Retrophin

New Research Study of the Prevalence of Cerebrotendinous Xanthomatosis (CTX) in Patients with Cataracts in Both Eyes

What is the purpose of the study?

The purpose of the CTX Prevalence Study is to help researchers understand how many people with early-onset cataracts in both eyes may have a rare but treatable disease called **cerebrotendinous xanthomatosis** (CTX).

What is CTX?

One of the important signs of CTX is having cataracts in both eyes at a young age. CTX is a disease where people do not make bile acids normally. This causes buildup of both cholesterol and cholestenol in many of the bodies' tissues over time leading to xanthomas (fatty skin growths that occur usually on the joints). CTX is normally a very progressive disease that may first appear at any time from birth through adulthood with changing appearances and progressing to long-lasting neurological (brain and nervous system), psychiatric (mental disorders) and musculoskeletal (bone) damage.

Who can take part in this study?

You may be eligible to take part in the CTX Prevalence study if you or your child:

- Have cataracts in both eyes
- Have no known cause for the cataracts
- Were between the ages of 2 to 21 years old when the cataracts were first diagnosed.

How long will the study last?

Study participation includes one visit to the study site but may last up to eight weeks until the results of the lab tests are known. .

Does it cost anything to participate?

There is no cost to you to take part in this study.

What are the potential risks and benefits?

During the clinic visit, the study doctor will tell you about the study and the possible risks and benefits of taking part. The study doctor can answer any questions you may have about study procedures or potential risks and benefits of study participation.

The information learned from the study and your participation may help doctors in the future diagnose and treat CTX at a younger age.

Study participation is voluntary. If you choose to participate, you may leave the study at any time for any reason.

Does the study involve any study medication?

No, there will be no study medication while you take part in the study. If you (or your child) is diagnosed with CTX as a result of this study, you will be referred for medical care and treatment. However, the cost of future referral and treatment would not be paid for as part of this study.

What do I have to do?

If you are eligible for this study, you will be asked to come into one of the study sites for one visit. Participants will be asked questions about their medical and family history and also asked to provide blood and urine samples.

Who do I contact with general questions?

For more information or questions about the study, please call 877-659-5518 or email medinfo@retrophin.com.

Where can I participate?

About 500 people in the United States will take part in the CTX Prevalence Study. The following is a map and list of actively enrolling CTX Prevalence Study site locations and the corresponding study doctors. This information will be updated as additional study sites open in the future:

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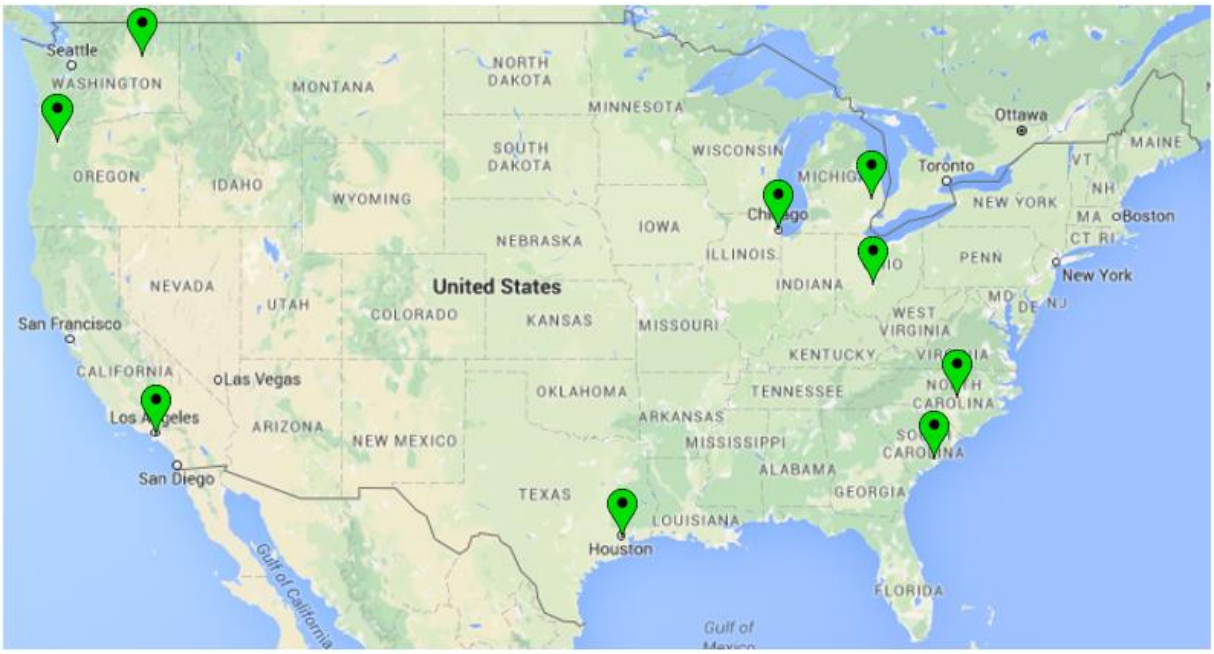

State	City	Hospital or Clinic	Study Doctor
CA	Los Angeles	Stein Eye Institute & Doheny Eye Institute (UCLA)	Federico Velez, MD
IL	Chicago	Lurie Children's Hospital of Chicago	Marilyn Mets, MD
MI	Ann Arbor	University Michigan/ Kellogg Eye Center	Monte DelMonte, MD
NC	Chapel Hill	UNC Kittner Eye Center	Natario Couser, MD
OH	Columbus	Pediatric Ophthalmology Associates	Cate Jordan, MD
OR	Tigard	Child Eye Care Associates	David Wheeler, MD
SC	Charleston	Storm Eye Institute (MUSC)	M. Edward Wilson, MD
TX	Houston	Houston Eye Associates	Ann Stout, MD
WA	Spokane	Spokane Eye Associates	Jeffrey Colburn, MD

*Updated as of January 13th, 2016

Additional information can be found on www.clinicaltrials.gov - CTX Prevalence Study.gov – ClinicalTrials.gov Identifier: NCT02638220