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

# FORM 8-K

**CURRENT REPORT**

**Pursuant to Section 13 or 15(d) of the Securities Exchange Act of 1934**

**Date of Report (Date of earliest event reported): May 06, 2024**

# DAY ONE BIOPHARMACEUTICALS, INC.

**(Exact name of registrant as specified in its charter)**

| **Delaware** | **001-40431** | **83-2415215** |
|---|---|---|
| **(State or other jurisdiction of incorporation)** | **(Commission File Number)** | **(IRS Employer Identification No.)** |

| | |
|---|---|
| **2000 Sierra Point Parkway, Suite 501** | |
| **Brisbane, California** | **94005** |
| **(Address of principal executive offices)** | **(Zip Code)** |

**Registrant's telephone number, including area code: (650) 484-0899**

**N/A**

**(Former name or former address, if changed since last report)**

Check the appropriate box below if the Form 8-K filing is intended to simultaneously satisfy the filing obligation of the registrant under any of the following provisions:

☐ Written communications pursuant to Rule 425 under the Securities Act (17 CFR 230.425)

☐ Soliciting material pursuant to Rule 14a-12 under the Exchange Act (17 CFR 240.14a-12)

☐ Pre-commencement communications pursuant to Rule 14d-2(b) under the Exchange Act (17 CFR 240.14d-2(b))

☐ Pre-commencement communications pursuant to Rule 13e-4(c) under the Exchange Act (17 CFR 240.13e-4(c))

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

| Title of each class | Trading Symbol(s) | 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 is an emerging growth company as defined in Rule 405 of the Securities Act of 1933 (§230.405 of this chapter) or Rule 12b-2 of the Securities Exchange Act of 1934 (§240.12b-2 of this chapter).

Emerging growth company ☐

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. ☐

**Item 2.02 Results of Operations and Financial Condition.**

On May 6, 2024, Day One Biopharmaceuticals, Inc. (the "Company") issued a press release announcing its financial results for the quarter ended March 31, 2024. A copy of the press release is attached as Exhibit 99.1 to this report.

**Item 7.01 Regulation FD Disclosure.**

On May 6, 2024, the Company updated its corporate presentation. A copy of the updated presentation is attached as Exhibit 99.2 to this report.

The information in this Current Report on Form 8-K, including Exhibit 99.1 and Exhibit 99.2 to this report, shall not be deemed to be "filed" for purposes of Section 18 of the Securities Exchange Act of 1934, as amended (the "Exchange Act"), or otherwise subject to the liabilities of that section or Sections 11 and 12(a)(2) of the Securities Act of 1933, as amended (the "Securities Act"). The information contained in this Current Report on Form 8-K and in the accompanying Exhibit 99.1 and Exhibit 99.2 shall not be incorporated by reference into any other filing under the Exchange Act or under the Securities Act, except as shall be expressly set forth by specific reference in such filing.

**Item 9.01 Financial Statements and Exhibits.**

(d) Exhibits

| Exhibit Number | Description |
|---|---|
| 99.1 | Press release issued by Day One Biopharmaceuticals, Inc. regarding its financial results for the quarter ended March 31, 2024, dated May 6, 2024. |
| 99.2 | Corporate Presentation. |
| 104 | Cover Page Interactive Data File (embedded within the Inline XBRL document). |

**SIGNATURES**

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

**DAY ONE BIOPHARMACEUTICALS, INC.**

Date: May 6, 2024

By: /s/ Charles N. York II, M.B.A.
Charles N. York II, M.B.A.
Chief Operating Officer and Chief Financial Officer

**Exhibit 99.1**

# Day One Reports First Quarter 2024 Financial Results and Corporate Progress

*OJEMDA™ (tovorafenib) launch underway following U.S. FDA accelerated approval for relapsed or refractory BRAF-altered Pediatric Low-Grade Glioma (pLGG)*

*First prescriptions received in the U.S.*

**BRISBANE, Calif., May 6, 2024 –** Day One Biopharmaceuticals (Nasdaq: DAWN) ("Day One" or the "Company"), a commercial-stage biopharmaceutical company dedicated to developing and commercializing targeted therapies for people of all ages with life-threatening diseases, today announced its first quarter 2024 financial results and highlighted recent corporate achievements.

"We are excited that OJEMDA is now approved and available here in the U.S., and we are grateful to the members of the pediatric brain tumor community whose support of the program has been invaluable," said Jeremy Bender, Ph.D., chief executive officer of Day One. "Our team is focused on executing our U.S. launch, on advancing our other programs, and on exploring opportunities to expand our pipeline."

**Program Highlights**

- In April 2024, the Company received U.S. Food and Drug Administration (FDA) accelerated approval of OJEMDA (tovorafenib), the first and only FDA approved therapy for the treatment of pediatric patients 6 months of age and older with relapsed or refractory pediatric low-grade glioma (pLGG) harboring a BRAF fusion or rearrangement, or BRAF V600 mutation.

- With OJEMDA now available and the first prescriptions written, patients have begun enrolling in EveryDay Support From Day One™, a comprehensive program that offers personalized services for OJEMDA patients and their care teams, including insurance coverage support, financial assistance options and educational resources throughout the treatment journey.

- The pivotal Phase 3 FIREFLY-2/LOGGIC clinical trial evaluating tovorafenib as a front-line therapy in patients aged 6 months to 25 years with pLGG continues to enroll in the United States, Canada, Europe, Australia and Asia, with more than 90 sites activated.

- Patient enrollment continues in the Phase 1b/2 substudy (102b) of the FIRELIGHT-1 trial evaluating the combination of tovorafenib with the Company's investigational MEK inhibitor, pimasertib.

**Corporate Highlights and Upcoming Milestones**

- The Company received a rare pediatric disease priority review voucher from the FDA upon OJEMDA's approval.

- Results from the FIRELIGHT-1 Phase 1b and next steps are expected in the second half of 2024.

**First Quarter 2024 Financial Highlights**

- **Cash Position:** Cash, cash equivalents and short-term investments totaled $317.9 million on March 31, 2024. Based on Day One's current operating plan, management believes it has sufficient capital resources to fund anticipated operations into 2026.

- **R&D Expenses:** Research and development expenses were $40.2 million for the first quarter of 2024 compared to $27.8 million for the first quarter of 2023. The increase was primarily due to additional employee compensation costs, a payment for the buyback of priority review voucher obligation, and increased clinical trial and manufacturing activities related to Day One's lead product, OJEMDA.

- **G&A Expenses:** General and administrative expenses were $26.6 million for the first quarter of 2024 compared to $18.0 million for the first quarter of 2023. The increase was primarily due to additional employee compensation costs, ongoing commercial buildout, and increased professional service expenses to support company growth.

- **Net Loss:** Net loss totaled $62.4 million for the first quarter of 2024 with non-cash stock compensation expense of $12.6 million, compared to $42.4 million for the first quarter of 2023 with non-cash stock compensation expense of $9.4 million.

**Upcoming Events**

- 2024 American Society of Clinical Oncology (ASCO) Annual Meeting, May 31-June 4, 2024
  - Abstract #10036 titled "Type II RAF inhibitor tovorafenib in relapsed/refractory pediatric low-grade glioma (pLGG): Reversible decreases in growth velocity in the phase 2 FIREFLY-1 trial" will be presented in a poster session on Saturday, June 1 from 1:30-4:30 pm CDT in Hall A

- Goldman Sachs 45th Annual Global Healthcare Conference, June 10-13, 2024

- 21st International Symposium on Pediatric Neuro-Oncology (ISPNO), June 28-29, 2024

**About OJEMDA™**
OJEMDA (tovorafenib) is a Type II RAF kinase inhibitor of mutant BRAF V600, wild-type BRAF, and wild-type CRAF kinases.

OJEMDA is indicated for the treatment of patients 6 months of age and older with relapsed or refractory pediatric low-grade glioma (LGG) harboring a BRAF fusion or rearrangement, or BRAF V600 mutation. This indication is approved under accelerated approval based on response rate and duration of response. Continued approval for this indication may be contingent upon verification and description of clinical benefit in a confirmatory trial(s).

Tovorafenib is under evaluation as a therapy for patients with pLGG requiring front-line treatment (Phase 3 FIREFLY-2/LOGGIC). It is also being studied in combination with the MEK inhibitor pimasertib for adolescent and adult patient populations with recurrent or progressive solid tumors with MAPK pathway alterations (FIRELIGHT-1).

Tovorafenib was granted Breakthrough Therapy and Rare Pediatric Disease designations by the FDA for the treatment of patients with pLGG harboring an activating RAF alteration, and it was evaluated by the FDA under priority review. Tovorafenib has also received Orphan Drug designation from the FDA for the treatment of malignant glioma and from the European Commission for the treatment of glioma.

For more information, please visit www.ojemda.com.

**About Day One Biopharmaceuticals**
Day One Biopharmaceuticals is a commercial-stage biopharmaceutical company that believes when it comes to pediatric cancer, we can do better. The Company was founded to address a critical unmet need: the dire lack of therapeutic development in pediatric cancer. Inspired by "The Day One Talk" that physicians have with patients and their families about an initial cancer diagnosis and treatment plan, Day One aims to re-envision cancer drug development and redefine what's possible for all people living with cancer—regardless of age—starting from Day One.

Day One partners with leading clinical oncologists, families, and scientists to identify, acquire, and develop important targeted cancer treatments. The Company's pipeline includes tovorafenib (OJEMDA™) and pimasertib.

Day One is based in Brisbane, California. For more information, please visit www.dayonebio.com or find the Company on LinkedIn or X.

**Cautionary Note Regarding Forward-Looking Statements**
This press release contains "forward-looking" statements within the meaning of the "safe harbor" provisions of the Private Securities Litigation Reform Act of 1995, including, but not limited to: Day One's plans to develop and commercialize cancer therapies, expectations from current clinical trials, the execution of the Phase 2 and Phase 3 clinical trial for tovorafenib as designed, any expectations about safety, efficacy, timing and ability to complete clinical trials, release data results and to obtain regulatory approvals for tovorafenib and other candidates in development, and the ability of tovorafenib to treat pLGG or related indications.

Statements including words such as "believe," "plan," "continue," "expect," "will," "develop," "signal," "potential," or "ongoing" and statements in the future tense are forward-looking statements. These forward-looking statements involve risks and uncertainties, as well as assumptions, which, if they do not fully materialize or prove incorrect, could cause our results to differ materially from those expressed or implied by such forward-looking statements.

Forward-looking statements are subject to risks and uncertainties that may cause Day One's actual activities or results to differ significantly from those expressed in any forward-looking statement, including risks and uncertainties in this press release and other risks set forth in our filings with the Securities and Exchange Commission, including Day One's ability to develop, obtain and retain regulatory approval for or commercialize any product candidate, Day One's ability to protect intellectual property, the potential impact of global business or macroeconomic conditions, including as a result of inflation, rising interest rates, instability in the global banking system, geopolitical conflicts and the sufficiency of Day One's cash, cash equivalents and investments to fund its operations. These forward-looking statements speak only as of the date hereof and Day One specifically disclaims any obligation to update these forward-looking statements or reasons why actual results might differ, whether as a result of new information, future events or otherwise, except as required by law.

**Day One Biopharmaceuticals, Inc.**
**Condensed Statements of Operations**
**(unaudited)**
**(in thousands, except shares)**

| | Three Months Ended March 31, | |
|---|---|---|
| | **2024** | **2023** |
| Operating expenses: | | |
| Research and development | $ 40,210 | $ 27,828 |
| General and administrative | 26,557 | 18,027 |
| Total operating expenses | 66,767 | 45,855 |
| Loss from operations | (66,767) | (45,855) |
| Investment income, net | 4,365 | 3,466 |
| Other expense, net | (10) | (4) |
| Net loss attributable to common stockholders | (62,412) | (42,393) |
| Net loss per share, basic and diluted | $ (0.72) | $ (0.59) |
| Weighted-average number of common shares used in computing net loss per share, basic and diluted | 86,679,282 | 71,972,888 |

**Day One Biopharmaceuticals, Inc.**
**Selected Condensed Balance Sheet Data**
**(unaudited)**
**(in thousands)**

| | **March 31, 2024** | **December 31, 2023** |
|---|---|---|
| Cash, cash equivalents and short-term investments | $ 317,944 | $ 366,347 |
| Total assets | 326,645 | 376,048 |
| Total liabilities | 29,839 | 29,508 |
| Accumulated deficit | (520,997) | (458,585) |
| Total stockholders' equity | 296,806 | 346,540 |

DAY ONE MEDIA
Laura Cooper, Head of Communications
media@dayonebio.com

DAY ONE INVESTORS
LifeSci Advisors, PJ Kelleher
pkelleher@lifesciadvisors.com

#####

# Day One Biopharmaceuticals

Targeted Therapies for People of All Ages

May 2024

# Disclaimer

This presentation and the accompanying oral commentary contain forward-looking statements that are based on our management's beliefs and assumptions and on information currently available to our management. Forward-looking statements are inherently subject to risks and uncertainties, some of which cannot be predicted or quantified. In some cases, you can identify forward-looking statements by terminology such as "may," "will," "should," "could," "expect," "plan," anticipate," "believe," "estimate," "predict," "intend," "potential," "would," "continue," "ongoing" or the negative of these terms or other comparable terminology. Forward-looking statements include all statements other than statements of historical fact contained in this presentation, including information concerning our future financial performance, including the sufficiency of our cash, cash equivalents and short-term investments to fund our operations, business plans and objectives, timing and success of our planned nonclinical and clinical development activities, the results of any of our strategic collaborations, including the potential achievement of milestones and provision of royalty payments thereunder, timing and results of nonclinical studies and clinical trials, efficacy and safety profiles of our products and product candidates, the ability of tovorafenib to treat pediatric low-grade glioma (pLGG) or related indications, the potential therapeutic benefits and economic value of our products and product candidates, potential growth opportunities, competitive position, industry environment and potential market opportunities, our ability to protect intellectual property and the impact of global business or macroeconomic conditions, including as a result of inflation, changing interest rates, cybersecurity incidents, potential instability in the global banking system, uncertainty with respect to the federal debt ceiling and budget and potential government shutdowns related thereto and global regional conflicts, on our business and operations.

Forward-looking statements are subject to known and unknown risks, uncertainties, assumptions and other factors. 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. These factors, together with those that are described under the heading "Risk Factors" contained in our most recent Quarterly Report on Form 10-Q filed with the Securities and Exchange Commission ("SEC") and other documents we file from time to time with the SEC, may cause our actual results, performance or achievements to differ materially and adversely from those anticipated or implied by our forward-looking statements.

In addition, statements that "we believe" and similar statements reflect our beliefs and opinions on the relevant subject. These statements are based upon information available to us as of the date of this presentation, and although we believe such information forms a reasonable basis for such statements, such information may be limited or incomplete, and our statements should not be read to indicate that we have conducted a thorough inquiry into, or review of, all potentially available relevant information. These statements are inherently uncertain and investors are cautioned not to unduly rely upon these statements. Furthermore, if our forward-looking statements prove to be inaccurate, the inaccuracy may be material. In light of the significant uncertainties in these forward-looking statements, you should not regard these statements as a representation or warranty by us or any other person that we will achieve our objectives and plans in any specified time frame, or at all. We undertake no obligation to publicly update any forward-looking statements, whether as a result of new information, future events or otherwise, except as required by law.

This presentation also contains estimates and other statistical data made by independent parties and by us relating to market size and growth and other data about our industry. This data involves a number of assumptions and limitations, and you are cautioned not to give undue weight to such estimates. In addition, projections, assumptions and estimates of our future performance and the future performance of the markets in which we operate are necessarily subject to a high degree of uncertainty and risk.

# Cancer Therapies for People of All Ages

Day One BIOPHARMACEUTICALS

## Our Approach

- Develop medicines for genomically-defined cancers
- Establish first-in-class position through rapid registration pathways
- Expand to adolescent and adult populations in parallel and pursue those opportunities with the same commitment we do for children



Nasdaq: DAWN | IPO: 2021 | Founded: 2018 | Financial Position: Runway into 2026

Day One BIOPHARMACEUTICALS

# Our Pipeline

| Product Candidate | Therapeutic Area | Preclinical | Phase 1 | Phase 2 | Phase 3/ Registrational | Approved | Recent & Anticipated Milestones |
|---|---|---|---|---|---|---|---|
| **Tovorafenib**<br>Type II RAF Inhibitor<br><br>OJEMDA brand name in U.S.[1] | BRAF-altered Relapsed pLGG | FIREFLY-1 (pivotal Phase 2) | | | | ojemda® (tovorafenib) | **FDA approval:**<br>April 2024 |
| | Frontline RAF-altered pLGG | FIREFLY-2 (pivotal Phase 3) | | | | | **First patient dosed:**<br>March 2023 |
| **Pimasertib**<br>MEK 1/2 Inhibitor | MAPK-altered solid tumors[†]<br>(Combo w/ tovorafenib) | FIREFLIGHT-1[††] | | | | | **Recommended Phase 2 dose & schedule expected:**<br>2H 2024 |
| **VRK1 Program**<br>VRK1 Inhibitor | Pediatric and adult cancers | | | | | | **In-licensed[§]:**<br>August 2023 |

[1] OJEMDA has received accelerated approval by the U.S. Food and Drug Administration. [†] Pimasertib Phase 1 dose escalation and expansion trial previously completed. [††] Includes patients ≥12 years of age.
[§] Research collaboration and license agreement with Sprint Bioscience AB for exclusive worldwide rights to a research-stage program targeting VRK1. pLGG, pediatric low-grade glioma.
The safety and efficacy of investigational agents and/or investigational uses of approved products have not been established.



Day One BIOPHARMACEUTICALS

# OJEMDA™ (tovorafenib)

Relapsed or Refractory BRAF-altered pLGG

# OJEMDA Now Approved In The U.S.

ojemda™
(tovorafenib)
100 mg tablets
25 mg/mL for oral suspension

OJEMDA is the **first and only FDA Approved therapy** for the treatment of pediatric patients 6 months of age and older with relapsed or refractory pediatric low-grade glioma harboring a BRAF fusion or rearrangement, or BRAF V600 mutation

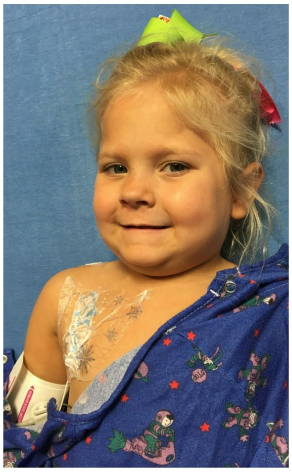


*This indication is approved under accelerated approval based on response rate and duration of response. Continued approval for this indication may be contingent upon verification of clinical benefit in a confirmatory trial.

# pLGG Impact On Patients' Lives

## Lily was diagnosed with an operable brain tumor at 5 months of age






# Pediatric Low-Grade Glioma: The Most Common Type Of Brain Tumor In Children

**pLGGs are chronic and relentless, with patients suffering profound tumor and treatment-associated morbidity that can impact their life trajectory over the long term[1]**

## A Serious and Life-Threatening Disease

- For the majority of pLGG patients in the relapsed setting, there is no standard of care and no approved therapies
- Up to 75% of pLGGs have a BRAF alteration*, of those ~80% are BRAF fusions and ~20% are BRAF V600 mutations[2-6]
- Despite surgery playing a significant role in treatment, the vast majority of patients still require systemic therapy[7,8]
- Due to high rate of disease recurrence, most patients will undergo multiple lines of systemic therapy over the course of their disease



*Incidence of BRAF alterations varies across pLGG subtypes. [1] Sievert AJ, Fisher MJ. Pediatric low-grade gliomas. *J Child Neurol.* 2009;24(11):1397-1408. doi:10.1177/0883073809342005 . [2] Penman CL et al. *Front Oncol.* 2015;5:54. [3] Cohen AR., *N Engl J Med.* 2020;386(20):1922-1931. [4] Lassaletta A, et al. *J Clin Oncol.* 2017;35(25):2934-2941. [5] Faulkner C, et al. J Neuropathol Exp Neurol. 2015;74(9):867-872. [6] Packer RJ, et al. Neuro Oncol. 2017;19(6):750-761. [7] Ostrum QT et al., Neuro Oncol. 2015; 16(Suppl 10):x1-x36; [8] De Blank P. et al., Curr Opin Pediatr. 2019 Feb; 31(1):21-27.

Day One BIOPHARMACEUTICALS

# Conventional Treatments Can Be Disruptive To Childhood And Can Have Significant Long-Term Consequences

## Surgery

- Significant recovery times
- Risks of complications
- Resection may be limited by location of tumor
- Potential for functional deficits based on location of tumor and extent of resection

## Chemotherapy

- Requirement for indwelling catheter and weekly infusions
- Risk of neutropenia, hypersensitivity reactions, nausea and vomiting and peripheral neuropathy

## Radiation

- Risk of secondary malignancy
- Risk of malignant transformation
- Risk of vascular proliferation and stroke
- Neurocognitive impact, depending on location of tumor and radiation field

**Goal of therapy is to control the tumor, minimize the burden of surgery, chemotherapy, and radiation, and reduce the risk of life-long treatment and disease-related effects**



Source: 1. Heitzer AM, Raghubar K, Ris MD, et al. Neuropsychological functioning following surgery for pediatric low-grade glioma: a prospective longitudinal study. J Neurosurg Pediatr. 2019;1-9. doi:10.3171/2019.9.PEDS19357. 2. Bryant R. Managing side effects of childhood cancer treatment. J Pediatr Nurs. 2003;18(2):113-125. doi:10.1053/jpdn.2003.11. 3. Zahnreich S, Schmidberger H. Childhood cancer: occurrence, treatment and risk of second primary malignancies. Cancers (Basel). 2021;13(11):2607. doi:10.3390/cancers/13112607. 4. National Cancer Institute. Fertility issues in girls and women with cancer. http://www.cancer.gov. Accessed June 13, 2022. 5. Alessi I., Caroleo A.M., de Palma L., Mastronuzzi A., Pro S., Colafati G.S., Boni A., Della Vecchia N., Velardi M., Evangelisti M., et al. Short and Long-Term Toxicity in Pediatric Cancer Treatment: Central Nervous System Damage. Cancers. 2022;14:1540. doi: 10.3390/cancers14061540.

Day One BIOPHARMACEUTICALS

# Overview U.S. Prescribing Information For OJEMDA™ (tovorafenib)

## Available in tablet formulation and pediatric-friendly powder for oral suspension

### INDICATION

OJEMDA is indicated for the treatment of pediatric patients 6 months of age and older with relapsed or refractory pediatric low-grade glioma harboring a BRAF fusion or rearrangement, or BRAF V600 mutation

### RECOMMENDED DOSE

380 mg/m$^2$ administered orally once weekly (not to exceed a dose of 600mg once weekly); OJEMDA can be taken with or without food


**For full prescribing information, visit dayonebio.com**

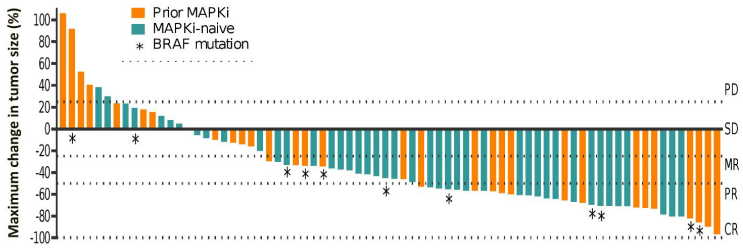


*This indication is approved under accelerated approval based on response rate and duration of response. Continued approval for this indication may be contingent upon verification of clinical benefit in a confirmatory trial.

Day One BIOPHARMACEUTICALS

# Efficacy Summary From OJEMDA™ (tovorafenib) Prescribing Information

**51%** **Overall response rate (RAPNO-LGG) in 76 evaluable patients**




| | RAPNO-LGG | | |
|---|---|---|---|
| Response (IRC) | n | n (%) | 95% CI |
| **ORR, n (%)** | **76** | **39 (51)** | **40-63** |
| BRAF fusion or rearrangement | 64 | 33 (52) | 39-64 |
| BRAF V600 mutation | 12 | 6 (50) | 21-79 |
| Prior MAPKi use | 45 | 22 (49) | 31-64 |
| MAPKi-naïve | 31 | 17 (55) | 36-73 |
| **Median DOR, months** | **39** | **13.8** | **11.3-NR†** |
| **Median TTR, months** | **39** | **5.3** | |
| Range | | 1.6-11.2 | |



June 5, 2023 data cutoff. CI, confidence interval; DOR, duration of response; IRC, independent radiology review committee; LGG, low-grade glioma; NR, not reached; ORR, overall response rate; RAPNO, Response Assessment in Pediatric Neuro-Oncology; TTR, time to response; CR, complete response; PR, partial response; MR, minor response; SD, stable disease; PD, progressive disease. † As of the data cutoff, 66% remain on tovorafenib.

# Safety Summary From OJEMDA™ (tovorafenib) Prescribing Information

## Warnings and Precautions

- Hemorrhage
- Skin toxicity, including photosensitivity
- Hepatotoxicity
- Effect on growth
- Embryo-fetal toxicity
- Use in NF1- associated tumors

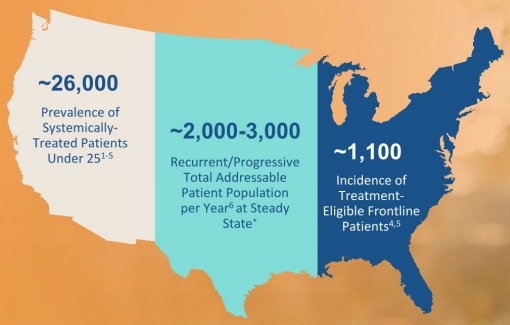
## No boxed warnings or contraindications

| | TEAEs (≥ 30% of patients [n=137]) | |
|---|---|---|
| **Preferred Term, n (%)** | **Any Grade** | **Grade ≥3** |
| Any AE | 137 (100) | 86 (63) |
| Hair color changes | 104 (76) | 0 |
| Anemia | 81 (59) | 15 (11) |
| Elevated CPK | 80 (58) | 16 (12) |
| Fatigue | 76 (55) | 6 (4) |
| Vomiting | 68 (50) | 6 (4) |
| Hypophosphatemia | 64 (47) | 0 |
| Headache | 61 (45) | 2 (1) |
| Maculo-papular rash | 60 (44) | 11 (8) |
| Pyrexia | 53 (39) | 5 (4) |
| Dry skin | 49 (36) | 0 |
| Elevated LDH | 48 (35) | 0 |
| Increased AST | 47 (34) | 4 (3) |
| Constipation | 45 (33) | 0 |
| Nausea | 45 (33) | 0 |
| Upper RTI | 43 (31) | 2 (1) |
| Dermatitis acneiform | 42 (31) | 1 (1) |
| Epistaxis | 42 (31) | 1 (1) |



June 5, 2023 data cutoff. OJEMDA safety data (n=137). Treatment-emergent AEs ≥20% any grade in arms 1 & 2. AE, adverse event; AST, aspartate aminotransferase; CPK, creatine phosphokinase; LDH, lactate dehydrogenase; RTI, respiratory tract infection; TEAEs, treatment-emergent adverse events.

# Estimated *BRAF*-Altered pLGG Patient Population In The U.S.




## Up to 75% of pLGG cases are BRAF-altered[7-14]

*Incidence of BRAF alterations varies across pLGG subtypes*


**of these cases have BRAF fusion, primarily KIAA1549-BRAF†**


of these cases have BRAF point mutations, primarily BRAF V600††



[1] Selt F, van Tilburg CM, Bison B, et al. Response to trametinib treatment in progressive pediatric low-grade glioma patients. J Neurooncol. 2020;149(3):499-510. doi:10.1007/s11060-020-03640-3.[2] Ryall S, Tabori U, Hawkins C. Pediatric low-grade glioma in the era of molecular diagnostics. Acta Neuropathol Commun. 2020;8(1):30. doi:10.1186/s40478-020-00902-z. [3] SEER US complete prevalence counts of patients aged under 25 with Brain and Other Nervous Systems tumors as of January 1, 2017.[4] CBTRUS, Qaddoumi et al 2009, Schreck et al 2019, ClearView Analysis. [5] US Census. Estimated annual incidence, estimated prevalence, and estimated recurrent/progressive total addressable patient population are Day One calculations based on publicly available data.[6] Source: Internal market research conducted by EpidStrategies, A Division of TovStrategies, Inc. on behalf of Day One.[7] Ryall S, et al. *Acta Neuropathol Commun.* 2020;8(1):30. [8] Behling F, et al. *Cancers (Basel).* 2019;11(6):794. [9] Penman CL, et al. *Front Oncol.* 2015;5:54. [10] Packer RJ, et al. *Neuro Oncol.* 2017;19(6):750-761. [11] Cohen AR, et al. *N Engl J Med.* 2022;386(20):1922-1931. [12] Ryall S, et al. *J Neuropathol Exp Neurol.* 2017;76(7):562-570. [13] Lassaletta A, et al. *J Clin Oncol.* 2017;35(25):2934-2941. [14] Faulkner C, et al. *J Neuropathol Exp Neurol.* 2015;74(9):867-872. * The estimated addressable pool of recurrent or progressive pLGG patients is based on progression free survival curves modeled from published literature.† Predominantly seen in pilocytic astrocytomas. †† May vary across pLGG subtypes. BRAF, V-Raf murine sarcoma viral oncogene homolog B; MAPK, mitogen-activated protein kinase; pLGG, pediatric low-grade glioma.

Day One BIOPHARMACEUTICALS

# What Physicians & Caregivers Are Looking For In A Therapy

## What HCP's are Seeking

Effective in stopping or shrinking tumors
Manageable safety profile
Minimal disruption to child's life

***"The goal is not interfering with the child's life."***
*– Ped Onc, Chicago Ad Board*

## What Caregivers are Seeking

Live as normal of a childhood as possible
Minimal impact from the disease
Minimal disruption to child's life

***"Our time with our kids is precious and not guaranteed, so the less time with meds and doctors the better."***
*– Caregiver for a child under 5 yrs*

# Product Profile Aligns With What Physicians Are Looking For In A Therapy

ojemda™ (tovorafenib)
100 mg tablets
25 mg/mL for oral suspension

| | |
|---|---|
| **Efficacy** | Meaningful tumor stabilization or shrinkage may be possible with OJEMDA.<br>In the clinical trial:<br>• 51% of children experienced tumor shrinkage by at least 25%<br>• 82% of children saw their tumors shrink or remain stable |
| **Safety** | Generally well-tolerated therapy, with 9 out of 10 patients staying on treatment in the clinical trial<br><br>Most common grade 3 / 4 adverse events include: anemia, elevated CPK, maculo-papular rash, fatigue & vomiting |
| **Dosing** | Once-weekly, taken with or without food conveniently from home can mean fewer daily interruptions |

**OJEMDA is indicated for the treatment of patients 6 months of age and older with relapsed or refractory pediatric low-grade glioma (LGG) harboring a BRAF fusion, rearrangement, or BRAF V600 mutation.**



Data from Pivotal Phase 2 FIREFLY-1 trial.

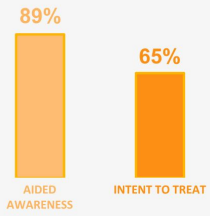
Day One BIOPHARMACEUTICALS

# Comprehensive Approach For A Successful Launch

ojemda™ (tovorafenib)
100 mg tablets
25 mg/mL for oral suspension

## Physicians

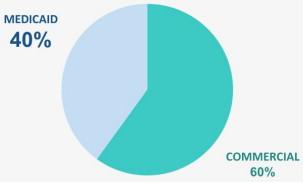
**Objective**: Establish OJEMDA™ as 1st choice in relapsed / refractory *BRAF*-altered pLGG patients




- Dedicated & experienced sales team to engage HCPs

## Payers

**Objective**: Rapidly establish coverage




- Pre-launch engagement to establish Day One & provide background information
- Plans in place for rapid engagement post-approval

## Patients & Families

**Objective**: Provide a positive & supportive experience when initiating therapy

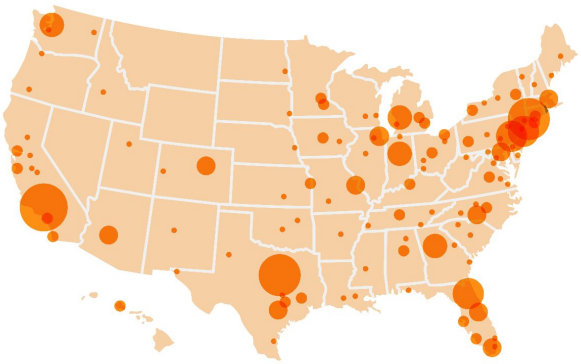
Biologics By McKesson | Onco360 ONCOLOGY PHARMACY

- SP distribution enables consistent patient experience
- Comprehensive patient support programs address patient needs and accelerates access to drug

# Targeted Launch With Highly Experienced Field Team

ojemda™ (tovorafenib)
100 mg tablets
25 mg/mL for oral suspension

## Targeting ~200 centers where 90% of pLGG patients receive treatment



## Deep oncology experience with relationships at top-tier accounts

**18 Account Managers** fully-dedicated to OJEMDA

**Average experience:**

**13** years of oncology

**4** years of rare disease

**2** years of pediatric oncology clinical experience

**Institutional experience and existing relationships with key accounts**



Day One BIOPHARMACEUTICALS

# Patient Support Program Supporting Access

ojemda™ (tovorafenib)
100 mg tablets
25 mg/mL for oral suspension

EveryDay Support.
FROM DAY ONE




Day One BIOPHARMACEUTICALS

# FIREFLY-2 / LOGGIC

## Pivotal Phase 3 Trial of Tovorafenib in Frontline pLGG

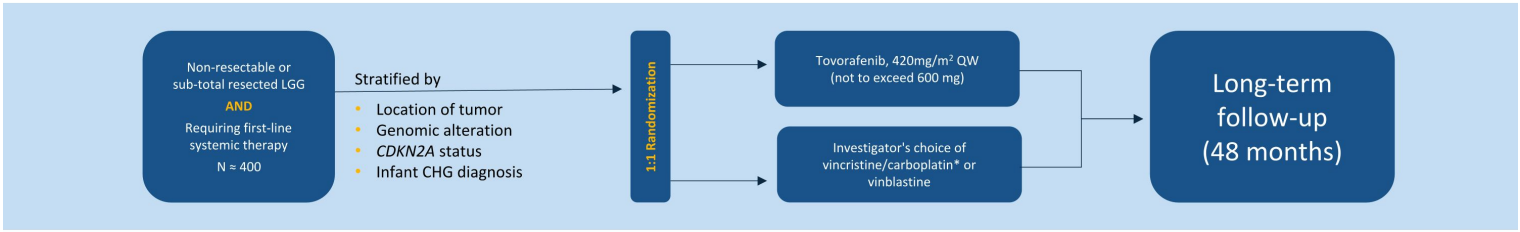
# FIREFLY-2/LOGGIC Pivotal Phase 3 Trial Of Tovorafenib In Frontline pLGG

## Trial Design

- Randomized, global, registrational Phase 3 trial of monotherapy tovorafenib vs SoC chemotherapy
- Eligibility: Patients aged 6 months to <25 years with LGG harboring a RAF alteration and requiring first-line systemic therapy
- Tovorafenib available as tablets and pediatric-friendly liquid suspension
- Patients who progress after stopping tovorafenib may be re-challenged
- Patients who progress in the SoC arm during or post-treatment may cross-over to receive tovorafenib

## Endpoints

- **Primary endpoint: ORR based on RANO-LGG criteria, assessed by blinded independent central review**
  - **The ORR primary analysis is expected to occur ~12 months after the last patient randomized**
- Key secondary endpoints: PFS and DoR by RANO criteria, ORR by RAPNO criteria
- Other secondary endpoints: changes in neurological and visual function, safety, and tolerability
- Key exploratory objectives: QoL and health utilization measures




* COG or SIOPe-LGG regimen. Abbreviations: CHG, chiasmatic, hypothalamic glioma; DoR, duration of response; LGG, low-grade glioma; ORR, objective response rate; QoL, quality of life; QW, once weekly; SoC, standard of care.

# FIRELIGHT-1

## Phase 1b/2 Trials Evaluating Tovorafenib as a Combination with Pimasertib

# Pimasertib: Investigational Allosteric MEK1/2 Inhibitor With Demonstrated Activity In MAPK-Driven Solid Tumors

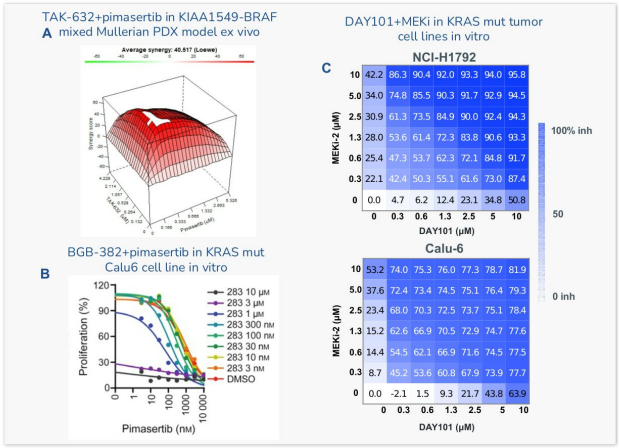
- Pimasertib is an investigational orally-bioavailable, selective, non-competitive MEK1/2 inhibitor in-licensed from Merck KGaA in February 2021
- Extensive non-clinical and clinical development work through Phase 2, including a solid tumor trial in Japan and combinations with other MOAs
- Main AEs typical for all in-class allosteric MEK inhibitors (GI, CPK elevation, skin rash, visual disturbances)
- Nearly three-fold higher CNS penetration than other MEKi inhibitors (trametinib or selumetinib)
- Pimasertib showed monotherapy clinical activity, including an improvement in median PFS versus dacarbazine in NRAS mutant melanoma
- Combination with tovorafenib and other targeted therapies may unlock the full value of pimasertib in advanced solid tumors



Sources: Pimasertib Investigator Brochure, v12, 2019; de Gooijer et al., Int J Cancer, 2018; Shaw et al., AACR LB-456, 2012; Lebbe et al., Cancers, 2020.

# Vertical MAPK Pathway Inhibition With Tovorafenib And Pimasertib May Unlock Potential Synergy For Adult Solid Tumors

| BRAF non-V600 mutant | KRAS or NRAS mutant |
|---|---|
| MEK → ERK | RAF/MEK/ERK; PI3K/m TOR; PI3K/m TOR |
| Proliferation, survival | Proliferation, survival |
| Type II RAFi + MEKI | Type II RAFi + MEKI |
| Non V600 BRAF dimers are effectively inhibited by type II RAFi , but not type I BRAFi | Targeting multiple nodes of MAPK pathway will drive deeper and more durable response |




A Type II RAFi + MEKi is synergistic in BRAF fusion melanoma PDX model ex vivo (internal data)

B Sensitivity of KRAS Q61 mutant cells to pimasertib is enhanced when cells are treated with the type II BRAF inhibitor BGB-283 (Yuan et al., Mol Onc 2020)

C Tovorafenib + MEK inhibitor is synergistic in KRAS G12C and Q61 mutant tumor cells (Venetsanakos et al., 2021 AACR poster presentation)



Day One BIOPHARMACEUTICALS

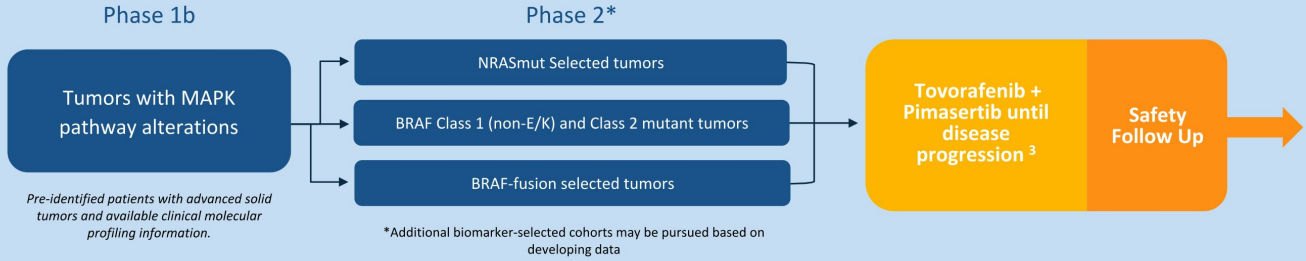
# Tovorafenib / Pimasertib Combination In Solid Tumors (FIRELIGHT-1)

## Trial Design[1]

- Combination dose escalation, global phase 1b/2 trial[2]
- Phase 1b, BOIN (adaptive), n = 10/cohort (approximately)
- Phase 2, Simon 2-stage, n = 25/cohort (approximately)
- Eligibility: Patients aged 12 years and older, dose escalation will be performed in advanced solid tumor patients with any MAPK alteration. Expansion cohorts will focus on indications with a potential path to accelerated approval

## Endpoints

- Phase 1b: PK, PD and Safety, MTD/RP2D
- Phase 2: Efficacy (ORR, DOR)






Abbreviations: BOIN, Bayesian Optimal Interval Design; BRAF, B-Raf proto-oncogene, serine/threonine kinase; MAPK, mitogen-activated protein kinase; NRAS, neuroblastoma rat sarcoma viral oncogene. [1]Umbrella master study – DAY101-102 (main protocol) DAY101 and MAPK pathway aberration, Sub-study 1 monotherapy (DAY101-102a), Sub-study 2 MEK combo (DAY101-102b). [2]Intend to open U.S. and ex-U.S. clinical sties. [3]DAY101 + Pimasertib until disease progression, intolerable toxicity, withdrawal of consent, or death

# Summary

# Financial Summary: DAWN

**Cash, cash equivalents and short-term investments as of March 31, 2024: $317.9 million (no debt)**

**~87.4 million shares of common stock outstanding as of May 1, 2024**

| $ Millions | Three Months Ended 3/31/24 | Three Months Ended 3/31/23 |
|---|---|---|
| R&D Expense | $40.2 | $27.8 |
| G&A Expense | $26.6 | $18.0 |
| Net Loss | $62.4 | $42.4 |

**Projected Cash Runway into 2026**

**FIREFLY-1: Pivotal Phase 2 clinical trial of tovorafenib**

- Data published in *Nature Medicine* and oral presentations at SNO in November 2023
- OJEMDA™ (tovorafenib) approved in the U.S. and received PRV in April 2024

**FIREFLY-2/LOGGIC: Pivotal Phase 3 clinical trial of tovorafenib in newly diagnosed pLGG**

- First patient dosed in March 2023

# Priorities as a Commercial-Stage Company

## Launch OJEMDA™ (tovorafenib)

- Expand awareness amongst physicians and establish broad coverage to enable patient access
- Establish OJEMDA as the standard of care for relapsed or refractory pLGG harboring a BRAF alteration
- Provide a positive and supportive experience when initiating OJEMDA therapy for patients and families

## Advance Portfolio

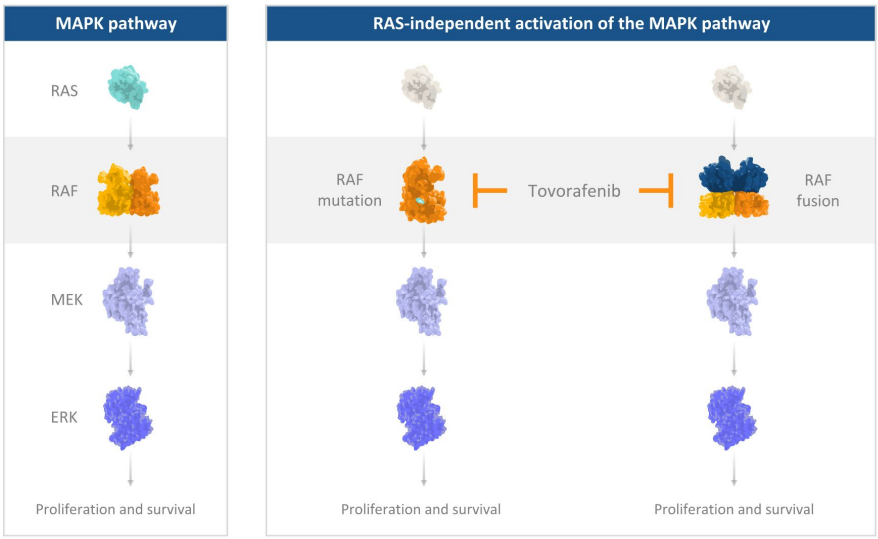
- FIREFLY-2: Study tovorafenib as a frontline therapy for treatment-naive patients with pLGG
- FIRELIGHT-1: Evaluate tovorafenib in combination with pimasertib in adolescent and adult populations
- Advance early stage VRK1 program to clinical development

## Expand Pipeline

- Grow Day One into a leading, biopharmaceutical company that is the partner of choice for oncology drug development
- Explore selective partnerships as a source of capital and risk sharing
- Further invest in business development activities to expand our multiple asset portfolio for both children and adults

# Appendix

# Tovorafenib Inhibits Both BRAF Fusions And BRAF V600 Mutations




Tovorafenib is an investigational, oral, selective, CNS-penetrant, type II RAF inhibitor that was designed to inhibit both monomeric and dimeric RAF kinase

- Activity in tumors driven by both RAF fusions and BRAF V600E mutations
- Tablet and pediatric-friendly liquid suspension
- Once weekly dosing

Currently approved type I BRAF inhibitors are indicated for use in patients with tumors bearing BRAF V600 mutations

- Type I BRAF inhibitors cause paradoxical MAPK activation in the setting of wild-type RAF, increasing the risk of tumor growth in BRAF fusion-driven


Source: 1. Sun Y et al., Neuro Oncol. 2017; 19: 774–85; 2. Sievert AJ et al., PNAS. 2013; 110:5957-62; 3. Karajannis MA et al., Neuro Oncol 2014;16(10):1408-16.

# Pivotal Phase 2 Trial Of Monotherapy Tovorafenib In Relapsed Or Progressive pLGG (FIREFLY-1)

## Trial Design

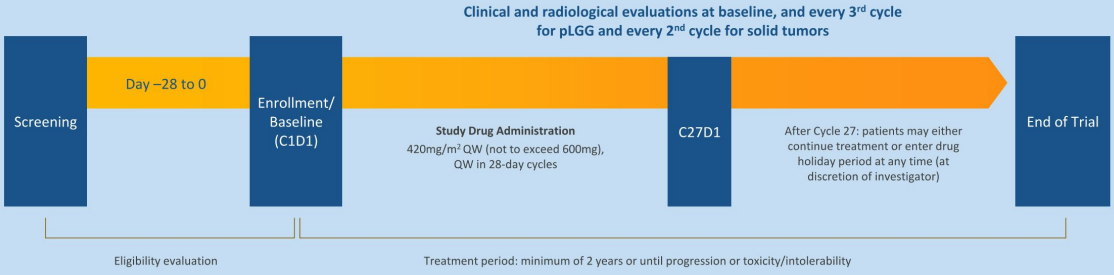
- Three arm, open-label, global registrational phase 2 trial
  - **Pivotal Arm 1 (recurrent/progressive pLGG, n=77): harboring a KIAA1549-BRAF fusion or BRAF V600E mutation**
  - Arm 2 (expanded access recurrent/progressive LGG, n=60): harboring an activating RAF alteration
  - Arm 3 (extracranial solid tumors): harboring an activating RAF fusion

## Endpoints (Pivotal Arm 1)

- **Primary endpoint: ORR based on RANO-HGG[1], assessed by blinded independent central review**
- Secondary endpoints: ORR by RAPNO-LGG[2] assessed by blinded independent central review; PFS, DoR; TTR, CBR; safety
- Exploratory analyses: ORR and CBR by RANO-LGG[3] assessed by blinded independent central review

**Key Inclusion Criteria**

- 6 months – 25 years of age
- RAF-altered tumor
- ≥1 prior line of systemic therapy with radiographic progression
- Prior use of MAPK pathway targeted therapy was permitted






June 5, 2023 data cutoff. [1] Wen PY, et al. *J Clin Oncol*. 2010;28(11):1963-1972. [2] Fangusaro J, et al. Lancet Oncol. 2020;21(6):e305–316. [3] van den Bent MJ, et al. Lancet Oncol. 2011;12(6):583-593. Abbreviations: CBR, clinical benefit rate; IRC, independent review committee; C, cycle; D, day; LGG, low-grade glioma; ORR, objective response rate; PFS, progression-free survival; DoR, duration of response; QW, once weekly; TTR, time to response; RANO, Response Assessment in Neuro-Oncology; RAPNO, Response Assessment in Pediatric Neuro-Oncology; MAPK, mitogen-activated protein kinase. For more information, please refer to NCT04775485

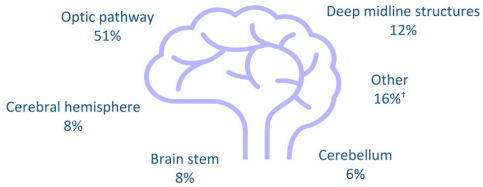
# Data from Pivotal Phase 2 FIREFLY-1 Trial

June 5, 2023 data cutoff

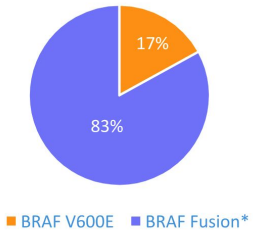
# FIREFLY-1 Baseline Patient Characteristics

| Characteristic | Arm 1 (n=77) |
|---|---|
| Median age, years (range) | 8 (2-21) |
| Sex, n (%) | |
| Male | 40 (52) |
| Female | 37 (48) |
| Race, n (%) | |
| White | 41 (53) |
| Asian | 5 (6) |
| Black | 2 (3) |
| Multiple | 3 (4) |
| Other | 6 (8) |
| Not specified | 20 (26) |
| Number of lines of prior systemic therapy | |
| Median (range) | 3 (1-9) |
| 1, n (%) | 17 (22) |
| 2, n (%) | 21 (27) |
| ≥3, n (%) | 39 (51) |
| Prior MAPK pathway targeted therapy, n (%) | |
| Prior MEK inhibitor | 43 (56) |
| Prior BRAF inhibitor | 8* (10) |
| Prior BRAF and MEK inhibitors‡ | 5 (7) |
| Any MAPK inhibitor | 46 (60) |

**Location (n=77)**



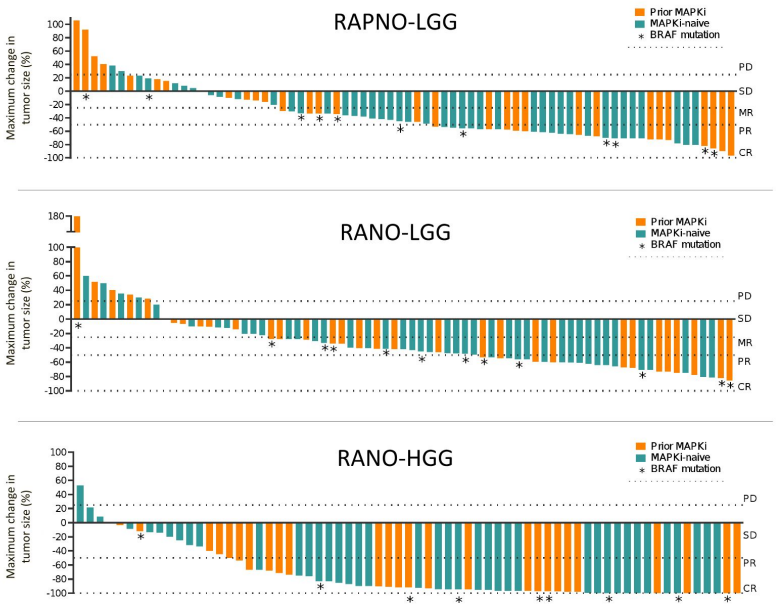

**BRAF alteration (n=77)**






June 5, 2023 data cutoff. *Includes 6 patients with BRAF duplication and 2 with BRAF rearrangement per fluorescence in situ hybridization or in situ hybridization. †Includes tumors that were extending into multiple regions of the brain, leptomeningeal disease, and/or spinal disease. ‡The 5 patients that had previously received both a MEK inhibitor and also a BRAF inhibitor are recorded in both the "Prior MEK inhibitor" and "Prior BRAF inhibitor" groups. MAPK, mitogen-activated protein kinase.

# Tumor Response To Tovorafenib Using RAPNO-LGG, RANO-LGG and RANO-HGG

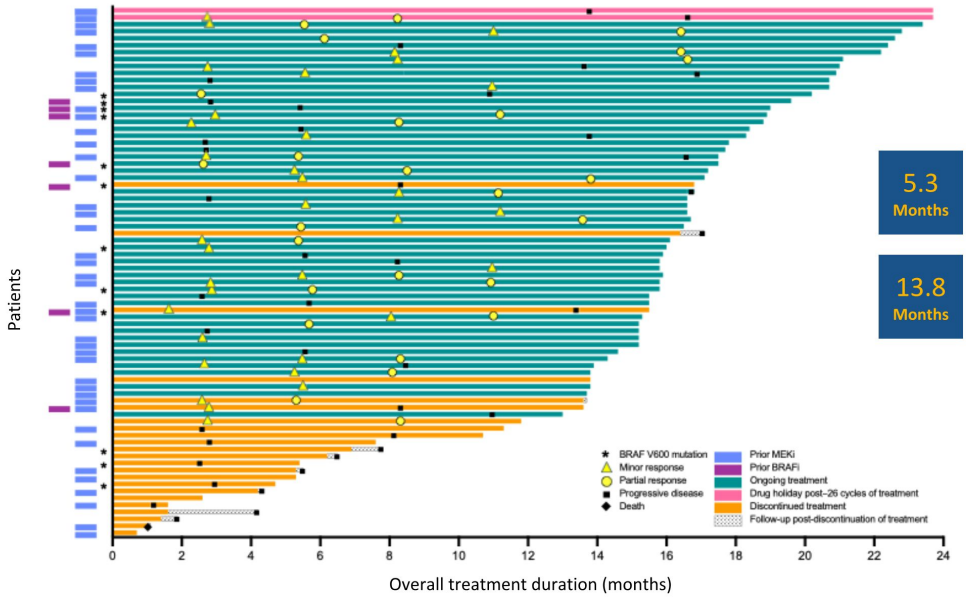



| Response (IRC) | RAPNO-LGG n=76 | RANO-LGG N=76 | RANO-HGG N=69 |
|---|---|---|---|
| **ORR,* n (%)** | **39 (51)** | **40 (53)** | **46 (67)** |
| 95% CI | 40-63 | 41-64 | 54-78 |
| **CBR,* n (%)** | | | |
| SD of any length of time | 62 (82) | 63 (83) | 64 (93) |
| SD ≥12 months | 43 (57) | 46 (61) | 54 (78) |
| **BOR,* n (%)** | | | |
| CR | 0 | 0 | 12 (17) |
| PR | 28 (37) | 20 (26) | 34 (49) |
| MR | 11 (14) | 20 (26) | n/a |
| SD | 23 (30) | 23 (30) | 18 (26) |
| SD <12 months | 19 (25) | 17 (22) | 10 (14) |
| SD ≥12 months | 4 (5) | 6 (8) | 8 (12) |
| PD | 13 (17) | 11 (14) | 4 (6) |
| NE | 1 (1) | 2 (3) | 1 (1) |
| **Median DOR, months** | **13.8** | **14.4** | **16.6** |
| 95% CI | 11.3-NR | 11.0-NR | 11.6-NR |
| **Median TTR, months** | **5.3** | **5.5** | **3.0** |
| Range | 1.6-11.2 | 1.6-11.3 | 2.6-16.6 |



June 5, 2023 data cutoff. BOR, best overall response; CBR, clinical benefit rate; CI, confidence interval; CR, complete response; DOR, duration of response; HGG, high-grade glioma; IRC, independent radiology review committee; LGG, low-grade glioma; MR, minor response; n/a, not applicable; NE, not evaluable; NR, not reached; ORR, overall response rate; PD, progressive disease; PR, partial response; RANO, Response Assessment in Neuro-Oncology; RAPNO, Response Assessment in Pediatric Neuro-Oncology; SD, stable disease; TTR, time to response. * ORR, CBR and BOR for RAPNO-LGG and RANO-LGG included MRs.

# Duration Of Tovorafenib Therapy For All Patients With RAPNO-LGG Evaluable Lesions



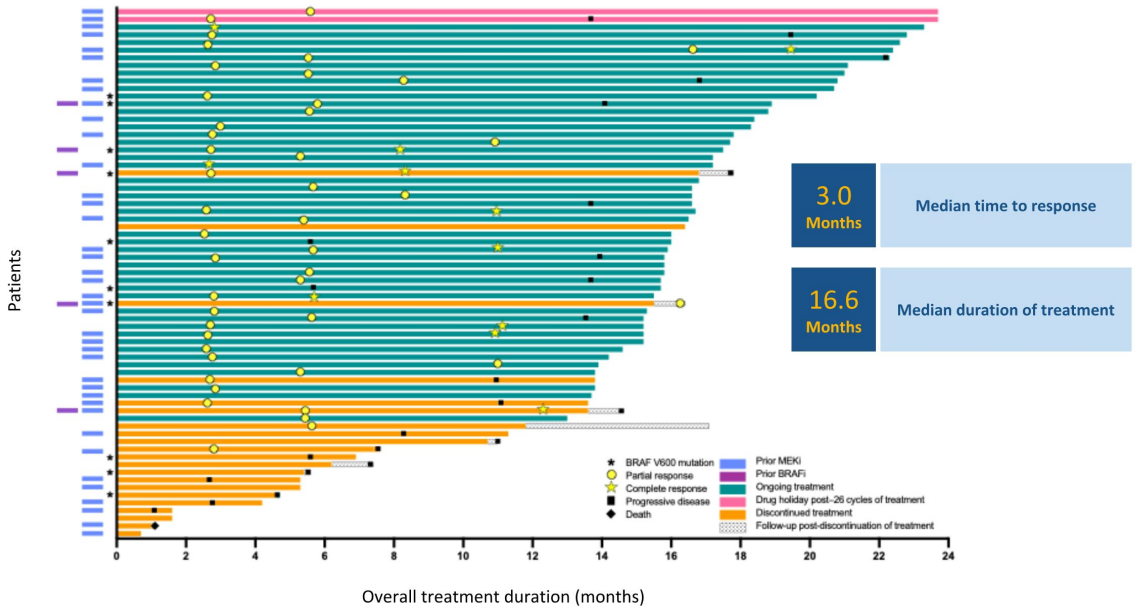

**5.3 Months** — Median time to response

**13.8 Months** — Median duration of treatment



June 5, 2023 data cutoff.

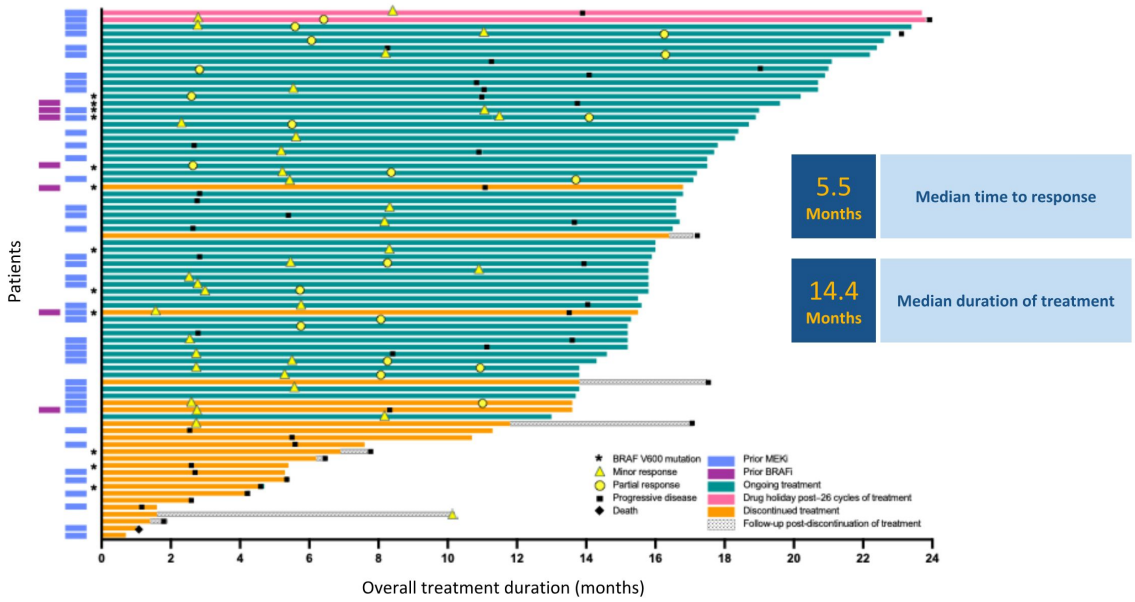
# Duration Of Tovorafenib Therapy For All Patients With RANO-HGG Evaluable Lesions






June 5, 2023 data cutoff.

# Duration Of Tovorafenib Therapy For All Patients With RANO-LGG Evaluable Lesions






June 5, 2023 data cutoff.

# Tumor Response To Tovorafenib Across Three Assessment Criteria Were Consistent Across BRAF Fusion And Mutation Patients, and Patients With Prior MAPK Treatment

| | RAPNO-LGG[2] | | RANO-LGG[3,4] | | RANO-HGG[1] | |
|---|---|---|---|---|---|---|
| Response (IRC) | n | | n | | n | |
| **ORR,* n (%)** | **76** | **39 (51)** | **76** | **40 (53)** | **69** | **46 (67)** |
| BRAF fusion | 64 | 33 (52) | 64 | 33 (52) | 59 | 41 (69) |
| BRAF mutation | 12 | 6 (50) | 12 | 7 (58) | 10 | 5 (50) |
| Prior MAPKi | 45 | 22 (49) | 45 | 23 (51) | 41 | 29 (71) |
| MAPKi-naive | 31 | 17 (55) | 31 | 17 (55) | 28 | 17 (61) |
| **CBR,* n (%) (SD of any length of time)** | **76** | **62 (82)** | **76** | **63 (83)** | **69** | **64 (93)** |
| BRAF fusion | 64 | 53 (83) | 64 | 53 (83) | 59 | 55 (93) |
| BRAF mutation | 12 | 9 (75) | 12 | 10 (83) | 10 | 9 (90) |
| Prior MAPKi | 45 | 38 (84) | 45 | 38 (84) | 41 | 37 (90) |
| MAPKi-naive | 31 | 24 (77) | 31 | 25 (81) | 28 | 27 (96) |
| **CBR,* n (%) (SD ≥12 months)** | **76** | **43 (57)** | **76** | **46 (61)** | **69** | **54 (78)** |
| BRAF fusion | 64 | 37 (58) | 64 | 39 (61) | 59 | 49 (83) |
| BRAF mutation | 12 | 6 (50) | 12 | 7 (58) | 10 | 5 (50) |
| Prior MAPKi | 45 | 25 (56) | 45 | 26 (58) | 41 | 33 (80) |
| MAPKi-naive | 31 | 18 (58) | 31 | 20 (65) | 28 | 21 (75) |
| **Median DOR, months (95% CI)**** | **39** | **13.8 (11.3-NR)** | **40** | **14.4 (11.0-NR)** | **46** | **16.6 (11.6-NR)** |
| BRAF fusion | 33 | 13.8 (11.3-NR) | 33 | 16.3 (11.0-NR) | 41 | 16.8 (11.6-NR) |
| BRAF mutation | 6 | NR (8.4-NR) | 7 | 12.0 (8.4-NR) | 5 | 15.1 (8.3-NR) |
| Prior MAPKi | 22 | 13.8 (11.3-NR) | 23 | 12.0 (8.5-NR) | 29 | 15.1 (9.0-16.8) |
| MAPKi-naive | 17 | NR (8.4-NR) | 17 | 16.3 (8.4-NR) | 17 | NR (11.6-NR) |



June 5, 2023 data cutoff. [1] Fangusaro J, et al. *Lancet Oncol.* 2020;21(6):e305–316. [2] Fangusaro J, et al. *Lancet Oncol.* 2020;21(6):e305–316. [3] van den Bent MJ, et al. *Lancet Oncol.* 2011;12(6):583-593. 4. Wen PY, et al. *J. Clin Oncol.* 2017;35(21),2439-2449. * ORR, CBR for RAPNO-LGG and RANO-LGG included MRs. ** the 95% CI were calculated using Kaplan-Meier method.

# Tovorafenib Safety Data (n=137)

| | TEAEs | | TRAEs | |
|---|---|---|---|---|
| Preferred Term, n (%) | Any Grade | Grade ≥3 | Any Grade | Grade ≥3 |
| Any AE | 137 (100) | 86 (63) | 134 (98) | 58 (42) |
| Hair color changes | 104 (76) | 0 | 104 (76) | 0 |
| Anemia | 81 (59) | 15 (11) | 67 (49) | 14 (10) |
| Elevated CPK | 80 (58) | 16 (12) | 77 (56) | 16 (12) |
| Fatigue | 76 (55) | 6 (4) | 60 (44) | 6 (4) |
| Vomiting | 68 (50) | 6 (4) | 28 (20) | 3 (2) |
| Hypophosphatemia | 64 (47) | 0 | 48 (35) | 0 |
| Headache | 61 (45) | 2 (1) | 29 (21) | 0 |
| Maculo-papular rash | 60 (44) | 11 (8) | 56 (41) | 11 (8) |
| Pyrexia | 53 (39) | 5 (4) | 17 (12) | 1 (1) |
| Dry skin | 49 (36) | 0 | 45 (33) | 0 |
| Elevated LDH | 48 (35) | 0 | 42 (31) | 0 |
| Increased AST | 47 (34) | 4 (3) | 41 (30) | 4 (3) |
| Constipation | 45 (33) | 0 | 31 (23) | 0 |
| Nausea | 45 (33) | 0 | 25 (18) | 0 |
| Upper RTI | 43 (31) | 2 (1) | 2 (1) | 0 |
| Dermatitis acneiform | 42 (31) | 1 (1) | 41 (30) | 1 (1) |
| Epistaxis | 42 (31) | 1 (1) | 27 (20) | 0 |
| Decreased appetite | 39 (28) | 5 (4) | 28 (20) | 4 (3) |
| Paronychia | 36 (26) | 2 (1) | 32 (23) | 2 (1) |
| Pruritus | 35 (26) | 1 (1) | 32 (23) | 1 (1) |
| COVID-19 | 34 (25) | 0 | 0 | 0 |

- The most common reasons for discontinuation were tumor hemorrhage (3 patients) and decrease in growth velocity (2 patients)
- 33 patients (24%) had TRAEs leading to dose reduction; 50 patients (37%) had TRAEs leading to dose interruption
- Median duration of dose interruption was 2 weeks
- 9 patients (7%) had TRAEs leading to discontinuation



June 5, 2023 data cutoff. Treatment-emergent AEs ≥25% any grade in arms 1 & 2. AE, adverse event; ALT, Alanine transaminase; AST, aspartate aminotransferase; COVID-19, Coronavirus disease 2019; CPK, creatine phosphokinase; LDH, lactate dehydrogenase; RTI, respiratory tract infection; TEAEs, treatment-emergent adverse events; TRAEs, treatment-related adverse events.