



Canavan Disease Information for Families

Aspa Therapeutics is working to develop a safe, effective gene therapy to treat patients with Canavan disease. The first step in Aspa's clinical program is CANinform, a natural history study of patients with Canavan disease that will help researchers better understand the disease and can provide a foundation for Aspa's gene therapy program.

WHAT IS A NATURAL HISTORY STUDY FOR CANAVAN DISEASE?

There are two aspects to CANinform: looking back on past medical history data (retrospective) and in-person assessments moving forward (prospective). Families affected by Canavan disease should know:

- Natural history studies like CANinform are becoming a more commonly used approach in rare diseases to understand the natural course of a disease.
- The data shared during the process may help define meaningful changes for families, physicians and regulators that can determine if a future treatment is effective.
- Families currently caring for a child and bereaved families are both eligible for CANinform and encouraged to consider participating.
- By participating in CANinform patients and families can contribute to a greater knowledge of the condition that benefits the whole Canavan community.

HOW CAN MY FAMILY PARTICIPATE IN CANinform?

By enrolling in the study and sharing patient information, Canavan families may enable clinicians to provide better care for those affected and could help speed up clinical development of Aspa's gene therapy. Patient medical records are a key component for participating in CANinform, so the company is offering eligible families – at no cost – a record retrieval service to obtain patient medical history from all their providers using three simple steps:



CALL

1 Families can **call** 1-833-764-2267 or email CanavanMedRec@veristat.com to register with the call center, and to ask about the free service to collect medical records



CONSENT

2 Fill out a short online form to provide **consent** to share medical provider information and begin the process of obtaining the records



CONTRIBUTE

3 Once records are retrieved, **contribute** to the Canavan community by enrolling in CANinform, the Canavan natural history study. Support other Canavan families by joining this important effort

LEARN MORE ABOUT CANinform:

Aspa has a dedicated website for families affected by Canavan disease that provides more information about CANinform and its clinical program. Families can visit www.treatcanavan.com to learn more.

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

VISITS TO A CANinform STUDY SITE

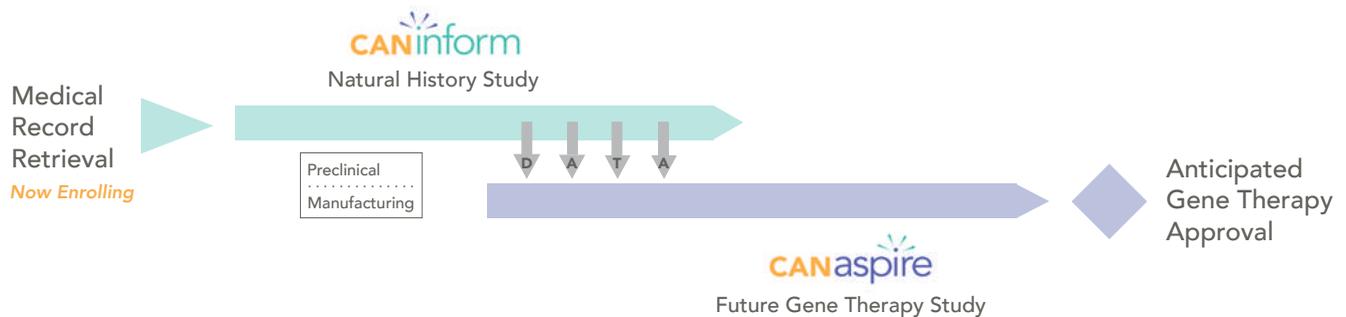
During study visits the Investigator will conduct assessments that will permit characterization of Canavan disease and its progression. These will include:

- Standard clinical evaluations of physical growth and development and collection of blood and urine samples, along with assessing Canavan disease-specific laboratory measures.
- Assessments that measure the impact of the disease on the patient's quality of life and the emotional and social aspects of individuals affected with Canavan disease may be administered as well.
- In-home assessments will be scheduled where available. A physical therapist trained on the required assessments will visit your home at a time that is convenient for the family.
- For those families able to participate in person, Aspa will cover the cost of travel expenses to attend the study visits.



ABOUT ASPA'S GENE THERAPY PROGRAM

Aspa's gene therapy will be part of a future clinical trial and use an AAV9 (adeno-associated virus serotype 9) vector. This approach differs from previous gene therapy trials in Canavan disease, and is designed to deliver functional copies of the ASPA gene throughout many tissues in the body as well as into the brain. The goal of this gene therapy is to address the underlying cause of Canavan disease and help improve signs and symptoms of the disease.



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

