

**UNITED STATES
SECURITIES AND EXCHANGE COMMISSION
WASHINGTON, D.C. 20549**

FORM 10-Q

(Mark One)

QUARTERLY REPORT PURSUANT TO SECTION 13 OR 15(d) OF THE SECURITIES EXCHANGE ACT OF 1934
For the quarterly period ended September 30, 2025

OR

TRANSITION REPORT PURSUANT TO SECTION 13 OR 15(d) OF THE SECURITIES EXCHANGE ACT OF 1934
For the transition period from _____ to _____
Commission File Number: 001-40431

DAY ONE BIOPHARMACEUTICALS, INC.

(Exact Name of Registrant as Specified in its Charter)

Delaware
(State or other jurisdiction of
incorporation or organization)

1800 Sierra Point Parkway, Suite 200
Brisbane, CA
(Address of principal executive offices)

83-2415215
(I.R.S. Employer
Identification No.)

94005

(Zip Code)

(650) 484-0899

(Registrant's telephone number, including area code)

N/A

(Former name, former address and former fiscal year, if changed since last report)

Securities registered pursuant to Section 12(b) of the Act:

Title of each class	Trading Symbol	Name of each exchange on which registered
Common Stock, par value \$0.0001 per share	DAWN	Nasdaq Global Select Market

Indicate by check mark whether the registrant (1) has filed all reports required to be filed by Section 13 or 15(d) of the Securities Exchange Act of 1934 during the preceding 12 months (or for such shorter period that the registrant was required to file such reports), and (2) has been subject to such filing requirements for the past 90 days. Yes No

Indicate by check mark whether the registrant has submitted electronically every Interactive Data File required to be submitted pursuant to Rule 405 of Regulation S-T (§232.405 of this chapter) during the preceding 12 months (or for such shorter period that the registrant was required to submit such files). Yes No

Indicate by check mark whether the registrant is a large accelerated filer, an accelerated filer, a non-accelerated filer, a smaller reporting company, or an emerging growth company. See the definitions of "large accelerated filer," "accelerated filer," "smaller reporting company," and "emerging growth company" in Rule 12b-2 of the Exchange Act.

Large accelerated filer	<input checked="" type="checkbox"/>	Accelerated filer	<input type="checkbox"/>
Non-accelerated filer	<input type="checkbox"/>	Smaller reporting company	<input type="checkbox"/>
		Emerging growth company	<input type="checkbox"/>

If an emerging growth company, indicate by check mark if the registrant has elected not to use the extended transition period for complying with any new or revised financial accounting standards provided pursuant to Section 13(a) of the Exchange Act.

Indicate by check mark whether the registrant is a shell company (as defined in Rule 12b-2 of the Exchange Act). Yes No

As of October 31, 2025, the registrant had 102,675,502 shares of common stock, \$0.0001 par value per share, outstanding.

CAUTIONARY NOTE REGARDING FORWARD-LOOKING STATEMENTS

This Quarterly Report on Form 10-Q contains forward-looking statements within the meaning of Section 21E of the Securities Exchange Act of 1934, as amended, or the Exchange Act, and section 27A of the Securities Act of 1933, as amended, or the Securities Act. All statements contained in this Quarterly Report other than statements of historical fact, including statements regarding our future results of operations and financial position, business strategy, market size, potential growth opportunities, nonclinical and clinical development activities, efficacy and safety profile of OJEMDA™ (tovorafenib) and our product candidates, potential therapeutic benefits and economic value of OJEMDA and our product candidates, our ability to market and sell OJEMDA while maintaining full compliance with applicable federal and state laws, rules and regulations, use of net proceeds from our public offerings, our ability to maintain and recognize the benefits of certain designations received by products and product candidates, the timing and results of nonclinical studies and clinical trials, commercial collaboration with third parties, and our ability to recognize milestone and royalty payments from commercialization agreements, the potential impact of global business or macroeconomic conditions, including as a result of inflation, changing interest rates, government shutdowns, cybersecurity incidents, significant political or regulatory developments or changes in tax or trade policy, including tariffs, shifting priorities within the U.S. Food and Drug Administration and reduced funding to federal healthcare programs, and global regional conflicts on our operations and the receipt and timing of potential regulatory designations, approvals and commercialization of product candidates, are forward-looking statements. The words “believe,” “may,” “will,” “potentially,” “estimate,” “continue,” “anticipate,” “predict,” “target,” “intend,” “could,” “would,” “should,” “project,” “plan,” “expect,” and other similar expressions that convey uncertainty of future events or outcomes are intended to identify forward-looking statements, although not all forward-looking statements contain these identifying words.

These forward-looking statements are subject to a number of risks, uncertainties and assumptions, including those described in Part II, Item 1A, “Risk Factors” and elsewhere in this Quarterly Report. Moreover, we operate in a very competitive and rapidly changing environment, and new risks emerge from time to time. It is not possible for our management to predict all risks, nor can we assess the impact of all factors on our business or the extent to which any factor, or combination of factors, may cause actual results to differ materially from those contained in any forward-looking statements we may make. In light of these risks, uncertainties and assumptions, the forward-looking events and circumstances discussed in this Quarterly Report may not occur and actual results could differ materially and adversely from those anticipated or implied in the forward-looking statements.

You should not rely upon forward-looking statements as predictions of future events. Although we believe that the expectations reflected in the forward-looking statements are reasonable, we cannot guarantee that the future results, levels of activity, performance or events and circumstances reflected in the forward-looking statements will be achieved or occur. We undertake no obligation to update publicly any forward-looking statements for any reason after the date of this report to conform these statements to actual results or to changes in our expectations, except as required by law. You should read this Quarterly Report with the understanding that our actual future results, levels of activity, performance and events and circumstances may be materially different from what we expect.

Unless the context indicates otherwise, as used in this Quarterly Report on Form 10-Q, the terms “Day One,” “the Company,” “we,” “us,” and “our” refer to Day One Biopharmaceuticals, Inc., a Delaware corporation unless otherwise noted. “Day One” and all product and product candidate names are our common law trademarks. This Quarterly Report contains additional trade names, trademarks and service marks of other companies, which are the property of their respective owners. We do not intend our use or display of other companies’ trade names, trademarks or service marks to imply a relationship with, or endorsement or sponsorship of us by, these other companies.

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PART I-FINANCIAL INFORMATION

Day One Biopharmaceuticals, Inc.
Condensed Balance Sheets
(in thousands, except share amounts)
(unaudited)

	September 30,		December 31,
	2025		2024
Assets			
Current assets:			
Cash and cash equivalents	\$ 43,277	\$	124,968
Short-term investments	408,303		406,752
Accounts receivable, net	16,697		13,876
Inventory	7,061		3,321
Prepaid expenses and other current assets	14,633		13,413
Total current assets	<u>489,971</u>		<u>562,330</u>
Property and equipment, net	2,227		2,285
Operating lease right-of-use asset	2,450		2,422
Intangible assets, net	18,815		15,630
Deposits and other long-term assets	317		121
Total assets	<u>\$ 513,780</u>	\$	<u>\$ 582,788</u>
Liabilities and stockholders' equity			
Current liabilities:			
Accounts payable	\$ 5,778	\$	3,262
Accrued expenses and other current liabilities	48,210		68,625
Current portion of deferred revenue	2,317		1,554
Current portion of operating lease liabilities	161		10
Total current liabilities	<u>56,466</u>		<u>73,451</u>
Long-term portion of deferred revenue	2,953		3,233
Long-term portion of operating lease liabilities	2,732		2,592
Other long-term liability	761		761
Total liabilities	<u>62,912</u>		<u>80,037</u>
Commitments and contingencies (Note 8)			
Stockholders' equity:			
Common stock, \$0.0001 par value; 500,000,000 shares authorized as of September 30, 2025 and December 31, 2024; 102,675,502 and 101,116,162 shares issued and outstanding as of September 30, 2025 and December 31, 2024, respectively	10		10
Additional paid-in-capital	1,090,914		1,056,738
Accumulated other comprehensive income	69		84
Accumulated deficit	(640,125)		(554,081)
Total stockholders' equity	<u>450,868</u>		<u>502,751</u>
Total liabilities and stockholders' equity	<u>\$ 513,780</u>	\$	<u>\$ 582,788</u>

See accompanying notes to the condensed financial statements.

Day One Biopharmaceuticals, Inc.
Condensed Statements of Operations
(in thousands, except share and per share amounts)
(unaudited)

	Three Months Ended September 30,		Nine Months Ended September 30,	
	2025	2024	2025	2024
Revenue:				
Product revenue, net	\$ 38,522	\$ 20,070	\$ 102,587	\$ 28,262
License revenue	1,273	73,691	1,877	73,691
Total revenues	39,795	93,761	104,464	101,953
Cost and operating expenses:				
Cost of product and license revenue	4,480	1,590	11,129	2,297
Research and development	31,419	33,563	107,187	165,879
Selling, general and administrative	28,147	28,972	86,440	85,715
Total cost and operating expenses	64,046	64,125	204,756	253,891
(Loss) income from operations	(24,251)	29,636	(100,292)	(151,938)
Non-operating income:				
Gain from sale of priority review voucher	—	—	—	108,000
Investment income, net	4,559	5,322	14,324	13,649
Other (expense) income, net	(34)	1,197	(76)	1,177
Total non-operating income, net	4,525	6,519	14,248	122,826
(Loss) income before income taxes	(19,726)	36,155	(86,044)	(29,112)
Income tax benefit (expense)	—	882	—	(670)
Net (loss) income	(19,726)	37,037	(86,044)	(29,782)
Net (loss) income per share - basic	\$ (0.19)	\$ 0.38	\$ (0.83)	\$ (0.33)
Net (loss) income per share - diluted	\$ (0.19)	\$ 0.38	\$ (0.83)	\$ (0.33)
Weighted-average number of common shares used in net (loss) income per share - basic	103,381,315	96,623,123	103,056,046	90,164,895
Weighted-average number of common shares used in net (loss) income per share - diluted	103,381,315	96,937,759	103,056,046	90,164,895

See accompanying notes to the condensed financial statements.

Day One Biopharmaceuticals, Inc.
Condensed Statements of Comprehensive Loss
(in thousands)
(unaudited)

	Three Months Ended September 30,		Nine Months Ended September 30,	
	2025	2024	2025	2024
Net (loss) income	\$ (19,726)	\$ 37,037	\$ (86,044)	\$ (29,782)
Other comprehensive income (loss):				
Unrealized gain (loss) on available-for-sale securities	161	16	(15)	(15)
Total comprehensive (loss) income	\$ (19,565)	\$ 37,053	\$ (86,059)	\$ (29,797)

See accompanying notes to the condensed financial statements.

Day One Biopharmaceuticals, Inc.
Condensed Statements of Stockholders' Equity
(in thousands, except share amounts)
(unaudited)

	Common Shares		Additional Paid-In Capital	Accumulated Other Comprehensive Income (Loss)	Accumulated Deficit	Total Stockholders' Equity
	Shares	Amount				
Balance at December 31, 2024	101,116,162	\$ 10	\$ 1,056,738	\$ 84	\$ (554,081)	\$ 502,751
Issuance of common stock upon release of restricted stock units	238,354	—	—	—	—	—
Unvested common stock forfeiture	(735)	—	—	—	—	—
Share-based compensation expenses	—	—	12,893	—	—	12,893
Unrealized loss on available-for-sale securities	—	—	—	(136)	—	(136)
Net loss	—	—	—	—	(35,996)	(35,996)
Balance at March 31, 2025	101,353,781	10	1,069,631	(52)	(590,077)	479,512
Issuance of common stock upon release of restricted stock units	246,250	—	—	—	—	—
Issuance of common stock pursuant to Employee Stock Purchase Plan	142,258	—	748	—	—	748
Issuance of common stock upon exercise of warrants	689,644	—	—	—	—	—
Share-based compensation expenses	—	—	10,936	—	—	10,936
Unrealized loss on available-for-sale securities	—	—	—	(40)	—	(40)
Net loss	—	—	—	—	(30,322)	(30,322)
Balance at June 30, 2025	102,431,933	10	1,081,315	(92)	(620,399)	460,834
Issuance of common stock upon release of restricted stock units	243,569	—	—	—	—	—
Share-based compensation expenses	—	—	9,599	—	—	9,599
Unrealized gain on available-for-sale securities	—	—	—	161	—	161
Net loss	—	—	—	—	(19,726)	(19,726)
Balance at September 30, 2025	102,675,502	\$ 10	\$ 1,090,914	\$ 69	\$ (640,125)	\$ 450,868

See accompanying notes to the condensed financial statements.

Day One Biopharmaceuticals, Inc.
Condensed Statements of Stockholders' Equity
(in thousands, except share amounts)
(unaudited)

	Common Shares		Additional Paid-In Capital	Accumulated Other Comprehensive Income (Loss)	Accumulated Deficit	Total Stockholders' Equity
	Shares	Amount				
Balance at December 31, 2023	87,227,132	\$ 9	\$ 805,107	\$ 9	\$ (458,585)	\$ 346,540
Issuance of common stock upon exercise of stock options	4,862	—	48	—	—	48
Issuance of common stock upon release of restricted stock units	157,724	—	—	—	—	—
Unvested common stock forfeiture	(12,555)	—	—	—	—	—
Share-based compensation expenses	—	—	12,644	—	—	12,644
Unrealized loss on available-for-sale securities	—	—	—	(14)	—	(14)
Net loss	—	—	—	—	(62,412)	(62,412)
Balance at March 31, 2024	87,377,163	9	817,799	(5)	(520,997)	296,806
Issuance of common stock upon exercise of stock options	22,151	—	324	—	—	324
Issuance of common stock upon release of restricted stock units	211,635	—	—	—	—	—
Issuance of common stock pursuant to Employee Stock Purchase Plan	94,827	—	973	—	—	973
Unvested common stock forfeiture	(12,860)	—	—	—	—	—
Share-based compensation expenses	—	—	13,052	—	—	13,052
Unrealized loss on available-for-sale securities	—	—	—	(17)	—	(17)
Net loss	—	—	—	—	(4,407)	(4,407)
Balance at June 30, 2024	87,692,916	9	832,148	(22)	(525,404)	306,731
Issuance of common stock upon exercise of stock options	67,540	—	977	—	—	977
Issuance of common stock upon release of restricted stock units	156,688	—	—	—	—	—
Issuance of common stock in connection with private placement, net of placement agent fees and offering costs	12,893,213	1	178,176	—	—	178,177
Issuance of prefunded warrants to purchase common stock in connection with private placement, net of issuance costs	—	—	20,941	—	—	20,941
Share-based compensation expenses	—	—	11,584	—	—	11,584
Unrealized gain on available-for-sale securities	—	—	—	16	—	16
Net income	—	—	—	—	37,037	37,037
Balance at September 30, 2024	100,810,357	\$ 10	\$ 1,043,826	\$ (6)	\$ (488,367)	\$ 555,463

See accompanying notes to the condensed financial statements.

Day One Biopharmaceuticals, Inc.
Condensed Statements of Cash Flows
(in thousands)
(unaudited)

	Nine Months Ended September 30,	
	2025	2024
Cash flows from operating activities:		
Net loss	\$ (86,044)	\$ (29,782)
Adjustments to reconcile net loss to net cash used in operating activities:		
Acquired in-process research and development assets	—	55,000
Share-based compensation expense	33,320	37,228
Depreciation expense	275	54
Accretion of discounts on short-term investments, net	(12,523)	(3,566)
Amortization of intangible assets	2,315	884
Amortization of operating right-of-use asset	202	332
Loss from sale of property and equipment	71	—
Gain from sale of priority review voucher	—	(108,000)
Changes in operating assets and liabilities:		
Accounts receivable, net	(2,821)	(8,695)
Inventory	(3,632)	(2,683)
Prepaid expenses and other current assets	(1,227)	(2,326)
Deposits and other long-term assets	(196)	97
Accounts payable	2,516	441
Accrued expenses and other current liabilities	(22,415)	8,665
Deferred revenue	483	4,523
Operating lease liability	68	(277)
Net cash used in operating activities	(89,608)	(48,105)
Cash flows from investing activities:		
Cash paid for purchase of short-term investments	(637,863)	(383,395)
Proceeds from maturity of short-term investments	648,820	307,482
Proceeds from sale of short-term investments	—	79,409
Cash paid for acquired intangible assets	(3,500)	(17,100)
Proceeds from sale of priority review voucher	—	108,000
Cash paid for acquired in-process research and development assets	—	(55,000)
Cash paid for purchase of property and equipment	(320)	(750)
Proceeds from sale of property and equipment	32	—
Net cash provided by investing activities	7,169	38,646
Cash flows from financing activities:		
Proceeds from issuance of common stock in connection with private placement, net of placement agent fees and offering costs	—	178,177
Proceeds from issuance of prefunded warrants to purchase common stock in connection with private placement, net of issuance costs	—	20,941
Proceeds from issuance of common stock upon stock option exercises	—	1,349
Proceeds from issuance of common stock upon Employee Stock Purchase Plan purchase	748	973
Cash provided by financing activities	748	201,440
Net (decrease) increase in cash and cash equivalents	(81,691)	191,981
Cash and cash equivalents, beginning of period	124,968	230,784
Cash and cash equivalents, end of period	\$ 43,277	\$ 422,765
Supplemental disclosure of cash flow information:		
Income taxes paid	5,316	883
Supplemental disclosures of noncash activities:		
Cash not yet paid for acquired intangible asset	2,000	—
Right-of-use asset obtained in exchange for new operating lease liabilities	230	2,554
Deferred offering costs not yet paid	—	66

See accompanying notes to the condensed financial statements.

Day One Biopharmaceuticals, Inc.
Notes to the Condensed Financial Statements

1. Description of Business and Organization

Organization and Business

Day One Biopharmaceuticals, Inc. is a biopharmaceutical company dedicated to developing and commercializing targeted therapies for people of all ages with life-threatening diseases. The Company was founded in November 2018 and is headquartered in Brisbane, CA.

2. Summary of Significant Accounting Policies

Basis of Presentation

The Company's unaudited condensed financial statements have been prepared in accordance with accounting principles generally accepted in the United States, or U.S. GAAP, for interim financial information and Article 10 of Regulation S-X of the Securities and Exchange Commission, or SEC, and should be read in conjunction with the Company's financial statements and notes thereto contained in the Company's Annual Report on Form 10-K for the year ended December 31, 2024, filed with the SEC on February 25, 2025. The condensed financial statements presented in this Quarterly Report on Form 10-Q are unaudited; however, in the opinion of management, such financial statements reflect all adjustments, consisting solely of normal recurring adjustments, necessary for a fair statement of the results for the interim periods presented.

Any reference in these notes to applicable guidance is meant to refer to the authoritative GAAP as found in Accounting Standards Codification, or ASC, and Accounting Standards Updates, or ASU, of the Financial Accounting Standards Board, or FASB.

Certain prior period amounts in the Notes to the Condensed Financial Statements have been reclassified to conform to the current period's presentation. These reclassifications did not affect the prior period's total assets, liabilities, stockholders' equity, net loss or cash flows. Specifically, the reclassifications separately present accrued rebates, accrued royalties and accrued federal and state taxes within Note 5.

Use of Estimates

The preparation of condensed financial statements in conformity with U.S. GAAP requires management to make estimates and assumptions that affect the reported amounts of assets, liabilities and disclosure of contingent assets and liabilities at the date of the condensed financial statements, and the reported amounts of expenses during the reporting period. Estimates and assumptions made in the accompanying condensed financial statements include, but are not limited to, accruals for research and development expenses, variable consideration and other relevant inputs impacting the gross and net revenue recognition, the valuation of share-based awards, and the valuation of deferred tax assets. The Company bases its estimates on historical experience and on various other assumptions that are believed to be reasonable. Actual results may differ from those estimates or assumptions.

Segments

Operating segments are defined as components of an entity about which separate discrete information is available for evaluation by the chief operating decision maker, or decision-making group, in deciding how to allocate resources and in assessing performance. The Company views its operations and manages its business in one operating segment.

Concentration of Credit Risk and Other Risks and Uncertainties

Financial instruments that subject the Company to significant concentrations of credit risk consist primarily of cash, cash equivalents, short-term investments, and accounts receivable. Amounts on deposit may at times exceed federally insured limits. The Company is exposed to credit risk in the event of default by the financial institutions holding its cash, cash equivalents and short-term investments that are recorded on its balance sheet. Per policy, the Company mitigates its risk by investing in high-grade instruments and limiting the concentration in any one non-United States government or government backed issuer, which limits its exposure. The Company has not experienced any losses on its cash, cash equivalents and short-term investments.

For the three months ended September 30, 2025, two individual customers accounted for 98.6% of total net product revenue, with these individual customers representing 74.0% and 24.6% of the Company's total net product revenue. For the nine months ended September 30, 2025, two individual customers accounted for 98.3% of total net product revenue, with these individual customers representing 66.4% and 31.9% of the Company's total net product revenue. As of September 30, 2025, two customers accounted for 91.7% of the accounts receivable balance, with these individual customers representing 68.5% and 23.2% of the accounts receivable balance. No other individual customers account for more than 10.0% of net product revenue or accounts receivable. The Company monitors the financial condition of its customers so that it can appropriately respond to

changes in their creditworthiness. To date, the Company has not experienced any losses with respect to the collection of its accounts receivable.

The Company is subject to certain risks and uncertainties and believes that changes in any of the following areas could have a material adverse effect on the Company's future financial position or results of its operations: ability to obtain future financing; regulatory requirements for approval and market acceptance of, and reimbursement for, product candidates; performance of third-party clinical research organizations and manufacturers upon which the Company relies; development of sales channels; protection of the Company's intellectual property; litigation or claims against the Company based on intellectual property, patent, product, regulatory or other factors; changes to the market landscape; and the Company's ability to attract and retain employees necessary to support its growth.

The Company is dependent on third-party manufacturers to supply products for commercial and research and development activities in its programs. In particular, the Company relies and expects to continue to rely on a small number of manufacturers to supply it with its requirements for the active pharmaceutical ingredients and formulated drugs related to these programs. These programs could be adversely affected by a significant interruption in the supply of active pharmaceutical ingredients and formulated drugs.

Recently Issued Accounting Pronouncements

In December 2023, the FASB issued ASU No. 2023-09, Income Taxes (Topic 740) – Improvements to Income Tax Disclosures, which enhances the transparency and decision usefulness of income tax disclosures by requiring disclosure of disaggregated income taxes paid, prescribes standard categories for the components of the effective tax rate reconciliation, and modifies other income tax-related disclosures. The ASU is effective for fiscal years beginning after December 15, 2024 and allows for adoption on a prospective basis, with a retrospective option. The Company is currently evaluating the effect of this update on its financial statements.

In November 2024, the FASB issued ASU No. 2024-03, Income Statement—Reporting Comprehensive Income—Expense Disaggregation Disclosures, which requires disclosure, in the notes to financial statements, of specified information about certain costs and expenses at each interim and annual reporting period. The ASU is effective for fiscal years beginning after December 15, 2026 and allows for adoption on a prospective basis, with a retrospective option. Early adoption is permitted. The Company is currently evaluating the effect of this update on its financial statements.

3. Recurring Fair Value Measurements

The following table sets forth the Company's financial instruments as of September 30, 2025 and December 31, 2024, which are measured at fair value on a recurring basis by level within the fair value hierarchy (in thousands):

	September 30, 2025			
	Level 1	Level 2	Level 3	Total
Financial assets:				
Money market funds	\$ 17,686	\$ —	\$ —	\$ 17,686
U.S. treasury securities	—	199,768	—	199,768
U.S. government agency securities	—	208,535	—	208,535
Total assets measured at fair value	\$ 17,686	\$ 408,303	\$ —	\$ 425,989
	December 31, 2024			
	Level 1	Level 2	Level 3	Total
Financial assets:				
Money market funds	\$ 16,728	\$ —	\$ —	\$ 16,728
U.S. treasury securities	—	311,487	—	311,487
U.S. government agency securities	—	183,375	—	183,375
Total assets measured at fair value	\$ 16,728	\$ 494,862	\$ —	\$ 511,590

The Company's money market funds are classified as Level 1 because they are measured using observable inputs from active markets for identical assets.

The Company's U.S. treasury securities and U.S. government agency securities are classified as Level 2 because they are measured with inputs that are either directly or indirectly observable for the asset which include quoted prices for similar assets in active markets and quoted prices for identical or similar assets in markets that are not active.

There were no assets or liabilities classified as Level 3 as of September 30, 2025 and December 31, 2024.

There were no transfers between Level 1, Level 2 or Level 3 categories during the periods presented.

The following tables summarize the estimated fair value of the Company's cash equivalents, available-for-sale securities classified as short-term investments, and associated unrealized gains and losses (in thousands):

	September 30, 2025			
	Amortized Cost	Unrealized Gains	Unrealized Losses	Estimated Fair Value
Cash equivalents:				
Money market funds	\$ 17,686	\$ —	\$ —	\$ 17,686
U.S. government agency securities	—	—	—	—
Total cash equivalents	17,686	—	—	17,686
Short-term investments				
U.S. government agency securities	208,516	41	(22)	208,535
U.S. treasury securities	199,717	61	(10)	199,768
Total short-term investments	\$ 408,233	\$ 102	\$ (32)	\$ 408,303

	December 31, 2024			
	Amortized Cost	Unrealized Gains	Unrealized Losses	Estimated Fair Value
Cash equivalents:				
Money market funds	\$ 16,728	\$ —	\$ —	\$ 16,728
U.S. government agency securities	75,163	—	—	75,163
U.S. treasury securities	12,947	—	—	12,947
Total cash equivalents	104,838	—	—	104,838
Short-term investments				
U.S. government agency securities	108,210	13	(11)	108,212
U.S. treasury securities	298,457	85	(2)	298,540
Total short-term investments	\$ 406,667	\$ 98	\$ (13)	\$ 406,752

The following table summarizes the maturities of our cash equivalents and available-for-sale securities (in thousands):

	September 30, 2025	
	Amortized Cost	Fair Value
Mature in one year or less	\$ 425,919	\$ 425,989
Total	\$ 425,919	\$ 425,989

	December 31, 2024	
	Amortized Cost	Fair Value
Mature in one year or less	\$ 511,505	\$ 511,590
Total	\$ 511,505	\$ 511,590

The Company regularly reviews the changes to the rating of its securities and monitors the surrounding economic conditions to assess the risk of expected credit losses. As of September 30, 2025 and December 31, 2024, there were no securities that were in an unrealized loss position for more than 12 months. As of September 30, 2025, the unrealized losses on the Company's short-term investments were primarily caused by interest rate changes. The Company does not expect the issuers to settle any security at a price less than the amortized cost basis of the investment with the contractual cash flows of these investments guaranteed by the issuer. No allowance for credit losses has been recorded since it is not more-likely-than-not that the Company will be required to sell the investments before recovery of their amortized cost basis.

4. Inventory

Inventory consisted of the following (in thousands):

	September 30, 2025	December 31, 2024
Raw materials	\$ —	\$ —
Work-in-process	6,005	2,448
Finished goods	1,056	873
Total inventory	\$ 7,061	\$ 3,321

The Company began capitalizing inventory for OJEMDA upon approval by the U.S. Food and Drug Administration, or FDA, in April 2024. Prior to regulatory approval, all direct and indirect manufacturing costs were charged to research and development expense in the period incurred.

Inventory is comprised of work-in-process and finished goods, and includes costs related to third-party contract manufacturing, packaging, freight-in and overhead. Inventory is stated at the lower of cost or net realizable value with cost based on the first-in-first-out method. Raw and intermediate materials that may be used for either research and development or commercial purposes where the intended use is not yet known are classified as inventory until the material is consumed or otherwise allocated for research and development. If the material is used or otherwise allocated for research and development, it is expensed as research and development in the period that determination is made.

The Company performs an assessment of the recoverability of capitalized inventory during each reporting period, and it writes down any excess and obsolete inventories to their estimated realizable value in the period in which the impairment is first identified. Such impairment charges, should they occur, are recorded within cost of product and license revenue. The determination of whether inventory costs will be realizable requires estimates by management. If actual market conditions are less favorable than projected by management, additional write-downs of inventory may be required, which would be recorded as a cost of product and license revenue in the statements of operations. There were no expenses recorded for excess inventory or other impairments during the three and nine months ended September 30, 2025 and 2024.

5. Balance Sheet Items

Prepaid Expenses and Other Current Assets

Prepaid expenses and other current assets consisted of the following (in thousands):

	September 30, 2025	December 31, 2024
Prepaid research and development expenses	\$ 9,816	\$ 8,216
Prepaid insurance	1,285	830
Other prepaid expenses and other assets	3,532	4,367
Total prepaid expenses and other current assets	<u>\$ 14,633</u>	<u>\$ 13,413</u>

Accrued Expenses and Other Current Liabilities

Accrued expenses and other current liabilities consisted of the following (in thousands):

	September 30, 2025	December 31, 2024
Accrued research and development expenses	\$ 19,832	\$ 18,760
Accrued payroll related expenses	8,972	13,943
Accrued rebates	8,298	4,518
Accrued professional service expenses	5,794	3,758
Accrued milestone payment	—	20,000
Accrued royalties	1,921	1,443
Accrued federal and state payables	—	4,966
Other accrued expenses and other current liabilities	3,393	1,237
Total accrued expenses and other current liabilities	<u>\$ 48,210</u>	<u>\$ 68,625</u>

6. Intangible Assets

Intangible assets, net consisted of the following:

	September 30, 2025	December 31, 2024
Intangible assets with finite lives:		
License agreement milestone payments	\$ 14,500	\$ 9,000
PRV-related obligation payment	8,100	8,100
Intangible assets, gross	22,600	17,100
Less accumulated amortization	(3,785)	(1,470)
Intangible assets, net	<u>\$ 18,815</u>	<u>\$ 15,630</u>

The Company incurred amortization expense of \$0.8 million and \$2.3 million for the three and nine months ended September 30, 2025, respectively. The Company incurred amortization expense of \$0.6 million and \$0.9 million for the three and nine months ended September 30, 2024.

The estimated aggregate amortization expense for amortizable, finite-lived intangible assets as of September 30, 2025 is as follows:

Year Ending December 31,	Estimated Amortization Expense
2025 (Remaining)	\$ 795
2026	3,180
2027	3,180
2028	3,180
2029	3,180
Thereafter	5,300
Total future expected amortization expense	<u>\$ 18,815</u>

7. Significant Agreements

Takeda asset purchase agreement

On December 16, 2019, a subsidiary of the Company entered into an asset purchase agreement, or the Takeda Asset Agreement, with Millennium Pharmaceuticals, Inc., a related party and an affiliate of Takeda Pharmaceutical Company Limited, or Takeda. Effective December 31, 2021, the subsidiary was merged with and into the Company, with the Company being the surviving corporation and assuming the subsidiary's obligations under the Takeda Asset Agreement. Pursuant to the Takeda Asset Agreement, the Company purchased certain technology rights and know-how related to TAK-580 (which is now OJEMDA™ (tovorafenib)) that provides a new approach for treating patients with primary brain tumors or brain metastases of solid tumors. The Company also received clinical inventory supplies to use in the Company's research and development activities of such RAF-inhibitor and an assigned investigator clinical trial agreement. Takeda also assigned to the Company its exclusive license agreement, or the Viracta License Agreement, with Viracta. Takeda also granted the Company a worldwide, sublicensable exclusive license under specified patents and know-how and non-exclusive license under other patents and know-how generated by Takeda under the Takeda Asset Agreement. The Company also granted Takeda a grant back license, as defined in the Takeda Asset Agreement, which is terminable either automatically or by the Company in the event Takeda does not achieve specified development milestones within the applicable timeframes set forth under the Takeda Asset Agreement. This grant back license to Takeda was terminated at the time of conversion of the Company from an LLC to a corporation in connection with the Millennium Stock Exchange Agreement.

The term of the Takeda Asset Agreement will expire on a country-by-country basis upon expiration of all assigned patent rights and all licensed patent rights in such country. Takeda can terminate the Takeda Asset Agreement in the event of the Company's bankruptcy. In the event of termination of the Takeda Asset Agreement by Takeda as a result of the Company's cessation of development or bankruptcy, all assigned patents, know-how and contracts (other than the Viracta License Agreement) will be assigned back to Takeda and Takeda will obtain a reversion license under patents and know-how generated to exploit all such terminated products.

In consideration for the sale and assignment of assets and the grant of the license under the Takeda Asset Agreement, the Company made an upfront payment of \$1.0 million in cash and issued 9,857,143 shares of Series A redeemable convertible preferred stock in the Company's subsidiary in December 2019. The fair value of issued shares was estimated as \$9.9 million, based on the price paid by other investors for issued shares in the Series A financing of the Company's subsidiary. Based on the terms of the Millennium Stock Exchange Agreement, Takeda exchanged the 9,857,143 shares of Series A redeemable convertible preferred stock of the Company's subsidiary for 6,470,382 shares of the Company's common stock upon the effectiveness of the conversion of the Company from an LLC to a corporation, on May 26, 2021.

License agreement with Viracta

On December 16, 2019, a subsidiary of the Company amended and restated the Viracta License Agreement that was assigned pursuant to the Takeda Asset Agreement. Effective December 31, 2021, the subsidiary was merged with and into the Company, with the Company being the surviving corporation and assuming the subsidiary's obligations under Viracta License Agreement. Under the Viracta License Agreement, the Company received a worldwide exclusive license under specified patent rights and know-how to develop, use, manufacture, and commercialize products containing compounds binding the RAF protein family.

The term of the Viracta License Agreement will expire on a licensed product-by-licensed product and country-by-country basis upon the expiration of the Company's obligation to pay royalties to Viracta with respect to such product in such country. The Company has the right to terminate the Viracta License Agreement with respect to any or all of the licensed products at will upon a specified notice period.

The Company paid \$2.0 million upfront in cash to Viracta in December 2019, which was recorded as research and development expenses as the technology does not have an alternative future use.

On March 4, 2024, the Company entered into an amendment to the Viracta License Agreement. As part of the amendment, the Company made a one-time payment in March 2024 to Viracta of \$5.0 million, which was recorded as research and development expenses during the year ended December 31, 2024, in exchange for reduced future payment obligations ranging from the mid-teens to the high single-digit percentage related to the future sale or use of the rare pediatric disease priority review voucher, or PRV, received.

On April 23, 2024, the FDA approved OJEMDA (a tablet formulation and powder solution formulation of tovorafenib) for the treatment of patients 6 months of age and older with relapsed or refractory pLGG harboring a BRAF fusion or rearrangement, or BRAF V600 mutation. The indication was approved under accelerated approval based on response rate and duration of response. With the approval, the Company received a rare pediatric disease PRV from the FDA. The Company made a \$9.0 million milestone payment to Viracta in May 2024 for the achievement of this milestone. The \$9.0 million milestone was accounted for as a finite-lived intangible asset and will be amortized over the life of the underlying asset. Related amortization expense will be recorded as cost of product and license revenue in the Company's statements of operations.

On May 29, 2024, the Company sold its rare pediatric disease PRV for \$108.0 million to an undisclosed buyer. As part of the transaction, \$8.1 million of the total consideration received from the sale of the rare pediatric disease PRV was paid to Viracta to fully satisfy PRV-related obligations under the Viracta License Agreement and was capitalized as a finite-lived intangible asset, which will be amortized on a straight-line basis over its estimated useful life. The gross proceeds of \$108.0 million were recorded as a gain from sale of priority review voucher in the accompanying statements of operations during the year ended December 31, 2024. As of September 30, 2025, the unamortized finite-lived intangible asset was \$6.6 million. Related amortization expense will be recorded as cost of product and license revenue in the Company's statements of operations.

On December 3, 2024, Viracta assigned the Viracta License Agreement to XOMA (US) LLC, or XOMA, pursuant to a Royalty Purchase Agreement dated March 22, 2021, between Viracta and XOMA, whereby Viracta sold its right, title, and interest in and to the Viracta License Agreement to XOMA. The Company has agreed to the assignment and novation of the Viracta License Agreement to XOMA as successor party, now referred to as the XOMA License Agreement. In connection with such assignment, the parties also agreed to assign all rights, title and interest in related intellectual property. No material terms of the XOMA License Agreement have been amended or modified in relation to the same.

On March 27, 2025, Ipsen Pharma SAS received validation of its completed submission of the Marketing Authorization Application to the European Medicines Agency, or EMA, for tovorafenib for the treatment of patients with relapsed or refractory pLGG. As a result, the Company paid \$4.0 million to XOMA for the achievement of this milestone in March 2025.

As of September 30, 2025, the Company could be required to make additional milestone payments of up to \$36.0 million upon achievement of specified development and regulatory milestones for each licensed product in two indications, with milestones payable for the second indication upon achievement of a specified milestone event being lower than milestones payable for the first indication. Commencing with the first commercial sale of OJEMDA in a country, the Company is obligated to pay tiered royalties ranging in the mid-single-digit percentages on net sales of licensed products. The obligation to pay royalties will end on a country-by-country and licensed product-by-licensed product basis commencing on the first commercial sale in a country and continuing until the later of: (i) the expiration of the last valid claim of the XOMA licensed patents, jointly owned collaboration patents or specified patents owned by the Company covering the use or sale of such product in such country, (ii) the expiration of the last statutory exclusivity pertaining to such product in such country or (iii) the tenth anniversary of the first commercial sale of such product in such country.

License agreement with Merck KGaA, Darmstadt, Germany

On February 10, 2021, a subsidiary of the Company entered into a license agreement, or the MRKDG License Agreement, with Merck KGaA, Darmstadt, Germany, a pharmaceutical corporation located in Darmstadt, Germany. Effective December 31, 2021, the subsidiary was merged with and into the Company, with the Company being the surviving corporation and assuming the subsidiary's obligations under the MRKDG License Agreement.

Under the MRKDG License Agreement, Merck KGaA, Darmstadt, Germany granted to the Company an exclusive worldwide license, with the right to grant sublicenses through multiple tiers, under specified patent rights and know-how for the Company to research, develop, manufacture and commercialize products containing and comprising the pimasertib and MSC2015103B compounds. The Company also received clinical inventory supplies to use in its research and development

activities. The Company's exclusive license grant is subject to a non-exclusive license granted by Merck KGaA, Darmstadt, Germany's affiliate to a cancer research organization and Merck KGaA, Darmstadt, Germany retains the right to conduct, directly or indirectly, certain ongoing clinical studies relating to pimasertib. Under the MRKDG License Agreement, the Company has obligations to use commercially reasonable efforts to develop and commercialize at least two licensed products in at least two specified major market countries by the year 2029.

The term of the MRKDG License Agreement will expire on a licensed product-by-licensed product and country-by-country basis upon the expiration of the Company's obligation to pay royalties to the licensor with respect to such licensed product in such country and will expire in its entirety upon the expiration of all of the Company's payment obligations with respect to all licensed products and all countries under the MRKDG License Agreement.

In consideration for the rights granted under the MRKDG License Agreement and clinical supplies, the Company made an upfront payment of \$8.0 million in March 2021, which was recorded as research and development expenses, as the technology does not have an alternative future use and supplies are used for research activities.

In November 2023, the Company discontinued its monotherapy substudy due to a limited duration of response in this rare patient population despite observing responses with a generally well tolerated therapy. In July 2024, the Company informed Merck KGaA, Darmstadt, Germany that the Company decided to close the program because the benefit/risk profile, as well as the market opportunity, did not justify the significant investment required to continue the trial despite observing some clinical responses. In September 2025, the Company finalized the termination agreement with Merck KGaA, Darmstadt, Germany and concluded material wind-down activities.

Research collaboration and license agreement with Sprint Bioscience AB

On August 15, 2023, the Company entered into a research collaboration and license agreement, or the Sprint License Agreement, with Sprint Bioscience AB, or Sprint, a Swedish corporation located in Huddinge, Sweden. Under the Sprint License Agreement, Sprint granted to the Company an exclusive, worldwide license, with the right to grant sublicenses through multiple tiers, to research, develop, and commercialize pharmaceutical products and to engage in research aimed at discovery, optimization and development of an inhibitor targeting Vaccinia Related Kinase 1, or VRK1. In consideration for the rights granted under the Sprint License Agreement, the Company paid \$3.0 million upfront in cash to Sprint in September 2023, which was recorded as research and development expenses as the technology does not have an alternative future use.

The term of the Sprint License Agreement will expire on a licensed product and country basis upon the expiration of the royalty term with respect to such licensed product and such country, unless terminated earlier. The Company has the right to terminate the Sprint License Agreement in its entirety, or on a licensed product-by-licensed product basis, at will upon a specified notice period.

On May 9, 2025, the Company notified Sprint of its decision to discontinue its investment in the VRK1 program and terminated the Sprint License Agreement, with termination taking effect on August 15, 2025. This decision was made following careful consideration of the current development status of the program and the Company's overall strategic objectives.

License agreement with MabCare Therapeutics

On June 17, 2024, the Company entered into a license agreement, or the MabCare License Agreement, with MabCare Therapeutics, or MabCare, a pharmaceutical corporation located in Shanghai, China. Under the MabCare License Agreement, MabCare granted to the Company an exclusive worldwide license, excluding Greater China, with the right to grant sublicenses through multiple tiers, under specified patent rights and know-how for the Company to develop, manufacture and commercialize DAY301 (formerly MTX-13), a novel Antibody Drug Conjugate, or ADC, targeting protein-tyrosine kinase 7, or PTK7. The Company will also receive clinical inventory supplies to use in its research and development activities. Under the MabCare License Agreement, the Company has obligations to use commercially reasonable efforts to develop, obtain regulatory approval for, and commercialize at least one licensed product in one indication in each of the United States, Japan, and three European countries.

The term of the MabCare License Agreement will expire in its entirety upon the expiration of the last to expire royalty term with respect to all licensed products in the Company's territory, unless terminated earlier. Following the expiration of the royalty term for a licensed product in a country, the license granted to the Company shall become non-exclusive, fully paid-up, royalty-free, perpetual, and irrevocable for such licensed product in such country. Upon the expiration of the term, the license granted to the Company shall become non-exclusive, transferable, sublicensable, fully paid, royalty free, perpetual, and irrevocable in its entirety.

In consideration for the rights granted under the MabCare License Agreement, the Company made a \$55.0 million upfront payment in July 2024. The upfront payment was recorded as research and development expenses, as the technology and supplies licensed do not have an alternative future use. As of September 30, 2025, the Company could be required to make additional payments of \$1,132.0 million based upon the achievement of specified development, regulatory, and commercial success-based milestones plus low-to-mid single-digit royalties on net sales outside of Greater China. Milestones and royalties are contingent

upon future events and will be recorded when the milestones are achieved and when payments are due. In January 2025, the Company cleared the first cohort (a single-patient accelerated titration cohort) in the Phase 1a portion of the DAY301 Phase 1a/b clinical trial. The Company made a \$20.0 million milestone payment to MabCare in January 2025 for the achievement of this milestone.

License agreement with Ipsen Pharma SAS

On July 23, 2024, the Company entered into the Ipsen License Agreement, pursuant to which, the Company licensed to Ipsen, on an exclusive basis, the right to commercialize tovorafenib in all territories outside the United States and agreed to provide certain research and development and manufacturing services. Ipsen shall have the right to grant sublicenses to third-parties.

Under the terms of the Ipsen License Agreement, (i) Ipsen paid the Company an upfront license fee in the amount of \$70.8 million in August 2024 and (ii) the Investor, a fully-owned United States affiliate of Ipsen, purchased 2,341,495 shares of the Company's common stock in a private placement for \$40.0 million, at a price per share representing a 17.0% premium to the VWAP of the Company's common stock as traded on The Nasdaq Stock Market LLC for the ten consecutive trading days prior to and including the date of the Revenue Release, and the ten consecutive trading days following the Revenue Release, in accordance with the terms set forth in an investment agreement by and between the Company and the Investor dated July 23, 2024.

As of September 30, 2025, the Company is also eligible to receive up to approximately \$380.0 million in additional commercial launch and sales-based milestone payments based on exchange rates as of the reporting date. The Company is also entitled to tiered, double-digit royalty payments starting at mid-teens percentage of annual net sales of tovorafenib, subject to customary adjustments specified in the Ipsen License Agreement. The royalty payment obligations under the Ipsen License Agreement expire on a country-by-country basis no earlier than ten years following the first commercial sale of tovorafenib in the applicable country.

In addition, the Ipsen License Agreement provides that the Company will supply to Ipsen, and Ipsen will purchase from the Company, all required quantities of tovorafenib for all territories outside the United States in accordance with a supply agreement to be entered into by and between the Company and Ipsen, or the Ipsen Supply Agreement. The Company determined that the cost-plus rate to be charged for the supply of tovorafenib does not represent a material right. Ipsen has the right to request a manufacturing technology transfer of the then-current manufacturing process of tovorafenib under the Ipsen License Agreement, and such consent shall not be unreasonably withheld, such that upon completion of the manufacturing technology transfer, Ipsen or a third-party would be solely responsible for the manufacture of tovorafenib for all territories outside the United States.

Following the two-year anniversary of July 23, 2024, the effective date of the Ipsen License Agreement, Ipsen may terminate the Ipsen License Agreement for convenience with six months' prior written notice or for certain other specified reasons. The Company may terminate the Ipsen License Agreement if Ipsen or any of its affiliates challenge the validity of any patents controlled by the Company that are licensed under the Ipsen License Agreement. Both Ipsen and the Company may terminate the Ipsen License Agreement (i) for material breach by the other party and a failure to cure such breach within the time period specified in the Ipsen License Agreement or (ii) the other party's bankruptcy event.

The Company evaluated the Ipsen License Agreement under Accounting Standards Codification, or ASC, 606 and concluded that Ipsen represents a customer in the transaction. The Company identified two distinct performance obligations for licenses to intellectual property in the form of the exclusive license to commercialize tovorafenib outside the United States for both (i) relapsed or refractory and (ii) front-line pLGG; and three distinct research and development performance obligations related to tovorafenib for completion of (i) the pivotal Phase 2 relapsed or refractory pLGG trial, or FIREFLY-1, (ii) the pivotal Phase 3 front-line pLGG trial, or FIREFLY-2, and (iii) the European Union, or EU, companion diagnostic for pLGG. Both the FIREFLY-1 and FIREFLY-2 trials related to pLGG pertain to later-stage intellectual property and only involve validating the efficacy of tovorafenib with respect to each distinct designation and are not expected to significantly modify or customize the licensed intellectual property. The FIREFLY-1 and FIREFLY-2 trials, and EU companion diagnostic research and development services related to pLGG could be performed by a third-party. The Company determined that the promise of the manufacturing technology transfer is a customer option that does not represent a material right given the value of the services is not material and fulfillment of this promise is ancillary to the main transaction. Accordingly, the manufacturing technology transfer is not a performance obligation at the outset of the arrangement.

Upon execution of the Ipsen License Agreement, the transaction price was determined to be \$78.2 million, representing the aggregate of the upfront license fee of \$70.8 million and the premium paid by Ipsen on its equity investment in the Company of \$7.4 million (the excess of the value of the shares of the Company issued to Ipsen), representing additional consideration from Ipsen for the rights under the Ipsen License Agreement. Commercial launch milestones related to first commercial sale(s) in certain territories, sales-based milestones and royalties on net sales upon commercialization by Ipsen were excluded from the transaction price and will be recognized when the related sales occur as they were determined to predominantly relate to the

intellectual property and, therefore, have been excluded from the transaction price in accordance with the sales-based royalty exception.

The Company allocated the transaction price to the performance obligations based on their relative standalone selling price. The Company developed the estimated stand-alone selling price for each license using discounted cash flow models. In developing this estimate, the Company applied judgment in the determination of the assumptions relating to forecasted future revenues, the discount rate, and the probability of success. The stand-alone selling price for each of the research and development services was estimated based on the Company's forecasted costs to be incurred to fulfill the obligations plus a reasonable margin. The portion of the transaction price allocable to the relapsed or refractory and front-line pLGG licenses to intellectual property was recognized as license revenue at the point in time in which Ipsen had the right to use the license/know-how, which occurred during the year ended December 31, 2024. The portion of the transaction price allocable to the relapsed or refractory, front-line and companion diagnostic research and development services performance obligations, as well as other services subsequently contracted by Ipsen with the Company on an ad-hoc basis that were not specifically identified as part of the Ipsen License Agreement and therefore considered separate performance obligations, will be recognized over time as the services are delivered based on costs incurred relative to the total estimated cost to deliver the services. During the three and nine months ended September 30, 2025, \$1.3 million and \$1.9 million of license revenue was recognized, respectively, with \$2.3 million and \$3.0 million of the undelivered relapsed or refractory, front-line and companion diagnostic research and development services included in current and non-current deferred revenue, respectively. During the three and nine months ended September 30, 2024, \$73.7 million of license revenue was recognized.

8. Commitments and Contingencies

Leases

In April 2022, the Company entered into a lease agreement for approximately 12,000 square feet of general use office space in Brisbane, California. The agreement was determined to be a lease since the right to control the use of the identified asset was conveyed to the Company for a period of time in exchange for consideration. The lease commenced in May 2022 and expired in November 2024. The Company was obligated to pay monthly rent expense and its pro rata share of the landlord's operating expenses which include utilities, common area maintenance expenses, and property taxes. Such expenses were a non-lease component and a variable consideration and included in the Company's operating expenses as incurred. The Company concluded that this lease was an operating lease.

In June 2024, the Company entered into a lease agreement for approximately 19,000 square feet of general use office space in Brisbane, California. The agreement was determined to be a lease since the right to control the use of the identified asset was conveyed to the Company for a period of time in exchange for consideration. The term of the lease is approximately 7.4 years and commenced in August 2024. There is no option to extend the lease term nor is there an option to terminate the lease term prior to its expiration. The Company is obligated to pay monthly rent expense and its pro rata share of the landlord's operating expenses which include utilities, common area maintenance expenses, and property taxes. Such expenses are a non-lease component and a variable consideration and included in the Company's operating expenses as incurred. The Company concluded that this lease is an operating lease. The total payments for base rent over the term of the lease is approximately \$4.4 million. Upon execution of the agreement, the Company paid a security deposit of approximately \$86,000 classified as deposits and other long-term assets on the condensed balance sheet.

The Company determined the lease incremental borrowing rate, or IBR, based on the information available at the applicable lease commencement date as the Company's leases do not provide an implicit rate. The IBR is determined by using the rate of interest that the Company would pay to borrow on a collateralized basis an amount equal to the lease payments for a similar term and in a similar economic environment where the asset is located. As of September 30, 2025, the weighted-average remaining lease term and weighted-average discount rate were 6.0 years and 12.9%, respectively.

The Company's leases do not require any contingent rental payments, impose financial restrictions, or contain any residual value guarantees.

Lease expense of right-of-use assets is recognized on a straight-line basis over the applicable lease term. Lease expense was \$0.1 million and \$0.2 million for the three months ended September 30, 2025 and 2024, respectively, and was \$0.5 million and \$0.4 million for the nine months ended September 30, 2025 and 2024, respectively. Cash paid for amounts included in the measurement of operating lease liabilities was \$0.2 million and \$0.4 million for the nine months ended September 30, 2025 and 2024, respectively. Variable payments expensed during the three and nine months ended September 30, 2025 and 2024 were immaterial.

As of September 30, 2025, the future lease obligations were as follows (in thousands):

Year Ending December 31,	
2025 (Remaining)	194
2026	527
2027	543
2028	492
2029	711
Thereafter	1,981
Total future minimum lease payments	4,448
Less: imputed interest	(1,555)
Present value of operating lease liabilities	2,893
Less: current portion of operating lease liabilities	(161)
Long-term portion of operating lease liabilities	<u>\$ 2,732</u>

Research and Development Agreements

The Company enters into contracts in the normal course of business with clinical research organizations, contract manufacturing organizations, and other third-party vendors for clinical trial, manufacturing, testing, and other research and development activities. These contracts generally provide for termination on notice, with the exception of one vendor where certain costs are non-cancellable after the approval of the project. As of September 30, 2025 and December 31, 2024, there were no amounts accrued related to termination and cancellation charges as these are not probable.

License Agreements

The Company entered into license agreements, as disclosed in Note 7, with various parties under which it is obligated to make contingent and non-contingent payments.

Purchase Commitments

To support product needs for OJEMDA, the Company entered into a manufacturing and supply agreement with Quotient Sciences - Philadelphia, LLC in July 2023 that requires the Company to meet minimum purchase obligations on an annual basis. The remaining amount of future minimum purchase obligations under the manufacturing and supply agreement over the next five years is approximately \$10.6 million, in aggregate, as of September 30, 2025. For the nine months ended September 30, 2025 and 2024, the Company made purchases of \$3.5 million and \$2.0 million, respectively, under the purchase obligation.

Legal Proceedings

The Company, from time to time, may be party to litigation, claims and assessments arising in the ordinary course of business. The Company accrues liabilities for such matters when it is probable that future expenditures will be made and such expenditures can be reasonably estimated. The Company is not subject to any material legal proceedings, and to the best of its knowledge, no material legal proceedings are currently pending or threatened.

Indemnification Agreements

In the normal course of business, the Company enters into contracts and agreements that contain a variety of representations and warranties and provide for indemnification for certain liabilities. The exposure under these agreements is unknown because it involves claims that may be made against it in the future but have not yet been made. To date, the Company has not paid any claims or been required to defend any action related to its indemnification obligations. However, the Company may record charges in the future as a result of these indemnification obligations. The Company also has indemnification obligations to its directors and executive officers for specified events or occurrences, subject to some limits, while they are serving at its request in such capacities. There have been no claims to date, and the Company believes the fair value of these indemnification agreements is minimal. Accordingly, the Company had not recorded any liabilities for these agreements as of September 30, 2025 and December 31, 2024.

9. Common Stock

Pursuant to its certificate of incorporation, the Company is authorized to issue 500.0 million shares of common stock at a par value of \$0.0001 per share. As of September 30, 2025, 102,675,502 shares of common stock were issued and outstanding.

The Company has reserved shares of common stock for future issuances as follows:

	September 30, 2025
Common stock options issued and outstanding	14,061,544
Common stock available for future grants	4,968,021
Common stock available for ESPP	3,305,854
Restricted stock units issued and outstanding	2,758,561
Pre-funded warrants	827,586
Total	25,921,566

2024 Private Placement

In July 2024, the Company entered into a securities purchase agreement with certain institutional and accredited investors, or the PIPE Investors, pursuant to which the Company agreed to sell and issue to the PIPE Investors in a private placement, or the Private Placement, an aggregate of (i) 10,551,718 shares of the Company's common stock, par value \$0.0001 per share, at a purchase price of \$14.50 per share and (ii) 1,517,241 pre-funded warrants, or the Pre-Funded Warrants, to purchase up to an aggregate of 1,517,241 shares of the Company's common stock, or the Warrant Shares, at a purchase price of \$14.4999 per Pre-Funded Warrant. Each Pre-Funded Warrant has an exercise price of \$0.0001 per Warrant Share.

The Pre-Funded Warrants are exercisable at any time after their original issuance at the option of each holder, in such holder's discretion, by (i) payment in full in immediately available funds for the number of shares of common stock purchased upon such exercise or (ii) a cashless exercise, in which case the holder would receive upon such exercise the net number of shares of common stock determined according to the formula set forth in the Pre-Funded Warrant. A holder will not be entitled to exercise any portion of any Pre-Funded Warrant if the holder's ownership of the Company's common stock would exceed 9.99% following such exercise.

In the event of certain fundamental transactions, the holders of the Pre-Funded Warrants will be entitled to receive upon exercise of the Pre-Funded Warrants the kind of amounts of securities, cash or other property that the holders would have received had they exercised the Pre-Funded Warrants immediately prior to such fundamental transaction without regard to any limitations on exercise contained in the Pre-Funded Warrants.

The Pre-Funded Warrants were classified as a component of permanent stockholders' equity within additional paid-in capital and were recorded at the issuance date using a relative fair value allocation method. The Pre-Funded Warrants are equity classified because they (i) are freestanding financial instruments that are legally detachable and separately exercisable from the equity instruments, (ii) are immediately exercisable, (iii) do not embody an obligation for the Company to repurchase its shares, (iv) permit the holders to receive a fixed number of shares of common stock upon exercise, (v) are indexed to the Company's common stock and (vi) meet the equity classification criteria. In addition, such Pre-Funded Warrants do not provide any guarantee of value or return. The Company valued the Pre-Funded Warrants at issuance, concluding that their sales price approximated their fair value, and allocated net proceeds from the Private Placement proportionately to the Company's common stock and Pre-Funded Warrants.

The Private Placement closed on August 1, 2024, and the Company received net proceeds of \$166.5 million, after deducting placement agent fees, offering costs, and other expenses, of which \$145.6 million was allocated to the common stock and \$20.9 million was allocated to the Pre-Funded Warrants. The net proceeds were recorded as a component of additional paid-in capital.

Investment agreement with Ipsen Biopharmaceuticals, Inc.

In July 2024, the Company entered into the Ipsen License Agreement, pursuant to which, the Company licensed to Ipsen, on an exclusive basis, the right to commercialize tovorafenib in all territories outside the United States and agreed to provide certain research and development and manufacturing services. Under the terms of the Ipsen License Agreement, (i) Ipsen paid the Company an upfront license fee in the amount of \$70.8 million in August 2024 and (ii) the Investor, a fully-owned United States affiliate of Ipsen, purchased 2,341,495 shares of the Company's common stock in a private placement for \$40.0 million, at a price per share representing a 17.0% premium to the VWAP of the Company's common stock as traded on The Nasdaq Stock Market LLC for the ten consecutive trading days prior to and including the date of the Revenue Release, and the ten consecutive trading days following the Revenue Release, in accordance with the terms set forth in an investment agreement by and between the Company and the Investor dated July 23, 2024. The Company valued the shares at issuance at \$32.6 million, concluding that the Company's common stock price as traded on The Nasdaq Stock Market LLC on the closing date of the transaction approximated fair value, which was recorded as a component of additional paid-in capital.

At-The-Market Offering

The Company has entered into an equity distribution agreement, or the Equity Distribution Agreement, with Piper Sandler & Co. and JonesTrading Institutional Services LLC, as sales agents, relating to the issuance and sale of shares of the Company's

common stock for an aggregate offering price of up to \$250.0 million under an at-the-market offering program, or the ATM. The Company has no obligation to sell any shares and could at any time suspend solicitations and offers under the ATM. No shares of the Company's common stock have been sold under the ATM as of September 30, 2025.

10. Share-based Compensation

Share-based compensation expense recorded in the accompanying condensed statements of operations is as follows (in thousands):

	Three Months Ended September 30,		Nine Months Ended September 30,	
	2025	2024	2025	2024
Research and development expense	\$ 2,991	\$ 3,816	\$ 10,830	\$ 13,178
Selling, general and administrative expense	6,570	7,738	22,490	24,050
Total share-based compensation expense	\$ 9,561	\$ 11,554	\$ 33,320	\$ 37,228

2022 Equity Inducement Plan

In October 2022, the board of directors approved the 2022 Equity Inducement Plan, or the 2022 Plan. The 2022 Plan provides for the grant of non-statutory stock options and restricted stock units. The number of shares of common stock reserved for issuance under the 2022 Plan is 1,000,000 shares.

2021 Equity Incentive Plan

In May 2021, in connection with the IPO, the board of directors and stockholders approved the 2021 Equity Incentive Plan, or the 2021 Plan, which became effective on the day before the date of the effectiveness of the IPO. The 2021 Plan provides for the grant of incentive stock options, non-statutory stock options, stock appreciation rights, awards of restricted stock, restricted stock units and other share-based awards. The number of shares of common stock reserved for issuance under the 2021 Plan is equal to the sum of: (x) 6,369,000 shares of common stock; plus (y) 4,719,605 shares of common stock issued in respect of the conversion of incentive shares that were subject to vesting immediately prior to the effectiveness of the registration statement for the IPO that expire, terminate or are otherwise surrendered, canceled, forfeited or repurchased by us at their original issuance price pursuant to a contractual repurchase right. The number of shares available for grant and issuance under the 2021 Plan will be automatically increased on the first day of each fiscal year, beginning with the fiscal year commencing on January 1, 2021 and continuing for each fiscal year until, and including, the fiscal year commencing on January 1, 2031, by the lesser of (a) 5% of the number of shares of all classes of the Company's common stock, plus the total number of shares of Company common stock issuable upon conversion of any preferred stock or exercise of any warrants to acquire shares of Company common stock for a nominal exercise price issued and outstanding on each December 31 immediately prior to the date of increase or (b) such number of shares determined by the board of directors.

Stock Options

The following table provides a summary of stock option activity during the nine months ended September 30, 2025.

	Options	Weighted-Average Exercise Price Per Share	Weighted-Average Remaining Contractual Term	Aggregate Intrinsic Value (in thousands)
Outstanding at December 31, 2024	12,127,435	\$ 16.50		
Granted	2,998,300	\$ 10.40		
Exercised	—	\$ —		\$ —
Forfeiture	(1,064,191)	\$ 15.92		
Outstanding at September 30, 2025	14,061,544	\$ 15.24	\$ 7.20	\$ 240
Vested and expected to vest at September 30, 2025	14,061,544	\$ 15.24	\$ 7.20	\$ 240
Exercisable at September 30, 2025	9,172,219	\$ 16.42	\$ 6.40	\$ 8

Aggregate intrinsic value represents the difference between the estimated fair value of the underlying common stock and the exercise price of outstanding, in-the-money options. There were no options exercised during the nine months ended September 30, 2025. The total intrinsic value of options exercised during the nine months ended September 30, 2024 was less than \$0.1 million.

The total fair value of options that vested during the nine months ended September 30, 2025 and 2024 was \$21.7 million and \$25.7 million, respectively. The weighted-average grant date fair value of options granted during the nine months ended September 30, 2025 and 2024 was \$6.81 per share and \$9.24 per share, respectively.

Unrecognized share-based compensation for stock options as of September 30, 2025 was \$40.3 million, which is expected to be recognized over a weighted-average period of 2.3 years.

The Company used the Black-Scholes option pricing model to estimate the fair value of stock option awards granted with the following assumptions:

	Three Months Ended September 30,		Nine Months Ended September 30,	
	2025	2024	2025	2024
Expected term (in years)	6.0 - 6.1	6.1	5.3 - 6.7	5.3 - 6.7
Expected volatility	69.44% - 74.96%	68.46% - 68.72%	68.30% - 75.07%	68.27% - 70.57%
Risk-free interest rate	3.77% - 4.05%	3.43% - 4.40%	3.77% - 4.48%	3.43% - 4.47%
Expected dividend yield	—	—	—	—

During the three and nine months ended September 30, 2025, the Company updated its methodology for estimating the expected volatility assumption used in the valuation of stock option awards to incorporate both the historical volatility of its common stock and the volatility of a group of publicly traded industry peers as the Company now has sufficient trading history for its common stock. This change provides a more representative measure of expected volatility by reflecting both Company-specific and market-based factors.

Restricted Stock Units

The following table provides a summary of restricted stock units activity under the 2021 Plan during the nine months ended September 30, 2025:

	Number of Shares	Weighted Average Grant Date Fair Value Per Share
Unvested restricted stock units at December 31, 2024	1,774,287	\$ 15.58
Granted	1,957,300	\$ 10.40
Vested	(728,173)	\$ 14.73
Forfeiture	(244,853)	\$ 14.45
Unvested restricted stock units at September 30, 2025	2,758,561	\$ 12.23

Unamortized share-based compensation for restricted stock units as of September 30, 2025 was \$31.4 million, which is expected to be recognized over a weighted-average period of 2.6 years.

Restricted Stock Awards

The following table provides a summary of the unvested common stock awards activity during the nine months ended September 30, 2025.

	Number of Shares	Weighted Average Grant Date Fair Value Per Share
Unvested common stock as of December 31, 2024	82,281	\$ 16.00
Vested	(81,546)	\$ 16.00
Forfeiture	(735)	\$ 16.00
Unvested common stock as of September 30, 2025	—	\$ —

As of September 30, 2025, there is no unamortized share-based compensation for restricted stock awards.

2021 Employee Stock Purchase Plan

In May 2021, the board of directors adopted and the stockholders approved the 2021 Employee Stock Purchase Plan, or the ESPP, which became effective on May 26, 2021. A total of 603,000 shares of common stock were initially reserved for issuance under the ESPP. The number of shares of the common stock reserved for issuance under the ESPP will automatically increase on the first day of each fiscal year, beginning with the fiscal year commencing on January 1, 2021 and continuing for each fiscal year until, and including, the fiscal year commencing on January 1, 2031, by the lesser of: (a) 1% of the total number of outstanding shares of common stock of the Company (on an as converted basis outstanding on the immediately preceding December 31 (rounded down to the nearest whole share)) and (b) an amount determined by the board of directors. 549,887 shares have been issued under the ESPP as of September 30, 2025. The Company recognized compensation expense related to the ESPP of \$0.1 million and \$0.2 million for the three months ended September 30, 2025 and 2024, respectively, and \$0.4 million and \$0.6 million for the nine months ended September 30, 2025 and 2024, respectively.

11. Income Taxes

The Company did not record an income tax provision for the three and nine months ended September 30, 2025 as it generated tax losses during each of the periods. For the three months ended September 30, 2024, the Company recognized an income tax benefit of \$0.9 million on pre-tax book income for the period, resulting in an effective tax rate of (2.4)%. For the nine months ended September 30, 2024, the Company recognized income tax expense of \$0.7 million on a pre-tax book loss for the period, resulting in an effective tax rate of (2.3)%. The primary reconciling items between the federal statutory rate of 21.0% for the three and nine months ended September 30, 2024 and the Company's overall effective tax rate of (2.4)% and (2.3)%, respectively, was the effect of equity compensation, generation of tax credits, deferred state income taxes and the valuation allowance recorded against the full amount of its net deferred tax assets.

A valuation allowance is established when it is more likely than not that some portion or all of a deferred tax asset will not be realized. The realization of deferred tax assets depends on the generation of future taxable income during the period in which related temporary differences become deductible. The Company continues to establish a valuation allowance against the full amount of its net deferred tax assets since it is more likely than not that benefits will not be realized, including those benefits created in the current year. This assessment is based on the Company's historical cumulative losses, which provide strong objective evidence that cannot be overcome with projections of income, as well as the fact that the Company expects continuing losses in the future.

In July 2025, the United States government passed the One Big Beautiful Bill Act into law. This legislation includes significant tax law changes with various effective dates, which may have an impact on the Company's business. The enactment of the One Big Beautiful Bill Act did not have a material impact on the Company's effective tax rate for the three and nine months ended September 30, 2025. The Company will continue to monitor the impact of this Act in future periods.

12. Net Loss Per Share

Basic and diluted net loss per share attributable to common stockholders is calculated as follows (in thousands except share and per share amounts):

	Three Months Ended September 30,		Nine Months Ended September 30,	
	2025	2024	2025	2024
Net (loss) income - basic and diluted	\$ (19,726)	\$ 37,037	\$ (86,044)	\$ (29,782)
Weighted average shares outstanding - basic	103,381,315	96,623,123	103,056,046	90,164,895
Effect of dilutive securities:				
Stock options	—	57,455	—	—
Restricted stock units	—	107,935	—	—
Restricted stock awards	—	149,246	—	—
Weighted average shares outstanding - diluted	103,381,315	96,937,759	103,056,046	90,164,895
Net (loss) income per share - basic	\$ (0.19)	\$ 0.38	\$ (0.83)	\$ (0.33)
Net (loss) income per share - diluted	\$ (0.19)	\$ 0.38	\$ (0.83)	\$ (0.33)

The following outstanding potentially dilutive securities have been excluded from the calculation of diluted net (loss) income per share, as their effect is anti-dilutive:

	Three Months Ended September 30,		Nine Months Ended September 30,	
	2025	2024	2025	2024
Stock options	14,061,544	11,454,704	14,061,544	11,926,321
Unvested common shares	—	—	—	165,840
Restricted stock units	2,758,561	406,092	2,758,561	1,887,246
Shares committed under ESPP	96,589	88,832	96,589	88,832
Total	16,916,694	11,949,628	16,916,694	14,068,239

13. Defined Contribution Plan

The Company maintains an employee savings plan pursuant to Section 401(k) of the Internal Revenue Code. All employees are eligible to participate provided that they meet the requirements of the plan. For each of the three months ended September 30, 2025 and 2024, the Company made matching contributions of \$0.4 million. For the nine months ended September 30, 2025 and 2024, the Company made matching contributions of \$1.6 million and \$1.4 million, respectively.

14. Segment Reporting

The Company views its operations and manages its business in one operating and one reportable segment, which includes all activities dedicated to developing and commercializing targeted therapies for people of all ages with life-threatening diseases. The determination of a single operating segment is consistent with the consolidated financial information regularly provided to the Company's chief operating decision maker, or CODM. The Company's CODM is its Chief Executive Officer, who reviews and evaluates net loss for purposes of assessing performance, making operating decisions, allocating resources, and planning and forecasting for future periods. Our CODM does not evaluate the operating segment using asset or liability information. The operating segment derives all net product revenues from the sales of OJEMDA in the United States through contractual arrangements with its specialty pharmacy and specialty distributor customers. All the Company's assets are located in the United States.

In addition to the significant expense categories included within net loss presented on the Company's condensed statements of operations, see below for disaggregated amounts that comprise research and development expenses (in thousands):

	Three Months Ended September 30,		Nine Months Ended September 30,	
	2025	2024	2025	2024
External costs:				
Third-party CRO, CMO and other third-party clinical trial costs (1)	\$ 19,131	\$ 19,405	\$ 62,038	\$ 61,497
License agreement related payments	—	—	4,000	60,000
Other research and development costs	1,930	1,907	5,952	6,421
Internal costs:				
Employee related expenses	10,358	12,251	35,197	37,961
Total research and development expenses	<u>\$ 31,419</u>	<u>\$ 33,563</u>	<u>\$ 107,187</u>	<u>\$ 165,879</u>

(1) Costs incurred under agreements with third-party CROs, CMOs, and other third parties that conduct clinical activities on the Company's behalf.

15. Subsequent Event

On October 7, 2025, the board of directors and compensation committee of the board of directors, or the Compensation Committee, approved an option repricing, or the Repricing, of the outstanding stock options held by certain then-current directors and employees, including the Company's named executive officers, or Eligible Participants, to be effective on the second full business day following the filing of the Company's Quarterly Report on Form 10-Q for the quarter ended September 30, 2025, or the Effective Date. As permitted under the terms of the Company's equity plans, the exercise price of each outstanding stock option with an exercise price per share of \$8.00 or greater held by the directors and employees providing services as of the Effective Date will be reduced to an amount equal to the closing price of the Company's common stock on the Effective Date, or the New Exercise Price. The board of directors approved the Repricing, upon the recommendation of the Compensation Committee, in order to retain and motivate key contributors of the Company without incurring dilution resulting from significant additional equity grants to the Company's employees or significant additional cash expenditures resulting from cash compensation.

In order to exercise the repriced options at the New Exercise Price, Eligible Participants are required to remain in service with the Company through the Retention Period (as defined herein). The "Retention Period" begins on the Effective Date and ends on the earlier of (i) the one-year anniversary of the Effective Date; or (ii) a Corporate Transaction (as defined in the 2021 Plan). However, if the Retention Period is not satisfied, the Eligible Participant will be required to pay to the Company an additional amount equal to the difference between the New Exercise Price and the prior exercise price of the corresponding option upon exercise of the option; provided that the additional premium payment will not be required if the Eligible Participant's service to the Company is terminated by reason of death or Disability (as defined in the 2021 Plan).

The Company is currently evaluating the effect of the Repricing and, at this time, cannot reasonably estimate the impact on its financial statements.

Item 2. Management’s Discussion and Analysis of Financial Condition and Results of Operations.

You should read the following discussion and analysis of our financial condition and results of operations together with our consolidated financial statements and related notes, our interim condensed financial statements and related notes, and other financial information appearing in our Annual Report on Form 10-K for the year ended December 31, 2024, or our Annual Report, and this Quarterly Report on Form 10-Q. Some of the information contained in this discussion and analysis includes forward-looking statements that involve risks and uncertainties. As a result of many factors, including those factors set forth in the “Risk Factors” in this Quarterly Report on Form 10-Q, our actual results could differ materially from the results described in or implied by the forward-looking statements contained in the following discussion and analysis.

Overview

Day One Biopharmaceuticals, Inc. is a biopharmaceutical company dedicated to developing and commercializing targeted therapies for people of all ages with life-threatening diseases.

At Day One, we aim to identify and develop breakthrough medicines with the goal of improving the outcomes and life trajectories of patients of any age facing serious diseases — starting from Day One. Our “search & development” strategy enables us to find, acquire, and develop potential best- or first-in-class programs with the goal of introducing new medicines that will make a real difference in the treatment of children and adults.

Our first commercial product, tovorafenib, is an oral, brain-penetrant, highly selective type II rapidly accelerated fibrosarcoma, or RAF, kinase inhibitor. Tovorafenib was granted breakthrough therapy designation by the U.S. Food and Drug Administration, or the FDA, in August 2020 for the treatment of relapsed or refractory low-grade glioma, or pLGG, based on initial results from a Phase 1 trial which showed evidence of rapid anti-tumor activity and durable responses in patients with pLGG. Pediatric low-grade glioma is the most common brain tumor diagnosed in children. While new targeted therapeutic options have recently become available for patients with pLGG, there is no consensual standard of care and a vast majority of patients with pLGG do not yet have access to approved therapies. Tovorafenib received orphan drug designation for the treatment of malignant glioma from the FDA in September 2020 and from the EU Commission for the treatment of glioma in May 2021. Additionally, the FDA granted rare pediatric disease designation in July 2021 to tovorafenib for treatment of low-grade gliomas, or LGGs, harboring an activating RAF alteration.

On April 23, 2024, we announced that the FDA approved OJEMDA™ (tovorafenib) for the treatment of patients 6 months of age and older with relapsed or refractory pLGG harboring a BRAF fusion or rearrangement, or BRAF V600 mutation. The indication was approved under accelerated approval based on a regulatory endpoint of overall response rate and the totality of the Company’s pivotal open-label Phase 2 trial, or FIREFLY-1, data, including duration of response. With the approval, we received, and subsequently sold, a rare pediatric disease priority review voucher, or PRV, from the FDA. We have commenced the commercial launch of OJEMDA in the United States. OJEMDA is the only systemic therapy for pLGG that offers once-weekly dosing, with or without food, as a tablet or oral suspension.

The accelerated approval of OJEMDA is based on data from FIREFLY-1, which enrolled a total of 137 relapsed or refractory BRAF-altered pLGG patients across two study arms. Arm 1, which accrued 77 patients, was used for the efficacy analyses. Arm 2 provided additional safety data from an incremental 60 patients and was initiated to enable access to OJEMDA once Arm 1 had fully accrued. Details of this trial were presented in November 2023 at the Society for Neuro-Oncology meeting through two oral plenary presentations and in parallel through a publication in *Nature Medicine*.

The approval of OJEMDA was based, in part, on the major efficacy outcome measure of overall response rate, or ORR, defined as the proportion of patients with complete response, partial response, or PR, or minor response, or MR, by independent review based on Response Assessment in Pediatric Neuro-Oncology Low-Grade Glioma, or RAPNO LGG.

In Arm 1, data from the 76 RAPNO LGG evaluable patients include (May 2024 data cutoff to support the ongoing evaluation of OJEMDA):

- A best ORR of 52.6% (95% CI: 40.8 - 64.2), which included 29% PRs and 11% MRs.
 - The ORR for OJEMDA was 53% among the 64 patients with BRAF fusions or rearrangements and 50% for the 12 patients with a BRAF V600 mutation.
 - The ORR was 51% among the 45 patients who had received a prior MAPK-targeted therapy, and 55% among the 31 patients who had not received a prior MAPK-targeted therapy.
- As of the May 10, 2024 data cutoff, the median duration of response by RAPNO LGG was 18.0 months (95% CI: 12.0, 22.8). In Arm 1, of the 77 patients enrolled, 66 (85.7%) patients have discontinued primary tovorafenib

treatment, most commonly due to being on drug holiday (33 [42.9%] patients); 10 (13.0%) patients discontinued the study.

- The median time to response, following initiation of treatment, with OJEMDA was 5.4 months (range 1.6 months, 17.5 months).
- Based on RANO LGG criteria, the ORR was 53.9% [95% CI: (42.1, 65.5)].

The safety of OJEMDA was evaluated in 137 patients with relapsed or refractory pLGG, with the majority of adverse events being Grade 1 or Grade 2. The most common side effects were rash, hair color changes, tiredness, viral infection, vomiting, headache, fever, decrease in growth velocity, dry skin, constipation, nausea, acne and upper respiratory tract infection.

We initiated a pivotal Phase 3 trial, or FIREFLY-2, evaluating tovorafenib as a front-line therapy in patients ages 6 months to 25 years with pLGG in June 2022. The first patient was dosed in FIREFLY-2 in March 2023. To date, patients continue to enroll in the United States, Canada, Europe, Australia, South America, Middle East and Asia, with approximately 135 sites activated. In June 2024, we announced the following changes to our FIREFLY-2 trial: the primary endpoint of objective response rate will be assessed according to the RAPNO-LGG criteria, key secondary endpoints of progression free survival and duration of response will be assessed according to RAPNO-LGG criteria, new patients will be initiated on a starting dose of 380 mg/m²/dose once weekly, and the addition of a once-monthly carboplatin regime as a fourth standard of care option for arm 2. We expect to complete enrollment of FIREFLY-2 in the first half of 2026. In June 2025, tovorafenib was added to the National Comprehensive Cancer Network, or NCCN, adult glioma treatment guidelines as a category 2a recommended treatment option for patients with recurrent or progressive BRAF-altered disease.

In July 2024, we entered into the Ipsen License Agreement, pursuant to which, we licensed to Ipsen, on an exclusive basis, the right to commercialize tovorafenib in all territories outside the United States and agreed to provide certain research and development and manufacturing services.

Under the terms of the Ipsen License Agreement, (i) Ipsen paid us an upfront license fee in the amount of \$70.8 million in August 2024 and (ii) Ipsen Biopharmaceuticals, Inc., or the Investor, a fully-owned Affiliate of Ipsen, purchased 2,341,495 shares of our common stock in a private placement for \$40.0 million, at a price per share representing a 17.0% premium to the volume weighted average price, or VWAP, of our common stock as traded on The Nasdaq Stock Market LLC for the ten consecutive trading days prior to and including the date of our public release of U.S. GAAP revenue for the quarter ended June 30, 2024 on July 30, 2024, or the Revenue Release, and the ten consecutive trading days following the Revenue Release, in accordance with the terms set forth in an investment agreement by and between us and the Investor dated July 23, 2024.

We are also eligible to receive up to approximately \$380.0 million in additional commercial launch and sales-based milestone payments based on exchange rates as of the reporting date. We are also entitled tiered, double-digit royalty payments starting at mid-teens percentage of annual net sales of tovorafenib, subject to customary adjustments specified in the Ipsen License Agreement. The royalty payment obligations under the Ipsen License Agreement expire on a country-by-country basis no earlier than ten years following the first commercial sale of tovorafenib in the applicable country.

On March 27, 2025, Ipsen Pharma SAS received validation of its completed submission of the Marketing Authorization Application to the EMA for tovorafenib for the treatment of patients with relapsed or refractory pLGG.

In August 2023, we entered into a research collaboration and license agreement, or the Sprint License Agreement, with Sprint Bioscience AB, or Sprint, a Swedish corporation located in Huddinge, Sweden. Under the Sprint License Agreement, Sprint granted to us an exclusive, worldwide license, with the right to grant sublicenses through multiple tiers, to research, develop, and commercialize pharmaceutical products and to engage in research aimed at discovery, optimization and development of an inhibitor targeting Vaccinia Related Kinase 1, or VRK1. In May 2025, we notified Sprint of our decision to discontinue our investment in the VRK1 program and terminated the Sprint License Agreement, with termination taking effect on August 15, 2025. This decision was made following careful consideration of the current development status of the program and our overall strategic objectives.

In June 2024, we entered into a license agreement, or the MabCare License Agreement, with MabCare Therapeutics, or MabCare, a pharmaceutical corporation located in Shanghai, China. Under the MabCare License Agreement, MabCare granted to us an exclusive worldwide license, excluding Greater China, with the right to grant sublicenses through multiple tiers, under specified patent rights and know-how for the Company to develop, manufacture and commercialize DAY301 (formerly MTX-13 or CB-002). DAY301 is a novel Antibody Drug Conjugate, or ADC, targeting protein-tyrosine kinase 7, or PTK7. In pre-clinical studies, DAY301 showed antitumor activity in a wide range of solid tumors. DAY301 targets PTK7, a highly-conserved, catalytically inactive transmembrane protein that is overexpressed in multiple adult cancers, including esophageal, ovarian, lung, and endometrial cancer, as well as pediatric cancers such as neuroblastoma, rhabdomyosarcoma and osteosarcoma. In April 2024, the FDA cleared the investigational new drug application for DAY301. In January 2025, we cleared the first cohort (a single-patient accelerated titration cohort) in the Phase 1a portion of the DAY301 Phase 1a/b clinical trial, and the study continues to enroll in the dose escalation phase of the program.

We believe our business development capabilities combined with our extensive experience in oncology drug development and deep ties within the research and patient advocacy communities, particularly within the pediatric setting, positions us to be a leader in

identifying, acquiring and developing therapies for patients of all ages. We hold exclusive rights to develop tovorafenib for all therapeutic areas worldwide and DAY301 for all therapeutic areas worldwide, excluding Greater China, subject to certain milestone and royalty payments. Further, we hold exclusive rights to commercialize tovorafenib in the United States subject to royalty payments. Pursuant to the Ipsen License Agreement, we licensed to Ipsen, on an exclusive basis, the right to commercialize tovorafenib outside of the United States, in exchange for certain milestone and royalty payments.

The following table summarizes our product and product candidate pipeline.

Our pipeline

Our goal is to take aim at the gaps that have left patients and their families behind.

Product Candidate	Therapeutic Area	Preclinical	Phase 1	Phase 2	Phase 3/ Registrational	Approved	Recent & Anticipated Milestones
Tovorafenib Type II RAF Inhibitor OJEMDA brand name in U.S. ¹ Ex-U.S. Rights:	BRAF-altered relapsed pLGG Front-line RAF-altered pLGG						FDA accelerated approval April 2024 EMA regulatory decision expected 2026 3-year follow-up data expected SNO Conference, November 2025
	Front-line RAF-altered pLGG						Enrollment completion expected 1H 2026
DAY301⁴ PTK7-Targeted ADC	Adult and pediatric solid tumors						First dose cohort cleared January 2025

¹ OJEMDA has received accelerated approval by the U.S. Food and Drug Administration. ² FIREFLY-1 is an open-label, pivotal Phase 2 trial. ³ Ex-U.S. license agreement with Ipsen to commercialize OJEMDA (tovorafenib) outside the U.S. ⁴ DAY301 is a license agreement with MabCell Therapeutics for exclusive worldwide rights, excluding Greater China, for MTX-13/CB-002, a novel ADC targeting PTK7. The safety and efficacy of investigational agents and/or investigational uses of approved products have not been established.



Significant Agreements

Takeda asset purchase agreement

On December 16, 2019, our subsidiary entered into an asset purchase agreement, or the Takeda Asset Agreement, with Millennium Pharmaceuticals, Inc., a related party and an affiliate of Takeda Pharmaceutical Company Limited, or Takeda. Effective December 31, 2021, the subsidiary was merged with and into our company, with our company being the surviving corporation and assuming the subsidiary's obligations under the Takeda Asset Agreement. Pursuant to the Takeda Asset Agreement, we purchased certain technology rights and know-how related to TAK-580 (which is now OJEMDA) that provides a new approach for treating patients with primary brain tumors or brain metastases of solid tumors. Takeda also assigned us its exclusive license agreement, or the Viracta License Agreement, with Viracta. Takeda also granted us a worldwide, sublicensable exclusive license under specified patents and know-how and non-exclusive license under other patents and know-how generated by Takeda under the Takeda Asset Agreement. We also granted Takeda a grant back license, as defined in the Takeda Asset Agreement, which is terminable either automatically or by us in the event Takeda does not achieve specified development milestones within the applicable timeframes set forth under the Takeda Asset Agreement. This grant back license to Takeda was terminated at the time of conversion of the Company from an LLC to a corporation in connection with the Millennium Stock Exchange Agreement.

The term of the Takeda Asset Agreement will expire on a country-by-country basis upon expiration of all assigned patent rights and all licensed patent rights in such country. Takeda can terminate the Takeda Asset Agreement in the event of our bankruptcy. In the event of termination of the Takeda Asset Agreement by Takeda as a result of our cessation of development or bankruptcy, all assigned patents, know-how and contracts (other than the Viracta License Agreement) will be assigned back to Takeda and Takeda will obtain a reversion license under patents and know-how generated to exploit all such terminated products.

In consideration for the sale and assignment of assets and the grant of the license under the Takeda Asset Agreement, we made an upfront payment of \$1.0 million in cash and issued 9,857,143 shares of our Series A redeemable convertible preferred stock in our subsidiary in December 2019. Based on the terms of the Millennium Stock Exchange Agreement, Takeda exchanged the 9,857,143 shares of Series A redeemable convertible preferred stock of our subsidiary for 6,470,382 shares of our common stock upon the effectiveness of the conversion of the Company from an LLC to a corporation, on May 26, 2021.

License agreement with Viracta

On December 16, 2019, our subsidiary amended and restated the Viracta License Agreement that was assigned pursuant to the Takeda Asset Agreement. Effective December 31, 2021, our subsidiary was merged with and into our company, with our company being the surviving corporation and assuming our subsidiary's obligations under Viracta License Agreement. Under the Viracta License Agreement, we received a worldwide exclusive license under specified patent rights and know-how to develop, use, manufacture, and commercialize products containing compounds binding the RAF protein family. We paid \$2.0 million upfront in cash to Viracta in December 2019.

The term of the Viracta License Agreement will expire on a licensed product-by-licensed product and country-by-country basis upon the expiration of our obligation to pay royalties to Viracta with respect to such product in such country. We have the right to terminate the Viracta License Agreement with respect to any or all of the licensed products at will upon a specified notice period.

On March 4, 2024, we entered into an amendment to the Viracta License Agreement. As part of the amendment, we made a one-time payment in March 2024 to Viracta of \$5.0 million in exchange for reduced future payment obligations related to the future sale or use of the rare pediatric disease PRV received.

On April 23, 2024, the FDA approved OJEMDA (a tablet formulation and powder solution formulation of tovorafenib) for the treatment of patients 6 months of age and older with relapsed or refractory pLGG harboring a BRAF fusion or rearrangement, or BRAF V600 mutation. The indication was approved under accelerated approval based on response rate and duration of response. With the approval, we received a rare pediatric disease PRV from the FDA. We made a \$9.0 million milestone payment to Viracta in May 2024 for the achievement of this milestone.

On May 29, 2024, we sold our rare pediatric disease PRV for \$108.0 million to an undisclosed buyer. As part of the transaction, \$8.1 million of the total consideration received from the sale of the rare pediatric disease PRV was paid to Viracta to fully satisfy PRV-related obligations under the Viracta License Agreement.

On December 3, 2024, Viracta assigned the Viracta License Agreement to XOMA (US) LLC, or XOMA, pursuant to a Royalty Purchase Agreement dated March 22, 2021, between Viracta and XOMA, whereby Viracta sold its right, title, and interest in and to the Viracta License Agreement to XOMA. We have agreed to the assignment and novation of the Viracta License Agreement to XOMA as successor party, now referred to as the XOMA License Agreement. No material terms of the XOMA License Agreement have been amended or modified in relation to the same.

On March 27, 2025, Ipsen Pharma SAS received validation of its completed submission of the Marketing Authorization Application to the EMA for tovorafenib for the treatment of patients with relapsed or refractory pLGG. As a result, we paid \$4.0 million to XOMA for the achievement of this milestone in March 2025.

As of September 30, 2025, we could be required to make additional milestone payments of up to \$36.0 million upon achievement of specified development and regulatory milestones for each licensed product in two indications. Commencing with the first commercial sale of OJEMDA in a country, we are obligated to pay tiered royalties ranging in the mid-single-digit percentages on net sales of licensed products. The obligation to pay royalties will end on a country-by-country and licensed product-by-licensed product basis commencing on the first commercial sale in a country and continuing until the later of: (i) the expiration of the last valid claim of the XOMA licensed patents, jointly owned collaboration patents or specified patents owned by us covering the use or sale of such product in such country, (ii) the expiration of the last statutory exclusivity pertaining to such product in such country or (iii) the tenth anniversary of the first commercial sale of such product in such country.

License agreement with Merck KGaA, Darmstadt, Germany

On February 10, 2021, our subsidiary entered into a license agreement, or the MRKDG License Agreement, with Merck KGaA, Darmstadt, Germany, a pharmaceutical corporation located in Darmstadt, Germany. Effective December 31, 2021, the subsidiary was merged with and into our company, with our company being the surviving corporation and assuming the subsidiary's obligations under the MRKDG License Agreement. Under the MRKDG License Agreement, Merck KGaA, Darmstadt, Germany granted to us an exclusive worldwide license, with the right to grant sublicenses through multiple tiers, under specified patent rights and know-how for us to research, develop, manufacture and commercialize products containing and comprising the pimasertib and MSC2015103B compounds. Under the MRKDG License Agreement, we have obligations to use commercially reasonable efforts to develop and commercialize at least two licensed products in at least two specified major market countries by the year 2029.

The term of the MRKDG License Agreement will expire on a licensed product-by-licensed product and country-by-country basis upon the expiration of our obligation to pay royalties to the licensor with respect to such licensed product in such country and will expire in its entirety upon the expiration of all of our payment obligations with respect to all licensed products and all countries under the MRKDG License Agreement.

In consideration for the rights granted under the MRKDG License Agreement and clinical supplies, we made an upfront payment of \$8.0 million in March 2021.

In November 2023, we discontinued our monotherapy substudy due to a limited duration of response in this rare patient population despite observing responses with a generally well tolerated therapy. In July 2024, we informed Merck KGaA, Darmstadt, Germany that we decided to close the program because the benefit/risk profile and the market opportunity did not justify the significant investment required to continue the trial despite observing some clinical responses. In September 2025, we finalized the termination agreement with Merck KGaA, Darmstadt, Germany and concluded material wind-down activities.

Research collaboration and license agreement with Sprint Bioscience AB

On August 15, 2023, we entered into the Sprint License Agreement. Under the Sprint License Agreement, Sprint granted to us an exclusive, worldwide license, with the right to grant sublicenses through multiple tiers, to research, develop, and commercialize pharmaceutical products and to engage in research aimed at discovery, optimization and development of an inhibitor targeting VRK1. In consideration for the rights granted under the Sprint License Agreement, we paid \$3.0 million upfront in cash to Sprint in September 2023.

The term of the Sprint License Agreement will expire on a licensed product and country basis upon the expiration of the royalty term with respect to such licensed product and such country, unless terminated earlier. We have the right to terminate the Sprint License Agreement in its entirety, or on a licensed product-by-licensed product basis, at will upon a specified notice period.

On May 9, 2025, we notified Sprint of our decision to discontinue our investment in the VRK1 program and terminated the Sprint License Agreement, with termination taking effect on August 15, 2025. This decision was made following careful consideration of the current development status of the program and our overall strategic objectives.

License agreement with MabCare Therapeutics

On June 17, 2024, we entered into the MabCare License Agreement. Under the MabCare License Agreement, MabCare granted to us an exclusive worldwide license, excluding Greater China, with the right to grant sublicenses through multiple tiers, under specified patent rights and know-how for us to develop, manufacture and commercialize DAY301, a novel ADC targeting PTK7. Under the MabCare License Agreement, we have obligations to use commercially reasonable efforts to develop, obtain regulatory approval for, and commercialize at least one licensed product in one indication in each of the United States, Japan, and three European countries.

The term of the MabCare License Agreement will expire in its entirety upon the expiration of the last to expire royalty term with respect to all licensed products in our territory, unless terminated earlier. Following the expiration of the royalty term for a licensed product in a country, the license granted to us shall become non-exclusive, fully paid-up, royalty-free, perpetual, and irrevocable for such licensed product in such country. Upon the expiration of the term, the license granted to us shall become non-exclusive, transferable, sublicensable, fully paid, royalty free, perpetual, and irrevocable in its entirety.

In consideration for the rights granted under the MabCare License Agreement, we made an upfront payment of \$55.0 million in July 2024. As of September 30, 2025, we could be required to make additional payments of \$1,132.0 million based upon the achievement of specified development, regulatory, and commercial success-based milestones plus low-to-mid single-digit royalties on net sales outside of Greater China.

License agreement with Ipsen Pharma SAS

On July 23, 2024, we entered into the Ipsen License Agreement, pursuant to which, we licensed to Ipsen, on an exclusive basis, the right to commercialize tovorafenib in all territories outside the United States and agreed to provide certain research and development and manufacturing services. Ipsen shall have the right to grant sublicenses to third-parties.

Under the terms of the Ipsen License Agreement, (i) Ipsen paid us an upfront license fee in the amount of \$70.8 million in August 2024 and (ii) the Investor, a fully-owned United States affiliate of Ipsen, purchased 2,341,495 shares of our common stock in a private placement for \$40.0 million, at a price per share representing a 17.0% premium to the VWAP of our common stock as traded on The Nasdaq Stock Market LLC for the ten consecutive trading days prior to and including the date of the Revenue Release, and the ten consecutive trading days following the Revenue Release, in accordance with the terms set forth in an investment agreement by and between us and the Investor dated July 23, 2024.

As of September 30, 2025, we are also eligible to receive up to approximately \$380.0 million in additional commercial launch and sales-based milestone payments based on exchange rates as of the reporting date. We are also entitled to tiered, double-digit royalty payments starting at mid-teens percentage of annual net sales of tovorafenib, subject to customary adjustments specified in the Ipsen License Agreement. The royalty payment obligations under the Ipsen License Agreement expire on a country-by-country basis no earlier than ten years following the first commercial sale of tovorafenib in the applicable country.

In addition, the Ipsen License Agreement provides that we will supply to Ipsen, and Ipsen will purchase from us, all required quantities of tovorafenib for all territories outside the United States in accordance with a supply agreement to be entered into by and between us and Ipsen, or the Ipsen Supply Agreement.

Following the two-year anniversary of July 23, 2024, the effective date of the Ipsen License Agreement, Ipsen may terminate the Ipsen License Agreement for convenience with six months' prior written notice or for certain other specified reasons. We may terminate the Ipsen License Agreement if Ipsen or any of its affiliates challenge the validity of any patents controlled by us that are licensed under the Ipsen License Agreement. Both we and Ipsen may terminate the Ipsen License Agreement (i) for material breach by the other party and a failure to cure such breach within the time period specified in the Ipsen License Agreement or (ii) the other party's bankruptcy event.

Intellectual Property

We strive to protect and enhance the proprietary technology, inventions and improvements that are commercially important to our business, including seeking, maintaining and defending patent rights, whether developed internally or licensed from our collaborators or other third parties. Our policy is to seek to protect our proprietary position by, among other methods, obtaining licenses to and filing patent applications in the United States and in jurisdictions outside of the United States related to our proprietary technology, inventions, improvements and product candidates that are important to the development and implementation of our business. We also may rely on trade secrets and know-how relating to our proprietary technology and product candidates, continuing innovation, and in-licensing opportunities to develop, strengthen and maintain our proprietary positions. We additionally plan to rely on data exclusivity, market exclusivity and patent term extensions if and when available, and if appropriate, seek and rely on regulatory protection afforded through orphan drug designations. Our commercial success may depend in part on our ability to obtain and maintain patent and other proprietary protection for our technology, inventions and improvements; to preserve the confidentiality of our trade secrets; to maintain our licenses to use intellectual property owned by third parties; to defend and enforce our proprietary rights, including our patents; and to operate without infringing on the valid and enforceable patents and other proprietary rights of third parties.

Our owned or co-owned patent portfolio, as of January 1, 2025, includes a co-owned United States patent directed to the compositions of matter and pharmaceutical compositions of tovorafenib, which provides patent protection in the United States through 2031, and multiple foreign patents and applications including granted patents in Germany, France, United Kingdom, Belgium, Switzerland, Denmark, Spain, Ireland, Italy, Netherlands, Australia, Brazil, Canada, China, India, Japan, Korea, Mexico, Russia, Singapore, South Africa, Taiwan, and Hong Kong, which are expected to expire in 2028. Furthermore, we have filed two applications for patent term extension of 5 years pertaining to the regulatory review period for OJEMDA (tovorafenib) for the United States patent directed to the compositions of matter and pharmaceutical compositions. One application involves NDA 217700 for a tovorafenib tablet and the other involves NDA 218033 for a tovorafenib oral suspension. The applications were submitted to the United States Patent and Trademark Office, or USPTO, in June 2024 and are under review by the USPTO. Approval of the applications for patent term extension would extend the United States patent to August 2036. We have additional patents and pending applications drawn to formulations, methods of use, and methods of manufacture for tovorafenib. Our patent portfolio also includes issued and pending patents covering formulations, methods of manufacture, and methods of use of tovorafenib. Certain issued patents are expected to provide protection through 2035, and pending applications, if granted, could extend coverage into the 2040s.

Where applicable, patent extensions will be requested for jurisdictions outside of the United States upon regulatory approval in these jurisdictions.

In the United States, OJEMDA has received both 5 years of New Chemical Entity exclusivity and 7 years of Orphan Drug Exclusivity to April 2029 and April 2031, respectively. During the seven-year exclusivity period, the FDA may not approve any other sponsor's application to market the same drug for the same indication, except in limited circumstances, such as a showing of clinical superiority to the product with orphan drug exclusivity by means of greater effectiveness, greater safety, or providing a major contribution to patient care, or in instances of drug supply issues or consent by the exclusivity holder.

Components of Results of Operations

Revenue

Product revenue, net

In April 2024, the FDA approved OJEMDA for the treatment of patients 6 months of age and older with relapsed or refractory pLGG harboring a BRAF fusion or rearrangement, or BRAF V600 mutation. We record product revenue net of estimated discounts, chargebacks, rebates, specialty distributor fees, copay assistance, and product returns.

License revenue

In July 2024, we entered into the Ipsen License Agreement, pursuant to which, we licensed to Ipsen, on an exclusive basis, the right to commercialize tovorafenib in all territories outside the United States and agreed to provide certain research and development and manufacturing services. The portion of the transaction price allocated to the licenses was recognized as revenue upon delivery of

the licenses in the year ended of December 31, 2024. The transaction price allocated to the research and development services is being recognized over time as services are delivered.

Operating expenses

Cost of product and license revenue

Cost of product and license revenue includes the cost of inventory sold, amortization expense of intangible assets and third-party royalties payable on our net product revenue. Cost of inventory sold may also include costs related to excess or obsolete inventory adjustment charges, abnormal costs, unabsorbed manufacturing and overhead costs, and manufacturing variances. We expect to use inventory previously expensed to research and development by the third quarter of 2025, and accordingly, we expect our cost of product and license revenue of OJEMDA to increase as a percentage of product revenue in future periods as we produce and sell inventory that reflects the full cost of manufacturing the product. Cost of license revenue includes the cost of delivering the performance obligations under the Ipsen License Agreement.

Research and development expenses

Research and development expenses consist primarily of external and internal expenses incurred for our research activities, including our discovery and in-licensing undertakings, and the development of our product candidates.

External expenses include:

- costs incurred under agreements with third-party contract research organizations, or CROs, contract manufacturing organizations, or CMOs, and other third parties that conduct clinical activities on our behalf;
- costs associated with acquiring technology and intellectual property licenses that have no alternative future uses; and
- other business operational costs, such as rent, facilities and maintenance, travel and information technology, incurred related to research and development activities, but are not allocable to a specific product or product candidate.

Internal expenses include:

- employee-related costs, including salaries, bonuses, benefits and share-based compensation expense, for our research and development personnel.

We expense research and development expenses as incurred. We track external costs by program, which currently consist of expenses for our tovorafenib, pimasertib, DAY301, and the VRK1 inhibitor program. We do not track indirect costs on a program specific basis because these costs are deployed across multiple programs and, as such, are not separately classified.

Research and development activities are central to our business model. We expect that our research and development expenses will increase for the foreseeable future as we continue to implement our business strategy; advance our product candidates through clinical trials and conduct larger clinical trials; expand our research and development efforts; and identify, acquire and develop additional product candidates, particularly as more of our product candidates move into clinical development and later stages of clinical development.

The successful development of our drug candidates is uncertain and subject to a number of risks. We cannot guarantee that results of clinical trials will be favorable or sufficient to support marketing authorizations for any of our product development programs. We could decide to abandon development or be required to spend considerable resources not otherwise contemplated. For additional discussion regarding the risks and uncertainties regarding our research and development programs, please refer to Part II, Item 1A “Risk Factors” in this Quarterly Report on Form 10-Q.

Selling, general and administrative expenses

Selling, general and administrative expenses consist primarily of employee-related costs, professional services and other operational costs. Employee-related costs include salaries, bonuses, benefits and share-based compensation expense for our selling, general and administrative personnel. Professional service expenses include legal fees; professional fees for accounting, marketing, human resources, business development, and other consulting services. Other operational costs include expenses for rent and facilities maintenance, travel, insurance and information technology.

We expect that our selling, general and administrative expenses will remain relatively consistent in the near-term.

Gain from sale of priority review voucher

Gain from the sale of priority review voucher represents the sale of our rare pediatric disease PRV, which was awarded to us in connection with the FDA’s approval of OJEMDA.

Results of operations

Comparison of three months ended September 30, 2025 and 2024

The following table summarizes our results of operations for the three months ended September 30, 2025 and 2024 (unaudited):

	Three Months Ended September 30,		\$ Change	% Change
	2025	2024		
Revenue:				
Product revenue, net	\$ 38,522	\$ 20,070	\$ 18,452	91.9%
License revenue	1,273	73,691	(72,418)	*
Total revenues	39,795	93,761	(53,966)	(57.6)%
Cost and operating expenses:				
Cost of product and license revenue	4,480	1,590	2,890	181.8%
Research and development	31,419	33,563	(2,144)	(6.4)%
Selling, general and administrative	28,147	28,972	(825)	(2.8)%
Total cost and operating expenses	64,046	64,125	(79)	(0.1)%
(Loss) income from operations	(24,251)	29,636	(53,887)	(181.8)%
Non-operating income:				
Investment income, net	4,559	5,322	(763)	(14.3)%
Other (expense) income, net	(34)	1,197	(1,231)	(102.8)%
Total non-operating income, net	4,525	6,519	(1,994)	(30.6)%
(Loss) income before income taxes	(19,726)	36,155	(55,881)	(154.6)%
Income tax benefit	—	882	(882)	*
Net (loss) income	(19,726)	37,037	(56,763)	(153.3)%

* Amount and/or percentage not meaningful

Product revenue, net

Product revenue, net increased \$18.4 million, from \$20.1 million for the three months ended September 30, 2024 to \$38.5 million for the three months ended September 30, 2025 due to increased sales of OJEMDA in the United States.

License revenue

License revenue decreased \$72.4 million, from \$73.7 million for the three months ended September 30, 2024 to \$1.3 million for the three months ended September 30, 2025 due to the recognition of the upfront consideration received from the Ipsen License Agreement for the pLGG license rights in the three months ended September 30, 2024.

Cost of product and license revenue

Cost of product and license revenue increased \$2.9 million, from \$1.6 million for the three months ended September 30, 2024 to \$4.5 million for the three months ended September 30, 2025 due to increased sales of OJEMDA in the United States and cost of license related services provided under the Ipsen License Agreement.

Research and development expenses

Research and development expenses decreased \$2.2 million, from \$33.6 million for the three months ended September 30, 2024 to \$31.4 million for the three months ended September 30, 2025. Personnel-related expenses decreased by \$1.9 million driven by lower share-based compensation and third-party expenses decreased by \$0.3 million due primarily to reductions in pimasertib clinical development costs.

The following table summarizes our external and internal research and development expenses for the three months ended September 30, 2025 and 2024:

	Three Months Ended September 30,	
	2025	2024
(in thousands)		
External costs:		
Third-party CRO, CMO and other third-party clinical trial costs (1)	\$ 19,131	\$ 19,405
Other research and development costs	1,930	1,907
Internal costs:		
Employee related expenses	10,358	12,251
Total research and development expenses	<u>\$ 31,419</u>	<u>\$ 33,563</u>

- (1) Third-party CRO, CMO and other clinical trial costs for the tovorafenib, pimasertib, DAY301, and the VRK1 inhibitor programs were \$15.4 million, \$0.3 million, \$2.8 million, and \$0.6 million, respectively, for three months ended September 30, 2025 compared to \$16.6 million, \$0.7 million, \$0.6 million, and \$1.5 million, respectively, for the three months ended September 30, 2024.

Selling, general and administrative expenses

Selling, general and administrative expenses decreased \$0.9 million, from \$29.0 million for the three months ended September 30, 2024 to \$28.1 million for the three months ended September 30, 2025. The decrease in selling, general and administrative expenses was primarily due to a decrease of \$1.8 million in personnel-related expenses driven by lower share-based compensation offset by an increase of \$0.8 million in other selling, general and administrative costs and an increase of \$0.1 million in professional services driven by increases in commercial launch activities.

Income tax expense

For the three months ended September 30, 2025, we did not record an income tax provision since we generated tax losses during this period. Income tax expense for the three months ended September 30, 2024, was \$0.9 million resulting in an effective tax rate of (2.4%) driven by the effect of equity compensation, generation of tax credits, deferred state income taxes and the valuation allowance recorded against the full amount of our net deferred tax assets.

Comparison of nine months ended September 30, 2025 and 2024

The following table summarizes our results of operations for the nine months ended September 30, 2025 and 2024 (unaudited):

	Nine Months Ended September 30,		\$ Change	% Change
	2025	2024		
Revenue:				
Product revenue, net	\$ 102,587	\$ 28,262	\$ 74,325	263.0%
License revenue	1,877	73,691	(71,814)	*
Total revenues	<u>104,464</u>	<u>101,953</u>	<u>2,511</u>	<u>2.5%</u>
Cost and operating expenses:				
Cost of product and license revenue	11,129	2,297	8,832	384.5%
Research and development	107,187	165,879	(58,692)	(35.4)%
Selling, general and administrative	86,440	85,715	725	0.8%
Total cost and operating expenses	<u>204,756</u>	<u>253,891</u>	<u>(49,135)</u>	<u>(19.4)%</u>
Loss from operations	<u>(100,292)</u>	<u>(151,938)</u>	<u>51,646</u>	<u>(34.0)%</u>
Non-operating income (expense):				
Gain from sale of priority review voucher	—	108,000	(108,000)	*
Investment income, net	14,324	13,649	675	4.9%
Other (expense) income, net	(76)	1,177	(1,253)	*
Total non-operating income, net	<u>14,248</u>	<u>122,826</u>	<u>(108,578)</u>	<u>(88.4)%</u>
Loss before income taxes	<u>(86,044)</u>	<u>(29,112)</u>	<u>(56,932)</u>	<u>195.6%</u>
Income tax expense	—	(670)	670	*
Net Loss	<u>\$ (86,044)</u>	<u>\$ (29,782)</u>	<u>\$ (56,262)</u>	<u>188.9%</u>

* Amount and/or percentage not meaningful

Product revenue, net

Product revenue, net increased \$74.3 million, from \$28.3 million for the nine months ended September 30, 2024 to \$102.6 million for the nine months ended September 30, 2025 due to increased sales of OJEMDA in the United States.

License revenue

License revenue decreased \$71.8 million, from \$73.7 million for the nine months ended September 30, 2024 to \$1.9 million for the nine months ended September 30, 2025 due to the recognition of the upfront consideration received from the Ipsen License Agreement for the pLGG license rights in the nine months ended September 30, 2024.

Cost of product and license revenue

Cost of product and license revenue increased \$8.8 million, from \$2.3 million for the nine months ended September 30, 2024 to \$11.1 million for the nine months ended September 30, 2025 due to increased sales of OJEMDA in the United States and cost of license related services provided under the Ipsen License Agreement.

Research and development expenses

Research and development expenses decreased \$58.7 million, from \$165.9 million for the nine months ended September 30, 2024 to \$107.2 million for the nine months ended September 30, 2025. License related payments decreased by \$56.0 million due to a \$4.0 million milestone payment related to the XOMA License Agreement in the nine months ended September 30, 2025 compared to a \$55.0 million upfront payment related to the MabCare License Agreement and a \$5.0 million amendment payment related to the Viracta License Agreement in the nine months ended September 30, 2024. Personnel-related expenses decreased by \$2.8 million driven by lower share-based compensation and other research and development costs decreased by \$0.4 million. This is offset by an increase in \$0.5 million in third-party expenses due primarily to an increase in clinical trial and manufacturing activities associated with DAY301.

The following table summarizes our external and internal research and development expenses for the nine months ended September 30, 2025 and 2024:

	Nine Months Ended September 30,	
	2025	2024
	(in thousands)	
External costs:		
Third-party CRO, CMO and other third-party clinical trial costs (1)	\$ 62,038	\$ 61,497
XOMA License Agreement milestone payment	4,000	—
MabCare license agreement upfront payment	—	55,000
Viracta license agreement payment	—	5,000
Other research and development costs	5,952	6,421
Internal costs:		
Employee related expenses	35,197	37,961
Total research and development expenses	<u>\$ 107,187</u>	<u>\$ 165,879</u>

- (1) Third-party CRO, CMO and other clinical trial costs for the tovorafenib, pimasertib, DAY301, and the VRK1 inhibitor programs were \$49.9 million, \$0.9 million, \$7.7 million, and \$3.5 million, respectively, for nine months ended September 30, 2025 compared to \$52.7 million, \$3.2 million, \$0.6 million, and \$5.0 million, respectively, for the nine months ended September 30, 2024.

Selling, general and administrative expenses

Selling, general and administrative expenses increased \$0.7 million, from \$85.7 million for the nine months ended September 30, 2024 to \$86.4 million for the nine months ended September 30, 2025. The increase in selling, general and administrative expenses was primarily due to an increase of \$0.1 million in professional services driven by increases in commercial launch activities and an increase of \$1.2 million in other selling, general and administrative costs offset by a decrease in \$0.6 million in personnel-related expenses driven by changes in headcount.

Income tax expense

For the nine months ended September 30, 2025, we did not record an income tax provision since we generated tax losses during this period. Income tax expense for the nine months ended September 30, 2024, was \$0.7 million resulting in an effective tax rate of (2.3%) driven by the effect of equity compensation, generation of tax credits, deferred state income taxes and the valuation allowance recorded against the full amount of our net deferred tax assets.

Liquidity and Capital Resources

Sources of liquidity

In July 2024, we entered into a securities purchase agreement with certain institutional and accredited investors, or the PIPE Investors, pursuant to which we agreed to sell and issue to the PIPE Investors in a private placement, or the Private Placement, an aggregate of (i) 10,551,718 shares of our common stock at a purchase price of \$14.50 per share and (ii) 1,517,241 pre-funded warrants, or the Pre-Funded Warrants, to purchase up to an aggregate of 1,517,241 shares of our common stock, or the Warrant Shares, at a purchase price of \$14.4999 per Pre-Funded Warrant. Each Pre-Funded Warrant has an exercise price of \$0.0001 per Warrant Share. The Pre-Funded Warrants are exercisable at any time after their original issuance and will not expire. The Private Placement closed on August 1, 2024 and we received net proceeds of \$166.5 million, after deducting placement agent fees, offering costs and other expenses, of which \$145.6 million was allocated to the common stock and \$20.9 million was allocated to the Pre-Funded Warrants. The net proceeds were recorded as a component of additional paid-in capital.

In July 2024, we entered into the Ipsen License Agreement, pursuant to which, we licensed to Ipsen, on an exclusive basis, the right to commercialize tovorafenib in all territories outside the United States and agreed to provide certain research and development and manufacturing services. Under the terms of the Ipsen License Agreement, (i) Ipsen paid us an upfront license fee in the amount of \$70.8 million in August 2024 and (ii) the Investor, a fully-owned United States affiliate of Ipsen, purchased 2,341,495 shares of our common stock in a private placement for \$40.0 million, at a price per share representing a 17.0% premium to the VWAP of our common stock as traded on The Nasdaq Stock Market LLC for the ten consecutive trading days prior to and including the date of the Revenue Release, and the ten consecutive trading days following the Revenue Release, in accordance with the terms set forth in an investment agreement by and between we and the Investor dated July 23, 2024. We valued the shares at issuance at \$32.6 million, concluding that our Company's common stock price as traded on The Nasdaq Stock Market LLC on the closing date of the transaction approximated fair value, which was then recorded as a component of additional paid-in capital.

We have entered into an equity distribution agreement, or the Equity Distribution Agreement, with Piper Sandler & Co. and JonesTrading Institutional Services LLC, as sales agents, relating to the issuance and sale of shares of our common stock for an aggregate offering price of up to \$250.0 million under an at-the-market offering program, or the ATM. No shares of our common stock have been sold under the ATM as of September 30, 2025.

As of September 30, 2025, we had an accumulated deficit of \$640.1 million and \$451.6 million in cash, cash equivalents and short-term investments. We believe our cash, cash equivalents and short-term investments will be sufficient to satisfy our capital requirements through at least twelve months after the date that this Quarterly Report is filed.

Our primary use of cash is to fund operating expenses, which consist of research and development expenditures and selling, general and administrative expenditures. Cash used to fund operating expenses is impacted by the timing of when we pay these expenses, as reflected in the change in our outstanding accounts payable and accrued expenses. Our material cash requirements include the following contractual and other obligations.

Leases

We have an operating lease obligation for office space. As of September 30, 2025, we had fixed lease payment obligations of approximately \$0.5 million payable, within 12 months.

Contract Research Organizations and Contract Manufacturing Organizations

We have entered into contracts in the normal course of business with CROs, CMOs, and other third-party vendors for clinical trial, manufacturing, testing, and other research and development activities. These contracts generally provide for termination on notice, with the exception of one vendor where certain costs are non-cancellable after the approval of the project. As of September 30, 2025, there were no amounts accrued related to termination and cancellation charges as these are not probable.

License Agreements

Under our license agreements, we have payment obligations that are contingent upon future events such as our achievement of specified development, regulatory and commercial milestones and are required to make royalty payments in connection with the sale of products developed under those agreements. The amount and timing of milestone obligations are unknown or uncertain as we are unable to estimate the timing or likelihood of achieving the milestone events. Additionally, the amount of royalty payments are based upon future product sales, which we are unable to predict with certainty. These potential obligations are further described in Note 7 to the financial statements.

Cash flows

The following table summarizes our sources and uses of cash for the periods presented:

	Nine Months Ended September 30,	
	2025	2024
Net cash used in operating activities	\$ (89,608)	\$ (48,105)
Net cash provided by investing activities	7,169	38,646
Cash provided by financing activities	748	201,440
Net (decrease) increase in cash and cash equivalents	<u>\$ (81,691)</u>	<u>\$ 191,981</u>

Operating activities

Net cash used in operating activities for the nine months ended September 30, 2025 was \$89.6 million consisting of our net loss of \$86.0 million and changes in net operating assets and liabilities of \$27.2 million, which were offset by non-cash charges of \$23.7 million. Changes in operating assets and liabilities were primarily related to a decrease in accrued expenses and other current liabilities of \$22.4 million, an increase in inventory of \$3.6 million, an increase in accounts receivable of \$2.8 million, an increase in prepaid expenses and other current assets of \$1.2 million, and an increase in deposits and other long-term assets of \$0.2 million. This was offset by an increase in accounts payable of \$2.5 million, an increase in deferred revenue of \$0.5 million, and an increase in our operating lease liability of \$0.1 million.

Net cash used in operating activities for the nine months ended September 30, 2024 was \$48.1 million, consisting of our net loss of \$29.8 million, non-cash charges of \$18.1 million and net changes in operating assets and liabilities of \$0.3 million. Non-cash charges are primarily related to a gain from the sale of our rare pediatric disease PRV of \$108.0 million and accretion of discounts on short-term investments of \$3.6 million, which was partially offset by acquired in-process research and development assets of \$55.0 million and share-based compensation expense of \$37.2 million. Net changes in operating assets and liabilities are primarily related to an increase in accrued expenses and other current liabilities of \$8.7 million, deferred revenue of \$5.3 million, and accounts payable of \$0.4 million, which was partially offset by an increase in accounts receivable of \$9.5 million, inventory of \$2.7 million, prepaid expenses and other current assets of \$2.3 million, and a decrease in operating lease liability of \$0.3 million.

Investing activities

Net cash provided by investing activities for the nine months ended September 30, 2025 was \$7.2 million attributable to the purchase of short-term investments of \$637.9 million, acquisition of intangible assets of \$3.5 million, and the acquisition of property and equipment of \$0.3 million. This was partially offset by the proceeds from maturity of short-term investments of \$648.8 million.

Net cash provided by investing activities for the nine months ended September 30, 2024 was \$38.6 million related to the proceeds from the maturity of short-term investments of \$307.5 million, proceeds from the sales of short-term investments of \$79.4 million, and from the sale of our rare pediatric disease PRV of \$108.0 million, partially offset by the purchase of short-term investments of \$383.4 million, acquisition of in-process research and development assets of \$55.0 million, acquisition of intangible assets of \$17.1 million, and purchase of property and equipment of \$0.8 million.

Financing activities

Cash provided by financing activities for the nine months ended September 30, 2025 was \$0.7 million related to net proceeds from the purchases made under our 2021 Employee Stock Purchase Plan.

Cash provided by financing activities for the nine months ended September 30, 2024 was \$201.4 million related to net proceeds from the issuance of common stock in connection with the private placement of \$178.2 million and issuance of prefunded warrants to purchase common stock of \$20.9 million, and upon stock option exercises and purchases made under our 2021 Employee Stock Purchase Plan of \$2.3 million.

Funding requirements

Since our inception, we have incurred significant operating losses. We expect to continue to incur significant expenses and increasing operating losses for the foreseeable future in connection with our ongoing activities. As of September 30, 2025, we had \$451.6 million in cash and cash equivalents and short-term investments. We believe our cash, cash equivalents and short-term investments will be sufficient to satisfy our capital requirements at least twelve months after the date that this Quarterly Report is filed.

If our cash, cash equivalents and short-term investments are not sufficient to meet capital needs until such time that we can generate substantial revenue, we expect to finance our cash needs through a combination of equity offerings, debt financings, collaborations, strategic alliances and marketing, distribution or licensing arrangements. Adequate additional funds may not be available to us on acceptable terms, or at all. If we are unable to raise capital when needed or on attractive terms, we may be required

to delay, limit, reduce or terminate our research, product development programs or any future commercialization efforts or grant rights to develop and market product candidates that we would otherwise prefer to develop and market ourselves.

To the extent that we raise additional capital through the sale of equity or convertible debt securities, stockholder ownership interest will be diluted, and the terms of these securities may include liquidation or other preferences that adversely affect stockholder rights as a common stockholder. Debt financing and preferred equity financing, if available, may involve agreements that include covenants limiting or restricting our ability to take specific actions, such as incurring additional debt, making acquisitions or capital expenditures or declaring dividends.

If we raise additional funds through collaborations, strategic alliances or marketing, distribution or licensing arrangements with third parties, we may have to relinquish valuable rights to our technologies, future revenue streams, research programs or product candidates or grant licenses on terms that may not be favorable to us.

Our ability to raise additional funds may be adversely impacted by potential worsening global economic conditions, and disruptions to and volatility in the credit and financial markets in the United States and worldwide resulting from inflation, changing interest rates, significant political, trade or regulatory developments, global regional conflicts, public health epidemics, or otherwise. Because of the numerous risks and uncertainties associated with product development, we cannot predict the timing or amount of increased expenses and cannot assure that we will ever be profitable or generate positive cash flow from operating activities.

Critical accounting policies and use of estimates

Our management's discussion and analysis of financial condition and results of operations is based on our condensed financial statements, which have been prepared in accordance with U.S. GAAP. The preparation of condensed financial statements in conformity with U.S. GAAP requires management to make estimates and assumptions that affect the reported amounts of assets, liabilities and disclosure of contingent assets and liabilities at the date of the financial statements and the reported amounts of expenses during the reporting period. We base our estimates on historical experience, known trends and events and various other factors that we believe are reasonable under the circumstances, the results of which form the basis for making judgments about the carrying values of assets and liabilities that are not readily apparent from other sources. We evaluate our estimates and assumptions on an ongoing basis. Our actual results may differ from these estimates under different assumptions or conditions.

While our significant accounting policies are described in more detail in the Notes to our Condensed Financial Statements appearing within Item 2 of this Quarterly Report, we believe that the following accounting policies are the most critical to the judgments and estimates used in the preparation of our condensed financial statements.

Accrued research and development expense

We record accrued liabilities for estimated costs of our clinical trials conducted by third-party service providers. We record the estimated costs of the clinical trials as research and development expense based upon the estimated amount of services provided but not yet invoiced. We accrue for these costs based on factors such as estimates of the work completed and in accordance with terms established with our third-party service providers under the service agreements.

We make payments in connection with the clinical trials under contracts with CROs who conduct and manage our clinical trials. The financial terms of these contracts are subject to negotiation, which vary by contract and may result in payments that do not match the periods over which materials or services are provided. Generally, these agreements set forth the scope of work to be performed at a fixed fee, unit price or on a time and materials basis. We accrue costs for clinical trial activities performed by CROs based upon the estimated amount of work completed on each trial. For clinical trials, the significant factors used in estimating accruals include the number of patients enrolled, the activities to be performed for each patient, the number of active clinical sites and the duration for which the patients will be enrolled in the trial. We monitor patient enrollment levels and related activities to the extent possible through internal reviews, correspondence with CROs and review of contractual terms. We base our estimates on the best information available at the time.

If we do not identify costs that have begun to be incurred or if we under- or over-estimate the level of services performed or the costs of these services, actual expenses could differ from our estimates. To date, we have not experienced any material differences between accrued costs and actual costs incurred. However, due to the nature of estimates, we cannot assure that we will not make changes to our estimates in the future as we become aware of additional information about the status or conduct of our clinical trials.

New Accounting Pronouncements

Refer to Note 2 of the Notes to our Financial Statements included in this Quarterly Report on Form 10-Q for a summary of recently issued and adopted accounting pronouncements.

Item 3. Quantitative and Qualitative Disclosures About Market Risk.

There were no material changes to our market risks from those described in Part II Item 7A. Quantitative and qualitative disclosures about market risk of our 2024 Form 10-K.

Item 4. Controls and Procedures.

Evaluation of Disclosure Controls and Procedures

As of September 30, 2025, management, with the participation of our Principal Executive Officer and Principal Financial Officer, performed an evaluation of the effectiveness of our disclosure controls and procedures as defined in Rules 13a-15(e) and 15d-15(e) of the Exchange Act. Our disclosure controls and procedures are designed to ensure that information required to be disclosed in the reports we file or submit under the Exchange Act is recorded, processed, summarized, and reported within the time periods specified in the SEC's rules and forms, and that such information is accumulated and communicated to our management, including the Principal Executive Officer and the Principal Financial Officer, to allow timely decisions regarding required disclosures. Any controls and procedures, no matter how well designed and operated, can provide only reasonable assurance of achieving the desired control objective and management necessarily applies its judgment in evaluating the cost-benefit relationship of possible controls and procedures. Based on this evaluation, our Principal Executive Officer and Principal Financial Officer concluded that, as of September 30, 2025, our disclosure controls and procedures were effective at a reasonable assurance level.

Changes in Internal Control over Financial Reporting

There were no changes in our internal control over financial reporting that occurred during the three months ended September 30, 2025 that have materially affected, or are reasonably likely to materially affect, our internal control over financial reporting.

PART II-OTHER INFORMATION

Item 1. Legal Proceedings.

From time to time, we may be involved in legal proceedings arising in the ordinary course of our business. We are not presently a party to any legal proceedings that, in the opinion of management, would have a material adverse effect on our business. Regardless of outcome, litigation can have an adverse impact on us due to defense and settlement costs, diversion of management resources, negative publicity and reputational harm, and other factors.

Item 1A. Risk Factors.

Investing in our common stock involves a high degree of risk. Before making your decision to invest in shares of our common stock, you should carefully consider the risks and uncertainties described below, together with the other information contained in this quarterly report, including our financial statements and the related notes and “Management’s Discussion and Analysis of Financial Condition and Results of Operations.” The risks and uncertainties described below are not the only ones we face. Additional risks and uncertainties that we are unaware of, or that we currently believe are not material, may also become important factors that affect us. We cannot assure you that any of the events discussed below will not occur. These events could have a material and adverse impact on our business, financial condition, results of operations and prospects. If that were to happen, the trading price of our common stock could decline, and you could lose all or part of your investment.

Summary of Risk Factors

Our business is subject to several risks and uncertainties, including those immediately following this summary. Some of these risks are:

- We are a commercial stage biopharmaceutical company with a limited operating history in the initial stages of the commercialization of our product, OJEMDA, which may make it difficult for investors to evaluate our current business and likelihood of success and viability.
- We have incurred significant net losses since our inception. We expect to incur continued losses for the foreseeable future and may never achieve or maintain profitability.
- Our near-term revenues are highly dependent on the successful commercialization of OJEMDA, which received marketing approval in April 2024 from the FDA for the treatment of patients 6 months of age and older with relapsed or refractory pLGG harboring a BRAF fusion or rearrangement, or BRAF V600 mutation. To the extent that OJEMDA is not commercially successful, our business, financial condition and results of operations would be materially and adversely affected and the price of our common stock would decline.
- Our ability to generate revenue and achieve profitability depends significantly on our ability to achieve several objectives relating to the discovery or identification, development and commercialization of OJEMDA and our product candidates.
- We may require additional capital to finance our operations and achieve our goals. If we are unable to raise capital when needed or on terms acceptable to us, we may be forced to delay, reduce or eliminate our research or product development programs, any future commercialization efforts or other operations.
- Clinical trials are very expensive, time-consuming and difficult to design and implement, and involve uncertain outcomes. Furthermore, results of earlier preclinical studies and clinical trials may not be predictive of results of future preclinical studies or clinical trials. OJEMDA and our product candidates may not have favorable results in later clinical trials, if any, or receive marketing authorization. If we fail to demonstrate the safety and effectiveness of our product candidates, our reputation may be harmed and our business will suffer.
- We may rely on data from investigator-initiated studies, as we did for the Phase 1 clinical trial, and we do not control the trial operations or reporting of the results of such trials.
- The development and commercialization of pharmaceutical products are subject to extensive regulation, and we may not obtain marketing authorizations for DAY301 or any future product candidates, on a timely basis or at all.
- The manufacture of pharmaceutical products, including OJEMDA and our product candidate, DAY301, is complex. Our third-party manufacturers may encounter difficulties in production, which could delay or entirely halt their ability to supply our product candidates for clinical trials or, if approved, our products for commercial sale.
- Our future success depends on our ability to retain our executive officers and key employees and to attract, retain and motivate qualified personnel and manage our human capital.

- We will need to grow the size and capabilities of our organization, and we may experience difficulties in managing this growth.
- If we are unable to obtain and maintain patent protection or other necessary rights for our products and technology, or if the scope of the patent protection obtained is not sufficiently broad or our rights under our patents (owned, co-owned or licensed) is not sufficiently broad, our competitors could develop and commercialize products and technology similar or identical to ours, and our ability to successfully commercialize our products and technology may be adversely affected.

Risks Related to Our Financial Position

We are a commercial-stage biopharmaceutical company with a limited operating history in the initial stages of the commercialization our product OJEMDA, which may make it difficult for investors to evaluate our current business and likelihood of success and viability.

We are a commercial-stage biopharmaceutical company with a limited operating history upon which you can evaluate our business and prospects. Investment in drug development is a highly speculative undertaking and involves a substantial degree of risk. We commenced operations in 2018, and, to date, we have devoted substantially all of our resources to identifying, acquiring and developing OJEMDA and our product candidates, including DAY 301, which we licensed from MabCare Therapeutics in June 2024, building our pipeline, organizing and staffing our company, business planning, building a commercial organization, establishing and maintaining our intellectual property portfolio, establishing arrangements with third parties for the manufacture of our product candidates, raising capital and providing selling, general and administrative support for these operations.

We are continuing to transition from a company with a research and development focus to a company capable of supporting commercial activities and we may not be successful in such transition. We are still at the early stages of demonstrating our ability to manufacture at commercial scale, or arrange for a third party to do so on our behalf, or conduct sales, marketing and distribution activities necessary for successful product commercialization. As a result, it may be more difficult for you to accurately predict our likelihood of success and viability than it could be if we had a longer operating history.

In addition, we may encounter unforeseen expenses, difficulties, complications, delays and other known and unknown factors and risks frequently experienced by biopharmaceutical companies in rapidly evolving fields and with recently approved therapies. If we do not adequately address these risks and difficulties or successfully make a commercial transition, our business will suffer.

We have incurred significant net losses since our inception. We expect to incur continued losses for the foreseeable future and may never achieve or maintain profitability.

We have incurred significant net losses in each reporting period since our inception, and as of September 30, 2025, we have generated approximately \$159.8 million of revenue from product sales of OJEMDA. We have financed our operations principally through the sale and issuance of redeemable convertible preferred shares, the completion of our initial public offering, or IPO, and follow-on public offerings of our common stock. For the nine months ended September 30, 2025 and 2024 we reported a net loss of \$86.0 million and \$29.8 million, respectively. We had an accumulated deficit of \$640.1 million as of September 30, 2025. We expect to incur increasing levels of operating losses for the foreseeable future, particularly as we advance tovorafenib and DAY301 through clinical development. Our prior losses, combined with expected future losses, have had, and will continue to have, an adverse effect on our stockholders' equity and working capital. We expect our research and development expenses to significantly increase in connection with our additional planned clinical trials for our product and product candidates, including our ongoing pivotal Phase 3 FIREFLY-2 trial of tovorafenib as a potential front-line therapy in pLGG, our post-marketing commitments and requirements for OJEMDA, our Phase 1a/b trial of DAY301 targeting PTK7 and development of and subsequent Investigational New Drug Applications, or INDs, for any future product candidates we may choose to pursue. In October 2023, the U.S. Food and Drug Administration, or FDA, accepted our New Drug Applications, or NDAs, and granted priority review for OJEMDA as a monotherapy in relapsed or refractory pLGG. On April 23, 2024, the FDA approved the NDAs for OJEMDA for use in the treatment of patients 6 months of age and older with relapsed or refractory pLGG harboring a BRAF fusion or rearrangement, or BRAF V600 mutation. We will incur significant sales, marketing and outsourced manufacturing expenses in connection with the commercialization of OJEMDA, or our product candidate, DAY301, if marketing authorization is received. We have also incurred, and will continue to incur, additional costs associated with operating as a public company.

As a result, we expect to continue to incur significant and increasing net losses for the foreseeable future. Because of the numerous risks and uncertainties associated with developing pharmaceutical products, we are unable to predict the extent of any future losses or when we will become profitable, if at all. Even if we do become profitable, we may not be able to sustain or increase our profitability on a quarterly or annual basis. In addition, we expect our financial condition and operating results to fluctuate significantly from quarter-to-quarter and year-to-year due to a variety of factors, many of which are beyond our control. Accordingly, you should not rely upon the results of any quarterly or annual periods as indications of future operating performance.

Our near-term revenues are highly dependent on the successful commercialization of OJEMDA, which received marketing approval in April 2024 from the FDA for the treatment of patients 6 months of age and older with relapsed or refractory pLGG harboring a BRAF fusion or rearrangement, or BRAF V600 mutation. To the extent that OJEMDA is not commercially successful, our business, financial condition and results of operations would be materially and adversely affected and the price of our common stock would decline.

Our future success is highly dependent on our ability to timely complete successful clinical trials, obtain marketing authorization for, and then successfully commercialize, OJEMDA and our product candidates. OJEMDA is our only drug that has been approved for sale and it has only been approved for the treatment of patients 6 months of age and older with relapsed or refractory pLGG harboring a BRAF fusion or rearrangement, or BRAF V600 mutation. Prior to OJEMDA, we have not, as an organization, launched or commercialized a product, and there is no guarantee that we will be able to do so successfully with OJEMDA. There are numerous examples of unsuccessful product launches and failures to meet high expectations of market potential. We are focusing a significant portion of our activities and resources on OJEMDA, and we believe our near-term revenues are highly dependent on, and a meaningful portion of the value of our company relates to, our ability to successfully commercialize OJEMDA in the United States. If the launch or commercialization of OJEMDA is unsuccessful or perceived as disappointing, our stock price could decline significantly and the long-term success of the product and our company could be harmed.

The success of OJEMDA will depend on several factors, including the following:

- making arrangements with third-party manufacturers, or establishing manufacturing capabilities, for both clinical and commercial supplies of OJEMDA and ensuring a resilient, effective supply chain that produces supply that outpaces demand;
- implementing marketing, pricing and reimbursement strategies, as well as adequate demand forecasts for supply and sales planning;
- establishing sales, marketing and distribution capabilities for OJEMDA, whether alone or in collaboration with others in a market where promotional sales approaches are rapidly moving to digital platforms and access of sales representatives to major institutions remains uncertain;
- acceptance of OJEMDA by patients, physicians, the medical community and third-party payors underpinned by adequate health economic data and a meaningful value proposition;
- obtaining and maintaining third-party payor coverage and adequate reimbursement in both public and private payor spaces across multiple countries;
- effectively competing with other therapies, including those that have not yet entered the market;
- effectively competing with other companies in the pharmaceutical and biotechnology industries, which are characterized by rapidly advancing technologies, intense competition and a strong emphasis on proprietary and novel products and product candidates;
- obtaining appropriate support from patient advocacy organizations;
- effectively shaping the market in the early years following launch to help providers understand a new way of thinking about treating relevant patients;
- whether our patents will be sufficient to prevent generic competition for OJEMDA after our orphan drug exclusivity expires;
- the successful completion of any required or committed post-marketing studies and available funding to perform any such post-marketing requirements or post-marketing commitments;
- maintaining a continued acceptable safety profile of the products following approval.

Many of these factors are beyond our control, and if we cannot address any of them in a timely manner or at all, we could experience significant delays or an inability to successfully commercialize OJEMDA and our product candidates, which would materially harm our business.

Our ability to generate revenue and achieve profitability depends significantly on our ability to achieve several objectives relating to the development and commercialization of OJEMDA and our product candidates.

Our business depends entirely on the successful commercialization of OJEMDA and development of our product candidates. We are early in our development efforts for other indications, and our product tovorafenib is currently in a pivotal Phase 3 clinical trial as a potential front-line therapy in pLGG. Our product candidate, DAY301, is in the earlier stages of development and is not approved for sale in any jurisdiction. There can be no assurance that tovorafenib and DAY301 or any future product candidates we develop, if any, will achieve success in their ongoing clinical trials or obtain marketing authorization.

Our ability to generate future revenue at the levels or timing we expect and achieve profitability depends on several factors, including, but not limited to, our ability to:

- successfully market and sell OJEMDA while maintaining full compliance with applicable federal and state laws, rules and regulations;
- complete a successful pivotal Phase 3 FIREFLY-2 trial with tovorafenib that achieves a competitive, clinically meaningful and generally well-tolerated target product profile for the front-line treatment of pLGG;
- complete a successful Phase 1a/b trial of DAY301;
- initiate and successfully complete all safety, pharmacokinetic and other studies required to support Ipsen to obtain foreign marketing authorization for OJEMDA as a treatment for patients with pLGGs;
- initiate and complete additional, successful late-stage clinical trials that meet their clinical endpoints;
- obtain favorable results from our clinical trials and apply for and obtain marketing authorizations for DAY301 from applicable regulatory authorities, including NDAs from the FDA, and maintaining such approvals;
- establish licenses, collaborations or strategic partnerships that allow for the commercialization of OJEMDA and our product candidates and/or may increase the value of our programs;
- successfully commercialize OJEMDA, DAY301, and any future product candidates we may develop, if approved, by building and maintaining a sales force and/or entering into collaborations with third parties;
- satisfy any post-marketing requirements imposed by, or post-marketing commitments made to, applicable regulatory authorities;
- demonstrate an acceptable safety profile of our product and our product candidate, DAY301, and continue to maintain a continued acceptable safety profile following marketing authorization, if any;
- identify, assess and develop new product candidates;
- establish and maintain patent and trade secret protection, statutory exclusivities and other intellectual property protections for our products;
- obtain, maintain, protect and defend our intellectual property portfolio, including any necessary licenses from third parties;
- address any competing therapies and technological and market developments;
- achieve market acceptance of our product candidate, DAY301, if approved, with patients, the medical community and third-party payors, both in the United States and internationally; and
- attract, hire and retain qualified personnel and management.

To become and remain profitable, we must succeed in developing and commercializing products that generate significant revenue. This will require us to be successful in a range of challenging activities, including completing clinical trials for OJEMDA and our product candidates, acquiring additional product candidates, establishing arrangements with third parties for the manufacture of clinical supplies of our product candidates, obtaining marketing authorization for our product candidates, obtaining and retaining patents, trade secrets, statutory exclusivities, and other intellectual property protections and marketing and selling products for which we may obtain marketing authorization, if any. We are in the early stages of most of these activities. We may never succeed in these activities and, even if we do, may never generate revenues that are significant enough to achieve profitability.

In cases where we are successful in obtaining marketing authorizations to market one or more of our product candidates, our revenue will be dependent, in part, upon the size of the markets in the territories for which we gain marketing authorizations, the pricing for the product, the duration of treatment with our product, the adoption of our product in treatment guidelines and by prescribers, the ability to obtain coverage and reimbursement and whether we own the commercial rights for that territory. If the number of our addressable patients is not as significant as we estimate, the approved indication is narrower than expected or the treatment population is narrowed by competition, physician choice, payor decisions or treatment guidelines, we may not generate significant revenue from sales of such products, even if approved.

If we decide to, or are required by the FDA or regulatory authorities in other jurisdictions to, perform studies or clinical trials in addition to those currently expected, or to modify ongoing or planned clinical trials, or if there are any delays in establishing appropriate manufacturing arrangements for, in initiating or completing our current and planned clinical trials for or in the development of, any of our product candidates, our expenses could increase significantly and profitability could be further delayed.

Our failure to become and remain profitable could depress the value of our company and could impair our ability to raise capital, expand our business, maintain our research and development efforts, diversify our product offerings or even continue our operations. A decline in the value of our company could also cause you to lose all or part of your investment.

We may require additional capital to finance our operations and achieve our goals. If we are unable to raise capital when needed or on terms acceptable to us, we may be forced to delay, reduce or eliminate our research or product development programs, any future commercialization efforts or other operations.

Developing pharmaceutical products, including conducting preclinical studies and clinical trials, is a very time-consuming, expensive and uncertain process that takes years to complete. Our operations have consumed substantial amounts of cash since inception, and we expect our expenses to increase in connection with our ongoing activities, particularly as we commercialize our product, OJEMDA, and advance product candidate, DAY301, and any future product candidates through clinical development. We expect increased expenses as we continue our research and development, initiate additional clinical trials, seek to expand our product pipeline, seek marketing authorization for our programs and future product candidates, if any, and invest in our organization. In addition, we expect to incur significant expenses related to the product manufacturing, marketing, sales and distribution of OJEMDA and, if we obtain marketing authorization, for our product candidate, DAY301. Furthermore, we have incurred and will continue to incur additional costs associated with operating as a public company, such as acquiring and retaining experienced personnel, developing new information technology systems and other costs associated with being a public company. Also, we expect to experience ongoing and additional costs related to preparing and filing patent applications, maintaining our intellectual property and potentially expanding our office facilities. Accordingly, we will need to obtain additional funding in connection with our continuing operations.

We had \$451.6 million in cash, cash equivalents and short-term investments as of September 30, 2025. Based on our cash, cash equivalents and short-term investments, as of September 30, 2025, we estimate that our current liquidity will be sufficient to satisfy our capital requirements at least twelve months after the date that this Quarterly Report is filed. We have based this estimate on assumptions that may prove to be wrong, and we could use our capital resources sooner than we currently expect. Changes beyond our control may occur that would cause us to use our available capital before that time, including changes in and progress of our drug development activities and changes in regulation. Our future capital requirements will depend on many factors, including:

- the progress, timing and results of preclinical studies and clinical trials for our current or any future product candidates;
- the extent to which we develop, in-license or acquire other pipeline product candidates or technologies;
- the number and development requirements of current or future product candidates that we may pursue, and other indications for our current product candidates that we may pursue;
- the costs, timing and outcome of obtaining marketing authorization for our current or future product candidates or the modification of ongoing or planned clinical trials;
- the successful development of and marketing authorization for any complementary or companion diagnostics that may be useful to or necessary for the commercialization of OJEMDA and our product candidates;
- the scope and costs of making arrangements with third-party manufacturers, or establishing manufacturing capabilities, for both clinical and commercial supplies of our current or future product candidates;
- the costs involved in growing our organization to the size needed to allow for the research, development and potential commercialization of our current or future product candidates;
- to the extent we pursue strategic collaborations, including collaborations to commercialize OJEMDA, DAY301 or any of our future pipeline products and product candidates, if any, our ability to establish and maintain collaborations on favorable terms, if at all, as well as the timing and amount of any milestone or royalty payments we are required to make or are eligible to receive under such collaborations or our current licenses;
- the cost associated with commercializing any approved products and product candidates, including establishing sales, marketing, market access and distribution capabilities;
- the cost associated with completing any post-marketing studies or trials requested or required by the FDA or other regulatory authorities, including for OJEMDA;
- the revenue, if any, received from commercial sales of OJEMDA, DAY301 or any of our future product candidates, if approved, or any other future pipeline product candidates that receive marketing authorization;
- the costs of preparing, filing and prosecuting patent applications, maintaining and enforcing our intellectual property rights and defending intellectual property-related claims that we may become subject to, including any litigation costs and the outcome of such litigation; and
- the costs associated with potential product liability claims, including the costs associated with obtaining insurance against such claims and with defending against such claims.

We may require additional capital to complete our planned clinical development programs for our current product candidates to obtain marketing authorization, and we anticipate needing to raise additional capital to complete the development of our product candidates.

Our ability to raise additional funds will depend on financial, economic and market conditions and other factors, over which we may have no or limited control. If adequate funds are not available on commercially acceptable terms when needed, we may be forced to delay, reduce or terminate the development or commercialization of all or part of our research programs or products and product candidates or we may be unable to take advantage of future business opportunities. Furthermore, any additional capital-raising efforts may divert our team's attention from their day-to-day activities, which may adversely affect our business, including our ability to develop and commercialize our current and future product candidates, if approved. Changing circumstances, some of which may be beyond our control, could cause us to consume capital significantly faster than we currently anticipate, and we may need to seek additional funds sooner than planned.

We may be required to obtain further funding through public or private equity financings, debt financings, collaborative agreements, licensing arrangements or other sources of financing, which may dilute our stockholders or restrict our operating activities. We do not have any committed external source of funds. We have entered into an equity distribution agreement, or the Equity Distribution Agreement, with Piper Sandler & Co. and JonesTrading Institutional Services LLC, as sales agents, relating to the issuance and sale of shares of our common stock for an aggregate offering price of up to \$250.0 million under an at-the-market offering program, or the ATM. No shares of our common stock have been sold under the ATM as of September 30, 2025. To the extent that we raise additional capital through the sale of equity or convertible debt securities, including pursuant to the ATM, each investor's ownership interests will be diluted, and the terms may include liquidation or other preferences that adversely affect each investor's rights as a stockholder. Debt financing may result in imposition of debt covenants, increased fixed payment obligations or other restrictions that may affect our business. If we raise additional funds through upfront payments or milestone payments pursuant to strategic collaborations with third parties, we may have to relinquish valuable rights to our product candidates or grant licenses on terms that are not favorable to us. Our ability to raise additional funds may be adversely impacted by potential worsening global economic conditions and disruptions to, and volatility in, the credit and financial markets in the United States and worldwide resulting from inflation, changes in interest rates, changes in domestic and foreign currency rates, significant political, trade or regulatory developments, global regional conflicts, public health epidemics or otherwise.

Our failure to raise capital as and when needed or on acceptable terms would have a negative impact on our financial condition and our ability to pursue our business strategy, and we may have to delay, reduce the scope of, suspend or eliminate one or more of our research or drug development programs, clinical trials or future commercialization efforts.

Risks Related to Development and Commercialization of OJEMDA and our Product Candidates

Clinical trials are very expensive, time-consuming and difficult to design and implement, and involve uncertain outcomes. Furthermore, results of earlier preclinical studies and clinical trials may not be predictive of results of future preclinical studies or clinical trials. OJEMDA and our product candidates may not have favorable results in later clinical trials, if any, and not all of our product candidates will receive marketing authorization. If we fail to demonstrate the safety and effectiveness of our product candidates, our reputation may be harmed and our business will suffer.

The risk of failure for our product candidates is high. It is impossible to predict when or if our product candidates will prove effective or safe in humans or if our product candidates will receive marketing authorization. To obtain the requisite marketing authorizations to market and sell our product candidates, we must demonstrate through extensive preclinical studies and clinical trials that our product candidates are safe and effective in humans for use in each target indication. Clinical testing is expensive and can take many years to complete, and the outcome is inherently uncertain. Failure can occur at any time during the clinical trial process.

In addition, the results of preclinical studies and earlier clinical trials may not be predictive of the results of later-stage preclinical studies or clinical trials. We have limited clinical data for our product candidates. Products and product candidates in later stages of clinical trials may fail to show similar or desired safety and efficacy traits despite having progressed through preclinical and earlier stage clinical trials.

In some instances, there can be significant variability in safety or efficacy results between different clinical trials of the same product or product candidate due to numerous factors, including changes in clinical trial procedures set forth in protocols, differences in the size and type of the patient populations, adherence to the dosing regimen and other clinical trial protocols and the rate of discontinuation among clinical trial participants.

If we fail to produce positive results in our planned clinical trials of any of our product candidates, the development timeline and marketing authorization and commercialization prospects for our product candidates, and, correspondingly, our business and financial prospects, would be materially and adversely affected.

OJEMDA has only been studied in a limited number of patients. Following commercial launch, OJEMDA is now available to a much larger number of patients, and we do not know whether the results of OJEMDA's use in such larger number of patients will be consistent with the results from our clinical studies.

OJEMDA has been administered only to a limited number of patients in clinical studies. While the FDA granted accelerated approval of OJEMDA based on the data included in the NDAs, we do not know whether the real-world safety and effectiveness of the product will be consistent with the safety and effectiveness profile seen in the clinical studies. New data relating to OJEMDA, including from adverse events reports and our post-marketing commitments in the United States, and from other ongoing clinical studies, may result in changes to the product label and may adversely affect sales, or result in withdrawal of OJEMDA from the market. If any of these actions were to occur, it could result in significant expense and delay and/or limit our ability to generate future sales revenues in line with our expectations.

We may rely on data from investigator-initiated studies, as we did for the Phase 1 clinical trial, and we do not control the trial operations or reporting of the results of such trials.

From time to time, we may rely on certain clinical data from investigator-sponsored clinical studies, and we do not control the trial operations or reporting of the results of such trials. This was the case for the initial Phase 1 study for our product, OJEMDA, which was run as an investigator-initiated, multi-center trial in patients with relapsed or refractory pLGG that is being conducted by the Dana Farber Cancer Institute in collaboration with the Pacific Pediatric Neuro-Oncology Consortium, or PNOC. The last data reported from that trial was in January 2023. It is possible that additional data, when reported, will not demonstrate similar results. We have no control over the timing of such clinical data announcements. Our pivotal Phase 2 FIREFLY-1 trial OJEMDA is a Day One-sponsored trial. In addition, in later-stage clinical trials, we will likely be subject to more rigorous statistical analyses than in completed earlier stage clinical trials. A number of companies in the pharmaceutical industry have suffered significant setbacks in later-stage clinical trials due to lack of efficacy or adverse safety profiles, notwithstanding promising results in earlier trials, and we cannot be certain that we will not face similar setbacks. Moreover, preclinical and clinical data are often susceptible to varying interpretations and analyses, and many companies that have believed their product candidates performed satisfactorily in preclinical studies and clinical trials have nonetheless failed to obtain marketing authorization for their product candidates.

Furthermore, we do not control the design or administration of investigator-sponsored trials, nor the submission or approval of any IND or foreign equivalent required to conduct these trials, and the investigator-sponsored trials could, depending on the actions of such third parties, jeopardize the validity of the clinical data generated, identify significant concerns with respect to our product candidates that could impact our findings or clinical trials and adversely affect our ability to obtain marketing authorization from the FDA or other applicable regulatory authorities. To the extent the results of this or other investigator-sponsored trials are inconsistent with, or different from, the results of our planned company-sponsored trials or raise concerns regarding our product candidates, the FDA or a foreign regulatory authority may question the results of the company-sponsored trial or subject such results to greater scrutiny than it otherwise would. In these circumstances, the FDA or such foreign regulatory authorities may require us to obtain and submit additional clinical data, which could delay clinical development or marketing authorization of our product candidates. While investigator-sponsored trials could be useful to inform our own clinical development efforts, we do not control the data or timing of data releases for investigator-sponsored trials, and there is no guarantee that we will be able to use the data from these trials to form the basis for marketing authorization of our product candidates.

Our clinical trials may be suspended, delayed or fail to adequately demonstrate the safety and effectiveness of OJEMDA and our product candidates, which would prevent or delay development, marketing authorization and commercialization.

Before obtaining marketing authorization from the FDA for our product candidates or comparable foreign regulatory authorities for the sale of OJEMDA and our product candidates, we must demonstrate through lengthy, complex and expensive clinical trials that our product candidates are both safe and effective for use in each target indication. Clinical testing is expensive, difficult to design and implement, can take many years to complete and its ultimate outcome is uncertain. Failure can occur at any time during the clinical trial processes and for any number of reasons, and, because our product candidates are in earlier stages of development, there is a high risk of failure and we may never succeed in developing marketable products.

We may experience numerous challenges and unforeseen events during, or as a result of, clinical trials that could delay or prevent receipt of marketing authorization for our product candidates, or our ability to successfully commercialize OJEMDA or our product candidates, including:

- the FDA or other regulators refusing to permit our clinical studies to proceed or placing studies on hold before or after the studies begin;
- a failure to demonstrate that the dose for a product candidate has been optimized;
- failure of our product candidates in clinical trials to demonstrate important functional, quality, or patient-reported outcomes;

- changes in the competitive landscape causing clinical trial enrollment challenges or preventing or delaying marketing authorization in one or several subsets studied in our programs, including in relapsed or front-line pLGG;
- receipt of feedback from regulatory authorities that requires us to modify the design of our clinical trials;
- negative or inconclusive clinical trial results that may require us to conduct additional clinical trials or abandon certain research and/or drug development programs;
- the number of patients required for clinical trials being larger than anticipated, enrollment in these clinical trials being slower than anticipated or participants dropping out of these clinical trials at a higher rate than anticipated;
- unanticipated delays in our preclinical studies or clinical trials;
- third-party contractors failing to comply with regulatory requirements, including Good Clinical Practice, or GCP, regulations, or meet their contractual obligations to us in a timely manner, or at all;
- the suspension or termination of our clinical trials for various reasons, including non-compliance with regulatory requirements or a finding that our product candidates have undesirable side effects or other unexpected characteristics or risks;
- the cost of clinical trials of our product candidates being greater than anticipated;
- the supply or quality of our product candidates or other materials necessary to conduct clinical trials of our product candidates being insufficient or inadequate;
- failure of our clinical trials to demonstrate the safety or effectiveness of our product candidates;
- regulators revising the requirements for approving our product candidates; and
- receipt of feedback from regulatory authorities that would require us to include data from additional patients or longer term efficacy and safety data.

We may also face unanticipated regulatory hurdles in our drug development program that may require additional data generation or delay our existing or planned trials and the timing of applications for marketing authorization. For instance, we may make formulation or manufacturing changes to our product candidates, in which case we may need to conduct additional preclinical studies to bridge our modified product candidates to earlier versions. Additionally, the FDA may determine that it has questions or concerns about our trials and may not permit our proposed clinical studies to move forward by imposing a partial or full clinical hold.

Further, we, the FDA or an institutional review board, or IRB, may suspend our clinical trials at any time if it appears that we or our collaborators are failing to conduct a trial in accordance with regulatory requirements, including GCP regulations, that we are exposing participants to unacceptable health risks or if the FDA finds deficiencies in our INDs or the conduct of these trials. Therefore, we cannot predict with any certainty the schedule for commencement and completion of future clinical trials.

We may also conduct clinical trials in foreign countries, which presents additional risks that may delay completion of our clinical trials. These risks include the failure of enrolled patients in foreign countries to adhere to clinical protocol as a result of differences in healthcare services or cultural customs, managing additional administrative burdens associated with foreign regulatory schemes, as well as political and economic risks relevant to such foreign countries. Further, data from trials conducted outside of the United States may be subject to additional scrutiny by the FDA, which may require that additional U.S. data be generated.

Because some of our product candidates are targeted towards the pediatric population, we may face additional hurdles and be subjected to greater scrutiny by regulatory agencies. Trials involving pediatric populations can be difficult to conduct, can be quite costly and, like other clinical trials, may not yield the anticipated results. In addition, pediatric studies are more dependent on a smaller number of specialized clinical trial sites, which in turn can limit site availability and make the trials more expensive to conduct. In addition, as interest in pediatric indications grows as a result of the Research to Accelerate Cures and Equity (RACE) for Children Act and other market forces, trial recruitment may become even more difficult due to competition for eligible patients. Moreover, it may be challenging to ensure that pediatric or adolescent patients adhere to clinical trial protocols. Our inability to enroll a sufficient number of pediatric patients for our clinical trial could result in significant delays, require us to abandon one or more clinical trials altogether, impact our ability to raise additional capital and delay or prevent our ability to obtain necessary marketing authorizations for any drug product candidate.

We cannot predict the outcome of our clinical trials, nor can we guarantee that the data we generate from our clinical trials will be acceptable to regulatory authorities so as to support marketing authorization.

The outcome of clinical trials is uncertain, and, because our product candidates are in earlier stages of development, there is a significant risk of failure. If we complete our clinical trials but the results of our clinical trials are inconclusive or only modestly positive, if there are safety concerns or serious adverse events associated with our product candidates or if our clinical trials are delayed or require unplanned changes, we may:

- incur additional, unplanned drug development and/or commercialization costs;
- be delayed in obtaining or unable to obtain marketing authorization;
- be required to perform additional clinical trials to support approval;
- obtain approval for indications or patient populations that are not as broad as intended or desired or may have contraindications, limitations of use or other restrictions that affect the market for the product;
- obtain marketing authorization with labeling that includes safety warnings, a risk evaluation and mitigation strategy, or REMS, and/or other restrictions on distribution or use that could affect market access;
- be subject to additional post-marketing testing requirements or commitments;
- have regulatory authorities withdraw, or suspend, their approval of the drug or impose post-marketing safety labeling changes or a REMS;
- be subject to civil or criminal investigations and litigation; or
- experience damage to our reputation.

If we experience delays in the commencement or completion of our clinical trials, or if we terminate a clinical trial prior to completion, the commercial prospects of our product candidates could be negatively impacted, and our ability to generate revenues from our product candidates may be delayed or eliminated entirely.

In addition, principal investigators for our clinical trials may serve as scientific advisors or consultants to us from time to time and receive compensation in connection with such services. Under certain circumstances, we may be required to report some of these relationships to the FDA or comparable foreign regulatory authorities. The FDA or a comparable foreign regulatory authority may conclude that a financial relationship between us and a principal investigator has created a conflict of interest or has affected the conduct or interpretation of the study. The FDA or a comparable foreign regulatory authority may therefore question the integrity of the data generated at the applicable clinical trial site and the utility of the clinical trial itself may be jeopardized. This could result in a delay in approval, or rejection, of our marketing applications by the FDA or comparable foreign regulatory authority, as the case may be, and may ultimately lead to the denial of marketing authorization of one or more of our product candidates.

If we experience delays or difficulties in enrolling patients in our ongoing or planned clinical trials, we may be delayed in or prevented from obtaining necessary marketing authorization for any or all of our product candidates.

We may not be able to initiate or continue our ongoing or planned clinical trials for our product candidates if we are unable to identify and enroll a sufficient number of eligible patients to participate in these trials as required by the FDA or comparable foreign regulatory authorities. In our OJEMDA program, we utilize genomic profiling of patients' tumors to identify suitable patients for recruitment into our clinical trials. We cannot be certain (i) how many patients will have the requisite alterations for inclusion in our clinical trials, (ii) that the number of patients enrolled in each program will suffice for marketing authorization or (iii) whether each specific BRAF mutation targeted will be included in the approved drug labeling. If our strategies for patient identification and enrollment prove unsuccessful, we may have difficulty enrolling or maintaining patients appropriate for our product candidates. Patient enrollment is also affected by other factors, including:

- severity of the disease under investigation;
- our ability to recruit clinical trial investigators of appropriate competencies and experience;
- the incidence and prevalence of our target indications;
- clinicians' and patients' awareness of, and perceptions as to, the potential advantages and risks of our product candidates in relation to other available therapies, including any new drugs that may be approved for the indications we are investigating;
- the availability and capacity of clinical researchers to conduct our clinical trials;
- the availability, expertise and selection of contract research organizations, or CROs, to manage operations related to clinical trial enrollment;
- competing studies or trials with similar eligibility criteria;
- any invasive procedures that may be required to enroll patients and to obtain evidence of the product candidate's performance during the clinical trial;
- availability and efficacy of approved medications for the disease under investigation;

- ongoing shortages of chemotherapy standard of care, which may be used in the control arm of certain of our clinical trials, including FIREFLY-2;
- eligibility criteria defined in the protocol for the trial in question;
- the size and nature of the patient population required for analysis of the trial's primary endpoints;
- efforts to facilitate timely enrollment in clinical trials;
- whether we are subject to a partial or full clinical hold on any of our clinical trials;
- reluctance of physicians or patient advocacy organizations to encourage patient participation in clinical trials;
- the ability to monitor patients adequately during and after treatment;
- our ability to obtain and maintain patient consents; and
- proximity and availability of clinical trial sites for prospective patients.

In addition, the conditions for which we currently plan to evaluate our product candidates are orphan or rare diseases with limited patient pools from which to draw for clinical trials. The eligibility criteria of our clinical trials, once established, will further limit the pool of available trial participants. Further, some of our competitors currently have ongoing clinical trials for product candidates that would treat the same patients as our clinical product candidates, and patients who would otherwise be eligible for our clinical trials may instead enroll in clinical trials of our competitors' product candidates. Moreover, if any of our competitors receive FDA approval for a product, it may limit our ability to enroll patients in our clinical trials if they decide to seek treatment with an approved product. For example, in March 2023, Novartis received approval for dabrafenib in combination with trametinib, which could in the future limit our ability to enroll patients whose tumors harbor V600E mutations in clinical trials for OJEMDA.

Our inability to enroll and maintain a sufficient number of patients for our clinical trials would result in significant delays or may require us to abandon one or more clinical trials or clinical programs altogether. There may be competing trials, as well as the limited bandwidth of pediatric oncology institutions for running trials, which can lead to the prioritization of certain trials, resulting in delays in our clinical trials. In addition, because our product candidates are initially targeted to pediatric populations, we may face additional challenges. For example, parents may be reluctant to enroll their children in our clinical trials or may decide to withdraw their children from our clinical trials to pursue other therapies.

Preliminary, interim, initial and topline data from our clinical trials that we announce or publish from time to time may change as more patient data become available and are subject to audit and verification procedures that could result in material changes in the final data.

From time to time, we may publicly disclose preliminary, interim or topline data from our clinical trials. These updates are based on a preliminary analysis of then-available data, and the results and related findings and conclusions are subject to change following a more comprehensive review of the data related to the particular study. Additionally, interim data from clinical trials that we may complete are subject to the risk that one or more of the clinical outcomes may materially change as patient enrollment continues and more patient data become available. Therefore, positive interim or initial results in any ongoing clinical trial may not be predictive of such results in the completed study. Initial or topline data also remain subject to audit and verification procedures that may result in the final data being materially different from the preliminary data we previously published.

Further, others, including regulatory agencies, may not accept or agree with our assumptions, estimates, calculations, conclusions or analyses or may interpret or weigh the importance of data differently, which could impact the value of the particular program, the approvability or commercialization of the particular product candidate or product and our company in general. For example, our FIREFLY-1 clinical trial was designed to use the Response Assessment for Neuro-Oncology – High Grade Glioma, or RANO-HGG, to measure the primary endpoint of overall response rate, or ORR, in alignment with the FDA, with ORR using Response Assessment for Pediatric Neuro-Oncology – Low-Grade Glioma, or RAPNO-LGG, as a secondary endpoint. Following discussions with the FDA and the March 2023 approval of dabrafenib, in combination with trametinib in BRAF V600E pLGG, we initially structured the primary endpoint in our FIREFLY-2/LOGGIC trial to be assessed using the Response Assessment for Neuro-Oncology Low-Grade Glioma, or RANO-LGG, and have included RANO-LGG as an exploratory endpoint in FIREFLY-1. Following further feedback from the FDA during review of the NDAs for OJEMDA, in June 2024 we updated the structure of the primary endpoint in our FIREFLY-2/LOGGIC trial to be assessed using the RAPNO-LGG criteria.

In addition, the information we choose to publicly disclose regarding a particular study or clinical trial is based on what is typically extensive information, and you or others may not agree with what we determine is the material or otherwise appropriate information to include in our disclosure, and any information we determine not to disclose may ultimately be deemed significant with respect to future decisions, conclusions, views, activities or otherwise regarding a particular drug, drug candidate or our business. If the topline data that we report differ from actual results, or if others, including regulatory authorities, disagree with the conclusions reached, our

ability to obtain approval for, and commercialize, our product candidates may be harmed, which could harm our business, operating results, prospects or financial condition.

We face substantial competition which may result in others discovering, developing or commercializing products before or more successfully than we do.

The pharmaceutical and biotechnology industries are characterized by rapidly advancing technologies, intense competition and a strong emphasis on proprietary and novel products and product candidates. Our competitors have developed, are developing or may develop products, product candidates and processes competitive with our product candidates. Any products or product candidates that we successfully develop and commercialize, including OJEMDA, may compete with existing therapies and new therapies that may become available in the future. We believe that a significant number of competing product candidates are currently under development, and may become commercially available in the future, for the treatment of conditions for which we are developing, or may in the future develop, product candidates. In addition, our product candidates may need to compete with drugs that are prescribed off-label to treat the indications for which we seek approval. This may make it difficult for us to replace existing therapies with our product candidates.

We also compete with these organizations to recruit and retain qualified scientific, management and sales and commercial and marketing personnel, which could negatively affect our level of expertise and our ability to execute our business plan. We will also face competition in establishing clinical trial sites and patient registration for clinical trials, as well as in acquiring technologies complementary to, or necessary for, our programs.

In particular, there is intense competition in the field of oncology. We have competitors both in the United States and internationally, including major multinational pharmaceutical companies, established biotechnology companies, specialty pharmaceutical companies, emerging and start-up companies, universities and other research institutions.

We expect to face competition from existing products and products in development for each of our programs. Drug discovery efforts focused on V600 mutations have led to clinical success in some cancers. Three BRAF inhibitors have been approved by the FDA for the treatment of tumors containing V600E or V600K mutations. These first-generation BRAF inhibitors, known more generally as Type I RAF inhibitors, are vemurafenib, marketed as Zelboraf® by Genentech; dabrafenib, marketed as Tafinlar® by Novartis; and encorafenib, marketed as Braftovi® by Pfizer. Dabrafenib, in combination with trametinib, marketed as Mekinist® by Novartis, has been approved for the treatment of adult and pediatric patients ≥ 6 years of age with unresectable or metastatic solid tumors with BRAF V600E mutation who have progressed following prior treatment and have no satisfactory alternative treatment options. This includes BRAF V600E pLGG, a subset of the greater RAF-altered pLGG clinical scope of the OJEMDA development program. We believe that current data indicates that the BRAF V600E subset represents 10%-20% of BRAF-altered pLGG, but additional epidemiologic data may emerge as more patients are profiled. Further, dabrafenib, in combination with trametinib, was granted full approval in the BRAF V600E pLGG indication in March 2023 to include the treatment of pediatric patients 1 year of age and older with low-grade glioma, or LGG, with a BRAF V600E mutation who require initial systemic therapy.

Five MEK inhibitors have been approved by the FDA. Three have been approved for the treatment of tumors containing BRAF V600E or V600K mutations, including cobimetinib, marketed as Cotellic® by Genentech; trametinib, marketed as Mekinist® by Novartis; and binimetinib, marketed as Mektovi® by Pfizer. Two have been approved for the treatment of pediatric patients, 2 years of age and older, with neurofibromatosis type 1, or NF1, who have symptomatic plexiform neurofibromas not amenable to complete resection, including selumetinib, marketed as Koselugo® by AstraZeneca and mirdametinib, marketed as Gomekli® by SpringWorks. While MEK inhibitors as monotherapy have been shown to be active in BRAF altered pLGG (both BRAF V600E mutant pLGG and BRAF fusion-driven pLGG), no MEK inhibitors have been approved by the FDA as a monotherapy for the treatment of patients with pLGG.

There are a number of next-generation BRAF inhibitors in clinical development. Fore Biotherapeutics (formerly NovellusDx) is developing the RAF dimer breaker plixorafenib (formerly FORE8394 or PLX-8394) in a Phase 2 trial in combination with cobicistat in patients with cancers harboring BRAF alterations. Black Diamond Therapeutics have the next-generation BRAF inhibitor BDTX-4933 in Phase 1 clinical trials in adult solid tumors (KRAS-mutant NSCLC and solid tumors with RAF/RAS-mutations). Jazz Pharmaceuticals and Redx Pharma's pan-RAF inhibitor JZP815 is currently being evaluated in a Phase 1 trial in adult solid tumors harboring alterations in the MAPK pathway. Erasca and Novartis' naporafenib, a pan-RAF inhibitor with a potential first-in-class and best-in-class profile in NRAS mutant melanoma and other RAS/MAPK pathway-driven tumors, is being evaluated in combination with trametinib, in a Phase 3 clinical trial in patients with NRAS-mutant melanoma. Nested Therapeutics has advanced NST-628, a pan-RAF/MEK "molecular glue" into a Phase 1 clinical trial. Pfizer's PF-07799933 (ARRY-440) is a brain-penetrant BRAF-selective monomer/dimer inhibitor that spares ARAF and CRAF, and is currently being evaluated in a phase 1 trial in adults with solid tumors.

With regard to the treatment of pLGG, some MEK inhibitors, some type I RAF inhibitors, and other targeted therapies have been studied, or are being studied, in academic investigator-initiated clinical trials, and in some regions may be being used in an off-label manner. The off-label use of these agents may represent competition for OJEMDA if it is approved and enters the market.

Pursuant to the MabCare License Agreement, we have the exclusive right to develop, manufacture and commercialize DAY301, a novel ADC targeting PTK7, worldwide, excluding Greater China. In January 2025, we cleared the first cohort (a single-patient accelerated titration cohort) in the Phase 1a portion of the DAY301 Phase 1a/b clinical trial. There are a few ADCs targeting PTK7 in clinical development. In February 2024, Profound Bio dosed its first patient in a Phase 1/2 Clinical Trial of PRO1107, a PTK7-targeted ADC with an auristatin payload. Profound Bio was acquired by Genmab A/S in May of 2024 and the program was renamed to GEN1107. In September 2025, Genmab announced that PRO1107 was being discontinued due to the overall benefit-risk balance of the program. In May 2024, Sichuan Kelun Pharmaceutical Research Institute also listed a Phase 1 study of SKB51, a PTK7-targeted ADC with a topoisomerase-1 payload, in adults with advanced or metastatic solid tumors. Eli Lilly and Company is also conducting a clinical trial, initiated in July 2025, for LY4175408, a PTK7 targeted ADC with an exatecan payload. Notable PTK7 in preclinical development include Whitehawk's HWK-007, IDEAYA's IDE-034 and Biocytogen's BCG003 and BCG017.

Many of our competitors, either alone or with their collaborators, have significantly greater financial resources, established presence in the market and expertise in research and development, manufacturing, preclinical and clinical testing, obtaining marketing authorizations and reimbursement and marketing approved products than we do.

Large pharmaceutical and biotechnology companies, in particular, have extensive experience in clinical testing, obtaining marketing authorizations, recruiting patients and manufacturing biotechnology product candidates. These companies also have significantly greater research, marketing and sales capabilities than we do and may also have product candidates that have been approved or are in late stages of development, and collaborative arrangements in our target markets with leading companies and research institutions. Established pharmaceutical and biotechnology companies may also invest heavily to accelerate discovery and development of novel compounds or to in-license novel compounds that could make the product candidates that we develop obsolete. Smaller or early-stage companies may also prove to be significant competitors, particularly through collaborative arrangements with large and established companies, as well as in acquiring technologies complementary to, or necessary for, our programs.

As a result of all of these factors, our competitors may succeed in obtaining approval from the FDA or comparable foreign regulatory authorities or in discovering, developing and commercializing product candidates in our field before we do, which could result in our competitors establishing a strong market position before we are able to enter the market with a particular product or product candidate or could make our development more complicated.

Our potential commercial opportunity could be reduced or eliminated if our competitors develop and commercialize products that are safer, more effective, have fewer or less severe side effects, are more convenient, have a broader label, are marketed more effectively, are more widely reimbursed or are less expensive than OJEMDA or our product candidates. Even if the product candidates we develop achieve marketing authorization, they may be priced at a significant premium over competitive products if any have been approved by then, resulting in reduced competitiveness. Technological advances or products developed by our competitors may render our technologies or product candidates obsolete, less competitive or not economical. If we are unable to compete effectively, our opportunity to generate future revenue from the sale of the product candidates we may develop, if approved, could be adversely affected.

Safety risks or other side effects associated with OJEMDA, DAY301 or any future products and product candidates we may develop could delay or preclude approval, cause us to suspend or discontinue clinical trials or abandon further development, limit the use of an approved product or result in significant negative consequences following marketing authorization, if any.

As is the case with pharmaceuticals generally, we have observed side effects and adverse events associated with our product, OJEMDA, and our product candidates. The most common side effects were rash, hair color changes, tiredness, viral infection, vomiting, headache, fever, decrease in growth velocity, dry skin, constipation, nausea, acne and upper respiratory tract infection.

Results of our ongoing and planned clinical trials could reveal a high and unacceptable severity and prevalence of side effects or unexpected characteristics. These side effects or unexpected characteristics may be subject to regulatory reporting requirements before and/or after approval. Undesirable side effects caused by OJEMDA or our product candidates could result in the delay, suspension or termination of clinical trials by us or regulatory authorities for a number of reasons. Furthermore, clinical trials by their nature utilize a sample of the potential patient population. With a limited number of subjects and limited duration of exposure, rare and severe side effects of OJEMDA or our product candidates or those of our competitors may only be uncovered with a significantly larger number of patients exposed to the drug.

Additionally, patients treated with OJEMDA and our product candidates have undergone, or may also be undergoing, medical, surgical, radiation and chemotherapy treatments, which can cause side effects or adverse events that are unrelated to OJEMDA or our product candidates but may still impact the success of our clinical trials. The inclusion of critically ill patients in our clinical trials may result in deaths or other adverse medical events due to other therapies or medications that such patients may be using or due to the gravity of such patients' illnesses. For example, it is expected that some of the patients to be enrolled in our future clinical trials will die or experience major clinical events either during the course of our clinical trials or after participating in such trials for non-treatment related reasons, which could impact development of OJEMDA, DAY301 or our other product candidates. If we elect or are required to delay, suspend or terminate any clinical trial, the commercial prospects of OJEMDA and our product candidates will be

harmed and our ability to generate product revenues from such product or product candidate will be delayed or eliminated. Serious adverse events, or SAEs, observed in clinical trials could hinder or prevent market acceptance of any approved products or reduce the duration of time that physicians expect to use our product in particular patients. Any of these occurrences may significantly harm our business, prospects, financial condition and results of operations.

Moreover, if OJEMDA or our product candidates are associated with undesirable side effects in clinical trials or have characteristics that are unexpected, we may elect to abandon or limit their development to more narrow uses or subpopulations in which the undesirable side effects or other characteristics are less prevalent, less severe or more acceptable from a risk-benefit perspective, which may limit the commercial expectations for our product candidates, if approved. We may also be required to modify our study plans based on findings in our clinical trials. Such side effects could also affect patient recruitment or the ability of enrolled patients to complete the trial. Many drugs that initially showed promise in early-stage testing have later been found to cause side effects that prevented further development. In addition, regulatory authorities may draw different conclusions, require additional testing to confirm these determinations, require more restrictive labeling or deny marketing authorization of the product candidate.

It is possible that, as we test OJEMDA or our product candidates in larger, longer and more extensive clinical trials, including with different dosing regimens, or as the use of our product candidates becomes more widespread following any marketing authorization, illnesses, injuries, discomforts and other adverse events that were observed, did not occur or went undetected in earlier trials, will be reported by patients. If such side effects become known later in development or upon approval, if any, such findings may significantly harm our business, financial condition, results of operations and prospects.

If any of our product candidates receive marketing authorization, and we or others later identify undesirable side effects caused by treatment with such drug, a number of potentially significant negative consequences could result, including:

- regulatory authorities may withdraw approval of the drug;
- we may be required to recall a product or change the way the drug is administered to patients;
- regulatory authorities may require additional warnings in the labeling, such as a contraindication or a boxed warning, or issue safety alerts, Dear Healthcare Provider letters, press releases or other communications containing warnings or other safety information about the product;
- we may be required to implement a REMS or create a medication guide outlining the risks of such side effects for distribution to patients;
- regulatory authorities may impose additional restrictions on the marketing or promotion of the particular product or the manufacturing processes for the product or any component thereof;
- we could be sued and held liable for harm caused to patients;
- we may be subject to regulatory investigations and government enforcement actions;
- the drug could become less competitive; and
- our reputation may suffer.

Any of these events could prevent us from achieving or maintaining market authorization or acceptance of our product candidates, if approved, and could significantly harm our business, financial condition, results of operations and prospects.

We may expend our limited resources to pursue a particular product or product candidate or indication and fail to capitalize on product candidates or indications that may be more profitable or for which there is a greater likelihood of success.

Because we have limited financial and managerial resources, we focus on research programs and products and product candidates that we identify for specific indications. As a result, we may forego or delay pursuit of opportunities with other product candidates or for other indications that later prove to have greater commercial potential. Our resource allocation decisions may cause us to fail to capitalize on viable commercial products or profitable market opportunities. Our spending on current and future research and development programs and products and product candidates for specific indications may not yield any commercially viable products. If we do not accurately evaluate the commercial potential or target market for a particular product candidate, we may relinquish valuable rights to that product candidate through collaboration, licensing or other royalty arrangements in cases in which it would have been more advantageous for us to retain sole development and commercialization rights to such product candidate.

The market opportunities for any products and product candidates we develop, if approved, may be limited to certain smaller patient subsets and may be smaller than we estimate them to be.

On April 23, 2024, the FDA approved the NDAs for the treatment of patients 6 months of age and older with relapsed or refractory pLGG harboring a BRAF fusion or rearrangement, or BRAF V600 mutation. We have commenced the commercial launch of

OJEMDA in the United States. There is no guarantee that OJEMDA or our product candidates will be approved for the front-line setting, and prior to any such approvals we may have to conduct additional clinical trials that may be costly, time-consuming and subject to risk.

Our projections of both the number of people who have the cancers we are targeting, as well as the subset of people with these cancers in a position to receive a particular line of therapy and who have the potential to benefit from treatment with OJEMDA and our product candidates, are based on our beliefs and estimates. For example, pLGG is a rare disease, and our projections of both the number of people who have this disease, as well as the subset of people with pLGG who have the potential to benefit from treatment with OJEMDA and our product candidates, are based on estimates. These estimates have been derived from a variety of sources, including scientific literature, surveys of clinics, patient foundations and market research. These estimates may prove to be incorrect. Additionally, new studies or information may change the estimated incidence or prevalence of the cancers that we are targeting, which could affect our eligibility for orphan designation for certain indications. The potentially addressable patient population for OJEMDA and our product candidates may be limited or may not be amenable to treatment with OJEMDA and our product candidates. Consequently, even if our product candidates are approved, the number of patients that may be eligible for treatment with our product candidates may turn out to be much lower than expected. Even if we obtain significant market share for our products, if the potential target populations are small, we may never achieve profitability without obtaining marketing authorization for additional indications, if at all.

Our clinical development activities are primarily focused on the development of targeted therapeutics for patients with genomically-defined cancers, which is a rapidly evolving area of science, and the approach we are taking to search and develop drugs is novel and may never lead to additional approved or marketable products.

The discovery and development of targeted therapeutics for patients with genomically-defined cancers is an emerging field, and the scientific discoveries that form the basis for our efforts to discover, identify and develop product candidates are relatively new. The scientific evidence to support the feasibility of developing product candidates based on these discoveries is both preliminary and limited. Although we believe, based on our products and product candidates' preclinical trial results and our clinical work, that the genomic alterations targeted by our programs are oncogenic drivers, clinical results may not confirm this hypothesis or may only confirm it for certain alterations or certain tumor types. The patient populations for OJEMDA and our product candidates are limited to those with specific target alterations and may not be completely defined but are substantially smaller than the general treated cancer population. In some cases, the target patient populations may not be completely defined. We will need to screen and identify appropriate patients with the targeted alterations. Successful identification of patients is dependent on several factors, including achieving certainty as to how specific alterations respond to OJEMDA and our product candidates and the ability to identify such alterations. Furthermore, even if we are successful in identifying patients, we cannot be certain that the resulting patient populations for each mutation will be large enough to allow us to successfully obtain approval for each mutation type and successfully commercialize OJEMDA and our product candidates and achieve profitability. In addition, even if our approach is successful in showing clinical benefit for RAF-driven cancers for our OJEMDA program, we may never successfully identify additional oncogenic alterations sensitive to OJEMDA in other MAPK-driven tumors. Therefore, we do not know if our approach of treating patients with genomically-defined cancers will be successful, and if our approach is unsuccessful, our business will suffer.

OJEMDA and our product candidate, DAY301, may not achieve adequate market acceptance among physicians, healthcare professionals, patients or their families, healthcare payors and others in the medical community necessary for commercial success.

Our product, OJEMDA, and product candidate, DAY301, if approved, may not achieve adequate market acceptance among physicians, healthcare professionals, patients or their families, healthcare payors and others in the medical community necessary for commercial success globally. The degree of market acceptance of any of our approved product candidates will depend on a number of factors, including:

- the efficacy, durability and safety profile as demonstrated in clinical trials compared to alternative treatments, in addition to functional, quality or patient-reported outcomes;
- the timing of market introduction of the product candidate and of any competitive products;
- the clinical indications for which a product candidate is approved;
- restrictions on the use of product candidates in the labeling approved by regulatory authorities, such as boxed warnings or contraindications in labeling, or REMS, which may not be required of alternative treatments and competitor products;
- the potential and perceived advantages of OJEMDA and our product candidates over alternative treatments;
- the cost of treatment in relation to alternative treatments and the cost/benefit ratios of each;
- the availability of coverage and adequate reimbursement by third-party payors, including government authorities, and timing of relevant formulary decision-making resulting in this coverage and reimbursement;

- relative convenience and ease of administration in relation to competition;
- the willingness of the target patient population (which may include willingness of our pediatric patients' parents) to try new therapies and undergo required diagnostic screening to determine treatment eligibility and of physicians to prescribe these therapies and diagnostic tests;
- the effectiveness of sales and marketing efforts and market access;
- unfavorable publicity relating to our product candidates; and
- the approval of other new therapies for the same indications.

If our product candidates are approved but do not achieve an adequate level of acceptance by physicians, hospitals, healthcare payors and patients, we may not generate or derive sufficient revenue from that product candidate and our financial results could be negatively impacted. With respect to OJEMDA specifically, successful commercialization globally will depend on negotiations with, and coverage, reimbursement, selection and/or acquisition decisions by, third-party payors, which we cannot predict. These decisions in turn may depend on value assessments conducted by various entities (e.g., formulary committees, such as pharmacy and therapeutics committees, healthcare systems and pharmacies, among others) that consider various factors (including the price of OJEMDA)—the outcomes of which we cannot predict.

Any products and product candidates we develop may become subject to unfavorable third-party coverage and reimbursement practices, as well as price restrictions.

The availability and extent of coverage and adequate reimbursement by third-party payors, including government health administration authorities, private health coverage insurers, managed care organizations and other third-party payors is essential for most patients to be able to afford expensive treatments. Sales of any of our products, including OJEMDA, and our product candidate, DAY301, should it receive marketing authorization, will depend substantially, both in the United States and internationally, on the extent to which the costs of such products and product candidates will be covered and reimbursed by third-party payors, as patients who are prescribed medicine for the treatment of their condition generally rely on third-party payors to reimburse all or part of the costs associated with their prescription drugs. Further, coverage and adequate reimbursement from governmental healthcare programs, such as Medicare and Medicaid, and commercial payors is critical to new product acceptance. If reimbursement is not available, or is available only to limited levels, we may not be able to successfully commercialize OJEMDA and product candidates. Even if coverage is provided, the approved reimbursement amount may not be high enough to allow us to establish or maintain pricing sufficient to realize an adequate return on our investment. Coverage and reimbursement may impact the demand for, or the price of, any product or product candidate for which we obtain marketing authorization.

There is significant uncertainty related to third-party payor coverage and reimbursement of newly approved products, particularly pediatric products. The payor mix for pediatric products in the United States is a fragmented combination of state-specific Medicaid policies and a broad universe of private insurance companies. There is no consistent policy or leading payor to inform other price-setting entities. Public and private payor policies are expected to be critical to our ability to achieve broad payment coverage. Further, to the extent one or more of our products obtain coverage by one third-party payor, that does not assure that other payors will also provide coverage for the product. As a result, the coverage determination process is often time-consuming and costly. This process will require us to provide scientific and clinical support for the use of our products to each third-party payor separately, with no assurance that coverage and adequate reimbursement will be applied consistently or obtained in the first instance.

As federal and state governments implement additional health care cost containment measures, including measures to lower prescription drug pricing, we cannot be sure that our products, if approved, will be covered by private or public payors, and if covered, whether the reimbursement will be adequate or competitive with other marketed products. These and other actions by federal and state governments and health plans may put additional downward pressure on pharmaceutical pricing and health care costs, which could negatively impact coverage and reimbursement for our products (if approved), our revenue and our ability to compete with other marketed products and to recoup the costs of our research and development.

Increasingly, third-party payors are requiring that drug companies provide them with predetermined discounts from list prices and are generally challenging the prices for medical products, including by examining the medical necessity and reviewing the cost effectiveness of medical product candidates. There may be especially significant delays in obtaining coverage and reimbursement for newly approved drugs. Third-party payors may limit coverage to specific products on an approved list, known as a formulary, which might not include all FDA-approved drugs for a particular indication. We plan to conduct pharmaco-economic studies to demonstrate the medical necessity and cost-effectiveness of our products, which may be costly. Nonetheless, our products and product candidates may not be considered medically necessary or cost-effective. Moreover, third-party payor coverage decisions may depend upon clinical and economic standards that disfavor new drug products when more established or lower cost therapeutic alternatives are already available or subsequently become available. We cannot be sure that coverage and reimbursement will be available for any product that we commercialize and, if reimbursement is available, what the level of reimbursement will be.

In addition, complementary and companion diagnostic tests require coverage and reimbursement separate and apart from the coverage and reimbursement for related pharmaceutical or biological products. Similar challenges to obtaining coverage and reimbursement, applicable to pharmaceutical or biological products, will apply to companion diagnostics. Additionally, if any companion diagnostic provider is unable to obtain reimbursement or is inadequately reimbursed, that may limit the availability of such companion diagnostic, which would negatively impact prescriptions for our product candidates, if approved.

Outside the United States, the commercialization of therapeutics is generally subject to extensive governmental price controls and other market regulations. We believe the increasing emphasis on cost containment initiatives in Europe, Canada and other countries has and will continue to put pressure on the pricing and usage of therapeutics such as our product candidates. In many countries, particularly the countries of the European Union, or EU, medicinal product prices are subject to varying price control mechanisms as part of national health systems. In these countries, pricing negotiations with governmental authorities can take considerable time after a product receives marketing authorization. To obtain reimbursement or pricing approval in some countries, we may be required to conduct a clinical trial that compares the cost-effectiveness of our product candidate to other available therapies. In general, product prices under such systems are substantially lower than in the United States. Other countries allow companies to fix their own prices for products but monitor and control company profits. Additional foreign price controls or other changes in pricing regulation could restrict the amount that we are able to charge for our product candidates. Accordingly, in markets outside the United States, the reimbursement for our products may be reduced compared with the United States and may be insufficient to generate commercially reasonable revenue and profits.

Coverage policies and third-party payor reimbursement rates may change at any time. Even if favorable coverage and reimbursement status is attained for one or more products for which we receive marketing authorization, less favorable coverage policies and reimbursement rates may be implemented in the future.

If we are unable to establish or sustain coverage and adequate reimbursement for any products from third-party payors, the adoption of those products and sales revenue will be adversely affected, which, in turn, could adversely affect the ability to market or sell those product, if approved.

Our business entails a significant risk of product liability and if we are unable to obtain sufficient insurance coverage such inability could have an adverse effect on our business and financial condition.

Our business exposes us to significant product liability risks inherent in the development, testing, manufacturing and commercialization of OJEMDA and any future products and marketing of therapeutic treatments. Product liability claims could delay or prevent completion of our development programs. If we succeed in marketing products, such claims could result in an FDA or other regulatory authority investigation of the safety and effectiveness of our products, our manufacturing processes and facilities or our marketing programs. The FDA or other regulatory authority investigations could potentially lead to a recall of our products or more serious enforcement action, limitations on the approved indications for which they may be used or suspension or withdrawal of approvals. Regardless of the merits or eventual outcome, liability claims may also result in decreased demand for our products, injury to our reputation, costs to defend the related litigation, a diversion of management's time, our resources and substantial monetary awards to trial participants or patients. We currently have product liability insurance that we believe is appropriate for our stage of development and may need to obtain higher levels prior to advancing our product candidates into clinical trials or marketing any of our product candidates, if approved. Any insurance we have or may obtain may not provide sufficient coverage against potential liabilities. Furthermore, clinical trial and product liability insurance is becoming increasingly expensive. As a result, we may be unable to obtain sufficient insurance at a reasonable cost to protect us against losses caused by product liability claims that could have an adverse effect on our business and financial condition.

Risks Related to Government Regulation

The development and commercialization of pharmaceutical products are subject to extensive regulation, and we may not obtain marketing authorizations for DAY301 or any future product candidates, on a timely basis or at all.

The clinical development, manufacturing, labeling, packaging, storage, recordkeeping, advertising, promotion, export, import, marketing, distribution, adverse event reporting, including the submission of safety and other post-marketing information and reports, and other possible activities relating to OJEMDA and DAY301, currently our only product and product candidate in planned or ongoing clinical trials, as well as any other product candidate that we may develop in the future, are subject to extensive regulation. Marketing authorization of drugs in the United States requires the submission of an NDA to the FDA. An NDA must be supported by extensive clinical and preclinical data, as well as extensive information regarding pharmacology, chemistry, manufacturing and controls. We are not permitted to market any product candidate in the United States until we obtain approval from the FDA of the NDA for that product.

The FDA may refer any application we submit to an advisory committee. An advisory committee is a panel of independent experts, including clinicians and other scientific experts, which reviews, evaluates and provides advice and recommendations to the FDA as to

whether the application should be approved and under what conditions. The FDA is not bound by the recommendations of an advisory committee, but it considers such recommendations carefully when making decisions.

FDA approval of an NDA is not guaranteed, and the review and approval process is an expensive and uncertain process over which the FDA has substantial discretion. The FDA approval process may also take several years. The timelines for the FDA review and approval process may be delayed as a result of future organizational changes and/or staffing reductions. The number and types of preclinical studies and clinical trials that will be required for NDA approval vary depending on the product candidate, the disease or the condition that the product candidate is designed to treat and the regulations applicable to any particular product candidate. Of the large number of drugs in development in the United States, only a small percentage will successfully complete the FDA marketing authorization process and will be commercialized. On April 23, 2024, the FDA approved the NDAs for the treatment of patients 6 months of age and older with relapsed or refractory pLGG harboring a BRAF fusion or rearrangement, or BRAF V600 mutation. In connection with its approval of OJEMDA, the FDA may impose restrictions, post-marketing requirements or post-marketing commitments that may limit our ability to commercialize OJEMDA or any other product. If we fail to comply with FDA-mandated requirements or if the results of certain required post-marketing studies are negative, the FDA could withdraw approval, add warnings or narrow approved indications, which could affect the commercial success of our products.

In addition, we may experience delays or rejections based upon additional government regulation from future legislation or administrative action, or changes in regulatory authority policy during the period of product development, clinical trials and the review process. For example, in May 2022, the Oncology Center of Excellence within the FDA advanced Project Optimus, an initiative to reform the dose optimization and dose selection paradigm in oncology drug development to emphasize selection of an optimal dose, which is a dose or doses that maximizes not only the efficacy of a drug but the safety and tolerability as well. This shift from the prior approach, which generally determined the maximum tolerated dose, may require sponsors to spend additional time and resources to further explore a product candidate's dose-response relationship to facilitate optimum dose selection in a target population. Other recent Oncology Center of Excellence initiatives have included Project FrontRunner, a new initiative with a goal of developing a framework for identifying candidate drugs for initial clinical development in the earlier advanced setting rather than for treatment of patients who have received numerous prior lines of therapies or have exhausted available treatment options.

Clinical trial failure may result from a multitude of factors, including flaws in trial design, dose selection, placebo effect, patient enrollment criteria, data integrity challenges or failure to demonstrate favorable safety or efficacy traits. Failure in clinical trials can occur at any stage. Companies in the pharmaceutical industry frequently suffer setbacks in the advancement of clinical trials due to lack of efficacy or adverse safety profiles, notwithstanding promising results in earlier trials. Based upon negative or inconclusive results, we may decide, or regulators may require us, to conduct additional clinical trials or preclinical studies. In addition, data obtained from clinical trials are susceptible to varying interpretations, and regulators may not interpret our data as favorably as we do, which may further delay, limit or prevent marketing authorization. On the basis of our clinical trials, the FDA could delay, limit or deny approval of a product candidate for many reasons, including because the FDA may:

- not deem our product candidate to be safe and effective;
- determine that the product candidate does not have an acceptable benefit-risk profile;
- determine in the case of an NDA seeking accelerated approval that the NDA does not provide evidence that the product candidate represents a meaningful advantage over available therapies and, therefore, may deny approval;
- determine that ORR as the primary endpoint, complemented by key secondary endpoints, is insufficient to reliably define clinical benefit;
- not agree that the data collected from preclinical studies and clinical trials are acceptable or sufficient to support the submission of an NDA or other submission or to obtain marketing authorization, and may impose requirements for additional preclinical studies or clinical trials;
- determine that adverse events experienced by participants in our clinical trials represent an unacceptable level of risk;
- determine that the population studied in the clinical trial may not be sufficiently broad or representative to assure safety in the full population for which we seek approval;
- not accept clinical data from trials, which are conducted at clinical facilities or in countries where the standard of care is potentially different from that of the United States;
- disagree regarding the formulation, labeling and/or the specifications;
- not approve the manufacturing processes associated with our product candidate or may determine that a manufacturing facility does not have an acceptable compliance status;
- change approval policies or adopt new regulations; or
- not file a submission due to, among other reasons, the content or formatting of the submission.

We have not yet obtained FDA approval for our product candidate, DAY301. While the FDA approved the NDAs for the treatment of patients 6 months of age and older with relapsed or refractory pLGG harboring a BRAF fusion or rearrangement, or BRAF V600 mutation, there is no assurance that we will receive similar approval for OJEMDA from comparable regulatory authorities in foreign jurisdictions, which may limit our addressable market and could adversely affect our business, prospects, financial condition and results of operations.

If we seek to utilize any of the FDA's expedited programs, the FDA may not find our product candidates to be eligible for these programs and, if granted, these programs may not lead to faster development, regulatory review or approval of our product candidates.

The FDA has several expedited programs, including Fast Track, Priority Review, Breakthrough Therapy and Accelerated Approval, which are authorized by the Federal Food, Drug and Cosmetic Act, or FD&C Act, and implemented pursuant to FDA regulations and guidance. None of these programs change the standard for FDA approval of a pharmaceutical product. We still must demonstrate substantial evidence of effectiveness and an acceptable safety profile to obtain marketing authorization.

We may seek to avail ourselves of one or more of the FDA's expedited programs. For example, we may seek Fast Track designation for one or more of our product candidates.

The FDA may grant a Fast Track designation to a drug that is intended for the treatment of a serious or life-threatening condition and nonclinical or clinical data demonstrates the potential to address unmet medical needs for this condition. The FDA has broad discretion whether to grant this designation, so even if we believe a particular product candidate is eligible for this designation, we cannot assure you that the FDA would decide to grant it. The FDA may withdraw Fast Track designation if it believes that the designation is no longer supported by data from our clinical development program.

We have applied for and have been granted breakthrough therapy designation for tovorafenib in patients with advanced pLGG, and we may apply for breakthrough therapy designation for other product candidates or indications in the future. The FDA may designate a drug candidate as a potential breakthrough therapy if the drug candidate is intended, alone or in combination with one or more other drugs or drug candidates, to treat a serious or life-threatening disease or condition and preliminary clinical evidence indicates that the drug candidate may demonstrate substantial improvement over existing therapies on one or more clinically significant endpoints. For drug candidates that have been designated as breakthrough therapies, interaction and communication between the FDA and the sponsor of the trial can help to identify the most efficient path for clinical development while minimizing the number of patients placed in ineffective control regimens. Drug candidates designated as breakthrough therapies by the FDA are also eligible for priority review if supported by clinical data at the time of the submission of the NDA. The FDA may withdraw breakthrough therapy designations if it determines that the criteria for the designation is no longer met.

We may seek priority review of one or more of our other applications for marketing authorization, or we may receive priority review as part of other designations we may seek for one or more of our other product candidates. The FDA may grant priority review to an application if an application is for a drug that treats a serious condition and, if approved, would provide a significant improvement in safety or effectiveness. The FDA may also grant priority review to supplements that propose a labeling change pursuant to a report on a pediatric study under Section 505A of the FD&C Act. Additionally, the FDA may grant priority review to any application or supplement for a drug submitted with a PRV. We cannot assure you that the FDA would decide to grant priority review of any of our product candidates.

Even if we do receive Fast Track designation, breakthrough therapy designation or priority review for any of our product candidates, we may not experience expedited development, review or faster action on our applications for marketing authorization compared to products without such designations.

The accelerated approval pathway may be unavailable or, if available, may not lead to faster development, regulatory review or marketing authorization, and the use of the accelerated approval pathway does not necessarily increase the likelihood that our product candidates will receive marketing authorization.

Under the FDA's Accelerated Approval Program, and subject to the conditions set forth in Section 506(c) of the FD&C Act and FDA regulations, the FDA may approve a product for a serious or life-threatening disease or condition based on a determination that the product has an effect on a surrogate endpoint that is reasonably likely to predict clinical benefit, or on a clinical endpoint that can be measured earlier than irreversible morbidity or mortality, that is reasonably likely to predict an effect on irreversible morbidity or mortality or other clinical benefit, taking into account the severity, rarity or prevalence of the condition and the availability or lack of alternative treatments. The FDA generally reserves the use of accelerated approvals for situations in which the product candidate at issue provides a meaningful therapeutic benefit over existing treatments.

We may seek accelerated approval for one or more of our product candidates on the basis of a surrogate endpoint that we believe is reasonably likely to predict clinical benefit, such as ORR. The FDA may not agree with our conclusion that an endpoint we select is

reasonably likely to predict clinical benefit, and thus the FDA may not agree that accelerated approval is appropriate based on that endpoint (even if the results on that endpoint are statistically significant), which could delay or preclude accelerated approval.

Products granted accelerated approval are subject to certain post-marketing requirements, which typically include a requirement to conduct one or more post-approval studies to confirm the clinical benefit of the product, which must be completed with due diligence. By the time of approval of the product, the FDA must set forth the conditions for the post-marketing studies which may include specific conditions and deadlines relating to the study protocol, enrollment targets, target completion date and other milestones. The FDA generally expects—and may require, as appropriate—the confirmatory study or studies to be underway at the time of the accelerated approval or within a specific time frame following approval. The FDA may disagree with our proposed clinical study designs for post-marketing confirmatory studies, and may require study conditions that are unfavorable to us, which could delay approval or lead to the withdrawal of a product approved under the accelerated approval pathway.

In addition, FDA regulations require that sponsors of products granted accelerated approval submit during the pre-approval review period copies of all promotional materials intended to be used within 120 days following marketing approval. After 120 days following marketing approval, unless otherwise informed by the FDA, the sponsor must submit all promotional materials at least 30 days prior to use.

The accelerated approval pathway has come under scrutiny within the FDA, by Congress and by other stakeholders. The FDA has put increased focus on ensuring that confirmatory studies are conducted with diligence and, ultimately, that such studies confirm the benefit. For example, the FDA has convened its Oncologic Drugs Advisory Committee to review what the FDA has called "dangling" or "delinquent" accelerated approvals where confirmatory studies have not been completed or where results did not confirm benefit. In addition, in 2021, the Oncology Center of Excellence announced Project Confirm, which is an initiative to promote the transparency of outcomes related to accelerated approvals for oncology indications and provide a framework to foster discussion, research and innovation in approval and post-marketing processes, with the goal to enhance the balance of access and verification of benefit for therapies available to patients with cancer and hematologic malignancies.

Finally, Congress passed the Food and Drug Omnibus Reform Act of 2022, or FDORA, which implemented key reforms to the FDA's authorities with respect to accelerated approval, including strengthening requirements around post-approval studies, codifying procedures for withdrawal of a product approved under the expedited approval pathway and establishing an intra-agency Accelerated Approval Council to address accelerated approval policy. FDORA also added the failure to conduct post-approval studies with due diligence or to submit timely progress reports on such studies to the list of prohibited acts under the FD&C Act, which means that any such failures, whether they result from our actions or the actions of third parties, could provide the basis for enforcement actions to be brought against us, which may be costly to defend or we may be unsuccessful in our defense.

The FDA also has the authority to withdraw products approved under the accelerated approval pathway using expedited withdrawal procedures. Circumstances that may lead to such withdrawal include:

- the failure to conduct any required post-approval study of a product candidate with due diligence, including with respect to conditions specified by the FDA;
- a study required to verify and describe the predicted clinical benefit of a product candidate fails to verify and describe such benefit;
- other evidence demonstrates that the product candidate is not shown to be safe or effective under the conditions of use; or
- the sponsor's dissemination of false or misleading promotional materials relating to the relevant product candidate.

If any of our competitors were to receive full approval for an indication for which we are seeking accelerated approval before we receive accelerated approval, the indication we are seeking may no longer qualify as a condition for which there is an unmet medical need, and accelerated approval of our product candidate would be more difficult or may not occur at all.

We may not be able to obtain or maintain orphan drug designation or exclusivity for our product candidates.

We have obtained orphan drug designation in the United States and in the EU for use of tovorafenib in treating malignant glioma and glioma, respectively. We may seek orphan drug designation for tovorafenib in additional geographies or indications, or for DAY301 or any product candidates we may develop in the future. Regulatory authorities in some jurisdictions, including the United States, may designate drugs for relatively small patient populations as "orphan drugs." Under the Orphan Drug Act, the FDA may designate a drug as an orphan drug if it is intended to treat a rare disease or condition, which is generally defined as a patient population of fewer than 200,000 individuals in the United States, or if the disease or condition affects more than 200,000 individuals in the United States and there is no reasonable expectation that the cost of developing and making available the drug for such disease or condition will be recovered from sales of the product in the United States.

Generally, if a product candidate with a U.S. orphan drug designation subsequently receives the first marketing authorization for the drug for the indication for which it has such designation, the product is entitled to a period of marketing exclusivity, which precludes the FDA from approving another marketing application for the same drug for the same indication for a period of seven years. Orphan

drug exclusivity in the United States may be lost if the FDA determines that the request for designation was materially defective or the drug in fact was ineligible for orphan-drug designation at the time the request for designation was submitted, or if the manufacturer is unable to assure a sufficient quantity of the drug to meet the needs of patients with the rare disease or condition.

The FDA may approve a subsequent application to market the same drug for the same indication during the exclusivity period in certain circumstances, such as if the subsequent product demonstrates clinical superiority (i.e., the subsequent product is safer, more effective or makes a major contribution to patient care) over the product with orphan exclusivity. Competitors, however, may receive approval of different products for the same indication for which the orphan product has exclusivity, or obtain approval for the same product but for a different indication than that for which the orphan product has exclusivity. Orphan drug designation also entitles a party to financial incentives, such as opportunities for grant funding towards clinical trial costs, tax advantages and user-fee waivers.

In the EU, if a medicinal product is granted marketing authorization as an orphan medicinal product, it benefits from a period of orphan market exclusivity during which the European Medicines Agency, or EMA, or a national regulator may not accept a marketing authorization application for a similar medicinal product in the same orphan indication. The applicable period of orphan exclusivity is ten years in the EU, but this can be reduced to six years if a drug no longer meets the criteria for orphan drug designation. The EMA or a national regulator may accept an application and grant a marketing authorization for a similar medicinal product for the orphan indication during the exclusivity period if the similar product is safer, more effective or otherwise clinically superior to the orphan product.

We cannot assure you that any future application for orphan drug designation with respect to any other product candidate will be granted. If we are unable to obtain orphan drug designation with respect to other product candidates in the United States or other jurisdictions, we will not be eligible to obtain the period of market exclusivity that could result from orphan drug designation or be afforded the other incentives associated with orphan drug designation.

Moreover, a recent Eleventh Circuit decision in *Catalyst Pharmaceuticals, Inc. vs. FDA* regarding interpretation of the Orphan Drug Act exclusivity provisions as applied to drugs approved for orphan indications narrower than the drug's orphan designation has the potential to significantly broaden the scope of orphan drug exclusivity for such products. Specifically, the Eleventh Circuit held that orphan drug exclusivity precludes the FDA from approving another marketing application for the same drug for the same orphan-designated disease or condition for a period of seven years. Although the FDA has announced that it will not apply the *Catalyst* decision beyond the facts at issue in that case, *Catalyst* could serve as a precedent for future challenges to the FDA's orphan drug-related decisions, and, accordingly, could fundamentally change how companies rely on, or seek to work around, orphan drug exclusivity in the United States. More recently, the U.S. Court of Appeals for the D.C. Circuit fully embraced the reasoning of the *Catalyst* decision in another decision challenging the scope of orphan drug exclusivity, *Jazz Pharmaceuticals, Inc. v. Kennedy*. Legislation has also been introduced that may reverse the *Catalyst* decision, but such legislation has not yet been passed.

We must comply with certain legal requirements and FDA policies, and may seek incentives under certain laws, relating to the development of drugs for pediatric patients, including the Pediatric Research Equity Act and the Best Pharmaceuticals for Children Act.

The Pediatric Research Equity Act, as amended, or PREA, requires that certain NDAs, Biologics License Applications, or BLAs, and NDA/BLA supplements contain assessment reports regarding the safety and efficacy of the product for the claimed indications in all relevant pediatric subpopulations to support dosing and administration for each pediatric subpopulation for which the product has been assessed to be safe and effective. In addition, PREA requires a molecularly targeted pediatric cancer investigation for an original NDA or BLA for a new active ingredient if the product candidate is intended to treat an adult cancer and is directed at a molecular target that the FDA determines to be substantially relevant to the growth or progression of a pediatric cancer, which may be different than the claimed adult cancer indication. PREA requires these pediatric studies be conducted using appropriate formulations for each age group that is studied, and an applicant must seek approval of any pediatric formulations that are used. The FDA may grant deferrals of PREA requirements or full or partial waivers. Unless otherwise required by regulation, PREA does not apply to a drug for an indication for which orphan designation has been granted, except that PREA will apply to an original NDA or BLA that is subject to the molecularly targeted pediatric cancer investigation requirement. Even if we are deemed exempt from PREA requirements for one application, any of our other applications may be subject to PREA requirements.

Under the Best Pharmaceuticals for Children Act, or the BPCA, the FDA can grant pediatric exclusivity to a sponsor that conducts pediatric studies requested by the FDA in a document called a Written Request. We may seek pediatric exclusivity for one or more of our product candidates under the BPCA, although we may not be granted such exclusivity. Pediatric exclusivity, if granted, adds six months to the end of certain unexpired statutory exclusivity periods and may also extend unexpired patent terms, depending on whether the application is an NDA or BLA. Whether this six-month extension is granted depends on the voluntary completion of pediatric studies in accordance with and in response to a Written Request for such studies, the submission of the study reports to the FDA within the timeframe required by the BPCA and the FDA's acceptance of the study reports. The FDA has indicated a strong preference to issue Written Requests only for studies that are in addition to and/or different from pediatric studies required under PREA (if applicable).

In general, pediatric drug development is an area that recently has been, and may continue to be, subject to evolving statutory requirements and regulatory standards, so some uncertainty exists with respect to expectations for pediatric drug development generally.

We may seek a rare pediatric disease designation for one or more of our product candidates under the FDA’s Rare Pediatric Disease Priority Review Voucher Program. Even if we were to obtain marketing authorization for a product with a rare pediatric disease designation, the Rare Pediatric Disease Priority Review Voucher Program may no longer be in effect at the time of such approval or we might not be able to capture the value of the Rare Pediatric Disease Priority Review Voucher Program.

OJEMDA was granted rare pediatric designation by the FDA in May 2021 for the treatment of LGGs harboring an activating RAF alteration that disproportionately affects children. We submitted the OJEMDA NDAs as a rare pediatric designation marketing application, and the FDA conditionally designated the marketing application as a “rare pediatric disease product application” pending the final determination at the time of approval or licensure on whether the application meets all of the eligibility criteria set forth in section 529(a)(4) of the FD&C Act. On April 23, 2024, the FDA approved the NDAs for the treatment of patients 6 months of age and older with relapsed or refractory pLGG harboring a BRAF fusion or rearrangement, or BRAF V600 mutation, and in connection with the accelerated approval, Day One received a rare pediatric disease PRV.

Congress authorized the FDA to award PRVs to sponsors of certain rare pediatric disease product applications that meet the specified criteria. These vouchers are designed to encourage development of new drug and biological products for prevention and treatment of certain rare pediatric diseases.

Specifically, under this program, a sponsor who receives an approval for a drug or biologic for a “rare pediatric disease” may qualify for a voucher that can be redeemed to receive a priority review of a subsequent marketing application for a different product. The sponsor of a rare pediatric disease drug product receiving a PRV may transfer (including by sale) the voucher to another sponsor. On May 29, 2024, we entered into an asset purchase agreement, pursuant to which we agreed to sell our rare pediatric disease PRV to an undisclosed buyer for gross proceeds of \$108.0 million. Following the sale, we are no longer eligible to take advantage of the incentives under the rare pediatric disease PRV, including priority review of a subsequent marketing application. The voucher may be further transferred any number of times before the voucher is used, as long as the sponsor making the transfer has not yet submitted the application. Although the voucher can be sold or transferred to third parties, there is no guarantee that we will be able to receive such voucher in the future for any of our current or future product candidates or that we will realize any value if we receive and were to sell any such voucher.

For the purposes of this program, a rare pediatric disease is a (i) serious or life-threatening disease in which the serious or life-threatening manifestations primarily affect individuals aged from birth to 18 years, including age groups often called neonates, infants, children, and adolescents; and (ii) rare disease or condition within the meaning of the Orphan Drug Act. The FDA may determine that an application for one or more of our product candidates does not meet the eligibility criteria for a PRV upon approval.

Moreover, under the current statutory sunset provisions, the FDA generally may not award rare pediatric disease PRVs after December 20, 2024. However, if the sponsor has received rare pediatric disease designation for a drug no later than September 30, 2024, the FDA may award a rare pediatric disease PRV if the drug is approved by September 30, 2026.

If we or a business partner are unable to successfully develop, validate, obtain marketing authorization for and commercialize any companion diagnostic tests that are deemed necessary for the use of any of our product candidates, or experience significant delays in doing so, we may not be able to obtain marketing authorization for, or realize the full commercial potential of, one or more of our product candidates.

Diagnostic tests can be useful in identifying patients who are most likely to benefit from a particular therapeutic drug product, among other potential uses. If a regulatory authority determines that an in vitro diagnostic test is necessary for the safe and effective use of a corresponding therapeutic product, that test is referred to as a “companion diagnostic.” Diagnostics that are not essential for the safe and effective use of a therapeutic product but that may aid in the benefit-risk decision-making about the use of the therapeutic product (such as to identify a subset of the indicated patient population for the therapeutic product that may respond particularly well) are typically referred to as “complementary diagnostics.” In the future, we may evaluate opportunities to develop, either by ourselves or with collaborators, companion or complementary diagnostic tests for our product candidates for certain indications.

If a companion diagnostic is needed for a therapeutic product, the companion diagnostic is generally developed in conjunction with the clinical program for an associated therapeutic product. To date, the FDA has required premarket approval of the vast majority of companion diagnostics for cancer therapies. Generally, when a companion diagnostic is essential to the safe and effective use of a drug product, the FDA generally requires that the companion diagnostic be approved before or concurrent with approval of the therapeutic product and before such product can be commercialized (except in limited circumstances). Where a companion diagnostic must be used to identify patients who are likely to benefit from the therapeutic product, the therapeutic product’s labeling typically limits the use of the therapeutic product to only those patients who express the specific genetic alteration or other biomarker that the companion diagnostic was developed to detect. By contrast, complementary diagnostics are not typically referenced in the indications

for the therapeutic product (i.e., the therapeutic product is not limited to use in biomarker positive patients) but the complementary diagnostic may be described in other areas of the therapeutic product labeling, such as when describing clinical study results for biomarker positive and negative patient subpopulations. While a complementary diagnostic is also typically developed in conjunction with the clinical program for an associated therapeutic product, the FDA may not require that the complementary diagnostic be approved before or concurrent with approval of the therapeutic product.

Development of a companion or complementary diagnostic could include additional meetings with regulatory authorities, such as a pre-submission meeting and the requirement to comply with the FDA's investigational device exemption regulations for clinical studies involving the diagnostic. In the case of an investigational diagnostic that is designated as "significant risk device," approval of an investigational device exemption application by an IRB and the FDA is required before such diagnostic may be used in conjunction with the clinical trials for a corresponding product candidate.

To be successful in developing, validating, obtaining approval of and commercializing a companion or complementary diagnostic, we or our collaborators will need to address a number of scientific, technical, regulatory and logistical challenges. We have no prior experience with medical device or diagnostic test development. If we choose to develop and seek FDA approval for companion diagnostic tests on our own, we will require additional personnel. We may rely on third parties for the design, development, testing, validation and manufacture of companion diagnostic tests for our therapeutic product candidates that require companion diagnostic tests or would benefit from complementary diagnostics, the application for and receipt of any required marketing authorizations and the commercial supply of these diagnostics. If these parties are unable to successfully develop companion diagnostics for these therapeutic product candidates, or experience delays in doing so, we may be unable to enroll enough patients for our current and planned clinical trials, the development of these therapeutic product candidates may be adversely affected, these therapeutic product candidates may not obtain marketing authorization and we may not realize the full commercial potential of any of these therapeutics that obtain marketing authorization. For any product candidate for which a companion diagnostic is necessary to select patients who may benefit from use of the product candidate, any failure to successfully develop a companion diagnostic may cause or contribute to delayed enrollment of our clinical trials, and may prevent us from initiating a pivotal trial. In addition, the commercial success of any of our product candidates that require a companion diagnostic will be tied to and dependent upon the receipt of required marketing authorizations and the continued ability of such third parties to make the companion diagnostic commercially available to us on reasonable terms in the relevant geographies. There is no guarantee that physicians will adopt any particular companion diagnostic, be willing to understand how to use it, how to obtain reimbursement for it or how to explain it to patients or dedicate staff to using it. Any failure to do so could materially harm our business, results of operations and financial condition.

For each product and product candidate for which marketing authorization is granted, including OJEMDA, the terms of approvals, ongoing regulation of our products or other post-approval restrictions may limit how we manufacture and market our products and compliance with such requirements may involve substantial resources, which could materially impair our ability to generate revenue in line with our expectations.

For each product and product candidate for which marketing authorization is granted, including OJEMDA, an approved product and the marketing authorization holder are subject to ongoing regulation by the FDA, the EMA and other regulators. Regulators may impose post-marketing requirements and elicit post-marketing commitments, which may be onerous and subject us to ongoing review and extensive regulation. For example, the FDA or EMA may request or require post-marketing clinical studies, enhanced pharmacovigilance programs, additional reporting requirements and other obligations at the time of approval or after approval. The FDA also may impose a REMS under Section 505-1 of the FD&C Act in order to ensure that the benefits of our product candidates outweigh their risks. Additionally, either at the time of approval or after approval, the FDA could invoke its authority under Section 505(o) of the FD&C Act and require costly post-marketing safety studies, including clinical trials, and/or epidemiologic surveillance to monitor the safety of our approved products in order to assess a known risk related to the product, assess signals of serious risks related to the product or identify an unexpected serious risk when available data indicates the potential for a serious risk.

In addition, any product candidates for which we receive accelerated approval from the FDA are required to undergo one or more clinical trials to confirm the clinical benefit of the product. If confirmatory studies fail to meet their efficacy endpoints, the FDA may withdraw approval of the product pursuant to expedited withdrawal authorities. There is no assurance that any such product will successfully advance through its confirmatory clinical trial(s). Therefore, even if a product candidate receives accelerated approval from the FDA, such approval may be withdrawn at a later date.

We must also comply with requirements concerning advertising and promotion for any of our product candidates for which we obtain marketing authorization. Further, there are additional requirements regarding promotional communications if our products are approved through the accelerated approval pathway. Promotional communications with respect to prescription drugs are subject to a variety of legal and regulatory restrictions and must be consistent with the information in the product's approved labeling. Thus, we will not be able to promote any products we develop for indications or uses for which they are not approved.

In addition, manufacturers of approved products and those manufacturers' facilities are required to ensure that quality control and manufacturing procedures conform to current good manufacturing practices, or cGMPs, which include requirements relating to quality control and quality assurance as well as the corresponding maintenance of records and documentation and reporting requirements. We

and our CMOs could be subject to periodic inspections by the FDA to monitor and ensure compliance with cGMPs, including pre-approval inspections of any manufacturing facilities proposed to commercially manufacture our product candidates, the success of which would be required prior to a commercial product launch. Accordingly, assuming we obtain marketing authorization for one or more of our product candidates, we and our CMOs will continue to expend time, money and effort in all areas of regulatory compliance, including manufacturing, production, testing, product surveillance and quality assurance.

If we are not able to comply with all of our post-approval regulatory requirements, we could have the marketing authorizations for our products withdrawn by regulatory authorities and our ability to market any future products could be limited, which could adversely affect our ability to achieve or sustain profitability. In addition, the cost of compliance with post-approval regulations may have a negative effect on our operating results and financial condition.

Any product for which we obtain marketing authorization, including OJEMDA, will be subject to ongoing enforcement of post-marketing requirements by regulatory agencies, and we could be subject to substantial penalties, including withdrawal of our product from the market, if we fail to comply with all regulatory requirements or if we experience unanticipated problems with our products, when and if any of them are approved.

Any product for which we obtain marketing authorization, such as OJEMDA, along with the manufacturing processes, post-approval clinical data, labeling, advertising and promotional activities for such product, will be subject to continual requirements of and review by the FDA, the EMA and other regulatory authorities. These requirements include, but are not limited to, restrictions governing promotion of an approved product, submissions of safety and other post-marketing information and reports, registration and listing requirements, cGMP requirements relating to manufacturing, quality control, quality assurance and corresponding maintenance of records and documents and requirements regarding drug distribution and the distribution of samples to physicians and recordkeeping.

The FDA and other federal and state agencies, including the Department of Justice, closely regulate compliance with all requirements governing prescription drug products, including requirements pertaining to marketing and promotion of drugs in accordance with the provisions of the approved labeling and manufacturing of products in accordance with cGMP requirements. For example, the FDA and other agencies actively enforce the laws and regulations prohibiting the promotion of off-label uses, and a company that is found to have improperly promoted off-label uses may be subject to significant liability. Violations of such requirements may lead to investigations alleging violations of the FD&C Act and other statutes, including the False Claims Act and other federal and state healthcare fraud and abuse laws as well as state consumer protection laws. Our failure to comply with all regulatory requirements, and later discovery of previously unknown adverse events or other problems with our products, manufacturers or manufacturing processes, may yield various results, including:

- litigation involving patients taking our products;
- restrictions on such products, manufacturers or manufacturing processes;
- restrictions on the labeling or marketing of a product;
- restrictions on product distribution or use;
- requirements to conduct post-marketing studies or clinical trials;
- warning or untitled letters;
- withdrawal of the products from the market;
- refusal to approve pending applications or supplements to approved applications that we submit;
- voluntary or mandatory recall of products;
- fines, restitution or disgorgement of profits or revenues;
- suspension or withdrawal of marketing authorizations;
- damage to relationships with any potential collaborators;
- unfavorable media coverage and damage to our reputation;
- refusal to permit the import or export of our products;
- product seizure; or
- injunctions or the imposition of civil or criminal penalties.

Non-compliance by us or any future collaborator with regulatory requirements, including safety monitoring or pharmacovigilance, and with requirements related to the development of products for the pediatric population can also result in significant financial penalties. Further, if any of these actions were to occur, we may have to discontinue the commercialization of OJEMDA and product candidates,

limit our sales and marketing efforts, conduct further post-approval studies and/or discontinue or change any other ongoing clinical studies, which in turn could result in significant expense and delay and/or limit our ability to generate sales revenues.

Our failure to obtain marketing authorization in foreign jurisdictions would prevent OJEMDA and our product candidates from being marketed in those jurisdictions, and any approval we are granted for our product candidates in the United States would not assure approval of product candidates in foreign jurisdictions.

In order to market and sell our products in any jurisdiction outside the United States, we must obtain separate marketing authorizations and comply with numerous and varying regulatory requirements. The approval procedure varies among countries and can involve additional testing and/or product release specifications. The time required to obtain approval may differ substantially from that required to obtain FDA approval. The marketing authorization process outside the United States generally includes all of the risks associated with obtaining FDA approval. In addition, in many countries outside the United States, it is required that the product be approved for reimbursement before the product can be approved for sale in that country. We may not obtain approvals from regulatory authorities outside the United States on a timely basis, if at all. Approval by the FDA or EMA does not ensure approval by regulatory authorities in other countries or jurisdictions, and approval by one regulatory authority outside the United States does not ensure approval by regulatory authorities in other countries or jurisdictions or by the FDA. We may not be able to submit and receive approval for marketing authorizations in other foreign jurisdictions and may not receive necessary approvals to commercialize our products in any market.

Our current and future relationships with customers and third-party payors may be subject to applicable anti-kickback, fraud and abuse, transparency, health privacy and other healthcare laws and regulations, which could expose us to significant penalties, including criminal, civil and administrative penalties, contractual damages, reputational harm and diminished profits and future earnings.

Healthcare providers, including physicians, and third-party payors will play a primary role in the recommendation and prescription of any product candidates for which we obtain marketing authorization. Our current and future arrangements with healthcare providers, third-party payors and customers may expose us to broadly applicable fraud and abuse and other healthcare laws and regulations that may constrain the business or financial arrangements and relationships through which we research, as well as market, sell and distribute, any products for which we obtain marketing authorization. Restrictions under applicable federal and state healthcare laws and regulations that may be applicable to our business include the following:

- the federal Anti-Kickback Statute prohibits, among other things, persons and entities from knowingly and willfully soliciting, offering, receiving or providing remuneration, directly or indirectly, in cash or in kind, to induce or reward, or in return for, either the referral of an individual for, or the purchase, order or recommendation of, any good or service, for which payment may be made under a federal healthcare program such as Medicare and Medicaid;
- the federal civil false claims laws, including the False Claims Act, which can be enforced by civil whistleblower or qui tam actions on behalf of the government, and criminal false claims laws and the civil monetary penalties law, prohibit individuals or entities from, among other things, knowingly presenting, or causing to be presented false or fraudulent claims for payment by a federal government program, or making a false statement or record material to payment of a false claim or avoiding, decreasing or concealing an obligation to pay money to the federal government;
- the federal Health Insurance Portability and Accountability Act of 1996, or HIPAA, prohibits, among other things, knowingly and willfully executing, or attempting to execute, a scheme to defraud any healthcare benefit program, regardless of the payor (e.g. public or private), and knowingly and willfully falsifying, concealing or covering up by any trick or device a material fact or making any materially false, fictitious or fraudulent statements in connection with the delivery of, or payment for, healthcare benefits, items or services relating to healthcare matters;
- HIPAA, as amended by the Health Information Technology for Economic and Clinical Health Act, or HITECH, and their implementing regulations, impose requirements on certain covered healthcare providers, health plans and healthcare clearinghouses, as well as their respective business associates and their subcontractors that perform services for them that involve the use, or disclosure of, individually identifiable health information, relating to the privacy, security and transmission of such individually identifiable health information;
- the federal transparency requirements under the Patient Protection and Affordable Care Act, as amended by the Health Care and Education Reconciliation Act of 2010, collectively referred to as the ACA, require certain manufacturers of drugs, devices, biologics and medical supplies to annually report to the Centers for Medicare & Medicaid Services, or CMS, information related to payments and other transfers of value provided to teaching hospitals, as well as ownership and investment interests held by physicians, defined to include doctors, dentists, optometrists, podiatrists and chiropractors, as well as ownership and investment interests held by physicians and their immediate family members. Since January 1, 2021, manufacturers are required to collect information regarding payments and transfers of value to physician assistants, nurse practitioners, clinical nurse specialists,

anesthesiologist assistants, certified nurse anesthetists and certified nurse-midwives for reporting in the following year. The reported information is made available on a public website; and

- analogous state laws and regulations such as state anti-kickback and false claims laws and analogous non-U.S. fraud and abuse laws and regulations, may apply to sales or marketing arrangements and claims involving healthcare items or services reimbursed by state payors and non-governmental third-party payors, including private insurers. Some state laws require pharmaceutical companies to comply with the pharmaceutical industry's voluntary compliance guidelines and the relevant compliance regulations promulgated by the federal government and may require drug manufacturers to report information related to payments and other transfers of value to physicians and other healthcare providers, marketing expenditures or drug pricing, including price increases. Certain state and local laws require the registration of pharmaceutical sales representatives. Certain state and non-U.S. laws, many of which differ from each other in significant ways and often are not preempted by HIPAA, also govern the privacy and security of health information in some circumstances, thus complicating compliance efforts.

Efforts to ensure that our internal business processes and business arrangements with third parties will comply with applicable healthcare laws and regulations will involve substantial costs. It is possible that governmental authorities will conclude that our business practices do not comply with current or future statutes, regulations or case law involving applicable fraud and abuse or other healthcare laws and regulations. If our operations are found to be in violation of any of these laws or any other governmental regulations that may apply to us, we may be subject to significant civil, criminal and administrative penalties, damages, fines, disgorgement, imprisonment, exclusion from government funded healthcare programs, such as Medicare and Medicaid and other federal healthcare programs, contractual damages, reputational harm, diminished profits and future earnings, additional integrity reporting and oversight obligations and the curtailment or restructuring of our operations, any of which could adversely affect our ability to operate our business and our results of operations. If any of the physicians or other healthcare providers or entities with whom we expect to do business is found to be not in compliance with applicable laws, they may be subject to significant criminal, civil and administrative sanctions, including exclusions from government funded healthcare programs, which could have a material adverse effect on our business, reputation, results of operations, financial condition and prospects. Although compliance programs can mitigate the risk of investigation and prosecution for violations of these laws, the risks cannot be entirely eliminated. Any action against us for violation of these laws, even if we successfully defend against it, could cause us to incur significant legal expenses and divert our management's attention from the operation of our business.

Existing, recently enacted and future legislation may increase the difficulty and cost for us to obtain marketing authorization of and commercialize our product candidates and decrease the prices we may obtain.

In the United States and some foreign jurisdictions, there have been a number of legislative and regulatory changes and proposed changes regarding the healthcare system that could prevent or delay marketing authorization of our product candidates, restrict or regulate post-approval activities and affect our ability to profitably sell any products for which we obtain marketing authorization.

For example, in March 2010, the ACA was signed into law. The ACA is a sweeping law intended to broaden access to health insurance, reduce or constrain the growth of healthcare spending, enhance remedies against fraud and abuse, add new transparency requirements for the healthcare and health insurance industries, impose new taxes and fees on the health industry and impose additional health policy reforms.

Among the provisions of the ACA of importance to our potential product candidates are the following:

- annual fees and taxes on manufacturers of certain branded prescription drugs;
- an annual, nondeductible fee on any entity that manufactures or imports specified branded prescription drugs and biologic products;
- a Medicare Part D coverage gap discount program, in which manufacturers must now agree to offer 70% point-of-sale discounts off negotiated prices of applicable brand drugs to eligible beneficiaries during their coverage gap period, as a condition for the manufacturer's outpatient drugs to be covered under Medicare Part D;
- a new methodology by which rebates owed by manufacturers under the Medicaid Drug Rebate Program are calculated for drugs that are inhaled, infused, instilled, implanted or injected;
- an increase in the statutory minimum rebates a manufacturer must pay under the Medicaid Drug Rebate Program and extended the rebate program to individuals enrolled in Medicaid managed care organizations;
- expansion of healthcare fraud and abuse laws, including the False Claims Act and the federal Anti-Kickback Statute, new government investigative powers and enhanced penalties for noncompliance;
- extension of manufacturers' Medicaid rebate liability;
- expansion of eligibility criteria for Medicaid programs;

- expansion of the entities eligible for discounts under the Public Health Service pharmaceutical pricing program;
- requirements to report financial arrangements with physicians, as defined by such law, and teaching hospitals;
- a requirement to annually report drug samples that manufacturers and distributors provide to physicians; and
- a Patient-Centered Outcomes Research Institute to oversee, identify priorities in and conduct comparative clinical effectiveness research, along with funding for such research.

There have been executive, judicial and Congressional challenges to repeal or replace certain aspects of the ACA, including measures taken during the first Trump administration. While Congress has not passed comprehensive repeal legislation, it has enacted laws that modify certain provisions of the ACA such as removing penalties, since January 1, 2019, for not complying with the ACA's individual mandate to carry health insurance, eliminating the implementation of certain ACA-mandated fees and increasing the point-of-sale discount that is owed by pharmaceutical manufacturers who participate in Medicare Part D. In addition, there have been legal challenges to the constitutionality of the ACA and certain requirements such as the individual mandate. Although these challenges have been unsuccessful to date, there may be other efforts to challenge the individual mandate or to challenge, repeal or replace the ACA. It is unclear how any pending or future litigation and the healthcare reform measures of the current presidential administration and Congress will impact the ACA and our business.

In addition, other legislative changes have been proposed and adopted since the ACA was enacted. On August 2, 2011, the Budget Control Act of 2011 was signed into law, which, among other things, created the Joint Select Committee on Deficit Reduction to recommend to Congress proposals for spending reductions. The Joint Select Committee did not achieve a targeted deficit reduction, triggering the legislation's automatic reduction to several government programs. These changes include aggregate reductions to Medicare payments to providers of up to 2% per fiscal year, which began in 2013, and due to subsequent legislative amendments to the statute, will remain in effect through 2030, with the exception of a temporary suspension from May 1, 2020 through December 31, 2021 due to the COVID-19 pandemic, unless additional Congressional action is taken. In January 2013, the American Taxpayer Relief Act of 2012 was signed into law, which, among other things, reduced Medicare payments to several providers and increased the statute of limitations period for the government to recover overpayments to providers from three to five years. In July 2025, the One Big Beautiful Bill Act, or the OBBBA, was signed into law, which reduces funding to federal healthcare programs and imposes additional requirements to be eligible for healthcare. These laws may result in additional reductions in Medicare and other healthcare funding.

Further, there has been heightened governmental scrutiny recently over the manner in which drug manufacturers set prices for their marketed products, which has resulted in several Congressional inquiries and proposed and enacted federal and state legislation designed to, among other things, bring more transparency to product pricing, review the relationship between pricing and manufacturer patient programs and reform government program reimbursement methodologies for drug products. At the federal level, recent presidential administrations were focused on drug pricing and have used several means to propose or implement drug pricing reform, including through federal budget proposals, executive orders and policy initiatives. For example, on September 9, 2021, the Biden administration published a wide-ranging list of policy proposals to lower prescription drug prices, including by allowing Medicare to negotiate prices and disincentivizing price increases, and to support market changes that strengthen supply chains, promote biosimilars and generic drugs and increase price transparency. These initiatives recently culminated in the enactment of the Inflation Reduction Act, or IRA, in August 2022, which will, among other things, allow the U.S. Department of Health and Human Services, or HHS, to negotiate the price of certain drugs and biologics that CMS reimburses under Medicare Part B and Part D. The IRA's negotiation program will apply to high-expenditure single-source drugs that have been approved for at least 7 years (11 years for biologics), among other negotiation selection criteria. One statutory exemption from the negotiation program is for a drug that has only a single orphan drug designation and is approved only for an indication or indications within the scope of such designation. The negotiated prices, which for the first round of selected drugs announced August 29, 2023 will become effective in 2026, will be capped at a statutorily-determined ceiling price. The IRA also penalizes drug manufacturers that increase prices of Medicare Part B and Part D drugs at a rate greater than the rate of inflation. In addition, the law eliminates the "donut hole" under Medicare Part D beginning in 2025 by significantly lowering the beneficiary maximum out-of-pocket cost and requiring manufacturers to subsidize, through a newly established manufacturer discount program, 10% of Part D enrollees' prescription costs for brand drugs below the out-of-pocket maximum, and 20% once the out-of-pocket maximum has been reached. The IRA permits the Secretary of HHS to implement many of these provisions through guidance, as opposed to regulation, for the initial years. Manufacturers that fail to comply with the IRA may be subject to various penalties, including civil monetary penalties. The IRA also extends enhanced subsidies for individuals purchasing health insurance coverage in ACA marketplaces through plan year 2025. These IRA provisions began taking effect progressively starting in 2023, although the drug negotiation provisions of the IRA are currently the subject of legal challenges. In addition, the Secretary of the HHS recently proposed testing three new models for pricing efficiency, including one that develops payment methods for drugs approved under accelerated approval, in consultation with the FDA, to encourage timely confirmatory trial completion and improve access to post-market safety and efficacy data with the goal of reducing Medicare spending on drugs that have no confirmed clinical benefit. Further, at the state level, individual states have increasingly introduced and passed legislation and implemented regulations designed to control pharmaceutical and biological product pricing, including: restricting price,

reimbursement, discounts, product access and marketing; imposing drug price and cost disclosure and transparency requirements; permitting importation from other countries; and encouraging bulk purchasing.

We expect that additional state and federal healthcare reform measures, including potentially significant additional changes to current drug pricing and reimbursement structures, such as Most Favored Nation provisions, will be adopted in the future. Current and future reform measures may result in more rigorous coverage criteria and in additional downward pressure on the prices that we receive for any approved product. The implementation of cost containment measures or other healthcare reforms may prevent us from being able to generate future revenue in line with our expectations, attain profitability or commercialize OJEMDA and our product candidates.

Legislative and regulatory proposals have been made to expand post-approval requirements and restrict sales and promotional activities for pharmaceutical products. We cannot be sure whether additional legislative changes will be enacted, or whether the FDA regulations, guidance or interpretations will be changed, or what the impact of such changes on the marketing authorizations of our product candidates, if any, may be. In addition, increased scrutiny by the U.S. Congress of the FDA's approval process may significantly delay or prevent marketing authorization, as well as subject us to more stringent product labeling and post-marketing testing and other requirements.

Further, in June 2024, the U.S. Supreme Court reversed its longstanding approach under the Chevron doctrine, which provided for judicial deference to regulatory agencies, including the FDA. As a result of this decision, we cannot be sure whether there will be increased challenges to existing agency regulations or how lower courts will apply the decision in the context of other regulatory schemes without more specific guidance from the U.S. Supreme Court. For example, this decision may result in more companies bringing lawsuits against the FDA to challenge longstanding decisions and policies of the FDA, which could undermine the FDA's authority, lead to uncertainties in the industry, and disrupt the FDA's normal operations, which could impact the timely review of any regulatory filings or applications we submit to the FDA. We cannot predict the full impact of this decision, future judicial challenges brought against the FDA, or the nature or extent of government regulation that may arise from future legislation or administrative action.

Governments outside of the United States tend to impose strict price controls, which may adversely affect our future revenues.

In some countries, including Canada and certain member states of the EU, the pricing of prescription pharmaceuticals is subject to governmental control. In these countries, pricing negotiations with governmental authorities can take considerable time after the receipt of marketing authorization for a product. To obtain reimbursement or pricing approval in some countries, we may be required to conduct a clinical trial that compares the cost-effectiveness of our product candidates to other available therapies. If reimbursement of our products is unavailable or limited in scope or amount, or if pricing is set at unsatisfactory levels, our business could be harmed. Political, economic and regulatory developments, including the imposition of new or additional tariffs or other trade barriers, may further complicate pricing negotiations, and pricing negotiations may continue after reimbursement has been obtained. Reference pricing used by various EU member states, and parallel trade, such as arbitrage between low-priced and high-priced member states, can further reduce prices. There can be no assurance that any country that has price controls or reimbursement limitations for pharmaceutical products will allow favorable reimbursement and pricing arrangements for any products, if approved in those countries. Publication of discounts by third-party payors or authorities may lead to further pressure on the prices or reimbursement levels within the country of publication or other countries.

Laws and regulations governing any international operations we may have in the future may preclude us from developing, manufacturing and selling certain product candidates and products outside of the United States and require us to develop and implement costly compliance programs.

If we expand our operations outside of the United States, we must dedicate additional resources to comply with numerous laws and regulations in each jurisdiction in which we plan to operate. The Foreign Corrupt Practices Act, or FCPA, prohibits certain U.S. individuals and entities and their party agents from paying, offering, authorizing payment or offering anything of value, directly or indirectly, to any foreign official, political party or candidate for the purpose of influencing any act or decision of such third party in order to assist the individual or business in obtaining or retaining business or gaining an improper advantage. The FCPA also obligates companies whose securities are listed in the United States to comply with certain accounting provisions requiring the company to maintain books and records that accurately and fairly reflect all transactions of the company, including international subsidiaries, and to devise and maintain an adequate system of internal accounting controls for international operations.

Compliance with the FCPA is expensive and difficult, particularly in countries where corruption is a recognized problem. In addition, the FCPA presents particular challenges in the pharmaceutical industry, because, in many countries, hospitals are operated by the government, and doctors and other hospital employees are considered foreign officials and therefore our interactions with these individuals are subject to regulation under the FCPA.

Various laws, regulations and executive orders also restrict the use and dissemination outside of the United States, or the sharing with certain non-U.S. nationals, of information classified for national security purposes, as well as certain products and technical data relating to those products. We are also subject to U.S. laws and regulations governing export controls, as well as economic sanctions

and embargoes on certain countries and persons. If we expand our presence outside of the United States, it will require us to dedicate additional resources to comply with these laws, and these laws may preclude us from developing, manufacturing or selling certain product candidates and products outside of the United States, which could limit our growth potential and increase our development costs.

The failure to comply with laws governing international business practices may result in substantial civil and criminal penalties and suspension or debarment from government contracting. The U.S. Securities and Exchange Commission, or the SEC, also may suspend or bar issuers from trading securities on U.S. exchanges for violations of the FCPA's accounting provisions.

Disruptions at the FDA may slow the time necessary for new products to be reviewed and/or approved, which would adversely affect our business. There is substantial uncertainty regarding new initiatives and how these might impact the FDA, its implementation of laws, regulations, policies and guidance and its personnel. Similar initiatives may also be directed toward other government agencies. These initiatives could prevent, limit or delay development and regulatory approval of our product candidates, which would adversely affect our business.

Disruptions at the FDA may slow the time necessary for new products to be reviewed and/or approved, which would adversely affect our business. Changes in FDA staffing could result in delays in the FDA's responsiveness or in its ability to review submissions or applications, issue regulations or guidance, or implement or enforce regulatory requirements in a timely fashion or at all. If any legislation, executive orders, or lapses in agency funding impose constraints on the FDA's ability to engage in oversight and implementation activities in the normal course, our business may be negatively impacted.

Similar consequences would also result in the event of another significant shutdown of the federal government. For example, in 2024, the U.S. government was on the verge of a shutdown and has previously shut down several times, and certain regulatory agencies, such as the FDA, had to furlough critical employees and stop critical activities. If a prolonged government shutdown occurs, or if geopolitical or global health concerns prevent the FDA from conducting their regular inspections, reviews, or other regulatory activities, it could significantly impact the ability of the FDA to timely review and process our regulatory submissions, which could have a material adverse effect on our business. Further, future government shutdowns or delays could impact our ability to access the public markets and obtain necessary capital in order to properly capitalize and continue our operations.

FDA-regulated industries, such as ours, face uncertainty with regard to the regulatory environment we will face as we proceed with research, development and commercialization. Some of these efforts have manifested to date in the form of personnel measures that could impact the FDA's ability to hire and retain key personnel, which could result in delays or limitations on our ability to obtain guidance from the FDA on our product candidates in development or obtain the requisite regulatory approvals in the future. There remains general uncertainty regarding future activities. New executive orders, regulations, policies or guidance could be issued or promulgated that adversely affect us or creates a more challenging or costly environment to pursue the development of new therapeutic products. Alternatively, state governments may attempt to address or react to changes at the federal level with changes to their own regulatory frameworks in a manner that is adverse to our operations. If we become negatively impacted by future governmental orders, regulations, policies or guidance, there could be a material adverse effect on us and our business.

If we fail to comply with environmental, health and safety laws and regulations, we could become subject to fines or penalties or incur costs that could harm our business.

We and our third-party contractors are subject to numerous foreign, federal, state and local environmental, health and safety laws and regulations, including those governing laboratory procedures and the handling, use, storage, treatment and disposal of hazardous materials and wastes. Our operations involve the use of hazardous and flammable materials, including chemicals and biological materials. Our operations also produce hazardous waste products. We generally contract with third parties for the disposal of these materials and wastes. We cannot eliminate the risk of contamination or injury from these materials. In the event of contamination or injury resulting from our use of hazardous materials, we could be held liable for any resulting damages, and any liability could exceed our resources, including any available insurance. We could also be held liable for unexpected safety events that could happen in our business offices.

In addition, our leasing and operation of real property may subject us to liability pursuant to certain of these laws or regulations. Under existing U.S. environmental laws and regulations, current or previous owners or operators of real property and entities that disposed or arranged for the disposal of hazardous substances may be held strictly, jointly and severally liable for the cost of investigating or remediating contamination caused by hazardous substance releases, even if they did not know of and were not responsible for the releases.

We could incur significant costs and liabilities which may adversely affect our financial condition and operating results for failure to comply with such laws and regulations, including, among other things, civil or criminal fines and penalties, property damage and personal injury claims, costs associated with upgrades to our facilities or changes to our operating procedures or injunctions limiting or altering our operations.

Although we maintain liability insurance to cover us for costs and expenses we may incur due to injuries to our employees, this insurance may not provide adequate coverage against potential liabilities. We do not maintain insurance for environmental liability or toxic tort claims that may be asserted against us in connection with our storage or disposal of biological, hazardous or radioactive materials.

In addition, we may incur substantial costs in order to comply with current or future environmental, health and safety laws and regulations. These current or future laws and regulations, which are becoming increasingly more stringent, may impair our research, development or production efforts. Our failure to comply with these laws and regulations also may result in substantial fines, penalties or other sanctions.

We are subject to certain U.S. and foreign anti-corruption, anti-money laundering, export control, sanctions and other trade laws and regulations. We can face serious consequences for violations.

U.S. and foreign anti-corruption, anti-money laundering, export control, sanctions and other trade laws and regulations prohibit, among other things, companies and their employees, agents, CROs, CMOs, legal counsel, accountants, consultants, contractors and other partners from authorizing, promising, offering, providing, soliciting, or receiving directly or indirectly, corrupt or improper payments or anything else of value to or from recipients in the public or private sector. Violations of these laws can result in substantial criminal fines and civil penalties, imprisonment, the loss of trade privileges, debarment, tax reassessments, breach of contract and fraud litigation, reputational harm and other consequences. We have direct or indirect interactions with officials and employees of government agencies or government-affiliated hospitals, universities and other organizations. We also expect our non-U.S. activities to increase over time. We expect to rely on third parties for research, preclinical studies and clinical trials and/or to obtain necessary permits, licenses, patent registrations and other marketing authorizations. We can be held liable for the corrupt or other illegal activities of our personnel, agents or partners, even if we do not explicitly authorize or have prior knowledge of such activities.

Any violations of the laws and regulations described above may result in substantial civil and criminal fines and penalties, imprisonment, the loss of export or import privileges, debarment, tax reassessments, breach of contract and fraud litigation, reputational harm and other consequences.

We have limited experience as a commercial company and the sales, marketing, and distribution of OJEMDA or any future approved products may be unsuccessful or less successful than anticipated.

We recently began commercializing our first product, OJEMDA, in the United States. As a company, we had no prior experience commercializing a product. The success of our commercialization efforts for OJEMDA and any future approved products is difficult to predict and subject to the effective execution of our business plan, including, among other things, the continued development of our internal sales, marketing, and distribution capabilities and our ability to navigate the significant expenses and risks involved with the development and management of such capabilities.

There are significant expenses and risks involved with establishing our own sales, marketing, and distribution capabilities, including our ability to hire, retain, and appropriately incentivize qualified individuals, provide adequate training to sales and marketing personnel, and effectively manage geographically dispersed sales and marketing teams to generate sufficient demand. Any failure or delay in the development of these capabilities could delay or negatively affect the success of our commercialization efforts and our business. For example, the ongoing commercialization of OJEMDA may not develop as planned or anticipated, which may require us to, among others, adjust or amend our business plan and incur significant expenses.

Alternatively, we may license certain rights with respect to our products or product candidates to collaborators and rely on the assistance and guidance of those collaborators. We may also seek collaborations to secure marketing authorizations and commercialize our products outside of the United States. We cannot assure that any collaboration(s) will result in short-term or long-term benefit to the company. If we choose to collaborate, either globally or on a territory-by-territory basis, with third parties that have direct sales forces and established distribution systems, either to augment our own sales force and distribution systems or in lieu of our own sales force and distribution systems, we will be required to negotiate and enter into arrangements with such third parties relating to the proposed collaboration and such arrangements may prove to be less profitable than commercializing the product on our own. If we are unable to build our own distribution and marketing capabilities or to find suitable partners for the commercialization of our products and product candidates, we may not generate substantial revenues, if any, from them or be able to reach or sustain profitability.

Given our lack of experience commercializing products, we do not have a track record of successfully executing on the commercialization of an approved product. If we are unsuccessful in accomplishing our objectives and executing on our business plan, or if the commercialization of OJEMDA or any future approved products does not develop as planned, we may require significant additional capital and financial resources, we may not become profitable, and we may not be able to compete against more established companies in our industry.

Risks Related to Our Reliance on Third Parties

We rely, and intend to continue to rely, on third parties to conduct our clinical trials and perform some of our research and potential preclinical studies. If these third parties do not satisfactorily carry out their contractual duties, fail to comply with applicable regulatory requirements or do not meet expected deadlines, our development programs may be delayed or subject to increased costs or we may be unable to obtain marketing authorization, each of which may have an adverse effect on our business, financial condition, results of operations and prospects.

We do not have the ability to independently conduct all aspects of our clinical trials ourselves. As a result, we are dependent on third parties to conduct our ongoing and planned clinical trials of tovorafenib, DAY301 and any preclinical studies and clinical trials of any future products and product candidates. The timing of the initiation and completion of these trials will therefore be partially controlled by such third parties and may result in delays to our development programs. Since such third parties partially control the progress of these trials, they may also publish the data related to these trials prior to obtaining or without our approval for doing so. Specifically, we expect CROs, independent clinical investigators and consultants to play a significant role in the conduct of these trials and the subsequent collection and analysis of data. For example, in addition to the Phase 1 clinical trial run by Dana Farber Cancer Institute in collaboration with PNOC, the Children's Oncology Group, a National Cancer Institute-supported clinical trials group and the world's largest organization devoted exclusively to childhood and adolescent cancer research, is developing a group-wide clinical trial of tovorafenib in relapsed Langerhans cell histiocytosis. However, these investigators, CROs and other third parties are not our employees, and we will not be able to control all aspects of their activities. Nevertheless, we are responsible for ensuring that each clinical trial is conducted in accordance with the applicable protocol and legal, regulatory and scientific standards, and our reliance on the investigators, CROs and other third parties does not relieve us of our regulatory responsibilities. We and our CROs are required to comply with GCP requirements, which are regulations and guidelines enforced by the FDA for products and product candidates in clinical development. Regulatory authorities enforce these GCP requirements through periodic inspections of trial sponsors, clinical trial investigators and clinical trial sites. If we or any of our CROs or clinical trial sites fail to comply with applicable GCP requirements, the data generated in our clinical trials may be deemed unreliable, and the FDA may require us to perform additional clinical trials before approving our marketing applications. We cannot assure you that, upon inspection, the FDA will determine that our clinical trials comply with GCPs. In addition, our clinical trials must be conducted with products and product candidates produced under cGMP regulations. Our failure or the failure of third parties on whom we rely to comply with these regulations may require us to stop and/or repeat clinical trials, which would delay the marketing authorization process.

There is no guarantee that any such CROs, clinical trial investigators or other third parties on which we rely will devote adequate time and resources to our development activities or perform as contractually required. In addition, these third parties may be subject to supply chain or inflationary pressures that limit their ability to achieve anticipated timelines or result a greater cost to us. For example, we are aware of a shortage of non-human primates available for preclinical studies and although that is not expected to impact our current business if we begin new product development programs we could be subject to longer development times or difficulty completing necessary research. If any of these third parties fail to meet expected deadlines, adhere to our clinical protocols or meet regulatory requirements, otherwise perform in a substandard manner or terminate their engagements with us, the timelines for our development programs may be extended or delayed or our development activities may be suspended or terminated. If our clinical trial site terminates for any reason, we may experience the loss of follow-up information on subjects enrolled in such clinical trial unless we are able to transfer those subjects to another qualified clinical trial site, which may be difficult or impossible.

In addition, with respect to investigator-sponsored trials that may be conducted, we would not control the design or conduct of these trials, and it is possible that the FDA will not view these investigator-sponsored trials as providing adequate support for future clinical trials or market approval, whether controlled by us or third parties, for any one or more reasons, including elements of the design or execution of the trials or safety concerns or other trial results. We expect that such arrangements will provide us certain information rights with respect to the investigator-sponsored trials, including access to and the ability to use and reference the data, including for our own regulatory submissions, resulting from the investigator-sponsored trials. However, we would not have control over the timing and reporting of the data from investigator-sponsored trials, nor would we own the data from the investigator-sponsored trials. If we are unable to confirm or replicate the results from the investigator-sponsored trials or if negative results are obtained, we would likely be further delayed or prevented from advancing further clinical development. Further, if investigators or institutions breach their obligations with respect to the clinical development of OJEMDA or our product candidates, or if the data proves to be inadequate compared to the firsthand knowledge we might have gained had the investigator-sponsored trials been sponsored and conducted by us, then our ability to design and conduct any future clinical trials ourselves may be adversely affected. The investigators may design clinical trials with clinical endpoints that are more difficult to achieve, or in other ways that increase the risk of negative clinical trial results compared to clinical trials that we may design on our own. Negative results in investigator-sponsored clinical trials could have a material adverse effect on our efforts to obtain marketing authorization for our product candidates and the public perception of our product candidates. Additionally, the FDA may disagree with the sufficiency of our right of reference to the preclinical, manufacturing or clinical data generated by these investigator-sponsored trials, or our interpretation of preclinical, manufacturing or clinical data from these investigator-sponsored trials. If so, the FDA may require us to obtain and submit additional preclinical, manufacturing or clinical data.

Furthermore, these third parties may also have relationships with other entities, some of which may be our competitors for whom they may also be conducting clinical trials or other pharmaceutical product development activities that could harm our competitive position. If these third parties do not successfully carry out their contractual duties, meet expected deadlines or conduct our clinical trials in accordance with regulatory requirements or our stated protocols, we will not be able to obtain, or may be delayed in obtaining, marketing authorizations for OJEMDA (outside of the United States), DAY301 or any future product candidates and will not be able to, or may be delayed in our efforts to, successfully commercialize our products.

The manufacture of pharmaceutical products, including OJEMDA and our product candidate, DAY301, is complex. Our third-party manufacturers may encounter difficulties in production, which could delay or entirely halt their ability to supply our product candidates for clinical trials or, if approved, for commercial sale.

We do not have any manufacturing facilities, and we currently contract with certain third-party manufacturers in China. We rely, and expect to continue to rely, on third parties for the manufacture of OJEMDA and our product candidates for clinical testing, product development purposes, to support regulatory application submissions, as well as for commercial manufacture of our product candidates. In addition, we expect to contract with analytical laboratories for release and stability testing of OJEMDA and our product candidates. This reliance on third parties increases the risk that we will not have sufficient quantities of OJEMDA or our product candidates or products or such quantities at an acceptable cost or quality, which could delay, prevent or impair our development or commercialization efforts and cause the FDA to withdraw certain designations, including orphan drug designation. For example, we cannot be sure to what extent the supply chain issues caused by geopolitical uncertainty, the imposition of new or additional tariffs or other trade barriers, and public health epidemics, may impact our ability to procure sufficient supplies for the development of OJEMDA and our product candidates and what, if any, impact that may have on our facilities and operations in the region, including but not limited to a decrease or disruption of production, increased costs of production or other interruptions in our supply chain.

In addition, any disruption in production or inability of our manufacturers, specifically in China, to produce adequate quantities to meet our needs, whether as a result of a natural disaster or other causes, could impair our ability to operate our business on a day-to-day basis and to continue our development of OJEMDA and our product candidates. Furthermore, since these manufacturers are located in China, we are exposed to the possibility of product supply disruption and increased costs in the event of economic sanctions, changes in the policies of the United States or Chinese governments, political unrest or unstable economic conditions in China. In April 2025, the U.S. presidential administration announced extensive tariffs on goods imported from China, where certain of our third-party manufacturers are located, and these tariffs remain in negotiation. We believe we currently have sufficient API inventory for commercial and clinical use and do not anticipate any near-term impact to our sales or clinical programs, we expect these tariffs to increase the cost of our API in the future, which would negatively impact our business, financial condition and results of operations. In addition, legislation was introduced in Congress to limit certain U.S. biotechnology companies from using equipment or services produced or provided by select Chinese biotechnology companies, including those affiliated with the manufacture of our API, Wuxi STA, and others in Congress have advocated for the use of existing executive branch authorities to limit those Chinese service providers' ability to engage in business in the United States. Although this legislation has not progressed to law, there is continued discussion on a modified BioSecure Act for potential inclusion in the National Defense Authorization Act, or NDAA, for the fiscal year beginning January 1, 2026. We cannot predict what actions may ultimately be taken with respect to trade relations between the United States and China or other countries, what products and services may be subject to such actions or what actions may be taken by the other countries in retaliation.

Any of these matters could materially adversely affect our business, financial condition and results of operations. In addition, disruptions in logistics routes and transportation capabilities could disrupt our supply chain. And, if we experience unexpected spikes in demand over time, we risk running out of our necessary supplies.

We entered into a manufacturing and supply agreement with Quotient for drug manufacturing of OJEMDA and a packaging agreement with Sharp Corporation, or Sharp, for the packaging and serialization of OJEMDA. Supply chain issues, such as those related to certain packaging material, may negatively impact our ability to package and deliver OJEMDA and our product candidates if not managed effectively. Moreover, if any of our existing or future contract manufacturers or suppliers fail to perform satisfactorily, it could delay development or regulatory approval of our drug candidates or commercialization of our drugs, which could negatively impact our results of operations and business.

We may be unable to enter into additional agreements with third-party manufacturers or suppliers or do so on favorable terms. Our anticipated reliance on a limited number of third party-manufacturers or suppliers exposes us to the following risks:

- reliance on the third party for regulatory, compliance and quality assurance;
- reliance on the third party for product development, analytical testing and data generation to support regulatory applications;
- operations of our third-party manufacturers or suppliers could be disrupted by conditions unrelated to our business or operations, including the bankruptcy of the manufacturer or supplier, the issuance of an FDA Form 483 notice or warning letter or other enforcement action by the FDA or other regulatory authority;

- the possible breach of the manufacturing agreement by the third party;
- the possible misappropriation of our proprietary information, including our trade secrets and know-how;
- the possible termination or nonrenewal of the agreement by the third party at a time that is costly or inconvenient for us;
- carrier disruptions or increased costs that are beyond our control; and
- failure to deliver our drugs under specified storage conditions and in a timely manner.

Third-party manufacturers may not be able to comply with cGMP regulations or similar regulatory requirements outside of the United States. If a Health Authority determines that our CMOs are not in compliance with laws and regulations, including those governing cGMPs, the Health Authority may not approve a regulatory submission until the deficiencies are corrected or we replace the manufacturer in our application with a manufacturer that is in compliance. Moreover, our failure, or the failure of our third-party manufacturers and suppliers, to comply with applicable regulations could result in sanctions being imposed on us, including clinical holds, fines, injunctions, civil penalties, seizures or recalls of product candidates or products, operating restrictions and criminal prosecutions, any of which could significantly and adversely affect supplies of our products. In addition, approved products and the facilities at which they are manufactured are required to maintain ongoing compliance with extensive FDA requirements and the requirements of other similar agencies, including ensuring that quality control and manufacturing procedures conform to cGMP requirements. As such, our CMOs are subject to continual review and periodic inspections to assess compliance with cGMPs. Furthermore, although we do not have day-to-day control over the operations of our CMOs, we are responsible for ensuring compliance with applicable laws and regulations, including cGMPs.

In addition, our third-party manufacturers and suppliers are subject to numerous environmental, health and safety laws and regulations, including those governing the handling, use, storage, treatment and disposal of waste products, and failure to comply with such laws and regulations could result in significant costs associated with civil or criminal fines and penalties for such third parties. Based on the severity of regulatory actions that may be brought against these third parties in the future, our clinical or commercial supply of drug and packaging and other services could be interrupted or limited, which could harm our business.

OJEMDA and our product candidates and any products that we may develop may compete with other product candidates and products for access to manufacturing facilities. As a result, we may not obtain access to these facilities on a priority basis or at all. There are a limited number of manufacturers that operate under cGMP regulations and that might be capable of manufacturing for us.

As we prepare for later-stage clinical trials and commercialization of OJEMDA, we will need to take steps to increase the scale of production. Other than for our product OJEMDA, we have not yet scaled up the manufacturing process for any of our product candidates and may need to scale further to support future supply needs for any of our product candidates. Third-party manufacturers may be unable to successfully increase the manufacturing capacity for any of our product candidates in a timely or cost-effective manner, or at all. In addition, quality issues may arise during scale-up or commercial activities. For example, if microbial, viral or other contaminations are discovered in our product candidates or in the manufacturing facilities in which our product candidates are made, such manufacturing facilities may need to be closed for an extended period of time to investigate and remedy the contamination.

Any performance failure on the part of our existing or future manufacturers could delay clinical development or marketing authorization. If our current CMOs for clinical testing cannot perform as agreed, we may be required to replace such CMOs. Although we believe that there are several potential alternative manufacturers who could manufacture OJEMDA or our product candidates, we may incur added costs and delays in identifying and qualifying any such replacement manufacturer or be able to reach agreement with any alternative manufacturer. Further, our third-party manufacturers may experience manufacturing or shipping difficulties due to resource constraints or as a result of natural disasters, labor disputes, unstable political environments or public health epidemics. If our current third-party manufacturers cannot perform as agreed, we may be required to replace such manufacturers and we may be unable to replace them on a timely basis or at all.

Our current and anticipated future dependence upon others for the manufacture of OJEMDA or our product candidates may adversely affect our future profit margins and our ability to commercialize any products that obtain marketing authorization on a timely and competitive basis.

We rely on a limited number of suppliers for raw materials and any disruptions arising from our sole suppliers could result in delays in our clinical trials or otherwise adversely affect our business and results of operations.

We rely on a limited number of suppliers, some of whom are our sole source for certain materials, and some of whom are based in foreign jurisdictions. Our small number of suppliers involves a number of additional risks, including risks related to supplier capacity constraints, component availability, price increases, timely delivery, component quality, failure of a key supplier to remain in business and adjust to market conditions, including inflation and changes in interest rates, changes in domestic and foreign currency rates, significant political, trade or regulatory developments, including the implementation of tariffs by the United States, China, and other governments, natural disasters, fire, regional geopolitical conflicts, acts of terrorism, pandemics, or other catastrophic events. Further,

in the case of materials for which we have a sole supplier, even if we are able to replace any raw materials or other materials with an alternative, such alternatives may cost more, result in lower yields or not be as suitable for our purposes. In addition, some of the materials that we use to manufacture OJEMDA and our product candidates are complex materials, which may be more difficult to substitute. Therefore, any disruptions arising from our sole suppliers could result in delays and additional regulatory submissions, which may adversely affect our business and results of operations.

The Company is sensitive to increased U.S. tariffs and other trade restrictions, including tariffs specifically on imports from China and tariffs on imports of pharmaceutical products from other jurisdictions. If such tariffs are expanded in scope, increased, or become permanent, the Company could experience higher costs on imported raw materials and product intermediates, which may compress margins. Because the timing, scope and enforceability of such tariffs are uncertain, we may not be able to fully predict or mitigate their impacts. Increased tariffs and other trade restrictions on China could increase annual API costs for OJEMDA. Although the effect of the more recently announced worldwide product-specific Pharmaceutical tariffs is only beginning to materialize, management expects that such measures could further increase costs.

To mitigate tariff risk, we are exploring steps such as (i) diversifying sourcing to lower-tariff jurisdictions, (ii) qualifying alternate domestic suppliers, and (iii) managing inventory levels. However, there can be no assurance these measures will fully offset the impacts of increased tariff.

Our existing License Agreement with Ipsen is important to our business. If Ipsen fails to fulfill its contract obligations, or if any of the Ipsen License Agreement is terminated, our ability to commercialize OJEMDA in territories outside the United States may be delayed or prevented and we may never receive milestone payments or future royalties under the License Agreement.

In July 2024, we entered into the Ipsen License Agreement, pursuant to which we licensed to Ipsen, on an exclusive basis, the right to commercialize tovorafenib in all territories outside the United States and agreed to provide certain research and development and manufacturing services. Ipsen shall have the right to grant sublicenses to third-parties. A significant portion of our future revenue and cash resources may be derived from the Ipsen License Agreement, or other similar agreements into which we may enter in the future.

Under the Ipsen License Agreement, we are eligible to receive up to approximately \$380.0 million in additional commercial launch and sales-based milestone payments based on exchange rates as of the reporting date, as well as tiered, double-digit royalty payments starting at mid-teens percentage of annual net sales of tovorafenib, subject to customary adjustments.

Under the terms of the Ipsen License Agreement, Ipsen will have significant discretion in determining the efforts and resources that they will apply to their marketing efforts and their management of the ex-U.S. regulatory activities and they may not perform their obligations as expected. Disputes may arise between Ipsen and us that result in the delay or termination of the research, development or commercialization of our products or product candidates or that result in costly litigation or arbitration that diverts management attention and resources. Furthermore, they may have changes in their strategic focus or available funding, or experience external factors, such as an acquisition, may divert resources or create competing priorities. Any of these events would have a material adverse effect on our business, financial condition and results of operations.

The Ipsen License Agreement may be terminated by either party for material breach or bankruptcy. In addition, Ipsen may terminate the Ipsen License Agreement after the second anniversary of the effective date for convenience with six months' prior written notice or for certain other specified reasons.

If the Ipsen License Agreement is terminated, then, depending on the event:

- our cash expenditures could increase significantly if it is necessary for us to hire additional employees and allocate internal resources to the commercialization or other activities that were previously shared by Ipsen;
- we would bear all of the risks and costs related to the further commercialization and development activities that were previously the subject of the Ipsen License Agreement;
- in order to fund further commercialization activities, we may need to seek out and establish alternative strategic collaborations with third-party partners, which may not be possible; or
- we may not be able to do so on terms which are acceptable to us, in which case it may be necessary for us to limit the size or scope of one or more of our programs or increase our expenditures and seek additional funding by other means.

Any of these events would have a material adverse effect on our business, financial condition and results of operations.

We may enter into collaborations with third parties for the development and commercialization of our product candidates. If those collaborations are not successful, we may not be able to capitalize on the market potential of our product candidates.

We may seek third-party collaborators for the development and commercialization of our product candidates on a select basis, such as our collaboration with Ipsen with respect to commercialization of tovorafenib in all territories outside the United States. Our likely

collaborators for any future collaboration arrangements include large and mid-size pharmaceutical companies, regional and national pharmaceutical companies and biotechnology companies. We face significant competition in seeking appropriate collaborators. Our ability to reach a definitive agreement for a future collaboration will depend, among other things, upon our assessment of the future collaborator's resources and expertise, the terms and conditions of the proposed collaboration and the proposed collaborator's evaluation of a number of factors.

If we do enter into any additional arrangements with any third parties, we will likely have limited control over the amount and timing of resources that our future collaborators dedicate to the development or commercialization of our product candidates. Our ability to generate revenues from these arrangements will depend on our future collaborators' abilities and efforts to successfully perform the functions assigned to them in these arrangements. Collaborations with future collaborators involving our product candidates would pose numerous risks to us, including the following:

- collaborators have significant discretion in determining the efforts and resources that they will apply to these collaborations and may not perform their obligations as expected;
- collaborators may de-emphasize or not pursue development and commercialization of our product candidates or may elect not to continue or renew development or commercialization programs based on clinical trial results, changes in the collaborator's strategic focus, including as a result of a sale or disposition of a business unit or development function, or available funding or external factors such as an acquisition that diverts resources or creates competing priorities;
- collaborators may delay clinical trials, provide insufficient funding for a clinical trial program, stop a clinical trial or abandon a product candidate, repeat or conduct new clinical trials or require a new formulation of a product candidate for clinical testing;
- collaborators could independently develop, or develop with third parties, products that compete directly or indirectly with our product candidates if the collaborators believe that competitive products are more likely to be successfully developed or can be commercialized under terms that are more economically attractive than ours;
- a collaborator with marketing and distribution rights to multiple products may not commit sufficient resources to the marketing and distribution of our product relative to other products;
- collaborators may reassign manufacturing responsibilities to themselves or a new CMO, which would require that any new manufacturing facility also comply with cGMPs. The FDA or another regulator could decide to conduct an inspection of any new manufacturing facility and a material noncompliance could delay the launch of commercial manufacturing at such facility;
- collaborators may not properly obtain, maintain, defend or enforce our intellectual property rights or may use our proprietary information and intellectual property in such a way as to invite litigation or other intellectual property related proceedings that could jeopardize or invalidate our proprietary information and intellectual property or expose us to potential litigation or other intellectual property related proceedings;
- disputes may arise between the collaborators and us that result in the delay or termination of the research, development or commercialization of our product candidates or that result in costly litigation or arbitration that diverts management attention and resources;
- collaborations may be terminated and, if terminated, may result in a need for additional capital to pursue further development or commercialization of the applicable products or product candidates;
- collaboration agreements may not lead to development or commercialization of our product candidates in the most efficient manner or at all; and
- if a future collaborator of ours were to be involved in a business combination, the continued pursuit and emphasis on our product development or commercialization program could be delayed, diminished or terminated.

If we establish additional collaborations in the future, all of the risks relating to product development, marketing authorization and commercialization described herein would also apply to the activities of any such future collaborators.

The loss of any large customer, or any cancellation or delay of a significant purchase by a large customer, could reduce our net sales and harm our operating results.

We have received a substantial portion of our revenue from a limited number of customers. For example, for the nine months ended September 30, 2025, two individual customers accounted for 98.3% of our total net product revenue, with these individual customers representing 66.4% and 31.9% of total net product revenue. As of September 30, 2025, two customers accounted for 91.7% of the accounts receivable balance, with these individual customers representing 68.5% and 23.2% of the accounts receivable balance.

We cannot provide any assurances that we will retain our current customers or groups of customers, that they will maintain their current or forecasted demand for our products, or that we will be able to attract and retain additional customers in the future. If for any

reason we were to lose our ability to sell to a specific group or class of customers, we could experience a significant reduction in revenue or loss of market share, which would adversely impact our operating results.

Risks Related to Employee Matters and Our Operations

Our future success depends on our ability to retain our executive officers and key employees and to attract, retain and motivate qualified personnel and manage our human capital.

Our ability to compete in the highly competitive biotechnology and pharmaceutical industries depends upon our ability to attract, motivate and retain highly qualified managerial, scientific, medical and commercial personnel. We are highly dependent on the development and management expertise of Jeremy Bender, Ph.D., M.B.A., our Chief Executive Officer, as well as the other members of our management team, other key employees and advisors. We currently do not maintain key person insurance on these individuals. Although we have entered into employment agreements with our executive officers, each of them may terminate their employment with us at any time.

Our industry has experienced a high rate of turnover in recent years. Our ability to compete in the highly competitive pharmaceuticals industry depends upon our ability to attract, retain and motivate highly skilled and experienced personnel with scientific, clinical, regulatory, manufacturing, quality, commercial and management skills and experience.

We largely conduct our operations in the greater San Francisco Bay Area, a region that is home to other pharmaceutical companies as well as many academic and research institutions, resulting in fierce competition for qualified personnel. We may not be able to attract or retain qualified personnel in the future due to the intense competition for a limited number of qualified personnel among pharmaceutical companies. Many of the other pharmaceutical companies against which we compete have greater financial and other resources, different risk profiles and a longer history in the industry than we do. Our competitors may provide higher compensation, more diverse opportunities and/or better opportunities for career advancement. In addition, as our business changes, key personnel may not want to work for a larger, commercial enterprise. Any or all of these competing factors may limit our ability to continue to attract and retain high quality personnel, which could negatively affect our ability to successfully develop and commercialize OJEMDA or our product candidates and to grow our business and operations as currently contemplated. We have adopted a greater level of flexibility in our recruiting practices to attract and hire candidates outside of the San Francisco Bay Area, which is intended to increase retention but could have a negative impact on employee engagement, resulting in greater employee turnover.

We will need to grow the size and capabilities of our organization, and we may experience difficulties in managing this growth.

We had 184 full-time employees as of September 30, 2025. We expect significant growth in the number of our employees and the scope of our operations, particularly in the areas of clinical development, clinical operations, manufacturing, regulatory affairs, sales, marketing and distribution. To manage our anticipated future growth, we must continue to implement and improve our managerial, operational and financial systems, expand our facilities and continue to recruit and train additional qualified personnel. Due to our limited financial resources and the limited experience of our management team in managing a company with such anticipated growth and with developing sales, marketing and distribution infrastructure, we may not be able to effectively manage the expansion of our operations or recruit and train additional qualified personnel. The expansion of our operations may lead to significant costs and may divert our management and business development resources.

Further, we currently rely, and for the foreseeable future will continue to rely, in substantial part on certain third-party contract organizations, advisors and consultants to provide certain services, including assuming substantial responsibilities for the conduct of our clinical trials and the manufacture of OJEMDA, DAY301 or any future product candidates. We cannot assure you that the services of such third-party contract organizations, advisors and consultants will continue to be available to us on a timely basis when needed, or that we can find qualified replacements. In addition, if we are unable to effectively manage our outsourced activities or if the quality or accuracy of the services provided by our vendors or consultants is compromised for any reason, our clinical trials may be extended, delayed or terminated, and we may not be able to obtain marketing authorization of DAY301 or any future product candidates or otherwise advance our business. We cannot assure you that we will be able to properly manage our existing vendors or consultants or find other competent outside vendors and consultants on economically reasonable terms, or at all.

If we are not able to effectively manage growth and expand our organization, we may not be able to successfully implement the tasks necessary to further develop and commercialize OJEMDA, DAY301, our other pipeline product candidates or any future product candidates and, accordingly, may not achieve our research, development and commercialization goals.

Our employees, clinical trial investigators, CROs, CMOs, consultants, vendors and any future commercial partners may engage in misconduct or other improper activities, including non-compliance with regulatory standards and requirements and insider trading.

We are exposed to the risk of fraud or other misconduct by our employees and third parties that we rely on, including, clinical trial investigators, CROs, CMOs, consultants, vendors and any future commercial partners. Misconduct by these parties could include

intentional, reckless and/or negligent conduct or disclosure of unauthorized activities to us that violates: (i) FDA regulations or those of comparable foreign regulatory authorities, including those laws that require the reporting of true, complete and accurate information, (ii) manufacturing (e.g., cGMP) and clinical practice (e.g., GCP) standards, (iii) federal and state health and data privacy, security, fraud and abuse, government price reporting, transparency reporting requirements and other healthcare laws and regulations in the United States and abroad, (iv) sexual harassment and other workplace misconduct, or (v) laws that require the true, complete and accurate reporting of financial information or data. In particular, research, sales, marketing and business arrangements in our industry are subject to a wide variety of laws and regulations that are intended to prevent fraud, misconduct, kickbacks and other abusive practices. Such misconduct could also involve the improper use of information obtained in the course of clinical trials, which could result in regulatory sanctions and cause serious harm to our reputation.

We have adopted a code of conduct applicable to all of our employees, as well as a disclosure program and other applicable policies and procedures, but it is not always possible to identify and deter employee misconduct, and the precautions we take to detect and prevent this activity may not be effective in controlling unknown or unmanaged risks or losses or in protecting us from governmental investigations or other actions or lawsuits stemming from a failure to comply with these laws or regulations.

Further, with respect to third parties, third parties are not our employees, and except for remedies available to us under our agreements, we have limited ability to control resources that any such third party will devote to our preclinical studies or our clinical trials. The third parties we rely on for these services may also have relationships with other entities, some of which may be our competitors, for whom they may also be conducting drug development activities, which could affect their performance on our behalf. Our reliance on third parties for drug development activities means that we will have less direct control over the conduct, timing and completion of studies and the management of data generated from such studies. Nonetheless, we remain responsible for ensuring that our studies and trials are conducted in accordance with applicable protocol, legal and regulatory requirements and scientific standards. In other words, our reliance on third parties does not relieve us of our regulatory responsibilities. For example, we will remain responsible for ensuring that each of our clinical trials is conducted in accordance with the investigational plan and relevant protocols and that any such trial complies with GCP standards. If we or any of our CROs or any clinical trial sites fail to comply with applicable GCP requirements, the clinical data generated in those trials may be deemed unreliable. This may cause the FDA or other comparable foreign regulatory authorities to require us to perform additional clinical trials before approving our marketing applications. If any of the third parties we rely on violates federal or state fraud and abuse or false claims laws and regulations or healthcare privacy and security laws, or other laws, actions may be instituted against us.

If any actions based on our conduct, our employees' conduct or third-party conduct are instituted against us, and we are not successful in defending ourselves or asserting our rights, those actions could have a significant impact on our business, including the imposition of significant civil, criminal and administrative penalties, damages, fines, disgorgement, imprisonment, exclusion from government funded healthcare programs, such as Medicare, Medicaid and other federal healthcare programs, injunctions, private actions brought by individual whistleblowers in the name of the government, debarment or refusal to allow us to enter into government contracts, contractual damages, reputational harm, diminished profits and future earnings, additional integrity reporting and oversight obligations and the curtailment or restructuring of our operations, any of which could adversely affect our ability to operate our business and our results of operations.

Additionally, there are risks that the third parties we rely on could become disqualified, debarred, suspended or otherwise penalized by the FDA or other comparable foreign regulatory authorities for violations of applicable regulatory requirements, in which case we may need to engage a substitute and may not be able to use some or all of the data produced by such contractors in support of our marketing applications.

If our security measures are compromised, or our information technology systems or those of our CROs, CMOs, vendors, contractors, consultants or other third-party partners fail or suffer security breaches, cyber-attacks, loss or leakage of data or other disruptions, this could result in a material disruption of our development programs, compromise sensitive information related to our business or other personal information or prevent us from accessing critical information, potentially exposing us to liability, harm our reputation or otherwise adversely affecting our business.

In the ordinary course of business, we may collect, process, store and transmit proprietary, confidential and sensitive information (including intellectual property, trade secrets, proprietary business information, personal information and protected health information). It is critical that we do so in a secure manner to maintain the confidentiality, integrity and availability of such information. We depend on information technology and telecommunications systems for significant elements of our operations and we utilize, and expect to expand, a number of enterprise software systems that affect a broad range of business processes and functional areas, including, for example, systems handling human resources, financial reporting and controls, customer relationship management, regulatory compliance and other infrastructure operations. We face a number of risks relative to protecting this critical information, including loss of access risk, inappropriate use or disclosure, inappropriate modification and the risk of our being unable to adequately monitor, audit and modify our controls over our critical information. These risks extend to the third parties with whom we work, as we rely on a number of third parties to operate our critical business systems and process confidential, proprietary and sensitive information.

Despite the implementation of security measures, given the size, complexity and increasing amounts of proprietary, confidential and sensitive information maintained by our internal information technology systems and those of our CROs, CMOs, vendors, contractors, consultants and other third-party partners are potentially vulnerable to breakdown, service interruptions, system malfunction, accidents by our personnel or third-party partners, natural disasters, terrorism, global pandemics, war and telecommunication and electrical failures, as well as security breaches from inadvertent or intentional actions by our personnel or those of our CROs, CMOs, vendors, contractors, consultants, business partners and/or other third-party partners, or from cyber-attacks by malicious third parties (including through viruses, worms, malicious code, malware, ransomware, distributed denial-of-service attacks, social engineering and other means to affect service reliability and the confidentiality, integrity and availability of information), which may compromise our system infrastructure, or that of our CROs, CMOs, vendors, contractors, consultants and other third-party partners, or lead to data leakage.

The risk of a security breach or disruption, particularly through cyber-attacks or cyber intrusion, including by computer hackers, viruses, foreign governments and cyber terrorists, has generally increased as the number, intensity and sophistication of attempted attacks and intrusions from around the world have increased. We may not be able to anticipate all types of security threats, nor may we be able to implement preventive measures effective against all such security threats. The techniques used by cyber criminals change frequently, may not be recognized until launched and can originate from a wide variety of sources, including outside groups such as external service providers, organized crime affiliates, terrorist organizations or hostile foreign governments or agencies. To the extent that any disruption or security breach were to result in a loss of, or damage to, our data or applications, or those of our CROs, CMOs, vendors, contractors, consultants and other third-party partners, or inappropriate disclosure of confidential, sensitive or proprietary information, we could incur liability and reputational damage and the further development and commercialization of OJEMDA, DAY301 or any future product candidates could be delayed. Any breach, loss or compromise of proprietary, confidential or sensitive information may also subject us to civil fines and penalties, including under HIPAA, and other relevant state and federal privacy laws in the United States.

The costs related to significant security breaches or disruptions could be material and exceed the limits of the cybersecurity insurance we maintain against such risks. If the information technology systems of our CROs, CMOs, vendors, contractors, consultants and other third-party partners become subject to disruptions or security breaches, we may have insufficient recourse against such third parties and we may have to expend significant resources to mitigate the impact of such an event, and to develop and implement protections to prevent future events of this nature from occurring.

We cannot assure you that our data protection efforts and our investment in information technology will prevent significant breakdowns, data leakages, breaches in our systems, or those of our CROs, CMOs, vendors, contractors, consultants and other third-party partners, or other cyber incidents that could have a material adverse effect upon our reputation, business, operations or financial condition. For example, if such an event were to occur and cause interruptions in our operations, or those of our third-party CROs, CMOs, vendors and other contractors and consultants, it could result in a material disruption of our programs and the development of OJEMDA or our product candidates could be delayed. In addition, the loss of clinical trial data for OJEMDA, DAY301 or any other product candidates could result in delays in our marketing authorization efforts and significantly increase our costs to recover or reproduce the data. Furthermore, significant disruptions of our internal information technology systems or those of our third-party CROs, CMOs, vendors and other contractors and consultants, or security breaches could result in the loss, misappropriation and/or unauthorized access, use or disclosure of, or the prevention of access to, confidential information (including trade secrets or other intellectual property, proprietary business information and personal information), which could result in financial, legal, business and reputational harm to us. If the information technology systems of our CROs, CMOs, vendors, contractors, consultants and other third-party partners become subject to disruptions or security incidents, we may have insufficient recourse against such third parties and we may have to expend significant resources to mitigate the impact of such an event, and to develop and implement protections to prevent future events of this nature from occurring.

Any event that leads to unauthorized access, use or disclosure of personal information, including personal information regarding our clinical trial subjects or personnel, could harm our reputation directly, compel us to comply with federal and/or state breach notification laws and foreign law equivalents, subject us to mandatory corrective action and otherwise subject us to liability under laws and regulations that protect the privacy and security of personal information, which could result in significant legal and financial exposure and reputational damages that could potentially have an adverse effect on our business.

We may have contractual and other legal obligations to notify relevant stakeholders of security breaches. Most jurisdictions have enacted laws requiring companies to notify individuals, regulatory authorities and others of security breaches involving certain types of data. Such mandatory disclosures are costly, could lead to negative publicity, may cause our customers to lose confidence in the effectiveness of our security measures and require us to expend significant capital and other resources to respond to and/or alleviate problems caused by the actual or perceived security breach.

The costs to respond to a security breach and/or to mitigate any security vulnerabilities that may be identified could be significant, our efforts to address these issues may not be successful and these issues could result in interruptions, delays, negative publicity, loss of customer trust or diminished use of our products, as well as other harms to our business and our competitive position. Remediation of

any potential security breach may involve significant time, resources and expenses. Any security breach may result in regulatory inquiries, litigation or other investigations, and can affect our financial and operational condition.

Unauthorized access to our systems, networks or physical facilities could result in litigation with our customers or other relevant stakeholders. These proceedings could force us to spend money in defense or settlement, divert management's time and attention, increase our costs of doing business or adversely affect our reputation.

While we maintain cybersecurity insurance coverage, we may not have adequate insurance coverage for security incidents or breaches, including fines, judgments, settlements, penalties, costs, attorney fees and other impacts that arise out of incidents or breaches. The successful assertion of one or more large claims against us that exceeds our available insurance coverage, or results in changes to insurance policies (including premium increases or the imposition of large deductible or co-insurance requirements), could have an adverse effect on our business. In addition, we cannot be sure that our existing insurance coverage and coverage for errors and omissions will continue to be available on acceptable terms or that our insurers will not deny coverage as to any future claim. Our risks are likely to increase as we continue to expand, grow our customer base and process, store and transmit increasingly large amounts of proprietary and sensitive data.

We are subject to stringent and changing laws, regulations and standards, and contractual obligations related to privacy, data protection and data security. The actual or perceived failure to comply with such obligations could lead to government enforcement actions (which could include civil or criminal penalties), fines and sanctions, private litigation and/or adverse publicity and could negatively affect our operating results and business.

We and third parties who we work with are or may become subject to numerous domestic and foreign data protection laws and regulations (i.e., laws and regulations that address privacy and data security), the scopes of which are changing, subject to differing applications and interpretations, and may be inconsistent among states, countries, or conflict with other requirements. We are or may become subject to the terms of contractual obligations related to privacy, data protection and data security. The actual or perceived failure by us or related third parties to comply with such obligations could increase our compliance and operational costs, expose us to regulatory scrutiny, actions, fines and penalties, result in reputational harm, lead to a loss of customers, result in litigation and liability and otherwise cause a material adverse effect on our business, financial condition and results of operations.

In the United States, numerous federal and state laws and regulations, including health information privacy and security laws, data breach notification laws, health information privacy laws and consumer protection laws that govern the collection, use, disclosure and protection of health-related and other personal information could apply to our operations or the operations of our collaborators. In addition, we may obtain protected health information from third parties (including research institutions from which we obtain clinical trial data) that are subject to privacy and security requirements under HIPAA, as amended by HITECH. Depending on the facts and circumstances, we could be subject to civil and criminal penalties if we obtain, use or disclose individually identifiable health information maintained by a HIPAA-covered entity in a manner that is not authorized or permitted by HIPAA.

Over a third of U.S. states have adopted comprehensive privacy and security laws and regulations, which govern the privacy, processing and protection of personal information, including certain specific requirements and laws with respect to health-related information. For example, Washington state has passed the My Health My Data Act, which is focused on the collection of consumer health data, which has a broader scope than HIPAA and includes a private right of action.

In California, the CCPA, grants individual privacy rights for California consumers and places increased privacy and data security obligations on entities handling personal information of consumers or households. The CCPA is enforced by the California Privacy Protection Agency, or the CPPA. The CCPA gives California residents expanded privacy rights, including the right to request correction, access and deletion of their personal information, the right to opt out of certain personal information sharing, and the right to receive detailed information about how their personal information is processed, including by California residents' employers, and provides for civil penalties and a private right of action for data breaches that is expected to increase data breach litigation.

We are also subject to foreign data protection laws, including the General Data Protection Regulation, or GDPR, which applies to personal information (including health-related data) obtained from individuals in the European Economic Area, or the EEA (as well as substantially similar laws that govern the collection of data from individuals in the UK and Switzerland). The GDPR imposes strict obligations on businesses, including requiring changes to informed consent practices and more detailed notices for clinical trial subjects and investigators, requiring limitations on data processing, establishing a legal basis for processing personal information, notification of data processing obligations, notification of security incidents to appropriate data protection authorities or data subjects, protecting the security and confidentiality of the personal information, and establishing means for data subjects to exercise rights in relation to their personal information. The GDPR subjects noncompliant companies to fines of up to the greater of 20 million Euros (17.5 million GBP in the UK) or 4% of their global annual revenues, potential bans on processing of personal information (including clinical trials), and private litigation. To the extent applicable, the GDPR will increase our responsibility and liability in relation to personal information that we process, and we may be required to put in place additional mechanisms and expend additional time and resources to ensure compliance with the EU data protection rules. Changes in international legislations may add additional complexity, variation in requirements, restrictions and potential legal risk, require additional investment in resources for compliance

programs, could impact strategies and availability of previously useful data, and could result in increased compliance costs and/or changes in business practices and policies. In addition, supervisory authorities in the EEA, Switzerland, and the UK have enforced data protection legislation inconsistently, which may result in us having to spend additional resources in order to comply with rules and guidance applicable only in certain, local jurisdictions.

Further, European data protection laws generally prohibit the transfer of personal information to countries outside of the EEA, UK and Switzerland, such as the United States, which are not considered by their relevant authorities to provide an adequate level of data protection. Switzerland has adopted similar restrictions. Although there are legal mechanisms to allow for the transfer of personal information from the EEA, UK, and Switzerland to the United States and other countries, they are or may become subject to legal challenges that, if successful, could invalidate these mechanisms, restrict our ability to process personal information of Europeans outside of Europe and adversely impact our business. If we cannot implement a valid compliance mechanism for cross-border data transfers, we may face increased exposure to regulatory actions, substantial fines, and injunctions against processing or transferring personal data from Europe or other foreign jurisdictions. In the EU and other markets, potential new rules and restrictions on the flow of data across borders could increase the cost and complexity of doing business in those regions.

In addition, further to the UK's exit from the EU on January 31, 2020, the GDPR ceased to apply in the UK at the end of the transition period on December 31, 2020. However, as of January 1, 2021, the United Kingdom's European Union (Withdrawal) Act 2018 incorporated the GDPR (as it existed on December 31, 2020 but subject to certain UK-specific amendments) into UK law, referred to as the UK GDPR. The UK GDPR and the UK Data Protection Act 2018 set out the UK's data protection regime, which is independent from but aligned to the EU's data protection regime. Non-compliance with the UK GDPR may result in monetary penalties of up to £17.5 million or 4% of worldwide revenue, whichever is higher. With respect to transfers of personal data from the EU to the United Kingdom, on June 28, 2021 the European Commission issued an adequacy decision in respect of the UK's data protection framework, enabling data transfers from EU member states to the UK to continue without requiring organizations to put in place contractual or other measures in order to lawfully transfer personal data between the territories. While it is intended to last for at least four years, the European Commission may unilaterally revoke the adequacy decision at any point, and if this occurs it could lead to additional costs and increase our overall risk exposure.

Other countries, including China, Brazil, Australia and Japan, for example, have adopted certain legal requirements for local storage and processing of data and cross-border transfers of personal information, any and all of which could increase the cost and complexity of conducting preclinical testing and clinical trials or delivering our future products, if any, and operating our business. These obligations may be interpreted and applied in a manner that is inconsistent from one jurisdiction to another and may conflict with other requirements or our practices.

We are or may become subject to the terms of external and internal privacy and security policies, representations, certifications and publications related to privacy and security.

Compliance with domestic and foreign privacy, data security and data protection laws, regulations and contractual and other obligations could require us to take on more onerous obligations in our contracts, restrict our ability to collect, use and disclose data, or in some cases, impact our ability to operate in certain jurisdictions. The actual or perceived failure to comply with domestic and foreign privacy, data privacy and data protection laws and regulations could result in government enforcement actions (which could include civil, criminal and administrative penalties), private litigation and/or adverse publicity and could negatively affect our operating results and business. Moreover, clinical trial subjects about whom we or our potential collaborators obtain information, as well as the providers who share this information with us, may contractually limit our ability to use and disclose the information. Claims that we have violated individuals' privacy rights, failed to comply with privacy, data security and data protection laws or breached our contractual obligations, even if we are not found liable, could be expensive and time consuming to defend and could result in adverse publicity that could harm our business.

Investors' expectations of our performance relating to environmental, social and governance factors may impose additional costs and expose us to new risks.

There is continued focus from certain regulators, investors, employees, users and other stakeholders concerning corporate responsibility, specifically related to ESG matters both in the United States and internationally. Some investors may use these non-financial performance factors to guide their investment strategies and, in some cases, may choose not to invest in us if they believe our policies and actions relating to corporate responsibility are inadequate. We may face reputational damage in the event that we do not meet the ESG standards set by various constituencies.

Further, ESG initiatives, goals or commitments could be difficult to achieve or costly to implement. If our competitors' corporate social responsibility performance is perceived to be better than ours, potential or current investors may elect to invest with our competitors instead. Moreover, California recently adopted two new climate-related bills, which require companies doing business in California that meet certain revenue thresholds to publicly disclose certain greenhouse gas emissions data and climate-related financial risk reports, and compliance with such requirements could require significant effort and resources. In addition, in recent years "anti-ESG" sentiment has gained momentum across the United States, with several states and Congress having proposed or enacted

“anti-ESG” policies, legislation, or initiatives or issued related legal opinions, and the U.S. presidential administration having recently issued an executive order opposing diversity equity and inclusion (“DEI”) initiatives in the private sector. Our business may face increased scrutiny related to these activities and our related disclosures, including from the investment community, and our failure to achieve progress or manage the dynamic public sentiment and legal landscape in these areas on a timely basis, or at all, could adversely affect our reputation, business, and financial performance.

We or the third parties upon whom we depend may be adversely affected by natural disasters and our business continuity and disaster recovery plans may not adequately protect us from a serious disaster.

Our current operations are primarily located in the San Francisco Bay Area. Any unplanned event, such as earthquake, flood, fire, explosion, extreme weather conditions, medical epidemic or pandemic, power shortage, telecommunication failure or other natural or man-made accident or incident that results in our being unable to fully utilize our facilities, or the manufacturing facilities of our third-party contract manufacturers, may have a material and adverse effect on our ability to operate our business, particularly on a daily basis, and have significant negative consequences on our financial and operating conditions. In addition, the long-term effects of climate change on general economic conditions and the pharmaceutical industry in particular are unclear, and may heighten or intensify existing risk of natural disasters. Loss of access to these facilities may result in increased costs, delays in the development of OJEMDA or our product candidates or interruption of our business operations, and have a material adverse effect on our business, financial condition, results of operations and prospects. If a natural disaster, power outage or other event occurred that prevented us from using all or a significant portion of our headquarters, that damaged critical infrastructure such as our research facilities or the manufacturing facilities of our third-party contract manufacturers, or that otherwise disrupted operations, it may be difficult or, in certain cases, impossible, for us to continue our business for a substantial period of time. The disaster recovery and business continuity plans we have in place may prove inadequate in the event of a serious disaster or similar event. We may incur substantial expenses as a result of the limited nature of our disaster recovery and business continuity plans, which could have a material adverse effect on our business. As part of our risk management policy, we maintain insurance coverage at levels that we believe are appropriate for our business. However, in the event of an accident or incident at these facilities, we cannot assure you that the amounts of insurance will be sufficient to satisfy any damages and losses. If our facilities, or the manufacturing facilities of our third-party contract manufacturers, are unable to operate because of an accident or incident or for any other reason, even for a short period of time, any or all of our research and development programs may be harmed. Any business interruption could have a material and adverse effect on our business, financial condition, results of operations and prospects.

Changes in tax laws or regulations that are applied adversely to us may have a material adverse effect on our business, cash flow, financial condition or results of operations.

New income, sales, use or other tax laws, statutes, rules, regulations or ordinances could be enacted at any time, which could adversely affect our business operations and financial performance. Further, existing tax laws, statutes, rules, regulations or ordinances could be interpreted, changed, modified or applied adversely to us. For example, future changes in corporate tax rates, the realization of net deferred tax assets relating to our operations, the taxation of foreign earnings, and the deductibility of expenses could have a material impact on the value of our deferred tax assets, could result in significant one-time charges, and could increase our future U.S. tax expense. In addition, under the Tax Cuts and Jobs Act, as amended by the OBBBA, for tax years beginning after December 31, 2021, taxpayers are required to capitalize and amortize certain research and development expenditures over fifteen years if incurred in foreign jurisdictions. For tax years beginning after December 31, 2021, and beginning on or before December 31, 2024, taxpayers generally were required to capitalize and amortize certain research and development expenditures over five years if incurred in the United States; however, beginning after that period, the OBBBA restored immediate deductibility of research and development expenditures incurred in the United States and also permits certain small business taxpayers to apply these changes retroactively to tax years beginning after December 31, 2021.

Effective for transactions occurring on or after January 1, 2023, the Inflation Reduction Act imposed a new one percent excise tax on certain repurchases of stock by publicly traded U.S. domestic corporations. The excise tax is imposed on the repurchasing corporation itself, not its shareholders from which shares are repurchased. For purposes of calculating the base excise tax, repurchasing corporations are permitted to net the fair market value of certain new stock issuances against the fair market value of stock repurchases during the same taxable year. Certain repurchases are not counted in the base of the excise tax.

Future changes in corporate tax rates, the realization of net deferred tax assets relating to our operations, the taxation of foreign earnings, and the deductibility of expenses under the Tax Cuts and Jobs Act, or future reform legislation could have a material impact on the value of our deferred tax assets, could result in significant one-time charges, and could increase our future U.S. tax expense.

Our ability to use our net operating loss carryforwards and certain other tax attributes may be limited.

We have incurred substantial losses during our history and do not expect to become profitable in the near future, and we may never achieve profitability. U.S. federal net operating losses generated in tax years beginning after December 31, 2017, will not expire and

may be carried forward indefinitely but the deductibility of such federal net operating losses is limited to 80% of current year taxable income (without regard to certain deductions).

In addition, both our current and our future unused losses and other tax attributes may be subject to limitation under Sections 382 and 383 of the Internal Revenue Code of 1986, as amended, or the Code, if we undergo, or have undergone, an “ownership change,” generally defined as a greater than 50 percentage point change (by value) in our equity ownership by certain stockholders over a three-year period. During the year ended December 31, 2024, the Company has completed a Section 382 study to determine whether an ownership change per the provisions of Section 382 of the Internal Revenue Code, as well as similar state provisions, has occurred. The study found that one of our predecessor companies experienced an ownership change on June 16, 2022 and that the tax attributes that it generated are subject to a change pursuant to Section 382; however, based on the study all of these attributes are fully available for use as of December 31, 2023. The Company’s current year utilization of net operating losses and income tax credits is not impacted by the provisions of Section 382 or 383. As a result, if we undergo an ownership change (or if we previously underwent such an ownership change), our ability to use our pre-change net operating loss carryforwards and other pre-change tax attributes (such as research tax credits) to offset our post-change income or taxes may be limited. Similar provisions of state tax law may also apply to limit our use of accumulated state tax attributes. In addition, at the state level, there may be periods during which the use of net operating losses is suspended or otherwise limited, which could accelerate or permanently increase state taxes owed. As a result, even if we attain profitability, we may be unable to use all or a material portion of our net operating losses and other tax attributes, which could adversely affect our future cash flows.

We have engaged, and will continue to engage, in strategic transactions that could impact our liquidity, increase our expenses and present significant distractions to our management.

We have engaged in strategic transactions, for instance, with affiliates of Takeda Pharmaceutical Company Limited, Viracta Therapeutics, Inc., Merck KGaA, Darmstadt, Germany, MabCare, and Ipsen, and from time to time, we may consider further strategic transactions, such as acquisitions of companies, businesses or assets and out-licensing or in-licensing of products, product candidates (such as DAY301) or technologies. Additional potential transactions that we may consider include a variety of different business arrangements, including spin-offs, strategic partnerships, joint ventures, restructurings, divestitures, business combinations and investments. Any such transaction may require us to incur non-recurring or other charges, may increase our near term or long-term expenditures and may pose significant integration challenges or disrupt our management or business, which could adversely affect our operations and financial results. For example, these transactions may entail numerous operational and financial risks, including:

- exposure to unknown liabilities;
- disruption of our business and diversion of our management’s time and attention in order to develop acquired products, product candidates or technologies;
- incurrence of substantial debt or dilutive issuances of equity securities to pay for acquisitions;
- higher than expected acquisition and integration costs;
- write-downs of assets or goodwill or impairment charges;
- increased amortization expenses;
- difficulty and cost in combining the operations, systems and personnel of any acquired businesses with our operations, systems and personnel;
- impairment of relationships with key suppliers or customers of any acquired businesses due to changes in management and ownership; and
- inability to retain key employees of any acquired businesses.

Risks Related to Our Intellectual Property

If we are unable to obtain and maintain patent protection or other necessary rights for our products and technology, or if the scope of the patent protection obtained is not sufficiently broad or our rights under our patents (owned, co-owned or licensed) is not sufficiently broad, our competitors could develop and commercialize products and technology similar or identical to ours, and our ability to successfully commercialize our products and technology may be adversely affected.

Our commercial success depends in part on our ability to obtain and maintain proprietary or intellectual property protection in the United States and other countries for OJEMDA and our current product candidates and future products, as well as our core technologies, including our manufacturing know-how. We strive to protect and enhance the proprietary technology, inventions and improvements that are commercially important to the development of our business by seeking, maintaining and defending our intellectual property, whether developed internally or licensed from third parties. We also rely on trade secrets, know-how, continuing

technological innovation and in-licensing opportunities to develop, strengthen and maintain our proprietary position in the field of cancer drug development. Additionally, we intend to rely on regulatory protection afforded through rare drug designations, data exclusivity and market exclusivity as well as patent term extensions, where available.

The patent position of biotechnology and pharmaceutical companies generally is highly uncertain, involves complex legal and factual questions and has in recent years been the subject of much litigation. The degree of patent protection we require to successfully compete in the marketplace may be unavailable or severely limited in some cases and may not adequately protect our rights or permit us to gain or keep any competitive advantage. We cannot provide any assurances that any of our own or licensed patent applications will mature into issued patents, and cannot provide any assurances that any such patents, if issued, will include claims with a scope sufficient to protect OJEMDA and our current and future product candidates or otherwise provide any competitive advantage. Additionally, patents can be enforced only in those jurisdictions in which the patent has issued. Furthermore, patents have a limited lifespan. In the United States, the natural expiration of a patent is generally 20 years after its first nonprovisional U.S. filing. The natural expiration of a patent outside of the United States varies in accordance with provisions of applicable local law, but is generally 20 years from the earliest local filing date. Various extensions may be available; however, the life of a patent, and the protection it affords, is limited. Given the amount of time required for the development, testing and regulatory review of new product candidates, patents protecting such candidates might expire before or shortly after such candidates are commercialized.

Moreover, our exclusive licenses may be subject to field restrictions and retained rights, which may adversely impact our competitive position. See “Management’s Discussion and Analysis of Financial Condition and Results of Operations—Significant Agreements.” Our licensed patent portfolio may not provide us with adequate and continuing patent protection sufficient to exclude others from commercializing products similar to OJEMDA and our product candidates, including generic versions of such products. In addition, the patent portfolio licensed to us is, or may be, licensed to third parties outside our licensed field, and such third parties may have certain enforcement rights. Thus, patents licensed to us could be put at risk of being invalidated or interpreted narrowly in litigation filed by or against another licensee or in administrative proceedings brought by or against another licensee in response to such litigation or for other reasons.

Other parties have developed technologies that may be related or competitive to our own and such parties may have filed or may file patent applications, or may have received or may receive patents, claiming inventions that may overlap or conflict with those claimed in our own patent applications or issued patents. Publications of discoveries in the scientific literature often lag behind the actual discoveries, and patent applications in the United States and in other jurisdictions are typically not published until 18 months after filing, or in some cases not at all. Therefore, we cannot know with certainty whether the inventors of our patents and applications were the first to make the inventions claimed in those patents or pending patent applications, or that they were the first to file for patent protection of such inventions. Further, we cannot assure you that all of the potentially relevant prior art relating to our patents and patent applications has been found. If such prior art exists, it can invalidate a patent or prevent a patent from issuing from a pending patent application. As a result, the issuance, scope, validity and commercial value of our patent rights cannot be predicted with any certainty. Further, if the breadth or strength of protection provided by our patents and patent applications is threatened, regardless of the outcome, it could dissuade companies from collaborating with us to license, develop or commercialize OJEMDA or our current or future product candidates.

In addition, the patent prosecution process is expensive and time-consuming, and we or our licensors may not be able to file and prosecute all necessary or desirable patent applications at a reasonable cost or in a timely manner. In addition, the scope of the claims initially submitted for examination may be significantly narrowed by the time they issue, if at all. It is also possible that we or our licensors will fail to identify patentable aspects of our research and development output before it is too late to obtain patent protection. We cannot provide any assurances that we will be able to pursue or obtain additional patent protection based on our research and development efforts, or that any such patents or other intellectual property we generate will provide any competitive advantage. Moreover, we do not have the right to control the preparation, filing and prosecution of patent applications, or to control the maintenance of the patents, covering technology that we license from third parties. Therefore, these patents and applications may not be filed, prosecuted or maintained in a manner consistent with the best interests of our business.

Further, recent reforms and changes at government agencies of the United States and those of non-U.S. jurisdictions could increase the delays, uncertainties and costs surrounding the prosecution of our patent applications, and the maintenance, enforcement, or defense of our issued patents. For example, the ability of the USPTO and other applicable patent authorities to properly administer their functions is highly dependent on the levels of funding available to the agency and their ability to retain personnel and fill key leadership appointments, among various factors. Termination of employees or delays in replacing or hiring for positions could significantly impact the ability of the USPTO and other applicable patent authorities to fulfill their functions and could greatly impact our ability to timely and adequately prosecute or maintain our patent applications, and our ability to timely and adequately maintain, enforce, or defend our issued patents.

Even if we acquire patent protection that we expect should enable us to maintain competitive advantage, the issuance of a patent is not conclusive as to its inventorship, scope, validity or enforceability. Third parties, including competitors, may challenge the inventorship, scope, validity or enforceability thereof, which may result in such patents being narrowed, invalidated or held unenforceable. If issued, our patents may be challenged in patent offices in the United States and abroad, or in court. For example, we

may be subject to a third-party submission of prior art to the U.S. Patent and Trademark Office, or USPTO, challenging the validity of one or more claims of our patents, once issued. Such submissions may also be made prior to a patent's issuance, precluding the granting of a patent based on one of our patent applications. We may become involved in opposition, reexamination, *inter partes* review, post-grant review, derivation, interference or similar proceedings in the United States or abroad challenging the claims of our patents, once issued. Furthermore, patents may be challenged in court, once issued.

Competitors may claim that they invented the inventions claimed in such patents or patent applications prior to the inventors of our patents, or may have filed patent applications before the inventors of our patents did. A competitor may also claim that we are infringing its patents and that we therefore cannot practice our technology as claimed under our patent applications and patents, if issued. As a result, one or more claims of our patents may be narrowed or invalidated. In litigation, a competitor could claim that our patents, if issued, are not valid for a number of reasons. If a court agrees, we would lose our rights to those challenged patents.

Even if they are unchallenged, our patents and pending patent applications, if issued, may not provide us with any meaningful protection or prevent competitors from designing around our patent claims to circumvent our patents by developing similar or alternative technologies or therapeutics in a non-infringing manner. For example, even if we have a valid and enforceable patent, we may not be able to exclude others from practicing our invention if the other party can show that they used the invention in commerce before our filing date or the other party benefits from a compulsory license. If the patent protection provided by the patents and patent applications we hold or pursue with respect to OJEMDA or our product candidates is not sufficiently broad to impede such competition, our ability to successfully commercialize OJEMDA or our product candidates could be negatively affected, which would harm our business. Certain regulatory exclusivities may be available, however, the scope of such regulatory exclusivities is subject to change and may not provide us with adequate and continuing protection sufficient to exclude others from commercializing products similar to OJEMDA and our product candidates.

If the scope of any patent protection we obtain is not sufficiently broad, or if we lose any of our patent protection, our ability to prevent our competitors from commercializing similar or identical products or product candidates would be adversely affected.

The patent position of biopharmaceutical companies generally is highly uncertain, involves complex legal and factual questions and has been the subject of much litigation in recent years. As a result, the issuance, scope, validity, enforceability and commercial value of our patent rights are highly uncertain. Our pending and future patent applications and those of our licensors may not result in patents being issued which protect our product candidates or which effectively prevent others from commercializing competitive product candidates.

Moreover, the coverage claimed in a patent application can be significantly reduced before the patent is issued, and its scope can be reinterpreted after issuance. Even if patent applications we own or in-license in the future issue as patents, they may not issue in a form that will provide us with any meaningful protection, prevent competitors or other third parties from competing with us or otherwise provide us with any competitive advantage. Any patents that we own or in-license may be challenged or circumvented by third parties or may be narrowed or invalidated as a result of challenges by third parties. Consequently, we do not know whether our product candidates will be protectable or remain protected by valid and enforceable patents. Our competitors or other third parties may be able to circumvent our patents or the patents of our licensors by developing similar or alternative technologies or products in a non-infringing manner which could materially adversely affect our business, financial condition, results of operations and prospects.

The issuance of a patent is not conclusive as to its inventorship, scope, validity or enforceability, and our patents or the patents of our licensors may be challenged in the courts or patent offices in the United States and abroad. We may be subject to a third-party pre-issuance submission of prior art to the USPTO, or become involved in opposition, derivation, revocation, reexamination, post-grant review and *inter partes* review, or other similar proceedings challenging our owned patent rights. An adverse determination in any such submission, proceeding or litigation could jeopardize patent term adjustment or otherwise reduce patent term, reduce the scope of or invalidate or render unenforceable, our patent rights, or allow third parties to commercialize our product candidates and compete directly with us, without payment to us. Moreover, our patents or the patents of our licensors may become subject to post-grant challenge proceedings, such as oppositions in a foreign patent office, that challenge our priority of invention or other features of patentability with respect to our patents and patent applications and those of our licensors. Such challenges may result in loss of patent rights, loss of exclusivity or in patent claims being narrowed, invalidated or held unenforceable, which could limit our ability to stop others from using or commercializing similar or identical technology and products, or limit the duration of the patent protection of our product candidates. Such proceedings also may result in substantial cost and require significant time from our scientists and management, even if the eventual outcome is favorable to us. In addition, if the breadth or strength of protection provided by our patents and patent applications or the patents and patent applications of our licensors is threatened, regardless of the outcome, it could dissuade companies from collaborating with us to license, develop or commercialize current or future product candidates.

Furthermore, the issuance of a patent does not give us the right to practice the patented invention. Third parties may have blocking patents that could prevent us from marketing our own patented product and practicing our own patented technology.

Intellectual property rights do not necessarily address all potential threats to our competitive advantage.

The degree of future protection afforded by our intellectual property rights is uncertain because intellectual property rights have limitations, and may not adequately protect our business or permit us to maintain our competitive advantage. For example:

- others may be able to develop products that are similar to OJEMDA and our product candidates but that are not covered by the claims of the patents that we own or license;
- we or our licensors or collaborators might not have been the first to make the inventions covered by the issued patents or patent application that we own or license;
- we or our licensors or collaborators might not have been the first to file patent applications covering certain of our inventions;
- others may independently develop similar or alternative technologies or duplicate any of our technologies without infringing our intellectual property rights;
- it is possible that the pending patent applications we own or license will not lead to issued patents;
- issued patents that we own or license may be held invalid or unenforceable, as a result of legal challenges by our competitors;
- our competitors might conduct research and development activities in countries where we do not have patent rights and then use the information learned from such activities to develop competitive products for sale in our major commercial markets;
- we may not develop additional proprietary technologies that are patentable;
- the patents of others may have an adverse effect on our business;
- we may fail to adequately protect and police our trademarks and trade secrets; and
- we may choose not to file a patent in order to maintain certain trade secrets or know-how, and a third party may subsequently file a patent covering such intellectual property.

Should any of these events occur, it could significantly harm our business, results of operations and prospects.

Our commercial success depends significantly on our ability to operate without infringing the patents and other proprietary rights of third parties. Claims by third parties that we infringe their proprietary rights may result in liability for damages or prevent or delay our developmental and commercialization efforts.

Our commercial success depends in part on avoiding infringement of the patents and proprietary rights of third parties. However, our research, development and commercialization activities may be subject to claims that we infringe or otherwise violate patents or other intellectual property rights owned or controlled by third parties. Other entities may have or obtain patents or proprietary rights that could limit our ability to make, use, sell, offer for sale or import our product candidates and products that may be approved in the future, or impair our competitive position. There is a substantial amount of litigation, both within and outside the United States, involving patent and other intellectual property rights in the biopharmaceutical industry, including patent infringement lawsuits, oppositions, reexaminations, *inter partes* review proceedings and post grant review proceedings before the USPTO and/or corresponding foreign patent offices. Numerous third-party U.S. and foreign issued patents and pending patent applications exist in the fields in which we are developing product candidates. There may be third-party patents or patent applications with claims to materials, formulations, methods of manufacture or methods for treatment related to the use or manufacture of our product candidates.

There may also be patent applications that, if issued as patents, could be asserted against us. Patent applications in the United States and elsewhere are typically published approximately 18 months after the earliest filing for which priority is claimed, with such earliest filing date being commonly referred to as the priority date. Certain U.S. patent applications that will not be filed outside the United States can remain confidential until patents issue. Therefore, patent applications covering our product candidates could have been filed by third parties without our knowledge. Additionally, pending patent applications that have been published can, subject to certain limitations, be later amended in a manner that could cover our product candidates and their uses or manufacturing processes. The scope of a patent claim is determined by an interpretation of the law, the written disclosure in a patent and the patent's prosecution history and can involve other factors such as expert opinion. Our interpretation of the relevance or the scope of claims in a patent or a pending application may be incorrect, which may negatively impact our ability to market our product candidates. Further, we may incorrectly determine that our product candidates and their uses and manufacturing processes are not covered by a third-party patent or may incorrectly predict whether a third-party's pending patent application will issue with claims of relevant scope. Our determination of the expiration date of any patent in the United States or abroad that we consider relevant may be incorrect, which may negatively impact our ability to develop and market our product candidates. Third-party intellectual property right holders may also actively bring infringement or other intellectual property-related claims against us, even if we have received patent protection for our product candidates and the relevant uses and processes.

As the biopharmaceutical industry expands and more patents are issued, the risk increases that our product candidates may be subject to claims of infringement of the patent rights of third parties. Because patent applications are maintained as confidential for a certain period of time, until the relevant application is published, we may be unaware of third-party patents that may be infringed by commercialization of any of our product candidates, and we cannot be certain that we were the first to file a patent application related to a product candidate or technology. Moreover, because patent applications can take many years to issue, there may be currently pending patent applications that may later result in issued patents that our product candidates may infringe. In addition, identification of third-party patent rights that may be relevant to our technology is difficult because patent searching is imperfect due to differences in terminology among patents, incomplete databases and the difficulty in assessing the meaning of patent claims. There is also no assurance that there is not prior art of which we are aware, but which we do not believe is relevant to our business, which may, nonetheless, ultimately be found to limit our ability to make, use, sell, offer for sale or import our products that may be approved in the future, or impair our competitive position. In addition, third parties may obtain patents in the future and claim that use of our technologies infringes upon these patents. Any claims of patent infringement asserted by third parties would be time consuming and could:

- result in costly litigation that may cause negative publicity;
- divert the time and attention of our technical personnel and management;
- cause development delays;
- prevent us from commercializing OJEMDA or any of our product candidates until the asserted patent expires or is held finally invalid or not infringed in a court of law;
- require us to develop non-infringing technology, which may not be possible on a cost-effective basis;
- subject us to significant liability to third parties; or
- require us to enter into royalty or licensing agreements, which may not be available on commercially reasonable terms, or at all, or which might be non-exclusive, which could result in our competitors gaining access to the same technology.

Although no third party has asserted a claim of patent infringement against us as of September 30, 2025, others may hold proprietary rights that could prevent OJEMDA or our product candidates from being marketed. It is possible that a third-party may assert a claim of patent infringement directed at OJEMDA or our product candidates. Any patent-related legal action against us claiming damages and seeking to enjoin commercial activities relating to OJEMDA or our product candidates, treatment indications, or processes could subject us to significant liability for damages, including treble damages if we were determined to willfully infringe, and require us to obtain a license to manufacture or market OJEMDA or our product candidates. Defense of these claims, regardless of their merit, would involve substantial litigation expense and would be a substantial diversion of employee resources from our business. We may also in the future pursue invalidity proceedings with respect to third-party patents. The outcome following legal assertions of invalidity is unpredictable. Additionally, we may be subject to claims of patent infringement during those proceedings, and delays caused by the federal agencies may increase the time period that we are subject to such claims. For example, administrative changes, including reduced personnel and budgets experienced by the Patent and Trial Appeal Board, could further delay our ability to timely challenge any such patents. We cannot predict whether we would prevail in any such actions or that any license required under any of these patents would be made available on commercially acceptable terms, if at all. Moreover, even if we or our current and/or future strategic partners were able to obtain a license, the rights may be nonexclusive, which could result in our competitors gaining access to the same intellectual property. In addition, we cannot be certain that we could redesign OJEMDA, our product candidates, treatment indications, or processes to avoid infringement, if necessary. Accordingly, an adverse determination in a judicial or administrative proceeding, or the failure to obtain necessary licenses, could prevent us from developing and commercializing our product candidates, which could harm our business, financial condition and operating results. In addition, intellectual property litigation, regardless of its outcome, may cause negative publicity and could prohibit us from marketing or otherwise commercializing OJEMDA or our product candidates and technology.

Parties making claims against us may be able to sustain the costs of complex patent litigation more effectively than we can because they have substantially greater resources. Furthermore, because of the substantial amount of discovery required in connection with intellectual property litigation or administrative proceedings, there is a risk that some of our confidential information could be compromised by disclosure. In addition, any uncertainties resulting from the initiation and continuation of any litigation could have a material adverse effect on our ability to raise additional funds or otherwise have a material adverse effect on our business, results of operations, financial condition and prospects.

Some of our current product candidates and research programs are licensed from third parties. If these license agreements are terminated or interpreted to narrow our rights, our ability to advance OJEMDA and our current product candidates or develop new product candidates based on these technologies will be materially adversely affected.

We now depend on, at least in part, Viracta Therapeutics, Inc., Takeda Pharmaceutical Company Limited, Dana Farber Cancer Institute, Millennium Pharmaceuticals, Inc., Merck KGaA, Darmstadt, Germany, MabCare, and Ipsen and will continue to depend on Viracta Therapeutics, Inc., Takeda Pharmaceutical Company Limited, Dana Farber Cancer Institute, Millennium Pharmaceuticals, Inc. and Merck KGaA, Darmstadt, Germany, MabCare, and Ipsen and on licenses and sublicenses from other third parties, as well as potentially on other strategic relationships with third parties, for the research, development, manufacturing and commercialization of OJEMDA and our current product candidates. If any of our licenses or relationships or any in-licenses on which our licenses are based are terminated or breached, we may:

- lose our rights to develop and market OJEMDA or our current product candidates;
- lose patent or trade secret protection for OJEMDA or our current product candidates;
- experience significant delays in the development or commercialization of OJEMDA or our current product candidates;
- not be able to obtain any other licenses on acceptable terms, if at all; or
- incur liability for damages.

Additionally, even if not terminated or breached, our intellectual property licenses or sublicenses may be subject to disagreements over contract interpretation which could narrow the scope of our rights to the relevant intellectual property or technology or increase our financial or other obligations.

If we experience any of the foregoing, it could have a materially adverse effect on our business and could force us to cease operations which could cause you to lose all of your investment.

If we breach our license agreements, it could have a material adverse effect on our commercialization efforts for OJEMDA and our product candidates.

If we breach any of the agreements under which we license the use, development and commercialization rights to OJEMDA and our product candidates or technology from third parties, we could lose license rights that are important to our business. Or if we fail to comply with our obligations in the agreements under which we license intellectual property rights from third parties or otherwise experience disruptions to our business relationships with our licensors, we could lose license rights that are important to our business.

OJEMDA and our current lead product candidates are protected by, among other intellectual property rights, patents and patent applications we co-own and exclusively in-license from Viracta Therapeutics, Inc. (f/k/a Sunesis Pharmaceuticals, Inc.). OJEMDA and our current lead product candidates and pipeline and our anticipated near-term pipeline may include technologies licensed from other third parties, including, for example, Merck KGaA, Darmstadt, Germany. Further, pursuant to the MabCare License Agreement, we have the exclusive right to develop, manufacture and commercialize DAY301 worldwide, excluding Greater China.

Under the license agreements, we are subject to various obligations, including diligence obligations such as development and commercialization obligations, as well as potential royalty payments and other obligations. If we fail to comply with any of these obligations or otherwise breach our license agreements, our licensors may have the right to terminate the applicable license in whole or in part. Generally, the loss of any one of our current licenses, or any other license we may acquire in the future, could harm our business, prospects, financial condition and results of operations.

Licensing of intellectual property is of critical importance to our business and involves complex legal, business and scientific issues. Disputes may arise between us and our licensors regarding intellectual property subject to a license agreement, including:

- the scope of rights granted under the license agreement and other interpretation-related issues;
- whether and the extent to which our technology and processes infringe on intellectual property of the licensor that is not subject to the licensing agreement;
- our right to sublicense patent and other intellectual property rights to third parties under collaborative development relationships;
- our diligence obligations with respect to the use of the licensed technology in relation to our development and commercialization of OJEMDA and our product candidates, and what activities satisfy those diligence obligations;
- the priority of invention of patented technology;
- the ownership of inventions and know-how resulting from the joint creation or use of intellectual property by our licensors and us and our partners; and
- whether and the extent to which inventors are able to contest the assignment of their rights to our licensors.

If disputes over intellectual property that we have licensed prevent or impair our ability to maintain our current licensing arrangements on acceptable terms or at all, we may be unable to successfully develop and successfully commercialize OJEMDA and the affected product candidates. In addition, if disputes arise as to ownership of licensed intellectual property, our ability to pursue or enforce the licensed patent rights may be jeopardized. If we or our licensors fail to adequately protect this intellectual property, our ability to commercialize our products could suffer.

In addition, the agreements under which we license intellectual property or technology from third parties, including our licenses with Viracta Therapeutics, Inc., Takeda Pharmaceutical Company Limited, Dana Farber Cancer Institute, Millennium Pharmaceuticals, Inc., Merck KGaA, Darmstadt, Germany, MabCare, and Ipsen are complex, and certain provisions in such agreements may be susceptible to multiple interpretations. The resolution of any contract interpretation disagreement that may arise could narrow what we believe to be the scope of our rights to the relevant intellectual property or technology, or increase what we believe to be our financial or other obligations under the relevant agreement, either of which could have a material adverse effect on our business, financial condition, results of operations, and prospects. Moreover, if disputes over intellectual property that we license prevent or impair our ability to maintain our licensing arrangements on commercially acceptable terms, we may be unable to successfully develop and commercialize the affected product candidates, which could have a material adverse effect on our business, financial conditions, results of operations and prospects.

In spite of our best efforts, our licensors might conclude that we materially breached our license agreements and might therefore terminate the license agreements, thereby removing our ability to develop and commercialize products and technology covered by these license agreements. If these in-licenses are terminated, or if the underlying patents fail to provide the intended exclusivity, competitors would have the freedom to seek marketing authorization of, and to market, products identical to ours. This could have a material adverse effect on our competitive position, business, financial conditions, results of operations and prospects.

While we cannot currently determine the amount of the royalty obligations we would be required to pay on sales of future products, if any, the amounts may be significant. The amount of our future royalty obligations will depend on the technology and intellectual property we use in products that we successfully develop and commercialize, if any. Therefore, even if we successfully develop and commercialize products, we may be unable to achieve or maintain profitability.

In the future, we may need to obtain additional licenses of third-party technology that may not be available to us or are available only on commercially unreasonable terms, and which may cause us to operate our business in a more costly or otherwise adverse manner that was not anticipated.

We seek to expand our product candidate pipeline in part by in-licensing the rights to key technologies. The future growth of our business will depend in part on our ability to in-license or otherwise acquire the rights to additional product candidates or technologies. We cannot assure you that we will be able to in-license or acquire the rights to any product candidates or technologies from third parties on acceptable terms or at all.

Other companies and academic institutions may also have filed or are planning to file patent applications potentially relevant to our business. From time to time, in order to avoid infringing these third-party patents, we may be required to license technology from third parties to further develop or commercialize our existing or future product candidates. Should we be required to obtain licenses to any third-party technology, including any such patents required to manufacture, use or sell our existing or future product candidates, such licenses may not be available to us on commercially reasonable terms, or at all. The inability to obtain any third-party license required to develop or commercialize any of our existing or future product candidates could cause us to abandon any related efforts, which could seriously harm our business and operations.

The in-licensing and acquisition of these technologies is a competitive area, and a number of more established companies are also pursuing strategies to license or acquire product candidates or technologies that we may consider attractive. These established companies may have a competitive advantage over us due to their size, cash resources and greater clinical development and commercialization capabilities. In addition, companies that perceive us to be a competitor may be unwilling to license rights to us. Furthermore, we may be unable to identify suitable product candidates or technologies within our area of focus. If we are unable to successfully obtain rights to suitable product candidates or technologies, our business, financial condition and prospects could suffer.

We may be involved in lawsuits to protect or enforce our own patents or our licensors' patents, which could be expensive, time consuming and unsuccessful. Further, our own issued patents or our licensors' patents could be found invalid or unenforceable if challenged in court.

Competitors may infringe our intellectual property rights. To prevent infringement or unauthorized use, we may be required to file infringement claims, which can be expensive and time-consuming. In addition, in a patent infringement proceeding, a court may decide that a patent we own or in-license is not valid, is unenforceable and/or is not infringed. If we or any of our collaborators were to initiate legal proceedings against a third-party to enforce a patent directed at OJEMDA or one of our product candidates, the defendant could counterclaim that our patent or the patent of our licensors is invalid and/or unenforceable in whole or in part. In patent litigation in the United States, defendant counterclaims alleging invalidity and/or unenforceability are commonplace. Grounds for a

validity challenge include an alleged failure to meet any of several statutory requirements, including lack of novelty, obviousness, lack of sufficient written description, non-enablement or obviousness-type double patenting. Grounds for an unenforceability assertion could include an allegation that someone connected with prosecution of the patent withheld relevant information from the USPTO or made a misleading statement during prosecution.

Third parties may also raise similar invalidity claims before the USPTO or patent offices abroad, even outside the context of litigation. Such mechanisms include re-examination, *inter partes* review proceedings, post grant review proceedings, derivation proceedings and equivalent proceedings in foreign jurisdictions (e.g., opposition proceedings). The outcome following legal assertions of invalidity and/or unenforceability is unpredictable. With respect to the validity question, for example, we cannot be certain that there is no invalidating prior art, of which we, our licensors and the patent examiners are unaware during prosecution. There is also no assurance that there is not prior art of which we are aware, but which we do not believe affects the validity or enforceability of a claim in our patents and patent applications or the patents and patent applications of our licensors, which may, nonetheless, ultimately be found to affect the validity or enforceability of a claim. If a third party were to prevail on a legal assertion of invalidity or unenforceability, we would lose at least part, and perhaps all, of the patent protection on our technology, or any product candidates that we may develop. Such a loss of patent protection would have a material adverse impact on our business, financial condition, results of operations and prospects.

In addition, if the breadth or strength of protection provided by our patents and patent applications or the patents and patent applications of our licensors is threatened, it could dissuade companies from collaborating with us to license, develop or commercialize current or future product candidates.

Even if resolved in our favor, litigation or other legal proceedings relating to our intellectual property rights may cause us to incur significant expenses, and could distract our technical and management personnel from their normal responsibilities. Such litigation or proceedings could substantially increase our operating costs and reduce the resources available for development activities or any future sales, marketing or distribution activities. We may not have sufficient financial or other resources to conduct such litigation or proceedings adequately. Some of our competitors may be able to sustain the costs of such litigation or proceedings more effectively than we can because of their greater financial resources. Uncertainties resulting from the initiation and continuation of patent litigation or other proceedings could compromise our ability to compete in the marketplace.

Furthermore, because of the substantial amount of discovery required in connection with intellectual property litigation or other legal proceedings relating to our intellectual property rights, there is a risk that some of our confidential information could be compromised by disclosure during this type of litigation or other proceedings.

Intellectual property litigation may lead to unfavorable publicity that harms our reputation and causes the market price of our common shares to decline.

During the course of any intellectual property litigation, there could be public announcements of the initiation of the litigation as well as results of hearings, rulings on motions and other interim proceedings or developments in the litigation. If securities analysts or investors regard these announcements as negative, the perceived value of our existing product candidates, approved products, programs or intellectual property could be diminished. Accordingly, the market price of shares of our common stock may decline. Such announcements could also harm our reputation or the market for our product candidates, which could have a material adverse effect on our business.

Derivation proceedings may be necessary to determine priority of inventions, and an unfavorable outcome may require us to cease using the related technology or to attempt to license rights from the prevailing party.

Derivation proceedings provoked by third parties or brought by us or declared by the USPTO may be necessary to determine the priority of inventions with respect to our patents or patent applications or those of our licensors. An unfavorable outcome could require us to cease using the related technology or to attempt to license rights to it from the prevailing party. Our business could be harmed if the prevailing party does not offer us a license on commercially reasonable terms. Our defense of derivation proceedings may fail and, even if successful, may result in substantial costs and distract our management and other employees from their regular responsibilities. In addition, the uncertainties associated with such proceedings could have a material adverse effect on our ability to raise the funds necessary to continue our clinical trials, continue our development programs, license necessary technology from third parties or enter into development or manufacturing partnerships that would help us bring our product candidates to market.

Because of the expense and uncertainty of litigation, we may not be in a position to enforce our intellectual property rights against third parties.

Because of the expense and uncertainty of litigation, we may conclude that even if a third party is infringing our issued patent, any patents that may be issued as a result of our pending or future patent applications or other intellectual property rights, the risk-adjusted cost of bringing and enforcing such a claim or action may be too high or not in the best interest of our company or our stockholders, or

it may be otherwise impractical or undesirable to enforce our intellectual property against some third parties. Our competitors or other third parties may be able to sustain the costs of complex patent litigation or proceedings more effectively than we can because of their greater financial resources and more mature and developed intellectual property portfolios. In such cases, we may decide that the more prudent course of action is to simply monitor the situation or initiate or seek some other non-litigious action or solution. In addition, the uncertainties associated with litigation could compromise our ability to raise the funds necessary to continue our product development, in-license needed technology or enter into development partnerships that would help us bring OJEMDA and our product candidates to market.

Recent patent reform legislation could increase the uncertainties and costs surrounding the prosecution of our patent applications and/or those of our licensors and the enforcement or defense of our issued patents and/or those of our licensors.

On September 16, 2011, the Leahy-Smith America Invents Act, or the Leahy-Smith Act, was signed into law. The Leahy-Smith Act includes a number of significant changes to U.S. patent law. These include provisions that affect the way patent applications will be prosecuted and may also affect patent litigation. In particular, under the Leahy-Smith Act, the United States transitioned in March 2013 to a “first inventor to file” system in which, assuming that other requirements of patentability are met, the first inventor to file a patent application will be entitled to the patent regardless of whether a third-party was first to invent the claimed invention. A third-party that files a patent application in the USPTO after March 2013 but before us could therefore be awarded a patent covering an invention of ours even if we had made the invention before it was made by such third-party. This will require us to be cognizant going forward of the time from invention to filing of a patent application. Furthermore, our ability to obtain and maintain valid and enforceable patents depends on whether the differences between our technology and the prior art allow our technology to be patentable over the prior art. Since patent applications in the United States and most other countries are confidential for a period of time after filing or until issuance, we may not be certain that we or our licensors are the first to either (1) file any patent application related to our product candidates or (2) invent any of the inventions claimed in the patents or patent applications.

The Leahy-Smith Act also includes a number of significant changes that affect the way patent applications will be prosecuted and also may affect patent litigation. These include allowing third-party submission of prior art to the USPTO during patent prosecution and additional procedures to attack the validity of a patent by USPTO administered post-grant proceedings, including post-grant review, *inter partes* review and derivation proceedings. An adverse determination in any such submission or proceeding could reduce the scope or enforceability of, or invalidate, our patent rights, which could adversely affect our competitive position.

Because of a lower evidentiary standard in USPTO proceedings compared to the evidentiary standard in United States federal courts necessary to invalidate a patent claim, a third party could potentially provide evidence in a USPTO proceeding sufficient for the USPTO to hold a claim invalid even though the same evidence would be insufficient to invalidate the claim if first presented in a district court action. Accordingly, a third party may attempt to use the USPTO procedures to invalidate our patent claims that would not have been invalidated if first challenged by the third party as a defendant in a district court action. Thus, the Leahy-Smith Act and its implementation could increase the uncertainties and costs surrounding the prosecution of our patent applications and/or those of our licensors and the enforcement or defense of our issued patents or those of our licensors, all of which could have a material adverse effect on our business, financial condition, results of operations and prospects.

Changes in U.S. patent law, or laws in other countries, could diminish the value of patents in general, thereby impairing our ability to protect OJEMDA and our product candidates.

As is the case with other pharmaceutical companies, our success is heavily dependent on intellectual property, particularly patents. Obtaining and enforcing patents in the biopharmaceutical industry involves a high degree of technological and legal complexity. Therefore, obtaining and enforcing biopharmaceutical patents is costly, time consuming and inherently uncertain. Changes in either the patent laws or in the interpretations of patent laws in the United States and other countries may diminish the value of our intellectual property and may increase the uncertainties and costs surrounding the prosecution of patent applications and the enforcement or defense of issued patents. We cannot predict the breadth of claims that may be allowed or enforced with respect to our patents or third-party patents. In addition, the U.S. Congress or other foreign legislative bodies may pass patent reform legislation that is unfavorable to us.

For example, the U.S. Supreme Court has ruled on several patent cases in recent years, either narrowing the scope of patent protection available in certain circumstances or weakening the rights of patent owners in certain situations. In addition to increasing uncertainty with regard to our ability to obtain patents in the future, this combination of events has created uncertainty with respect to the value of patents, once obtained. Depending on decisions by the U.S. Congress, the U.S. federal courts, the USPTO or similar authorities in foreign jurisdictions, the laws and regulations governing patents could change in unpredictable ways that would weaken our ability to obtain new patents or to enforce our existing patent and the patents we might obtain or license in the future.

Additionally, starting from June 1, 2023, European applications have the option, upon grant of a patent, of becoming a Unitary Patent which will be subject to the jurisdiction of the Unitary Patent Court, or the UPC. This will be a significant change in European patent practice. As the UPC is a new court system, there is limited precedent for the court, increasing the uncertainty of any litigation.

Patents granted before the implementation of the UPC will have the option of opting out of the jurisdiction of the UPC and remaining as national patents in the UPC countries. Patents that remain under the jurisdiction of the UPC may be potentially vulnerable to a single UPC-based revocation challenge that, if successful, could invalidate the patent in all countries who are signatories to the UPC. We cannot predict with certainty the long-term effects of the new unitary patent system.

We may be subject to claims challenging the inventorship or ownership of our patents and other intellectual property.

We and/or our licensors may be subject to claims that former employees, collaborators or other third parties have an interest in our patents or other intellectual property as an inventor or co-inventor. In addition, we cannot assure you that all inventors have been or will be identified by us and/or by our collaborators despite diligent effort. The failure to name the proper inventors on a patent application can result in the patents issuing thereon being unenforceable. Inventorship disputes may arise from conflicting views regarding the contributions of different individuals named as inventors, the effects of foreign laws where foreign nationals are involved in the development of the subject matter of the patent, conflicting obligations of third parties involved in developing our product candidates or as a result of questions regarding co-ownership of potential joint inventions. Litigation may be necessary to resolve these and other claims challenging inventorship and/or ownership. Alternatively, or additionally, we may enter into agreements to clarify the scope of our rights in such intellectual property. If we fail in defending any such claims, in addition to paying monetary damages, we may lose valuable intellectual property rights, such as exclusive ownership of, or right to use, valuable intellectual property. Such an outcome could have a material adverse effect on our business. Even if we are successful in defending against such claims, litigation could result in substantial costs and be a distraction to management and other employees.

Our licensors may have relied on third-party consultants or collaborators such that our licensors are not the sole and exclusive owners of the patents we in-licensed. If other third parties have ownership rights or other rights to our in-licensed patents, they may be able to license such patents to our competitors, and our competitors could market competing products. This could have a material adverse effect on our competitive position, business, financial conditions, results of operations, and prospects.

In addition, while it is our policy to require our employees and contractors who may be involved in the conception or development of intellectual property to execute agreements assigning such intellectual property to us, we may be unsuccessful in executing such an agreement with each party who, in fact, conceives or develops intellectual property that we regard as our own. The assignment of intellectual property rights may not be self-executing, or the assignment agreements may be breached, and we may be forced to bring claims against third parties, or defend claims that they may bring against us, to determine the ownership of what we regard as our intellectual property. Such claims could have a material adverse effect on our business, financial condition, results of operations and prospects.

Patent terms may be inadequate to protect our competitive position on our product candidates for an adequate amount of time.

Patents have a limited lifespan. In the United States, if all maintenance fees are timely paid, the natural expiration of a patent is generally 20 years from its earliest U.S. non-provisional filing date. Various extensions may be available, but the life of a patent, and the protection it affords, is limited. Even if patents covering our product candidates are obtained, once the patent life has expired, we may be open to competition from competitive products. Given the amount of time required for the development, testing and regulatory review of new product candidates, patents protecting such candidates might expire before or shortly after such candidates are commercialized. As a result, our patent portfolio may not provide us with sufficient rights to exclude others from commercializing products similar or identical to ours.

If we do not obtain patent term extension for OJEMDA and product candidates, our business may be materially harmed.

Depending upon the timing, duration and specifics of FDA marketing authorization of our product and product candidates, one or more of our U.S. patents or those of our licensors may be eligible for limited patent term restoration under the Drug Price Competition and Patent Term Restoration Act of 1984, or the Hatch-Waxman Amendments. The Hatch-Waxman Amendments permit a patent restoration term of up to five years as compensation for patent term lost during product development and the FDA regulatory review process. A maximum of one patent may be extended per FDA approved product as compensation for the patent term lost during the FDA regulatory review process. A patent term extension cannot extend the remaining term of a patent beyond a total of 14 years from the date of product approval and only those claims covering such approved drug product, a method for using it or a method for manufacturing it may be extended. Patent term extension may also be available in certain foreign countries upon marketing authorization of our product candidates. However, we may not be granted an extension because of, for example, failing to apply within applicable deadlines, failing to apply prior to expiration of relevant patents or otherwise failing to satisfy applicable requirements. Additionally, administrative changes at the USPTO or other applicable patent authorities, such as reduced hiring and/or funding, may result in delays in issuance of a patent or in accrual of patent term extension, thereby reducing the amount of patent term extension that could otherwise be received. Administrative changes (e.g., at the FDA or USPTO) may also lead to delays in review and analysis of regulatory submissions or requests for patent term extension, which could result in a patent term extension not being timely granted (e.g., before the expiration of the patent) and there may be no patent eligible for extension. Moreover, the applicable time period or the

scope of patent protection afforded could be less than we request. If we are unable to obtain patent term extension or restoration or the term of any such extension is less than we request, our competitors may obtain approval of competing products following our patent expiration, and our revenue could be reduced, possibly materially. Further, if this occurs, our competitors may take advantage of our investment in development and trials by referencing our clinical and preclinical data and may launch their product earlier than might otherwise be the case.

We may not be able to protect our intellectual property rights throughout the world.

Although we have pending patent applications in the United States and other countries, filing, prosecuting and defending patents in all countries throughout the world would be prohibitively expensive, and our intellectual property rights in some countries outside the United States can be less extensive than those in the United States. In addition, the laws of some foreign countries do not protect intellectual property rights to the same extent as federal and state laws in the United States. Consequently, we may not be able to prevent third parties from practicing our inventions in all countries outside the United States or from selling or importing products made using our inventions in and into the United States or other jurisdictions. Competitors may use our technologies in jurisdictions where we have not obtained patent protection to develop their own products and, further, may export otherwise infringing products to territories where we have patent protection, but enforcement is not as strong as that in the United States. These products may compete with our product candidates, and our patents, the patents of our licensors or other intellectual property rights may not be effective or sufficient to prevent them from competing.

Many companies have encountered significant problems in protecting and defending intellectual property rights in foreign jurisdictions. The legal systems of many foreign countries do not favor the enforcement of patents and other intellectual property protection, which could make it difficult for us to stop the infringement of our patents or our licensors' patents or marketing of competing products in violation of our proprietary rights. Proceedings to enforce our patent rights in foreign jurisdictions could result in substantial costs and divert our efforts and attention from other aspects of our business, could put our patents or the patents of our licensors at risk of being invalidated or interpreted narrowly and our patent applications or the patent applications of our licensors at risk of not issuing and could provoke third parties to assert claims against us. We may not prevail in any lawsuits that we initiate, and the damages or other remedies awarded, if any, may not be commercially meaningful. Accordingly, our efforts to enforce our intellectual property rights around the world may be inadequate to obtain a significant commercial advantage from the intellectual property that we develop or license.

Many countries have compulsory licensing laws under which a patent owner may be compelled to grant licenses to third parties. In addition, many countries limit the enforceability of patents against government agencies or government contractors. In these countries, the patent owner may have limited remedies, which could materially diminish the value of such patent. If we are forced to grant a license to third parties with respect to any patents relevant to our business, our competitive position may be impaired, and our business, financial condition, results of operations and prospects may be adversely affected.

Further, trade barriers and unexpected changes in tariffs, including tariffs that have been or may in the future be imposed by the United States or other countries, could impact our intellectual property rights in unpredictable ways. For example, trade barriers and changes in tariffs may require us to change our supply chain and move our current or future production to countries with more favorable trade agreements but less favorable or no intellectual property rights protection. The United States and other countries may also take protectionist or retaliatory measures in response to the changes in tariffs, leading to a plethora of unpredictable consequences that could weaken our intellectual property rights throughout the world.

Obtaining and maintaining our patent protection depends on compliance with various procedural, documentary, fee payment and other requirements imposed by regulations and governmental patent agencies, and our patent protection could be reduced or eliminated for non-compliance with these requirements.

Periodic maintenance fees, renewal fees, annuity fees and various other governmental fees on patents and/or applications will be due to the USPTO and various foreign patent offices at various points over the lifetime of our patents and/or applications and those of our licensors. We have systems in place to remind us to pay these fees, and we rely on our outside patent annuity service to pay these fees when due. Additionally, the USPTO and various foreign patent offices require compliance with a number of procedural, documentary, fee payment and other similar provisions during the patent application process. We employ reputable law firms and other professionals to help us comply, and in many cases, an inadvertent lapse can be cured by payment of a late fee or by other means in accordance with rules applicable to the particular jurisdiction. However, there are situations in which noncompliance can result in abandonment or lapse of the patent or patent application, resulting in partial or complete loss of patent rights in the relevant jurisdiction. If such an event were to occur, it could have a material adverse effect on our business.

If our trademarks and trade names are not adequately protected, then we may not be able to build name recognition in our markets of interest and our business may be adversely affected.

We intend to use registered or unregistered trademarks or trade names to brand and market ourselves and our products. Our trademarks or trade names may be challenged, infringed, circumvented or declared generic or determined to be infringing on other marks. We may not be able to protect our rights to these trademarks and trade names, which we need to build name recognition among potential partners or customers in our markets of interest. At times, competitors may adopt trade names or trademarks similar to ours, thereby impeding our ability to build brand identity and possibly leading to market confusion. In addition, there could be potential trade name or trademark infringement claims brought by owners of other registered trademarks or trademarks that incorporate variations of our registered or unregistered trademarks or trade names. Over the long term, if we are unable to establish name recognition based on our trademarks and trade names, then we may not be able to compete effectively, and our business may be adversely affected. Our efforts to enforce or protect our proprietary rights related to trademarks, trade secrets, domain names, copyrights or other intellectual property may be ineffective and could result in substantial costs and diversion of resources and could adversely affect our financial condition or results of operations.

If we are unable to protect the confidentiality of our trade secrets, our business and competitive position would be harmed.

We rely in part on the protection of our trade secrets, including unpatented know-how, technology and other proprietary information, to maintain our competitive position. Although we have taken steps to protect our trade secrets and unpatented know-how, including entering into confidentiality agreements with third parties, and confidential information and inventions agreements with employees, consultants and advisors, we cannot provide any assurances that all such agreements have been duly executed, and any of these parties may breach the agreements and disclose our proprietary information, including our trade secrets, and we may not be able to obtain adequate remedies for such breaches. Enforcing a claim that a party illegally disclosed or misappropriated a trade secret is difficult, expensive and time-consuming, and the outcome is unpredictable. In addition, some courts inside and outside the United States are less willing or unwilling to protect trade secrets.

Moreover, third parties may still obtain this information or may come upon this or similar information independently, and we would have no right to prevent them from using that technology or information to compete with us. If any of these events occurs or if we otherwise lose protection for our trade secrets, the value of this information may be greatly reduced, and our competitive position would be harmed. If we do not apply for patent protection prior to such publication or if we cannot otherwise maintain the confidentiality of our proprietary technology and other confidential information, then our ability to obtain patent protection or to protect our trade secret information may be jeopardized.

We may be subject to claims that we or our employees have wrongfully used or disclosed alleged confidential information or trade secrets.

We have entered into or may enter in the future into non-disclosure and confidentiality agreements to protect the proprietary positions of third parties, such as outside scientific collaborators, CROs, third-party manufacturers, consultants, advisors, potential partners and other third parties. We may become subject to litigation where a third party asserts that we or our employees inadvertently or otherwise breached the agreements and used or disclosed trade secrets or other information proprietary to the third parties. Defense of such matters, regardless of their merit, could involve substantial litigation expense and be a substantial diversion of employee resources from our business. We cannot predict whether we would prevail in any such actions. Moreover, intellectual property litigation, regardless of its outcome, may cause negative publicity and could prohibit us from marketing or otherwise successfully commercializing OJEMDA and our product candidates and technology. Failure to defend against any such claim could subject us to significant liability for monetary damages or prevent or delay our developmental and commercialization efforts, which could adversely affect our business. Even if we are successful in defending against these claims, litigation could result in substantial costs and be a distraction to our management team and other employees.

Parties making claims against us may be able to sustain the costs of complex intellectual property litigation more effectively than we can because they have substantially greater resources. Furthermore, because of the substantial amount of discovery required in connection with intellectual property litigation, there is a risk that some of our confidential information could be compromised by disclosure. In addition, any uncertainties resulting from the initiation and continuation of any litigation could have a material adverse effect on our ability to raise additional funds or otherwise have a material adverse effect on our business, operating results, financial condition and prospects.

We may be subject to claims that we have wrongfully hired an employee from a competitor or that we or our employees have wrongfully used or disclosed alleged confidential information or trade secrets of their former employers.

As is common in the biopharmaceutical industry, in addition to our employees, we engage the services of consultants to assist us in the development of OJEMDA and our product candidates. Many of these consultants, and many of our employees, were previously employed at, or may have previously provided or may be currently providing consulting services to, other pharmaceutical companies including our competitors or potential competitors. We may become subject to claims that we, our employees or a consultant inadvertently or otherwise used or disclosed trade secrets or other information proprietary to their former employers or their former or

current clients. Litigation may be necessary to defend against these claims. If we fail in defending any such claims, in addition to paying monetary damages, we may lose valuable intellectual property rights or personnel, which could adversely affect our business. Even if we are successful in defending against these claims, litigation could result in substantial costs and be a distraction to our management team and other employees.

The patent protection and patent prosecution for OJEMDA and some of our product candidates may be dependent on third parties.

While we normally seek to obtain the right to control prosecution, maintenance and enforcement of the patents relating to OJEMDA and our product candidates, there may be times when the filing and prosecution activities for patents and patent applications relating to OJEMDA and our product candidates are controlled by our licensors or collaboration partners. If any of our licensors or collaboration partners fail to prosecute, maintain and enforce such patents and patent applications in a manner consistent with the best interests of our business, including by payment of all applicable fees for patents covering OJEMDA and our product candidates, we could lose our rights to the intellectual property or our exclusivity with respect to those rights, our ability to develop and commercialize OJEMDA and those product candidates may be adversely affected and we may not be able to prevent competitors from making, using and selling competing products. In addition, even where we have the right to control patent prosecution of patents and patent applications we have licensed from third parties, we may still be adversely affected or prejudiced by actions or inactions of our licensors and their counsel that took place prior to the date upon which we assumed control over patent prosecution.

Currently, our intellectual property protection includes patents and patent applications that we have in-licensed from, among others, Viracta Therapeutics, Inc., Takeda Pharmaceutical Company Limited, Merck KGaA, Darmstadt, Germany, MabCare, and Ipsen. Our exclusive and non-exclusive licenses may be subject to certain retained rights, which may adversely impact our competitive position. We do not control the prosecution and maintenance of several of the licensed patent portfolios; thus, we cannot assure you that the licensed patent families will be prepared, filed, prosecuted, or maintained in a manner consistent with the best interests of our business. See “Management’s Discussion and Analysis of Financial Condition and Results of Operations— Significant Agreements.” Our licensed patent portfolio may not provide us with adequate and continuing patent protection sufficient to exclude others from commercializing products similar to OJEMDA and our product candidates.

Intellectual property discovered through government funded programs may be subject to federal regulations such as “march-in” rights, certain reporting requirements and a preference for U.S.-based companies. Compliance with such regulations may limit our exclusive rights and limit our ability to contract with non-U.S. manufacturers.

Some of our own issued patents or pending patent applications may have been generated through the use of U.S. government funding, and we may acquire or license in the future intellectual property rights that have been generated through the use of U.S. government funding or grants. Pursuant to the Bayh-Dole Act of 1980, the U.S. government has certain rights in inventions developed with government funding. These U.S. government rights include a non-exclusive, non-transferable, irrevocable worldwide license to use inventions for any governmental purpose. In addition, the U.S. government has the right, under certain limited circumstances, to require us to grant exclusive, partially exclusive or non-exclusive licenses to any of these inventions to a third party if it determines that: (1) adequate steps have not been taken to commercialize the invention; (2) government action is necessary to meet public health or safety needs; or (3) government action is necessary to meet requirements for public use under federal regulations (also referred to as “march-in rights”). If the U.S. government exercised its march-in rights in our existing or future intellectual property rights that are generated through the use of U.S. government funding or grants, we could be forced to license or sublicense intellectual property developed by us or that we license on terms unfavorable to us, and there can be no assurance that we would receive compensation from the U.S. government for the exercise of such rights. The U.S. government also has the right to take title to these inventions if the grant recipient fails to disclose the invention to the government or fails to file an application to register the intellectual property within specified time limits. Intellectual property generated under a government funded program is also subject to certain reporting requirements, compliance with which may require us to expend substantial resources. In addition, the U.S. government requires that any products embodying any of these inventions or produced through the use of any of these inventions be manufactured substantially in the United States. This preference for U.S. industry may be waived by the federal agency that provided the funding if the owner or assignee of the intellectual property can show that reasonable but unsuccessful efforts have been made to grant licenses on similar terms to potential licensees that would be likely to manufacture substantially in the United States or that under the circumstances domestic manufacture is not commercially feasible. This preference for U.S. industry may limit our ability to contract with non-U.S. product manufacturers for products covered by such intellectual property.

Geo-political actions in the United States and in foreign countries could increase the uncertainties and costs surrounding the prosecution or maintenance of our patent applications or those of any current or future licensors and the maintenance, enforcement or defense of our issued patents or those of any current or future licensors.

Certain geo-political actions in the United States or other countries may increase the uncertainties and costs related to the prosecution or maintenance of our patent applications, or those of our current or future licensors. For example, the United States and foreign government actions related to Russia’s invasion of Ukraine may limit or prevent filing, prosecution and maintenance of patent

applications in Russia. Government actions may also prevent maintenance of issued patents in Russia. These actions could result in abandonment or lapse of our patents or patent applications, resulting in partial or complete loss of patent rights in Russia. If such an event were to occur, it could have a material adverse effect on our business. In addition, a decree was adopted by the Russian government in March 2022, allowing Russian companies and individuals to exploit inventions owned by patentees that have citizenship or nationality in, are registered in, or have predominately primary place of business or profit-making activities in the United States and other countries that Russia has deemed unfriendly without consent or compensation. Consequently, we would not be able to prevent third parties from practicing our inventions in Russia or from selling or importing products made using our inventions in and into Russia.

The laws of some jurisdictions do not protect intellectual property rights to the same extent as the laws in the United States, and many companies have encountered significant difficulties in protecting and defending such rights in such jurisdictions. If we or our licensors or collaborators encounter difficulties in protecting, or are otherwise precluded from effectively protecting, the intellectual property rights important for our business in such jurisdictions, the value of these rights may be diminished and we may face additional competition from others in those jurisdictions. Many countries have compulsory licensing laws under which a patent owner may be compelled to grant licenses to third parties. In addition, many countries limit the enforceability of patents against government agencies or government contractors. In these countries, the patent owner may have limited remedies, which could materially diminish the value of such patent. If we or any of our licensors or collaborators are forced to grant a license to third parties with respect to any patents relevant to our business, our competitive position in the relevant jurisdiction may be impaired and our business, financial condition, results of operations and prospects may be adversely affected.

Risks Related to Our Common Stock

An active and liquid trading market for our common stock may never be sustained. As a result, you may not be able to resell your shares of common stock at or above the purchase price.

An active trading market for our common stock may never be sustained. The market value of our common stock may decrease from the purchase price. As a result of these and other factors, you may be unable to resell your shares of our common stock at or above the purchase price. The lack of an active market may impair your ability to sell your shares at the time you wish to sell them or at a price that you consider reasonable. The lack of an active market may also reduce the fair market value of your shares.

Furthermore, an inactive market may also impair our ability to raise capital by selling shares of our common stock and may impair our ability to enter into strategic collaborations or acquire companies or products by using our shares of common stock as consideration.

Our quarterly operating results may fluctuate significantly or may fall below the expectations of investors or securities analysts, each of which may cause our stock price to fluctuate or decline.

We expect our operating results to be subject to quarterly fluctuations. Our operating results will be affected by numerous factors, including:

- our ability to generate revenue from the sales of our product, OJEMDA;
- timing and variations in the level of expense related to the current or future development of our programs;
- timing and status of enrollment for our clinical trials;
- results of clinical trials, or the addition or termination of clinical trials or funding support by us or potential future partners;
- our execution of any collaboration, licensing or similar arrangements, and the timing of payments we may make or receive under potential future arrangements or the termination or modification of any such potential future arrangements;
- any intellectual property infringement, misappropriation or violation lawsuit or opposition, interference or cancellation proceeding in which we may become involved;
- additions and departures of key personnel;
- strategic decisions by us or our competitors, such as acquisitions, divestitures, spin-offs, joint ventures, strategic investments or changes in business strategy;
- if a product candidate we develop receives marketing authorization, the timing and terms of such approval and market acceptance and demand for such product;
- the timing and cost to establish a sales, marketing and distribution infrastructure to commercialize any products for which we may obtain marketing authorization and intend to commercialize on our own or jointly with future collaborators;
- regulatory developments affecting current or future product candidates or products, if any, or those of our competitors;

- the amount of expense or gain associated with the change in value of the success payments and contingent consideration;
- changes in general market and economic conditions, such as due to rising interest rates, inflation, changes in domestic and foreign currency rates, significant political or trade regulatory developments or changes in trade policy, global regional conflicts and public health epidemics;
- business development activities, such as additional program in-licensing, which could result in up-front payments or increased development expenses; and
- cybersecurity incidents.

If our quarterly operating results fall below the expectations of investors or securities analysts, the price of our common stock could decline substantially. Furthermore, any quarterly fluctuations in our operating results may, in turn, cause the price of our common stock to fluctuate substantially. We believe that quarterly comparisons of our financial results are not necessarily meaningful and should not be relied upon as an indication of our future performance.

The market price of our common stock is likely to be highly volatile, which could result in substantial losses for purchasers of our common stock.

The market price of our common stock is likely to continue to be highly volatile and subject to wide fluctuations in response to various factors, some of which we cannot control. As a result of this volatility, you may not be able to sell your shares of common stock at or above the price paid. The market price for our common stock may be influenced by many factors, including the other risks described in this “Risk Factors” section and the following:

- the success of our commercialization efforts for our product, OJEMDA;
- results of preclinical studies or clinical trials by us or those of our competitors or by existing or future collaborators or licensing partners;
- the timing and enrollment status of our clinical trials;
- changes in the development status of our product candidates, including variations in the level of expense related to the development of our programs or funding support by us or by existing or future collaborators or licensing partners;
- regulatory or legal developments in the United States and other countries, especially changes in laws or regulations applicable to our business;
- the success of competitive products or technologies;
- introductions and announcements of new product candidates by us, our future collaboration partners, or our competitors, and the timing of these introductions or announcements;
- actions taken by regulatory agencies with respect to our product candidates, clinical studies, manufacturing process or sales and marketing terms;
- our execution of any collaboration, licensing or similar arrangements, and the timing of payments we may make or receive under existing or future arrangements or the termination or modification of any such existing or future arrangements;
- actual or anticipated variations in our financial results or those of companies that are perceived to be similar to us;
- the success of our efforts to acquire or in-license additional technologies or product candidates;
- announced or completed significant acquisitions, strategic collaborations, joint ventures or capital commitments by us or our competitors;
- developments or disputes concerning our intellectual property and proprietary rights;
- the recruitment or departure of key personnel;
- changes in the structure of healthcare payment systems;
- actual or anticipated changes in earnings estimates or changes in stock market analyst recommendations regarding our common stock, other comparable companies or our industry generally;
- our failure or the failure of our competitors to meet analysts’ projections or guidance that we or our competitors may give to the market;
- speculation in the press or investment community;

- share price and fluctuations of trading volume of our common stock;
- the impact of interest rate increases on the overall stock market and the market for biopharmaceutical company stocks;
- fluctuations in the valuation of companies perceived by investors to be comparable to us;
- sales of shares of our common stock by us, insiders or our stockholders;
- our ability or inability to raise additional capital and the terms on which we raise it;
- the concentrated ownership of our common stock;
- changes in accounting principles;
- natural disasters and other calamities;
- general economic, industry and market conditions, including inflation, changes in interest rates, changes in domestic and foreign currency rates and significant political or regulatory developments or changes in trade policy, many of which are beyond our control;
- other events or factors, including those resulting from global pandemics, such as the COVID-19 pandemic, or war, incidents of terrorism or responses to these events, including global regional conflicts; and
- cybersecurity incidents.

In addition, the stock market in general, and the markets for pharmaceutical, biopharmaceutical and biotechnology stocks in particular, have experienced extreme price and volume fluctuations, including as a result of the COVID-19 pandemic, increase in inflation and changes in interest rates, as well as disruptions to the supply chain, that have been often unrelated or disproportionate to the operating performance of the issuer. Furthermore, the trading price of our common stock may be adversely affected by third parties trying to drive down the market price. Short sellers and others, some of whom post anonymously on social media, may be positioned to profit if our stock declines and their activities can negatively affect our stock price. These broad market and industry factors may seriously harm the market price of our common stock, regardless of our actual operating performance. The realization of any of the above risks or any of a broad range of other risks, including those described in this “Risk Factors” section, could have a dramatic and adverse impact on the market price of our common stock.

In the past, securities class action litigation has often been brought against public companies following declines in the market price of their securities. This risk is especially relevant for biopharmaceutical companies, which have experienced significant stock price volatility in recent years. If we face such litigation, it could result in substantial costs and a diversion of management’s attention and our resources, which could harm our business.

We do not currently intend to pay dividends on our common stock and, consequently, our stockholders’ ability to achieve a return on their investment will depend on appreciation of the value of our common stock.

We have never declared or paid cash dividends on our common stock. We currently intend to retain all available funds and any future earnings to support operations and to finance the growth and development of our business. We do not intend to declare or pay any cash dividends on our capital stock in the foreseeable future. As a result, any investment return on our common stock will depend upon increases in the value for our common stock, which is not certain.

A sale of a substantial number of shares of our common stock may cause the price of our common stock to decline.

Sales of a substantial number of shares of our common stock in the public market could occur at any time. If our stockholders sell, or the market perceives that our stockholders intend to sell, substantial amounts of our common stock in the public market, the market price of our common stock could decline significantly.

The holders of an aggregate of 8,502,776 shares of our outstanding common stock as of September 30, 2025 will have rights, subject to some conditions, to require us to file registration statements covering their shares or to include their shares in registration statements that we may file for ourselves or our stockholders. We also have registered shares of common stock that we may issue under our equity incentive plans. These shares are freely tradeable in the public market upon issuance.

We cannot predict what effect, if any, sales of our shares in the public market or the availability of shares for sale will have on the market price of our common stock. However, future sales of substantial amounts of our common stock in the public market, including shares issued upon exercise of our outstanding options or vesting of outstanding restricted stock unit awards, or the perception that such sales may occur, could adversely affect the market price of our common stock.

We also expect that significant additional capital may be needed in the future to continue our planned operations. To raise capital, we may sell common stock, convertible securities or other equity securities in one or more transactions at prices and in a manner we

determine from time to time. To the extent that additional capital is raised through the sale and issuance of shares or other securities convertible into shares, our stockholders will be diluted. These sales, or the perception in the market that the holders of a large number of shares intend to sell shares, could reduce the market price of our common stock.

Our principal stockholders and management own a significant percentage of our stock and will be able to exert significant control over matters subject to stockholder approval.

Based on the beneficial ownership of our common stock as of September 30, 2025, our executive officers, directors, holders of 5% or more of our capital stock and their respective affiliates beneficially owned 37.9% of our voting stock. The voting power of this group may increase to the extent they convert shares of non-voting common stock they hold into common stock. As a result, these stockholders, if acting together, will continue to have significant influence over the outcome of corporate actions requiring stockholder approval, including the election of directors, amendment of our organizational documents, any merger, consolidation or sale of all or substantially all of our assets and any other significant corporate transaction. The interests of these stockholders may not be the same as or may even conflict with your interests. For example, these stockholders could delay or prevent a change of control of our company, even if such a change of control would benefit our other stockholders, which could deprive our stockholders of an opportunity to receive a premium for their common stock as part of a sale of our company or our assets and might affect the prevailing market price of our common stock.

Anti-takeover provisions in our charter documents and under Delaware law could prevent or delay an acquisition of us, which may be beneficial to our stockholders, and may prevent attempts by our stockholders to replace or remove our current management.

Our restated certificate of incorporation and our amended and restated bylaws contain provisions that could delay or prevent a change in control of our company. These provisions could also make it difficult for stockholders to elect directors who are not nominated by current members of our board of directors or take other corporate actions, including effecting changes in our management. These provisions:

- establish a classified board of directors so that not all members of our board are elected at one time;
- permit only the board of directors to establish the number of directors and fill vacancies on the board;
- provide that directors may only be removed “for cause” and only with the approval of two-thirds of our stockholders;
- require super-majority voting to amend some provisions in our restated certificate of incorporation and restated bylaws;
- authorize the issuance of “blank check” preferred stock that our board could use to implement a stockholder rights plan;
- eliminate the ability of our stockholders to call special meetings of stockholders;
- prohibit stockholder action by written consent, which requires all stockholder actions to be taken at a meeting of our stockholders;
- prohibit cumulative voting; and
- establish advance notice requirements for nominations for election to our board or for proposing matters that can be acted upon by stockholders at annual stockholder meetings.

In addition, Section 203 of the Delaware General Corporation Law, or DGCL, may discourage, delay or prevent a change in control of our company. Section 203 imposes certain restrictions on mergers, business combinations and other transactions between us and holders of 15% or more of our common stock.

The exclusive forum provision in our organizational documents may limit a stockholder’s ability to bring a claim in a judicial forum that it finds favorable for disputes with us or any of our directors, officers or other employees, or the underwriters of any offering giving rise to such claim, which may discourage lawsuits with respect to such claims.

Our restated certificate of incorporation provides that, to the fullest extent permitted by law, the Court of Chancery of the State of Delaware is the exclusive forum for: any derivative action or proceeding brought on our behalf; any action asserting a breach of fiduciary duty; any action asserting a claim against us arising pursuant to the DGCL, our restated certificate of incorporation or our amended and restated bylaws; or any action asserting a claim against us that is governed by the internal affairs doctrine. This exclusive forum provision does not apply to suits brought to enforce a duty or liability created by the Securities Exchange Act of 1934, as amended, or the Exchange Act. It could apply, however, to a suit that falls within one or more of the categories enumerated in the exclusive forum provision.

This choice of forum provision may limit a stockholder’s ability to bring a claim in a judicial forum that it finds favorable for disputes with us or any of our directors, officers, or other employees, or the underwriters of any offering giving rise to such claims, which may

discourage lawsuits with respect to such claims. Alternatively, if a court were to find the choice of forum provisions contained in our restated certificate of incorporation to be inapplicable or unenforceable in an action, we may incur additional costs associated with resolving such action in other jurisdictions, which could harm our business, results of operations and financial condition.

Section 22 of the Securities Act of 1933, as amended, or the Securities Act, creates concurrent jurisdiction for federal and state courts over all claims brought to enforce any duty or liability created by the Securities Act or the rules and regulations thereunder. Our amended and restated bylaws provide that the federal district courts of the United States of America will, to the fullest extent permitted by law, be the exclusive forum for resolving any complaint asserting a cause of action arising under the Securities Act, or a Federal Forum Provision, including for all causes of action asserted against any defendant named in such complaint. For the avoidance of doubt, this provision is intended to benefit and may be enforced by us, our officers and directors, the underwriters to any offering giving rise to such complaint and any other professional entity whose profession gives authority to a statement made by that person or entity and who has prepared or certified any part of the documents underlying the offering. Our decision to adopt a Federal Forum Provision followed a decision by the Supreme Court of the State of Delaware holding that such provisions are facially valid under Delaware law. While federal or state courts may not follow the holding of the Delaware Supreme Court or may determine that the Federal Forum Provision should be enforced in a particular case, application of the Federal Forum Provision means that suits brought by our stockholders to enforce any duty or liability created by the Securities Act must be brought in federal court and cannot be brought in state court, and our stockholders cannot waive compliance with the federal securities laws and the rules and regulations thereunder.

Section 27 of the Exchange Act creates exclusive federal jurisdiction over all claims brought to enforce any duty or liability created by the Exchange Act or the rules and regulations thereunder. In addition, neither the exclusive forum provision nor the Federal Forum Provision applies to suits brought to enforce any duty or liability created by the Exchange Act. Accordingly, actions by our stockholders to enforce any duty or liability created by the Exchange Act or the rules and regulations thereunder must be brought in federal court, and our stockholders cannot waive compliance with the federal securities laws and the rules and regulations thereunder.

Any person or entity purchasing or otherwise acquiring or holding any interest in any of our securities shall be deemed to have notice of and consented to our exclusive forum provisions, including the Federal Forum Provision. These provisions may limit a stockholders' ability to bring a claim and may result in increased costs for a stockholder to bring such a claim, in a judicial forum of their choosing for disputes with us or our directors, officers, or other employees, or the underwriters of any offering giving rise to such claim, which may discourage lawsuits against us and our directors, officers, and other employees.

If securities or industry analysts do not publish research or reports about our business, or if they issue an adverse or misleading opinion regarding our company, our common stock price and trading volume could decline.

The trading market for our common stock is influenced by the research and reports that industry or securities analysts publish about us or our business. We do not have any control over the analysts, or the content and opinions included in their reports. If any of the analysts who cover us issue an adverse or misleading opinion regarding us, our business model, our intellectual property or our stock performance, or if our preclinical studies and future clinical trials and operating results fail to meet the expectations of analysts, our stock price would likely decline. If one or more of such analysts cease coverage of us or fail to publish reports on us regularly, we could lose visibility in the financial markets, which in turn could cause a decline in our stock price or trading volume.

General Risk Factors

We incur increased costs as a result of operating as a public company, and our management will be required to devote substantial time to new compliance initiatives and corporate governance practices.

As a public company, we incur significant legal, accounting and other expenses that we did not incur as a private company. The Sarbanes-Oxley Act, the Dodd-Frank Wall Street Reform and Consumer Protection Act, the listing requirements of the Nasdaq Global Select Market, or Nasdaq, and other applicable securities rules and regulations impose various requirements on public companies, including establishment and maintenance of effective disclosure and financial controls and corporate governance practices. Our management and other personnel will need to devote a substantial amount of time to these compliance initiatives. Moreover, we expect these rules and regulations to substantially increase our legal and financial compliance costs and to make some activities more time consuming and costly. For example, we expect that these rules and regulations may make it more difficult and more expensive for us to obtain director and officer liability insurance and we may be required to incur substantial costs to maintain sufficient coverage. We cannot predict or estimate the amount or timing of additional costs we may incur to respond to these requirements. The impact of these requirements could also make it more difficult for us to attract and retain qualified persons to serve on our board of directors, our board committees or as executive officers. The increased costs may require us to reduce costs in other areas of our business or increase the prices of our products once commercialized. Moreover, these rules and regulations are often subject to varying interpretations, in many cases due to their lack of specificity, and, as a result, their application in practice may evolve over

time as new guidance is provided by regulatory and governing bodies. This could result in continuing uncertainty regarding compliance matters and higher costs necessitated by ongoing revisions to disclosure and governance practices.

If we fail to maintain proper and effective internal controls over financial reporting our ability to produce accurate and timely financial statements could be impaired.

Pursuant to Section 404 of the Sarbanes-Oxley Act, our management is required to report upon the effectiveness of our internal control over financial reporting and our independent registered public accounting firm is required to attest to the effectiveness of our internal control over financial reporting in our annual reports on Form 10-K. The rules governing the standards that must be met for our management and our independent registered public accounting firm to assess our internal control over financial reporting are complex and require significant documentation, testing, and possible remediation. In connection with our and our independent registered public accounting firm's evaluations of our internal control over financial reporting, we will need to continue to dedicate internal resources, potentially engage outside consultants and adopt a detailed work plan to assess and document the adequacy of internal control over financial reporting, continue steps to improve control processes as appropriate, validate through testing that controls are functioning as documented and implement a continuous reporting and improvement process for internal control over financial reporting. This process will be time-consuming, costly and complicated.

Any failure to maintain internal control over financial reporting, including any failure to implement required new or improved controls, or difficulties encountered in their implementation, could severely inhibit our ability to accurately report our financial condition, results of operations or cash flows. If we or our independent registered public accounting firm are unable to conclude that our internal control over financial reporting is effective, or if our independent registered public accounting firm determines we have a material weakness or significant deficiency in our internal control over financial reporting, investors may lose confidence in the accuracy and completeness of our financial reports, the market price of our common stock could decline, and we could be subject to sanctions or investigations by Nasdaq, the SEC or other regulatory authorities. Failure to remedy any material weakness in our internal control over financial reporting, or to implement or maintain other effective control systems required of public companies, could also restrict our future access to the capital markets.

Our disclosure controls and procedures may not prevent or detect all errors or acts of fraud.

We are subject to the periodic reporting requirements of the Exchange Act. We designed our disclosure controls and procedures to reasonably assure that information we must disclose in reports we file or submit under the Exchange Act is accumulated and communicated to management, and recorded, processed, summarized and reported within the time periods specified in the rules and forms of the SEC. We believe that any disclosure controls and procedures or internal controls and procedures, no matter how well-conceived and operated, can provide only reasonable, not absolute, assurance that the objectives of the control system are met.

These inherent limitations include the realities that judgments in decision-making can be faulty, and that breakdowns can occur because of simple error or mistake. For example, our directors or executive officers could inadvertently fail to disclose a new relationship or arrangement causing us to fail to make any related party transaction disclosures. Additionally, controls can be circumvented by the individual acts of some persons, by collusion of two or more people or by an unauthorized override of the controls. Accordingly, because of the inherent limitations in our control system, misstatements due to error or fraud may occur and not be detected. In addition, we do not have a formal risk management program for identifying and addressing risks to our business in other areas.

We may be subject to securities litigation, which is expensive and could divert management attention.

The market price of our common stock may be volatile. The stock market in general, and Nasdaq and biopharmaceutical companies in particular, have experienced extreme price and volume fluctuations that have often been unrelated or disproportionate to the operating performance of these companies. In the past, companies that have experienced volatility in the market price of their stock have been subject to securities class action litigation. We may be the target of this type of litigation in the future. Securities litigation against us could result in substantial costs and divert our management's attention from other business concerns, which could seriously harm our business.

Unfavorable global economic conditions could adversely affect our business, financial condition, stock price and results of operations.

Our results of operations could be adversely affected by general conditions in the global economy and in the global financial markets. The global economy, including credit and financial markets, has recently experienced extreme volatility and disruptions, including severely diminished liquidity and credit availability, volatile interest rates and increasing inflation, changes in domestic and foreign currency rates, declines in consumer confidence, declines in economic growth, increases in unemployment rates, changes in tariffs and trade policy and uncertainty about economic stability. A severe or prolonged economic downturn or recession and a continued

increase in inflation rates or interest rates could result in a variety of risks to our business, including weakened demand for OJEMDA and our ability to raise additional capital when needed on acceptable terms, if at all. Likewise, the capital and credit markets may be adversely affected by global regional conflicts, and the possibility of wider or additional global conflicts, global sanctions imposed in response thereto or an energy crisis. A weak or declining economy also could strain our suppliers, possibly resulting in supply disruption. We cannot anticipate all of the ways in which the foregoing, and the current economic climate and financial market conditions generally, could adversely impact our business. Furthermore, our stock price may decline due in part to the volatility of the stock market and any general economic downturn.

Further, our business and operations may be impacted by the political instability and military hostilities in multiple geographies including Ukraine, the Middle East and the tensions between China and Taiwan.

Item 2. Unregistered Sales of Equity Securities, Use of Proceeds and Issuer Purchases of Equity Securities.

Unregistered Sales of Equity Securities

None.

Use of Proceeds

None.

Issuer Purchases of Equity Securities

None.

Item 3. Defaults Upon Senior Securities.

None.

Item 4. Mine Safety Disclosures.

Not applicable.

Item 5. Other Information.

Insider Trading Policies and Arrangements

The Company's directors and Section 16 officers (as defined in Rule 16a-1(f) under the Exchange Act) are only permitted to trade in the Company's securities pursuant to a prearranged trading plan intended to satisfy the affirmative defense of Rule 10b5-1(c) under the Exchange Act, or a Rule 10b5-1 Plan. During the three months ended September 30, 2025, none of the Company's Section 16 officers or directors adopted or terminated any Rule 10b5-1 Plan.

Item 6. Exhibits.

The exhibits filed or furnished as part of this Quarterly Report on Form 10-Q are set forth on the Exhibit Index below.

Exhibit Number	Description	Incorporated by Reference				Filed/Furnished Herewith
		Form	File No.	Filing Date	Exhibit	
31.1	Certification of Principal Executive Officer Pursuant to Rules 13a-14(a) and 15d-14(a) under the Securities Exchange Act of 1934, as Adopted Pursuant to Section 302 of the Sarbanes-Oxley Act of 2002.					X
31.2	Certification of Principal Financial Officer Pursuant to Rules 13a-14(a) and 15d-14(a) under the Securities Exchange Act of 1934, as Adopted Pursuant to Section 302 of the Sarbanes-Oxley Act of 2002.					X
32.1*	Certification of Principal Executive Officer Pursuant to 18 U.S.C. Section 1350, as Adopted Pursuant to Section 906 of the Sarbanes-Oxley Act of 2002.					X
32.2*	Certification of Principal Financial Officer Pursuant to 18 U.S.C. Section 1350, as Adopted Pursuant to Section 906 of the Sarbanes-Oxley Act of 2002.					X
101.INS	Inline XBRL Instance Document (the instance document does not appear in the Interactive Data File because its XBRL tags are embedded within the Inline XBRL document).					X
101.SCH	Inline XBRL Taxonomy Extension Schema With Embedded Linkbase Documents.					X
104	Cover Page Interactive Data File (embedded within the Inline XBRL document)					X

* This certification is deemed not filed for purposes of section 18 of the Exchange Act or otherwise subject to the liability of that section, nor shall it be deemed incorporated by reference into any filing under the Securities Act or the Exchange Act.

SIGNATURES

Pursuant to the requirements of the Securities Exchange Act of 1934, as amended, the registrant has duly caused this report to be signed on its behalf by the undersigned thereunto duly authorized.

DAY ONE BIOPHARMACEUTICALS, INC.

Date: November 4, 2025

By: /s/ Jeremy Bender, Ph.D., M.B.A.

Jeremy Bender, Ph.D., M.B.A.
Chief Executive Officer and President
Principal Executive Officer

Date: November 4, 2025

By: /s/ Charles N. York II, M.B.A.

Charles N. York II, M.B.A.
Chief Operating Officer and Chief Financial Officer
Principal Financial and Accounting Officer

**CERTIFICATION PURSUANT TO RULE 13a-14(a) OR 15d-14(a) OF
THE SECURITIES EXCHANGE ACT OF 1934,
AS ADOPTED PURSUANT TO SECTION 302 OF THE SARBANES-OXLEY ACT OF 2002**

I, Jeremy Bender, certify that:

1. I have reviewed this Quarterly Report on Form 10-Q of Day One Biopharmaceuticals, Inc.;
2. Based on my knowledge, this report does not contain any untrue statement of a material fact or omit to state a material fact necessary to make the statements made, in light of the circumstances under which such statements were made, not misleading with respect to the period covered by this report;
3. Based on my knowledge, the financial statements, and other financial information included in this report, fairly present in all material respects the financial condition, results of operations and cash flows of the registrant as of, and for, the periods presented in this report;
4. The registrant's other certifying officer and I are responsible for establishing and maintaining disclosure controls and procedures (as defined in Exchange Act Rules 13a-15(e) and 15d-15(e)) and internal control over financial reporting (as defined in Exchange Act Rules 13a-15(f) and 15d-15(f)) for the registrant and have:
 - a. Designed such disclosure controls and procedures, or caused such disclosure controls and procedures to be designed under our supervision, to ensure that material information relating to the registrant is made known to us by others within those entities, particularly during the period in which this report is being prepared;
 - b. Designed such internal control over financial reporting, or caused such internal control over financial reporting to be designed under our supervision, to provide reasonable assurance regarding the reliability of financial reporting and the preparation of financial statements for external purposes in accordance with generally accepted accounting principles;
 - c. Evaluated the effectiveness of the registrant's disclosure controls and procedures and presented in this report our conclusions about the effectiveness of the disclosure controls and procedures, as of the end of the period covered by this report based on such evaluation; and
 - d. Disclosed in this report any change in the registrant's internal control over financial reporting that occurred during the registrant's most recent fiscal quarter (the registrant's fourth fiscal quarter in the case of an annual report) that has materially affected, or is reasonably likely to materially affect, the registrant's internal control over financial reporting; and
5. The registrant's other certifying officer and I have disclosed, based on our most recent evaluation of internal control over financial reporting, to the registrant's auditors and the audit committee of the registrant's board of directors (or persons performing the equivalent functions):
 - a. All significant deficiencies and material weaknesses in the design or operation of internal control over financial reporting which are reasonably likely to adversely affect the registrant's ability to record, process, summarize and report financial information; and
 - b. Any fraud, whether or not material, that involves management or other employees who have a significant role in the registrant's internal control over financial reporting.

Date: November 4, 2025

/s/ Jeremy Bender, Ph.D., M.B.A.
Jeremy Bender, Ph.D., M.B.A.
Chief Executive Officer and President
(Principal Executive Officer)

**CERTIFICATION PURSUANT TO RULE 13a-14(a) OR 15d-14(a) OF
THE SECURITIES EXCHANGE ACT OF 1934,
AS ADOPTED PURSUANT TO SECTION 302 OF THE SARBANES-OXLEY ACT OF 2002**

I, Charles N. York II, certify that:

1. I have reviewed this Quarterly Report on Form 10-Q of Day One Biopharmaceuticals, Inc.;
2. Based on my knowledge, this report does not contain any untrue statement of a material fact or omit to state a material fact necessary to make the statements made, in light of the circumstances under which such statements were made, not misleading with respect to the period covered by this report;
3. Based on my knowledge, the financial statements, and other financial information included in this report, fairly present in all material respects the financial condition, results of operations and cash flows of the registrant as of, and for, the periods presented in this report;
4. The registrant's other certifying officer and I are responsible for establishing and maintaining disclosure controls and procedures (as defined in Exchange Act Rules 13a-15(e) and 15d-15(e)) and internal control over financial reporting (as defined in Exchange Act Rules 13a-15(f) and 15d-15(f)) for the registrant and have:
 - a. Designed such disclosure controls and procedures, or caused such disclosure controls and procedures to be designed under our supervision, to ensure that material information relating to the registrant is made known to us by others within those entities, particularly during the period in which this report is being prepared;
 - b. Designed such internal control over financial reporting, or caused such internal control over financial reporting to be designed under our supervision, to provide reasonable assurance regarding the reliability of financial reporting and the preparation of financial statements for external purposes in accordance with generally accepted accounting principles;
 - c. Evaluated the effectiveness of the registrant's disclosure controls and procedures and presented in this report our conclusions about the effectiveness of the disclosure controls and procedures, as of the end of the period covered by this report based on such evaluation; and
 - d. Disclosed in this report any change in the registrant's internal control over financial reporting that occurred during the registrant's most recent fiscal quarter (the registrant's fourth fiscal quarter in the case of an annual report) that has materially affected, or is reasonably likely to materially affect, the registrant's internal control over financial reporting; and
5. The registrant's other certifying officer and I have disclosed, based on our most recent evaluation of internal control over financial reporting, to the registrant's auditors and the audit committee of the registrant's board of directors (or persons performing the equivalent functions):
 - a. All significant deficiencies and material weaknesses in the design or operation of internal control over financial reporting which are reasonably likely to adversely affect the registrant's ability to record, process, summarize and report financial information; and
 - b. Any fraud, whether or not material, that involves management or other employees who have a significant role in the registrant's internal control over financial reporting.

Date: November 4, 2025

/s/ Charles N. York II, M.B.A.
Charles N. York II, M.B.A.
Chief Operating Officer and Chief Financial Officer
(Principal Financial Officer and Principal Accounting Officer)

**CERTIFICATION PURSUANT TO
18 U.S.C. SECTION 1350, AS ADOPTED PURSUANT TO
SECTION 906 OF THE SARBANES-OXLEY ACT OF 2002**

I, Jeremy Bender, Chief Executive Officer of Day One Biopharmaceuticals, Inc. (the “Company”), do hereby certify, pursuant to 18 U.S.C. Section 1350, as adopted pursuant to Section 906 of the Sarbanes-Oxley Act of 2002, that to the best of my knowledge:

1. the Quarterly Report on Form 10-Q of the Company for the fiscal quarter ended September 30, 2025 (the “Report”) fully complies with the requirements of Section 13(a) or 15(d) of the Securities Exchange Act of 1934, as amended; and
2. the information contained in the Report fairly presents, in all material respects, the financial condition and results of operations of the Company.

Dated: November 4, 2025

/s/ Jeremy Bender, Ph.D., M.B.A.

Jeremy Bender, Ph.D., M.B.A.

Chief Executive Officer and President

(Principal Executive Officer)

**CERTIFICATION PURSUANT TO
18 U.S.C. SECTION 1350, AS ADOPTED PURSUANT TO
SECTION 906 OF THE SARBANES-OXLEY ACT OF 2002**

I, Charles N. York II, Chief Financial Officer of Day One Biopharmaceuticals, Inc. (the “Company”), do hereby certify, pursuant to 18 U.S.C. Section 1350, as adopted pursuant to Section 906 of the Sarbanes-Oxley Act of 2002, that to the best of my knowledge:

1. the Quarterly Report on Form 10-Q of the Company for the fiscal quarter ended September 30, 2025 (the “Report”) fully complies with the requirements of Section 13(a) or 15(d) of the Securities Exchange Act of 1934, as amended; and
2. the information contained in the Report fairly presents, in all material respects, the financial condition and results of operations of the Company.

Dated: November 4, 2025

/s/ Charles N. York II, M.B.A.

Charles N. York II, M.B.A.

Chief Operating Officer and Chief Financial Officer

(Principal Financial Officer and Principal Accounting Officer)
