Inozyme Pharma Reports Full Year 2022 Financial Results and Provides Business Highlights

March 22, 2023

- Founding CEO, Axel Bolte, MSc, MBA, to retire; Douglas A. Treco, Ph.D., to succeed as CEO -

- Matthew Winton, Ph.D., appointed COO -

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

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

BOSTON, March 22, 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 full year ended December 31, 2022, and provided recent business highlights. The Company also today announced that founding chief executive officer (CEO), Axel Bolte, MSc, MBA, will retire from his current role and that Douglas A. Treco, Ph.D., chairman of the Company's board of directors (Board), will succeed Mr. Bolte as the CEO of the Company, effective April 1, 2023.

"With the recent release of positive topline data for INZ-701 in the ongoing ENPP1 Deficiency and ABCC6 Deficiency trials, the planned initiation of pivotal trials this year, and a strong cash balance, Inozyme is well-positioned for its next chapter. After more than seven years leading Inozyme from concept to Phase 3 readiness, I am excited to pass the baton to Doug to lead the Company into late-stage clinical development and pre-commercial activities," said Mr. Bolte. "Doug's experience as a leader of multiple rare disease companies and deep knowledge of our team and science uniquely positions him to lead Inozyme and further unlock its potential value. I look forward to assisting the Company and Doug in this next phase."

"The Board and I are immensely grateful for Axel's leadership and accomplishments from forming the Company to advancing its lead programs to Phase 3 readiness. I look forward to continuing to benefit from his experience as a senior advisor and Board member," said Dr. Treco. "Throughout my career, I have been driven to make a difference for patients living with rare disease and I believe INZ-701 is a unique product candidate which has the potential to transform the treatment of multiple severe disorders."

Recent Management Updates

- **CEO Transition.** Axel Bolte, MSc, MBA, the Company's founding CEO will retire and continue to work with Inozyme as a senior advisor and serve on the Board. Douglas A. Treco, Ph.D., an industry veteran and Chairman of the Board, will succeed Mr. Bolte as the CEO of the Company, effective April 1, 2023. Dr. Treco was co-founder, president, and chief executive officer of Ra Pharmaceuticals, Inc. (acquired in 2020 by UCB S.A. for $2.5 billion) where he oversaw the discovery and development of zilucoplan in myasthenia gravis, which is currently under review for marketing approval in the United States and European Union. Previously, he co-founded Transkaryotic Therapies, Inc. (TKT; acquired in 2005 by Shire plc), where he directed research and development efforts which led to the approval of Replagal®, Elaprase®, Dynepo™, and Vpriv®. Dr. Treco is a member of the Board of Directors of CRISPR Therapeutics AG.

- **COO Appointment.** Matthew Winton, Ph.D. has been appointed chief operations officer, effective April 3, 2023. Dr. Winton most recently served as senior vice president and head of the Multiple Sclerosis franchise for Biogen's US organization. Previously, he was the head of Biogen's Spinal Muscular Atrophy franchise in the US, where he was responsible for setting strategic direction for the infant, pediatric, and adult markets. Matt also served as director, Payer and Channel Marketing at Biogen, where he was responsible for the development and execution of pricing, access, and reimbursement strategies across various therapeutic franchises. In particular, he helped successfully prepare the organization for the approval and launch of its first orphan disease drug, SPINRAZA™.

"Matt's extensive strategic and commercial experience in rare diseases will be critical as we plan to execute our pivotal trials in ENPP1 Deficiency and continue to develop the market opportunity across our indications. We are excited to begin working with him," added Dr. Treco.

Recent Clinical and Regulatory Updates

- **Expanded Access Program (EAP).** The Company recently dosed the first pediatric patient with ENPP1 Deficiency with INZ-701 under its EAP.

- **Regulatory Progress.** The Company has initiated discussions with the U.S. Food and Drug Administration (FDA) regarding the design and clinical endpoints for pivotal trials in ENPP1 Deficiency.

- **Phase 1/2 Clinical Trial of INZ-701 in Adults with ENPP1 Deficiency.** The Company recently announced positive topline pharmacokinetic (PK), pharmacodynamic (PD) and safety data and 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 investigate the potential for once-weekly dosing in the

ongoing trial.

- **Phase 1/2 Clinical Trial of INZ-701 in Adults with ABCC6 Deficiency (pseudoxanthoma elasticum or PXE).** The Company recently announced positive topline PK, PD and safety 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 fourth quarter of 2023.

- **Calciphylaxis Program.** The Company recently received allowance of its investigational new drug (IND) application from the FDA to evaluate INZ-701 in a clinical trial in patients with end stage kidney disease (ESKD) and calciphylaxis.

Anticipated Milestones in 2023

- **ENPP1 Deficiency**
  - Initiation of ENERGY-1 - Phase 1b clinical trial to evaluate the safety, tolerability, PK and PD of INZ-701 in infants—Q2 2023
  - Interim clinical data from ongoing Phase 1/2 trial in adults—Q3 2023
  - Initiation of pivotal trial in pediatric patients, subject to regulatory approval—Q3 2023
  - Start of European Medicines Agency protocol assistance meetings—Q4 2023

- **ABCC6 Deficiency**

  - Interim clinical data from ongoing Phase 1/2 trial in adults—Q4 2023

Financial Results for the Year Ended December 31, 2022

- **Cash Position and Financial Guidance** – Cash, cash equivalents, and short-term investments were $127.9 million as of December 31, 2022. Based on its current plans, the Company anticipates its cash, cash equivalents, and short-term investments as of December 31, 2022, together with the additional $20.0 million borrowed on February 15, 2023 under its existing debt facility, will enable the Company to fund cash flow requirements into the fourth quarter of 2024.

- **Research and Development (R&D) Expenses** – R&D expenses were $47.8 million for the year ended December 31, 2022, compared to $37.7 million for the year ended December 31, 2021. This increase was primarily due to an increase in clinical trial costs due to the progression of the clinical trials of INZ-701 for ENPP1 Deficiency and ABCC6 Deficiency, costs for consultants to support our ongoing trials, and personnel-related costs, partially offset by a decrease in research costs.

- **General and Administrative (G&A) Expenses** – G&A expenses were $20.8 million for the year ended December 31, 2022, compared to $18.9 million for the year ended December 31, 2021. The increase was primarily due to an increase in personnel costs.

- **Net Loss** – Net loss was $67.1 million, or $1.78 loss per share, for the year ended December 31, 2022, compared to $56.6 million, or $2.40 loss per share, for the year ended December 31, 2021.

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 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 initially enrolled nine adult patients with ENPP1 Deficiency at sites in North America and Europe. The trial is designed to 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 was designed 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 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 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 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 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.

Condensed Consolidated Balance Sheet Data
(Unaudited)

(in thousands)

	December 31, 2022	December 31, 2021
Cash, cash equivalents and investments	$ 127,866	$ 111,801
Total assets	$ 139,195	$ 123,541
Total liabilities	$ 20,801	$ 14,273

Additional paid-in-capital	$ 333,356	$ 256,948
Accumulated deficit	$ (214,761)	$ (147,700)
Total stockholders' equity	$ 118,394	$ 109,268

Condensed Consolidated Statements of Operations and Comprehensive Loss
(Unaudited)

(in thousands, except share and per share data)

	Year Ended December 31,	
	2022	**2021**
Operating expenses:		
Research and development	$ 47,849	$ 37,720
General and administrative	20,826	18,926
Total operating expenses	68,675	56,646
Loss from operations	(68,675)	(56,646)
Other income (expense):		
Interest income, net	1,933	211
Other expense, net	(319)	(189)
Other income, net	1,614	22
Net loss	$ (67,061)	$ (56,624)
Other comprehensive (loss) income:		
Unrealized (losses) on available-for-sale securities	(198)	(4)
Foreign currency translation adjustment	(25)	20
Total other comprehensive (loss) income	(223)	16
Comprehensive loss	$ (67,284)	$ (56,608)
Net loss attributable to common stockholders—basic and diluted	$ (67,061)	$ (56,624)
Net loss per share attributable to common stockholders—basic and diluted	$ (1.78)	$ (2.40)
Weighted-average common shares outstanding—basic and diluted	37,763,168	23,558,306

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