Clinical Trial Results

NOVARTIS

Research Sponsor:	Novartis
Location of Headquarters:	Basel, Switzerland
Treatment Studied:	HSC835
Protocol #:	CHSC835X2202
Full Trial Title:	A single-arm, open-label study to evaluate the safety and tolerability of infusing HSC835 in patients with hematological malignancies undergoing UCB transplantation using a non-myeloablative conditioning regimen
Full Scientific Summary:	www.novctrd.com
Trial Date:	October 2014 to August 2016

Thank you!

Thank you to the patients who took part in the clinical trial for the treatment HSC835. The patients helped researchers learn how HSC835 works in people with blood cancer.

Novartis sponsored this trial and thinks it is important for them to know the results of this trial. An independent non-profit organization called CISCRP prepared this summary of the trial results for the patients. We hope it helps the patients understand and feel proud of their important role in medical research.

If the patients have questions about the results, they should speak with the doctor, research nurse, or other team member at the trial site.

What's happened since the trial ended?

The patients were in this trial for about 1 year, but the trial took almost 2 years to complete. This is because the patients started and stopped the trial at different times.

The trial included 9 patients from 1 trial site in the United States. When the trial ended, the sponsor reviewed the data and created a report of the results. This is a summary of that report.

Why was the research needed?

The researchers were looking for a better way to treat blood cancer. One common treatment for blood cancer is to give patients blood stem cell transplants. The blood stem cells can be taken from bone marrow or from umbilical cord blood. A blood stem cell can turn into all the common types of cells normally found in the blood.

Sometimes doctors cannot always find stem cells from bone marrow that are a safe 'match' for the patient. In this case, umbilical cord blood may be used. But the amount of stem cells from 1 unit of umbilical cord blood may not be enough to help treat a patient with blood cancer. Often patients need more stem cells in order to recover from the transplant quickly enough. So researchers are looking for new ways to help speed up the patient's recovery.

The researchers in this trial wanted to learn whether a treatment called HSC835 could help patients with blood cancer recover from the transplant faster. In this treatment, some of the cells from the umbilical cord blood are grown in the laboratory to increase the number of blood stem cells. Because the HSC835 treatment has more blood stem cells, only 1 unit of umbilical cord blood is needed for the transplant.

In this trial the researchers wanted to know:

- How many patients had successful transplants?
- How many patients grew new blood cells?
- How many patients died for reasons other than their cancer?
- How many patients lived for at least 1 year after getting HSC835?
- What medical problems did patients have during the trial?

To answer these questions, the researchers asked for the help of people with blood cancer. The patients in this trial were men and women who were 29 to 70 years old and had blood cancer.

What kind of trial was this?

This trial was “open-label”. This means that the patients, doctors, staff, and the sponsor knew what each patient got. In this trial, all patients got HSC835.

What happened during the trial?

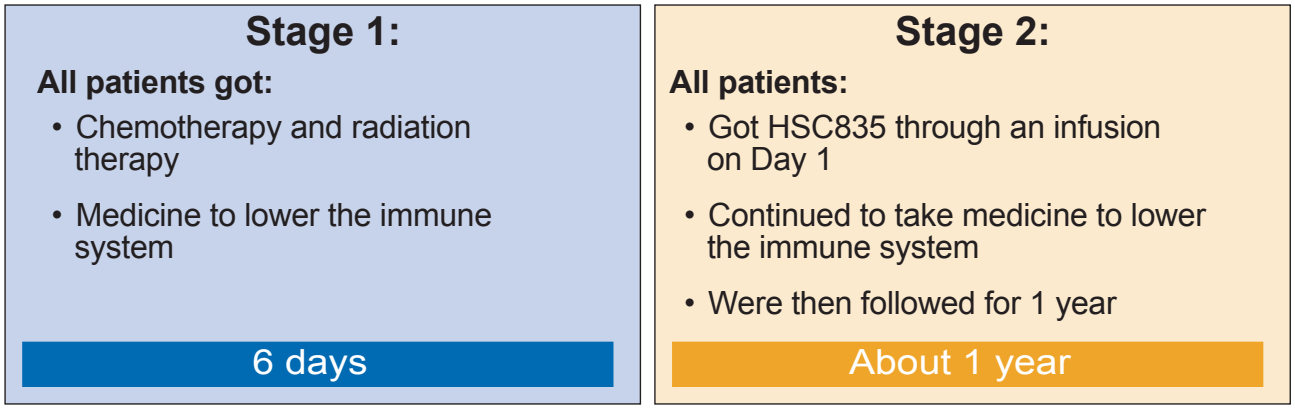
Before the trial started the doctors did a full checkup of all patients to make sure they could join the trial. The doctors did a physical exam, took blood and urine samples, did other tests, and checked the lung and heart health of the patients. The doctors also asked the patients questions about any medical problems and other drugs that the patients were taking.

This trial had 2 stages. All of the patients went through both stages. During both stages they took medicine to lower their immune system. These medicines helped make sure the immune system did not reject the transplanted stem cells.

- **Stage 1 lasted 6 days.** During this stage each patient got chemotherapy, radiation therapy to help treat their blood cancer.
- **Stage 2 lasted about 1 year.** During this stage, each patient got HSC835 on Day 1 through an infusion. An infusion is the delivery of a liquid drug into the body through a vein at a controlled rate. The patients were followed for 1 year to see how well they were doing after the transplant and how they were feeling.

The chart below shows how the trial was done.

Open-Label Trial



What were the results of the trial?

This is a summary of the overall results of this trial, not individual patient results. The results presented here are for a single trial. Researchers look at the results of many trials to decide which treatments work best and are safest for patients.

Other trials may provide new information or different results. Patients should not make changes to their treatment based on the results of a single trial without first talking to their doctor. Always talk to a doctor before making any treatment changes.

How many patients had successful transplants?

All 9 of the patients had successful transplants of HSC835. The transplant was successful if the patient did not have a "graft failure". Graft failure means the body rejected the stem cells that were transplanted. None of the patients had graft failure 42 days after getting the transplant.

How many patients grew new blood cells?

The transplanted blood stem cells can turn into all the common types of cells normally found in the blood. All 9 of the patients grew new blood cells after the HSC835 transplant. The researchers wanted to know if the patients grew a certain kind of blood cell called a "neutrophil". All of the patients had neutrophils grow in their blood from the transplanted blood stem cells within 42 days of the transplant.

How many patients died for reasons other than their cancer?

The researchers wanted to know how many patients died during the trial for reasons other than blood cancer. The reasons could have been because of the transplant or something else.

- None of the patients died in the first 100 days after getting the transplant for reasons other than their cancer.
- One patient (11.1%) died within 1 year of getting the transplant for reasons other than their cancer. This was 1 of the 9 patients.

How many patients lived for at least 1 year after getting HSC835?

The researchers wanted to know how many patients lived for at least 1 year before dying for any reason. They also wanted to know how many patients did not have their cancer come back.

- 55.6% of the patients were alive 1 year after getting the HSC835 transplant. This was 5 of the 9 patients.
- 44.4% did not have their cancer come back 1 year after getting the HSC835 transplant. This was 4 of the 9 patients.

What medical problems did patients have?

A lot of research is needed to know whether a treatment causes a medical problem. So, when new treatments are being studied, researchers keep track of all medical problems that patients have. These medical problems are called “adverse events”. An adverse event is any unwanted sign or symptom that may or may not be caused by the trial treatment.

An adverse event is considered “serious” when it is life-threatening, causes lasting problems, or leads to hospitalization. During a trial, all serious adverse events are reported and written down, whether or not they are caused by the trial drug.

How many patients had adverse events during the trial?

Overall, all 9 patients (100.0%) had adverse events during the trial. The table below shows how many patients had adverse events during this trial.

Adverse events in this trial

	Time from transplant until 48 hours after transplant Total (Out of 9 patients)	Time from Day 3 to the end of the trial Total (Out of 9 patients)
How many patients had adverse events?	88.9% (8)	100.0% (9)
How many patients had serious adverse events?	0.0% (0)	100.0% (9)
How many patients left the trial due to adverse events?	0.0% (0)	55.6% (5)

Did any patients have serious adverse events?

During the 1 year trial period, 44.4% of the patients died. This was 4 out of the 9 patients.

- 3 patients died within 1 year of getting HSC835 because their cancer came back.
- 1 patient died because of acute graft versus host disease. In this disease, the transplanted blood stem cells attack the body.

The doctors did not think any of the deaths were related to HSC835 treatment.

None of the patients had serious adverse events during the first 48 hours after getting HSC835. From Day 3 after the transplant to the end of the trial, all 9 patients had serious adverse events. The serious adverse event that happened the most often was acute graft versus host disease.

The table below shows the most common serious adverse events that happened to at least 2 patients from Day 3 to the end of the trial. Other serious adverse events happened in fewer total patients.

Most common serious adverse events from Day 3 to end of the trial

Serious adverse event	Total (Out of 9 patients)
Acute graft versus host disease (when the transplanted stem cells attack the body soon after the transplant)	66.7% (6)
Intestinal infection due to C. difficile bacteria	22.2% (2)
Pneumonia	22.2% (2)
Aspiration pneumonia (infection caused by breathing in food, drink, vomit, or saliva into the lungs)	22.2% (2)

What were the most common non-serious adverse events?

In the first 48 hours after getting HSC835, adverse events happened in 88.9% of patients. This was 8 out of 9 patients. The most common adverse event that happened in the first 48 hours after getting HSC835 was high blood pressure.

The table below shows the most common adverse events that happened to at least 2 patients in the first 48 hours after getting HSC835. Other adverse events happened in fewer total patients.

Most common adverse events in the first 48 hours after getting HSC835

Adverse event	Total (Out of 9 patients)
High blood pressure	44.4% (4)
Too much fluid in the blood	33.3% (3)
Headache	33.3% (3)

More adverse events happened from Day 3 to the end of the trial than in the first 48 hours after getting HSC835. The most common adverse event that happened from Day 3 to the end of the trial was acute graft versus host disease.

The table below shows the adverse events that happened to at least 2 patients from Day 3 to the end of the trial. Other adverse events happened in fewer total patients.

Most common adverse events from Day 3 to end of the trial

Adverse event	Total (Out of 9 patients)
Acute graft versus host disease	66.7% (6)
Blood infection due to cytomegalovirus	44.4% (4)
Pneumonia	44.4% (4)
Blood infection due to Enterococcus bacteria	33.3% (3)
Skin infection	22.2% (2)
Intestinal infection due to C. difficile bacteria	22.2% (2)
Blood clot in a vein	22.2% (2)
Fever and low neutrophil count	22.2% (2)
Infection with herpes virus	22.2% (2)
Aspiration pneumonia	22.2% (2)

For more information about adverse events in this trial, please refer to the full scientific summary of the results available on the Novartis Clinical Trial Results website (www.novctrd.com).

Where can patients find more information?

Researchers look at the results of many trials to decide which drugs work best and are safest for patients. It takes volunteers in many trials all around the world to advance medical science.

More information about the results for this trial can be found in the scientific results summary available on the Novartis Clinical Trial Results website (www.novctrd.com).

Once on the site, click **"Clinical trial results"** at the bottom of the page. After agreeing to enter the Novartis website, type **CHSC835X2202** into the keyword search box and click **"Search"**. If you have questions about the results, please speak with the doctor or staff at the trial site.

This trial was registered on the following website:

- Clinical Trials.gov (https://clinicaltrials.gov/) - National Clinical Trial # NCT01930162

If more clinical trials are planned, they will be listed on the above public websites or www.novartisclinicaltrials.com. Search for **"HSC835"**.

Thank you

Clinical trial patients belong to a large community of patients around the world. Patients help researchers answer important health questions and test new medical treatments.

CISCRP

The Center for Information & Study on Clinical Research Participation (CISCRP) is a non-profit organization focused on educating and informing the public about clinical research participation. CISCRP is not involved in recruiting patients for clinical trials, nor is it involved in conducting clinical trials.

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