# Inozyme Pharma Reports Third Quarter 2024 Financial Results and Provides Business Highlights

November 5, 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 –*

*- Topline data from ENERGY 3, a pivotal trial of INZ-701 in pediatric patients with ENPP1 Deficiency, expected in early 2026 -*

*- Company plans to initiate registrational trials in calciphylaxis and ABCC6 Deficiency in 2025 subject to regulatory alignment and sufficient funding -*

*- Cash, cash equivalents, and short-term investments as of September 30, 2024, expected to fund operations into the fourth quarter of 2025 –*

BOSTON, Nov. 05, 2024 (GLOBE NEWSWIRE) -- **Inozyme Pharma, Inc.** (Nasdaq: INZY) ("the Company" or "Inozyme"), a clinical-stage biopharmaceutical company developing innovative therapeutics for rare diseases that affect bone health and blood vessel function, today reported financial results for the third quarter ended September 30, 2024, and provided business highlights.

"As we close in on the end of a highly productive year, our focus remains firmly on advancing INZ-701 across each of our clinical programs," said Douglas A. Treco, Ph.D., CEO and Chairman of Inozyme Pharma. "The recent presentation of promising interim data from our Phase 1 data from our calciphylaxis program highlights our progress, and we remain on track to report interim data from our Phase 1b ENERGY 1 trial in ENPP1 Deficiency by year-end. With a growing body of clinical evidence supporting the potential for INZ-701 to serve as a meaningful therapy across multiple indications of high unmet need, we are committed to our mission to bring novel treatment options to patients facing rare diseases affecting bone health and blood vessel function."

**Recent Highlights**

*Pipeline*

- **Presentations at American Society for Bone and Mineral Research (ASBMR) 2024 Annual Meeting.** In September 2024, the Company presented new data at ASBMR 2024 in Toronto, Canada, demonstrating the progression and impact of ENPP1 Deficiency and ABCC6 Deficiency in children. These findings underscored the urgent need for innovative therapies to address the severe cardiovascular and musculoskeletal complications associated with these conditions. In addition, the Company and GACI Global highlighted the launch of the PROPEL Registry designed to further understanding of the burden of illness and progressive nature of ENPP1 Deficiency and early-onset ABCC6 Deficiency (NCT06302439).

*ENPP1 Deficiency*

- **ENPP1 Deficiency Review Series Publication**. In September 2024, a series of comprehensive review articles on ENPP1 Deficiency was published in the French journal of pediatrics, *Archives de Pédiatrie*. Authored by expert clinicians and researchers in bone health, the articles collectively highlight the complexities of ENPP1 Deficiency, underscoring both established and emerging insights into the disease's presentation and management.

*Calciphylaxis*

- **Phase 1 Trial of INZ-701 in Patients with End-Stage Kidney Disease (ESKD) Undergoing Hemodialysis.** In October 2024, the Company announced positive interim data demonstrating INZ-701 was well-tolerated and significantly increased plasma pyrophosphate (PPi) levels in patients with ESKD on dialysis. Low PPi levels are linked to the development of calciphylaxis, a rare and life-threatening complication of ESKD, as well as the associated morbidity and mortality. Data were featured at the American Society of Nephrology's *Kidney Week 2024* in San Diego, CA. Subject to regulatory review and sufficient funding, the Company plans to initiate a registrational study in calciphylaxis in 2025.

*Corporate*

- **Board of Directors Appointment.** In October 2024, the Company announced the appointment of Erik Harris to its Board of Directors. Mr. Harris, who currently serves as Chief Commercial Officer and Executive Vice President at Ultragenyx Pharmaceutical Inc., brings to the Company over 20 years of commercial expertise within the biopharmaceutical industry.

**Anticipated Milestones**

- **ENPP1 Deficiency**
  - Complete enrollment of ENERGY 3 pivotal trial in pediatric patients by the end of 2024
  - Initiation of the ENERGY 2 pivotal trial in infants with ENPP1 Deficiency outside the United States in the fourth quarter of 2024
  - Release interim data from the ENERGY 1 Phase 1b trial in infants in the fourth quarter of 2024
  - Release topline data from the ENERGY 3 pivotal trial in pediatric patients in early 2026

- **ABCC6 Deficiency**
  - Initiation of pivotal clinical trial of INZ-701 in pediatric patients with ABCC6 Deficiency in 2025, subject to regulatory

alignment and sufficient funding.

- **Calciphylaxis**
  - Initiation of a pivotal trial of INZ-701 in patients with calciphylaxis in 2025, subject to regulatory alignment and sufficient funding.

**Third Quarter 2024 Financial Results**

- **Cash Position and Financial Guidance.** Cash, cash equivalents, and short-term investments were $131.6 million as of September 30, 2024. Based on its current plans, the Company anticipates its cash, cash equivalents, and short-term investments as of September 30, 2024, will enable the Company to fund cash flow requirements into the fourth quarter of 2025.
- **Research and Development (R&D) Expenses.** R&D expenses were $19.9 million for the quarter ended September 30, 2024, compared to $13.3 million for the prior-year period.
- **General Administrative (G&A) Expenses.** G&A expenses were $5.0 million for the quarter ended September 30, 2024, compared to $4.7 million for the prior-year period.
- **Net Loss.** Net loss was $24.6 million, or $0.39 net loss per share, for the quarter ended September 30, 2024, compared to $16.6 million or $0.29 net 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 Calciphylaxis and the PPi-Adenosine Pathway**
Calciphylaxis (also known as calcific uremic arteriolopathy, or CUA) is a rare disorder with a high mortality rate that predominantly 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. This leads to poor blood flow, blood clots, painful skin ulcers, serious infections, and often death, with a reported one-year survival rate of approximately 50%. Currently, there are no approved therapies for calciphylaxis. 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 addressable markets.

The PPi-Adenosine Pathway plays a critical role in regulating both pathologic mineralization and intimal proliferation. The ENPP1 enzyme generates PPi, a potent inhibitor of pathologic mineralization, by hydrolyzing extracellular adenosine triphosphate. Additionally, adenosine, produced by the CD73 enzyme regulates intimal proliferation, preventing the abnormal growth of smooth muscle cells within blood vessels, which can contribute to vascular occlusion. Recent genetic research has shown that polymorphisms in the *ENPP1* or *CD73* genes have been linked to an increased risk of arterial calcification in ESKD patients and/or calciphylaxis, further substantiating the role of the PPi-Adenosine Pathway in this condition.

INZ-701 is designed to restore PPi levels and increase adenosine production, addressing both key elements of the PPi-Adenosine Pathway. By normalizing these processes, INZ-701 has the potential to prevent the progression of calciphylaxis, which could offer a promising therapeutic solution for this high-risk and underserved patient population.

**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, calciphylaxis, 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 calciphylaxis. 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 and calciphylaxis programs, 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)**

| | September 30, 2024 | December 31, 2023 |
|---|---|---|
| Cash, cash equivalents and investments | $ 131,608 | $ 188,589 |
| Total assets | $ 143,361 | $ 200,847 |
| Total liabilities | $ 60,573 | $ 60,368 |
| Additional paid-in-capital | $ 443,476 | $ 426,362 |
| Accumulated deficit | $ (360,880) | $ (285,930) |
| Total stockholders' equity | $ 82,788 | $ 140,479 |

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

| | Three Months Ended September 30, | |
|---|---|---|
| | 2024 | 2023 |
| **Operating expenses:** | | |
| Research and development | $ 19,890 | $ 13,341 |
| General and administrative | 4,961 | 4,733 |
| Total operating expenses | 24,851 | 18,074 |
| Loss from operations | (24,851) | (18,074) |
| Other income (expense): | | |
| Interest income | 1,778 | 2,369 |
| Interest expense | (1,416) | (953) |
| Other (expense)income, net | (81) | 20 |
| Other income, net | 281 | 1,436 |
| **Net loss** | $ (24,570) | $ (16,638) |
| Other comprehensive income (loss): | | |
| Unrealized gains on available-for-sale securities | 290 | 53 |
| Foreign currency translation adjustment | (1) | (182) |
| Total other comprehensive income (loss) | 289 | (129) |
| **Comprehensive loss** | $ (24,281) | $ (16,767) |
| Net loss attributable to common stockholders—basic and diluted | $ (24,570) | $ (16,638) |
| Net loss per share attributable to common stockholders—basic and diluted | $ (0.39) | $ (0.29) |
| Weighted-average common shares outstanding—basic and diluted | 63,276,851 | 56,758,395 |

| | Nine Months Ended September 30, | |
|---|---|---|
| | 2024 | 2023 |
| **Operating expenses:** | | |
| Research and development | $ 60,758 | $ 36,864 |
| General and administrative | 16,101 | 15,973 |
| Total operating expenses | 76,859 | 52,837 |

| | | |
|---|---|---|
| Loss from operations | (76,859) | (52,837) |
| Other income (expense): | | |
| Interest income | 6,182 | 5,306 |
| Interest expense | (4,136) | (2,052) |
| Other expense, net | (137) | (42) |
| Other income (expense), net | 1,909 | 3,212 |
| **Net loss** | $ (74,950) | $ (49,625) |
| Other comprehensive income (loss): | | |
| Unrealized gains on available-for-sale securities | 137 | 279 |
| Foreign currency translation adjustment | 8 | (152) |
| Total other comprehensive income | 145 | 127 |
| **Comprehensive loss** | $ (74,805) | $ (49,498) |
| Net loss attributable to common stockholders—basic and diluted | $ (74,950) | $ (49,625) |
| Net loss per share attributable to common stockholders—basic and diluted | $ (1.20) | $ (1.02) |
| Weighted-average common shares outstanding—basic and diluted | 62,334,482 | 48,494,175 |

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