# Glanzmann's Thrombasthenia (GT) with Refractoriness to Platelets

## IN YOUR WORLD, EXPERIENCE MATTERS



# **Understanding GT**

GT is a rare genetic disorder that affects both men and women and occurs because of mutations in either of two genes: *ITGA2B* or *ITGB3*. Having one copy of either of these gene mutations makes each parent a carrier. Although a carrier is affected by the condition, they typically do not show signs or symptoms of GT. When two copies of the mutated gene from both parents are passed down to their child, a child may be affected by GT.

~1 in 1,000,000 people

Beginning from birth, GT is often recognized by lengthy or spontaneous bleeding. The intensity and frequency of these bleeding episodes can be hard to predict.

### Common signs and symptoms of GT

- Easy bruising
- Frequent bleeding from the nose and gums
- Bleeding beneath the skin that causes red or purple spots to form on the skin (petechiae)
- Tissue bleeding that results in swelling (hematoma)
- Prolonged bleeding from injury, trauma, or surgery (including dental work)
- Abnormally heavy menstrual bleeding
- Increased risk of heavy blood loss during pregnancy and childbirth

Less common bleeding episodes can happen inside the skull or joints.

Additionally, 1 in 4 people with GT have gastrointestinal bleeding as adults.

### The role of platelets

Your blood contains small cells called platelets. When you begin to bleed, platelets rush to the injury site and clump together to help stop the bleeding. However, due to the genetic mutation people with GT are born with, platelets are not able to properly function.

### Use of a bypassing agent

One treatment option for GT is platelet transfusion; some patients who receive platelet transfusions may develop antibodies and become resistant. You may be able to use a bypassing agent to control your bleeds if platelets do not work for you.



Bypassing agents help platelets work around the genetic mutation in order to help your body form normal clots



### NovoSeven® RT:

## **Experience where it matters**

### Controlling bleeds, wherever and whenever they happen

Proven effective to treat GT at home

### Safety supported by clinical experience

• A low rate (<0.2%) of unintended blood clots has been reported

\*Administer as a slow bolus injection over 2-5 minutes, depending on the dose administered.

<sup>b</sup>Compassionate use, also known as expanded access, began enrolling in 1988: FDA approval received in 1999.

### Speed when it's needed

• Fast to mix, fast to infuse, and fast to control bleeds<sup>a</sup>

### NovoSeven® RT—committed to your experience

• More than 30 years of research and long-term clinical experience<sup>b</sup>

#### What is NovoSeven® RT?

NovoSeven® RT (coagulation Factor VIIa, recombinant) is an injectable medicine used for:

- Treatment of bleeding and prevention of bleeding for surgeries and procedures in adults and children with hemophilia A or B with inhibitors, congenital Factor VII (FVII) deficiency, and Glanzmann's thrombasthenia with a decreased or absent response to platelet transfusions
- Treatment of bleeding and prevention of bleeding for surgeries and procedures in adults with acquired hemophilia

### **Important Safety Information**

What is the most important information I should know about NovoSeven® RT? NovoSeven® RT may cause serious side effects, including:

- Serious blood clots that form in veins and arteries with the use of NovoSeven® RT have been reported
- Your healthcare provider should discuss the risks and explain the signs and symptoms
  of blood clots to you. Some signs of a blood clot may include pain, swelling, warmth,
  redness, or a lump in your legs or arms, chest pain, shortness of breath, or sudden
  severe headache and/or loss of consciousness or function
- Your healthcare provider should monitor you for blood clots during treatment with NovoSeven® RT



KALI has GT with refractoriness to platelets.







### **Important Safety Information**

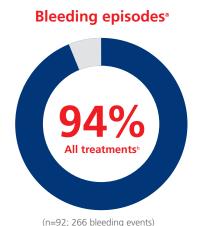
What is the most important information I should know about NovoSeven® RT? (cont'd)

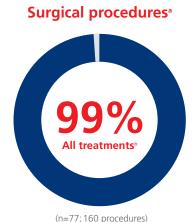
NovoSeven® RT may cause serious side effects, including: (cont'd)

• You should not use NovoSeven® RT if you have ever had allergic (hypersensitivity) reactions, including severe, whole body reactions (anaphylaxis) to NovoSeven® RT, any of its ingredients, or mice, hamsters, or cows. Signs of allergic reaction include shortness of breath, rash, itching (pruritus), redness of the skin (erythema), or fainting/dizziness

## Experience proven bleed control with NovoSeven® RT

### Bleed control seen in a large clinical study





### Effectiveness in children seen in a large clinical study

- 94% bleed control in patients 0 to 12 years
- 100% bleed control in patients >12 to 16 years
- •100% efficacy in surgical procedures in children 0 to 16 years

<sup>a</sup>Effectiveness seen in a prospective, observational, multinational study. bNovoSeven® RT only and NovoSeven® RT with platelets and/or antifibrinolytics.



NovoSeven®RT Coagulation Factor VIIa (Recombinant)

# NovoSeven® RT: **Established safety** profile that comes from experience

### The safety of NovoSeven® RT is well studied

- Works at the site of injury
- < 0.2% rate of unintended blood clots has been reported

# Recombinant manufacturing minimizes the possibility of viral contamination

- NovoSeven® RT is the only recombinant therapy indicated for GTa
- Made without any human blood or plasma

<sup>a</sup>In patients with refractoriness to platelet transfusions, with or without antibodies to platelets.

Serious side effects, including serious blood clots that form in veins and arteries with the use of NovoSeven® RT, have been reported.

### **Important Safety Information**

### What should I tell my healthcare provider before using NovoSeven® RT?

- Tell your healthcare provider if you have any of the following, as these may increase your risk of blood clots:
- congenital hemophilia and are also receiving treatment with aPCCs (activated prothrombin complex concentrates)
- are an older patient particularly with acquired hemophilia and receiving other agents to stop bleeding
- history of heart or blood vessel diseases









### **Important Safety Information**

What should I tell my healthcare provider before using NovoSeven® RT? (cont'd)

 Tell your healthcare provider and pharmacist about all the medicines you take, including all prescription and non-prescription medicines, such as over-the-counter medicines, supplements, or herbal remedies

### What are the possible side effects of NovoSeven® RT?

- The most common and serious side effects are blood clots
- Tell your healthcare provider about any side effects that bother you or do not go away, and seek medical help right away if you have signs of a blood clot or allergic reaction

# Experience **fast mixing and infusing** with NovoSeven® RT

Stopping bleeds quickly is important. The faster you infuse treatment, the sooner it can start to work.

#### NovoSeven® RT with MixPro® is fast to mix

• Prefilled syringe means no extra steps to fill a syringe with diluent

### Fast to infuse

- NovoSeven® RT is given as an intravenous (IV) bolus injection
- NovoSeven® RT provides low infusion volumeª
- Infusing NovoSeven® RT takes only 2 to 5 minutes
- NovoSeven® RT offers a range of vial sizes to fit your needs

### **Room temperature stable**

- Stable at room temperature (77° F) until the expiration date
- After mixing, NovoSeven® RT may be stored at room temperature in the vial (or refrigerated) for up to 3 hours

<sup>a</sup>Administer as a slow bolus injection over 2 to 5 minutes, depending on the dose administered.

Visit **NovoSevenRT.com** to find a local Novo Nordisk Representative to connect you to additional resources, including a Patient Starter Kit





### NovoSeven® RT:

### **Experience where it matters**

# Controlling bleeds, wherever and whenever they happen

• Proven effective to treat GT at home

### **Safety** supported by clinical experience

• A low rate (<0.2%) of unintended blood clots has been reported

### Speed when it's needed

• Fast to mix, fast to infuse, and fast to control bleeds<sup>a</sup>

### NovoSeven® RT—committed to your experience

• More than 30 years of research and long-term clinical experience

### Learn more at NovoSevenRT.com

<sup>a</sup>Administer as a slow bolus injection over 2-5 minutes, depending on the dose administered. <sup>b</sup>Compassionate use, also known as expanded access, began enrolling in 1988; FDA approval received in 1999.

### **Important Safety Information**

What is the most important information I should know about NovoSeven® RT?

### NovoSeven® RT may cause serious side effects, including:

- Serious blood clots that form in veins and arteries with the use of NovoSeven® RT have been reported
- Your healthcare provider should discuss the risks and explain the signs and symptoms of blood clots to you. Some signs of a blood clot may include pain, swelling, warmth, redness, or a lump in your legs or arms, chest pain, shortness of breath, or sudden severe headache and/or loss of consciousness or function
- Your healthcare provider should monitor you for blood clots during treatment with NovoSeven® RT

Please see additional Important Safety Information throughout. Please see accompanying Prescribing Information.

Novo Nordisk Inc., 800 Scudders Mill Road,



Plainsboro, New Jersey 08536 U.S.A.
MixPro® and NovoSeven® are registered trademarks
of Novo Nordisk Health Care AG.
Novo Nordisk is a registered trademark of Novo Nordisk A/S.
© 2019 Novo Nordisk Printed in the U.S.A.
US18NSVN00085 January 2019





**CATHY** has GT with refractoriness to platelets.

