Blog for ED Society Website for XLHED Families

Two Partners Re-Launch XLHED Research Study

By Mary Fete, Executive Director, NFED

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Today, I am excited to share with you some great news about our quest to develop a treatment for X-Linked Hypohidrotic Ectodermal Dysplasia (XLHED).

EspeRare has partnered with Dermelix Biotherapeutics to develop DMX-101 as an in-utero protein replacement therapy for the treatment of XLHED. They plan to launch their pivotal study later this year. If this treatment is approved, it has the potential to be the very first in utero administered drug to correct a genetic condition before birth! Extraordinary.

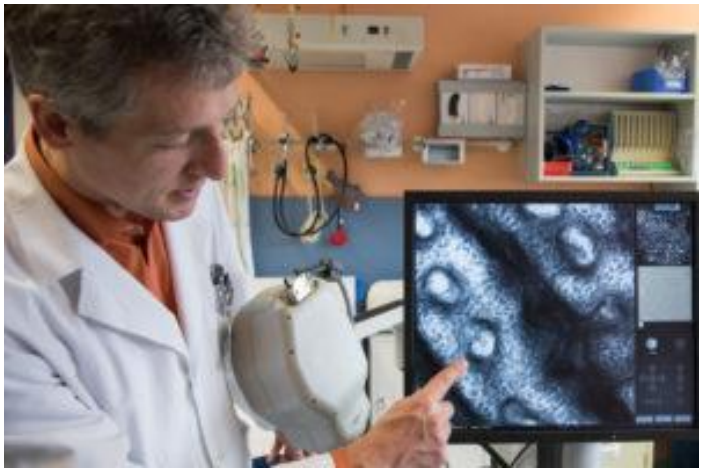
The Partners Leading the Way

EspeRare is a Swiss non-profit organization dedicated to accelerating the development of rare disease treatments. We have been working closely with them since they acquired the rights to this research when Edimer Pharmaceuticals shut its doors.

Dermelix Biotherapeutics is a biopharmaceutical company based in New York City focused on the development of innovative treatments for rare genetic skin diseases. The two entities announced their partnership today.

What is DMX-101?

You may remember this recombinant protein from some of the other names it has had through the years – ER-004, EDI200, and APO200. It's the same protein but it is now called DMX-101. It was developed as a replacement for the protein that is missing in XLHED.



Professor Holm Schneider points to the sweat glands that developed in a baby he treated in utero with DMX-101 in a Trial to a Cure

In a Trial to a Cure, Dr. Holm Schneider administered the protein in utero, through the amniotic fluid to three babies in the late stages of fetal development. His published findings documented its significant benefits. Among them, the babies had an increased number of teeth and working sweat glands!

These twins were the first two babies affected by XLHED to receive DMX-101 in utero in a Trial to a Cure. They developed working sweat glands!

The next step is to conduct a larger study to confirm these findings.

The Study

EspeRare and Dermelix announced that they plan to launch a single study of DMX-101 first in Europe, including the UK, in the second half of 2019 and then in the United States. The study calls for up to 15 male patients with XLHED to participate. Their goal? To bring this treatment to market as an option for all XLHED families!

EspeRare will sponsor the development of DMX-101 in Europe. The study has received accelerated development from the European Medicines Agency's PRIME (PRIority Medicines).

"This is such an exciting time for the EspeRare team which warmly thanks all of its supporters who contributed greatly to giving a second life to this therapy, bringing back hope to the XLHED patient community," said Caroline Kant, Founder and CEO of EspeRare. "This innovative treatment approach has the potential to fundamentally change the lives of these patients and may also pave the way for other prenatal treatments to correct genetic diseases before birth".

Dermelix will sponsor the development of DMX-101 outside Europe. It has received an Orphan Drug Designation in the United States. Dermelix will be responsible for the commercialization of DMX-101 worldwide.

Dr. Nick France, Chief Medical Officer of Dermelix said, "Our partnership with EspeRare represents a major milestone in establishing Dermelix as a leader in the orphan dermatology space and is emblematic of our vision in accelerating the translation of scientific discoveries into approved therapies for patients with some of the highest unmet medical needs".

How to Volunteer

We will share more detailed information about the study with our XLHED community when we receive it from EspeRare and Dermelix. This includes how to volunteer to be considered for enrollment in the study. Please keep in mind that the study will be limited to male unborn babies affected by XLHED. Here's why. XLHED is fully expressed in males. Females may or may not manifest symptoms. In order to determine if DMX-101 was successful in treating the symptoms, they must study males. Our hope in the long term is to see how females with XLHED could also benefit from the treatment. For now, if you are interested or considering participating in this study, please email me at mary@nfed.org.

A Moment to Celebrate

"Our research journey has been typical: filled with stops and starts, hills and valleys. But, for 30+ years, we persisted" said Mary Fete NFED.

What's not typical, is that it has the potential to be the very first treatment to correct a genetic condition before birth.

Today, we stand with pride at what's been accomplished and with great optimism for what lies ahead. We are humbled by all of the XLHED families who volunteered and supported the XLHED research projects in the last three decades. This success rests on your willingness to help us find answers.

Thank you, EspeRare and Dermelix, for sharing our vision to provide a treatment for XLHED families for future generations.

Think about this. If their study is successful, a baby boy affected by XLHED could receive a single course of treatment in utero and develop working sweat glands and an increased number of teeth, salivary glands, and meibomian glands. That child would have less respiratory challenges, hospitalisations or asthma. It almost sounds too impossible to be true. But it is!

This dream propels us forward.

We stand committed to working with both EspeRare and Dermelix to support their study. You are invited to join us!

If you live in the UK, please contact diana@edsociety.co.uk