Inozyme Pharma Reports First Quarter 2024 Financial Results and Provides Business Highlights

May 7, 2024

- Interim data from SEAPORT-1, a Phase 1 trial of INZ-701 in patients with end-stage kidney disease receiving hemodialysis, on track for fourth quarter of 2024 -

- Interim data from ENERGY-1, a Phase 1b trial of INZ-701 in infants with ENPP1 Deficiency, on track for second half of 2024 -

- Cash, cash equivalents, and short-term investments as of March 31, 2024, expected to fund operations into the fourth quarter of 2025 -

BOSTON, May 07, 2024 (GLOBE NEWSWIRE) -- Inozyme Pharma, Inc. (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 reported financial results for the first quarter ended March 31, 2024, and provided business highlights.

"We were extremely pleased to see preliminary evidence of improved vascular health with INZ-701 treatment in adults with ABCC6 Deficiency, providing strong support for further clinical development in this disease," said Douglas A. Treco, Ph.D., CEO and Chairman of Inozyme. "We found that children with ABCC6 Deficiency are at high risk for neurological and visual impairment and represent the most pressing unmet need in this disease, substantially expanding the addressable population beyond impacted adults. We look forward to working with regulators to establish a path to approval for our ABCC6 Deficiency program, as well as presenting additional data from current trials in our calciphylaxis and ENPP1 Deficiency programs."

Recent Highlights

- **Phase 1/2 Clinical Trial of INZ-701 in Adults with ABCC6 Deficiency.** In April 2024, the Company announced positive topline safety and immunogenicity data, with clinical improvements in vascular pathology, visual function, and patient reported outcomes (PROs).

- **Natural History Studies and Development Plans for INZ-701 in Pediatric ABCC6 Deficiency.** In April 2024, the Company reported initial findings from natural history studies which indicate a substantial disease burden among pediatric patients with ABCC6 Deficiency, manifesting as a high incidence of major clinical events, notably stroke, severe neurological disease, and severe cardiovascular disease, occurring early in life. Subject to regulatory review and sufficient funding, the Company expects to initiate a pivotal trial in pediatric patients with ABCC6 Deficiency in Q1 2025.

- **Phase 1/2 Clinical Trial of INZ-701 in Adults with ENPP1 Deficiency.** In April 2024, the Company announced positive topline data indicating that the previously-reported favorable safety, immunogenicity, and clinical outcome data were maintained through 48 weeks in Cohorts 1-3. Data from Cohort 4 support once weekly dosing in ongoing and future clinical trials.

Anticipated Milestones

- **ENPP1 Deficiency**
  - Initiation of the ENERGY-2 pivotal trial in infants, Ex-U.S. – 2H 2024
  - Interim data from the ENERGY-1 Phase 1b trial in infants – 2H 2024
  - Topline data from the ENERGY-3 pivotal trial in pediatric patients – Mid-2025

- **ABCC6 Deficiency**
  - Initiation of pivotal clinical trial in pediatric patients, subject to regulatory review and sufficient funding – Q1 2025

- **Calciphylaxis**
  - Interim data from SEAPORT-1 Phase 1 trial in patients with end-stage kidney disease (ESKD) receiving hemodialysis – Q4 2024

First Quarter 2024 Financial Results

- **Cash Position and Financial Guidance.** Cash, cash equivalents, and short-term investments were $166.2 million as of March 31, 2024. Based on its current plans, the Company anticipates its cash, cash equivalents, and short-term investments as of March 31, 2024, will enable the Company to fund cash flow requirements into Q4 2025.

- **Research and Development (R&D) Expenses.** R&D expenses were $19.1 million for the quarter ended March 31, 2024, compared to $11.9 million for the prior-year period.

- **General and Administrative (G&A) Expenses.** G&A expenses were $5.2 million for the quarter ended March 31, 2024,

compared to $6.5 million for the prior-year period.

- **Net Loss.** Net loss was $23.3 million, or $0.38 loss per share, for the quarter ended March 31, 2024, compared to $17.4 million or $0.40 loss per share for the prior-year period.

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.

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.

About Calciphylaxis

Calciphylaxis is a rare disorder with a high mortality rate that mostly affects patients with end-stage kidney disease (ESKD). The disease is associated with low levels of pyrophosphate (PPi) and is characterized by pathologic mineralization (i.e., calcification) and intimal proliferation (the overgrowth of smooth muscle cells inside blood vessels) of the vasculature in the skin and fatty tissue leading to poor blood flow, blood clots, painful skin ulcers, serious infections, and death. Patients with calciphylaxis have a reported one-year survival rate of approximately 50%. The estimated incidence of calciphylaxis is approximately 3.5 per 1,000 patients with ESKD with approximately 5,000 new patients presenting annually across major geographies. There are no approved therapies for calciphylaxis.

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 initiation, timing, and design of our planned clinical trials, the availability and timing of data from clinical trials, the potential benefits of INZ-701, our regulatory strategy, including our plan to work with regulators to establish a path to approval for our ABCC6 Deficiency program, and the period over which we believe that our existing cash, cash equivalents, and short-term investments will be sufficient to fund our cash flow requirements. 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 ABCC6 Deficiency, ENPP1 Deficiency, and calciphylaxis; enroll patients in ongoing and planned trials; obtain and maintain necessary approvals from the Food and Drug Administration 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)

	March 31, 2024	December 31, 2023
Cash, cash equivalents, and investments	$ 166,153	$ 188,589
Total Assets	$ 176,943	$ 200,847
Total Liabilities	$ 58,107	$ 60,368
Additional paid-in-capital	$ 428,212	$ 426,362
Accumulated deficit	$ (309,277)	$ (285,930)
Total Stockholders' Equity	$ 118,836	$ 140,479

Condensed Consolidated Statements of Operations and Comprehensive Loss
(Unaudited)

	Three Months Ended March 31,	
	2024	2023
Operating expenses:		
Research and development	$ 19,111	$ 11,857
General and administrative	5,234	6,512
Total operating expenses	24,345	18,369
Loss from operations	(24,345)	(18,369)
Other income (expense):		
Interest income	2,374	1,327
Interest expense	(1,325)	(328)
Other expenses	(51)	(34)
Other income (expense), net	998	965
Net loss	$ (23,347)	$ (17,404)
Other comprehensive (loss) income:		
Unrealized (losses) gains on available-for-sale securities	(156)	150
Foreign currency translation adjustment	10	19
Total other comprehensive (loss) income	(146)	169
Comprehensive loss	$ (23,493)	$ (17,235)
Net loss attributable to common stockholders—basic and diluted	$ (23,347)	$ (17,404)
Net loss per share attributable to common stockholders—basic and diluted	$ (0.38)	$ (0.40)
Weighted-average common shares outstanding—basic and diluted	61,772,279	43,720,578

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