Clinical Study Results

CSL Behring

Research Sponsor: CSL Behring
Treatment Studied: IgPro20
Device Studied: Investigational Wearable Infusor (IWI)
Study Purpose: A study to learn how much IgPro20 got into the blood using 2 different infusion devices in participants with primary immunodeficiency

Thank you!

Thank you for taking part in the clinical study for IgPro20, also called Hizentra® or human normal immunoglobulin. You and all of the participants helped researchers learn more about using 2 different infusion devices to give IgPro20 to patients with primary immunodeficiency.

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

What is happening with the study now?

The participants were in the study for up to 13 weeks. The entire study took about 14 months to finish. The study started in June 2018 and ended in August 2019. It included 23 participants in 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 the main results of that report.

Why was the research needed?

Researchers are looking for a better way to treat people with primary immunodeficiency. Before a treatment can be approved for patients to take, researchers do clinical studies to find out how it works and how safe it is.

IgPro20 is already approved to use in people with primary immunodeficiency. In this study, the researchers wanted to find out how much IgPro20 got into the blood in participants with primary immunodeficiency.

In people with primary immunodeficiency, their immune system does not work properly. This means their immune system cannot properly defend the body against infection.

Current treatment for people with primary immunodeficiency is called “immunoglobulin replacement therapy”. It helps to prevent infections and other medical problems by replacing missing proteins called antibodies in the immune system.

IgPro20 is used as an immunoglobulin replacement therapy for treating people who have primary immunodeficiency. It is given through a needle under the skin, also called an injection or infusion. An infusion device is needed to help give the infusions. In this study, the researchers compared how much IgPro20 got into the blood using 2 different types of infusion devices.

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

- What was the average total amount of IgPro20 in the blood using different infusion devices?
- Did the participants feel happy with their study treatment?
- Did any participants experience any medical problems during the study?

What kind of study was this?

To answer the questions in this study, the researchers asked for the help of people with primary immunodeficiency who were already on a steady dose of IgPro20, also called Hizentra®. The participants in this study were males and females who were 14 to 75 years old.

This was an “open-label” study. This means the researchers and the participant knew which treatment group the participant was in.

In this study, all the participants got their IgPro20 through an infusion using 2 different types of infusion device. The “Investigational Wearable Infusor” was a new device that was also known as IWI. The researchers wanted to compare this device to the standard infusion device called “Crono Pump”, also known as CP. The participants got the same dose of IgPro20 with both devices that they were already getting before the study.

This study happened in 2 parts. It was planned that all of the participants would complete both parts of the study. Each participant would use the IWI in 1 part, and the CP in the other part. The order that each participant used each device was chosen randomly using a computer program, purely by chance, like the toss of a coin. Researchers do this so that comparing the results of each treatment is as accurate as possible.

What happened during the study?

About 5 weeks before the participants got any study treatment, the participants visited their study site 1 time. The doctors:

- checked the participants' overall health to make sure they could join the study
- checked the participants' blood pressure, height and weight, and took blood samples
- asked about the participants' medical history

During the study, each part lasted for 4 weeks. The participants visited their study site 4 times in each part, and got IgPro20 once a week at the study site. The participants used either the CP or IWI device in each part. In the study:

- 23 participants used the CP device
- 22 participants used the IWI device

There was 1 participant who left the study before it ended and did not get IgPro20 using the IWI device.

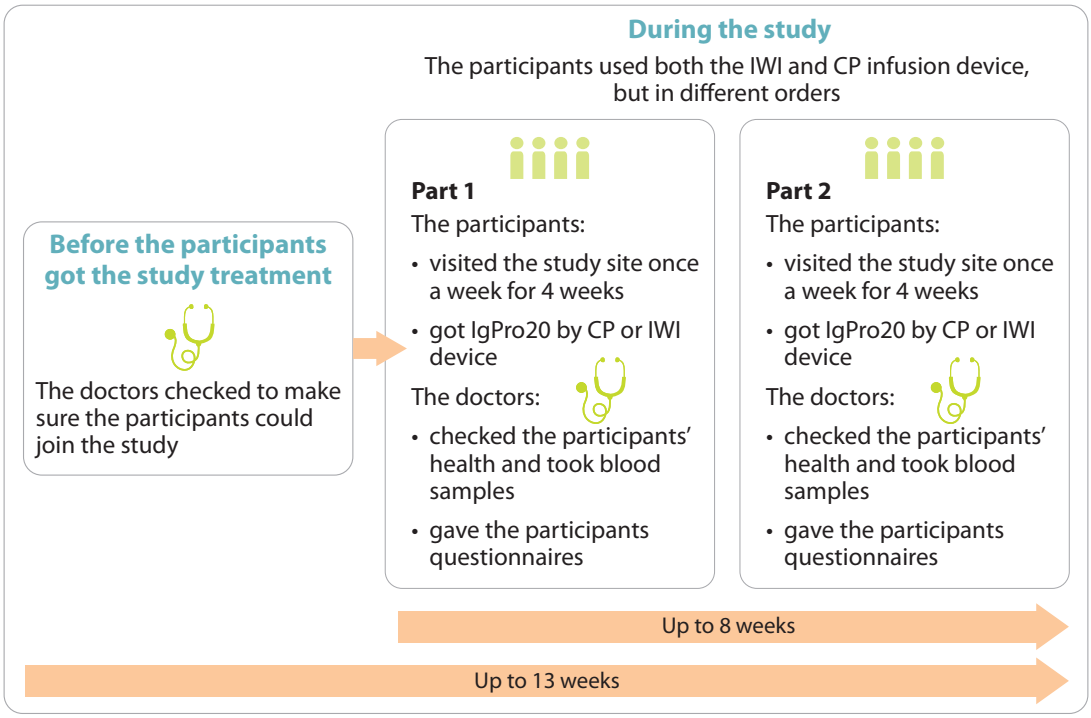
At the first visit of Part 1, the study staff showed the participants how to give their own infusion. At the first visit of each part and throughout the study, the study staff:

- checked the participants' overall health
- did a physical exam
- took blood samples
- gave the participants some questionnaires about their treatment

After the first visit, the participants visited the study site once every week, for 4 weeks. The study staff checked if the participants were having any problems using the CP or IWI device.

After the last dose, the participants stopped using the IWI and CP devices and started using the infusion device they were using before the study.

The chart on the next page shows how the study 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 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.

Not all the participants had all the blood measurements done. The results below are from 20 participants who used both the IWI device and the CP device.

What was the average total amount of IgPro20 in the blood using different infusion devices?

The researchers wanted to know the average total amount of IgPro20 in the blood after the participants used each infusion device.

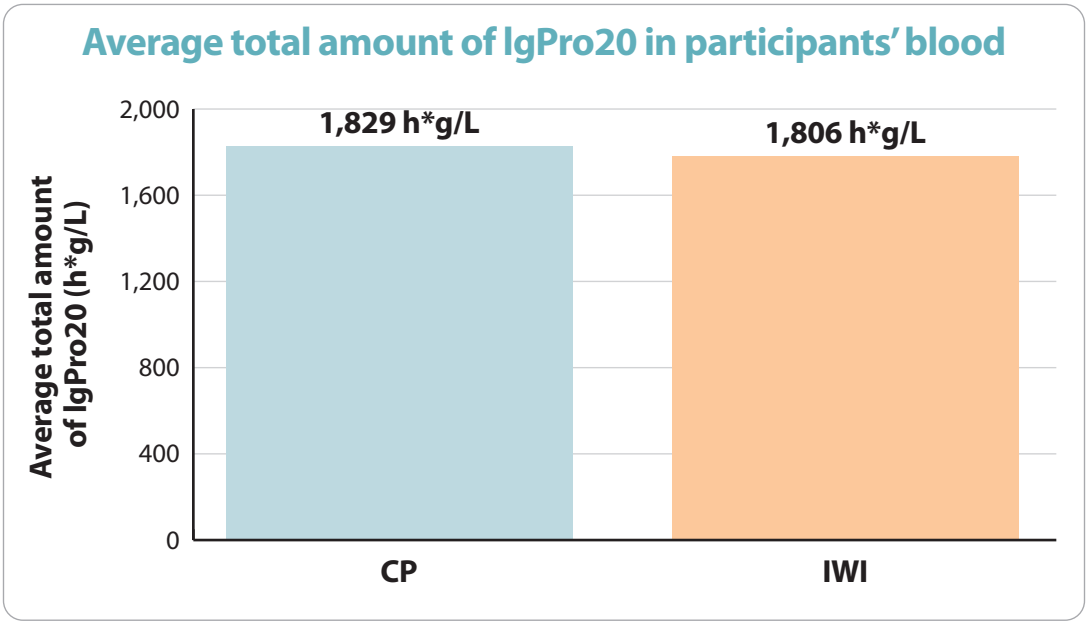
To answer this question, the doctors took blood samples in each part just before the participants got their infusion in week 4. Then, in week 4, they took blood samples 6 times after the participants got their infusion.

The researchers calculated the average total amount of IgPro20 in the participants blood. This was measured in units of grams per liter per hour, also known as h*g/L.

Overall, the average total amount of IgPro20 in the participants' blood was similar for both devices. The amounts were:

- 1,829 h*g/L with the CP device
- 1,806 h*g/L with the IWI device

The chart below shows these results.



Did the participants feel happy with their study treatment?

To answer this question, the doctors asked the participants to answer 3 questionnaires about their treatment using each infusion device. The doctors gave "scores" based on the participants' answers. These questionnaires were the:

- Treatment Satisfaction Questionnaire, also called the TSQ
- Self-Injection Assessment Questionnaire, also called the SIAQ
- Device Preference Questionnaire, also called the DPQ

The TSQ included questions about the convenience of each device and how happy the participants were overall with the treatment. The higher the score, the happier the participant was with the treatment. Overall, TSQ scores were higher when participants used the IWI device compared to when they used the CP device.

The SIAQ included questions that asked the participants how they felt about self-injection. The higher the score, the happier the participant felt about self-injection. Overall, SIAQ scores were higher when the participants used the IWI device compared to when they used the CP device.

The DPQ asked the participants to select the device they preferred. All 22 participants who provided answers preferred the IWI device to the CP device.

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 medical problem that participants have 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.

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 treatment.

How many participants had adverse events?

Overall, 82.6% of participants had adverse events during the study. This was 19 out of 23 participants.

69.6% of participants had adverse events during the study using the CP device. This was 16 out of 23 participants.

54.5% of participants had adverse events during the study using the IWI device. This was 12 out of 22 participants.

None of the participants left the study early because of adverse events.

How many participants had serious adverse events?

None of the participants had serious adverse events during the study.

What adverse events did the participants have?

The most common adverse event during this study was injection site skin hardening.

The table below shows the adverse events that happened during the study in 5% or more of participants. Some participants may have had more than 1 adverse event. There were other adverse events, but these happened in fewer participants.

Adverse events during the study

	CP (23 participants)	IWI (22 participants)
Injection site skin hardening	21.7% (5)	22.7% (5)
Injection site pain	13.0% (3)	4.5% (1)
Injection site redness	13.0% (3)	4.5% (1)
Injection site itching	13.0% (3)	4.5% (1)
Infection of the nose, throat, sinuses, or upper breathing tubes	13.0% (3)	4.5% (1)
Inflammation of the sinuses	13.0% (3)	0.0% (0)

How has this study helped patients and researchers?

This study helped researchers learn more about using 2 different infusion devices to give IgPro20 to participants with primary immunodeficiency.

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. Always talk to a doctor before making any treatment changes.

Further clinical studies with the IWI device are not currently planned.

Where can I learn more about this study?

This summary can be viewed online at www.TrialSummaries.com.

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

- www.clinicaltrialsregister.eu Once you are on the website, click **"Home and Search"**. Then, type **"2016-003798-16"** in the search box and click **"Search"**.

Full study title: Comparison of 2 Infusion Devices With Respect to Pharmacokinetics, Safety, and Tolerability of Hizentra®: An Investigational Wearable Infusor and the Crono S-PID-50 Infusion Pump

EU Clinical Trials number: 2016-003798-16

CSL Behring protocol number: IgPro20_1001

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.

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

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