Inozyme Pharma Reports First Quarter 2023 Financial Results and Provides Business Highlights

May 9, 2023

- Upcoming clinical and regulatory milestones remain on track with previous guidance –

- Current cash, cash equivalents and short-term investments anticipated to fund cash flow requirements into the fourth quarter of 2024 -

BOSTON, May 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 reported financial results for the first quarter ended March 31, 2023, and provided business highlights.

“We are continuing to advance INZ-701 and look forward to initiating a pivotal trial for pediatric patients with ENPP1 Deficiency in the third quarter of this year, subject to receipt of regulatory approval. These patients represent a population with high unmet medical need and they and their caregivers eagerly anticipate the planned clinical trial," said Douglas A. Treco, Ph.D., chief executive officer and chairman of the Company’s board of directors. “Our team is executing well, and we continue to collect clinical data from our ongoing trials in adults with ENPP1 Deficiency and ABCC6 Deficiency, which will provide important insights into the clinical effects of INZ-701.”

Business Highlights

- **Regulatory Update.** The Company has reached agreement with the European Medicines Agency (EMA) on a Pediatric Investigational Plan (PIP) for the study of INZ-701 in pediatric patients with ENPP1 Deficiency.

- **Phase 1/2 Clinical Trial of INZ-701 in Adults with ENPP1 Deficiency.** The Company dosed the first adult patient with ENPP1 Deficiency in an additional dose cohort designed to investigate the potential for once-weekly dosing of INZ-701 in the ongoing trial.

- **Phase 1/2 Clinical Trial of INZ-701 in Adults with ABCC6 Deficiency (pseudoxanthoma elasticum or PXE).** The Company announced the first self-administration of INZ-701 in the open-label Phase 2 portion of the ongoing clinical trial.

- **Medical Conference Presentations.** Yves Sabbagh, Ph.D., the Company’s senior vice president and chief scientific officer, presented the recently announced topline data from the ongoing Phase 1/2 clinical trial of INZ-701 in patients with ENPP1 Deficiency at the European Calcified Tissue Society (ECTS) on April 17, 2023. Kurt Gunter, M.D., the Company’s senior vice president and chief medical officer, will present these data at the European Congress of Endocrinology (ECE 2023) scheduled May 13-16.

Anticipated Milestones

- **ENPP1 Deficiency**
  - Initiation of ENERGY-1 – Phase 1b clinical trial to evaluate the safety, tolerability, pharmacokinetic and pharmacodynamic profile of INZ-701 in infants – Q2 2023
  - Initiate scientific advice process with EMA regarding our comprehensive development plan covering all age groups– Q2 2023
  - Interim data from the Phase 2 portion of the ongoing trial in adults – Q3 2023
  - Initiation of pivotal trial in pediatric patients, subject to regulatory approval – Q3 2023

- **ABCC6 Deficiency**
  - Interim data from the Phase 2 portion of the ongoing trial in adults – Q4 2023

First Quarter 2023 Financial Results

- **Cash Position and Financial Guidance** – Cash, cash equivalents, and short-term investments were $130.9 million as of March 31, 2023. Based on its current plans, the Company anticipates its cash, cash equivalents, and short-term investments as of March 31, 2023 will enable the Company to fund cash flow requirements into the fourth quarter of 2024.

- **Research and Development (R&D) Expenses** – R&D expenses of $11.9 million for the quarter ended March 31, 2023 were relatively consistent with R&D expenses of $11.8 million for the prior-year period.

- **General and Administrative (G&A) Expenses** – G&A expenses were $6.5 million for the quarter ended March 31, 2023, compared to $5.0 million for the prior-year period. The increase was primarily related to the expenses recorded for the transition and separation agreement entered into with our former chief executive officer.

- **Net Loss** – Net loss was $17.4 million, or $0.40 loss per share, for the quarter ended March 31, 2023, compared to $16.9 million, or $0.71 loss per share, for the prior-year period.

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 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 initially enrolled nine adult patients with ABCC6 Deficiency at sites in the United States and Europe. The trial is designed to 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 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 was designed to identify a safe, tolerable dose for further development that increases PPi levels. 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 include evaluations of ectopic calcification, vascular and retinal function, patient-reported outcomes and exploratory biomarkers.

About INZ-701

INZ-701 is a clinical-stage enzyme replacement 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 adult 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, 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.

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 planned clinical trials, the availability of data from clinical trials, timing of planned regulatory meetings, the potential benefits of INZ-701, 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; comply with the 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.

Condensed Consolidated Balance Sheet Data
(Unaudited)
(in thousands)

	March 31, 2023	December 31, 2022
Cash, cash equivalents and short-term investments	$ 130,930	$ 127,866
Total assets	$ 141,796	$ 139,195
Total liabilities	$ 38,449	$ 20,801
Additional paid-in-capital	$ 335,544	$ 333,356
Accumulated deficit	$ (232,165)	$ (214,761)
Total stockholders' equity	$ 103,347	$ 118,394

Condensed Consolidated Statements of Operations and Comprehensive Loss
(Unaudited)
(in thousands, except share and per share data)

	Three Months Ended March 31,	
	2023	2022
Operating expenses:		
Research and development	$ 11,857	$ 11,814
General and administrative	6,512	5,025
Total operating expenses	18,369	16,839
Loss from operations	(18,369)	(16,839)
Other income (expense):		
Interest income, net	999	60
Other expense, net	(34)	(105)
Other income (expense), net	965	(45)
Net loss	$ (17,404)	$ (16,884)
Other comprehensive income (loss):		
Unrealized gains (losses) on available-for-sale securities	150	(132)
Foreign currency translation adjustment	19	(15)
Total other comprehensive income (loss)	169	(147)
Comprehensive loss	$ (17,235)	$ (17,031)
Net loss attributable to common stockholders—basic and diluted	$ (17,404)	$ (16,884)
Net loss per share attributable to common stockholders—basic and diluted	$ (0.40)	$ (0.71)
Weighted-average common shares outstanding—basic and diluted	43,720,578	23,686,351

Contacts

Investors:
Inozyme Pharma
Stefan Riley, Director of IR and Corporate Communications
(857) 330-8871

stefan.riley@inozyme.com

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