BioMarin Rare Connections™

# PATIENT SUPPORT PROGRAM ENROLLMENT GUIDE

## One-time infusion. One-to-one treatment support.

ROCTAVIAN™ is the only FDA approved gene therapy for eligible adults with severe hemophilia A. This one-time infusion is matched by one-to-one, personalized product and education support from BioMarin RareConnections™.


**Indication**

ROCTAVIAN is indicated for the treatment of adults with severe hemophilia A (congenital Factor VIII deficiency with Factor VIII activity <1 IU/dL) without antibodies to adeno-associated virus serotype 5 detected by an FDA-approved test.

**Contraindications**

Patients with active infections, either acute (such as acute respiratory infections or acute hepatitis) or uncontrolled chronic (such as chronic active hepatitis B). Patients with known significant hepatic fibrosis (stage 3 or 4 on the Batts-Ludwig scale or equivalent), or cirrhosis, and patients with known hypersensitivity to mannitol.

**Please see Important Safety Information throughout and in the included Prescribing Information and Patient Information.**

# Why enroll in BioMarin RareConnections?

Our dedicated team is here to offer product support and education tailored to your unique needs. We can help you start and continue your ROCTAVIAN™ (valoctocogene roxaparvovec-rvox) journey—from pre-infusion eligibility testing to the one-time infusion and post-infusion follow-up.

## Financial Navigation Support

- **Understand potential out-of-pocket costs** associated with one-time ROCTAVIAN gene therapy
- **Navigate the insurance process** and learn about coverage options to gain access to gene therapy
- **Identify potential financial assistance options**, including co-pay* support for commercially insured patients
  With the ROCTAVIAN Co-Pay Assistance Program*, qualified patients could be reimbursed (up to an annual maximum amount) for eligible out-of-pocket expenses related to ROCTAVIAN drug co-pay, administration, and eligible lab services that are not fully covered by insurance
- **Educate and work with your healthcare provider's office** on insurance coverage requirements for access to gene therapy

## Lab Support

### For Qualified Commercially Insured Patients

- **Mobile blood draw services** for pre-infusion eligibility testing and post-infusion follow-up†
- **Walk-in lab support** (e.g., Quest Patient Service Center) for pre-infusion eligibility and post-infusion follow-up†

# Get started

Enroll today to learn more about pre-treatment eligibility testing and how to gain access to ROCTAVIAN. Complete the Patient Consent Form (PCF) online by **clicking the button** or visiting **ROCTAVIAN-PCF.com**

**Enroll here**

Click the button

*Valid only for those patients with commercial prescription insurance coverage for products who meet eligibility criteria. Offer not valid for prescriptions, administration, or related labs reimbursed, in whole or in part, by any federal, state, or government-funded insurance programs (for example, Medicare, Medicare Advantage, Medigap, Medicaid, VA, DoD, or TRICARE), for cash-paying patients, where product, administration, or related labs are not covered by patient's commercial insurance, where patient's commercial insurance plan reimburses them for entire cost of their prescription drug, administration, and/or related labs, or where prohibited by law or by the patient's health insurance provider. Patients who are residents of certain states (MA or RI) are not eligible for drug administration co-pay support. Patients who are residents of certain states (MI, MN, or RI) are not eligible for laboratory services co-pay support. If at any time a patient begins receiving prescription drug, administration, or related lab coverage under any such federal, state, or government-funded healthcare program, patient will no longer be able to use the BioMarin Co-Pay Assistance Program and patient must notify BioMarin RareConnections at 1-833-ROCTAVIAN (1-833-762-8284) to stop participation. Patients may not seek reimbursement for the value of the out-of-pocket expense amount covered by the Program from any third-party payer, whether public or private. The Program is valid ONLY for qualifying patients residing in the 50 U.S. states or in Puerto Rico with commercial insurance who have a valid prescription for an FDA-approved indication for the qualifying BioMarin therapy. This program is not health insurance. Offer may not be combined with any other rebate, coupon, or offer. Co-payment assistance under the Program is not transferable. BioMarin Pharmaceutical Inc. reserves the right to rescind, revoke, or amend the Program without notice. Patient/caregiver certifies responsibility for complying with applicable limitations, if any, of any commercial insurance and reporting receipt of program rewards, if necessary, to any commercial insurer. The Program is subject to termination or modification at any time. Some restrictions apply.

†Mobile blood draw services and walk-in lab support for blood draws related to ROCTAVIAN gene therapy are for commercially insured patients only. These services are not available to residents of certain states (MI, MN, RI). Some restrictions apply.

## Enroll today to get product support for one-time gene therapy

***Have questions? We're here to help.***

| Visit | Email | Call |
|---|---|---|
| **ROCTAVIAN.com** for product information<br>**BioMarin-RareConnections.com/ROCTAVIAN** for patient support information | **roctaviansupport@biomarin-rareconnections.com** | **1.833.ROCTAVIAN (1.833.762.8284),**<br>hours M–F, 8 AM–8 PM (ET) |

# The only way to know if you're eligible for ROCTAVIAN is to schedule a test with your healthcare team.

Andrew, ROCTAVIAN clinical trial patient

## Indication and Important Safety Information

### What is ROCTAVIAN?

ROCTAVIAN is a one-time gene therapy used for the treatment of adults with severe hemophilia A who do not have antibodies to the virus, AAV5 which is determined by a blood test. ROCTAVIAN uses a modified virus, called a vector, to deliver a working copy of the Factor VIII gene to liver cells to enable your body to produce clotting factor on its own, which helps the blood to clot and prevents or reduces the occurrence of bleeding. The modified virus does not contain viral DNA and does not cause disease in humans.

### Do not take ROCTAVIAN if you:

- Have an active infection or if you have a long-term infection that is not controlled by the medicines you take
- Have scarring of the liver (significant liver fibrosis or cirrhosis)
- Are allergic to mannitol (an inactive ingredient in ROCTAVIAN)

### What is the most important information I should know about ROCTAVIAN?

ROCTAVIAN may cause serious side effects during the infusion and afterward:

- During and in the hours following the infusion, tell your doctor or nurse immediately about any symptoms you experience, including hives or other rashes, itching, sneezing, coughing, difficulty breathing, runny nose, watery eyes, tingling throat, nausea (feeling sick), diarrhea, low blood pressure, rapid heartbeat, light-headedness (near-fainting), fever, chills, or shivering. Talk to your doctor about what to do if you experience any side effects after you leave the infusion
- Before and regularly following administration of ROCTAVIAN, your doctor will perform blood tests to check your liver health. Make sure you obtain these blood tests during the specified time your doctor instructs you to. Based on your liver test results, you may need to take corticosteroids or another medicine for a period of time (several

months or longer) to help decrease liver enzyme levels, which may cause side effects while you receive them. Talk to your doctor about these side effects and what you need to do to improve and maintain your liver's health

- Patients with active Factor VIII inhibitors should not take ROCTAVIAN. Following administration your doctor will monitor you for inhibitors and you will have regular factor level testing. Talk to your doctor if you start bleeding following ROCTAVIAN, in order for your doctor to assess the need for additional tests or treatments
- Depending on your risk factors, an improvement in Factor VIII levels may mean an increased possibility of unwanted blood clots (so called "thromboses," in either veins or arteries). You and your doctor should discuss your risk factors before and after treatment and how to recognize symptoms of unwanted clots and what to do if you think you may have one
- ROCTAVIAN can insert itself into the DNA of human body cells. The effect that insertion may have on those cells is unknown, but such events may contribute to a theoretical risk of cancer. There have been no reported cases of cancer caused by treatment with ROCTAVIAN. Your doctor may perform regular monitoring if you have pre-existing risk factors for developing liver cancer. In the event of cancer, your doctor may send a sample to BioMarin Pharmaceutical Inc. for further testing

## What should I tell my doctor before I get ROCTAVIAN?

Talk to your doctor about the following:

- **Your medical conditions including:**
  - Any general risk factors for unwanted blood clots and for cardiovascular disease
  - If your immune system's ability to fight infections is reduced
  - If you have inhibitors or a history of inhibitors to Factor VIII
- **All medicines you take or new medicines you plan to take,** including prescription and nonprescription drugs, vitamins, herbal supplements, and vaccines
- If you have a female partner that plans to become pregnant within 6 months of treatment

## What should I avoid after taking ROCTAVIAN?

- Avoid alcohol use for the first year. Talk to your doctor about how much alcohol may be acceptable after the first year
- You and any female partner must prevent becoming pregnant for 6 months. Discuss with your doctor which methods of contraception are suitable
- Do not donate semen for at least 6 months after treatment
- Do not donate blood, organs, tissues, or cells

## What are the possible side effects of ROCTAVIAN?

- **The most common side effects of ROCTAVIAN are:**
  - Nausea, fatigue, headache, infusion-related reactions, vomiting, and abdominal pain
  - Changes to laboratory results from blood tests that measure your liver health and other ways your body is responding to ROCTAVIAN

## What other information should I know before getting ROCTAVIAN?

- **Receiving gene therapy again in the future:** It is not yet known whether or under which conditions you may be able to repeat ROCTAVIAN treatment or use another gene therapy
- **Hemophilia treatment registry:** After treatment with ROCTAVIAN, you will be asked to enroll in a 15-year registry to help study the long-term safety of the treatment and how well it continues to work
- **Understanding the risks and benefits of ROCTAVIAN:** While the majority of patients experience a benefit from ROCTAVIAN, the treatment response and duration may vary. Some patients do not experience a benefit from ROCTAVIAN. It is not possible to predict if and how much a patient may benefit. After administration, your doctor will monitor your lab tests and talk to you about whether you can stop prophylaxis, whether you should start prophylaxis again, and whether and how you should treat any surgeries, procedures, injuries, or bleeds

Talk to your doctor about the potential risks and benefits of ROCTAVIAN. Whether a patient experiences a benefit or not, the risks discussed here and with your doctor still apply.

These are not all the possible side effects of ROCTAVIAN. Talk to your doctor for medical advice about side effects. You may report side effects to BioMarin Pharmaceutical Inc. at 1-866-906-6100 or FDA at 1-800-FDA-1088.

**Please see additional safety information in the Prescribing Information and Patient Information.**

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