Clinical Study Results

CSL Behring

Research Sponsor: CSL Behring

Medicine Studied: IgPro20

Study Purpose: A study to find out how safe IgPro20 is in people with chronic inflammatory demyelinating polyneuropathy

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Thank you!

Thank you for taking part in the clinical study for IgPro20, also called Hizentra®. You and all of the participants helped study doctors learn more about IgPro20 and how safe it is in people with chronic inflammatory demyelinating polyneuropathy, also called CIDP.

CSL Behring sponsored this study and thinks it is important to share the results of the study with you and the public. CSL Behring hopes it helps you understand and feel proud of your important role in medical research.

If you have questions about the results, please speak with the doctor or staff at your study site.

What is happening with the study now?

The study started in July 2014 and ended in July 2017. You were treated with IgPro20 for up to 48 weeks during the study.

The study included 82 participants in Australia, Canada, the Czech Republic, France, Germany, Italy, Japan, the Netherlands, Spain, the United Kingdom, and the United States.

CSL Behring reviewed the data collected when the study ended and created a report of the results. This is a summary of that report.

Why was the research needed?

Researchers are looking for a better way to prevent the relapse, or return, of CIDP symptoms. People with CIDP have immune cells that wrongly attack their own nerves in the body. This can cause pain, numbness, tingling, or muscle weakness that gets worse over time.

IgPro20 is a type of medicine called an immunoglobulin. Immunoglobulins are made using certain antibodies from the blood of healthy donors. Antibodies are made by the body's immune system to fight off infection. Doctors are now able to use immunoglobulins as medicines to treat a variety of medical conditions, including CIDP. Current treatments for CIDP that use an antibody medicine have to be given directly into a vein, called an intravenous, or IV, treatment. This can require hospitalization and can be inconvenient for patients.

In this study, doctors wanted to learn more about IgPro20 given as an infusion under the skin as a more convenient treatment option. An infusion under the skin is similar to an IV treatment, but the infusion gets medicine into the body slower, more steadily, and can be done at home.

Before a medicine can be approved for patients to take, study doctors do clinical studies to find out if it works in participants with the disease and if it is safe. In this study, doctors wanted to find out if participants had any medical problems after IgPro20 treatment.

The main questions study doctors wanted to answer in this study were:

- How many adverse events happened per infusion?
- What medical problems did participants have during the study?

What kind of study was this?

To answer the questions in this study, study doctors asked for the help of men and women with CIDP who had previously participated in another study with IgPro20. The participants in this study were 28 to 83 years old.

This was an "open-label" study. This means the researchers and the participant knew what the participant was taking. In this study, all participants took IgPro20.

What happened during the study?

To join this study, participants either:

- Completed a previous IgPro20 study called IgPro20_3003.
- Used the rescue treatment IgPro10 during that previous study to treat the return of any CIDP symptoms. Rescue medicine is medicine that patients could take if they needed urgent help for their CIDP symptoms.

During this study, treatment lasted for 48 weeks. Participants took 1 of the following treatments once a week through an infusion under the skin:

- 0.2 grams per kilogram of body weight, also called g/kg, of IgPro20
- 0.4 g/kg of IgPro20

Participants got a higher or lower dose of IgPro20 depending on when they started the study:

- Participants who started with 0.2 g/kg IgPro20 could stay with this dose until finishing the study if their CIDP symptoms did not return. Participants who started with 0.2 g/kg IgPro20 increased their dose to 0.4 g/kg IgPro20 if their CIDP symptoms returned. If they recovered from those symptoms, they could stay on 0.4 g/kg IgPro20 until finishing the study. If they did not recover from those symptoms, they left the study.
- Participants who started with 0.4 g/kg IgPro20 decreased their dose to 0.2 g/kg IgPro20 if their CIDP symptoms did not return. If their CIDP symptoms returned after that, they could go back to taking 0.4 g/kg IgPro20. If they did not recover from those symptoms, they left the study. If their CIDP symptoms returned after starting with 0.4 g/kg IgPro20, they could choose to continue taking 0.4 g/kg IgPro20 to help recover from the symptoms.

Throughout the study, the study doctors:

- checked participants' overall health and took blood samples
- asked participants how they were feeling and what medicines they were taking
- checked participants' CIDP symptoms

Participants had a final follow-up visit about 1 week after their last treatment dose.

The chart below shows how the study was done:

Before this study	Open-label treatment: 82 participants	Follow-up
• Participants completed previous IgPro20 study OR • Participants used IgPro10 as rescue medicine to treat the return of CIDP symptoms in the previous study	• Participants got either 0.2 g/kg or 0.4 g/kg of IgPro20 once a week 48 weeks	• Participants had a final follow-up visit 1 week later

What were the results of the study?

This is a summary of the main results from this study overall. The results each participant had might be different from the overall summary results. A full list of the questions researchers wanted to answer can be found on the websites listed at the end of this summary. Once a full report of the study results is available, it may also be found on these websites.

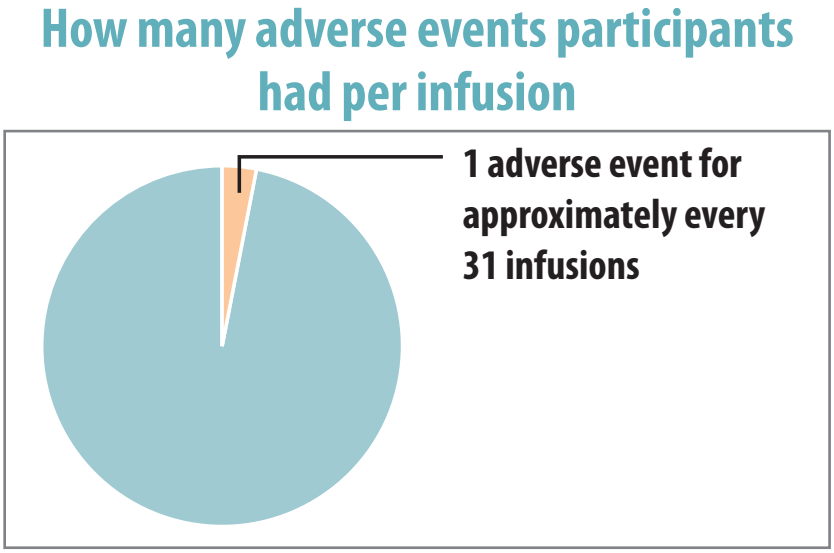
It takes many studies to decide which treatments work best and are safest. Other studies may provide new information or different results.

How many adverse events happened per infusion?

The researchers wanted to know how many adverse events happened per infusion. To find out, the researchers counted how many adverse events happened in the study and compared that to the number of infusions given to the participants.

During this study, there were 180 adverse events and 5,553 infusions. This idea can also be expressed as the number of adverse events participants had per number of infusions. The researchers found that there was 1 adverse event for approximately every 31 infusions.

The number of adverse events the participants had per infusion during the study is shown in the chart below.



What medical problems did participants have?

This section is a summary of the “adverse events” that happened during the study. An adverse event is any sign or symptom that participants have. An adverse event is considered “serious” when it is life-threatening, causes lasting problems, is medically significant, requires hospital care, or results in death.

Adverse events may or may not be caused by the treatments in this study. A lot of research is needed to know whether a treatment causes an adverse event. Doctors keep track of all the adverse events that happen in studies, even if they do not think the adverse events might be related to the treatments.

How many participants had adverse events?

There were 75.6% of participants who had adverse events during the study. This was 62 out of 82 participants.

The table below shows how many participants in the study had adverse events.

Adverse events during this study

	IgPro20 (Out of 82 total participants)
How many participants had adverse events?	75.6% (62)
How many participants had serious adverse events?	8.5% (7)
How many participants stopped treatment because of adverse events?	3.7% (3)

How many participants had serious adverse events?

There were 8.5% of participants who had serious adverse events during the study. This happened in 7 out of 82 participants.

The serious adverse events that happened in this study were:

- Hole in the gallbladder
- Abnormally quick heart beat
- Trapped nerve
- Life-threatening complication from a chest infection
- Urinary tract infection
- Buildup of thickened stool in the large intestine or rectum
- Return of serious CIDP symptoms
- Back pain

Study doctors did not think any of these serious adverse events were caused by the treatment.

None of the participants in this study died because of serious adverse events.

What adverse events did the participants have?

The most common adverse event was the common cold. The adverse events that happened in at least 5% of total participants during the study are listed below.

Most common adverse events during this study

Adverse event	IgPro20 (Out of 82 total participants)
Common cold	13.4% (11)
Swelling where the infusion was given	11.0% (9)
Redness where the infusion was given	8.5% (7)

How has this study helped patients and researchers?

In this study, doctors learned how safe IgPro20 was as a treatment for patients with CIDP.

Researchers look at the results of many studies to decide which treatment doses work best and are the safest for patients. This summary shows only the main results from one study. Other studies may provide new information or different results. Further clinical studies with IgPro20 are planned.

Always talk to your doctor before making any treatment changes.

Where can I learn more about this study?

You can find more information about this study by searching on the websites listed below. Once a full report of the study results is available, it may also be found there.

- www.clinicaltrials.gov Once you are on the website, type **"NCT02027701"** into the search box and click **"Search"**.
- www.clinicaltrialsregister.eu Once you are on the website, click **"Home and Search"**, then type **"2013-004157-24"** in the search box and click **"Search"**.

The full title of your study is: Multicenter, open-label extension study to investigate the long-term safety and efficacy of IgPro20 in maintenance treatment of chronic inflammatory demyelinating polyneuropathy (CIDP) in subjects completing Study IgPro20_3003

The protocol number of your study is: IgPro20_3004

CSL Behring sponsored this study and has its headquarters at 1020 First Avenue, King of Prussia, PA 19406 USA.

The phone number for the CSL Behring Information Center is 610-878-4000.

The email address for CSL Behring Clinical Trial information is clinicaltrials@cslbehring.com.

Thank you

Clinical study participants belong to a large community of people who take part in clinical research around the world. They help researchers answer important health questions and find medical treatments for patients.

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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 participants for clinical studies, nor is it involved in conducting clinical studies.

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