Synlogic Proprietary Synthetic Biotic Receives FDA Orphan Drug Designation

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The Company Has Developed The First Live *E. Coli* Nissle Bacterium Modified To Assimilate Ammonia For The Potential Treatment Of Urea Cycle Disorders

CAMBRIDGE, Mass.–(BUSINESS WIRE)–Synlogic, a privately-held biopharmaceutical company developing novel medicines based on its proprietary synthetic biology and microbiome platform, today announced that the U.S. Food and Drug Administration (FDA) has granted orphan drug designation to Synlogic's investigational program involving a live *E. coli* Nissle bacterium modified to assimilate ammonia for treatment of urea cycle disorders (UCDs). UCDs are a family of rare but serious genetic mutations that allow the build-up of toxic levels of ammonia in the bloodstream, causing severe neurological complications and even death. Synlogic's lead candidate, SYNB1020, is currently in preclinical development and the company plans to file an investigational new drug application (IND) with the FDA for the potential treatment of UCDs in the first quarter of 2017.

"We are pleased that the FDA recognizes the significant need that exists among children and adults living with UCD," said Jose Carlos Gutierrez-Ramos, CEO of Synlogic. "Receiving orphan drug designation is an important step forward in our shared goal of bringing this novel treatment approach to the patients and families affected by urea cycle disorders. We look forward to evaluating our lead candidate SYNB1020 for the potential treatment of this debilitating disease."

The FDA's Orphan Drug program offers orphan status to drugs and biologics that are intended for the treatment of rare diseases affecting fewer than 200,000 people in the U.S. The designation provides sponsors with development and commercial incentives for designated compounds and medicines, including eligibility for a seven-year period of market exclusivity in the U.S. after product approval, FDA assistance in clinical trial design and an exemption from FDA user fees.

About Urea Cycle Disorders

UCDs are rare but serious, potentially fatal, genetic disorders caused by mutations in one or more genes encoding enzymes in the urea cycle. These genetic mutations lead to the build-up of toxic levels of ammonia in the blood that can cause significant neurological and behavioral complications. The overall frequency of congenital UCDs varies within literature and is most conservatively estimated at 1 per 8000 births. UCD patients are prone to significant morbidity and mortality, primarily due to neurocognitive and developmental abnormalities and frequently suffer from hepatic abnormalities. Chronic management of abnormal plasma ammonia levels (hyperammonemia) in UCD patients involves long-term dietary modification with nutritional oversight to restrict protein intake, thereby minimizing the flux of nitrogen through the urea cycle and preventing acute episodes of hyperammonemic crises. Despite recent advances in the acute and chronic management of UCDs, current treatment strategies are not sufficient, and reducing the concentration of blood ammonia in patients with hyperammonemia still represents a significant medical need. A truly transformative therapy for patients with UCDs would be that of maintaining safe concentrations of blood ammonia levels to the upper limit of normal, while allowing for diets with normal or less restrictive protein intake. SYNB1020 is a live probiotic bacterium that is designed to provide enhanced ability to metabolize excess ammonia in the gut by modifying a widely used probiotic *E. coli* Nissle.

About Synlogic

Synlogic is a privately-held biopharmaceutical company based in Cambridge, Massachusetts, pioneering the development of a novel class of therapeutics, called synthetic biotics, based on its proprietary synthetic biology and microbiome platform. Synlogic's two lead therapeutic programs are being developed for the potential treatment of rare inborn errors of metabolism of Urea Cycle Disorder (UCD) and Phenylketonuria (PKU). In addition to the company's proprietary pipeline focused on rare diseases, the company is leveraging the broad potential of its synthetic biotics platform for novel drug development in major disease areas through partnerships with pharmaceutical and biotechnology companies. Synlogic is collaborating with AbbVie to develop synthetic biotics-based treatment for inflammatory bowel disease (IBD). Synlogic is backed by leading life sciences investors, including Atlas Venture, New Enterprise Associates (NEA), Orbimed, Deerfield and the Bill & Melinda Gates Foundation. For more information, please visit http://synlogictx.com/.

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