Plain Language Summary of Study Results

Astellas is grateful to the people who took part in this clinical study. Thank you.

What was the Study Called?

Long Term Follow up of Sub-retinal Transplantation of hESC Derived RPE Cells in Patients With Age-Related Macular Degeneration

Why was this Study Needed?

The macula is a small central part of the retina, the light-sensitive part at the back of the eye. It is the part of the eye that allows you to see fine detail. In some people, age, along with other factors, may cause cells in the macula to die. They can develop a condition called dry age-related macular degeneration (also called dry AMD). With this condition, people lose their clear, sharp central vision. Central vision is needed to be able to read and drive a car.

One treatment for dry age-related macular degeneration is surgery. However, not all people can have surgery or, it may not work for all people. One possible new treatment is to transplant healthy cells into the retina. This is called cell therapy. Stem cells are cells that can be turned into specialized cells. Astellas has developed stem cells into specialized cells found in the retina. These cells are also known as ASP7316. In an earlier study, different dose levels of ASP7316 were transplanted into the retina of people with dry age-related macular degeneration. During that study, these people were followed for 1 year after their transplant.

Cell therapy is a new type of treatment. Researchers do not know if this treatment causes medical problems over time. Researchers have been asked to follow people who have had cell therapy for longer. This will help them better understand the condition and its treatment.

In this study, the people from that earlier study were followed for longer to check if they had any long-term medical problems after their ASP7316 transplant. Medical problems are also known as adverse events. The people in this study were followed for up to 5 years after their transplant.

The study started in February 2013 and ended in August 2019. The sponsor of this study (Astellas Institute for Regenerative Medicine) reviewed all the study information and created a report of the results. This is a summary of that report.

What Kind of Study was this and Who Took Part in It?

This study was a long-term follow-up study. It followed people from an earlier study in which they had 1 eye treated with ASP7316. That study ended 1 year after treatment. This study continued to follow these people from 1 year up to 5 years.

Only people with dry age-related macular degeneration who had an ASP7316 transplant in the earlier study could take part in this study. People in the earlier study received different dose levels of ASP7316.

This study took place at 4 clinics in the United States. 11 people were in the study.

	Number of People
Age Group	
Aged less than 65 years	1
Aged 65 up to 85 years	9
Aged 85 years or older	1
Sex	
Men	4
Women	7

What Were the Study Results?

A lot of research is needed to know whether a medicine causes a medical problem. So when new medicines are being studied, researchers keep track of all medical problems that patients have while they are in the study. These problems are called "adverse events" and are recorded whether or not they might be caused by the treatment taken.

The study was mainly aimed to check for any adverse events that were considered serious. It was also aimed to check for other important adverse events. In this study, important adverse events were adverse events of particular interest to the researchers. Studies also routinely report adverse events that were possibly related to treatment.

*****Did people have any adverse events that were serious during this study?**

An adverse event is considered "serious" when it is life-threatening, causes lasting problems or needs hospital care.

No one had a serious adverse event that involved the eye.

8 people (72.7%%, or 8 out of 11 people) had serious adverse events that did not involve the eyes. None of the events were in more than 1 person each. None of them were judged by the study doctor to be possibly caused by ASP7316.

*****Did people have any adverse reactions during this study?**

An "adverse reaction" is any medical problem or "adverse event" that is judged by the study doctor to be possibly caused by a medicine or treatment used in the study.

3 people (27.3% or 3 out of 11 people) had adverse reactions that involved the eyes.

The table below shows the adverse reactions that involved the eyes.

Adverse Reaction	**Eye Treated with ASP7316 (out of 11 people)**	**Untreated Eye (out of 11 people)**
Coloring of the retina, the light-sensitive part at the back of the eye.	0	1 (9.1%, 1 out of 11)
Extra fiber tissue found in the macula of the eye which can lead to distortion of vision.	1 (9.1%, 1 out of 11)	0
Abnormal results from eye examination of the retina, the light-sensitive part at the back of the eye.	1 (9.1%, 1 out of 11)	0

Study doctors considered these adverse reactions to be mild or they did not require treatment.

No one had adverse reactions that did not involve the eyes.

*****What other important adverse events did people have during this study?**

Some adverse events were considered important as they were of particular interest to the researchers. Some of the important adverse events in this study involved the eye treated with ASP7316. Other important adverse events did not involve the eyes.

7 people (63.6% or 7 out of 11 people) had important adverse events that involved the eye treated with ASP7316.

The most common important adverse events that involved the eye treated with ASP7316 are shown in the table below:

Important Adverse Event	**Eye Treated with ASP7316 (out of 11 people)**
Clouding of the center of the lens in the eye	4 (36.4%, 4 out of 11)
New damaging blood vessels that grow behind the retina in an area called the choroid	3 (27.3%, 3 out of 11)

6 people (54.5%, or 6 out of 11) had important adverse events that did not involve the eyes. None of these adverse events happened in more than 1 person each. None of them were judged by the study doctor to be possibly caused by ASP7316.

There were no signs of failure or rejection of the ASP7316 transplant during this study. Also there were no signs of an immune response to the ASP7316 transplant. The immune response is how the body fights foreign objects or infections.

The results in this study were similar across all dose levels of ASP7316.

2 people passed away during the 5 year follow-up period. Neither of them passed away because of the study medicine.

Where Can I Learn More About This Study?

This document is a short summary of the main results from this study. You can find this summary and more information about this study online at http://www.astellasclinicalstudyresults.com.

Please remember that researchers look at the results of many studies to find out how well medicines work and which adverse reactions they might cause. This summary only shows the results of this 1 study. Your doctor may help you understand more about the results of this study.

Sponsor contact details:

Astellas Institute for Regenerative Medicine
9 Technology Drive
Westborough, MA 01581, USA