Third Quarter 2026 Financial Results

November 2, 2026

Forward-Looking Statements

This presentation and the associated conference call and webcast contain forward-looking statements about the business prospects of BioMarin Pharmaceutical Inc. (BioMarin), including, without limitation, statements about: future financial performance, including the expectations of Revenues and Non-GAAP Diluted EPS for, in certain instances, the full-year 2026 and fourth quarter of 2026, and future periods, and the underlying drivers of those results, such as the expected demand and continued growth of BioMarin's Metabolic Conditions portfolio, including PALYNZIQ, GALAFOLD and POMBILITI + OPFOLDA, and VOXZOGO, and the expected impact of the acquisition of Alesta Therapeutics B.V. (Alesta) and the BMN 450 license agreement; the anticipated benefits of the acquisition of Amicus Therapeutics, Inc., including the expected amount and timing of cost synergies as well as expected revenue from the addition of GALAFOLD and POMBILITI + OPFOLDA; the anticipated benefits of the acquisition of Alesta; the anticipated benefits of the BMN 450 license agreement; the anticipated benefits of the global settlement with Ascendis Pharma A/S, including BioMarin's expected receipt of royalty payments on net sales of Yuviwel in the United States, the European Union, Brazil and South Korea; BioMarin's plans for investment in innovation and future growth; the timing of orders for commercial products; plans and expectations regarding the development, commercialization and commercial prospects of BioMarin's product candidates and commercial products, including the prospects and timing of actions relating to clinical studies and trials and product approvals, such as study initiations, study advancements, data readouts, submissions, filings, approvals, and label expansions; the expected benefits and availability of BioMarin's commercial products and product candidates, including with respect to the potential new indication for VOXZOGO in hypochondroplasia; and potential growth opportunities and trends, including the assumptions and expectations regarding total addressable patient population (TAPP) with respect to the conditions targeted by BioMarin's product candidates and commercial products. These risks and uncertainties include, among others, those factors detailed in BioMarin's press release issued on November 2, 2026, and BioMarin's filings with the Securities and Exchange Commission, including, without limitation, the factors contained under the caption "Risk Factors" in BioMarin's Quarterly Report on Form 10-Q for the quarter ended June 30, 2026, as such factors may be updated by any subsequent reports. Investors are urged not to place undue reliance on forward-looking statements, which speak only as of the date hereof. BioMarin is under no obligation, and expressly disclaims any obligation to update or alter any forward-looking statement, whether as a result of new information, future events or otherwise.

Non-GAAP Financial Measures

This presentation includes both GAAP information and Non-GAAP information. Non-GAAP Income is defined by the company as GAAP Net Income (Loss) excluding amortization, stock-based compensation expense and, in certain periods, certain other specified items, as detailed below when applicable. The company also includes a Non-GAAP adjustment for the estimated tax impact of the reconciling items. Non-GAAP Cost of Sales (COS), Non-GAAP Research and Development (R&D) expenses and Non-GAAP Selling, General and Administrative (SG&A) expenses are defined by the company as GAAP COS, GAAP R&D expenses and GAAP SG&A expenses, respectively, excluding stock-based compensation expense and, in certain periods, certain other specified items, as detailed below when applicable. Non-GAAP Operating Margin percentage is defined by the company as GAAP Income (Loss) from Operations, excluding amortization of intangible assets, stock-based compensation expense and, in certain periods, certain other specified items, divided by GAAP Total Revenues. Non-GAAP Diluted EPS is defined by the company as Non-GAAP Income divided by Non-GAAP Weighted-Average Diluted Shares Outstanding. Non-GAAP Weighted-Average Diluted Shares Outstanding is defined by the company as GAAP Weighted-Average Diluted Shares Outstanding, adjusted to include any common shares issuable under the company's equity plans or convertible debt in periods when they are dilutive under Non-GAAP. Non-GAAP synergies is defined by the company as Amicus' legacy labor and external spend cost reductions, excluding the impact of stock-based compensation. BioMarin regularly uses both GAAP and Non-GAAP results and expectations internally to assess its financial operating performance and evaluate key business decisions related to its principal business activities: the discovery, development, manufacture, marketing and sale of innovative biologic therapies. Because such Non-GAAP metrics are important internal measurements for BioMarin, BioMarin believes that providing this information in conjunction with GAAP information enhances investors' and analysts' ability to meaningfully compare the company's results from period to period and to its forward-looking guidance, and to identify operating trends in the company's principal business. Non-GAAP financial measures are not meant to be considered in isolation or as a substitute for, or superior to comparable GAAP measures and should be read in conjunction with the consolidated financial information prepared in accordance with GAAP. Investors should note that the Non-GAAP information is not prepared under any comprehensive set of accounting rules or principles and does not reflect all of the amounts associated with the company's results of operations as determined in accordance with GAAP. Investors should also note that these Non-GAAP financial measures have no standardized meaning prescribed by GAAP and, therefore, have limits in their usefulness to investors. In addition, from time to time in the future there may be other items that the company may exclude for purposes of its Non-GAAP financial measures; likewise, the company may in the future cease to exclude items that it has historically excluded for purposes of its Non-GAAP financial measures. Because of the non-standardized definitions, the Non-GAAP financial measure as used by BioMarin in this presentation may be calculated differently from, and therefore may not be directly comparable to, similarly titled measures used by other companies. BioMarin does not provide guidance for GAAP reported financial measures (other than revenue) or a reconciliation of forward-looking Non-GAAP financial measures to the most directly comparable GAAP reported financial measures because the company is unable to predict with reasonable certainty the financial impact of changes resulting from its strategic portfolio and business operating model reviews; potential future asset impairments; gains and losses on investments; and other unusual gains and losses without unreasonable effort. These items are uncertain, depend on various factors, and could have a material impact on GAAP reported results for the guidance period. As such, any reconciliations provided would imply a degree of precision that could be confusing or misleading to investors. With respect to historical Non-GAAP adjusted financial information, see the appendix beginning on slide 19 for the reconciliations to the comparable information reported under U.S. GAAP.

Q3’26 Financial Results Agenda:

1. Key Business Updates
2. Commercial Update
3. Research & Development Update
4. Financial Results
5. Q&A



Alexander Hardy
Chief Executive Officer



Cristin Hubbard
Chief Commercial Officer



Greg Friberg
Chief R&D Officer



Brian Mueller
Chief Financial Officer

Q3’26 Key Business Updates



Alexander Hardy
Chief Executive Officer

Q3: Execution Across the Business while Investing in the Future

STRONG Q3'26 PERFORMANCE

$1.025B ▲ 32%Y/Y

Total Revenue Growth;
First $1B+ Revenue Quarter

- 44% Y/Y growth across Metabolic Conditions, led by strong GALAFOLD, POMBILITI + OPFOLDA and VIMIZIM contributions
- 12% Y/Y growth of VOXZOGO; driven by international expansion and U.S. starts under age 2; large order timing shifted from Q3 to Q4
- Record top line and disciplined spend drove significant Non-GAAP Diluted EPS[1] growth from underlying operations

AMICUS VALUE CREATION

+13% GALAFOLD

+69% POMBILITI + OPFOLDA

Q3'26 Patient Growth, Y/Y

- Seamless commercial integration continues
- Q3 revenue of $152M for GALAFOLD and $49M for POMBILITI + OPFOLDA
- Expanding diagnosis and treatment for GALAFOLD and driving switching for POMBILITI + OPFOLDA
- Cost synergies realization tracking ahead of expectations in 2026; significant cost synergies[1,2] expected to be fully realized in 2028

BUILDING PIPELINE MOMENTUM

4 New Pipeline Assets in 2026; Data and Approvals Expected Across Pipeline

- In 2026: Added BMN 450 for Fabry, BMN 440 for hypophosphatasia (HPP), BMN 430 for ReNU syndrome, and BMN 820 for focal segmental glomerulosclerosis (FSGS)[3]
- VOXZOGO for achondroplasia (ACH) full approval PDUFA action date: February 2027
- VOXZOGO for hypochondroplasia (HCH) PDUFA action date: May 2027
- BMN 351 update expected by YE; BMN 333 and BMN 440 data anticipated in 2027

[1]Refer to slide 2 for more detail on Non-GAAP financial measures; [2]Represents approximately $280 million of cost reductions on a GAAP basis, and approximately $220 million of cost reductions on a Non-GAAP basis; [3]BMN 450 (HM15421/GC1134A), BMN 440 (ALE1), BMN 820 (DMX-200)

Commercial Update



Cristin Hubbard
Chief Commercial Officer

Strong GALAFOLD and POMBILITI + OPFOLDA Patient Growth in Q3

Galafold® (migalastat)

- **+13%** Y/Y patient growth
- **$152M** Q3 revenue
- **Expanded diagnosis and treatment**, with deeper penetration across established and newer markets
- **In international markets,** increased KOL engagement and family cascade testing contributed to patient growth

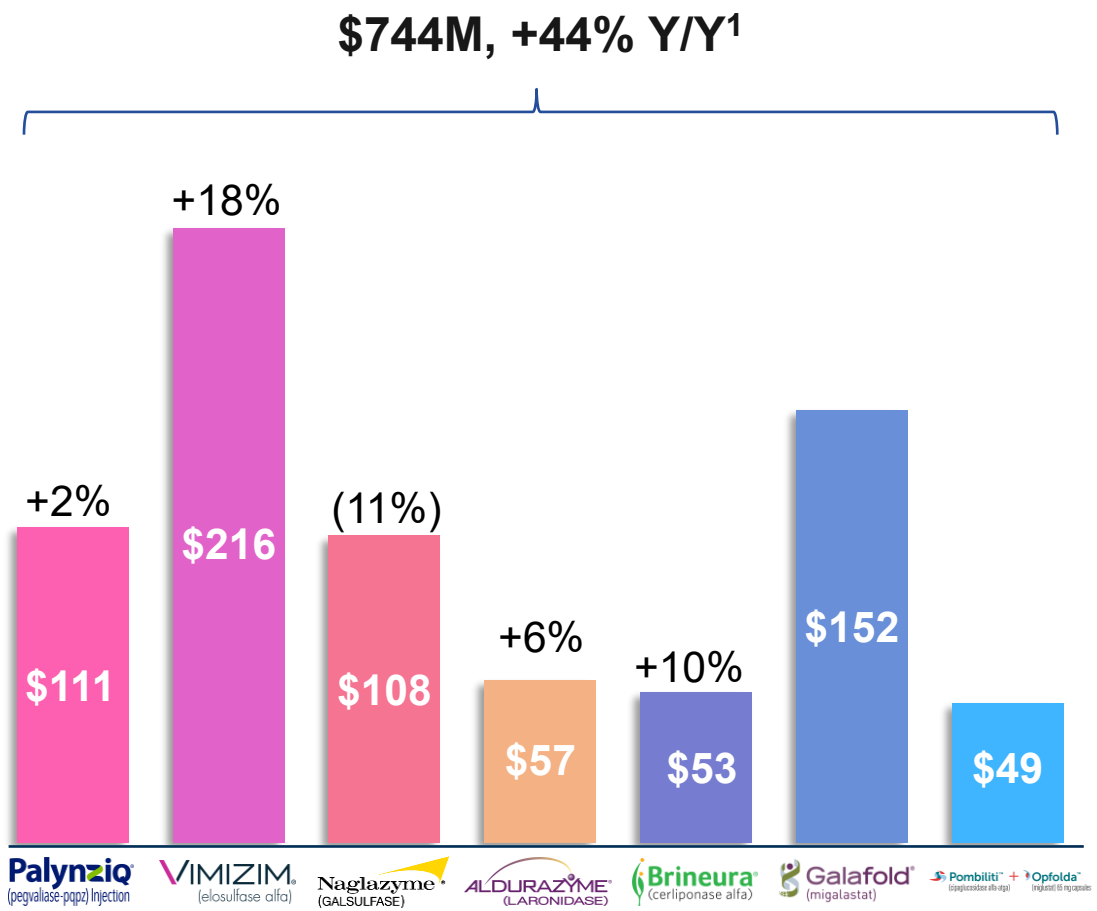
Pombiliti™ (cipaglucosidase alfa-atga) + Opfolda™ (miglustat) 65 mg capsules

- **+69%** Y/Y patient growth
- **$49M** Q3 revenue
- **Patient switching supported by a differentiated clinical profile** and growing clinical and real-world evidence, including five-year PROPEL data
- **Underlying growth complemented** by new patient starts due to supply constraints for other Pompe therapies

Metabolic Conditions: Diversified Portfolio Drove Record Results

Metabolic Conditions Q3'26 Revenue

In millions, except percentages



Q3'26 Dynamics

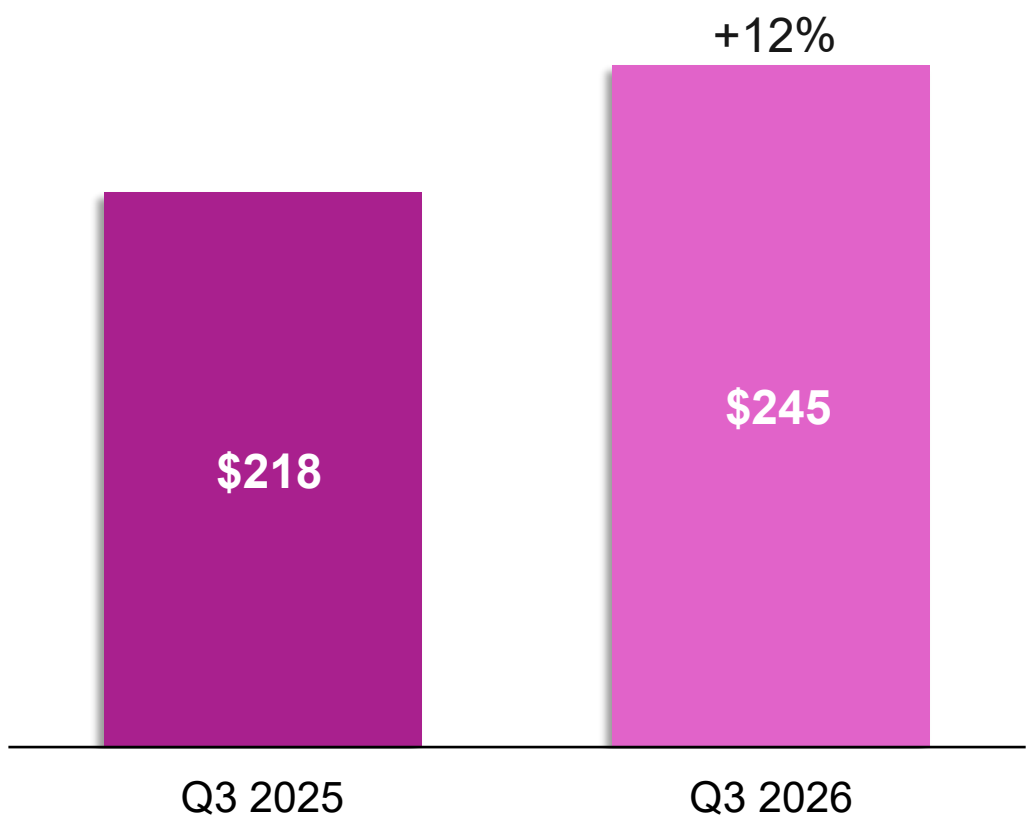
- **+44%** Y/Y revenue growth, driven largely by the first full quarter of GALAFOLD and POMBILITI + OPFOLDA
- Patients on therapy increased across all BioMarin-marketed Metabolic Conditions products, Y/Y
- The number of patients on PALYNZIQ increased 9% Y/Y, led by adolescent launch in U.S.
- VIMIZIM revenue increased 18% and NAGLAZYME decreased (11%) Y/Y, reflecting international order timing

[1]Year-over-year revenue comparisons are not available for GALAFOLD and POMBILITI + OPFOLDA because the prior-year periods occurred before BioMarin's acquisition of Amicus Therapeutics

Skeletal Conditions: Global Growth and Continued Leadership

VOXZOGO® Revenue

In millions, except percentages



Q3'26 Dynamics

- +12% Y/Y revenue growth; ~17% increase in the number of children treated globally Y/Y
- > 75% of revenue generated outside of the U.S.
- Large international order previously expected in Q3 now anticipated in Q4
- Children < 2 y/o represented majority of U.S. patient starts

HCH Launch Preparations

- ~14,000 total addressable patient population (TAPP)
- Focused on sharing strong evidence, increasing diagnosis and patient identification, and laying a foundation for rapid access
- HCH diagnosis to benefit from recent international consensus recommendations published in August 2026 in Nature Reviews Endocrinology[1]

Percentages reflect year-over-year change; [1]Dauber, A., Cheung, M.S., Hoover-Fong, J. et al. Diagnosis of hypochondroplasia: international Delphi consensus recommendations. Nat Rev Endocrinol (2026). https://doi.org/10.1038/s41574-026-01296-z

Research & Development Update



Greg Friberg
Chief R&D Officer

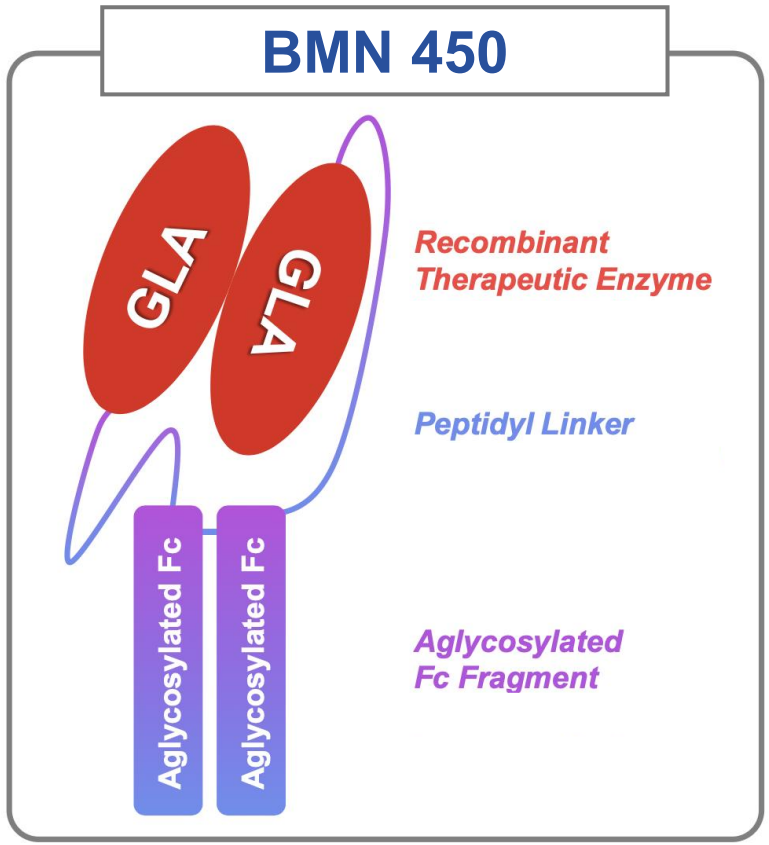
Pipeline Momentum: Four New Assets in 2026, Multiple Catalysts Ahead

Program	Preclinical	Phase 1	Phase 2	Phase 3	Key Highlights
VOXZOGO Hypochondroplasia					• sNDA filing accepted • PDUFA action date: May 30, 2027
VOXZOGO Idiopathic Short Stature					• Phase 2 data expected in 2027
BMN 333 Achondroplasia					• Phase 2/3 registration-enabling study enrolling; Phase 2 data expected in 2027
★ **BMN 440 (ALE1)** Hypophosphatasia (HPP)					• Phase 1/2a ongoing in healthy volunteers and adults with HPP • PK/PD data expected in 2027
POMBILITI + OPFOLDA Late-onset Pompe disease (LOPD)					• sNDA filing accepted (for adolescents) • PDUFA action date: Jan. 10, 2027
★ **BMN 450 (HM15421/GC1134A)** Fabry disease					• Phase 1/2 study ongoing
★ **BMN 820 (DMX-200)** Focal Segmental Glomerulosclerosis (FSGS)					• Phase 3 endpoint agreed with FDA • Data expected in 2028
BMN 351 Duchenne Muscular Dystrophy					• Phase 1/2 enrollment completed • Program update expected by year-end
★ **BMN 430** ReNU syndrome					• Pre-clinical studies underway • Identifying lead ASO candidate

Skeletal Conditions Metabolic Conditions Pipeline ★ New assets added in 2026

BMN 450: Potential First Subcutaneous ERT for Fabry Disease

Long-acting enzyme replacement therapy designed for subcutaneous dosing



Target Product Profile

- Human recombinant α-Gal A designed to extend half-life and improve stability relative to currently available ERTs
- Subcutaneous dosing could reduce treatment burden for patients and physicians
- Potential to treat all ERT-eligible Fabry patients

Pipeline Highlights

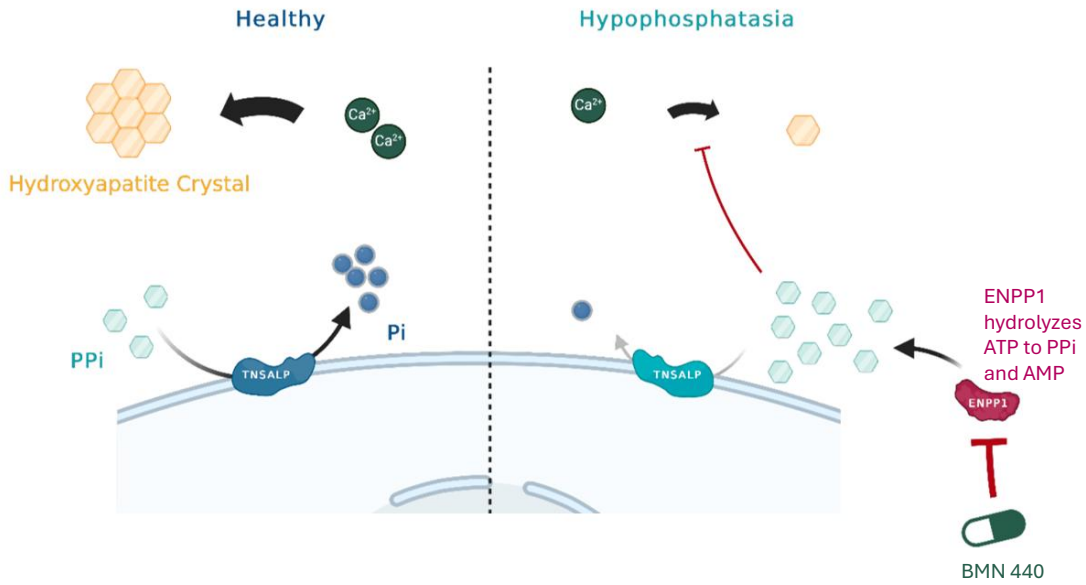
- Global Phase 1/2 study ongoing; FDA Orphan Drug Designation
- Preclinical data show differentiated efficacy in renal function, vascular health and peripheral nervous system outcomes

BMN 450 has the potential to expand the Fabry market with the addition of a therapy amenable to more patients

BMN 440 for Hypophosphatasia: Differentiated Mechanism of Action

Directly inhibits ENPP1 upstream of PPi, offering a substrate reduction approach for HPP

Deficient alkaline phosphatase activity causes PPi accumulation, impairing bone mineralization and contributing to musculoskeletal pain and weakness



Target Product Profile

- Oral small molecule targeting ENPP1 to reduce PPi at its source
- Potential alternative to ERT for HPP patients (both pediatric + adult-onset)
- Potential to address systemic symptoms beyond bone, including muscle weakness, pain and fatigue

Pipeline Highlights

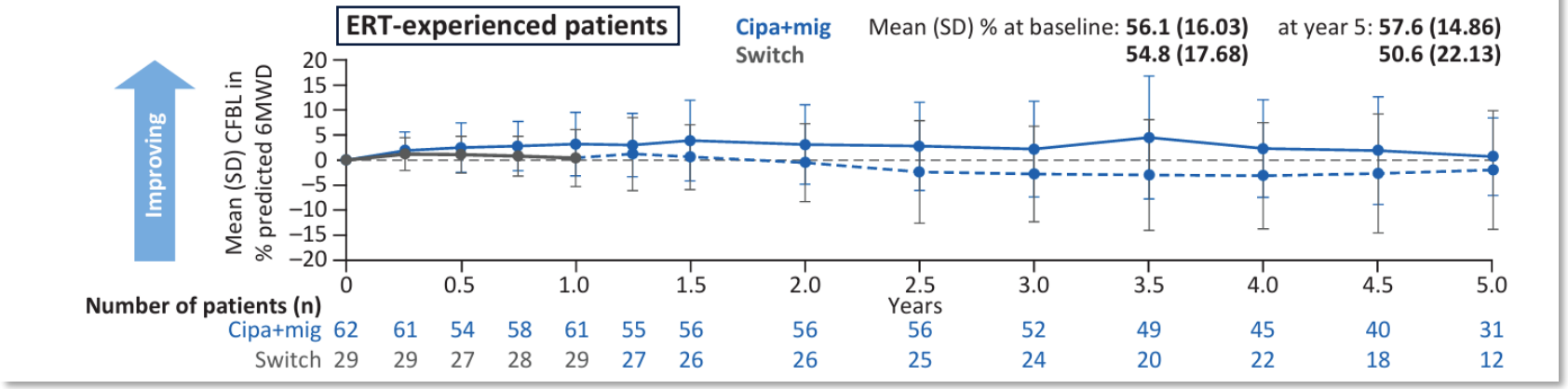
- Phase 1/2a ongoing in healthy volunteers and adults with HPP
- Data readout expected in 2027

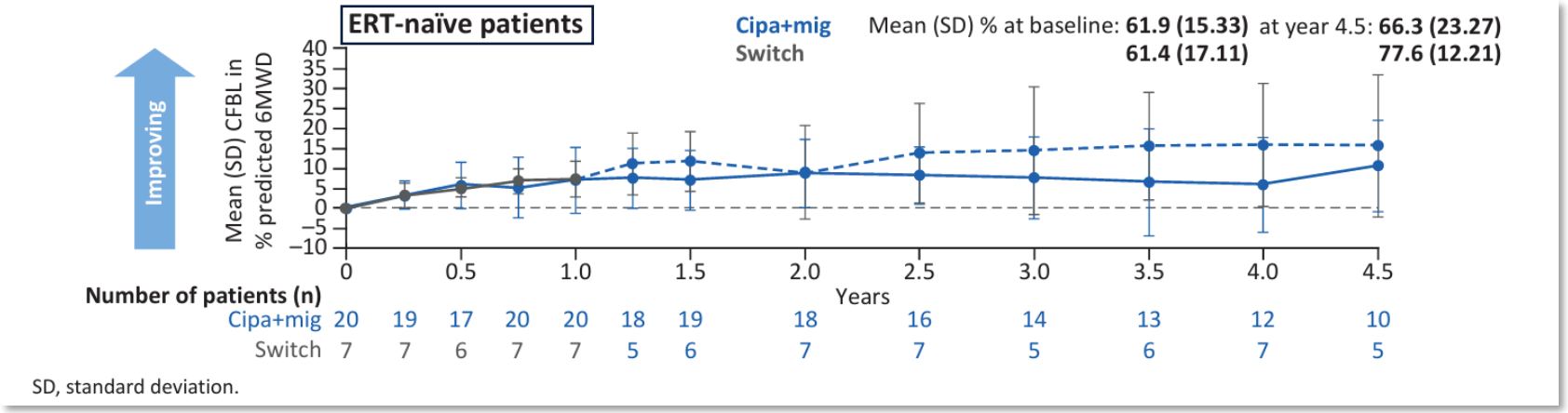
BMN 440 has the potential to be the first oral therapy for HPP, including adults who are largely untreated today

POMBILITI + OPFOLDA Demonstrates Durable Outcomes Through Five Years in LOPD

Long-term durability across motor function, pulmonary function, biomarker response, and safety

In % predicted 6MWD, ERT-experienced patients experienced stability to year 5, while ERT-naïve patients experienced sustained improvements to year 4.5





SD, standard deviation.

Cipa+mig Switch

Key Highlights

Biomarker improvement: Sustained serum CK reductions in both ERT-experienced and ERT-naïve patients.

Long-term pulmonary stability: % predicted sitting FVC remained relatively stable in ERT-experienced and ERT-naïve patients.

Consistent long-term safety through Year 5, consistent with published 2-year data.

Roberts M, et al. Long-term (5-year) efficacy and safety of cipaglucosidase alfa plus miglustat in adults with late-onset Pompe disease treated from PROPEL baseline. Poster presented at: World Muscle Society (WMS) Annual Congress; September 29–October 3, 2026; Hiroshima, Japan.

Q3’26 Financial Results



Brian Mueller
Chief Financial Officer

Q3 Financial Highlights

Q3 2026 Results

In millions, except percentages and per share amounts	Q3'26	Y/Y Change
Total Revenues	$1,025	+32%
GAAP R&D	$181	(4%)
Acquired IPR&D	$288	+30%
GAAP SG&A	$334	+24%
GAAP Operating Margin	(8.0%)	(2.0) ppts
Non-GAAP R&D[1]	$167	(4%)
Non-GAAP SG&A[1]	$249	+12%
Non-GAAP Operating Margin[1]	13.4%	+10.6 ppts
GAAP Diluted EPS	($0.63)	+294%
Non-GAAP Diluted EPS[1]	$0.23	+92%
Interest Income	$5	(74%)
Interest Expense	($56)	N.M.[2]

Total Revenues

- $1,025M, +32% Y/Y, primarily driven by addition of GALAFOLD and POMBILITI + OPFOLDA
- Royalty and Other Revenues now include royalties from Ascendis

Operating Expenses

- Acquired IPR&D expenses are primarily related to Alesta acquisition
- Non-GAAP SG&A increased Y/Y, driven by addition of sales and marketing spend from Amicus to support the growth of GALAFOLD and POMBILITI + OPFOLDA

Non-GAAP Diluted EPS

- $0.23 EPS, +92% Y/Y, primarily driven by revenue growth, partially offset by higher acquired IPR&D charges and interest expense

[1]Refer to slide 2 for more detail on Non-GAAP financial measures; [2]N.M. = not meaningful

Updated Full-year 2026 Guidance

(In millions, except per share amounts)	Prior Guidance As of August 6, 2026	Updated Guidance As of November 2, 2026	Midpoint Growth (Y/Y)
Total Revenues	**$3,875 to $3,925**	**$3,800 to $3,900**	**↑ 20%**
Metabolic Conditions	**$2,725 to $2,775**	**$2,675 to $2,750**	**↑ 29%**
VOXZOGO	**$1,000 to $1,050**	**$975 to $1,025**	**↑ 8%**
Other Revenues[1]	**$100 to $125**	**$125 to $150**	
Non-GAAP Diluted EPS[2,3]	**$4.90 to $5.10**	**$2.80 to $3.10**	**(6%)**

- **Revenue:** Total Revenues, Metabolic Conditions, and VOXZOGO guidance updated to reflect uncertainty in the timing of multiple orders from a single international customer.
- **Non-GAAP Diluted EPS:** BioMarin expects the Alesta acquisition and BMN 450 license agreement to result in total pre-tax charges of approximately $320 million and $83 million, respectively, for the full year 2026, consisting of the acquired IPR&D charges and ongoing operating expenses, representing an unfavorable impact of approximately $1.55 and $0.32, respectively, to both GAAP Diluted EPS and Non-GAAP Diluted EPS for the full year 2026.

[1]Other Revenues includes KUVAN, ROCTAVIAN, and royalties. [2]Refer to slide 2 for more detail on Non-GAAP financial measures; [3]Non-GAAP Diluted EPS guidance assumes approximately 200 million Weighted-Average Diluted Shares Outstanding

Q&A

Appendix

Reconciliation of GAAP Reported to Selected Non-GAAP Adjusted Information

Reconciliation of GAAP Reported Net Income (Loss) to Non-GAAP Income[(1)]

(In millions)

	Three Months Ended September 30,		Nine Months Ended September 30,	
	2026	2025	2026	2025
GAAP Reported Net Income (Loss)	**$ (123)**	**$ (31)**	**$ 27**	**$ 395**
Adjustments				
Stock-based compensation expense - COS	4	4	13	11
Stock-based compensation expense - R&D	14	15	41	41
Stock-based compensation expense - SG&A[(2)]	36	30	119	83
Amortization of intangible assets	99	5	177	15
Amortization of acquired inventory step-up - COS[(3)]	18	—	29	—
Acquisition and restructuring-related costs - SG&A[(4)]	47	15	143	15
Loss on investments [(5)]	—	—	—	3
Income tax effect of adjustments	(50)	(16)	(120)	(38)
Non-GAAP Income	$ 45	$ 22	$ 430	$ 525

Please refer to slide 24 for footnotes to the table

Reconciliation of GAAP and Non-GAAP COS, R&D, and SG&A Expenses[1]

(In millions)

	Three Months Ended September 30,						Nine Months Ended September 30,					
	2026			2025			2026			2025		
	COS	R&D	SG&A	COS	R&D	SG&A	COS	R&D	SG&A	COS	R&D	SG&A
GAAP expenses	$ 205	$ 181	$ 334	$ 140	$ 189	$ 268	$ 603	$ 566	$ 987	$ 442	$ 509	$ 707
Adjustments												
Stock-based compensation expense[2]	(4)	(14)	(36)	(4)	(15)	(30)	(13)	(41)	(119)	(11)	(41)	(83)
Amortization of acquired inventory step-up[3]	(18)	—	—	—	—	—	(29)	—	—	—	—	—
Acquisition and restructuring-related costs[4]	—	—	(47)	—	—	(15)	—	—	(143)	—	—	(15)
Non-GAAP expenses	$ 184	$ 167	$ 249	$ 136	$ 174	$ 223	$ 561	$ 525	$ 724	$ 431	$ 468	$ 609

Please refer to slide 24 for footnotes to the table

Reconciliation of GAAP Income (Loss) from Operations to Non-GAAP Income from Operations[1]

(In millions, except percentages)

	Three Months Ended September 30,				Nine Months Ended September 30,			
	2026	Percent of GAAP Total Revenue	2025	Percent of GAAP Total Revenue	2026	Percent of GAAP Total Revenue	2025	Percent of GAAP Total Revenue
GAAP Income (Loss) from Operations	**$ (82)**	**(8.0)%**	**$ (47)**	**(6.0)%**	**$ 158**	**5.7 %**	**$ 454**	**19.4 %**
Adjustments								
Stock-based compensation expense[2]	54	5.3	49	6.3	173	6.2	135	5.8
Amortization of intangible assets	99	9.7	5	0.6	177	6.4	15	0.6
Amortization of acquired inventory step-up[3]	18	1.8	—	—	29	1.0	—	—
Acquisition and restructuring-related costs[4]	48	4.7	15	1.9	143	5.1	15	0.6
Non-GAAP Income from Operations	$ 137	13.4 %	$ 22	2.8 %	$ 683	24.6 %	$ 618	26.3 %

Please refer to slide 24 for footnotes to the table

Reconciliation of GAAP Diluted EPS to Non-GAAP Diluted EPS[1]

(In U.S. dollars)

	Three Months Ended September 30,		Nine Months Ended September 30,	
	2026	2025	2026	2025
GAAP Diluted EPS	**$ (0.63)**	**$ (0.16)**	**$ 0.14**	**$ 2.04**
Adjustments				
Stock-based compensation expense[2]	$ 0.28	$ 0.25	0.87	0.69
Amortization of intangible assets	$ 0.51	$ 0.03	0.89	0.08
Amortization of acquired inventory step-up[3]	$ 0.09	$ —	0.15	—
Acquisition and restructuring-related costs[4]	$ 0.24	$ 0.08	0.72	0.08
Loss on investments [5]	$ —	$ —	—	0.02
Income tax effect of adjustments	$ (0.26)	$ (0.08)	(0.60)	(0.19)
Non-GAAP Diluted EPS	$ 0.23	$ 0.12	$ 2.18	$ 2.69

Please refer to slide 24 for footnotes to the table

Reconciliation of GAAP Weighted-Average Diluted Shares Outstanding to Non-GAAP Weighted-Average Diluted Shares Outstanding[1]

(In millions)

	Three Months Ended September 30,		Nine Months Ended September 30,	
	2026	2025	2026	2025
GAAP Weighted-Average Diluted Shares Outstanding	**193.6**	**192.0**	**195.2**	**196.9**
Adjustments[6]				
Common stock issuable under the company's equity plans	2.5	0.8	—	—
Common stock issuable under the company's convertible debt	—	—	4.4	—
Non-GAAP Weighted-Average Diluted Shares Outstanding	196.0	192.8	199.6	196.9

(1) Certain amounts may not sum or recalculate due to rounding.
(2) Stock-based compensation expense recorded in SG&A for the nine months ended September 30, 2026, includes approximately $13 million related to the post-combination service period for unvested Amicus stock options.
(3) Represents amortization of the inventory step-up for the inventory acquired in the Amicus transaction.
(4) The 2026 amounts represent severance, transaction and integration costs resulting from the Amicus acquisition, as well as restructuring costs incurred in connection with corporate initiatives. The 2025 amounts represent severance costs incurred in connection with the acquisition of Inozyme Pharma, Inc.
(5) Represents impairment loss on non-marketable equity securities recorded in Other income, net, in the first quarter of 2025.
(6) Common stock issuable under the company's equity plans and convertible debt were excluded from the computation of GAAP and Non-GAAP Weighted-Average Diluted Shares Outstanding in the periods they were anti-dilutive.