First Quarter 2025 Financial Results & Corporate Progress

MAY 2025

Day One
BIOPHARMACEUTICALS

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 commercialization and marketing efforts, 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, efficacy and safety profiles of our products and product candidates, the ability of OJEMDA™ (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, significant political, trade or regulatory developments, including tariffs or shifting priorities within the U.S. Food and Drug Administration, 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.

Agenda & Day One Participants

Opening Remarks	**Jeremy Bender** (Chief Executive Officer)
OJEMDA™ (tovorafenib)	**Lauren Merendino** (Chief Commercial Officer)
Financial Performance	**Charles York** (Chief Operating & Chief Financial Officer)
Q&A Session	**All, joined by: Elly Barry** (Chief Medical Officer)

Opening Remarks

Jeremy Bender

Chief Executive Officer

First quarter 2025 highlights

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

$87.7M in net product revenue since launch[1]

$30.5M in net product revenue in Q1 2025

Pipeline Progress

Tovorafenib EMA regulatory submission accepted

Building value across multiple programs with inflection points on the horizon

Financial Position

Strong balance sheet with $473M in cash[2]

Financial independence from capital markets

[1] OJEMDA received U.S. FDA accelerated approval for relapsed or refractory BRAF-altered pediatric low-grade glioma on April 23, 2024. [2] Represents cash, cash equivalents and short-term investments as of March 31, 2025.

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OJEMDA Launch Performance

Lauren Merendino

Chief Commercial Officer

Commercial performance by the numbers

$87.7M

OJEMDA Net Product Revenue Since Launch[1]

$30.5M

(+11%) QoQ[2,3]

Q1 2025 OJEMDA Net Product Revenue

2,571

Cumulative Prescriptions Since Launch[1,4]

[1] OJEMDA received U.S. FDA accelerated approval for relapsed or refractory BRAF-altered pediatric low-grade glioma on April 23, 2024. [2] Q4 2024 revenue included approximately $1.4M of revenue associated with Ex-U.S. sales which has been removed for the purposes of calculating quarter over quarter recurring revenue growth. [3] QoQ represents the comparison of Q1 2025 to Q4 2024. [4] Prescriptions are approximations based on data available as of March 31, 2025.

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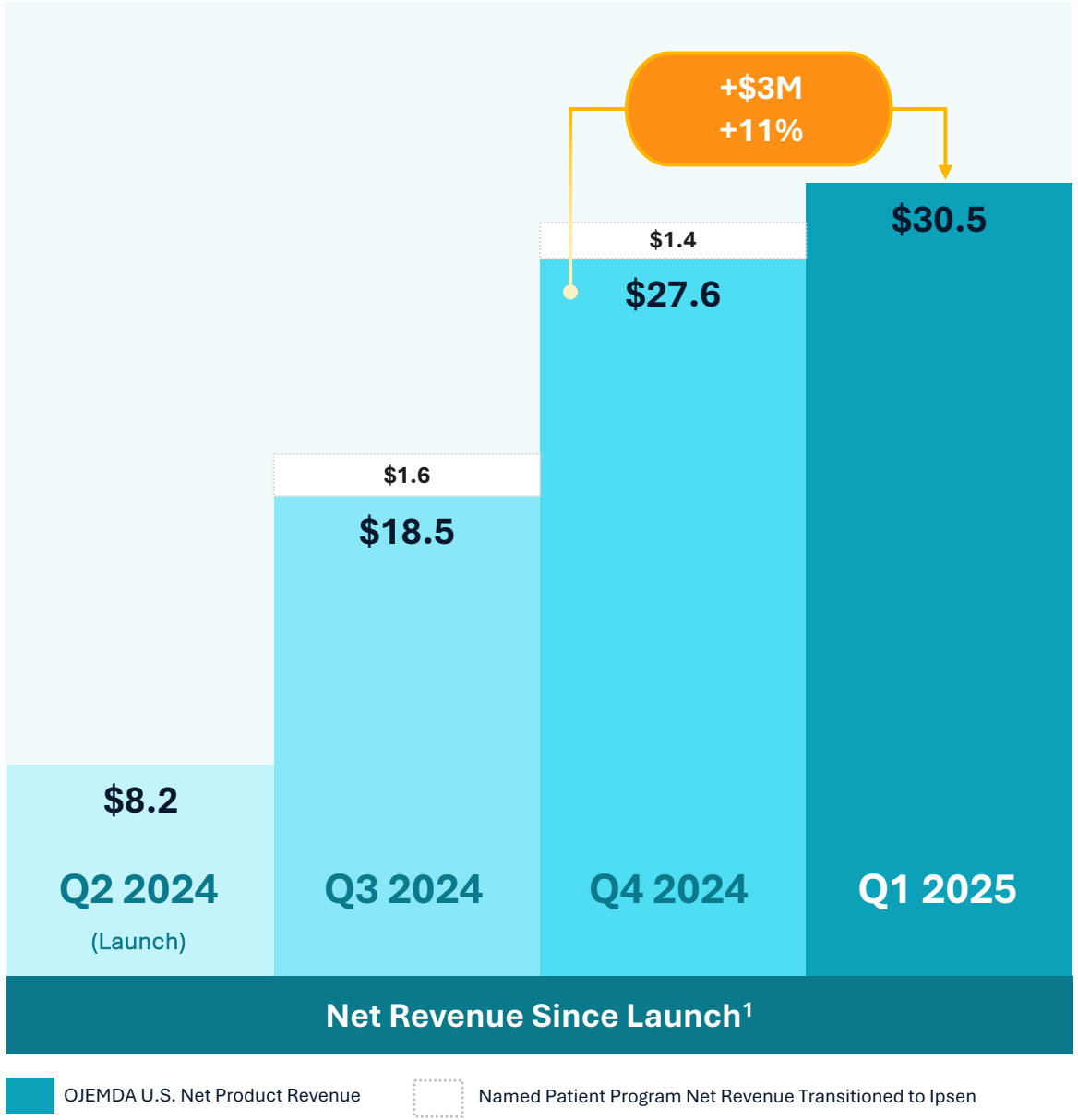
Continued OJEMDA revenue growth in Q1 2025

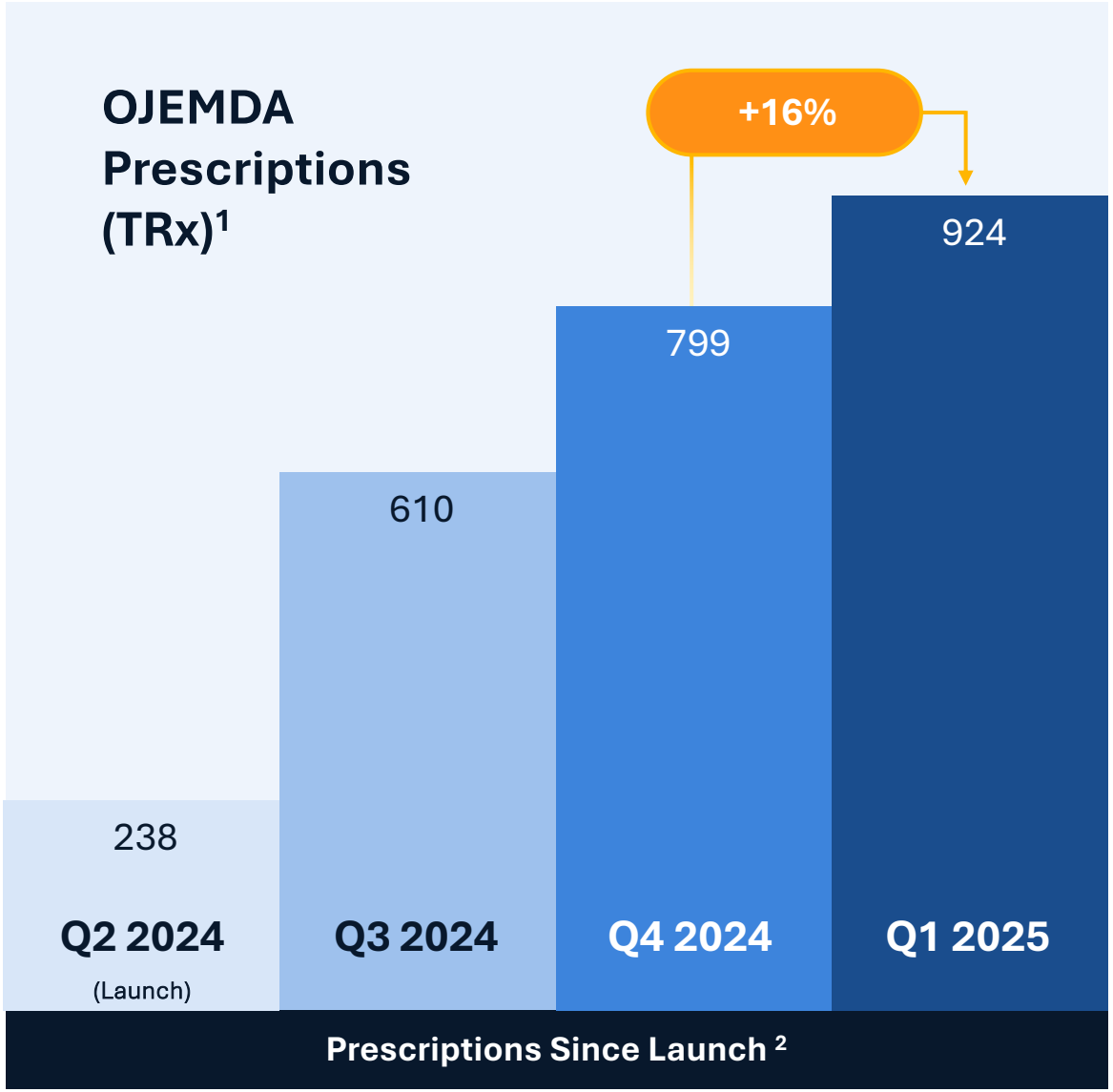
Achieved $87.7M in OJEMDA net product revenue since launch[1]

Net Revenue Highlights

- Achieved $30.5M in Q1 2025 OJEMDA net product revenue
- Represents $3.0M (+11%) U.S. net product revenue growth over Q4 2024[2]



[1]OJEMDA received U.S. FDA accelerated approval for relapsed or refractory BRAF-altered pediatric low-grade glioma on April 23, 2024. [2]Q4 2024 revenue included approximately $1.4M of revenue associated with Ex-US sales which has been removed for the purposes of calculating quarter over quarter recurring revenue growth.



Strong commercial execution led to continued OJEMDA prescription growth in Q1 2025

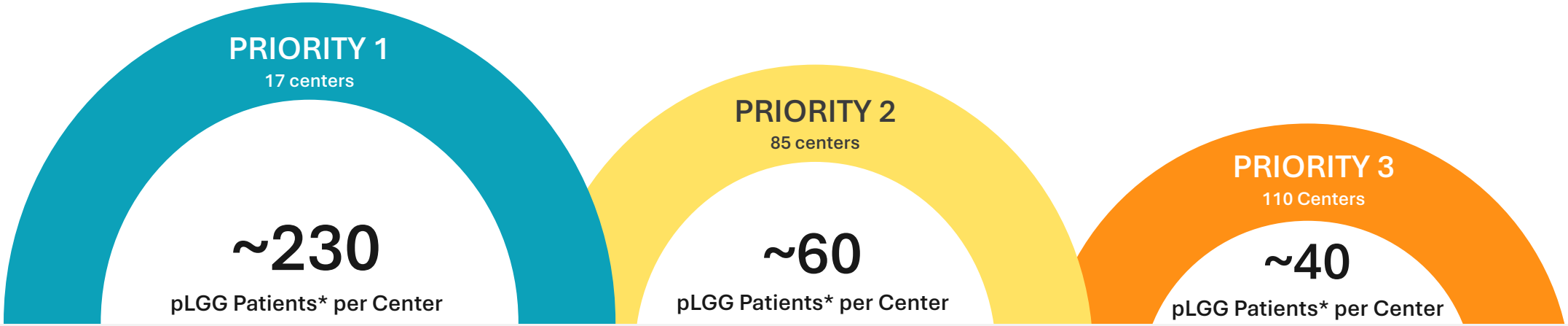
Achieved
2,571 cumulative
scripts since launch

Prescription Highlights

- Growth was driven by new patient starts and a high percentage of on-label patients continuing on therapy each month
- Continued prescriber adoption accompanied by growing breadth & depth

[1]Prescriptions are approximations based on data available as of March 31, 2025. [2] OJEMDA received U.S. FDA accelerated approval for relapsed or refractory BRAF-altered pediatric low-grade glioma on April 23, 2024.

OJEMDA has significant patient opportunity across priority centers



~4,000	**Estimated total patients managed by priority 1 centers**
~5,000	**Estimated total patients managed by priority 2 centers**
~4,000	**Estimated total patients managed by priority 3 centers**

Estimated patients managed by priority account is calculated by taking the prevalence of pLGG systemically-treated patients under 25 years (~26,000), less the 50% (~13,000) estimated to be in long-term remission and not likely to need an additional systemic therapy, and then broken down by the estimated patients managed within each priority (priority 1 = 30%, priority 2 = 40%, priority 3 = 30%). Estimated patients per center is calculated by taking the estimated patients managed within each priority and divided by the number of centers within each priority. Estimated patients managed by priority is rounded to nearest 1,000 and estimated patients per center is rounded to nearest 10. *Relapsed or refractory pLGG BRAF altered patient who has received a systemic treatment.

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OJEMDA priorities to drive revenue growth in 2025

- Drive depth of prescribing with current prescribers
- Encourage non-user HCPs to try OJEMDA in their next r/r pLGG patient
- Establish OJEMDA as standard of care in 2nd line relapsed or refractory BRAF-altered pLGG
- Support prescribers and patients to allow for optimal duration of treatment

r/r, relapsed or refractory.

Financial Performance

Charles York

Chief Operating Officer &
Chief Financial Officer

First quarter 2025 financial results

Financial Summary *($ in millions)*	Three Months Ended 3/31/25	Three Months Ended 3/31/24
OJEMDA Net Revenue	30.5	--
License Revenue	0.3	--
Total Revenue	**$30.8**	**$--**
Cost of Product Revenue	2.9	--
Research and Development Expense[1]	39.6	40.2
Selling, General and Administrative Expense[2]	29.3	26.6
Total Cost and Operating Expenses	**$71.8**	**$66.8**
Non-operating Income	5.0	4.4
Income Tax Expense	--	--
Net Loss	**($36.0)**	**($62.4)**

	3/31/25	12/31/24
Cash, cash equivalents and short-term investments	$473.0	$531.7

All financial information is unaudited. [1] Includes stock-based compensation expense of $4.3 million for the three months ended 3/31/25, and $4.7 million for the three months ended 3/31/24. [2] Includes stock-based compensation expense of $8.6 million for the three months ended 3/31/25, and $8.0 million for the three months ended 3/31/24.

Thank You

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