Inozyme Pharma Provides Update for Phase 1/2 Clinical Trials in ABCC6 Deficiency and ENPP1 Deficiency

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- First patient dosed in Phase 1/2 clinical trial in ABCC6 Deficiency –

- Preliminary biomarker and safety data from Phase 1/2 clinical trial in ABCC6 Deficiency expected in the second quarter of 2022 –

- Second cohort in Phase 1/2 clinical trial in ENPP1 Deficiency fully enrolled; topline data expected in the second half of 2022 -

BOSTON, April 12, 2022 (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 Phase 1/2 clinical trial of INZ-701 in adult patients with ABCC6 Deficiency and the second cohort in the ongoing Phase 1/2 clinical trial in ENPP1 Deficiency has been fully enrolled.

“With momentum in patient enrollment in the trials for our lead indications and our recently-reported positive preliminary data in ENPP1 Deficiency, we believe we are another step closer to potentially bringing a much-needed therapeutic option to patients suffering from these devastating diseases,” said Axel Bolte, MSc, MBA, Inozyme’s co-founder, president, and chief executive officer. “In preclinical models of ABCC6 Deficiency and ENPP1 Deficiency, INZ-701 increased plasma pyrophosphate (PPi) levels and prevented soft tissue calcification, a key manifestation of both conditions. We expect to report preliminary biomarker and safety data from our ABCC6 Deficiency trial in the second quarter of 2022 and topline data from the ENPP1 Deficiency trial in the second half of 2022.”

The Company recently reported positive preliminary biomarker, safety, and pharmacokinetic (PK) data from the first three patients (cohort 1) treated in the Phase 1 portion of its ongoing first-in-human Phase 1/2 clinical trial of INZ-701 in adult patients with ENPP1 Deficiency. At the 0.2 mg/kg dose level of INZ-701, all three patients showed rapid, significant, and sustained increases in PPi levels.

INZ-701 in ABCC6 Deficiency Phase 1/2 Clinical Trial Design

The ongoing Phase 1/2 open-label clinical trial is expected to enroll up to nine adult patients with ABCC6 Deficiency at sites in the United States and Europe. The trial will primarily assess the safety and tolerability of INZ-701 in adult patients with ABCC6 Deficiency, as well as characterize the pharmacokinetic (PK) and pharmacodynamic (PD) profile of INZ-701, including the evaluation of levels of plasma PPi and other biomarkers. In the Phase 1 dose-escalation portion of the trial, Inozyme is assessing INZ-701 for 32-days at doses of 0.2 mg/kg, 0.6 mg/kg, and 1.8 mg/kg administered via subcutaneous injection twice weekly, with three patients per dose cohort. Doses were selected based on preclinical studies and PK/PD modeling. The Phase 1 dose-escalation portion of the trial seeks 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, where patients may receive doses of INZ-701 at home depending on site-specific protocols. Exploratory endpoints will include evaluations of vascular, ophthalmologic, physical function and patient-reported outcomes.

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, which we believe affects more than 67,000 individuals worldwide. The condition is characterized by pathological mineralization in blood vessels and soft tissues clinically affecting the skin, eyes, and cardiovascular system 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 are no approved therapies for ABCC6 Deficiency.

INZ-701 in ENPP1 Deficiency Phase 1/2 Clinical Trial Design

The ongoing Phase 1/2 open-label clinical trial is expected to enroll up to nine adult patients with ENPP1 Deficiency at sites in North America 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 PK and pharmacodynamic (PD) profile of INZ-701, including evaluation of PPi and other biomarker levels. In the Phase 1 dose-escalation portion of the trial, Inozyme is assessing INZ-701 for 32-days at doses of 0.2 mg/kg, 0.6 mg/kg, and 1.8 mg/kg administered via subcutaneous injection twice weekly, with three patients per dose cohort. Doses were selected based on preclinical studies and PK/PD modeling. The Phase 1 dose-escalation portion of the trial seeks to identify a safe, tolerable dose that increases PPi levels, and that can be used for further clinical development. The open-label Phase 2 extension portion of the trial is assessing long-term safety, pharmacokinetics, and pharmacodynamics of continued treatment with INZ-701 for up to 48 weeks, where patients may receive doses of INZ-701 at home depending on site-specific protocols. Exploratory endpoints will include evaluations of skeletal, vascular, physical function and patient-reported outcomes.

About ENPP1 Deficiency

ENPP1 Deficiency is a progressive condition that manifests as a spectrum of diseases. 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 neointimal proliferation (overgrowth of smooth muscle cells inside blood vessels), resulting in myocardial infarction, stroke, or cardiac or multiorgan failure. Approximately 45% to 50% of infants with ENPP1 Deficiency die within six months of birth. Children with ENPP1 Deficiency typically experience rickets, a condition also known as autosomal-recessive hypophosphatemic rickets type 2 (ARHR2) while adults experience osteomalacia (softened

bones), and they can exhibit a range of signs and symptoms that include hearing loss, arterial calcification, and cardiac and/or neurological involvement. There are no approved therapies for ENPP1 Deficiency.

About INZ-701

INZ-701 is a clinical-stage enzyme replacement therapy 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 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 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 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 Phase 1/2 clinical trials for the treatment of ENPP1 Deficiency and ABCC6 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 trial results, 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 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; 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.

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