Inozyme Pharma Announces Investor and Analyst Event and Highlights 2022 Progress

January 9, 2023

- Company to share topline data from ongoing Phase 1/2 trials of INZ-701 in ENPP1 Deficiency and ABCC6 Deficiency at virtual event on Thursday, Feb. 16, 2023 –

- Significant clinical and scientific milestones across ENPP1 Deficiency and ABCC6 Deficiency programs achieved in 2022 -

BOSTON, Jan. 09, 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 that it will share topline pharmacokinetic, pharmacodynamic (PK/PD) and safety data from the ongoing Phase 1/2 clinical trials of INZ-701 in ENPP1 Deficiency and ABCC6 Deficiency at a virtual Investor and Analyst Event on Thursday, Feb. 16, 2023.

"In 2022 we generated the first evidence that INZ-701 can drive a pharmacodynamic effect on PPi, a key regulator of mineralization, which is integral to the biology of our lead indications. In our ENPP1 Deficiency trial, we observed a rapid, significant, and sustained elevation of PPi at the lowest dose cohort. We also saw promising PPi elevation in our ABCC6 Deficiency trial, with a rapid initial increase at the lowest dose of INZ-701. We look forward to sharing data from all dose cohorts in these ongoing Phase 1/2 trials at our virtual Investor and Analyst event in February," said Axel Bolte, MSc, MBA, Inozyme's co-founder, president, and chief executive officer.

Virtual Investor and Analyst Event

The Company will share topline pharmacokinetic, pharmacodynamic (PK/PD) and safety data from ongoing Phase 1/2 trials of INZ-701 in ENPP1 Deficiency and ABCC6 Deficiency. The event will also feature presentations from members of the Inozyme management team, as well as from key opinion leaders in ENPP1 Deficiency and ABCC6 Deficiency.

The webcast will be accessible through the Investor Relations section of Inozyme's website under events and will be available for a limited time following the event.

2022 Milestones for ENPP1 Deficiency and ABCC6 Deficiency Programs

- **Phase 1/2 Clinical Trial of INZ-701 in Adults with ENPP1 Deficiency.** In April 2022, the Company reported positive preliminary PK/PD and safety data from its ongoing Phase 1/2 trial of INZ-701 in patients with ENPP1 Deficiency. The Phase 1 dose escalation portion of this trial has been fully enrolled, and dosing is ongoing in the open-label Phase 2 extension of the trial. In November 2022, the Company announced the first self-administration of INZ-701 in the open-label Phase 2 extension portion of the trial.

- **Phase 1/2 Clinical Trial of INZ-701 in Adults with ABCC6 Deficiency (pseudoxanthoma elasticum or PXE).** In July 2022, the Company reported positive preliminary PK/PD and safety data from its ongoing Phase 1/2 trial of INZ-701 in patients with ABCC6 Deficiency. The Phase 1 dose escalation portion of this trial has been fully enrolled, and dosing is ongoing in the open-label Phase 2 extension portion of the trial.

- **Natural History Studies.** Patient enrollment is underway in a prospective natural history study in ENPP1 Deficiency and ABCC6 Deficiency. Patient enrollment is also underway in a longitudinal, retrospective natural history study in ENPP1 Deficiency and ABCC6 Deficiency. These studies are designed to test and validate findings from the Company's previously published cross-sectional retrospective natural history study.

- **ENPP1 Patient Population Publications.** Peer-reviewed article in *Human Mutation* titled "ENPP1 Deficiency: A Clinical Update on the Relevance of Individual Variants Using a Locus-Specific Database," reported a three-fold increase in pathogenic/likely pathogenic *ENPP1* variants. Analysis also identified severe phenotypes in patients with monoallelic heterozygous *ENPP1* variants. Leading disease experts Carlos Ferreira, M.D., of the National Institutes of Health (NIH) and Frank Rutsch, M.D., of Münster University Children's Hospital, together with Genomenon, an AI-driven genomics company, analyzed these data and found the estimated prevalence of ENPP1 Deficiency to be 1 in 64,000 pregnancies, more than tripling the prior estimate. This research was published in a peer-reviewed article in *Orphanet Journal of Rare Diseases* titled "Estimation of ENPP1 Deficiency Genetic Prevalence Using a Comprehensive Literature Review and Population Databases."

- **Additional Peer-reviewed Scientific Publications.** Peer-reviewed article on Burden of Illness study in *PLOS One* titled "Lifelong impact of ENPP1 Deficiency and the early onset form of ABCC6 Deficiency from patient or caregiver perspective," showed that individuals with ENPP1 Deficiency or infant onset ABCC6 Deficiency experience lifelong morbidity causing substantial physical and emotional burden to patients and caregivers. Peer-reviewed article in *Experimental Dermatology* titled "INZ-701, a recombinant ENPP1 enzyme, prevents ectopic calcification in an

Abcc6 $^{-/-}$ mouse model of pseudoxanthoma elasticum," showed that INZ-701 increased PPi and prevented skin calcification in an Abcc6-deficient mouse model and therefore might provide therapeutic benefit in ABCC6 Deficiency.

- **Collaboration with Rady Children's Institute for Genomic Medicine (RCIGM).** Inozyme became a founding member of the Public-Private BeginNGS™ Consortium established by RCIGM to advance and evaluate a novel newborn screening technology to facilitate the diagnosis of genetic diseases. The collaboration focuses on a diagnostic and precision medicine guidance tool called BeginNGS™, which incorporates rapid Whole Genome Sequencing (rWGS®) to currently screen newborns for approximately 400 genetic diseases, including generalized arterial calcification of infancy (GACI), the infant form of ENPP1 Deficiency.

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 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.

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 pharmacokinetic (PK) and pharmacodynamic (PD) profile of INZ-701, including evaluation of plasma pyrophosphate (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 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 affects more than 67,000 individuals worldwide. Infants with ABCC6 Deficiency are diagnosed with generalized arterial calcification of infancy (GACI) type 2, a condition that resembles GACI type 1, the infant form of ENPP1 Deficiency. In older individuals, ABCC6 Deficiency presents as pseudoxanthoma elasticum (PXE), which is characterized by pathological mineralization in blood vessels and soft tissues clinically affecting the skin, eyes, and vascular system. There are no approved therapies for ABCC6 Deficiency.

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 INZ-701

INZ-701 is a clinical-stage enzyme 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, a potential first-in-class enzyme 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 timing of our ongoing and planned clinical trials and other studies, the availability of data from clinical trials, the potential benefits of INZ-701, the impact of the debt facility on the Company's balance sheet and the sufficiency of the Company's cash resources. 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 and Quarterly Report on Form 10-Q 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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