June 22, 2022

CAN*aspire* Gene Therapy Trial for Canavan Disease

Aspa Therapeutics, a BridgeBio company, is excited to announce that new data from the CAN*aspire* gene therapy clinical trial were shared in a recent press release. Decreases in N-acetylaspartate (NAA), a chemical marker in Canavan Disease, in the urine, cerebrospinal fluid (CSF), and the brain suggest the investigational treatment's potential impact on this disease. Details can be found here [link].

Dr. Florian Eichler, lead investigator of the CAN*aspire* trial, noted: *"To see this biochemical change suggests that we are reaching cells critical to the disease process, a milestone in this disease. The ongoing myelination seen on MRI and the new interactions witnessed between children and their parents are both encouraging."*

We are continuing to recruit new participants for CAN*aspire* and have an ample supply of our investigational gene therapy on hand to dose new eligible participants.

Aspa remains grateful to be able to collaborate with families and advocacy organizations in the Canavan community in the pursuit of meaningful therapeutic advances for Canavan patients.

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

We look forward to sharing updates about the progress of our trial on an ongoing basis.

Sincerely,
The Aspa Therapeutics Team

For additional information, please visit:

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

The FDA has granted Rare Pediatric Disease designation, Orphan Drug designation and Fast Track status to Aspa's investigational gene therapy for Canavan disease

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