

## LIVING WITH TYPE 1 GAUCHER



## OF PERSON WITH TYPE 1 GAUCHER

# YOUR EXPERIENCE WITH TYPE 1 GAUCHER IS AS UNIQUE AS YOU ARE.

## THIS IS WHY WE CREATED A PRODUCT SUPPORT EXPERIENCE TO MATCH.

It's designed to put you and your very specific needs first. From answering your insurance questions to helping to coordinate your first infusion, we will be here to help you every step of the way. **And that all starts now.**

### INDICATION

VPRIV® (velaglugcerase 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

Hypersensitivity reactions, including serious allergic reactions (anaphylaxis) have occurred. VPRIV should be administered under the supervision of a healthcare professional. VPRIV is given every other week by intravenous infusion that typically takes up to 60 minutes. Appropriate medical support should be available when VPRIV is administered. The most serious side effects in patients treated with VPRIV were hypersensitivity reactions.



## YOU'VE BEEN DIAGNOSED...

### WHAT HAPPENS NOW?

We know that there's a lot to consider right now. Being diagnosed with a condition you may not have heard of before can be overwhelming. Know that we'll be here with you now, just as we've been there for others living with type 1 Gaucher.

It's important to know that you've got options. Those options begin with understanding the condition itself and speaking with your doctor about treatment options.

EMILY

LIVING WITH TYPE 1 GAUCHER

### 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.

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

Please see additional Important Safety Information on pages 11-12 and click [here](#) for Full Prescribing Information.

# WHAT IS TYPE 1 GAUCHER?

Type 1 Gaucher is a rare, inherited genetic disorder. Those affected have a deficiency of an enzyme called glucocerebrosidase, which is responsible for breaking down a fatty substance in the body, known as glucocerebroside. When this enzyme doesn't work properly, glucocerebroside builds up in the cells. These enlarged cells accumulate in the organs (particularly the spleen and liver) and tissues.

Even though all patients are different and may experience type 1 Gaucher differently, there are several common signs and symptoms that occur most frequently in patients. **They are\*:**



.....  
**LOW RED BLOOD CELL COUNT**



.....  
**ENLARGED SPLEEN** and/or liver†



.....  
**LOW PLATELET COUNT**



.....  
**A FULL EVALUATION  
SHOULD BE COMPLETED  
BY YOUR DOCTOR.**

\*This is not the complete list of signs and symptoms.

†**In a clinical trial of treatment-naïve patients for VPRIV® (velaglucerase alfa for injection), a significant reduction in liver size was not demonstrated.**

# WHO GETS TYPE 1 GAUCHER, AND HOW?

Type 1 Gaucher is hereditary, and in order to get the condition, a person must inherit 2 genes with a Gaucher mutation from their parents. If they inherit only one mutated Gaucher gene, then they are considered a Carrier. People can be Carriers without having the condition themselves. They can, however, pass the condition to their children. The best way to confirm Carrier status is to test through genetic screening.

IF BOTH PARENTS  
ARE CARRIERS



=



Then there is a **50% CHANCE** of the child also being a Carrier.



And a **25% CHANCE** that the child will have Gaucher disease.



And a **25% CHANCE** the child will be unaffected.

## HERE IS WHO TYPE 1 GAUCHER AFFECTS:

~1 in 100,000 in the overall population have type 1 Gaucher



~1 in 12 within the Ashkenazi Jewish population are a Carrier of a Gaucher mutation



~1 in 855 within the Ashkenazi Jewish population have type 1 Gaucher



# WHY VPRIV (VELAGLUCERASE ALFA FOR INJECTION)?

## LET'S TALK FOR A MOMENT ABOUT WHAT VPRIV IS, AND HOW IT WORKS.

VPRIV is an enzyme replacement therapy (ERT). VPRIV is designed to replace the deficient enzyme (glucocerebrosidase) that patients with type 1 Gaucher are missing. It is made from a human cell line, and has the same amino acid sequence as the naturally produced enzyme that occurs in your body.



ERTs are administered by infusion, which means that they are injected directly into your bloodstream.

**If you've got more questions about ERTs, the infusion process, or anything else dealing with treatment, talk to your doctor.**



**"WHEN WE FIRST ACTUALLY STARTED GETTING INFUSIONS WE WOULD GO**

to the infusion center. Now that we've switched to every other week home infusions, it's great because we can coordinate Emily's care around our schedules."

SUSAN

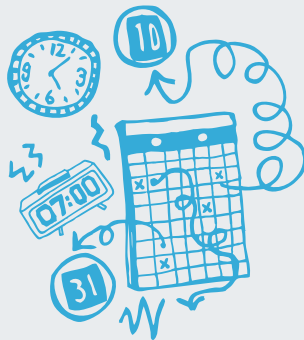
mother of Emily, who lives with type 1 Gaucher

## IMPORTANT SAFETY INFORMATION (CONTINUED)

If anaphylactic or other acute reactions occur, 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 WAS STUDIED IN THE LARGEST-EVER CLINICAL TRIAL OF ENZYME REPLACEMENT THERAPY (ERT) FOR TYPE 1 GAUCHER DISEASE.

Data on file. Largest and most comprehensive clinical trial registration program for ERT in type 1 GD, including 94 patients treated with VPRIV. Shire; Lexington, MA.



For patients 4 years and older  
who are new to treatment,

**VPRIV IS A 60-MINUTE INFUSION  
TAKEN ONCE EVERY OTHER WEEK**

under the supervision of a healthcare professional.



**YOU'VE GOT SUPPORT FROM THE MOMENT  
YOU'RE PRESCRIBED VPRIV.**

### 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.

Please see additional Important Safety Information on pages 11-12 and click [here](#) for Full Prescribing Information.

  
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## SUPPORT AS INDIVIDUAL AS YOU ARE

Our patient programs are delivered with personal attention to help ensure your specific questions are answered and your individual needs are met. Let's spend some time getting to know each of the programs now.



### PRODUCT SUPPORT

**OnePath is designed to provide eligible, enrolled patients, their family members, and caregivers with product support.** As part of OnePath, a dedicated Patient Support Manager will work with you one-on-one to help manage everything from infusion appointments to resources for financial assistance. If you'd like to find out more, please call the number below.

**OnePath Patient Support Managers** are available from 8:30 am-8:00 pm (ET) at 1-866-888-0660. Support is also available 24/7 at [OnePath.com](https://www.onepath.com) or through the OnePath Mobile App for iOS and Android.

### IMPORTANT SAFETY INFORMATION (CONTINUED)

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



AMANDA  
LIVING WITH TYPE 1 GAUCHER





## QUICKSTART: HELP STREAMLINE STARTING TREATMENT

Some insurance plans may require additional paperwork called a prior authorization. QuickStart allows you to:

- **receive your infusion** while this prior authorization is still being reviewed
- **receive up to two free doses of VPRIV**, if eligible and prescribed by your doctor

Additional terms and conditions may apply.

## PREPPEDAHEAD™: EXPEDITE INFUSION PREPARATION



Another service we offer patients using VPRIV is PreppedAhead. What some patients don't realize when first starting VPRIV is that it takes the site of care some time upfront to ready the infusion.

**PreppedAhead provides you with the option of having the infusion center prepare your treatment before you arrive – so you don't have to wait as long before your infusion begins.**

PreppedAhead is available only to patients enrolled in OnePath, and whose site of care is enrolled in the PreppedAhead program.

**Talk to your OnePath Patient Support Manager for more details.**

## CONNECT WITH VPRIV PATIENTS

Want to hear personal stories from another patient? Or maybe you'd like to ask questions of someone who has been where you are now? We offer both a phone-based mentor program and live patient events featuring Takeda Ambassadors who are living with type 1 Gaucher. **Check out [VPRIV.com](https://www.vpriv.com) for more info.**

## 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.



Please see additional Important Safety Information on pages 11-12 and click [here](#) for Full Prescribing Information.

# MEET OTHERS LIVING WITH TYPE 1 GAUCHER



**"I crave adventure and experiencing new things.** It's not easy when you have this type of disease to balance travel with treatment, but after talking with my doctor, I now have a plan in place to make it more manageable."

- AMANDA, LIVING WITH TYPE 1 GAUCHER



TO HEAR MORE PATIENT STORIES,  
VISIT [VPRIV.COM/COMMUNITY](https://vpriv.com/community)

**"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



**"Sharing my story with others, I think, will help people understand a bit more about what the condition is.** And to hear it from a patient can make it more real and help you understand it a lot better."

- KATIE, LIVING WITH TYPE 1 GAUCHER

## IMPORTANT SAFETY INFORMATION (CONTINUED)

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 patients who had not previously been treated with ERT, who were then treated with VPRIV, developed antibodies. 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.

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# OTHER PLACES TO TURN TO FOR INFO ON TYPE 1 GAUCHER

The Gaucher community may be small, but that means it can be close-knit. Check the list below to see where else you can turn to for information and support:

## THE NATIONAL GAUCHER FOUNDATION (NGF)

The NGF helps support the Gaucher community through financial, educational, and research programs. Follow them for the latest info and explore the resources on their website.

## NATIONAL ORGANIZATION FOR RARE DISORDERS (NORD)

A rare disease umbrella organization that provides advocacy, research, education, and patient services in the US. See how they help and find out how you can get involved.

For more resources regarding type 1 Gaucher, visit [VPRIV.com/community](https://vpriv.com/community).

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- If anaphylactic or other acute reactions occur, 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.



Please see additional Important Safety Information on back cover and click [here](#) for Full Prescribing Information.

For more information about VPRIV and the support we offer

VISIT VPRIV.COM

Each patient's experience with diagnosis and treatment of type 1 Gaucher can vary.

**Talk to your healthcare professional to see if VPRIV could be right for you.**

### 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.
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For additional safety information, please click [here](#) for Full Prescribing Information and discuss with your doctor.

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.



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