



### FDA Approval Call

April 24, 2024

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#### **Forward Looking Statements**

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, including as a result of inflation, changing interest rates, cybersecurity incidents, 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.

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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.





#### **Agenda**

#### **Opening Remarks**

Jeremy Bender (Chief Executive Officer)

#### **OJEMDA™** (tovorafenib) Prescribing Information & Clinical Data

Sam Blackman (Co-Founder & Head of R&D)

#### **U.S. Launch Plans for OJEMDA**

Lauren Merendino (Chief Commercial Officer)

#### **Closing Remarks**

Jeremy Bender (Chief Executive Officer)

#### **Q&A Session**

 All, joined by: Charles York (Chief Operating Officer & Chief Financial Officer)





## **Opening Remarks**

Jeremy Bender

Chief Executive Officer

#### **OJEMDA Now Approved In The U.S.**



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



# OJEMDA™ (tovorafenib) Prescribing Information And Clinical Data

Sam Blackman

Co-Founder & Head of R&D

#### 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<sup>1</sup>

#### 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<sup>2-6</sup>
- Despite surgery playing a significant role in treatment, the vast majority of patients still require systemic therapy<sup>7,8</sup>
- Due to high rate of disease recurrence, most patients will undergo multiple lines of systemic therapy over the course of their disease



## 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



#### 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<sup>2</sup> 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

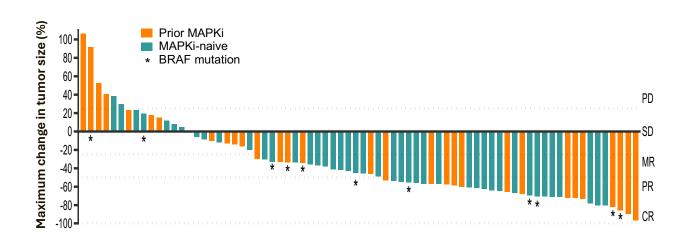


## Efficacy Summary From OJEMDA™ (tovorafenib) Prescribing Information



**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 <sup>†</sup>
Median TTR, months	39	5.3	
Range		1.6-11.2	



## 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	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)		



Thank you



## U.S. Launch Readiness For OJEMDA™ (tovorafenib)

Lauren Merendino

Chief Commercial Officer

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

~26,000 Prevalence of Systemically-Treated Patients Under 25 1-5

~2,000-3,000

Recurrent/Progressive Total Addressable Patient Population per Year<sup>6</sup> at Steady State\*

#### Up to **75%** of pLGG cases are BRAF-altered<sup>7-14</sup>

Incidence of BRAF alterations varies across pLGG subtypes



of these cases have BRAF fusion, primarily KIAA1549-BRAF<sup>†</sup>



of these cases have BRAF point mutations, primarily BRAF V600<sup>††</sup>





#### 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



#### **Efficacy**

Meaningful tumor stabilization or shrinkage may be possible with OJEMDA. In the clinical trial:

- 51% of children experienced tumor shrinkage by at least 25%
- 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

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.

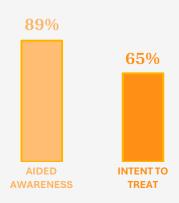


#### Comprehensive Approach For A Successful Launch



#### Physicians

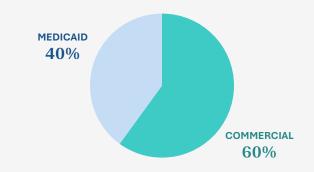
**Objective:** Establish OJEMDA<sup>TM</sup> as  $1^{st}$  choice in relapsed / refractory BRAF-altered pLGG patients



 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 postapproval

#### Patients & Families

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

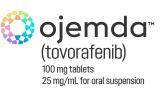




- SP distribution enables consistent patient experience
- Comprehensive patient support programs address patient needs and accelerates access to drug

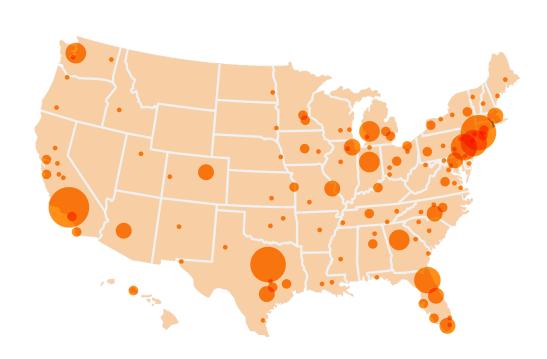


#### **Targeted Launch With Highly Experienced Field Team**



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



#### **Patient Support Program Supporting Access**





FROM DAY ONE





## **Closing Remarks**

Jeremy Bender

Chief Executive Officer

Our commitment will not stop here

Thank You



