Inozyme Pharma Announces Dosing of First Infant with ENPP1 Deficiency in a Phase 1b Trial of INZ-701

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BOSTON, June 27, 2023 (GLOBE NEWSWIRE) -- Inozyme Pharma, Inc. (Nasdaq: INZY), a clinical-stage rare disease biopharmaceutical company developing novel therapeutics for the treatment of pathologic mineralization and intimal proliferation, today announced dosing of the first patient in its ENERGY-1 trial, a Phase 1b clinical trial of INZ-701 in infants with ENPP1 Deficiency.

“Initiation of the ENERGY-1 trial in infants is an important milestone as we continue to advance INZ-701 with the goal of improving the lives of patients with ENPP1 Deficiency across all age groups. We are committed to a global program to identify and treat all newborns with this condition,” said Kurt Gunter, M.D., senior vice president and chief medical officer of Inozyme.

“Infants diagnosed with ENPP1 Deficiency face a high mortality risk in the first months of life. Those who survive this critical period often develop severe symptoms that adversely affect lifelong health and quality of life. I am excited to serve as a principal investigator in this trial as a first step towards delivering a potentially lifesaving therapy for this patient population,”, said David R. Weber, M.D., MSCE, Medical Director of the Center of Bone Health, Division of Endocrinology and Diabetes at the Children’s Hospital of Philadelphia (CHOP).

ENERGY-1 is a Phase 1b, single arm, open label clinical trial designed to primarily assess the safety, tolerability, pharmacokinetics (PK), and pharmacodynamics (PD) of INZ-701 in infants with ENPP1 Deficiency. The trial is expected to enroll up to eight infants between the ages of one and 12 months across multiple sites in the United States and Europe. Patients will receive subcutaneous doses of INZ-701 during the treatment period of 52 weeks and may continue to receive INZ-701 in an extension period beyond 52 weeks. Doses range from 0.2 mg/kg once weekly through 0.6 mg/kg twice weekly, with the ability to increase the dose further depending on the results of PK/PD and safety data. Additional outcome measures include evaluation of plasma pyrophosphate (PPi) levels, survival, growth, development, functional performance, cardiac function, and exploratory biomarkers.

Planned ENPP1 Deficiency Program Updates

The Company plans to host a conference call in July 2023 to provide a program update on global development plans for INZ-701 in patients with ENPP1 Deficiency. The update will cover regulatory agreements on pivotal trial designs, including plans for our pivotal trial of INZ-701 in pediatric patients which is planned to begin in Q3 2023, an overview of the ENPP1 Deficiency opportunity, and ongoing patient identification efforts.

About ENPP1 Deficiency

ENPP1 Deficiency is a progressive condition that manifests as a spectrum of diseases. The estimated genetic prevalence of ENPP1 Deficiency is approximately 1 in 64,000 pregnancies. Individuals who present in utero or in infancy are typically diagnosed with generalized arterial calcification of infancy (GACI), which is characterized by extensive vascular calcification and intimal proliferation (overgrowth of smooth muscle cells inside blood vessels), resulting in myocardial infarction, stroke, or cardiac or multiorgan failure. Approximately 50% of infants with ENPP1 Deficiency die within six months of birth. Children with ENPP1 Deficiency typically develop rickets, a condition diagnosed as autosomal-recessive hypophosphatemic rickets type 2 (ARHR2), while adults can develop osteomalacia (softened bones). ARHR2 and osteomalacia lead to pain and mobility issues. Patients can also exhibit signs and symptoms of hearing loss, arterial and joint calcification, and cardiovascular complications. There are no approved therapies for ENPP1 Deficiency.

About INZ-701

INZ-701, a recombinant Fc fusion protein, is an ENPP1 enzyme replacement therapy in development for the treatment of rare disorders of the vasculature, soft tissue, and skeleton. In preclinical studies, the experimental therapy has shown potential to prevent pathologic mineralization and intimal proliferation (the overgrowth of smooth muscle cells inside blood vessels), which can drive morbidity and mortality in devastating genetic disorders such as ENPP1 Deficiency and ABCC6 Deficiency. INZ-701 is currently in Phase 1/2 clinical trials for the treatment of ENPP1 Deficiency and ABCC6 Deficiency.

About Inozyme Pharma

Inozyme Pharma, Inc. (Nasdaq: INZY) is a clinical-stage rare disease biopharmaceutical company developing novel therapeutics for the treatment of diseases impacting the vasculature, soft tissue, and skeleton. We are developing INZ-701, an enzyme replacement therapy, to address pathologic mineralization and intimal proliferation which can drive morbidity and mortality in these severe diseases. INZ-701 is currently in Phase 1/2 clinical trials for the treatment of ENPP1 Deficiency and ABCC6 Deficiency.

For more information, please visit www.inozyme.com and follow us on LinkedIn, Twitter, and Facebook.

Cautionary Note Regarding Forward-Looking Statements

Statements in this press release about future expectations, plans, and prospects, as well as any other statements regarding matters that are not historical facts, may constitute "forward- looking statements" within the meaning of The Private Securities Litigation Reform Act of 1995. These statements include, but are not limited to, statements relating to the design of our clinical trials, the potential benefits of INZ-701 and timing and contents of our planned program update. The words "anticipate," "believe," "continue," "could," "estimate," "expect," "intend," "may," "plan," "potential," "predict," "project," "should," "target," "will," "would," and similar expressions are intended to identify forward-looking statements, although not all forward-looking statements contain these identifying words. Any forward-looking statements are based on management's current expectations of future events and are subject to a number of risks and uncertainties that could cause actual results to differ materially and adversely from those set

forth in, or implied by, such forward-looking statements. These risks and uncertainties include, but are not limited to, risks associated with the Company's ability to conduct its ongoing Phase 1/2 clinical trials of INZ-701 for ENPP1 Deficiency and ABCC6 Deficiency; obtain and maintain necessary approvals from the FDA and other regulatory authorities; continue to advance its product candidates in preclinical studies and clinical trials; replicate in later clinical trials positive results found in preclinical studies and early-stage clinical trials of its product candidates; advance the development of its product candidates under the timelines it anticipates in planned and future clinical trials; obtain, maintain, and protect intellectual property rights related to its product candidates; manage expenses; comply with the covenants under its outstanding loan agreement; and raise the substantial additional capital needed to achieve its business objectives. For a discussion of other risks and uncertainties, and other important factors, any of which could cause the Company's actual results to differ from those contained in the forward-looking statements, see the "Risk Factors" section in the Company's most recent Annual Report on Form 10-K filed with the Securities and Exchange Commission, as well as discussions of potential risks, uncertainties, and other important factors, in the Company's most recent filings with the Securities and Exchange Commission. In addition, the forward-looking statements included in this press release represent the Company's views as of the date hereof and should not be relied upon as representing the Company's views as of any date subsequent to the date hereof. The Company anticipates that subsequent events and developments will cause the Company's views to change. However, while the Company may elect to update these forward-looking statements at some point in the future, the Company specifically disclaims any obligation to do so.

Contacts

Investors:
Inozyme Pharma
Stefan Riley, Director of IR and Corporate Communications
(857) 330-8871
stefan.riley@inozyme.com

Media:
SmithSolve
Matt Pera
(973) 886-9150
matt.pera@smithsolve.com