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 primary or secondary immune deficiencies when taken every 2 weeks

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 its safety in people with primary or secondary immune deficiencies.

CSL Behring sponsored this study and thinks it is important to share the results of the study with you and the public. We hope 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 March 2016 and ended in January 2018. You were treated with IgPro20 for up to 64 weeks during the study.

The study included 25 participants at 2 study sites in Canada.

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

Why was the research needed?

Researchers are looking for a better way to treat primary immune deficiency, also called PID, and secondary immune deficiency, also called SID. In healthy people, the immune system makes proteins called antibodies to fight infections. People with PID have abnormal genes that make it difficult for their immune system to make antibodies. This can cause them to get infections easily. People with SID have problems with their immune systems due to other illnesses or because they take certain medicines. They can also get infections easily, and their immune systems also have difficulty making antibodies.

IgPro20 is a type of medicine called an immunoglobulin. Immunoglobulins are made using antibodies from the blood of healthy donors. Doctors are now able to use immunoglobulins as medicines to treat a variety of medical conditions, including PID and SID. Current treatments for PID and SID use an immunoglobulin medicine that is given directly into a vein, called an intravenous or IV treatment. It can also be taken as an infusion under the skin, called a subcutaneous or SC treatment. IV treatment requires hospitalization and can be inconvenient for patients.

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

In this study, the doctors also wanted to find out more about how safe IgPro20 is when taken every 2 weeks in people with PID or SID. IgPro20 was already approved to use once a week in people with PID or SID.

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

- How many medical problems happened per year when a participant got the study treatment every 2 weeks?
- What medical problems did participants have during the study?

What kind of study was this?

To answer the questions in this study, the study doctors asked for the help of men, women, and children with PID or SID who were already getting immunoglobulin treatment. The participants in this study were 6 to 66 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?

Before the study started, the doctors did a physical exam and asked about your health to make sure you could join the study. They also checked your PID or SID symptoms.

This study had 2 parts:

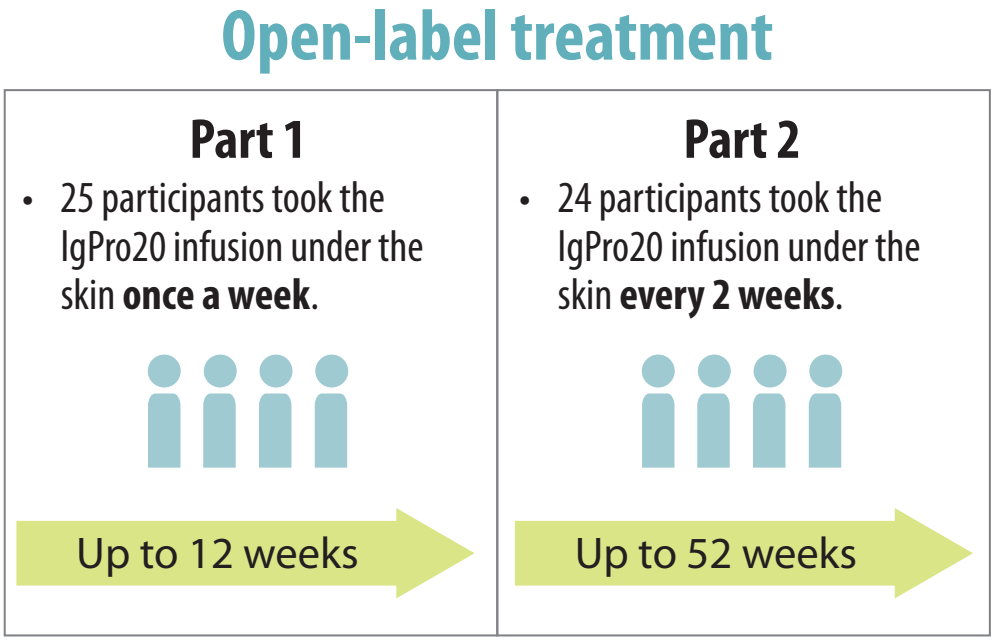
- **Part 1 lasted up to 12 weeks.** During Part 1, 25 participants took IgPro20 once a week at home through an infusion under the skin.
- **Part 2 lasted up to 52 weeks.** During Part 2, 24 participants took IgPro20 every 2 weeks at home through an infusion under the skin. Participants took the first infusion 2 weeks after their last weekly infusion in Part 1. One participant from Part 1 left the study before taking the treatment in Part 2.

Participants had a final visit about 4 weeks after their last treatment dose in Part 2.

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' PID or SID symptoms.

The chart below shows how the treatment was done.



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 medical problems happened per year when a participant got the study treatment every 2 weeks?

An adverse event is any sign or symptom that participants have. Adverse events may or may not be caused by the study treatment.

The researchers wanted to know the average number of adverse events that happened per year when a participant got the study treatment every 2 weeks. They also wanted to know the same average for adverse events that happened at or near where on the body a participant got the infusion. This location is called the infusion site. To find out, the researchers counted how many adverse events happened in the study in a year for participants who got IgPro20 every 2 weeks.

The researchers found that an individual participant who got IgPro20 every 2 weeks had an average of:

- 4.1 adverse events per year
- 0.2 adverse events per year that happened at or near the infusion site

The number of adverse events the participants got per year during the study when they took IgPro20 every 2 weeks is shown in the graphic below.

How many adverse events happened per year for an individual participant?

4.1 the average number of adverse events that happened	**0.2** the average number of adverse events that happened at or near the infusion site

What medical problems did the participants have?

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

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 treatment.

How many participants had adverse events?

There were 24 out of 25 participants who had adverse events during the study. This was 96.0% of all participants. The adverse events seen in this study are commonly seen in patients who get treatment with IgPro20.

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

Adverse events during this study

	IgPro20 (Out of 25 total participants)
How many participants had adverse events?	96.0% (24)
How many participants had serious adverse events?	4.0% (1)
How many participants stopped treatment because of adverse events?	0.0% (0)

How many participants had serious adverse events?

There was 1 out of 25 participants who had a serious adverse event during the study. This was 4.0% of all participants. During Part 2 of the study, this participant had a serious case of the stomach flu. Study doctors did not think this serious adverse event was caused by the treatment.

None of the participants died during this study.

What adverse events did the participants have?

The most common adverse event was the common cold. The adverse events that happened in at least 5.0% of participants during the study are listed on the next page. There were other adverse events, but these happened in a single participant.

Most common adverse events during this study	
Adverse event	IgPro20 (Out of 25 total participants)
Common cold	36.0% (9)
Headache	20.0% (5)
Infection of the nose, throat, or upper airways	16.0% (4)
Joint pain	16.0% (4)
Abdominal pain	12.0% (3)
Nausea	12.0% (3)
Sinus infection	12.0% (3)
Bruising at the injection site	8.0% (2)
Ear infection	8.0% (2)
Inflammation of the tendon	8.0% (2)
Migraine	8.0% (2)
Pain at the injection site	8.0% (2)
A feeling of pins and needles	8.0% (2)
Rash	8.0% (2)
Urinary tract infection	8.0% (2)

How has this study helped patients and researchers?

In this study, doctors learned how safe IgPro20 was as a treatment for patients with PID or SID when taken every 2 weeks.

Researchers look at the results of many studies to decide which treatments work best and are 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 **"NCT02711228"** into the search box and click **"Search"**.
- www.clinicaltrialsregister.eu Once you are on the website, click **"Home and Search"**. Then, type **"2015-004977-34"** in the search box and click **"Search"**.

The full title of your study is: Study of immune deficiency patients treated with subcutaneous immunoglobulin (IgPro20, Hizentra®) on weekly and biweekly schedules

The protocol number of your study is: IgPro20_4005

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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