Inozyme Announces Acceptance of First European Clinical Trial Application for Phase 1/2 Clinical Trial of INZ-701 in ABCC6 Deficiency

June 9, 2021

- Clinical trial initiation expected in mid-2021 –
- Preliminary safety and biomarker data expected by the end of 2021 -

BOSTON, June 09, 2021 (GLOBE NEWSWIRE) -- Inozyme Pharma, Inc. (Nasdaq: INZY), a rare disease biopharmaceutical company developing novel therapeutics for the treatment of abnormal mineralization, today announced the acceptance of its Clinical Trial Application (CTA) from the National Agency for the Safety of Medicines and Health Products (ANSM) in France to allow initiation of its Phase 1/2 clinical trial of INZ-701, as a potential treatment for ABCC6 Deficiency. Inozyme plans to initiate its Phase 1/2 trial in mid-2021. This CTA was submitted and accepted as part of ANSM's Fast Track procedure designed to reduce processing times for clinical trial authorization requests for innovative medical products.

ABCC6 Deficiency is a rare, inherited disorder that can present as generalized arterial calcification of infancy (GACI) type 2 in infants and as pseudoxanthoma elasticum (PXE) in children and adults. This is one of several disorders with a significant decrease in plasma pyrophosphate (PPi) levels, a potent regulator of mineralization. In patients with ABCC6 Deficiency, the abnormal calcification caused by low PPi can result in vision loss and life-threatening cardiovascular complications, among other morbidities. There is no approved treatment for ABCC6 Deficiency.

"The acceptance of this CTA marks another important regulatory milestone for Inozyme and a key advancement for INZ-701 as a potential treatment for people living with ABCC6 Deficiency," said Axel Bolte, MSc, MBA, co-founder, president, and chief executive officer of Inozyme Pharma. "We are well-positioned to execute on our planned clinical study and the notable level of interest from the ABCC6 Deficiency community underscores the urgent need for therapeutic options. I want to express my thanks to the team at Inozyme and our external collaborators, all of whom have been instrumental in our continued progress."

INZ-701 is an ENPP1 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 PPi and to restore it to appropriate physiological levels, thereby preventing calcification in the vasculature and kidneys, while at the same time correcting bone abnormalities in Enpp1-deficient mice. In Abcc6-deficient mice, subcutaneous administration of INZ-701 (2 and 10 mg/kg every other day for two or eight weeks) led to a dose-dependent increase in plasma PPi levels at both two and eight weeks after initiation of treatment, leading to significantly lower levels of soft tissue mineralization.

About ABCC6 Deficiency
ABCC6 Deficiency is a rare, severe, inherited disorder caused by mutations in the *ABCC6* gene, leading to low levels of PPi. PPi is essential for preventing harmful soft tissue calcification and regulating bone mineralization. ABCC6 Deficiency is a systemic and progressively debilitating condition estimated to affect more than 67,000 individuals worldwide. The condition is characterized by pathological mineralization in blood vessels and soft tissues throughout the body that can drive devastating medical problems.

Some infants with ABCC6 Deficiency are diagnosed with generalized arterial calcification of infancy (GACI) type 2, a vascular condition that resembles GACI type 1, the acute infantile form of ENPP1 Deficiency. In older patients, ABCC6 Deficiency presents as pseudoxanthoma elasticum (PXE), a rare, inherited disorder in which individuals develop calcification of soft connective tissues, including in the eyes, cardiovascular system, and skin. There is no approved treatment for ABCC6 Deficiency.

About INZ-701
INZ-701 is an ENPP1 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 correcting bone abnormalities. 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.

Inozyme is preparing to initiate a Phase 1/2 clinical trial in patients with ENPP1 Deficiency in the first half of 2021 and a separate Phase 1/2 clinical trial in patients with ABCC6 Deficiency in mid-2021.

About Inozyme Pharma
Inozyme Pharma (Nasdaq: INZY) is a 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 to treat the rare genetic diseases of ENPP1 and ABCC6 Deficiencies.

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 potential of our lead product candidate, INZ-701, the initiation and timing of our

future clinical trials and our research and development programs. 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 its 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, 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
Alex Van Rees
(973) 442-1555 ext. 111
Alex.vanrees@smithsolve.com