Inozyme Pharma Announces Partnership with Rady Children's Institute for Genomic Medicine to Advance Newborn Screening for Genetic Diseases

New technology aims to expand testing to approximately 1,000 diseases and sequencing to 3.7 million newborns annually

BOSTON, June 16, 2022 (GLOBE NEWSWIRE) -- Inozyme Pharma, Inc. (Nasdaq: INZY), a clinical-stage rare disease biopharmaceutical company developing novel therapeutics for the treatment of abnormal mineralization, today announced a partnership with Rady Children's Institute for Genomic Medicine (RCIGM) to advance and evaluate a novel newborn screening technology to facilitate diagnosis of genetic diseases. The partnership includes several leading genomics, biotechnology companies and patient advocacy groups and focuses specifically on a diagnostic and precision medicine guidance tool called BeginNGS™, which incorporates rapid Whole Genome Sequencing (rWGS®) to currently screen newborns for approximately 400 genetic diseases.

"Newborn screening will be essential to identifying and initiating timely intervention in children with rare genetic disorders like GACI (generalized arterial calcification of infancy) as we advance INZ-701 through clinical testing," said Catherine Nester, vice president, physician and patient strategies at Inozyme Pharma. "We look forward to working with Rady Children's Institute for Genomic Medicine, and with the BeginNGS consortium, to advance the use of this promising screening technology."

RCIGM is in a pilot evaluation that aims to supplement existing newborn screening protocols at birthing hospitals throughout the United States. The pilot program's goal is for BeginNGS to become the genetic disease screening standard, with testing expanding to approximately 1,000 [disorders] and sequencing of 3.7 million newborns annually. Founding members of the public-private BeginNGS consortium include Inozyme, Alexion, Travere Therapeutics, and several patient advocacy groups that are helping to advance this program.

"RCIGM helped pioneer the use of rWGS for diagnosis of genetic disease in intensive care settings," said Stephen Kingsmore, MD, DSc, president and CEO of RCIGM. "With the proven clinical utility of diagnostic rWGS , we are using that experience to screen, diagnose, and help treat genetic conditions at or before onset of symptoms. Through a public-private consortium of leading organizations such as Inozyme, and advocacy groups in pediatrics, genetics, biopharma, biotech, and information technology, we aim to scale newborn sequencing to every life-threatening childhood genetic disease, RCIGM believes now is the time to end the diagnostic and therapeutic odyssey for all children with treatable genetic diseases."

BeginNGS developed through a research collaboration with Alexion; AstraZeneca's Rare Disease group; Illumina, Inc.; TileDB; Fabric Genomics; and Genomemon, which uses rWGS to diagnose and identify treatment options for genetic conditions before symptoms begin. This approach represents an advance over current pediatric uses of rWGS that focus mainly on children who are already critically ill. Once a diagnosis is made, BeginNGS uses Genome-to-Treatment (GTRx™), a tool that provides immediate treatment guidelines to help physicians understand genetic conditions and their available treatment options.

Addressing the Need for Enhanced Newborn Screening Tools

Traditional newborn screening is one of the most successful public health programs in the United States. Of nearly 4 million babies born annually, 98 percent are tested in the first days of life. The BeginNGS test identifies serious

childhood diseases that have effective treatments. States currently screen for only 31 to 76 of the hundreds of severe, childhood genetic diseases that have available treatments. Adding a new condition to the screening protocol is slow (5 to 6 years per condition), laborious, and costly. In the last decade, WGS has increased in speed, diagnostic performance, and scalability. BeginNGS will not replace the current biochemical newborn screening paradigm; rather, it is designed to complement the newborn screening processes and infrastructure that are already in place.

"We are thrilled at the prospect of newborn screening to assist in early identification of infants affected by ENPP1 Deficiency and ABCC6 Deficiency via Inozyme's collaboration with Rady Children's Institute for Genomic Medicine. Early diagnosis is crucial to improving a baby's chances of survival and long-term health if they have these rare and devastating diseases," said Christine O'Brien and Liz Molloy, co-presidents of GACI Global.

About Rady Children's Institute for Genomic Medicine

Rady Children's Institute for Genomic Medicine is transforming pediatric critical care by advancing disease-specific healthcare for infants and children with rare disease. Discoveries at the Institute are enabling rapid diagnosis and targeted treatment of critically ill newborns and pediatric patients at Rady Children's Hospital-San Diego and a growing network of more than 60 children's hospitals nationwide. The vision is to expand delivery of this life-changing technology to enable the practice of Rapid Precision Medicine™ at children's hospitals across the nation and the world. RCIGM is a non-profit, research institute embedded within Rady Children's Hospital and Health Center.

About Inozyme Pharma

Inozyme Pharma, Inc. (Nasdaq: INZY) is a clinical-stage 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, INZ-701, to treat the rare genetic diseases of ENPP1 and ABCC6 Deficiencies. INZ-701 is currently in Phase 1/2 clinical trials for the treatment of ENPP1 Deficiency and ABCC6 Deficiency.

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.

About ENPP1 Deficiency

ENPP1 Deficiency is a heterogenous, progressive condition with high infant mortality in the first six months of life. Individuals who present in utero or in infancy are typically diagnosed with generalized arterial calcification of infancy (GACI), which is characterized by extensive vascular calcification and neointimal proliferation (overgrowth of smooth muscle cells inside blood vessels), resulting in stroke, cardiac or multiorgan failure. Children with ENPP1 Deficiency typically experience rickets and other skeletal manifestations, a condition also known as autosomal-recessive hypophosphatemic rickets type 2 (ARHR2). Adults may experience osteomalacia (softened bones), pain, stiffness and impaired quality of life. There are no approved therapies for ENPP1 Deficiency.

About ABCC6 Deficiency

ABCC6 Deficiency is a rare, severe, inherited disorder caused by mutations in the ABCC6 gene, leading to low levels of PPi. PPi is essential for preventing harmful soft tissue calcification and regulating bone mineralization. ABCC6 Deficiency is a systemic and progressively debilitating condition, which affects more than 67,000 individuals worldwide. Infants with ABCC6 Deficiency are diagnosed with GACI type 2, a condition that resembles GACI type 1, the infant form of ENPP1 Deficiency. In older patients, ABCC6 Deficiency presents as pseudoxanthoma elasticum (PXE), which

is characterized by pathological 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 is a clinical-stage enzyme replacement therapy in development for the treatment of mineralization disorders of the circulatory system, bones, and kidneys. In preclinical studies, the experimental therapy has shown potential to generate PPi and to restore it to appropriate physiological levels, thereby preventing calcification in the vasculature and kidneys, while at the same time normalizing bone mineralization. Inozyme is developing INZ-701 for certain rare, life-threatening, and devastating genetic disorders such as ENPP1 Deficiency and ABCC6 Deficiency in which PPi levels are below the normal physiological levels. INZ-701 is currently in Phase 1/2 clinical trials for the treatment of ENPP1 Deficiency and ABCC6 Deficiency.

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 the partnership with Rady Children's Institute for Genomic Medicine. 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 Phase 1/2 clinical trials of INZ-701 for ENPP1 Deficiency and ABCC6 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 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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6/16/2022 7:30:00 AM