5 YEARS OF DATA NOW AVAILABLE

For eligible adults with severe hemophilia A

Spontaneity can be your focus

One infusion. Years of freedom from continuous prophylaxis is possible.

Individual results may vary. Not all patients respond to ROCTAVIAN.

Actor portrayal.

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).

Please see Important Safety Information throughout and in the accompanying Prescribing Information and Patient Information in pocket.

ROCTAVIAN®
(valoctocogene roxaparvovec-rvox)
Suspension for intravenous infusion

Could ROCTAVIAN be right for you?

ROCTAVIAN is a one-time infusion that is FDA approved and backed by the longest and largest Phase 3 gene therapy clinical study in severe hemophilia A.*

ROCTAVIAN helps people make their own Factor VIII, so they may be able to†:

- Maintain Factor VIII levels in the mild range or higher
- Experience improved bleed control for years
- Stop and stay off continuous prophylaxis for years

Now with 5 years of safety profile data

134 people

with severe hemophilia A received one-time ROCTAVIAN in the clinical trial‡

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

> **Since my ROCTAVIAN infusion, I don't have to dose up before I do something now. I can just go.**
>
> —Andrew S., received ROCTAVIAN in 2018 as part of a clinical trial

*At FDA approval, ROCTAVIAN had the longest and largest Phase 3 clinical study for gene therapy in severe hemophilia A.

†Individual Factor VIII levels produced and durability of levels reached can vary. ROCTAVIAN did not work for everyone. Some patients did not respond to treatment or lost response to treatment.

‡Results in this brochure are based on 5 years of data from people in the ROCTAVIAN clinical study. 134 people were part of the ROCTAVIAN clinical study. 112 people had their data collected for at least 6 months before their infusion (rollover population) and 22 people immediately received their infusion (directly enrolled population).

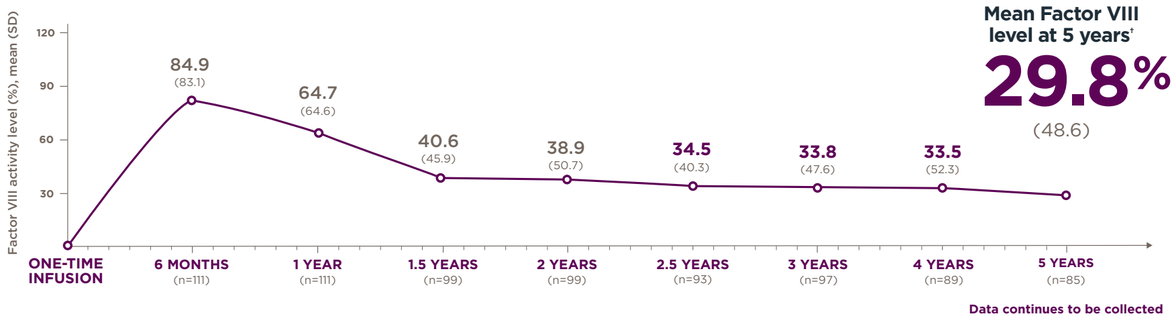
Please see Important Safety Information throughout and in the accompanying Prescribing Information and Patient Information in pocket.

ROCTAVIAN is not intended for administration in women.

ROCTAVIAN®
(valoctocogene roxaparvovec-rvox)
Suspension for intravenous infusion

ROCTAVIAN helped most people make their own Factor VIII for years

5 years after ROCTAVIAN, the average Factor VIII activity level was in the mild hemophilia range*



Mean Factor VIII level at 5 years†

29.8%

(48.6)

Data continues to be collected

What is the most important information I should know about ROCTAVIAN? (cont'd)

- 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

> **It's nice being able to go more places knowing that I don't have to bring Factor VIII with me.**
>
> —Dave N., received ROCTAVIAN in 2024

- ROCTAVIAN was studied in 112 people whose data were collected for at least 6 months before their infusion (rollover population) and 22 people who immediately received their infusion (directly enrolled population)
- Factor VIII activity levels are based on definitions from the World Federation of Hemophilia. Here, we define mild as 5% to <40% and near-normal to normal as 40% to ≤150%
- Some people did not respond to treatment or lost response to treatment. It is not possible to predict if and how much you may benefit from ROCTAVIAN

*The follow-up period began 5 weeks or more after administration and consisted of a median follow-up of 5 years with a range of 1.7 to 5.2 years.

†Factor VIII levels can be measured using either an OSA (shown here) or a chromogenic assay. One-stage results consistently show higher Factor VIII levels.

Please see Important Safety Information throughout and in the accompanying Prescribing Information and Patient Information in pocket.

ROCTAVIAN is indicated for adults only.

ROCTAVIAN®
(valoctocogene roxaparvovec-rvox)
Suspension for intravenous infusion

Most people maintained Factor VIII activity levels in the mild range or higher for years*

84%

of people who had severe hemophilia A before ROCTAVIAN increased Factor VIII levels to the mild range or higher at 5 years (per OSA, n=85)†‡§

What is the most important information I should know about ROCTAVIAN? (cont'd)

- 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

> **Being able to say that my body makes its own factor now, really does mean everything to me.**
>
> —Andrew S., received ROCTAVIAN in 2018 as part of a clinical trial

OSA, one-stage assay.

*Here, we define Factor VIII activity levels: mild as 5% to <40% and near-normal to normal as 40% to ≤150%. Some patients did not respond to treatment or lost response to treatment. It is not possible to predict if and how much you may benefit from ROCTAVIAN. Some patients had elevated Factor VIII levels but didn't respond to ROCTAVIAN.

†Factor VIII levels can be measured using either an OSA (shown here) or a chromogenic assay. One-stage results consistently show higher Factor VIII levels.

‡Available data from rollover population (n=112) at week 260.

§Patients in the clinical trial experienced Factor VIII levels of <1%. Under the 5-year analysis of available, valid data, the minimum range value was 1.5 to 321.2.

Please see Important Safety Information throughout and in the accompanying Prescribing Information and Patient Information in pocket.

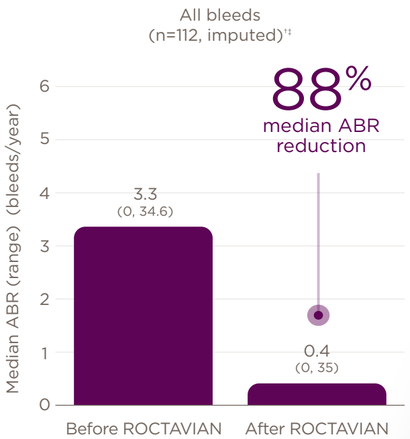
ROCTAVIAN is indicated for adults only. ROCTAVIAN is not intended for administration in women.

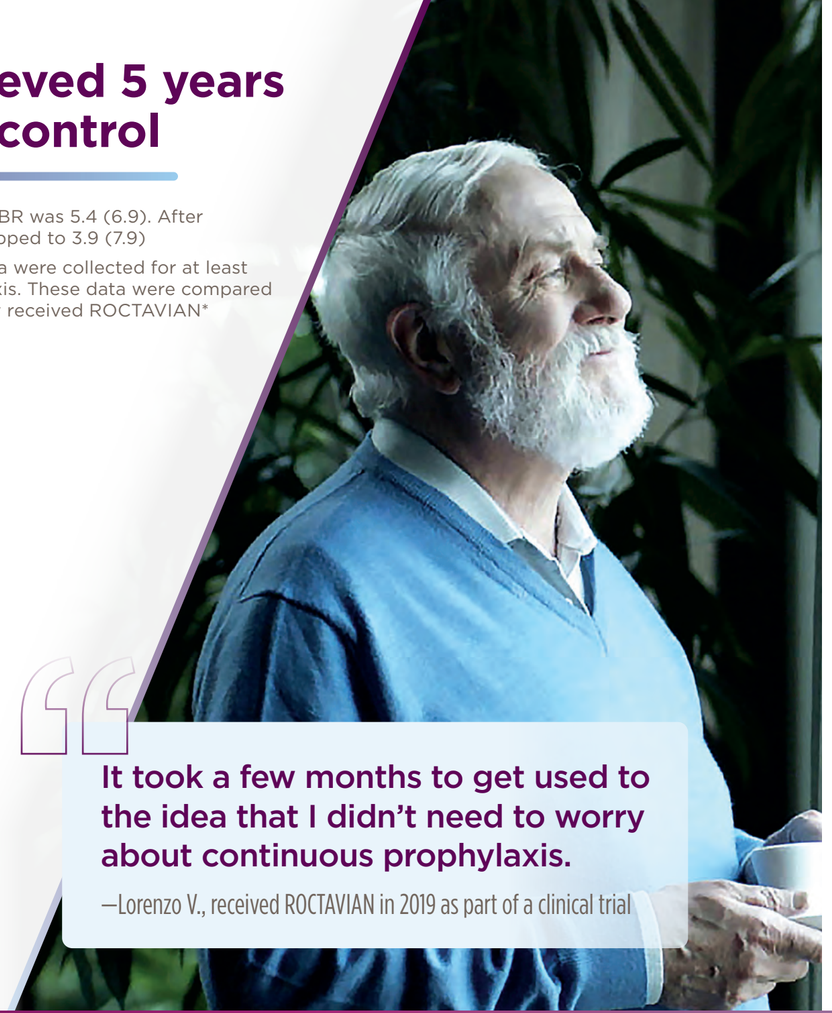
ROCTAVIAN®
(valoctocogene roxaparvovec-rvox)
Suspension for intravenous infusion

In the clinical study

Most people achieved 5 years of durable bleed control

- Before ROCTAVIAN, the average (mean) ABR was 5.4 (6.9). After ROCTAVIAN, the average (mean) ABR dropped to 3.9 (7.9)
- Results are based on 112 people whose data were collected for at least 6 months while they were taking prophylaxis. These data were compared with data from the 5-year period after they received ROCTAVIAN*





> **It took a few months to get used to the idea that I didn't need to worry about continuous prophylaxis.**
>
> —Lorenzo V., received ROCTAVIAN in 2019 as part of a clinical trial

Observed bleeds (n=112)*†‡§

78%
reduction
in **overall** mean ABR
5.4 before ROCTAVIAN vs
1.17 after ROCTAVIAN

77%
reduction
in **spontaneous** bleed mean ABR
2.26 before ROCTAVIAN vs
0.5 after ROCTAVIAN

80%
reduction
in **joint** bleed mean ABR
3.1 before ROCTAVIAN vs
0.6 after ROCTAVIAN

ABR, annualized bleeding rate.

*The 5-year follow-up period began 5 weeks or more after administration and consisted of a median follow-up of 5 years with a range of 1.7 to 5.2 years.

†Median is the middle number in a list of numbers arranged from smallest to largest.

‡19 of 112 people (17%) returned to continuous prophylaxis after ROCTAVIAN, with a median start time at 984 days with a range of 33 to 1467 days. An ABR of 35 was added to account for the periods when these people were on prophylaxis, regardless of the patients' actual bleed rate.

§Observed bleeds are actual bleeding events that are directly documented and recorded during the study without imputation.

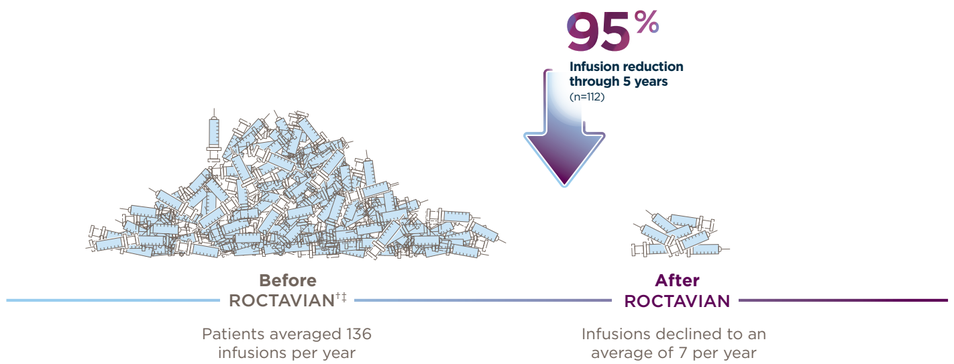
What is the most important information I should know about ROCTAVIAN? (cont'd)

- 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

Please see Important Safety Information throughout and in the accompanying Prescribing Information and Patient Information in pocket.

ROCTAVIAN®
(valoctocogene roxaparvovec-rvox)
Suspension for intravenous infusion

You could be free from prophylaxis for years*



5 years after ROCTAVIAN, more than 70% of people (80 out of 112) had responded to ROCTAVIAN then stopped—and stayed off—continuous prophylaxis.†§

Some people did not respond to treatment or lost response to treatment. In clinical studies, these people were able to resume prophylaxis. Your healthcare team will talk with you to decide what's right for you.

After ROCTAVIAN, your doctor will monitor your lab results and talk to you about whether you can stop prophylaxis and whether you should start prophylaxis again if stopped. In ROCTAVIAN clinical studies, patients who did not respond to treatment or lost response to treatment were able to resume prophylaxis.

Your doctor will also discuss with you whether and how you should treat for any surgeries, procedures, injuries, or bleeds.

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

> **I never imagined a day where I wouldn't have to actively treat with factor.**
>
> —Andrew W., received ROCTAVIAN in 2018 as part of a clinical trial

*Prophylaxis is defined as the ongoing use of Factor VIII or another treatment to prevent bleeds.

†The follow-up period began 5 weeks or more after administration and consists of a median follow-up of 5 years with a range of 1.7 to 5.2 years.

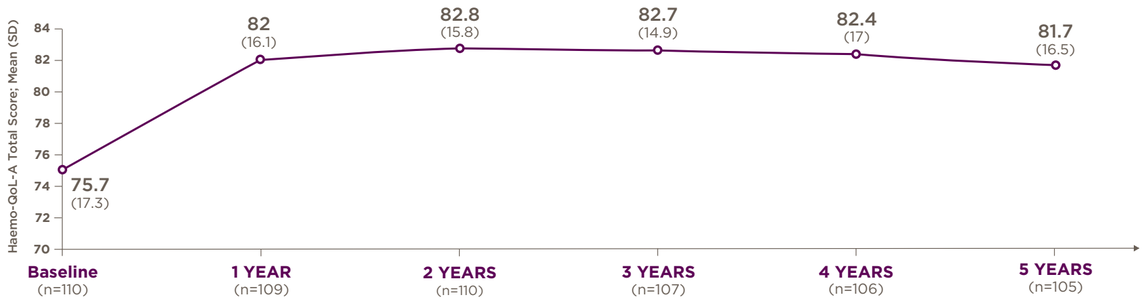
‡Data were collected for 6 months and those results were annualized.

§ROCTAVIAN worked for 71% (80/112) of people in the rollover population and 59% (13/22) of people in the directly enrolled population throughout the 5-year follow-up period.

Please see Important Safety Information throughout and in the accompanying Prescribing Information and Patient Information in pocket.

Health-related quality-of-life changes were measured over 5 years

Average Haemo-QOL-A Total Score (n=112)



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

The Haemo-QoL-A (Haemophilia-Specific Quality of Life Questionnaire) is designed to measure health-related quality of life specifically for people with hemophilia.

Haemo-QoL-A consists of a Total Score and six domain scores: Physical Functioning, Role Functioning, Consequences of Bleeding, Worry, Emotional Impact, and Treatment Concern. Each domain measures specific aspects of HRQoL for people with hemophilia. Participants answer each question on a scale from 0, “none of the time,” to 5, “all of the time.” For analysis, this scale is averaged and converted to 0–100, with higher scores indicating better HRQoL or less impairment.

Study limitations: The ROCTAVIAN Phase 3 study was a single-arm study, which may impact the interpretation of patient-reported outcome findings. The absence of a comparator arm may result in an under- or overestimation of treatment effect. Patient-reported nature of data may impact the reliability of findings. Since formal hypothesis testing was completed after the Year 2 data cutoff per the statistical analysis plan, all data reported here are for descriptive purposes.

I can spend more time with my family. I can do more things with less worry about having a bleed.

—Steven S., dosed with ROCTAVIAN in 2019 as part of a clinical trial

Please see Important Safety Information throughout and in the accompanying Prescribing Information and Patient Information in pocket.

ROCTAVIAN is not intended for administration in women.

ROCTAVIAN®
(valoctocogene roxaparvovec-rvox)
Suspension for intravenous infusion

Safety profile of ROCTAVIAN after 5 years

The safety of ROCTAVIAN is being evaluated in a clinical trial

134 people enrolled*

5 years of data so far

Long-term safety continues to be studied, and data will be collected for years

*Included 112 people whose data were collected for at least 6 months before their infusion (rollover population) and 22 people who immediately received their infusion (directly enrolled population).

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)

In the clinical study, the most common side effects of ROCTAVIAN were:

- nausea (32.1%)
- fatigue (15.7%)
- infusion-related reactions (9%)
- headache (7.5%)
- vomiting (6%)
- abdominal pain (6%)
- Elevated liver enzyme levels that were temporary, asymptomatic, and low grade: 82.8% of people experienced ALT levels above the upper limit of normal

ALT=alanine aminotransferase.

In the clinical study:

ZERO

Zero people developed inhibitors to Factor VIII

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.

Zero people developed blood clots

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 blood clots and what to do if you think you may have one.

Zero people experienced treatment-related deaths

Based on data available at the time of approval. Clinical studies are ongoing.

Select Warnings and Precautions

Before receiving ROCTAVIAN, there are a few things you should know and some precautions that you and your healthcare team will need to take.

Your doctor will monitor your liver health

- 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

Tell your doctor if you experience side effects*

- 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 have your infusion

Other considerations

- 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

*The most common adverse reactions (≥5%) to ROCTAVIAN in the Phase 3 clinical trial were nausea (32.1%), fatigue (15.7%), infusion-related reactions (9%), headache (7.5%), vomiting (6%), and abdominal pain (6%).

Please see Important Safety Information throughout and in the accompanying Prescribing Information and Patient Information in pocket.

ROCTAVIAN®
(valoctocogene roxaparvovec-rvox)
Suspension for intravenous infusion

Getting started with ROCTAVIAN

ROCTAVIAN is for eligible adults with severe hemophilia A. Eligibility testing is important because people with certain conditions, such as pre-existing immunity to the AAV5 virus, cannot receive ROCTAVIAN. The risk for this immunity increases over time, so it's important to test early for eligibility. The good news is that this can be done with simple blood tests at no extra cost to you.

3 steps to get started

STEP 1

Connect with your healthcare team

Consider ROCTAVIAN if you're aged 18+ years with severe hemophilia A

STEP 2

Confirm eligibility

Simple blood tests check for any active infections, Factor VIII inhibitors, pre-existing immunity to the AAV5 virus, and confirm liver health

STEP 3

Schedule your one-time ROCTAVIAN infusion

What other information should I know before getting ROCTAVIAN?

- **Receiving gene therapy again in the future:** ROCTAVIAN is a one-time treatment. Currently, treatment with ROCTAVIAN means you cannot receive another gene therapy for hemophilia
- **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

> **ROCTAVIAN has impacted my treatment routine because I don't have a routine anymore.**
>
> —Dave N., received ROCTAVIAN in 2024

Find healthcare professionals who have been trained on ROCTAVIAN with our Doctor Finder tool

Please see Important Safety Information throughout and in the accompanying Prescribing Information and Patient Information in pocket.

What to expect with ROCTAVIAN

ROCTAVIAN works in the liver, so it's important to keep it healthy

Talk to your doctor about maintaining a health lifestyle before receiving ROCTAVIAN. Other considerations to discuss include:

- Do not drink alcohol for the first 12 months after treatment
- Check with your doctor before taking any medicines, herbal products, or supplements
- Use an effective form of birth control with your female partner for 6 months after ROCTAVIAN
- Do not donate blood, organs, tissues, or cells for transplantation

What other information should I know before getting ROCTAVIAN? (cont'd)

- **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

See how ROCTAVIAN is working at follow-up appointments

For the first 6 months after your ROCTAVIAN infusion, your doctor will perform weekly blood tests to monitor liver health and liver enzyme levels. These show **when your body starts to make its own Factor VIII, how your body is responding, and when you can stop routine prophylaxis**. Over time, you'll need these tests less often.

If your liver enzymes become elevated, your doctor may give you corticosteroids and will monitor you until your levels return to normal. Corticosteroids may cause side effects, which are important to report to your doctor.

Additionally, your doctor will perform ultrasounds if you have pre-existing risk factors to HCC, a type of liver cancer, and monitor for Factor VIII inhibitors if bleeding is not controlled.

Dosing day

Plan ahead

Expect to spend the full day at the infusion center. The process, including testing and monitoring, could take 10 hours or longer.

Stay nearby

If you don't live near the infusion center, we may be able to help with logistics and travel support.

Stream away

Bring a tablet to catch up on your favorite show, a book, or loved one to help pass the time.

Our team of experts is here to help with information about ROCTAVIAN, cost and insurance coverage, and more

Please see Important Safety Information throughout and in the accompanying Prescribing Information and Patient Information in pocket.

ROCTAVIAN®
(valoctocogene roxaparvovec-rvox)
Suspension for intravenous infusion

Our team is here to help you at every step

Enrolling in BioMarin Rare Connections™ connects you to our team of experts

Testing support
Get support for pre-treatment eligibility testing and post-infusion monitoring for qualifying commercially insured patients*

Insurance support
We can help navigate the insurance process and coverage options

Financial support
We can help identify financial assistance for which you may be eligible*

Expedite access
We may be able to help expedite the process of receiving ROCTAVIAN

The one-time cost of ROCTAVIAN is covered by most insurance plans

*Only for commercially insured patients. Eligibility criteria and other program requirements apply. See full terms and conditions at biomarin-rareconnections.com/roctavian/co-pay-assistance/.

What other information should I know before getting ROCTAVIAN (cont'd)

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 Important Safety Information throughout and in the accompanying Prescribing Information and Patient Information in pocket.

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:** ROCTAVIAN is a one-time treatment. Currently, treatment with ROCTAVIAN means you cannot receive another gene therapy for hemophilia
- **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 accompanying Prescribing Information and Patient Information in pocket.

ROCTAVIAN®
(valoctocogene roxaparvovec-rvox)
Suspension for intravenous infusion

One infusion. Years of freedom from continuous prophylaxis is possible.

ROCTAVIAN helps people make their own Factor VIII, so they may be able to*:

- Maintain Factor VIII levels in the mild range or higher
- Experience improved bleed control for years
- Stop and stay off continuous prophylaxis

Now with 5 years of safety profile data

> **It's possible to live with severe hemophilia A without continuous prophylaxis.**
>
> —Dave N., received ROCTAVIAN in 2024

*Individual Factor VIII levels produced and durability of levels reached can vary. ROCTAVIAN did not work for everyone. Some patients did not respond to treatment or lost response to treatment.

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).

BIOMARIN®

ROCTAVIAN®
(valoctocogene roxaparvovec-rvox)
Suspension for intravenous infusion