BioMarin to Acquire Alesta Therapeutics to Gain ALE1, a Potential First Oral Therapy for Hypophosphatasia, Adding an Important Clinical Program to BioMarin's Pipeline

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Visit the Alesta Therapeutics website

BioMarin to pay $275 million upfront, plus additional payments upon achievement of development and regulatory milestones

Alesta to spin out all non-ALE1 assets to a new entity and Alesta employees to transfer to the spinout entity prior to close

ALE1 has significant potential to help BioMarin expand into larger rare disease markets with a medicine intended to address a high unmet need for patients and offers strong strategic fit for the company

SAN RAFAEL, Calif., Aug. 18, 2026 – BioMarin Pharmaceutical Inc. (Nasdaq: BMRN) announced today that BioMarin has entered into a definitive agreement to acquire Alesta Therapeutics to gain Alesta's lead clinical-stage asset, ALE1. Alesta plans to spin out all non-ALE1 assets prior to the close of the transaction, which has been approved by the boards of directors of both companies and is expected to be completed this quarter, subject to customary closing conditions.

ALE1 is an orally active, small molecule for the potential treatment of hypophosphatasia (HPP), a rare genetic bone disease caused by mutations in the ALPL gene. ALE1 has the potential to be the first oral therapy for HPP and is currently being evaluated in an ongoing Phase 1/2a clinical trial assessing safety, tolerability and pharmacokinetics/pharmacodynamics in healthy volunteers and adults with HPP.

HPP is a serious condition that can affect bone and tooth mineralization, resulting in easy or frequent bone breaks, early tooth loss, and, in adults, clinically significant muscle weakness, fatigue and pain. If approved, ALE1 is expected to be the first oral therapy approach that targets the central disease metabolite, PPi (inorganic pyrophosphate), with the potential to impact both skeletal and broader manifestations of HPP through systemic correction of disease biology. The program will become part of BioMarin's Skeletal Conditions Business Unit following close.

"ALE1 is a strong strategic fit for BioMarin, bringing a potential oral alternative to the injectable therapies available today for people living with HPP around the world while meaningfully strengthening our early-stage clinical pipeline," said Alexander Hardy, President and Chief Executive Officer of BioMarin. "This is exactly the kind of opportunity to address a significant unmet need that lets us compete in larger rare disease markets – adding an asset that has the potential to reach our largest addressable patient population. We plan to continue to seek these kinds of opportunities as we focus on clinical-stage innovation to drive durable growth for BioMarin."

Under the terms of the agreement, BioMarin will acquire Alesta and Alesta shareholders will receive an upfront payment of $275 million plus up to $215 million in subsequent payments upon achievement of certain development and regulatory milestones. Additionally, immediately prior to the close of BioMarin's acquisition of Alesta, Alesta will spin out all non-ALE1 assets to a new entity and former Alesta employees will transfer to the spinout entity. As a result, no Alesta employees will become employees of BioMarin in connection with the transaction. BioMarin intends to fund the transaction with cash on hand. BioMarin expects to provide updated full-year 2026 guidance reflecting the acquisition of Alesta following the closing of the transaction. Excluding the upfront consideration, the transaction is expected to have a modestly dilutive impact on 2026 financial results.

"We chose to partner with BioMarin due to their deep commitment to people living with rare diseases," said Ilan Ganot, Chief Executive Officer of Alesta Therapeutics. "BioMarin's global reach, scale, and proven expertise in rare disease drug development make it an ideal partner to advance ALE1 and realize its potential as a promising treatment for patients with HPP worldwide. This acquisition is also a testament to the extraordinary work, scientific expertise, and drug development capabilities of the Alesta team."

Morgan Stanley & Co. LLC is acting as the exclusive financial advisor to BioMarin, and Jones Day is serving as legal counsel in connection with the acquisition. J.P. Morgan Securities LLC is acting as exclusive financial advisor to Alesta, and Goodwin Procter LLP and NautaDutilh N.V. are serving as legal counsel.

About ALE1

ALE1 is designed to inhibit a novel target that regulates levels of inorganic pyrophosphate (PPi), the metabolite at the center of HPP pathology. By lowering excess PPi, ALE1 aims to restore healthier bone and mineral metabolism across the full spectrum of HPP.

More than 9,000 people have been diagnosed with HPP in the U.S., however, the disease is often underdiagnosed due to a broad spectrum of symptoms that can mimic more common conditions.

About BioMarin

BioMarin is a leading, global rare disease biotechnology company focused on delivering medicines for people living with genetically defined conditions. Founded in 1997, the San Rafael, California-based company has a proven track record of innovation, with nine 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 have a profound impact on patients.

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

About Alesta Therapeutics

Alesta Therapeutics is a clinical-stage biotechnology company committed to developing novel oral

small-molecule therapies for underserved diseases. The company's lead asset, ALE1, is being developed for hypophosphatasia (HPP), a rare genetic disorder with significant unmet need.

For more information, visit www.alestatherapeutics.com.

Forward-Looking Statements

This press release contains forward-looking statements about, among other things, the proposed acquisition of ALE1, the lead clinical-stage asset, of Alesta Therapeutics (Alesta) by BioMarin Pharmaceutical Inc. (BioMarin) and the business prospects of BioMarin, including, without limitation, statements about: the anticipated occurrence, manner, funding and timing of the closing of the proposed acquisition; BioMarin's plans to update financial guidance; the potential impact of the acquisition on BioMarin's financial results and financial guidance; the prospective benefits of the proposed acquisition, including expectations that it will be a strong strategic fit for BioMarin and will meaningfully strengthen BioMarin's early-stage clinical pipeline; expectations regarding ALE1 and its ongoing development, including its potential to be a first-in-class oral therapy for the treatment of hypophosphatasia (HPP) and the potential benefits of ALE1 to patients with HPP around the world; BioMarin's plans to drive durable growth and strengthen its pipeline for the future; BioMarin's ability to compete in larger rare disease markets; BioMarin's expectations regarding unmet need and opportunities in HPP that may potentially be addressed by ALE1, including BioMarin's estimates regarding the prevalence of HPP; and other statements that are not historical facts.

These forward-looking statements are predictions and involve risks and uncertainties such that actual results may differ materially from these statements. These risks and uncertainties include, among others: consummating the proposed acquisition in the anticipated timeframe, if at all; Alesta's ability to complete the contemplated spinout of non-ALE1 assets prior to closing of the proposed acquisition, if at all; 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 BioMarin's stock price and/or BioMarin's operating results; unknown or inestimable liabilities; the development, launch and commercialization of products and product candidates; BioMarin's 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 integration will not be successful or that such integration may be more difficult, time-consuming or costly than expected; the time-consuming and uncertain regulatory approval process for pharmaceutical product development; 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 business operations and financial results; the sufficiency of BioMarin's cash flows and capital resources; BioMarin's ability to fund the acquisition; BioMarin's evaluation of the potential impact of the transaction on its financial results and financial guidance; BioMarin'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; 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 Alesta'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 those factors detailed in 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.

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