



# WITH YOU ALL THE WAY



## YOUR GUIDE TO MEDICAL SPECIALISTS FOR TYPE 1 GAUCHER DISEASE

### INDICATION

VPRIV<sup>®</sup> (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 15, and click [here](#) for Full Prescribing Information, including WARNING for Risk of Anaphylaxis, and discuss with your doctor.



## INTRODUCTION

Whether you have just started your type 1 Gaucher disease (GD1) and VPRIV journey, are far along, or anywhere in between, this brochure is designed to introduce you to some of the key specialists you may encounter.

Included is an overview of their roles in supporting your overall care and treatment journey, as well as some questions that you can ask each specialist to help have meaningful conversations with your healthcare team.

Your Gaucher treatment provider will help you to establish your VPRIV treatment goals and coordinate your care. They can help determine when you need specialist care or additional support for your individual symptoms.

Some insurance plans allow you to schedule appointments directly with your specialist, while others require you to be referred by your primary doctor. Make sure to check your medical insurance plan details to confirm what process should be followed.

When you are prescribed VPRIV and enrolled in Takeda Patient Support, a specialist can address your questions and concerns and help get you the answers, resources, and tools you need to access your VPRIV therapy.

You can get in touch with Takeda Patient Support at: **1-866-888-0660**

Our [VPRIV Discussion Guide](#) can help you prepare for your regular appointments.



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## CARE FACILITIES

It is important to familiarize yourself with the different care facilities you may visit, so you can be as prepared as possible for wherever your individual journey takes you. See some examples below:



### INDEPENDENT PHYSICIANS OR PROVIDERS

An independent physician or provider works separately from larger healthcare organizations, but can still refer you to larger systems and specialists when required.



### HOSPITALS OR HOSPITAL NETWORKS

Sometimes referred to as healthcare organizations (HCOs), networks can include hospitals, clinics, or foundations. Some networks are owned by one organization, so they can provide you with coordinated care services throughout your journey.



### SITES OF CARE (SOC) OR INFUSION SITES

Your SOC or infusion site is where you receive your regular VPRIV infusions. This will often be a specific infusion center but may be within a larger hospital. Learn more about the staff that will manage your infusions at your SOC or infusion site on [page 12](#).

### 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.

## HEMATOLOGIST (BLOOD SPECIALIST)

### HOW CAN THEY HELP?

A hematologist will address any issues that come up relating to the blood and its components, such as hemoglobin or platelets. They will perform tests to better understand your symptoms and their underlying causes, for example:

- Low levels of red blood cells (anemia), which can cause tiredness
- Low levels of platelets, the cells that help blood to clot, a condition known as thrombocytopenia. This can result in excessive bleeding and bruising

How often you will need blood tests can depend on how you are progressing toward your treatment goals.

### WHAT SYMPTOMS MAY YOUR HEMATOLOGIST HELP MANAGE?

You should tell your doctor about all your symptoms, but a hematologist may have interest in hearing about symptoms that relate specifically to your blood health, including:

- Tiredness/fatigue
- Easy bruising
- Nosebleeds

### WHAT QUESTIONS MIGHT YOU HAVE FOR THEM?

- ? What kinds of tests do you perform?
- ? How might VPRIV alter my hemoglobin levels?
- ? How might VPRIV alter my platelet levels?

## HEPATOLOGIST (LIVER SPECIALIST)

### HOW CAN THEY HELP?

A hepatologist will work to identify, monitor, and address any issues with your liver. They will test your liver function and measure your liver volume.

An enlarged liver is one of the more common signs of GD1; it can cause a swollen abdomen, which can lead to stomach discomfort and pain. If you experience any of these symptoms, speak to your doctor as soon as possible, as the earlier symptoms are recognized, the sooner you may be able to receive treatment if it is required. It is recommended that you see a hepatologist every 6–12 months as a pediatric patient, and every 1–2 years as an adult.

### WHAT SYMPTOMS MAY YOUR HEPATOLOGIST HELP MANAGE?

You should tell your doctor about all your symptoms, but a hepatologist may have interest in hearing about symptoms that relate specifically to your liver health including:

- Loss of appetite
- Jaundice
- Swollen abdomen
- Feelings of fullness
- Abdominal pain
- Diarrhea

### WHAT QUESTIONS MIGHT YOU HAVE FOR THEM?

- ? How might VPRIV help me?
- ? Are there any lifestyle changes I can make to help my liver function?

### 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.

## **INTERNIST** (INTERNAL ORGAN SPECIALIST)

### HOW CAN THEY HELP?

An internist specializes in the workings of your internal organs, including the interactions between them. They may consult other Gaucher specialists and assist in the coordination of your treatment.

Gaucher cells can build up in different organs, particularly in the liver and spleen. If this occurs, an internist may be able to help manage any potential organ-related complications.

### WHAT SYMPTOMS MAY YOUR INTERNIST HELP MANAGE?

You should tell your doctor about all your symptoms, but a hepatologist may have interest in hearing about symptoms that relate specifically to your liver health including:

- Loss of appetite
- Abdominal pain
- Feelings of fullness
- Swollen abdomen

### WHAT QUESTIONS MIGHT YOU HAVE FOR THEM?

- ? What other specialists are you likely to work with for my symptoms/a specific symptom?
- ? How might VPRIV affect my spleen size?

## **PHYSICAL THERAPIST**

### HOW CAN THEY HELP?

Working with a trained physical therapist can help you strengthen your muscles and maintain movement in your joints. This can help to sustain your overall mobility and help with pain management in affected areas of the body.

A physical therapist can put together a plan for you, tailored specifically to your needs and abilities.

### WHAT SYMPTOMS MAY YOUR PHYSICAL THERAPIST HELP MANAGE?

You should tell your doctor about all your symptoms, but a physical therapist may have interest in hearing about symptoms that are relate specifically to your physical health, including:

- Joint pain
- Restricted mobility
- Stiffness

### WHAT QUESTIONS MIGHT YOU HAVE FOR THEM?

- ? How often should I have a physical therapy session?
- ? Are there any exercises I can practice between sessions to maintain my mobility?
- ? Should I plan my VPRIV infusion schedule around my physical therapy appointments?

### IMPORTANT SAFETY INFORMATION (CONTINUED)

The most commonly reported side effects during clinical studies (in  $\geq 10\%$  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.

## MENTAL HEALTH THERAPIST

### HOW CAN THEY HELP?

Receiving a diagnosis for a lifelong condition can be emotionally and psychologically challenging. Talking through your thoughts and feelings with a trained professional can help you to come to terms with various aspects of living with GD1.

Mental health professionals, such as therapists, can provide practical help by providing you with coping strategies, new perspectives, and positive ways of thinking.

### WHAT SYMPTOMS MAY YOUR MENTAL HEALTH THERAPIST HELP MANAGE?

You should tell your doctor about all your symptoms, but a mental health therapist may have interest in hearing about symptoms that relate specifically to your mental health, including:

- Mood changes
- Anxiety
- Low self-esteem
- Feelings of distress, sadness, or isolation

### WHAT QUESTIONS MIGHT YOU HAVE FOR THEM?

- ? How can I speak to my friends and colleagues about my condition?
- ? How can I help to manage any negative emotions I may feel in relation to my diagnosis?
- ? How can I explain to my workplace why I may need accommodations for my VPRIV infusion schedule?

### 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.

## OB/GYN

### HOW CAN THEY HELP?

An obstetrician-gynecologist (OB/GYN) specializes in the care of women during pregnancy and childbirth, and in female reproductive health. An OB/GYN can also help advise women on birth control and infertility issues.

Your OB/GYN will be an important part of your team if you choose to start a family, providing you with guidance before and during pregnancy.

GD1 symptoms can worsen during pregnancy, but your OB/GYN can advise on a management plan to support you throughout your pregnancy.

You will be closely monitored throughout your pregnancy, and your OB/GYN can help you to build your birth plan around any additional needs you may have relating to GD1. They may also refer you to a genetic counselor (see [page 10](#)) for additional support.

### WHAT QUESTIONS MIGHT YOU HAVE FOR THEM?

- ? How will being pregnant affect my GD1?
- ? What information is available about being pregnant and having GD1 and/or being on treatment with VPRIV?
- ? What should I know or consider before planning to become pregnant while on VPRIV?

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



## GENETICIST

### HOW CAN THEY HELP?

Geneticists specialize in working with patients with genetic conditions, such as GD1.

They aim to understand how genetics influence both a patient's current and future symptoms. Geneticists can also help you to understand how GD1 is passed down and answer any questions you have related to family planning.

### WHAT QUESTIONS MIGHT YOU HAVE FOR THEM?

- ? What kind of tests do you perform?
- ? What is the benefit of getting a genetic test?
- ? How should I prepare before the test?
- ? When will I find out the results?

## GENETIC COUNSELOR

### HOW CAN THEY HELP?

Genetic counselors often work with geneticists and can help you navigate some of the decisions you may make in relation to your life with GD1.

For example, if you are thinking of starting a family, they will be there to help explain how GD1 is inherited, the potential ways it can affect your children, and the likelihood of each outcome. A genetic counselor can also help you to organize genetic screenings as part of your family planning.

They will also be there to help you understand the results of genetic tests, answer any questions you have, and equip you with the information you need to make decisions about your future.

Sometimes, genetic counselors may also have a role in your disease management and monitoring.

### WHAT QUESTIONS MIGHT YOU HAVE FOR THEM?

- ? If my partner and I are both affected or carriers of GD1, what are the chances of our children having it or being carriers too?
- ? What does it mean to be a carrier of GD1?

### IMPORTANT SAFETY INFORMATION (CONTINUED)

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.



## INFUSION NURSE

### HOW CAN THEY HELP?

Your infusion nurse will be there to set up your infusions, as well as to support and monitor you throughout. They should be able to answer questions you have about your infusions.

Office staff at infusion sites will also be an important part of your care team: they will help you book follow-up appointments and keep track of the ones you have.

### WHAT QUESTIONS MIGHT YOU HAVE FOR THEM?

- ? How long will my VPRIV infusions take?
- ? Is it possible to have my infusions at home?
- ? Who should I speak to at the infusion center about scheduling any follow-up appointments?

### PreppedAhead<sup>®</sup>

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.

This service is only available to patients enrolled in Takeda Patient Support at sites of care that are enrolled in the PreppedAhead program. Talk to your Takeda Patient Support Manager about PreppedAhead today.

Ask your doctor about PreppedAhead or [click here](#) for more information.

### IMPORTANT SAFETY INFORMATION (CONTINUED)

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.

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## HOME CARE NURSE

### HOW CAN THEY HELP?

If you are eligible and choose to, you can get your infusions at home with a home care nurse. Much like at an infusion center, your home care nurse should be equipped to answer questions you have about your infusions and will monitor you throughout your infusions.

### WHAT QUESTIONS MIGHT YOU HAVE FOR THEM?

- ? How will the home infusion process differ from what I am used to at an infusion center or other SOC?
- ? Is there anything I need to do at home in preparation for my VPRIV infusions?



## DIETICIAN

### HOW CAN THEY HELP?

Dietitians can make recommendations for your diet and tailor meal plans best suited to your individual needs and GD1 symptoms.

GD1 directly affects the immune system, meaning that your diet can play a role in your overall health throughout your GD1 journey. Your dietitian can help you with this by advising on a meal plan that offers the right balance of foods, and making recommendations on vitamins that support a healthy immune system.

### WHAT QUESTIONS MIGHT YOU HAVE FOR THEM?

- ? Are there changes I can make to my diet that could help my overall health?
- ? What types of food are good for supporting a healthy immune system?
- ? Are there certain foods I should eat or avoid on my VPRIV infusion days?
- ? Will I need to change my diet as a result of being on VPRIV?

**Tell your healthcare provider about all the medicines you take, including prescription medicines, over-the-counter medicines, vitamins, or herbal supplements.**

## SUMMARY

**We hope you continue to use this guide as you navigate life with GD1 and VPRIV.**

Your multidisciplinary team may evolve over time; you will likely encounter the specialists in this guide, and maybe others too. Remember, your primary Gaucher treatment provider will be there to coordinate your care and answer any questions.

For more information about VPRIV, click [here](#) or talk to your doctor.

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**You are encouraged to report negative side effects of prescription drugs to the FDA. Visit [www.fda.gov/medwatch](http://www.fda.gov/medwatch), or call 1-800-FDA-1088.**

For more information, contact Takeda at 1-877-TAKEDA-7 (1-877-825-3327), or by email at [medinfous@takeda.com](mailto:medinfous@takeda.com)

  
**VPRIV**<sup>®</sup>  
velaglucerase alfa  
for injection

To learn more, visit  
[www.vpriv.com](http://www.vpriv.com). Talk to  
your healthcare professional  
to see if VPRIV could be  
the right choice for you.



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