

1. Introduction

What does this Summary Cover?

This is a summary of a clinical study that was done in people with diabetic macular oedema (DME). The summary has information about how the study was done and what its most important results were. The summary only has information about this specific study. Researchers need to look at the results of more studies to make sure that a medicine works and that it is safe.

Short Study Title

A study to evaluate the safety of THR-687 in subjects with DME.

Official Study Title

A Phase 1, open-label, multicenter, dose escalation study to evaluate the safety of a single intravitreal injection of THR-687 for the treatment of DME.

Protocol Number

THR-687-001

US Study Number

NCT03666923

2. Who Sponsored the Study?

Who Was the Sponsor of the Study?

The sponsor of the study was Oxurion (previously called ThromboGenics). Oxurion paid for the study to be done.

How to Contact Oxurion?

You can contact Oxurion at +32 16 75 13 10.

3. General Information About the Study

Where and When Was the Study Done?

The study was done in 7 study centers in the United States. The study started in September 2018 and was finished in November 2019.

Why Was the Study Done?

The study was done to test a new medicine to treat DME. DME is an eye problem that can develop in people who have diabetes. People with diabetes have too much sugar in their blood, which can damage blood vessels, including the ones in the back of their eye. As a result, these blood vessels can break easily and leak, causing the back of the eye (the retina) to swell. As the retina, and especially its central part (the macula), is very important for vision, DME can lead to vision problems. If left untreated, it can lead to blindness.

There are already some medicines available for DME. They work well for most of the people with DME, but not for everyone. That is why new medicines are needed.

The new medicine that was tested in this study is called THR-687. This was the first study in which THR-687 was tested. That is why the main goal of the study was to make sure that THR-687 is safe. The other goals of the study were to check if THR-687 could reduce swelling of the retina and improve vision in people with DME.

4. Who Took Part in the Study?

The people who took part in the study had DME. Twelve (12) people in total, 9 men and 3 women, took part in the study. Their average age was 58 years. The youngest person in the study was 38 years old and the oldest person was 72 years old.

5. Which Medicine Was Tested?

The medicine that was tested is called THR-687. THR-687 blocks proteins called integrins. Integrins can make damaged blood vessels grow and leak fluid. That is why THR-687 may be a good treatment for DME.

All people in the study received 1 injection with THR-687 into one of their eyes. Three (3) concentrations of THR-687 were tested. The first 3 people who took part in the study got the lowest concentration of THR-687, the next 3 people got the middle concentration and the last 6 people got the highest concentration. The people who took part in the study did not know which concentration they got, but the study doctors and the researchers did.

6. How Was the Study Done?

For each person who took part in the study, there were 6 check-ups after the injection with THR-687 was given. These check-ups were done over the course of 3 months.

During each check-up, the study doctor did a complete eye examination, including a vision test. For the vision test, the people were asked to read letters from a vision chart with 100 letters in total, going from very big to very small in size (called ETDRS chart). The better the vision, the more letters a person can read. The study doctor also used a machine to measure the swelling of the retina.

In addition, everyone in the study had to tell the study doctor about any medical problems they had while they were in the study. This was needed to make sure that the researchers learn about any side effects that the injection with THR-687 may cause.

7. What Were the Side Effects?

What Are Side Effects?

Side effects are unwanted medical problems that happen when a medicine is given. Side effects can be serious (for instance when they are life threatening, when they lead to having to stay in the hospital, or when they cause lasting damage), or non-serious (all other side effects). In a clinical study, all medical problems that start after the medicine is given are considered side effects. For each side effect, the study doctor decides whether it may be caused by the medicine. In this study, side effects that the study doctor decided were caused by the medicine could either be caused by THR-687 itself, or by the procedure of injecting it into the eye.

Which Side Effects Happened in the Study?

After the injection with THR-687, there were 3 (out of 12) people who had side effects that the study doctor decided were caused by the medicine. Each of those 3 people got a different concentration of THR-687. The person who got the lowest concentration of THR-687 had bleeding on the surface of the eye as well as an increased pressure in the eye. The person who got the middle concentration of THR-687 had bleeding on the surface of the eye. The person who got the highest concentration of THR-687 had eye pain. All of these side effects got better again without treatment. The researchers think that these side effects were caused by the procedure of injecting THR-687 into the eye rather than by THR-687 itself.

8. What Were the Overall Results of the Study?

Safety of THR-687

The study found that one injection of THR-687 is safe.

Improvement of Vision

After the injection with THR-687, on average, people could read more letters on the vision chart than before the injection. The improvement started on the day after the injection and vision was still improved at the last check-up, 3 months after the injection. While vision improved with all of

the concentrations of THR-687 that were tested, on average, the vision of the people who got the highest concentration of THR-687 improved the most.

Reduction of the Swelling in the Back of the Eye

After the injection with THR-687, on average, there was a small reduction in the swelling in the back of the eye that lasted up to 1 month after the injection. The largest reduction was seen in people who got the highest concentration of THR-687.

9. How Has This Study Helped Patients and Researchers?

The study found that one injection of THR-687 is safe. The results of the study also suggest that THR-687 can improve vision and reduce the swelling of the retina in people with DME. However, this was only the first study in which THR-687 was tested. More studies will need to be done to better understand if THR-687 is a safe and efficacious medicine to treat DME.

10. Are There Plans for Further Studies?

The next study, called the INTEGRAL study, is ongoing. The people who take part in this study will several injections into their eye. In the first part of the study, 2 different concentrations of THR-687 are tested: the highest concentration that was used in study THR-687-001 as well as a slightly lower concentration. The protocol number of this study is THR-687-002 and the EU study number is 2020-000362-42. The US study number was not yet known at the time this summary was prepared.

Information on this study will appear in the EU Clinical Trials Register (<https://www.clinicaltrialsregister.eu/ctr-search/search>) and on ClinicalTrials.gov (<https://clinicaltrials.gov/ct2/home>)

11. Where Can I Find More Information About This Study?

To learn more about study THR-687-001, you can find more detailed information on this website:

<https://clinicaltrials.gov/ct2/home>

Search for US study number: NCT03666923,
or for protocol number: THR-687-001