



Inozyme Pharma to Present Recently Announced Data from Phase 1/2 Trials of INZ-701 in Adults with ENPP1 Deficiency and ABCC6 Deficiency (PXE) at Upcoming Medical Conferences

May 23, 2024

BOSTON, May 23, 2024 (GLOBE NEWSWIRE) -- [Inozyme Pharma, Inc.](https://www.inozyme.com) (Nasdaq: INZY) ("the Company" or "Inozyme"), a clinical-stage rare disease biopharmaceutical company developing novel therapeutics for the treatment of pathologic mineralization and intimal proliferation, today announced that the Company will present [recently announced topline data](#) from the Company's ongoing Phase 1/2 clinical trials of INZ-701 in adults with ENPP1 Deficiency and ABCC6 Deficiency (manifesting as pseudoxanthoma elasticum, or PXE), during oral presentations at two upcoming medical conferences.

Details regarding the presentations are as follows:

The European Calcified Tissue Society Congress (ECTS) 2024 being held May 25-28, 2024 in Marseille, France.

Title: Impact of INZ-701 on Bone and Mineral Metabolism Biomarkers and Clinical Outcomes in Adults with ENPP1 Deficiency – Results from 48-week Phase 1/2 Open Label Study

Presentation Number: COP05

Session Title: Concurrent Oral Presentations 1: Rare Bone Diseases

Date: Sunday, May 26, 2024

Time: 9:45 – 9:55 CEST / 3:45am – 3:55am ET

Location: Callelongue

Presenter: Yves Sabbagh, Ph.D., Senior Vice President and Chief Scientific Officer

Title: Safety and Exploratory Efficacy of INZ-701 in Adults with ABCC6 Deficiency Manifesting as Pseudoxanthoma Elasticum – Results from 48-week Phase 1/2 Open Label Study

Presentation Number: P237

Session Title: Concurrent Oral Poster Presentations 1: Clinical/Public Health

Date: Sunday, May 26, 2024

Time: 18:15 – 18:20 CEST/ 12:15pm – 12:20pm ET

Location: Callelongue

Presenter: Yves Sabbagh, Ph.D., Senior Vice President and Chief Scientific Officer

The Endocrine Society's Annual Meeting (ENDO) 2024 being held June 1-4, 2024, in Boston, Massachusetts.

Title: Impact of INZ-701 on Bone and Mineral Metabolism Biomarkers and Clinical Outcomes in Adults with ENPP1 Deficiency-Results from 48-week Phase 1/2 Open Label Study

Abstract Number: 7217

Session Title: Oral Abstract and Rapid-Fire

Date: Monday, June 3, 2024

Time: 2:45pm – 3:00pm ET

Location: Boston Convention & Exhibition Center (BCEC): 258ABC – BCEC

Presenter: Kurt Gunter, M.D., Senior Vice President and Chief Medical Officer

About ENPP1 Deficiency

ENPP1 Deficiency is a progressively debilitating condition of the vasculature, soft tissue, and skeleton with a prevalence of approximately 1 in 64,000 pregnancies worldwide. Although ENPP1 Deficiency was initially described in patients with biallelic ENPP1 Deficiency (homozygous or compound heterozygous mutations), many patients with monoallelic ENPP1 Deficiency (heterozygous mutations) have clinical symptoms, potentially increasing the worldwide prevalence. Individuals who present in utero or in infancy are typically diagnosed with generalized arterial calcification of infancy (GACI Type 1) and approximately 50% of infants 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 adolescents and 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 the PD marker, 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 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 sought to identify a safe, tolerable dose that increases PPI levels, and that can be used for further clinical development. Following completion of the Phase 1 portion of the first three cohorts, Inozyme dosed patients in a fourth cohort at 1.2 mg/kg to investigate the potential for once-weekly dosing of INZ-701. The open-label Phase 2 extension portion of the trial is assessing long-term safety, PK, and PD of continued treatment with INZ-701 for at least 48 weeks, where patients may self-administer INZ-701. Exploratory endpoints include evaluations of skeletal, vascular, physical function and patient-reported outcomes.

About ABCC6 Deficiency

ABCC6 Deficiency is a progressively debilitating condition of the vasculature and soft tissue that is estimated to affect approximately 1 in 25,000 to 1 in 50,000 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. Pediatric patients who survive the first year of life may develop neurological

disease, including stroke, and cardiovascular disease secondary to ongoing vascular calcification and stenosis. In older individuals, ABCC6 Deficiency presents as pseudoxanthoma elasticum (PXE), which is characterized by pathologic 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 enrolled ten 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 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 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 sought to identify a safe, tolerable dose for further development that increases PPI levels. The open-label Phase 2 extension portion of the trial is assessing long-term safety, PK, and PD of continued treatment with INZ-701 for at least 48 weeks, where patients may self-administer INZ-701. Exploratory endpoints will include evaluations of vascular, ophthalmologic, physical function and patient-reported outcomes.

About INZ-701

INZ-701, a recombinant Fc fusion protein, is an ENPP1 enzyme replacement therapy (ERT) in development for the treatment of rare disorders of the vasculature, soft tissue, and skeleton. INZ-701 metabolizes adenosine triphosphate (ATP) to generate PPI, a natural inhibitor of mineralization, and AMP, which can be processed to phosphate and adenosine, the latter being a natural inhibitor of intimal proliferation. In preclinical studies, the experimental therapy has shown potential to prevent pathologic mineralization and intimal proliferation, which can drive morbidity and mortality in devastating disorders such as, ENPP1 Deficiency, ABCC6 Deficiency, and calciphylaxis. Clinical data to date have demonstrated that INZ-701 was generally well tolerated, exhibited a favorable safety profile, and meaningfully increased PPI levels in multiple clinical trials.

About Inozyme Pharma

Inozyme Pharma, Inc. is a clinical-stage rare disease biopharmaceutical company developing novel therapeutics for the treatment of diseases impacting the vasculature, soft tissue, and skeleton. Inozyme is 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 clinical development for the treatment of ENPP1 Deficiency, ABCC6 Deficiency and calciphylaxis.

For more information, please visit <https://www.inozyme.com/> or follow Inozyme on [LinkedIn](#), [X](#), 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 availability of data from clinical trials, 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 clinical trials of INZ-701 for ENPP1 Deficiency and ABCC6 Deficiency; enroll patients in ongoing and planned trials; 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; comply with covenants under its outstanding loan agreement; 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.

Contacts

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

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