BioMarin Strengthens Enzyme Therapy Business with Acquisition of Inozyme Pharma

May 16, 2025

Acquisition is Strong Strategic Fit for BioMarin, Adding INZ-701, a Phase 3 Enzyme Replacement Therapy Being Developed for Treatment of ENPP1 Deficiency

First Pivotal Data Readout in Children Expected in Early 2026 with Potential Launch in 2027; Additional Clinical Programs to Expand to Patients of all Ages

Potential First-in-Disease Treatment for ENPP1 Deficiency

Conference Call and Webcast Scheduled Today at 8:45 a.m. ET

SAN RAFAEL, Calif. and BOSTON, May 16, 2025 /PRNewswire/ -- BioMarin Pharmaceutical Inc. (Nasdaq: BMRN) and Inozyme Pharma, Inc. (Nasdaq: INZY) announced today that BioMarin has entered into a definitive agreement to acquire Inozyme for $4.00 per share in an all-cash transaction for a total consideration of approximately $270 million. The transaction has been unanimously approved by the Boards of Directors of both companies and is expected to close in the third quarter of 2025, subject to regulatory approval, successful completion of a tender offer and other customary closing conditions.

The acquisition will strengthen BioMarin's enzyme therapies portfolio, adding a late-stage enzyme replacement therapy, INZ-701, which is currently being assessed for the treatment of ectonucleotide pyrophosphatase/phosphodiesterase 1 (ENPP1) Deficiency, a rare, serious and progressive genetic condition that affects blood vessels, soft tissues and bones. The condition is associated with increased cardiovascular mortality risk across all age groups, especially in infants. It is also associated with severe rickets and osteomalacia in children and adults. Data from the first Phase 3 pivotal study of INZ-701 in children is expected in early 2026, with potential regulatory approval in 2027.

"BioMarin has been deeply committed to advancing enzyme therapies for children and adults living with serious genetic conditions for more than 25 years, and today's agreement builds on our legacy," said Alexander Hardy, President and Chief Executive Officer of BioMarin. "This acquisition brings to BioMarin an important medicine that has the potential to be the first treatment for children and adults with ENPP1 Deficiency, improving care for people living with this serious condition. As BioMarin continues our transformation and delivers on our corporate strategy, we will continue to evaluate external innovation alongside internal innovation. We are in a strong financial position to bring in additional assets as we accelerate the development of medicines for patients with significant unmet need."

"Today's announcement gives greater hope to patients who may benefit from INZ-701, a potentially transformative therapy that aims to address the underlying causes and systemic impacts of ENPP1 Deficiency," said Douglas A. Treco, Ph.D., Chief Executive Officer and Chairman of Inozyme. "BioMarin has paved the way over the past two and a half decades, successfully launching five first-in-disease enzyme therapies. I'd like to thank the team at Inozyme and our partners for their outstanding work and dedication, as we pass this important potentially life-changing therapy to the leading innovator in genetically defined conditions."

INZ-701 and ENPP1 Deficiency
INZ-701 is a potential first-in-class, subcutaneous enzyme replacement therapy that is being developed for infants, pediatric and adult patients with ENPP1 Deficiency across a continuum of phenotypes. In addition to the ongoing Phase 3 pivotal study in children, Inozyme is currently enrolling infants in a pivotal study, and a supportive study for adolescents and adults is being planned.

In a Phase 1/2 study of adults living with ENPP1 Deficiency, INZ-701 demonstrated a favorable safety profile, with no serious adverse events attributed to INZ-701. Improvements in pyrophosphate levels, bone mineralization biomarkers and quality of life were all observed, suggesting prospect for benefit in patients.

ENPP1 Deficiency is a lifelong, rare, progressive, multisystemic condition, caused by mutations in the *ENPP1* gene, leading to loss of ENPP1 enzymatic activity that results in low pyrophosphate, upregulation of fibroblast growth factor-23 and phosphate wasting. The condition affects blood vessels, soft tissues and bones. It is associated with high risk of cardiovascular mortality in patients of all ages, especially infants. It is also associated with severe rickets and osteomalacia in children and adults. Patients require considerable multidisciplinary lifelong medical and surgical management of complications. Currently there are no approved therapies for ENPP1 Deficiency.

Terms of the Transaction
Under the terms of the merger agreement, BioMarin will promptly commence a cash tender offer to acquire all of the outstanding shares of Inozyme common stock at a price of $4.00 per share. Inozyme's Board of Directors unanimously recommends that Inozyme's stockholders tender their shares in the tender offer.

The consummation of the tender offer is subject to customary closing conditions, including the tender of at least a majority of the outstanding shares of Inozyme, the expiration or termination of the waiting period under the Hart-Scott-Rodino Antitrust Improvements Act of 1976, and other customary conditions. Following the successful completion of the tender offer, a wholly owned subsidiary of BioMarin will merge with Inozyme and the outstanding Inozyme shares not tendered in the tender offer will be converted into the right to receive the same $4.00 per share in cash paid in the tender offer. The transaction is not subject to any financing condition.

BioMarin also today reaffirmed previously provided full-year 2025 financial guidance, excluding the impact of the accounting treatment of this transaction in accordance with Generally Accepted Accounting Principles (GAAP) upon closing, as well as its plan to achieve 40% Non-GAAP Operating Margin in 2026.

Advisors
Goldman Sachs & Co. LLC is acting as exclusive financial advisor to BioMarin, and Cooley LLP is serving as legal counsel. Centerview Partners LLC is acting as exclusive financial advisor to Inozyme, and Goodwin Procter LLP is serving as legal counsel.

Conference Call
BioMarin will host a conference call and webcast to discuss the acquisition today, May 16, 2025, at 8:45 a.m. ET. This event can be accessed through

this link or on the investor section of the BioMarin website at www.biomarin.com.

U.S./Canada Dial-in Number: 888-596-4144 Replay Dial-in Number: 800-770-2030

International Dial-in Number: 646-968-2525 Replay International Dial-in Number: 609-800-9909

Conference ID: 2239224 Conference ID: 2239224

About BioMarin
BioMarin is a global biotechnology company dedicated to translating the promise of genetic discovery into medicines that make a profound impact on the life of each patient. The San Rafael, California-based company, founded in 1997, has a proven track record of innovation with eight commercial therapies and a strong clinical and preclinical pipeline. Using a distinctive approach to drug discovery and development, BioMarin seeks to unleash the full potential of genetic science by pursuing category-defining medicines that offer new possibilities for people living with genetically defined conditions around the world.

To learn more, please visit www.biomarin.com.

About Inozyme
Inozyme Pharma is a clinical-stage biopharmaceutical company, with approximately 50 employees based in Boston. The company is dedicated to developing innovative therapeutics that target the PPi-Adenosine Pathway, a key regulator of bone health and blood vessel function. Disruptions in this pathway underlie a range of severe diseases, including ENPP1 Deficiency. Our lead investigational therapy, INZ-701, is an ENPP1 Fc fusion protein enzyme replacement therapy designed to restore pyrophosphate and adenosine levels. INZ-701 is currently in late-stage clinical development in ENPP1 Deficiency, with the potential to expand into additional indications where deficiencies in the Pyrophosphate-Adenosine Pathway contribute to disease pathology, including ABCC6 Deficiency and calciphylaxis. Through our pioneering work, we aim to transform treatment options for patients affected by these devastating conditions.

To learn more, please visit www.inozyme.com.

Forward-Looking Non-GAAP Financial Information
As described above, today BioMarin reaffirmed its plan to achieve 40% Non-GAAP Operating Margin in 2026. 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 BioMarin 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.

Non-GAAP Information
Non-GAAP Operating Margin percentage is defined by BioMarin as GAAP Income from Operations, excluding amortization of intangible assets, stock-based compensation expense and, in certain periods, certain other specified items, divided by GAAP Total Revenues.

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, manufacturing, marketing and sale of innovative biologic therapies. Because Non-GAAP Operating Margin percentage is an important internal measurement for BioMarin, BioMarin believes that providing this information in conjunction with BioMarin's GAAP information enhances investors' and analysts' ability to meaningfully compare BioMarin's results from period to period and to its forward-looking guidance, and to identify operating trends in BioMarin'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 BioMarin'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 BioMarin may exclude for purposes of its Non-GAAP financial measures; likewise, BioMarin 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 press release may be calculated differently from, and therefore may not be directly comparable to, similarly titled measures used by other companies.

Forward-Looking Statements
This press release and the associated conference call contain forward-looking statements about, among other things, the proposed acquisition of Inozyme Pharma, Inc. (Inozyme) by BioMarin Pharmaceutical Inc. (BioMarin) and the business prospects of Inozyme and BioMarin, including, without limitation, statements about: the anticipated occurrence, manner and timing of the proposed tender offer and the closing of the proposed acquisition; the prospective benefits of the proposed acquisition, including expectations that it will strengthen BioMarin's enzyme therapies portfolio and be a strong strategic fit for BioMarin; Inozyme's product candidate INZ-701 and expectations regarding its ongoing development, including the potential for INZ-701 to be the first treatment for children and adults with ENPP1 Deficiency, the potential benefits of INZ-701 for patients, the anticipated timing for data from the first Phase 3 pivotal study of INZ-701 in children, the anticipated costs of developing INZ-701 and the potential regulatory approval of INZ-701 in 2027; potential revenue for INZ-701; additional INZ-701 clinical programs intended to expand to patients of all ages; the anticipated market for INZ-701; plans for an INZ-701 pivotal study for adolescents and adults; INZ-701's potential to expand into additional indications where deficiencies in the Pyrophosphate-Adenosine Pathway contribute to disease pathology; the accounting treatment of the potential acquisition under GAAP and its potential impact on BioMarin's financial results and financial guidance; BioMarin's plans for external innovation, including BioMarin being in a strong financial position to acquire additional assets; BioMarin's ability to execute additional transactions in future quarters; statements about BioMarin's future financial performance, including the expectations of Non-GAAP Operating Margin percentage; 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 Inozyme 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 Inozyme's 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 in the anticipated timeframe, if at all; how many of Inozyme's stockholders will tender their stock in the tender offer; 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 Inozyme's or BioMarin's stock price and/or BioMarin's or Inozyme's operating results; unknown or inestimable liabilities; the development, launch and commercialization of products and product candidates such as INZ-701, if approved; the successful completion of regulatory activities with respect to INZ-701; 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 Inozyme 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 Inozyme's 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 of INZ-701; global economic, financial, and healthcare system disruptions and the current and potential future negative impacts to BioMarin's or Inozyme's business operations and financial results; the sufficiency of BioMarin's or Inozyme's cash flows and capital resources; BioMarin's ability to fund the acquisition with existing cash and investments; BioMarin's evaluation of the accounting treatment of the potential acquisition and its potential impact on its financial results and financial guidance; BioMarin's or Inozyme's 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 Inozyme's 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 Inozyme, including those risk factors detailed in BioMarin's and Inozyme's 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 March 31, 2025 and Inozyme's Quarterly Report on Form 10-Q for the fiscal quarter ended March 31, 2025, as such risk factors may be updated by any subsequent reports, as well as the Tender Offer Statement on Schedule TO and related tender offer documents to be filed by BioMarin and its acquisition subsidiary, Incline Merger Sub, Inc., and the Solicitation/Recommendation Statement on Schedule 14D-9 to be filed by Inozyme. Stockholders of BioMarin and Inozyme are urged not to place undue reliance on forward-looking statements, which speak only as of the date hereof. BioMarin and Inozyme 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.

Additional Information about the Acquisition and Where to Find It

The tender offer for all of the outstanding shares of Inozyme described in this communication has not yet commenced. This communication is for informational purposes only and is neither an offer to purchase nor a solicitation of an offer to sell any securities, nor is it a substitute for the tender offer materials that Inozyme, BioMarin or its acquisition subsidiary will file with the SEC upon commencement of the tender offer. The solicitation and offer to tender and the offer to buy outstanding shares of Inozyme will only be made pursuant to a tender offer statement on Schedule TO, including an Offer to Purchase and related tender offer materials that BioMarin and its acquisition subsidiary, Incline Merger Sub, Inc., are expected to file with the SEC. At the time the tender offer is commenced, BioMarin and its acquisition subsidiary will file a Tender Offer Statement on Schedule TO, and Inozyme will file a Solicitation/Recommendation Statement on Schedule 14D-9 with the SEC with respect to the tender offer. THE TENDER OFFER MATERIALS (INCLUDING AN OFFER TO PURCHASE, A RELATED LETTER OF TRANSMITTAL AND CERTAIN OTHER TENDER OFFER DOCUMENTS), AS WELL AS THE SOLICITATION/RECOMMENDATION STATEMENT ON SCHEDULE 14D-9, WILL CONTAIN IMPORTANT INFORMATION ABOUT THE PROPOSED ACQUISITION AND THE PARTIES THERETO. INVESTORS AND STOCKHOLDERS OF INOZYME ARE URGED TO READ THESE DOCUMENTS CAREFULLY WHEN THEY BECOME AVAILABLE (AND EACH AS IT MAY BE AMENDED OR SUPPLEMENTED FROM TIME TO TIME) BECAUSE THEY WILL CONTAIN IMPORTANT INFORMATION THAT INVESTORS AND STOCKHOLDERS OF INOZYME SHOULD CONSIDER BEFORE MAKING ANY DECISION WITH RESPECT TO THE TENDER OFFER. The tender offer materials (including the Offer to Purchase, the related Letter of Transmittal and certain other tender offer documents), as well as the Solicitation/Recommendation Statement, will be made available to all investors and stockholders of Inozyme at no expense to them at SEC's website at www.sec.gov. Copies of the documents filed with the SEC by BioMarin will be available free of charge on BioMarin's website at www.biomarin.com. Copies of the documents filed with the SEC by Inozyme will be available free of charge on Inozyme's website, www.inozyme.com, or by contacting Inozyme's investor relations department at investorrelations@inozyme.com. The information contained in, or that can be accessed through, BioMarin's and Inozyme's websites is not a part of, or incorporated by reference herein. In addition to the Offer to Purchase, related Letter of Transmittal and certain other tender offer documents, and Solicitation/Recommendation Statement, BioMarin and Inozyme file annual, quarterly, and current reports, proxy statements and other information with the SEC. You may read any reports, statements or other information filed by BioMarin and Inozyme with the SEC for free on the SEC's website at www.sec.gov.

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