NEWS RELEASE

data published in nature communications demonstrates coa therapeutics' potential to treat genetically driven pantothenate kinase-associated neurodegeneration (pkan)

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PALO ALTO, Calif., Oct. 23, 2018 /PRNewswire/ — **CoA Therapeutics**, a company developing small molecules that target Coenzyme-A for patients with PKAN and other diseases of CoA sequestration, today announced key data published by researchers from St. Jude Children's Research Hospital in Nature Communications demonstrating the potential of small molecule activators of pantothenate kinase for the treatment of pantothenate kinase-associated neurodegeneration (PKAN) and other diseases involving CoA sequestration. In addition, the company announced that it has nominated the development candidate molecule and will commence IND-enabling studies. Finally, CoA announced the formation of a scientific advisory board that will help guide future development.

PKAN is a neurological disorder that arises from mutations in the human PANK2 gene, which codes for an enzyme called pantothenate kinase 2 (PanK2). The mutations lead to progressive difficulty with movement, speech and vision, usually beginning in childhood. There are currently no treatments approved for PKAN, which typically results in death in early adulthood.

The study, authored by St. Jude researcher **Suzanne Jackowski**, Ph.D., describes the discovery of a new class of compounds, the pantazines, that are designed to allosterically modulate pantothenate kinases, allowing PanK enzymes to make more CoA than they would typically make in an untreated system. The specific tool compound used in the study, PZ-2891, was found to be orally bioavailable and cross the blood brain barrier, a critical characteristic for treating neurological disorders like PKAN. In addition, in a mouse model of brain CoA deficiency,

animals on PZ-2891 therapy gained weight, had improved locomotor activity and a longer life span, suggesting the compound could be a novel treatment approach for PKAN. For her role in advancing research in the field, Dr. Jackowski was awarded the Lippman Medal from the International Association for Cellular Coenzymes in July 2018.

“Dr. Jackowski’s novel approach to modulating PanK enzymes is the scientific foundation of CoA Therapeutics. In this study, we see the potential of novel allosteric modulators of PanK in increasing brain CoA levels resulting in phenotypic correction in knock-out mice. These compounds have been designed to be highly brain penetrating which is critical for diseases primarily affecting the brain such as PKAN,” said Shafique Virani, M.D., chief executive officer for CoA Therapeutics. “We will be moving rapidly to translate this exciting science to support entry of this therapeutic approach into clinical trials in PKAN and other diseases of CoA sequestration.”

Dr. Jackowski, who is a member of the faculty of St. Jude Children’s Research Hospital, will be joining CoA’s scientific advisory board. In addition to Dr. Jackowski, **Hyder Jinnah**, M.D., professor at Emory University School of Medicine; **Nicola Longo**, M.D., Ph.D., professor of pediatrics at the University of Utah, and **Vernon R. Sutton**, M.D., professor of molecular and human genetics at Baylor College of Medicine will join the advisory board.

About CoA Therapeutics

CoA Therapeutics, a subsidiary of BridgeBio Pharma, is developing small molecules as targeted therapeutics for PKAN and other diseases involving CoA sequestration. The company’s mission is to improve and prolong the lives of patients suffering from PKAN and other CoA-implicated genetic disorders. Founded in 2017, CoA Therapeutics is led by a team of veteran biotechnology executives. Together with patients and physicians, the company aims to bring a safe, effective treatment to market as quickly as possible. To learn more, visit **www.coatherapeutics.com**.