



NEWS RELEASE

## bridgebio pharma launches coa therapeutics to target coenzyme-a for rare genetic disorders

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PALO ALTO, Calif., June 13, 2018 /PRNewswire/ — **BridgeBio Pharma** today announced the launch of CoA Therapeutics, a biopharmaceutical company developing novel small-molecules designed to increase Coenzyme-A (CoA) levels in genetic disorders where CoA deficiency is implicated. BridgeBio has committed funding to drive the program toward clinical studies. CoA Therapeutics' lead compound will be entering the clinic in 2019 with a planned initial focus on pantothenate kinase-associated neurodegeneration, or PKAN.

PKAN is an autosomal recessive genetic disorder caused by mutations in the pantothenate kinase 2 (PANK2) gene. The PANK2 enzyme plays a critical role in the synthesis of CoA, which is crucial in energy metabolism and implicated in a large number of developmental disorders. Patients with PKAN either have a complete absence or a significant deficiency of the PANK2 enzyme, which may lead to reduced CoA levels in the brain. There are currently no treatments approved for PKAN.

The CoA Therapeutics' lead compounds, which were obtained under a license from St. Jude Children's Research Hospital, were discovered and developed by St. Jude investigators Suzanne Jackowski, Ph.D., Charles Rock, Ph.D., Richard Lee, Ph.D., and Stephen White, Ph.D.

The prevalence of PKAN is estimated to be three in every 1,000,000 people. In the typical form of PKAN, symptoms present before the age of 10, and patients may rapidly experience neuronal degeneration, causing problems with movement, speech and vision.

"PKAN is a progressive, debilitating disease with no current approved therapy, and patients and their families

currently rely on supportive treatments, which partially address the symptoms but not the root cause,” said Shafique Virani, M.D., CEO of CoA. “We believe that our novel approach, using a highly brain-penetrant compound to directly target enzyme activity in neurons, can safely increase CoA levels, ease patients’ symptoms and make a meaningful difference in their quality of life. Our novel approach also holds promise for other diseases with defects in CoA metabolism, including the organic acidemias.”

Joining Dr. Virani is Adam Shaywitz, M.D., Ph.D., who will serve as chief medical officer at CoA as well as CMO-in-residence at BridgeBio. Shaywitz was most recently executive director in clinical sciences at BioMarin Pharmaceuticals where he was involved in the design and planning of a number of clinical studies in rare disease including serving as program lead for the natural history and clinical treatment studies in Sanfilippo Syndrome B (MPS IIIB).

“We are privileged to be working with Drs. Jackowski, Rock, Lee and White, who have been at the forefront of CoA metabolism research in health and disease,” said Neil Kumar, Ph.D., CEO of BridgeBio. “Their novel approach, which relies on increasing activity of the PANK enzyme family, is a great example of an allosteric agonist approach to potentially treat patients who have very few options.”

#### About BridgeBio Pharma

BridgeBio is a clinical-stage biotech company developing novel, genetically targeted therapies to improve the lives of patients. The BridgeBio approach combines a traditional focus on drug development with a unique corporate model, allowing rapid translation of early stage science into medicines that treat disease at its source. Founded in 2015 by a team of industry veterans, the company has built a robust portfolio of nineteen transformative assets, each housed in its own subsidiary, ranging from pre-clinical to late stage development in multiple therapeutic areas including oncology, cardiology, neurology, dermatology and endocrinology. The company’s focus on scientific excellence and rapid execution aims to translate today’s discoveries into tomorrow’s medicines.

#### About CoA Therapeutics

CoA Therapeutics, a subsidiary of BridgeBio Pharma, is developing a small molecule as a targeted therapeutic for PKAN. 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.