BioMarin to Acquire Amicus Therapeutics

- Expands BioMarin’s position as a leader in rare diseases
- Creates value from Day 1

December 19, 2025

Forward Looking Statement

This presentation and the associated conference call and webcast contain forward-looking statements about, among other things, the proposed acquisition of Amicus Therapeutics (Amicus) by BioMarin Pharmaceutical Inc. (BioMarin) and the business prospects of Amicus and BioMarin, including, without limitation, statements about: the anticipated occurrence, manner and timing of the closing of the proposed acquisition and the availability of financing, including the proposed non-convertible debt financing; the prospective benefits of the proposed acquisition, including expectations that it will accelerate BioMarin's revenue growth and strengthen its financial outlook immediately upon close, increase BioMarin's long-term CAGR through 2030 and beyond, strengthen and diversify BioMarin's commercial portfolio and be a strong strategic fit for BioMarin by expanding BioMarin's rare disease product portfolio, create substantial shareholder value by being accretive to non-GAAP diluted earnings per share in the first year after close and substantially accretive beginning in 2027; BioMarin's commitment to deleveraging and the expectation that BioMarin will reach gross leverage <2.5x within two years after close; expectations regarding Amicus' products, Galafold and Pombiliti + Opfolda, including ability to expand access to patients in new markets across BioMarin's global footprint and potential label expansion opportunities; BioMarin's capital allocation strategy to leverage its financial strength to diversify its pipeline and add innovative new therapies for patients; the potential impact of the acquisition on BioMarin's financial results and financial guidance; BioMarin's plans for external innovation, including BioMarin's ability to execute additional transactions in future quarters; statements about BioMarin's future financial performance; and other statements that are not historical facts. Actual results could differ materially from those anticipated in these forward-looking statements. Except as required by law, each of BioMarin and Amicus assume no obligation to update these forward-looking statements, whether as a result of new information, future events or otherwise. These statements, which represent each of BioMarin's and Amicus' current expectations or beliefs concerning various future events that are subject to significant risks and uncertainties, may contain words such as "may," "will," "would," "could," "expect," "anticipate," "intend," "plan," "believe," "estimate," "project," "seek," "should," "strategy," "future," "opportunity," "potential" or other similar words and expressions indicating future results. These forward-looking statements are predictions and involve risks and uncertainties such that actual results may differ materially from these statements. Forward-looking statements reflect current beliefs and expectations; however, these statements involve inherent risks and uncertainties, including, without limitation, with respect to: consummating the proposed acquisition and financing in the anticipated timeframe, if at all; whether Amicus' stockholders will approve the acquisition; the possibility that competing offers or acquisition proposals will be made; the possibility that various closing conditions for the transaction may not be satisfied or waived, including that a governmental entity may prohibit, delay, or refuse to grant approval for the consummation of the transaction (or only grant approval subject to adverse conditions or limitations); the difficulty of predicting the timing or outcome of regulatory approvals or actions, if any; the effects of the proposed acquisition (or the announcement thereof) on Amicus' or BioMarin's stock price and/or Amicus' or BioMarin's operating results; unknown or inestimable liabilities; the development, launch and commercialization of products and product candidates; the parties' ability to realize the anticipated benefits of the proposed acquisition, including the possibility that the expected benefits from the proposed acquisition will not be realized or will not be realized within the expected time period and that BioMarin and Amicus will not be integrated successfully or that such integration may be more difficult, time-consuming or costly than expected; obtaining and maintaining adequate coverage and reimbursement for BioMarin's or Amicus' products; the time-consuming and uncertain regulatory approval process; the costly and time-consuming pharmaceutical product development process and the uncertainty of clinical success, including risks related to failure or delays in successfully initiating or completing clinical trials and assessing patients, including with respect to current and planned future clinical trials; global economic, financial, and healthcare system disruptions and the current and potential future negative impacts to BioMarin's or Amicus' business operations and financial results; the sufficiency of BioMarin's or Amicus' cash flows and capital resources; BioMarin's ability to fund the acquisition, including BioMarin's ability to obtain financing on terms satisfactory to BioMarin or at all; BioMarin's evaluation of the potential impact of the transaction on its financial results and financial guidance; BioMarin's or Amicus' ability to achieve targeted or expected future financial performance and results and the uncertainty of future tax, accounting and other provisions and estimates; the effects of the transaction on relationships with key third parties, including employees, customers, suppliers, other business partners or governmental entities, including the risk that the proposed acquisition adversely affects employee retention; transaction costs; risks that the proposed acquisition disrupts current plans and operations; risks that the proposed transaction diverts management's attention from ongoing business operations; changes in Amicus' business during the period between announcement and closing of the proposed acquisition; any legal proceedings and/or regulatory actions that may be instituted related to the proposed acquisition; and other risks and uncertainties affecting BioMarin and Amicus, including those risk factors detailed in BioMarin's and Amicus' filings with the Securities and Exchange Commission (SEC), including, without limitation, the risk factors contained under the caption "Risk Factors" in BioMarin's Quarterly Report on Form 10-Q for the fiscal quarter ended September 30, 2025 and Amicus' Quarterly Report on Form 10-Q for the fiscal quarter ended September 30, 2025, as such risk factors may be updated by any subsequent reports, as well as the Proxy Statement on Schedule 14A to be filed by Amicus. Stockholders of BioMarin and Amicus are urged not to place undue reliance on forward-looking statements, which speak only as of the date hereof. BioMarin and Amicus are under no obligation, and expressly disclaim any obligation, to update (publicly or otherwise) or alter any forward-looking statement, including without limitation any financial projection or guidance, whether as a result of new information, future events or otherwise.

Agenda

- BioMarin and Amicus: An Ideal Fit
- Overview of Financial Terms
- Expansion and Diversification of BioMarin's Core Enzyme Business
- Reaching More Patients Globally who can Benefit from Innovative Therapies

Galafold® (migalastat) 123 mg capsule

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

Q&A with BioMarin Team:

Alexander Hardy
Chief Executive Officer

Brian Mueller
Chief Financial Officer

Cristin Hubbard
Chief Commercial Officer

Greg Friberg
Chief R&D Officer

A Strategic Fit that Accelerates BioMarin's Growth Trajectory

BIOMARIN® + Amicus Therapeutics

Shared Focus on Rare Disease

- Shared commitment to prioritize finding and treating patients with rare diseases
- BioMarin's global scale and manufacturing capabilities will enable more patients to benefit from Galafold® and Pombiliti® + Opfolda®
- Executes on capital allocation strategy to maximize and diversify value creating growth drivers

Accelerates & Diversifies Revenue

- Adds two marketed, high-growth products with multiple global expansion opportunities
- Expected to increase BioMarin's long-term revenue CAGR through 2030 and beyond and diversifies revenue mix

Substantially Accretive Beginning in 2027

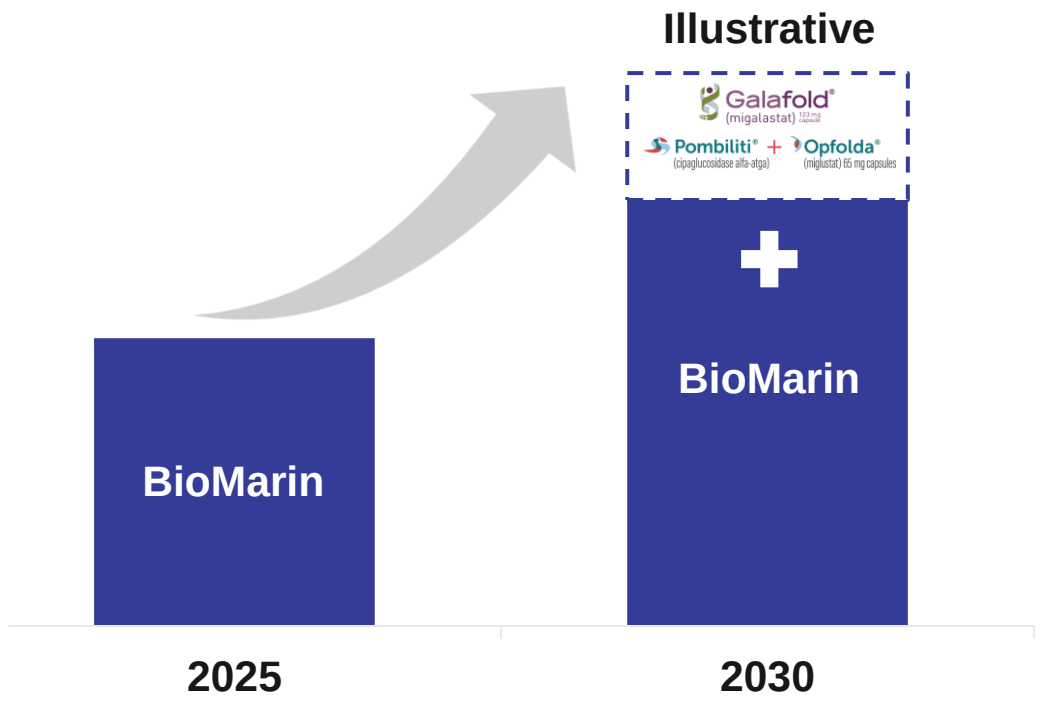
- Expected to be accretive to Non-GAAP Diluted Earnings Per Share (EPS) in first 12 months after close and substantially accretive beginning in 2027
- Increases long-term profitability and cash flow and drives efficiencies that align with margin expansion priorities
- Commitment to deleveraging, targeting gross leverage < 2.5x within two years after close

Attractive Transaction Terms and Leverage Profile

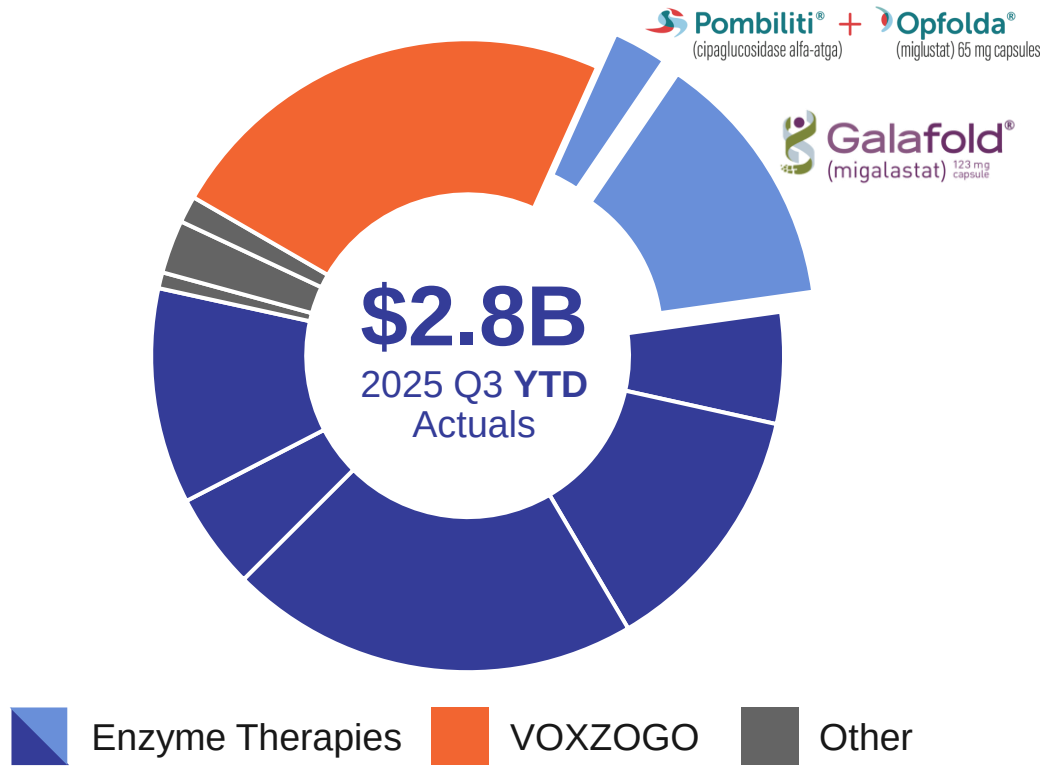
Purchase Price	Funding & Capital Impact	Approvals & Timing
$14.50 per share BioMarin has entered into a definitive agreement to acquire Amicus Therapeutics in an all-cash transaction	**$4.8B Equity Value** Transaction to be financed through a combination of cash on hand and ~$3.7 billion of non-convertible debt financing	**BOD Approved** Transaction has been unanimously approved by the Boards of Directors of both companies
33% Premium to Amicus' closing stock price on 12/18/2025	**Targeting < 2.5x Gross Leverage within Two Years after Close** Strong cash flow enables deleveraging	**Q2 2026** Expected close, subject to regulatory clearance, approval by the stockholders of Amicus and other customary closing conditions

Two New Therapies Expected to Accelerate and Diversify Revenues

Accelerates Total BioMarin Revenues



Expands and Diversifies BioMarin's Revenue Streams



Products and Indications Fit Seamlessly into BioMarin's Enzyme Therapies Portfolio

	Galafold® (migalastat) 123 mg capsule	Pombiliti® (cipaglucosidase alfa-atga) + Opfolda® (miglustat) 65 mg capsules
Indication	• **Fabry disease:** A rare disease caused by mutation in galactosidase alpha gene (GLA) and deficiency of α-Gal A enzyme. Without fully functioning α-Gal A, fatty waste substances build up in the body, causing damage to tissues and organs. • **Galafold** is indicated for individuals with Fabry disease and an amenable GLA variant	• **Late-onset Pompe Disease (LOPD):** Rare, inherited genetic disorder caused by mutation in GAA gene and deficiency of α-glucosidase (GAA) enzyme. Symptoms include systemic muscle weakness that worsens over time. • **Pombiliti + Opfolda** is indicated for adults with late-onset Pompe disease (LOPD)[1]
Mechanism of Action	• Oral pharmacological chaperone – binds to active sites of amenable mutant forms of a-Gal A to stabilize them and prevent misfolding	• Pombiliti – GAA enzyme replacement • Opfolda – Oral pharmacological chaperone, binds with and stabilizes Pombiliti, reducing its inactivation in the bloodstream
Diagnosed Population	• Fabry disease: > 18,000	• Late-onset Pompe disease: ~5,000-10,000
Date of Approval	• EMA Approval – 2016 • FDA Approval – 2018	• EMA Approval – 2023 • FDA Approval – 2023
Overlap with BioMarin Call Points	• Neurology • Medical Geneticists	• Neurology • Medical Geneticists
Key Advantages	✓ Oral convenience and safety profile are the major driver of uptake in milder patient population ✓ Life-long therapy with limited discontinuation	✓ Two-component therapy is attractive ✓ No concerns on safety & tolerability

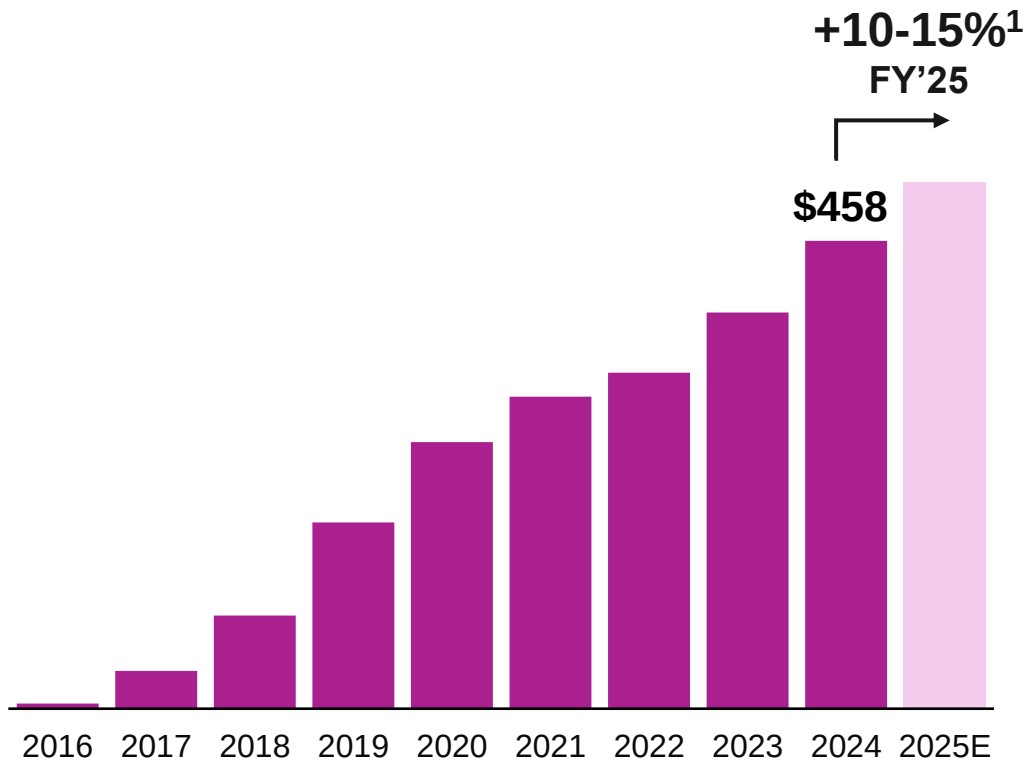
[1] For adults weighing 88 lbs or more who are not improving on their current enzyme replacement therapy

Galafold is the First and Only Oral Therapy for Fabry Disease

Galafold® (migalastat) 123 mg capsule for Fabry Disease

- **Strong strategic fit** within BioMarin's Enzyme Therapies business unit
- **Approved in more than 40 countries,** including the U.S., EU, UK, and Japan
- **Opportunity** to **deepen reach in existing markets** and **expand into multiple additional countries** with BioMarin's global footprint and capabilities
- Approved age groups:
  - U.S.: ≥18 years
  - EU, UK, Canada: ≥12 years
  - Japan: ≥16 years

Galafold® (migalastat) 123 mg capsule Annual Revenue ($M)



[1]FY'25 guidance as of Amicus Q3'25 earnings call on November 4, 2025; constant exchange rates

Pending U.S. Galafold Intellectual Property Litigation Resolved

All Pending U.S. Galafold IP Litigation Resolved

- Amicus entered into license agreements with Aurobindo and Lupin for Galafold 123mg capsules
- Amicus will grant Aurobindo and Lupin licenses to market generic versions of Galafold in the U.S. beginning on January 30, 2037
- In accordance with the agreements, parties will terminate all ongoing litigation between Amicus and Aurobindo and Lupin regarding U.S. Galafold patents
- Litigation with Teva previously settled in October 2024 (with license to market beginning on January 30, 2037)

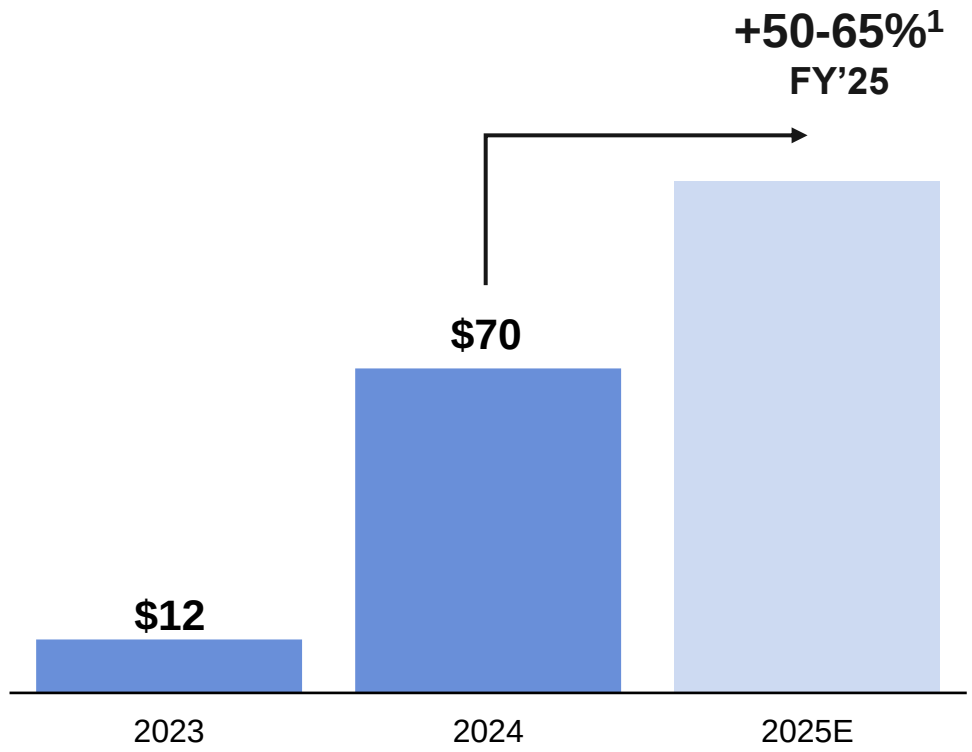
Resolution of IP litigation supports **growth outlook for Galafold**

Pombiliti + Opfolda is the Only Two-Component Therapy for the Treatment of Pompe Disease

Pombiliti® (cipaglucosidase alfa-atga) + Opfolda® (miglustat) 65 mg capsules **for Pompe Disease**

- **Reimbursed in 15 countries** with 10 countries added in 2025, opening access to **~2,400** people with Pompe
- **Approved in adults** with LOPD
- **Potential label expansion** opportunities:
  - Phase 3 study (ZIP) ongoing in children < 18 y/o with LOPD
    - **Mid-2026:** Potential approval for 12 to 17 y/o
    - Completing enrollment in < 12 y/o cohort
- Phase 3 study (ROSELLA) enrolling children < 18 y/o with Infantile-onset Pompe disease (IOPD)

Pombiliti® (cipaglucosidase alfa-atga) + Opfolda® (miglustat) 65 mg capsules **Annual Revenue ($M)**



[1]FY'25 guidance as of Amicus Q3'25 earnings call on November 4, 2025; constant exchange rates

Transforming BioMarin For The Next Phase of Growth

Expands BioMarin's position as a leader in rare diseases with addition of two, high-growth products with numerous expansion opportunities, including geographies, age label, indications

Opportunity to find and treat more patients with Fabry disease and Pompe disease by leveraging BioMarin's global footprint, manufacturing capabilities and experience finding and treating patients with rare diseases

Immediate revenue contributions increasing long-term CAGR and diversifying revenue mix

Anticipated to be accretive to Non-GAAP Diluted EPS in first 12 months after close and substantially accretive beginning in 2027

Strong cash flow generation – commitment to rapid deleveraging, targeting gross leverage < 2.5x within two years after transaction close

Accelerates revenue and profitability, and strengthens outlook

Q&A