Inozyme Pharma Receives Orphan Drug Designation for INZ-701 in the United States and European Union

Novel Enzyme Replacement Therapy in Development for Treatment of ENPP1 Deficiency

Boston, Mass., July 17, 2018 – Inozyme Pharma (Inozyme), a biopharmaceutical company dedicated to developing treatments for rare and debilitating diseases, today announced that both the Food and Drug Administration's (FDA) Office of Orphan Products Development and the European Medicines Agency's (EMA) Committee for Orphan Medical Products (COMP) granted Orphan Drug Designation to INZ-701 for the treatment of ENPP1 Deficiency. INZ-701, the Company's lead therapeutic candidate, is in pre-clinical development for the treatment of patients with ENPP1 Deficiency, a serious and life-threatening calcification disorder that manifests as generalized arterial calcification of infancy (GACI) in infants and as autosomal recessive hypophosphatemic rickets type 2 (ARHR2) post-infancy.

"Orphan Drug Designation, both in the United States and the European Union, is an important regulatory milestone for Inozyme as we continue our quest to develop INZ-701 for patients with rare and life-threatening calcification disorders," said Axel Bolte, co-founder and chief executive officer of Inozyme. "The dual designations from the FDA and EMA provide crucial momentum for INZ-701, putting us in an excellent position to rapidly advance the clinical development program for this novel enzyme replacement therapy."

The FDA and EMA respectively grant Orphan Drug Designation to drugs intended for safe and effective treatment of rare, life-threatening or chronically debilitating conditions that affect fewer than 200,000 people in the United States or fewer than one in 2,000 individuals in Europe. By receiving Orphan Drug Designation, Inozyme qualifies for certain regulatory and financial incentives, including scientific assistance, fee reductions, tax credits and seven years of market exclusivity in the U.S., as well as 10 years of market exclusivity post-authorization in the European Union.

About ENPP1 Deficiency

The *ENPP1* gene produces a critical enzyme called ectonucleotide pyrophosphatase/ phosphodiesterase 1 (ENPP1), which regulates inorganic pyrophosphate (PPi) levels in plasma. PPi is essential for preventing harmful soft tissue calcification and for regulating normal bone mineralization. ENPP1 Deficiency manifests as either generalized arterial calcification of infancy (GACI) type 1 or autosomal recessive hypophosphatemic rickets type 2 (ARHR2). GACI type 1 is a devastating and often fatal disease affecting infants and is characterized by calcification and narrowing of large and medium-sized arteries, resulting in heart failure and death in about half

of patients within the first six months of life. ARHR2 manifests in the post-infancy stage and causes rickets, weakened bones, repeated bone fractures, skeletal deformities, short stature, muscle weakness, fatigue, and bone pain.

About INZ-701

INZ-701 is an enzyme replacement therapy under development with the intention to be used for the treatment of calcification disorders of the circulatory system, bones, and kidneys. In pre-clinical studies, the experimental therapy has shown potential to generate plasma pyrophosphate (PPi) and to restore it to appropriate physiological levels, thereby preventing calcification in the vasculature and kidneys and normalizing bone.

Inozyme is developing INZ-701 for certain rare, life-threatening and devastating genetic disorders such as ENPP1 Deficiency (GACI and ARHR2) and pseudoxanthoma elasticum (PXE) in which PPi levels are below the normal physiological levels. For more information about INZ-701, please visit: http://www.inozyme.com/our-science/.

About Inozyme Pharma

Inozyme Pharma is a biotechnology company committed to developing novel medicines for the treatment of rare metabolic diseases of calcification. The company was founded in 2016 with technology licensed from Yale University. For more information, please visit: www.inozyme.com.

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