



Inozyme Pharma to Present Recently Announced Preliminary Data from Ongoing Phase 1/2 Trial in ENPP1 Deficiency at the European Calcified Tissue Society Congress (ECTS)

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BOSTON, April 14, 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 Yves Sabbagh, Ph.D., the company's senior vice president and chief scientific officer, will present recently [announced](#) preliminary data from the ongoing Phase 1/2 clinical trial of INZ-701 in patients with ENPP1 Deficiency in an oral presentation on Monday, April 17 at the European Calcified Tissue Society Congress (ECTS) in Liverpool, UK.

In February 2023, the Company announced positive preliminary pharmacokinetic (PK), pharmacodynamic (PD), and safety data with encouraging patient reported outcome data from the ongoing trial. Dosing is ongoing in the Phase 2 portion of the trial and the Company is on track to report interim clinical data in the third quarter of 2023. The Company also plans to add an additional cohort to investigate the potential for once-weekly dosing in the ongoing trial.

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 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.

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

The ongoing Phase 1/2 open-label clinical trial initially enrolled 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 assessed 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 with three patients per dose cohort. Patients received a single dose and then began twice weekly dosing one week later. 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 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 ectopic calcification, skeletal, vascular, and physical function, patient-reported outcomes, and exploratory biomarkers.

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](#).

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