



Dear Families

You are being sent this letter because you are affected by X-Linked Hypohidrotic Ectodermal Dysplasia (XLHED) or a carrier of the gene for XLHED. You may be interested in learning more about a clinical trial being conducted by the Esperare Foundation and their partner, Pierre Fabre Medicament, to treat boys affected by XLHED before birth.

The purpose of this study is to look at the safety and efficacy of an investigational drug, ER004. This drug was initially developed by Edimer Pharmaceuticals and was then known as EDI200. The study drug is investigational, which means that the regulatory authorities have not approved the drug.

This study is now recruiting women pregnant with XLHED-affected male foetuses in the second trimester of their pregnancy (between weeks 18 and 23). Mothers must be genetically confirmed as XLHED carriers while their unborn son will have to be confirmed as XLHED-affected as part of the study and before receiving the drug.

As part of their participation in this study, the unborn boys will get three doses of ER004. The drug will be injected into the amniotic fluid surrounding the foetus during the latter stages of pregnancy, in a procedure resembling an amniocentesis. From birth and until they are 5 years old, the treated boys will undergo several examinations to assess the efficacy and long-term safety of the drug (eg: blood taken, growth and development assessments, dental x-rays, sweat ducts counted, sweat collected, eyes and skin analysed, questionnaires to answer).

This study is being conducted at the University Hospital of Wales, in Cardiff, but you do not need to live in Cardiff or Wales to participate. If you choose to participate in this study you will need to be able to travel to these clinical research sites for an overnight stay 3 times during your pregnancy, at weeks 25, 28 and 31. While it is preferred that you give birth at the clinical site where your unborn son was treated, you can choose to give birth at a different place. Once your baby is born, you will need to come back to the clinical research site for 9 additional visits with your child as part of a long-term follow up, starting when your son is 1-month old and until he is 5 years old.

The sponsors of this clinical study, the Esperare Foundation and Pierre Fabre Medicament, will pay for your travel, meals and accommodation for each visit and will work with you to ensure you can comply with the schedule of visits as best as possible.

If you or one of your family members is expecting a baby or thinking about getting pregnant and is interested in learning more about participation in this study or learning more about ER004 or XLHED please contact us:

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Additional information on this clinical study can be found at <http://clinicaltrials.gov/> and search for EDELIFE.

Sincerely,

Prof Angus Clarke ([ClarkeAJ@cardiff.ac.uk](mailto:ClarkeAJ@cardiff.ac.uk))