Inozyme Pharma Reports Third Quarter 2020 Financial Results and Provides Business Highlights

November 12, 2020

– Submitted CTA for INZ-701 for the treatment of ENPP1 deficiency to United Kingdom regulatory agency –

– Received Rare Pediatric Disease and Fast Track Designations for INZ-701 for the treatment of ENPP1 deficiency –

– Expect to initiate INZ-701 Phase 1/2 clinical trials for ENPP1 and ABCC6 deficiencies in first half of 2021 –

BOSTON, Nov. 12, 2020 (GLOBE NEWSWIRE) -- **Inozyme Pharma, Inc. (Nasdaq: INZY),** a rare disease biopharmaceutical company developing novel therapeutics for the treatment of diseases of abnormal mineralization impacting the vasculature, soft tissue and skeleton, today reported financial results for the third quarter ended September 30, 2020 and provided recent business highlights.

"ENPP1 deficiency is a systemic, progressive and continuous disease occurring over the course of a patient's lifetime, starting as early as fetal development and spanning into adulthood. The fact that INZ-701 had previously received orphan drug designation and now rare pediatric disease and fast track designations underscores the significant unmet medical need for a treatment for this disease," said Axel Bolte, MSc, MBA, co-founder, president and chief executive officer of Inozyme Pharma. "I'm pleased with the progress we have made with U.S. and European regulatory authorities, and we remain on track to initiate our planned Phase 1/2 clinical trials in the first half of 2021, subject to clearance of our regulatory applications."

Recent Business Highlights

- **Submitted Clinical Trial Application (CTA) for INZ-701 for the treatment of ENPP1 deficiency** – Inozyme recently submitted its first CTA to initiate a Phase 1/2 clinical trial of INZ-701 for the treatment of ENPP1 deficiency to the United Kingdom's Medicines and Healthcare products Regulatory Agency (MHRA).
- **Received Rare Pediatric Disease Designation and Fast Track Designation from the U.S. Food and Drug Administration (FDA) for INZ-701 for the treatment of ENPP1 deficiency** – The FDA grants rare pediatric disease designation to drugs for serious and life-threatening diseases in which the serious or life-threatening manifestations primarily affect children aged from birth through 18 years and affect fewer than 200,000 people in the U.S. Under the FDA's Rare Pediatric Disease Priority Review Voucher program, a sponsor who receives approval of a biologics license application (BLA) for a rare pediatric disease product application may be eligible for a voucher which can be redeemed to obtain priority review for a subsequent marketing application for a different product. Separately, Fast Track Designation facilitates the potential expedited development and review of a drug for the treatment of a serious or life-threatening disease and that has demonstrated the potential to address unmet medical needs. Benefits of this designation include frequent engagements with the FDA to discuss the drug's clinical development plan, eligibility for priority review, and a rolling review of a BLA. Previously, the FDA and the European Medicines Agency (EMA) had granted orphan drug designation to INZ-701 for the treatment of ENPP1 deficiency.
- **Completed disease burden study in ENPP1 deficiency and ABCC6 deficiency** – Inozyme and GACI Global, a patient advocacy organization dedicated to bettering the lives of families affected by Generalized Arterial Calcification of Infancy and/or Autosomal Recessive Hypophosphatemic Rickets Type 2 (GACI/ARHR2), completed a study to characterize the burden of disease and understand the systemic progression of disease for the rare genetic diseases of both ENPP1 deficiency and ABCC6 deficiency from the perspective of a patient and/or parent. Inozyme expects to share data from this study in 2021.

Upcoming Anticipated Milestones, Subject to COVID-19 Dynamics

- **INZ-701 for ENPP1 deficiency**
  - **Early 2021:** Clearance of IND and CTAs
  - **H1 2021:** Initiation of Phase 1/2 clinical trial
  - **H1 2021:** Initiation of prospective natural history study
  - **H2 2021:** Preliminary safety and biomarker data from Phase 1/2 clinical trial
- **INZ-701 for ABCC6 deficiency**
  - **Early 2021:** Clearance of CTAs
  - **H1 2021:** Initiation of Phase 1/2 clinical trial
  - **H2 2021:** Preliminary safety and biomarker data from Phase 1/2 clinical trial

Upcoming Investor Conference

- Piper Sandler 32nd Annual Healthcare Conference, November 30 – December 3, 2020

Third Quarter 2020 Financial Results

- **Cash Position and Financial Guidance** – Cash, cash equivalents and investments were $171.7 million as of September 30, 2020. Based on its current plans, the Company expects that its existing cash, cash equivalents and investments will be sufficient to enable it to fund its operating expenses and capital expenditure requirements at least into the second half of 2022.
- **Research and Development (R&D) Expenses**– R&D expenses were $25.2 million for the third quarter ended September 30, 2020, compared to $3.3 million for the same period in 2019. The increase was primarily due to an increase of $17.8 million resulting from the non-recurring, non-cash purchase of in-process research and development intellectual property assets from Alexion in exchange for stock of the Company in July 2020, costs associated with preclinical studies and clinical preparation activities with the Company's CRO, and growth in the number of R&D employees.
- **General and Administrative (G&A) Expenses –** G&A expenses were $3.1 million for the third quarter ended September 30, 2020, compared to $1.0 million for the same period in 2019. The increase was primarily due to the growth in the number of G&A employees, an increase in legal fees related to patents, new contracts and operations as a public company, and generally higher fees in areas such as audit, tax and information technology to support the Company's growth.
- **Net Loss** – Net loss was $28.1 million, or $1.55 loss per share, for the third quarter ended September 30, 2020, compared to $4.0 million, or $3.38 loss per share, for the same period in 2019.

About Inozyme Pharma

Inozyme Pharma, Inc. (Nasdaq: INZY), is a rare disease biopharmaceutical company developing novel therapeutics for the treatment of diseases of abnormal mineralization impacting the vasculature, soft tissue and skeleton. Through our in-depth understanding of the biological pathways involved in mineralization, we are pursuing the development of therapeutics to address the underlying causes of these debilitating diseases. It is well established that two genes, ENPP1 and ABCC6, play key roles in a critical mineralization pathway and that defects in these genes lead to abnormal mineralization. We are initially focused on developing a novel therapy to treat the rare genetic diseases of ENPP1 and ABCC6 deficiencies.

Inozyme Pharma was founded in 2017 by Joseph Schlessinger, Ph.D., Demetrios Braddock, M.D., Ph.D., and Axel Bolte, MSc, MBA, with technology developed by Dr. Braddock and licensed from Yale University. For more information, please visit www.inozyme.com.

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 and timing of our future clinical trials, our research and development programs, the availability of preclinical study and clinical trial data, the timing of our regulatory applications and the period over which we believe that our existing cash, cash equivalents and investments will be sufficient to fund our operating expenses. 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 successfully resolve the clinical hold with regard to its planned Phase 1/2 clinical trial of INZ-701 for ENPP1 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; 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, 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)

	September 30, 2020	December 31, 2019
Cash, cash equivalents and investments	$ 171,709	$ 47,132
Total assets	178,993	47,944
Total liabilities	11,077	3,236
Convertible preferred stock	—	77,927
Additional paid-in-capital	247,872	1,428
Accumulated deficit	(79,958)	(34,652)
Total stockholders' equity (deficit)	167,916	(33,219)

Condensed Consolidated Statements of Operations and Comprehensive Loss
(Unaudited)

(in thousands, except share and per share data)

	Three Months Ended September 30,		Nine Months Ended September 30,	
	2020	**2019**	**2020**	**2019**
Operating expenses:				
Research and development	$ 25,174	$ 3,317	$ 39,457	$ 10,941
General and administrative	3,142	1,003	6,313	3,097
Total operating expenses	28,316	4,320	45,770	14,038
Loss from operations	(28,316)	(4,320)	(45,770)	(14,038)
Other income (expense):				
Interest income	64	288	306	892
Other income (expense), net	157	(3)	158	(34)
Other income (expense), net	221	285	464	858
Net loss	$ (28,095)	$ (4,035)	$ (45,306)	$ (13,180)
Other comprehensive (loss) income:				
Unrealized (losses) gains on available-for-sale securities	(13)	(2)	(5)	8
Total other comprehensive (loss) income	(13)	(2)	(5)	8
Comprehensive loss	$ (28,108)	$ (4,037)	$ (45,311)	$ (13,172)
Net loss attributable to common stockholders—basic and diluted	$ (28,095)	$ (4,035)	$ (45,306)	$ (13,180)
Net loss per share attributable to common stockholders—basic and diluted	$ (1.55)	$ (3.38)	$ (6.57)	$ (11.20)
Weighted-average common shares outstanding —basic and diluted	18,101,496	1,195,309	6,893,745	1,176,769

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