DO YOU HAVE A GENETIC EYE DISEASE?

Consider learning more about clinical trials

This brochure explains important terms and answers frequently asked questions about clinical trial participation for people with genetic eye diseases such as Leber’s congenital amaurosis, Usher syndrome, retinitis pigmentosa, or Fuchs’ endothelial corneal dystrophy.

What are clinical trials for genetic eye diseases?

Clinical trials, also called clinical studies, help researchers and doctors learn more about genetic eye conditions and new medications, devices, or tests that may help people with a genetic eye disease. Before any medication, device, or test can be approved and made available to the public, it must go through several phases of clinical trials with volunteer participants.

Accessibility
Scan the QR code to read this brochure on your mobile device.
Clinical trials help confirm if the new medication, device, or test is safe and effective in treating vision impairment. Treatments may aim to slow down or stop worsening of vision, or even improve vision.

**Key Terms**

These terms are used throughout this brochure.

**Clinical trial team**
The team at the clinic that you will interact with when you join a clinical trial. Clinical trial team members can include your current or another ophthalmologist, other doctors, nurses, technicians, and study staff.

**Ophthalmologist**
A doctor specialized in eye conditions and disorders.

**Study clinic**
The doctor’s office or hospital where clinical trial participants receive tests during a clinical trial.

**Informed Consent Form (ICF)**
A document that explains the clinical trial in detail, including its purpose, background, study tests, visits, and potential risks and benefits. The ICF also outlines the responsibilities of clinical trial participants. Participants must sign the ICF before they receive any clinical trial tests.

**Study medication**
A new medication that has not been approved by official regulatory groups like the United States Food and Drug Administration (FDA) or European Medicines Agency (EMA) to use outside of controlled clinical trials. This may also be called *study drug* or *active medication*.

**Intravitreal injection**
Shot of medicine into the eye. The inside of the eye is filled with a jelly-like fluid (vitreous). During this procedure, your clinical trial doctor injects medicine into the vitreous, near the retina at the back of the eye. This method is most often used to get a higher level of medicine to the retina.

**Sham**
A fake procedure that closely imitates the real procedure of an intravitreal injection into the eye, however there is no injection and no active medication delivered. Many clinical trials have one group of participants receiving the study
medication via intravitreal injection into the eye, and another group receiving the sham procedure. By using a sham procedure, neither study participants, nor the clinical trial team will know who has received active medication or nothing, which helps researchers confirm that any effects participants experience are caused by the active medication and not by something else.

**Study visits**
Appointments at the study clinic that clinical trial participants must attend during a clinical trial.

**What happens during a clinical trial?**

All clinical trials are unique but usually follow a similar pattern:

**Signing Informed Consent Form**
If you consider participating in a clinical trial, the clinical trial team will discuss the informed consent form (ICF) with you and allow you time to review the information before you decide to participate or not. You can only start the clinical trial after you have signed the informed consent form. Participation is always voluntary.

**Screening**
Screening involves receiving tests to make sure you qualify for the clinical trial. This includes several eye tests but also health checks, blood tests, and often genetic testing. These tests can sometimes take up to 2 to 3 days at the study clinic. Some study visits are long because study tests must sometimes be repeated to provide better information about your disease. Other tests require adaptation to dim light for a period of time, before the actual test can start. There often are breaks between these tests to make it easier for you.

**Receiving study treatment**
Study treatment may be provided as a pill, drops, a sham procedure or by intravitreal injection administered by a clinical trial doctor. Depending on the study design, you will be assigned to receive either the study medication, a sham procedure, or standard treatment.

**Attending study visits**
You will visit the study clinic regularly for health checks and eye tests. Depending on the clinical trial, study visits will usually be more frequent and take longer than your
regular doctor visits. You may get support from the clinical trial team for traveling to the study clinic and for arranging overnight stays, if applicable.

Responding to follow up visits or calls
After your final study visit, you may still be contacted by clinical trial team by phone or email to monitor your health.

Why do people participate in clinical trials for genetic eye disease?

People participate in clinical trials for different reasons. Some participate because they want to learn more about their disease. Others participate because they want to help with the development of new treatments that could help them and others in the future. If you take part in a clinical trial, you may be one of the first people to benefit from new study medication. However, there is always a chance that the new medication is not better, or worse, than the standard treatment (if a standard treatment exists) or that you will receive the sham procedure.

Are clinical trials safe?

Clinical trials follow a specific set of standards and are strictly regulated to help keep all participants safe. All clinical trials are reviewed and approved by a committee of independent experts (Ethics Committee or Institutional Review Board). During a clinical trial, participants are carefully monitored. In addition to eye tests, general safety tests may be performed during study visits, such as blood tests, blood pressure, heart rate and temperature.

What should I know before joining a clinical trial for genetic eye disease?

It is important that you know as much as you can about a clinical trial before joining. You should carefully review any requirements and risks and talk with your clinical trial doctor to make sure participating in the clinical trial is a good choice for you. Remember the following when considering if you should join a clinical trial:

• Clinical trial participation can be time consuming. You may be
expected to attend multiple visits at the study clinic where you will undergo several tests to monitor if there is any improvement and if the study treatment is safe.

• You may see different members of the clinical trial team, like technicians who specialize in specific eye tests. Your regular ophthalmologist may or may not be involved in the clinical trial but will be kept informed of your participation and progress.

• Some clinical trials require you to stay overnight for some visits. In some trials, visits may be spread out over 2 or more days to increase convenience and reduce burden of participation. Your clinical trial team will inform you exactly about what will be required from you during clinical trial participation.

• There may be restrictions on what you can and cannot do. For example, you may be asked to avoid or stop taking certain medications or to prevent pregnancy during study participation.

• There is a chance that you may experience unknown side effects from the study treatment.

Who can join a clinical trial?

Every clinical trial has a clinical trial plan, also known as a study protocol, which describes what will be done during the clinical trial, how it will be done, and why it is necessary. It also includes criteria on who can and cannot join. Some common criteria include:

• age
• type of eye disease
• medical history
• past treatments
• specific genetic profile

Will I get paid?

You may be reimbursed for time and travel during a clinical trial, but this depends on what is allowed at your study clinic. The ICF for the specific clinical trial will provide details on what reimbursement or payment you may receive for a clinical trial.
Can I leave a clinical trial once it starts?

Yes, clinical trial participation is completely voluntary. You do not need to take part in a clinical trial, and you can end your participation at any time, for any reason. If you leave a clinical trial early, the clinical trial doctor may ask that you return to the study clinic for a final visit.

Can I see my regular ophthalmologist during the clinical trial?

Yes, you can visit any doctor to meet other health care needs during the clinical trial. Your clinical trial doctor may be different than your regular ophthalmologist.

What if I have questions?

You can ask the clinical trial team questions at any time before, during and after the clinical trial. Contact information will be provided to you.

If you are interested in participating in a clinical trial for a genetic eye disease, please talk to your ophthalmologist.