**PATIENT INFORMATION**
**ROCTAVIAN™ (rok-'tā-vē-in)**
**(valoctocogene roxaparvovec-rvox)**
**suspension for intravenous infusion**

**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, and 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 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 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

**How will I get ROCTAVIAN?**

- ROCTAVIAN is **a single, one-time infusion given into a vein**. The infusion will be given to you in a medical facility by a healthcare professional. The infusion may take 2 to 5 hours or longer depending on your weight and how you respond to the infusion.
- During and after the infusion, you will be monitored for any possible side effects. Your healthcare provider will determine when you can go home (usually later the same day).

**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.

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.

**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.

**What are the ingredients in ROCTAVIAN?**
**Active ingredient:** valoctocogene roxaparvovec-rvox
**Inactive ingredients:** mannitol, poloxamer 188, sodium chloride, sodium phosphate monobasic dihydrate, sodium phosphate dibasic dodecahydrate, and Water for Injection

Manufactured by:
BioMarin Pharmaceutical Inc.
Novato, CA 94949



For more information, go to www.ROCTAVIAN.com or call 1-866-906-6100.

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