Inozyme Pharma Announces FDA Fast Track Designation for INZ-701 in ABCC6 Deficiency

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Agency program will support and expedite clinical studies addressing severe unmet need in children with ABCC6 Deficiency

BOSTON, July 02, 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 announced that the U.S. Food and Drug Administration (FDA) has granted Fast Track designation to INZ-701 for the treatment of ABCC6 Deficiency.

"Through Fast Track designation, the FDA recognizes the potential of INZ-701 in ABCC6 Deficiency. We plan to work closely with the agency to establish an efficient path to approval. Receipt of Fast Track designation underscores our belief that INZ-701 could serve as an important therapy for patients living with ABCC6 Deficiency, notably for pediatric patients in whom this condition increases the risk of major clinical events such as stroke and severe neurological and cardiovascular disease," said Douglas A. Treco, Ph.D., CEO and Chairman of Inozyme Pharma. "We look forward to presenting our development plans to regulatory agencies and reaching agreement on a pivotal study in pediatric patients with ABCC6 Deficiency by year-end 2024."

Fast Track is an FDA program designed to facilitate and expedite the development and review of new medicines that are intended to treat or prevent serious conditions and have the potential to address an unmet medical need. The designation has been granted based on nonclinical pharmacology data and preliminary safety and efficacy data from the Company's ongoing Phase 1/2 trial of INZ-701 in adults with ABCC6 Deficiency. With Fast Track designation, the development of INZ-701 can benefit from more frequent engagement with the FDA and expedited regulatory review.

In April 2024, the Company announced positive topline safety and immunogenicity data from its ongoing Phase 1/2 trial of INZ-701 in adults with ABCC6 Deficiency. Clinical improvements were observed in vascular pathology, visual function, and patient reported outcomes (PROs). The Company also 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.

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 INZ-701

INZ-701, a recombinant Fc fusion protein, is an ENPP1 enzyme replacement 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, which can drive morbidity and mortality in devastating genetic disorders such as ENPP1 Deficiency, ABCC6 Deficiency and calciphylaxis. INZ-701 is currently in clinical development for the treatment of ENPP1 Deficiency, ABCC6 Deficiency and calciphylaxis.

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 availability of data from clinical trials, and the potential benefits of INZ-701. 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 and ABCC6 Deficiency; 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.

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