Inozyme Pharma Announces Dosing of First Patient in First-in-Human Clinical Trial of INZ-701 in Patients with ENPP1 Deficiency

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- Preliminary biomarker and safety data expected in the first half of 2022 -

BOSTON, Nov. 15, 2021 (GLOBE NEWSWIRE) -- Inozyme Pharma, Inc. (Nasdaq: INZY), a clinical-stage rare disease biopharmaceutical company developing novel therapeutics for the treatment of abnormal mineralization, today announced that the first patient has been dosed in its first-in-human Phase 1/2 clinical trial of INZ-701 in adult patients with ENPP1 Deficiency. INZ-701 is the Company's enzyme replacement therapy in development for the treatment of mineralization disorders.

"This milestone represents an important step forward for Inozyme and, more importantly, for patients with ENPP1 Deficiency who are in dire need of an effective therapeutic option," said Axel Bolte, MSc, MBA, Inozyme's co-founder, president, and chief executive officer. "Data from preclinical studies of INZ-701 in ENPP1 Deficiency support inorganic pyrophosphate (PPi) levels as a key predictive marker of therapeutic benefit and show that INZ-701 has the potential to increase PPi, prevent clinical symptoms, and decrease mortality. We are committed to unlocking the full potential of INZ-701 in patients with ENPP1 Deficiency, and in parallel, we remain on track to commence our Phase 1/2 clinical trial in adult patients with ABCC6 Deficiency by year-end."

The Phase 1/2 open-label multiple ascending dose trial is expected to enroll up to nine adult patients at sites in the United States and Europe. The trial will primarily assess the safety and tolerability of INZ-701 in adult patients with ENPP1 Deficiency, as well as characterize the pharmacokinetic and pharmacodynamic profile of INZ-701, including evaluation of plasma PPi and other biomarker levels. The Phase 1 dose-escalation portion of the trial will use a standard 3+3 escalation design to assess INZ-701 for seven weeks at doses of 0.2 mg/kg, 0.6 mg/kg, and 1.8 mg/kg, which were selected based on preclinical studies and pharmacokinetic/pharmacodynamic modeling. The dose-escalation portion of the trial will seek to identify a safe, tolerable dose for further development that increases PPi levels. The open-label Phase 2 extension portion of the trial will assess long-term safety, pharmacokinetics, and pharmacodynamics of continued treatment with INZ-701 for up to 48 weeks. Exploratory endpoints will include evaluations of skeletal, vascular, and physical function, as well as patient-reported outcomes. The Company expects to present preliminary biomarker and safety data in the first half of 2022.

About ENPP1 Deficiency

ENPP1 Deficiency is a progressive condition that manifests as a spectrum of diseases. Those who present in utero or infancy are typically diagnosed with generalized arterial calcification of infancy (GACI), which is characterized by extensive vascular calcification and neointimal proliferation (overgrowth of smooth muscle cells inside blood vessels), resulting in myocardial infarction, stroke, or cardiac or multiorgan failure. The condition is lethal in an estimated 50% of affected babies. Children and adults with ENPP1 Deficiency typically experience rickets and osteomalacia (softened bones), also termed autosomal-recessive hypophosphatemic rickets type 2 (ARHR2), and can exhibit a range of signs and symptoms that can include hearing loss, arterial calcification, cardiac, and neurological involvement. There are no approved treatments for ENPP1 Deficiency.

About INZ-701

INZ-701 is a clinical-stage enzyme replacement therapy (ERT) in development for the treatment of mineralization disorders of the circulatory system, bones, and kidneys. In preclinical studies, the experimental therapy has shown potential to generate plasma pyrophosphate (PPi) and to restore it to appropriate physiological levels, thereby preventing calcification in the vasculature and kidneys, while at the same time normalizing bone mineralization. Inozyme is developing INZ-701 for certain rare, life-threatening, and devastating genetic disorders such as ENPP1 Deficiency and ABCC6 Deficiency in which PPi levels are below the normal physiological levels. INZ-701 is currently in a Phase 1/2 clinical trial for ENPP1 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 of abnormal mineralization impacting the vasculature, soft tissue, and skeleton. Through our in-depth understanding of the biological pathways involved in mineralization, we are pursuing the development of therapeutics to address the underlying causes of these debilitating diseases. It is well established that two genes, *ENPP1* and *ABCC6*, play key roles in a critical mineralization pathway and that defects in these genes lead to abnormal mineralization. We are initially focused on developing a novel therapy, INZ-701, to treat the rare genetic diseases of ENPP1 and ABCC6 Deficiencies. INZ-701 is currently in a Phase 1/2 clinical trial for ENPP1 Deficiency.

Inozyme Pharma was founded in 2017 by Joseph Schlessinger, Ph.D., Demetrios Braddock, M.D., Ph.D., and Axel Bolte, MSc, MBA, with technology developed by Dr. Braddock and licensed from Yale University. For more information, please visit www.inozyme.com.

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 initiation and timing of our clinical trials, trial design, the availability of clinical trial data and the potential benefits of INZ-701. 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 initiate and conduct its ongoing and planned 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; 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 Investor Relations
stefan.riley@inozyme.com

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