

May 2023



Dear Canavan Community,

Aspa Therapeutics, a BridgeBio company, shared additional encouraging [data](#) from the CANaspire gene therapy trial during an invited presentation at the 26<sup>th</sup> Annual Meeting of the American Society of Gene and Cell Therapy (ASGCT). The ASGCT holds this scientific conference to showcase the latest research from international experts in gene and cell therapy. Dr. Florian Eichler, principal investigator of the CANaspire clinical trial, presented new findings from the study on Saturday, May 20, 2023. The presentation summarized early results from the first six CANaspire participants who received Aspa's investigational gene therapy, BBP-812. The main findings presented:

#### **N-acetylaspartate (NAA)**

- All participants showed a rapid and lasting decrease in levels of NAA, a key chemical marker which is elevated in children with Canavan disease, after dosing with BBP-812. The reduced levels of NAA were measured in the urine, cerebrospinal fluid (CSF), and brain of all participants. Lower NAA levels have been observed for over a year in the earliest dosed participants. A reduction in NAA levels is an indicator that the investigational gene therapy may be able to influence the disease.
- Aspa's CANinform natural history study collects information from untreated children affected by Canavan disease. Information from the natural history study as well as prior scientific research suggest that the NAA levels of CANaspire participants after receiving BBP-812 at this point are generally consistent with levels seen in individuals with less severe disease.

#### **Magnetic Resonance Imaging (MRI): White Matter**

- Brain MRI scans of participants after dosing demonstrate improvement in myelin, also known as white matter (a type of tissue that is essential for brain function and does not form properly in Canavan disease).

#### **Motor Function**

- Positive changes in sitting ability and head control have also been observed using multiple assessments including the Gross Motor Function Measure-88 (GMFM-88), a clinical outcome measure used by physical therapists to assess movement function in children.

Aspa Therapeutics continues to enroll new participants for the CANaspire gene therapy clinical trial. To be considered for screening for potential participation, a child must meet the following criteria:

- 30 months of age or less at the time of dosing
- Clinical, biochemical, and genetic diagnosis of Canavan disease
- Agreement of the clinical investigator(s) on potential eligibility

We at Aspa Therapeutics are grateful for the continued partnership with advocacy organizations and families of the Canavan community as we work together to advance a more meaningful understanding of the natural history of Canavan disease as well as a potential therapeutic option for affected children. A special thanks to all the children and families who have participated and expressed interest in participating in these important studies. Updates about the program can always be found on [www.treatcanavan.com](http://www.treatcanavan.com), <https://clinicaltrials.gov/ct2/show/NCT04998396>, or [www.aspatx.com](http://www.aspatx.com). Questions and comments are welcome. Please write to [canaspire@aspatx.com](mailto:canaspire@aspatx.com).

Sincerely,

The Aspa Therapeutics Team