JOHNS HOPKINS MEDICINE

Approved January 4, 2024

CALYX

A clinical study in adult cALD

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.

CALYX

A clinical study in adult cALD

To learn more about this study, please contact:

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minoryx therapeutics

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CALYX

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Have you been diagnosed with

Cerebral X-linked Adrenoleukodystrophy (cALD)?

minoryx therapeutics

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[illegible] diagnosed with Cerebral X-linked Adrenoleukodystrophy (cALD)?

LEARN MORE ABOUT MT-3-01 STUDY, THE CLINICAL STUDY THAT IS ASSESSING THE EFFICACY AND SAFETY [illegible] LERIGLITAZONE IN ADULT MALE SUBJECTS WITH CEREBR[illegible] 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 glyceryl 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 proposed.

The [illegible] study will help provide more information about [illegible]he investigational drug, the disease, [illegible]al 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:

- ≥18 years — Be male and aged ≥18 years.
- ≥0.5 and ≤12 — 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.
- Not have a major functional disability excepting "wheelchair bound" or "total incontinence" and not have major cognitive impairment.

All study-related visits, tests, and study medicine will b[illegible] provided without costs to you. In addition, assistanc[illegible] travel to the study clinical will be provided, and you w[illegible] reimbursed for the expenses related to the study travel[illegible]

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.

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 st[illegible] leriglitazone or placebo.

During this double-blind period, you will h[illegible] visit each month for 3 months, and then 1 visit every 3 months d[illegible] this period. Visits at months 1, 3, and 9 may be conducted either a[illegible] study site or at your home. At months 2, 15, 21, 24, 30, and 33 yo[illegible] receive phone calls from your doctor, and the remaining visits wi[illegible] place at the study clinic. Home visits will be supported by a nurse.

Your participation in the double-[illegible]riod is expected to last a maximum of 36 months. But, on 2 occasion[illegible] this period (Month 18 and Month 27), independent experts will ch[illegible] study results to assess if MIN-102 is working. This is called an "interi[illegible]ment". If, at either of these interim assessments, it is determined th[illegible]zone is working, the double-blind period will end, the open-label [illegible] will begin, and all subjects will start receiving leriglitazone.

If the results of the interim assessm[illegible] do not confirm that leriglitazone is working, you will continue receiving th[illegible] treatment (placebo or leriglitazone) until Month 36. After this time, [illegible] open-label extension will begin, and you will start receiving leriglitazone.

In the **open-label extension**, all subjects will receive leriglitazone. The duration of [illegible] pen-label extension is unknown. Throughout the open-label exten[illegible] hone calls with the study staff will be performed every 3 months a[illegible] you will 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 study.

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.