



## Inozyme Pharma Announces First Self-Administration of INZ-701 in Ongoing ENPP1 Deficiency Phase 1/2 Clinical Trial

November 3, 2022

BOSTON, Nov. 03, 2022 (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 the first self-administration of INZ-701 in the open-label Phase 2 extension portion of the ongoing Phase 1/2 clinical trial of INZ-701 in adult patients with ENPP1 Deficiency.

The implementation of self-administration of INZ-701 followed Inozyme's submission of a protocol amendment to the U.S. Food and Drug Administration (FDA) and approval of a protocol amendment submitted to the German Federal Institute for Drugs and Medical Devices (BfArM). The Company has also submitted documentation to additional ex-U.S. regulatory agencies to allow self-administration. These submissions were supported by preliminary safety data from the ongoing trial.

"This is a significant milestone for a product candidate at this stage of its clinical and regulatory pathway and we believe highlights the safety profile of INZ-701 to date in our ongoing Phase 1/2 clinical trial," said Kurt Gunter, M.D., Inozyme's senior vice president and chief medical officer. "Self-administration opens up a convenient dosing option for patients in the Phase 2 extension. The extension portion of the trial is designed to provide important long-term safety and clinical data and support the development of INZ-701 as the first potential approved therapy for ENPP1 Deficiency."

Inozyme is facilitating self-administration in the Phase 2 extension portion of the clinical trial after patients complete the 32-day dose evaluation period in the clinic. INZ-701 is supplied as a lyophilized powder intended for reconstitution and subcutaneous injection. In its lyophilized form, INZ-701 has been shown to be shelf stable for up to three years. Long-term shelf stability along with the ability for self-administration significantly improves convenience for patients.

### 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 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.

### 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, 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](http://www.inozyme.com) and follow us on [LinkedIn](#), [Twitter](#), and [Facebook](#).

### Contacts

Investors:  
Inozyme Pharma  
Stefan Riley, Director of IR and Corporate Comms  
(857) 330-8871  
[stefan.riley@inozyme.com](mailto:stefan.riley@inozyme.com)

Media:  
SmithSolve  
Matt Pera  
(973) 886-9150  
[matt.pera@smithsolve.com](mailto:matt.pera@smithsolve.com)