Medical Record Retrieval Service Available to Canavan Families

In recent months, there have been references made within the community to natural history studies in Canavan disease. As you may know, expert pediatric neurologists have been collecting natural history data for the past few years. Recently, Aspa Therapeutics began working with these disease experts to update the study.

Information on the importance of a natural history study and the role a family plays in this effort can be found at the end of this message.

Aspa Therapeutics is in the final stages of starting up the Canavan Natural History Study, also known as ***CAN-Inform***. Families who wish to enroll in this study will need their medical records. Recognizing that medical record retrieval can be overwhelming for families dealing with Canavan disease, Aspa is providing a service to aid families in the medical record collection process by utilizing the services of a company called Telegenisys, which is very experienced in this process. This service is being offered free of charge.

To provide extra support for this service, Aspa has hired Veristat, a Contract Research Organization, who has established a Call Center dedicated to this effort. The first step in obtaining your child's medical records is to contact the Call Center at 1-833-764-2267 or ***CanavanMedRec@veristat.com***. A short telephone call will be scheduled at your convenience to speak with a representative who will ask you a few details about you and your child. Once the representative confirms your details, you will be provided instructions for how to access this service. After providing consent, you will then be asked to provide healthcare provider information to Telegenisys, as they will manage the collection of your medical records.

Aspa is offering this service at the suggestion of the patient advocacy organizations. **There will be no charge to the families.**

Aspa and the study physicians will NOT have access to your records through this effort. Once the records are provided to you, you are free to do with your child's records as you wish. You can choose to enroll in the ***CAN-Inform*** Study and provide your records to the study site but using this service to retrieve your records does not mean you are required to participate. If you decide to enroll in ***CAN-Inform***, after you sign consent for the ***CAN-Inform*** Study, instructions will be provided on how to submit the medical records to your assigned study site.

Bereaved families are welcomed and encouraged to join this effort as well.

Although the clinical sites that will conduct the natural history study are not yet open, we encourage you to begin your medical record collection process as soon as possible, as it could take up to 2 months to collect all records. At this time, this service is for US families only. Families living outside the US are still welcome to contact the Call Center for updates.

Thank you in advance for considering the important role *YOU MAY CHOOSE TO PLAY* in documenting the course of Canavan disease. If you have any questions, please contact the Call Center @ 1-833-764-2267 or ***CanavanMedRec@veristat.com***.

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What is a Natural History Study?

A natural history study is a research study where physicians monitor patients over time to record the signs, symptoms, and changes in a disease. Natural history studies are often an integral part of developing effective treatments. Natural history data helps physicians and researchers understand complex diseases like leukodystrophies and what they might expect to see if there is an improvement after administering a new therapy. It is important to keep in mind that when we talk about improvement in a disease, it could mean an improvement in a patient's abilities, or it might also indicate a slowing or stopping of disease progression.

Why is a Natural History Study Needed?

Rare diseases such Canavan disease typically have very small patient populations, vary widely in their presentation, and have progressive symptoms. Collecting and analyzing patient data can help measure the natural progression. As treatments are developed, this data can be used to define clear changes that are meaningful, which are also called clinical endpoints (a change that is meaningful). These endpoints help determine if a treatment is effective or not. It may be possible to use the data in a treatment study in place of a group that receives the same treatment procedure *without* therapy, which is also called a placebo group (the same treatment procedure without any therapy).

The Regulatory Component

Having good natural history data is also crucial from a regulatory perspective. Agencies like the Food and Drug Administration (FDA) and European Medicines Agency (EMA) require that natural history data be included in the development of treatments for rare diseases. Regulators want to be sure a disease is well understood as they evaluate if a treatment truly works.

What is the Role of Families?

Families are a critical source of information about Canavan disease. By participating in a natural history study, parents are able to report their child's symptoms, characteristics, and changes in health status to the researchers who are conducting the study. Bereaved families can also make a very significant contribution to the natural history study, as allowing their child's medical records to be studied provides equally important information about the course of Canavan disease over time. Participating in a natural history study advances the scientific knowledge about the disease and helps to advance clinical programs.