VPRIV®
velaglucerase alfa
for injection

DISCUSSION GUIDE FOR
PATIENTS & CAREGIVERS

WITH YOU
ALL THE WAY

When starting a new treatment, it's natural to have questions. This guide has been created as a tool to help you discuss these questions with your doctor, so you know what to expect if you decide VPRIV is the right choice for you or a loved one.

INDICATION

VPRIV® (velaglucerase alfa) for injection is a prescription medication indicated for long-term enzyme replacement therapy (ERT) for patients with type 1 Gaucher disease.

IMPORTANT SAFETY INFORMATION

Life-threatening hypersensitivity reactions, including anaphylaxis may occur with VPRIV treatment. This reaction may occur early in treatment or after many doses. Seek immediate help if you experience wheezing, shortness of breath, trouble breathing, itching, hives, rapid heartbeat, swelling of the tongue or throat. VPRIV should be administered under the supervision of a healthcare professional. Appropriate medical support should be available when VPRIV is administered.

Please see additional Important Safety Information throughout and on page 14, and click here for Full Prescribing Information, including WARNING for Risk of Anaphylaxis, and discuss with your doctor.

VPRIV®
velaglucerase alfa
for injection

DOCTOR DISCUSSION GUIDE FOR PATIENTS AND CAREGIVERS

How to use this guide

If you have been recently diagnosed with type 1 Gaucher disease (GD1) or are new to VPRIV, you may have questions or wish to learn more about your condition.

Because your doctor will be your main source of information in your journey, this guide is intended to help you navigate your doctor discussions. It contains a list of questions that may help you, and several resources listed that you may find useful.

WHAT WOULD YOU LIKE TO DISCUSS AT YOUR APPOINTMENT?

☐ Type 1 Gaucher disease
☐ Safety and efficacy of VPRIV treatment
☐ VPRIV dosing and infusions
☐ Other (please specify below)

IMPORTANT SAFETY INFORMATION (CONTINUED)

Hypersensitivity reactions were the most commonly observed side effects in patients treated with VPRIV in clinical studies. The most commonly observed symptoms of hypersensitivity reactions were: headache, dizziness, low blood pressure, high blood pressure, nausea, tiredness/weakness, and fever. Hypersensitivity reactions in the clinical trials include any event considered related to and occurring within up to 24 hours of VPRIV infusion, including one case of anaphylaxis. Generally the reactions were mild and, in patients not previously treated, occurred mostly during the first 6 months of treatment and tended to occur less frequently with time. After the drug was approved, additional hypersensitivity reactions of chest discomfort, difficulty breathing, itching and vomiting have been reported. In some cases, vomiting can be serious and require hospitalization and/or stopping the medication.

WHAT IS TYPE 1 GAUCHER DISEASE?

You may have learned quite a bit about type 1 Gaucher disease already, but often the more we learn, the more questions we have. Are there any topics about GD1 you would like to discuss? (please tick all that apply)

☐ What is type 1 Gaucher disease?
☐ What are the signs and symptoms of type 1 Gaucher disease?
☐ Who does type 1 Gaucher disease affect and how it is inherited?
☐ Should my family members get tested for type 1 Gaucher disease?

Are there any additional aspects of GD1 that you would like to discuss with your doctor? If so, please give some more details below.

Appointment notes.

Please see additional Important Safety Information throughout and on page 14, and click here for Full Prescribing Information, including WARNING for Risk of Anaphylaxis.

DESCRIBE YOUR EXPERIENCE

Type 1 Gaucher disease affects everyone differently. It can be useful to outline the key features of your own unique experience with GD1 to help paint a clearer picture for your doctor. This will help them to help you.

How would you describe your current experience of GD1 to your doctor?

Type 1 Gaucher disease impacts each individual differently, so signs and symptoms may present in different ways for different people. Below are some of the more common signs of GD1, and the presenting symptoms that may link to underlying causes:

- Low red blood cell count – tiredness and weakness
- Enlarged spleen and/or liver – stomach discomfort and pain
- Low platelet (blood-clotting cell) count – bruising and bleeding

How frequently, if at all, are you experiencing these, or other signs or symptoms?

SYMPTOMS	FREQUENCY (1 being not at all and 5 being frequently)
Tiredness and/or weakness	
Stomach discomfort and/or pain	
Bruising and/or bleeding	
Other (please specify below):	

Please remember, not all symptoms of GD1 are easily noticeable or interpretable; that's why it's important to talk to your doctor who will consistently monitor your condition, to ensure the most effective management of your symptoms.

WHAT IS VPRIV?

VPRIV is an enzyme replacement therapy (ERT), indicated for long-term use by patients with type 1 Gaucher disease.

VPRIV is designed to replace the enzyme glucocerebrosidase that patients with GD1 are missing. VPRIV may affect individuals differently and each person's experience with VPRIV will be unique.

VPRIV was studied in the largest clinical study program of an ERT for type 1 Gaucher disease; up to 99 patients in various life stages (adults and children aged 4 years and older) were evaluated across three clinical trials and a long-term extension study. During these trials, the safety and efficacy of VPRIV were established in patients during various stages of life, in both those new to treatment or switching from imiglucerase.

You can find further info about VPRIV in our patient brochure or at www.vpriv.com

Some questions you may want to ask your doctor about the efficacy of VPRIV:

☐ How does VPRIV help manage type 1 Gaucher disease?

☐ Is VPRIV a new medication?

☐ What evidence is there to support the efficacy of VPRIV in patients like me, or with my treatment history?

Are there any other questions you have about the efficacy of VPRIV that you would like to discuss with your doctor? If so, please give some more details below.

IMPORTANT SAFETY INFORMATION (CONTINUED)

If anaphylactic or other acute reactions occur, seek immediate medical care. Your healthcare provider will immediately discontinue the infusion of VPRIV and initiate the appropriate medical treatment. A hypersensitivity reaction should be treated based on the severity of the reaction. Your healthcare provider may manage a reaction by slowing the infusion rate or treating with medicine such as antihistamines, fever-reducing agents and/or corticosteroids or possibly stopping the medication and then restarting with a longer infusion time. For patients who have had symptoms of hypersensitivity reaction to enzyme replacement therapy, the doctor may consider treating the patient with antihistamines and/or corticosteroids before an infusion to help prevent such a reaction from happening.

VPRIV SAFETY AND TOLERABILITY

Here are some topics regarding VPRIV safety and tolerability that you may wish to discuss with your doctor:

- ☐ What are the most common side effects of VPRIV?
- ☐ Does VPRIV have any interactions with other drugs?
- ☐ What information is available if I am, or plan to become, pregnant or start a family?

Are there any other questions you have about the safety of VPRIV that you would like to discuss with your doctor? If so, please give some more details below.

Appointment notes.

IMPORTANT SAFETY INFORMATION (CONTINUED)

The most commonly reported side effects during clinical studies (in ≥10% of patients) were hypersensitivity reactions, headache, dizziness, abdominal pain, nausea, back pain, joint pain, increased time it takes for blood to clot, tiredness/weakness, and fever. In clinical studies, the overall frequency of side effects was generally higher in the patients not previously treated with ERT than in the patients who switched from imiglucerase to VPRIV.

Talk to your doctor if you are pregnant, plan to be pregnant, are breastfeeding, or plan to breastfeed.

HOW TO TAKE VPRIV

VPRIV is a 60-minute infusion taken once every other week at an infusion center, under the supervision of a trained healthcare professional. 'Infusion' means that is administered intravenously (directly into the bloodstream).

Some questions you may like to ask your doctor about VPRIV infusions:

- ☐ How much time should I plan around my infusion (before/after)?
- ☐ Where is my nearest infusion center?
- ☐ What should I bring to my first infusion?
- ☐ What can I do during an infusion?
- ☐ Can anyone come with me to my infusions?
- ☐ Can I have my infusions at home?*
- ☐ How will my VPRIV dose be calculated?
- ☐ Will my dose of VPRIV ever need to change?
- ☐ What should I do if I am due for an infusion, but feeling under the weather?

**Where permitted by local healthcare and reimbursement policy, patients who tolerate VPRIV infusions well over several months may be able to receive infusions at home under the supervision of a healthcare professional.*

Are there any other questions you have about VPRIV infusions that you would like to discuss with your doctor? If so, please give some more details below.

Appointment notes.

Please see additional Important Safety Information throughout and on page 14, and click here for Full Prescribing Information, including WARNING for Risk of Anaphylaxis.

NOTES

Please see additional Important Safety Information throughout and on page 14, and click here for Full Prescribing Information, including WARNING for Risk of Anaphylaxis.

> “You’re going to be OK. You’re going to be brave and do something new. You can do it.”
>
> Emily
>
> *Living with Type 1 Gaucher*

Images throughout this booklet are for illustrative purposes only, and are not of actual patients.

RESOURCES AND SUPPORT

Wherever your type 1 Gaucher disease treatment journey takes you, we will be with you all the way. Below you can find some further resources for more information on VPRIV, as well as places to find support from the GD1 community:

GAUCHER COMMUNITY ALLIANCE (GCA)

The GCA provides emotional support, information, and patient and family resources for anyone affected by Gaucher disease:

www.gauchercommunity.org

NATIONAL ORGANIZATION FOR RARE DISORDERS (NORD)

Discover the advocacy, research, education, and patient services provided by NORD to patients with rare diseases throughout the US:

www.rarediseases.org

NATIONAL GAUCHER FOUNDATION (NGF)

Find out more about the financial, educational, and research programs carried out by the NGF to support the Gaucher community:

www.gaucherdisease.org

JEWISH GENETIC DISEASE CONSORTIUM

A group dedicated to preventing Jewish genetic diseases through awareness:

www.jewishgeneticdiseases.org

NATIONAL SOCIETY OF GENETIC COUNSELORS

An organization that helps people find genetic counselors in their area: these are healthcare professionals who can help guide and support patients with inherited diseases and conditions:

www.nsgc.org

Please see additional Important Safety Information throughout and on page 14, and click here for Full Prescribing Information, including WARNING for Risk of Anaphylaxis.

VPRIV EMAILS

Stay up to date with the latest VPRIV news and updates by signing up to receive occasional emails from us:

www.vpriv.com/stayupdated

VPRIV WEBSITE

Visit for more information about VPRIV, including the largest clinical trial program for GD1 and available support programs, plus further resources:

www.vpriv.com

Takeda Patient Support
Your Source for Education and Access

Takeda Patient Support is a no-cost program offered by Takeda that provides support services to eligible patients and caregivers who are prescribed VPRIV.

www.takedapatientsupport.com

PreppedAhead

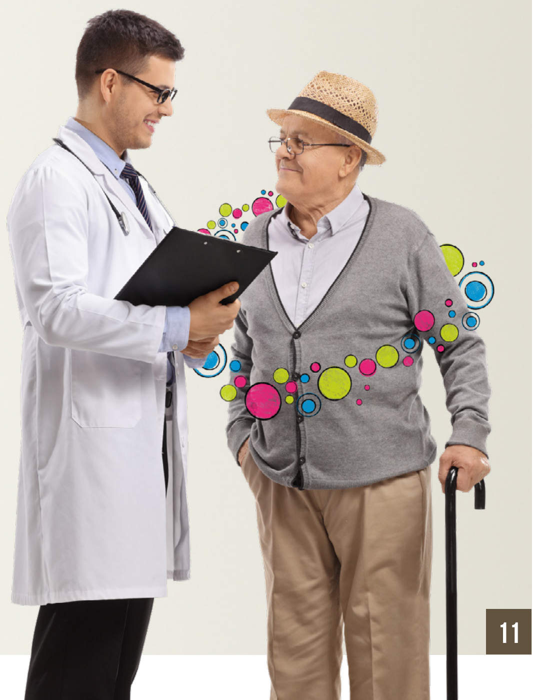
PreppedAhead is a program that provides you with the option of having your site of care prepare your treatment before you arrive - so you don't have to wait as long before your infusion begins.

Ask your doctor about PreppedAhead

IMPORTANT SAFETY INFORMATION (CONTINUED)

The safety and efficacy profiles were similar in pediatric (ages 4 to 17) and adult patients. The safety of VPRIV has not been established in patients under 4 years of age. Side effects more commonly seen in pediatric patients compared to adult patients include (>10% difference): rash, increased time it takes for blood to clot, and fever.

The side effect profile in elderly patients was generally similar to that seen in pediatric and other adult patients. In general, dose selection for an elderly patient should be approached cautiously, considering other existing medical conditions.



> “My piece of advice is just, don’t let it define you. There’s going to be things that you have to change, but at the end of the day, you’re still you.”
>
> Aaron
> *Living with Type 1 Gaucher*

This image is for illustrative purposes only, and does not depict an actual patient.

NOTES

Please see additional Important Safety Information throughout and on page 14, and click here for Full Prescribing Information, including WARNING for Risk of Anaphylaxis.

IMPORTANT SAFETY INFORMATION

INDICATION

VPRIV® (velaglucerase alfa) for injection is a prescription medication indicated for long-term enzyme replacement therapy (ERT) for patients with type 1 Gaucher disease.

IMPORTANT SAFETY INFORMATION

Life-threatening hypersensitivity reactions, including anaphylaxis may occur with VPRIV treatment. This reaction may occur early in treatment or after many doses. Seek immediate help if you experience wheezing, shortness of breath, trouble breathing, itching, hives, rapid heartbeat, swelling of the tongue or throat. VPRIV should be administered under the supervision of a healthcare professional. Appropriate medical support should be available when VPRIV is administered.

Hypersensitivity reactions were the most commonly observed side effects in patients treated with VPRIV in clinical studies. The most commonly observed symptoms of hypersensitivity reactions were: headache, dizziness, low blood pressure, high blood pressure, nausea, tiredness/weakness, and fever. Hypersensitivity reactions in the clinical trials include any event considered related to and occurring within up to 24 hours of VPRIV infusion, including one case of anaphylaxis. Generally the reactions were mild and, in patients not previously treated, occurred mostly during the first 6 months of treatment and tended to occur less frequently with time. After the drug was approved, additional hypersensitivity reactions of chest discomfort, difficulty breathing, itching and vomiting have been reported. In some cases, vomiting can be serious and require hospitalization and/or stopping the medication.

If anaphylactic or other acute reactions occur, seek immediate medical care. Your healthcare provider will immediately discontinue the infusion of VPRIV and initiate the appropriate medical treatment. A hypersensitivity reaction should be treated based on the severity of the reaction. Your healthcare provider may manage a reaction by slowing the infusion rate or treating with medicine such as antihistamines, fever-reducing agents and/or corticosteroids or possibly stopping the medication and then restarting with a longer infusion time. For patients who have had symptoms of hypersensitivity reaction to enzyme replacement therapy, the doctor may consider treating the patient with antihistamines and/or corticosteroids before an infusion to help prevent such a reaction from happening.

The most commonly reported side effects during clinical studies (in ≥10% of patients) were hypersensitivity reactions, headache, dizziness, abdominal pain, nausea, back pain, joint pain, increased time it takes for blood to clot, tiredness/weakness, and fever. In clinical studies, the overall frequency of side effects was generally higher in the patients not previously treated with ERT than in the patients who switched from imiglucerase to VPRIV.

Talk to your doctor if you are pregnant, plan to be pregnant, are breastfeeding, or plan to breastfeed.

The safety and efficacy profiles were similar in pediatric (ages 4 to 17) and adult patients. The safety of VPRIV has not been established in patients under 4 years of age. Side effects more commonly seen in pediatric patients compared to adult patients include (>10% difference): rash, increased time it takes for blood to clot, and fever.

The side effect profile in elderly patients was generally similar to that seen in pediatric and other adult patients. In general, dose selection for an elderly patient should be approached cautiously, considering other existing medical conditions.

As with all therapeutic proteins, there is a potential for developing antibodies to VPRIV. In clinical studies, 1 of 54 (2%) patients who had not previously been treated with ERT, who were then treated with VPRIV, developed antibodies. One additional patient developed antibodies to VPRIV during an extension study. It is unknown if having antibodies to VPRIV is associated with a higher risk of infusion reactions. Patients with an immune response to other enzyme replacement therapies who are switching to VPRIV should continue to be monitored for antibodies to VPRIV.

For additional safety information, please click here for Full Prescribing Information, including WARNING for Risk of Anaphylaxis, and discuss with your doctor.

You are encouraged to report negative side effects of prescription drugs to the FDA. Visit www.fda.gov/medwatch, or call 1-800-FDA-1088.

KEY INFORMATION

PCP's Name

Geneticist's Name

Appointment Time

Address

Phone Number

Infusion Center

Infusion Nurse

Takeda Patient Support Specialist

VPRIV®
velaglucerase alfa
for injection

To learn more about how VPRIV is made and how it works, visit www.vpriv.com. Talk to your healthcare professional to see if VPRIV could be the right choice for you.

Takeda

US-VEL-0536v2.0 08/24