

Clinical Trial for Prenatal Treatment of Boys Affected by X-linked Hypohidrotic Ectodermal Dysplasia

Information for Families

An investigational drug is being tested in a pivotal clinical trial for x-linked hypohidrotic ectodermal dysplasia (XLHED). This drug has previously been developed by another sponsor, but a clinical trial where newborn XLHED-affected babies were treated with it was unsuccessful, and the drug development was stalled.

Prof. Holm Schneider, a German pediatrician actively involved in this clinical development, revived hope for the XLHED community when he showed that while the drug does not work in newborn babies, it shows promise when given before birth to XLHED-affected fetuses.

Sweat function was shown to be normalised in a small number of XLHED-affected boys treated before birth <https://www.nejm.org/doi/full/10.1056/NEJMoa1714322>. It is the intention of the Sponsors to reproduce and confirm these results in a robust pivotal clinical trial.

If approved, this drug will be the first to improve the main symptoms of this rare genetic disorder, and it will do so through a single-course treatment of 3 injections given before birth. It may be the first drug to improve the quality of life for boys with XLHED.

What are Clinical Trials?

Clinical trials are an important part of developing new medical treatments. They help doctors and scientists determine if new drugs are safe, effective, and prescribed at the correct dosage and timing. The party responsible for overseeing and financing a clinical trial is known as the Sponsor of the trial.

The Prenatal XLHED Clinical Trial is a Phase II pivotal study. Trials conducted in Phase II determine how well the proposed drug meets the goals of the study for safety, effectiveness, and optimal dosing to achieve the best results. A pivotal trial is a study that intends to generate evidence and data that Regulatory Agencies use to decide whether a drug can be approved as a new treatment.

Who may participate?

Sponsors of this trial are looking for women who know or suspect that they are XLHED carriers and who are thinking of becoming pregnant or are in the early stages of their pregnancy. Women pregnant with boys in the next 24 months may be eligible to participate in the study.

How will I know if my unborn son is a candidate?

A simple blood test can determine whether you are a carrier of XLHED and whether your future child could develop XLHED. If you are pregnant, the XLHED status of your future baby boy can be determined via genetic testing (such as amniocentesis).

Knowing that you are a carrier of XLHED and that you are pregnant with a boy is sufficient information to consider taking part in the study. Confirmation of the XLHED status of your unborn son will determine whether you can enter the study or not.

Available Genetic Testing

Women who suspect they are carriers of XLHED should have a genetic test to determine XLHED carrier status and whether their child may develop XLHED. Genetic testing may be available to women or to their unborn sons at risk to develop XLHED.

For information regarding genetic testing, please speak with your family doctor.

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Will my child receive the new drug?

Women pregnant with XLHED-affected male fetuses taking part in the study will:

- Receive 3 doses of the investigational drug before birth through a series of 3 injections into the amniotic fluid:
- At pregnancy weeks 25, 28 and 31
- Be required to attend a number of visits at the treatment site, following the injections and until their child is 5 years old, to evaluate long-term safety and efficacy effects of the given drug

What if I decide not to participate?

Participation in a clinical trial is always voluntary—and choosing not to participate will have no effect on current medical care patients are receiving or may receive in the future. It's also important to know that if as a patient you decide to participate in a clinical trial, you may withdraw at any time—and for any reason.

For More Information

Pregnant women and their unborn XLEHD-affected sons must be enrolled in the clinical trial during the second trimester, between pregnancy weeks 18 and 23.

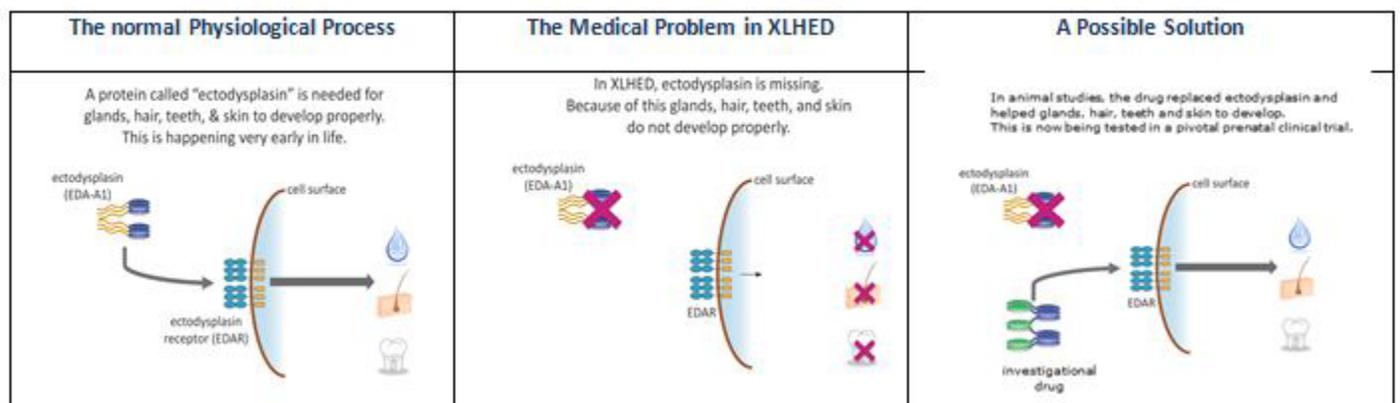
Therefore, it is important that families consider this information as early as possible. For more information about the clinical trial visit: www.clinicaltrials.gov (search for clinical trial EDELIFE) or contact the following:

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Clinical Trial of a Possible Treatment

Under typical conditions, a signalling molecule called ectodysplasin-A (EDA1) starts a pathway that is very important to the growth of ectodermal tissues in the developing fetus (such as sweat glands, teeth, and hair). This pathway starts early on in embryonic life. For people affected by XLHED, this signalling molecule is missing due to a change in their genetic information. Without this signal, the ectodermal tissues don't grow as expected, or in some cases don't grow at all, leading to the symptoms people affected by XLHED experience.

The investigational drug is a version of the signalling molecule EDA1 that is made in the laboratory. It is currently in a clinical trial as a potential treatment for XLHED. The rationale for use of the proposed drug is to provide XLHED patients this molecule as a replacement for their missing EDA1 during key development periods in the fetal development period to positively impact the formation of ectodermal structures.



Resources for You

To learn more about XLHED, clinical studies, research updates, and other resources for you and your patients, visit www.nfed.org.