



About Research Studies

Pharmaceutical companies use research studies like this one to learn more about investigational drugs before they are made available to the public as approved treatments. The results of this study will provide information about the effectiveness of the investigational drug being evaluated. By taking part in this study, you will be making an important contribution to Cerebral X-Linked adrenoleukodystrophy (cALD) treatment research.



To learn more about this study, please contact:

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Have you been diagr Cerebral X-linked Adrenoleukodystrophy (cALD)?



JOHNS HOPKINS MERICINE In diagnose with Cerebral X-linked Adrenoleukodystrophy



LEARN MORE ABOUT MT-3-01 STUDY, THE CLINICAL STUDY THAT IS ASSESSING THE EFFICACY AND SAFET LERIGLITAZONE IN ADULT MALE SUBJECTS WITH CEREE ADRENOLEUKODYSTROPHY (CALD).

MT-3-01 is a clinical study where the effect of leriglitazone on the progression of cerebral X-linked Adrenoleukodystrophy (cALD) will be tested and compared to placebo.

There is currently no approved treatment for cALD in adults.

Hematopoietic (blood) stem-cell transplantation is used in some subjects with cALD. Corticosteroids are used to treat the adrenal insufficiency. Dietary supplementation with a 4:1 mixture of trioleate and glyceryl trierucate (Lorenzo's oil) can normalize

plasma

very long chain fatty acids levels, but clinical studies have shown that this does not prevent disease progression.

The leriglitazone has showed in studies performed on the

laboratory

that it may have various protective effects on the nervous tissues that are affected in cALD and may slow the progression.

Leriglitazone showed a manageable safety profile; considering the severity of the disease and the unmet medical need, a positive benefit-risk balance for male subjects with cALD has been

tudy will help provide more information about

investigational drug, the disease, drug could one day be used to

Who is eligible to participate in this study?

Your doctor will let you know whether you are eligible to take part in the study. The following are just some of the criteria that your doctor will assess:

Be male and aged ears ≥18 vears

Have a Loes score ≥ 0.5 and ≤ 12 .

X-ALD

Have a genetic confirmation of X-ALD.



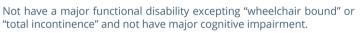
Have a normal adrenal function.



HSCT not recommended by the investigator or not willing to undergo HSCT.



Have a progressive cALD, defined as GdE+ brain lesions.





All study-related visits, tests, and study medicine wi provided without costs to you. In addition, assistan travel to the study clinical will be provided, a d vou ' reimbursed for the expenses related to the study trave

What will happen during this study?

If your doctor considers that you meet all the study's eligibility criteria, he will schedule a first study visit, named Screening Visit, and he will hand you over an informed consent form with further information about this study that you will have to sign in case you agree to take part in the study and then, he will assess and test you and determine if you could be enrolled in this study or not.



study.

This study has 2 parts: a double-blind period and an open-label extension.

In the **double-blind period** of this study, the leriglitazone will be compared to a placebo. A placebo is a treatment that looks and tastes exactly like the study medicine but does not contain any active ingredient. The first visit of this part will be the Baseline Visit, where the study doctor re-confirm your eligibility and you will be randomly assigned the stu leriglitazone or placebo.

During this double-blind period, you will months, and then 1 visit every 3 months 1, 3, and 9 may be conducted either a months 2, 15, 21, 24, 30, and 33 yo doctor, and the remaining visits w visits will be supported by a nurse.

Your participation in the doubleof 36 months. But, on 2 occasion 27), independent experts will ch working. This is called an "interil assessments, it is determined th period will end, the open-label start receiving leriglitazone.

If the results of the interim assessm working, you will continue receiving t or leriglitazone) until Month 36. After this th begin, and you will start receiving leriglitazone.

visit each month for 3 this period. Visits at months study site or at your home. At receive phone calls from your place at the study clinic. Home

riod is expected to last a maximum g this period (Month 18 and Month tudy results to assess if MIN-102 is nent". If, at either of these interim zone is working, the double-blind will begin, and all subjects will

> that leriglitazone is itment (placebo en-label extension will

In the ope duration of label exten months a

n-label extension, all subjects will receive leriglitazone. The pen-label extension is unknown. Throughout the openone calls with the study staff will be performed every 3 ill make 1 visit to the study clinic every 9 months.

What are the benefits and risks related to this study?

As with any research study, you may not personally benefit from participation in this study. Results from this study may benefit others in the future. It is also possible that you could experience a side effect while in this study. Before you begin the study, the study staff will discuss with you the risks related to participating in this

Because research studies can affect the health and safety of participants, you

will be closely monitored during this study. Researchers for this study designed a protocol, which describes all study procedures in complete detail, and they will ensure that the procedures will be followed exactly. An independent review board responsible for ethical considerations reviewed and approved this protocol. There is also an independent safety monitoring board that monitors the participants' safety throughout the entire study.