Data Demonstrating ENPP1 Inhibition as a Therapeutic Approach for Later-onset Hypophosphatasia Presented at the American Society for Bone and Mineral Research 2024 Annual Meeting

September 30, 2024 at 8:00 AM EDT

NEW HAVEN, Conn.--(BUSINESS WIRE)--Sep. 30, 2024-- Rallybio Corporation (Nasdaq: RLYB), a clinical-stage biotechnology company translating scientific advances into transformative therapies for patients with devastating rare diseases, announced today that nonclinical data demonstrating ENPP1 inhibition as a therapeutic approach for the treatment of patients with hypophosphatasia (HPP) was presented at the American Society for Bone and Mineral Research (ASBMR) 2024 Annual Meeting. ASBMR is being held September 27 – 30, 2024 in Toronto, Canada.

“From my team’s earlier work published in 2002 and 2005, we knew that ENPP1 could be a targetable molecule to modulate TNAP’s all-important substrate PP_i. I am delighted that we have now been able to demonstrate the efficacy of this principle in our mouse model of later-onset HPP,” said José Luis Millán, Ph.D., Professor, Human Genetics Program at Sanford Children’s Health Research Center, Sanford Burnham Prebys Medical Discovery Institute, and author of the study.

HPP is a rare, genetic, metabolic disorder characterized by poor bone mineralization. The disease has a broad spectrum of symptoms and severity ranging from the life-threatening perinatal- and infantile-onset form to the less severe juvenile-onset form that can manifest with frequent bone fractures, significant joint and bone pain, and joint swelling. HPP is caused by loss-of-function mutations in the gene that encodes tissue-nonspecific alkaline phosphatase (TNAP), whose deficiency results in the accumulation of extracellular inorganic pyrophosphate (PP_i), an inhibitor of bone mineralization.

“The data presented at ASBMR supports ENPP1 inhibition as a therapeutic approach for patients with hypophosphatasia,” said Stephen Uden, MD, Chief Executive Officer of Rallybio. “There is significant unmet patient need in HPP, particularly in adults. We believe this data with an early lead ENPP1 inhibitor from our joint venture with Exscientia is very promising and gives us enthusiasm that the development candidate we expect to nominate in the fourth quarter could be a safe and effective treatment to meaningfully improve the lives of patients suffering from HPP.”

This nonclinical study was designed to assess whether ENPP1 could be a druggable target to treat the non-lethal forms of HPP using an early lead ENPP1 inhibitor and the $Alpl^{-/Prx1}$ mouse, which is a model of later-onset HPP.

Results indicate that oral dosing of an early lead ENPP1 inhibitor, REV101, to adult HPP mice lowered PP_i by 30%, leading to improvements in mineralization of long and vertebrate bones. Furthermore, data showed that ENPP1 inhibition was safe and well-tolerated, and, for the first time, showed that ENPP1 is a druggable target for later-onset HPP.

Rallybio and Exscientia plc (Nasdaq: EXAI) are developing an ENPP1 inhibitor with improved properties compared with REV101 as a differentiated therapy to address the unmet need in patients with HPP. Rallybio and Exscientia expect to nominate a development candidate in the fourth quarter of 2024.

Details of yesterday’s poster presentation:

Title: ENPP1 Inhibition as a Therapeutic Approach for Later-onset Hypophosphatasia
Date/Time: September 29, 2024, 2:15pm – 3:45pm EDT
Presenter: Dr. José Luis Millán
Poster Number: Sun-LB 552

The poster will be available in the Publications & Presentations section of Rallybio’s website following the conclusion of the conference.

About Rallybio
Rallybio (NASDAQ: RLYB) is a clinical-stage biotechnology company with a mission to develop and commercialize life-transforming therapies for patients with severe and rare diseases. Rallybio has built a broad pipeline of promising product candidates aimed at addressing diseases with unmet medical need in areas of maternal fetal health, complement dysregulation, hematology, and metabolic disorders. The Company has two clinical stage programs: RLYB212, an anti-HPA-1a antibody for the prevention of fetal and neonatal alloimmune thrombocytopenia (FNAIT) and RLYB116, an inhibitor of complement component 5 (C5), with the potential to treat several diseases of complement dysregulation, as well as additional programs in preclinical development. Rallybio is headquartered in New Haven, Connecticut. For more information, please visit www.rallybio.com and follow us on LinkedIn and Twitter.

Forward-Looking Statements
This press release contains forward-looking statements that are based on our management’s beliefs and assumptions and on currently available information. All statements, other than statements of historical facts contained in this press release are forward-looking statements. In some cases, forward-looking statements can be identified by terms such as “may,” “will,” “should,” “expect,” “plan,” “anticipate,” “could,” “intend,” “target,” “project,” “contemplate,” “believe,” “estimate,” “predict,” “potential” or “continue” or the negative of these terms or other similar expressions, although not all forward-looking statements contain these words. Forward-looking statements in this press release include, but are not limited to, statements concerning the timing of development candidate nomination for Rallybio’ ENPP1 inhibitor, whether Rallybio’s small molecule would successfully inhibit ENPP1, or demonstrate efficacy in later-onset HPP, or otherwise be safe when administered to humans. The forward-looking statements in this press release are only predictions and are based largely on management’s current expectations and projections about future events and financial trends that management believes may affect Rallybio’s business, financial condition and results of operations. These forward-looking statements speak only as of the date of this press release and are subject to a number of known and unknown risks, uncertainties and assumptions, including, but not limited to, our ability to successfully initiate and conduct our planned clinical trials, including the FNAIT natural history study, and the Phase 2 clinical trial for

RLYB212, and complete such clinical trials and obtain results on our expected timelines, or at all, whether our cash resources will be sufficient to fund our operating expenses and capital expenditure requirements and whether we will be successful raising additional capital, our ability to enter into strategic partnerships or other arrangements, competition from other biotechnology and pharmaceutical companies, and those risks and uncertainties described in Rallybio's filings with the U.S. Securities and Exchange Commission (SEC), including Rallybio's Quarterly Report on Form 10-Q for the period ended June 30, 2024, and subsequent filings with the SEC. The events and circumstances reflected in our forward-looking statements may not be achieved or occur and actual future results, levels of activity, performance and events and circumstances could differ materially from those projected in the forward-looking statements. Except as required by applicable law, we are not obligated to publicly update or revise any forward-looking statements contained in this press release, whether as a result of any new information, future events, changed circumstances or otherwise.

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