15 March 2024

**Pause in Phase 2/3 clinical study in PKAN, CoA conducting additional research on BBP-671**

Dear Members of the Pantothenate kinase-associated neurodegeneration (PKAN) Community,

CoA Therapeutics ("CoA") was founded in 2018 with the aim of developing novel therapies for difficult to treat rare genetic disorders by harnessing advances in understanding of the coenzyme A pathway. Our investigational small molecule therapy, BBP-671, is in development for individuals with PKAN as well as propionic and methylmalonic acidemia (PA and MMA) through research led by St Jude Children's Research Hospital in Memphis, Tennessee. BBP-671 is "investigational" which means that the potential therapy being studied has not been approved by any regulatory agency in any country for the treatment of PA and/or MMA or PKAN.

In April 2021, the first-in-human Phase 1 study of BBP-671, CoA-101, was conducted in 77 healthy volunteers to provide single- and multiple-dose safety and tolerability data. Pharmacokinetic (PK) and pharmacodynamic (PD) data to support future development of BBP-671 were also gathered. Based on these data, in June 2022 we launched the patient cohort of our CoA-101 study of BBP-671 (NCT04836494), which was designed to assess the safety and tolerability of BBP-671 in individuals with PA and MMA, a group of disorders in which the coenzyme A is affected. This study was designed to look at the safety and tolerability of multiple doses of BBP-671 in up to 16 individuals with PA and MMA, as well as to evaluate the potential effect of BBP-671 on selected exploratory disease-related biomarkers.

Although this study was evaluating BBP-671 in PA and MMA, every study allows us to learn more about BBP-671, and this study was intended to provide valuable information to support the development of our pivotal Phase 2/3 clinical study in PKAN. In particular, we had hoped that results from this study would provide information on different dose levels of BBP-671 that could inform dose selection for our PKAN study and longer-term safety data with BBP-671 in humans.

Unfortunately, there are many challenges with researching investigational treatments in ultra-rare genetic conditions such as PA, MMA, and PKAN. Enrollment in small patient populations can present a challenge. In the CoA-101 study for BBP-671, we did not reach sufficient enrollment and were not able to gather all the information we hoped to collect. Due to these challenges, the study was closed.

Closing the PA/MMA study impacts our plans for our Phase 2/3 PKAN study. Since we did not gather the information needed, it is necessary for us to do additional laboratory studies of BBP-671 in cells, tissues, and/or animals. While we do these additional studies, we have determined that it is unfortunately necessary to pause the start of Phase 2/3 PKAN study. At this time, we do not have a new estimated start date for the study. During this time, we are also reevaluating the design of our Phase 2/3 PKAN study.

We are disappointed to share this news, but we understand the importance of keeping the community informed. We will continue to keep the community updated regarding our progress on the development of BBP-671 as it becomes available.

Sincerely,
The CoA Therapeutics Team