GENE THERAPY FOR CANAVAN DISEASE

Clinical Trial Information for Families

Aspa Therapeutics, working closely with medical experts, is developing an investigational gene therapy for patients with Canavan disease. CANaspire is an open-label clinical trial and is designed to assess safety and potential effectiveness of the investigational treatment.

WHAT IS GENE THERAPY?

Gene therapy is designed to treat genetic disorders by providing the body with a working copy of the missing or faulty gene that causes the disorder.

Gene therapy uses viruses known as adeno-associated viruses or AAVs to deliver the healthy gene. AAVs are not known to cause disease in people.

Aspa's investigational gene therapy uses adeno-associated virus serotype 9 (AAV9) which is designed to deliver functional copies of the ASPA gene into the brain as well as throughout the body, to address the underlying cause of Canavan disease.

AAV gene therapy has been studied extensively by researchers around the world. Over 3,000 patients have already participated in AAV gene therapy clinical trials, and currently there are two approved AAV gene therapies for rare conditions. One of the approved treatments uses AAV9 to treat a neurological disease in children.

ABOUT ASPA'S GENE THERAPY TRIAL

To be considered for participation, patients must:

- Have a diagnosis and signs of Canavan disease
- Be 30 months of age or younger on the expected date the investigational gene therapy would be given
- Meet other requirements that the study doctor will assess during screening

Aspa's investigational gene therapy is given intravenously into the bloodstream and does not require surgery.

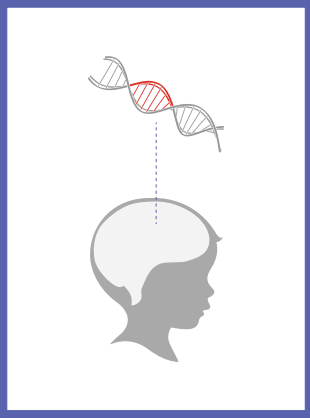
The trial will be conducted at research centers in the US and Germany.

- Participation is open to Canavan disease patients around the world

Aspa will pay for all trial-related expenses, including travel; there will be no cost to families for participating.

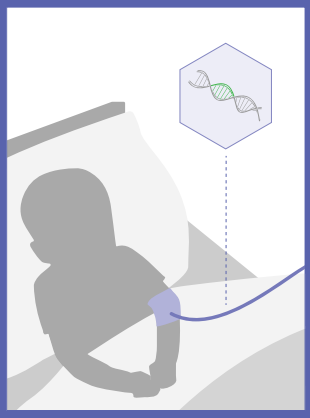
OVERVIEW OF ASPA'S INVESTIGATIONAL GENE THERAPY PROGRAM

Children with Canavan disease have mutations in the *ASPA* gene, which is responsible for making aspartoacylase, an enzyme that breaks down a compound called N-acetyl-L-aspartic acid (NAA).



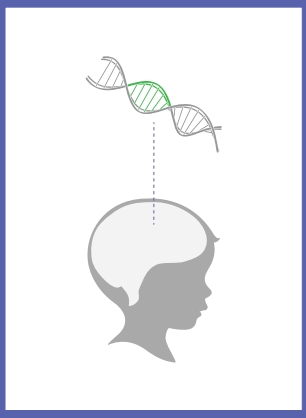
MUTATED *ASPA* GENE

NAA is found throughout the brain. Faulty NAA breakdown prevents the proper development of myelin, a substance that protects neurons and supports their function.



GENE THERAPY

Aspa's investigational gene therapy is given in a single intravenous dose and aims to provide working copies of the ASPA gene throughout the body, as well as into the brain.



FUNCTIONAL *ASPA* GENE

Once the functional ASPA gene is delivered, it may help restore myelin in the brain. This can potentially improve the course of Canavan disease by addressing the underlying genetic cause.

TO LEARN IF YOUR CHILD MAY BE ELIGIBLE:

CONTACT

1 Families can call **1-833-764-2267** (toll-free) or **1-617-861-4617** or email **CANaspire@aspatx.com** to reach a trial representative.

PRESCREEN

2 A trial representative will conduct a brief initial eligibility screening.

CONNECT

3 Families whose child meets initial eligibility criteria will be referred to the nearest available research site.

LEARN MORE ABOUT CAN*aspire*

Visit:
www.treatcanavan.com
www.clinicaltrials.gov

For questions related to Aspa's clinical program, email:
clinicaltrials@aspatx.com.

Information and support for families with Canavan disease are available from advocacy organizations such as:

NTSAD

Canavan Foundation
for the Prevention & Cure of Canavan Disease

Canavan Research Illinois