



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

August 6, 2024

- Complete enrollment in ENERGY 3, a pivotal trial of INZ-701 in pediatric patients with ENPP1 Deficiency, expected third quarter of 2024 -
- Interim data from ENERGY 1, a Phase 1b trial of INZ-701 in infants with ENPP1 Deficiency, on track for fourth quarter of 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 -
- Cash, cash equivalents, and short-term investments as of June 30, 2024, expected to fund operations into the fourth quarter of 2025 -

BOSTON, Aug. 06, 2024 (GLOBE NEWSWIRE) -- [Inozyme Pharma, Inc.](#) (Nasdaq: INZY) ("the Company" or "Inozyme"), a clinical-stage rare disease biopharmaceutical company developing innovative therapeutics for rare diseases that affect bone health and blood vessel function, today reported financial results for the second quarter ended June 30, 2024, and provided business highlights.

"We are making strides in advancing INZ-701 through our clinical programs, with several significant milestones expected by year-end," said Douglas A. Treco, Ph.D., Chief Executive Officer and Chairman of Inozyme's Board of Directors. "Notably, we expect to provide an update on our planned pathway to approval for the ABCC6 Deficiency program and present the first look at clinical data from our ongoing infant ENPP1 Deficiency trial, along with interim data from the SEAPORT 1 trial in calciphylaxis. Our unwavering dedication to developing transformative treatments for patients with rare diseases linked to the PPI-Adenosine Pathway drives us forward with a profound sense of urgency."

Recent Highlights

Pipeline

- **Publication of Preclinical Data in Cells.** In July 2024, the Company published preclinical data supporting the potential of INZ-701 to treat a broad range of diseases mediated by the PPI-Adenosine Pathway, which regulates mineralization and intimal proliferation (the overgrowth of smooth muscle cells inside blood vessels). The article titled, "[Inhibition of Vascular Smooth Muscle Cell Proliferation by ENPP1: The Role of CD73 and the Adenosine Signaling Axis](#)", was published in the journal *Cells*.

ENPP1 Deficiency

- **Presentation and Symposium at the 11th International Conference on Children's Bone Health (ICCBH)** . In June 2024, results from a radiographic study describing skeletal features of pediatric patients with ENPP1 Deficiency [were presented](#) at the 11th International Conference on Children's Bone Health in Salzburg, Austria. In addition, the Company hosted a sponsored symposium titled, "Recognizing ENPP1 Deficiency - An overlooked cause of hypophosphatemic rickets".
- **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. Data were [subsequently featured](#) at the European Calcified Tissue Society Congress (ECTS) 2024 in May in Marseille, France and the Endocrine Society's Annual Meeting (ENDO) 2024 in June in Boston.

ABCC6 Deficiency

- **FDA Fast Track Designation Granted for INZ-701 in ABCC6 Deficiency.** In July 2024, the Company [announced](#) that the U.S. Food and Drug Administration (FDA) has granted Fast Track designation to INZ-701 for the treatment of ABCC6 Deficiency (manifesting as pseudoxanthoma elasticum, or PXE).
- **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). Data were [subsequently featured](#) at the ECTS 2024. Subject to regulatory review and sufficient funding, the Company expects to initiate a pivotal trial in pediatric patients with ABCC6 Deficiency in Q1 2025.
- **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 indicated 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.

Anticipated Milestones

- **ENPP1 Deficiency**

- Complete enrollment of ENERGY 3 pivotal trial in pediatric patients – Q3 2024
- Initiation of the ENERGY 2 pivotal trial in infants, Ex-U.S. – Q4 2024
- Interim data from the ENERGY 1 Phase 1b trial in infants – Q4 2024
- Topline data from the ENERGY 3 pivotal trial in pediatric patients – 2H 2025

- **ABCC6 Deficiency**

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

- **Calciophylaxis**

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

Second Quarter 2024 Financial Results

- **Cash Position and Financial Guidance.** Cash, cash equivalents, and short-term investments were \$144.5 million as of June 30, 2024. Based on its current plans, the Company anticipates its cash, cash equivalents, and short-term investments as of June 30, 2024, will enable the Company to fund cash flow requirements into Q4 2025.
- **Research and Development (R&D) Expenses.** R&D expenses were \$21.8 million for the quarter ended June 30, 2024, compared to \$11.7 million for the prior-year period.
- **General Administrative (G&A) Expenses.** G&A expenses were \$5.9 million for the quarter ended June 30, 2024, compared to \$4.7 million for the prior-year period.
- **Net Loss.** Net loss was \$27.0 million, or \$0.44 loss per share, for the quarter ended June 30, 2024, compared to \$15.6 million or \$0.35 loss per share for the prior-year period.

About ENPP1 Deficiency

ENPP1 Deficiency is a serious and progressive rare disease that affects blood vessels, soft tissues, and bones. Individuals who present in utero or in infancy are typically diagnosed with generalized arterial calcification of infancy (GACI Type 1), with about 50% of these infants not surviving beyond six months. Children with this condition typically develop rickets, specifically autosomal-recessive hypophosphatemic rickets type 2 (ARHR2), while adolescents and adults may develop osteomalacia, or softened bones. ARHR2 and osteomalacia cause pain and difficulty with movement. Additionally, patients may experience hearing loss, calcification in arteries and joints, and heart problems.

Biallelic ENPP1 Deficiency affects approximately 1 in 64,000 pregnancies worldwide. Initially, it was believed to only impact individuals with two copies of the mutated gene. However, many individuals with just one copy of the mutated gene (monoallelic ENPP1 Deficiency) also exhibit severe symptoms. This suggests that the worldwide prevalence of ENPP1 Deficiency may be much higher than current estimates, which are based solely on biallelic cases. Currently, there are no approved therapies for ENPP1 Deficiency.

About ABCC6 Deficiency

ABCC6 Deficiency is a progressive and debilitating rare disease that affects blood vessels and soft tissues. Infants with ABCC6 Deficiency are diagnosed with generalized arterial calcification of infancy (GACI Type 2), which is similar to GACI Type 1, the infant form of ENPP1 Deficiency. Pediatric patients who survive beyond the first year of life may develop neurological disease, including strokes, and cardiovascular diseases due to ongoing vascular calcification and stenosis. In older individuals, ABCC6 Deficiency manifests as pseudoxanthoma elasticum (PXE), characterized by abnormal mineralization in blood vessels and soft tissues, affecting the skin, visual function, and vascular system.

Biallelic ABCC6 Deficiency is estimated to affect 1 in 25,000 to 1 in 50,000 individuals worldwide. Initially, it was believed to only impact individuals with two copies of the mutated gene. However, many people with just one copy of the mutated gene (monoallelic ABCC6 Deficiency) also exhibit severe symptoms. This suggests that the worldwide prevalence of ABCC6 Deficiency may be much higher than current estimates, which are based solely on biallelic cases. Currently, there are no approved therapies for ABCC6 Deficiency.

About Calciophylaxis

Calciophylaxis (also known as calcific uremic arteriopathy, CUA) 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 inorganic 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 calciophylaxis have a reported one-year survival rate of approximately 50%. The estimated incidence of calciophylaxis is approximately 3.5 per 1,000 patients with ESKD with approximately 5,000 new patients presenting annually across major addressable markets. Currently, there are no approved therapies for calciophylaxis.

About Inozyme Pharma

Inozyme Pharma is a pioneering clinical-stage biopharmaceutical company dedicated to developing innovative therapeutics for rare diseases that affect bone health and blood vessel function. We are experts in the PPI-Adenosine Pathway, where the ENPP1 enzyme generates inorganic pyrophosphate (PPi), which regulates mineralization, and adenosine, which controls intimal proliferation (the overgrowth of smooth muscle cells inside blood vessels). Disruptions in this pathway impact the levels of these molecules, leading to severe musculoskeletal, cardiovascular, and neurological conditions, including ENPP1 Deficiency, ABCC6 Deficiency, calciophylaxis, and ossification of the posterior longitudinal ligament (OPLL).

Our lead candidate, INZ-701, is an ENPP1 Fc fusion protein enzyme replacement therapy (ERT) designed to increase PPi and adenosine, enabling the potential treatment of multiple diseases caused by deficiencies in these molecules. It is currently in clinical development for the treatment of ENPP1 Deficiency, ABCC6 Deficiency, and calciophylaxis. By targeting the PPI-Adenosine Pathway, INZ-701 aims to correct pathological mineralization and intimal proliferation, addressing the significant morbidity and mortality in these devastating diseases.

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, availability of data from clinical trials, the potential benefits of INZ-701, our regulatory strategy, including our planned pathway to approval for the 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 ENPP1 Deficiency, ABCC6 Deficiency, and calciphylaxis; 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.

**Condensed Consolidated Balance Sheet Data
(Unaudited)**

	June 30, 2024	December 31, 2023
Cash, cash equivalents and investments	\$ 144,523	\$ 188,589
Total Assets	\$ 155,712	\$ 200,847
Total Liabilities	\$ 61,292	\$ 60,368
Additional paid-in-capital	\$ 430,827	\$ 426,362
Accumulated deficit	\$ (336,310)	\$ (285,930)
Total stockholders' equity	\$ 94,420	\$ 140,479

**Condensed Consolidated Statements of Operations and Comprehensive Loss
(Unaudited)**

	Three Months Ended June 30,	
	2024	2023
Operating expenses:		
Research and development	\$ 21,758	\$ 11,666
General and administrative	5,907	4,728
Total operating expenses	27,665	16,394
Loss from operations	(27,665)	(16,394)
Other income (expense):	—	—
Interest income	2,030	1,610
Interest expense	(1,395)	(771)
Other expenses	(3)	(28)
Other income (expense), net	632	811
Net loss	\$ (27,033)	\$ (15,583)
Other comprehensive income (loss):		
Unrealized gains (losses) on available-for-sale securities	3	76
Foreign currency translation adjustment	(1)	11
Total other comprehensive income (loss)	2	87
Comprehensive loss	\$ (27,031)	\$ (15,496)
Net loss attributable to common stockholders—basic and diluted	\$ (27,033)	\$ (15,583)
Net loss per share attributable to common stockholders—basic and diluted	\$ (0.44)	\$ (0.35)
Weighted-average common shares outstanding—basic and diluted	61,943,960	44,860,279

	Six Months Ended June 30,	
	2024	2023
Operating expenses:		
Research and development	\$ 40,868	\$ 23,523

General and administrative	11,141	11,240
Total operating expenses	52,009	34,763
Loss from operations	(52,009)	(34,763)
Other income (expense):		
Interest income	4,404	2,937
Interest expense	(2,720)	(1,099)
Other expenses	(55)	(62)
Other income (expense), net	1,629	1,776
Net loss	\$ (50,380)	\$ (32,987)
Other comprehensive income (loss):		
Unrealized gains (losses) on available-for-sale securities	(153)	226
Foreign currency translation adjustment	9	30
Total other comprehensive income (loss)	(144)	256
Comprehensive loss	\$ (50,524)	\$ (32,731)
Net loss attributable to common stockholders—basic and diluted	\$ (50,380)	\$ (32,987)
Net loss per share attributable to common stockholders—basic and diluted	\$ (0.81)	\$ (0.74)
Weighted-average common shares outstanding—basic and diluted	61,858,119	44,293,577

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