October 13, 2022

CAN*aspire* Gene Therapy Trial for Canavan Disease

Aspa Therapeutics, a BridgeBio company, shared additional encouraging data from the CAN*aspire* gene therapy trial during a poster presentation at the Child Neurology Society meeting held October 12-15, 2022 in Cincinnati, Ohio. The complete press release can be found here.

Ongoing data collected from the first three participants show levels of N-acetylaspartate (NAA), a chemical marker elevated in Canavan disease and measured in the urine, cerebrospinal fluid (CSF), and the brain, remain markedly lower than NAA levels recorded prior to dosing. A reduction in NAA levels throughout the body is an indicator of the gene therapy's potential influence on the disease. Of course, more data are needed to establish the full efficacy and safety of the therapy.

> *"The most critical biomarker, in my mind, is NAA. Reduction in NAA levels, whether in the brain, CSF fluids, or in the urine, may indicate a sign of improvement because the gene missing in Canavan disease directly leads to accumulation of NAA. So, showing that NAA is decreasing is very important."*
>
> Guangping Gao, Ph.D., Scientific Founder of Aspa Therapeutics

Aspa Therapeutics is grateful for the ongoing collaboration with families and advocacy organizations in the Canavan community as we work together to advance meaningful therapeutic options for families affected by Canavan disease.

- Canavan Foundation
- Canavan Research Illinois
- National Tay-Sachs & Allied Diseases Association (NTSAD)

A special thanks to all the children and families in our natural history and gene therapy trials, as well as those who have expressed interest in participating. Aspa Therapeutics continues to recruit new participants for the CAN*aspire* trial. We welcome questions and comments at canaspire@aspatx.com.

Sincerely,
The Aspa Therapeutics Team

For additional information:

- About Aspa's gene therapy program: **www.treatcanavan.com**
- About the Aspa clinical study: visit **https://clinicaltrials.gov/ct2/show/NCT04998396**

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