

NOHF MISSION

TO ENHANCE THE QUALITY OF LIFE FOR PEOPLE WITH BLEEDING DISORDERS AND THEIR FAMILIES, THROUGH ADVOCACY, EDUCATION, RESEARCH AND OTHER CONSTITUENCY SERVICES.

NOTE to the Community

By Tanya Ricchi

Happy Fall! It may not look like the harvest seasons of past, but we hope that you and your families are staying healthy and well and finding some joy in the change of the season.

The staff at NOHF are still busy planning programs and events. While nothing matches the warmth of in-person connection, it has still been nice to see familiar community faces in the zoom boxes every few weeks! We have some fun and educational virtual programs planned for the coming months: The Unite for Bleeding Disorders Walk, a bingo night, ladies wreath making, a joint health program, Family Camp, the Annual Meeting, Black and Blue Ball and even a pumpkin carving competition withe the Northwest Ohio Hemophilia Foundation! Check out our calendar on page 7 and join us!

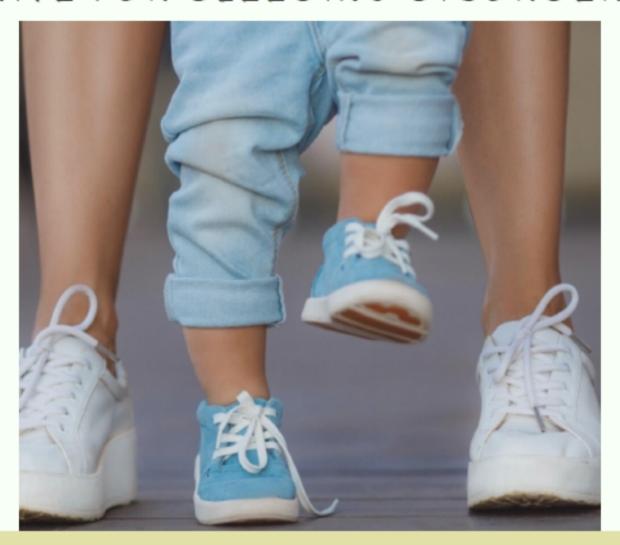
We also have a NEW website that we will keep updated with event information and other pertinent news. Check it out when you have a minute. We hope you find it easy to navigate with plenty of good information and resources.

In closing, we know these times are difficult for so many of our families. Please remember that WE ARE HERE TO HELP YOU. It's our mission and the reason we exist. If you are struggling and need assistance, please reach out to me and we will do what we can to help. We have a separate assistance fund for those whose financial situations have been impacted by COVID and have received additional grant funds from many of our industry, regional and national partners for just this purpose.

We hope to hear from you and see you on our screens soon! -Tanya



UNITE FOR BLEEDING DISORDERS



OUR WALK IS VIRTUAL IN 2020! UNITE TOGETHER, EVEN APART

JOIN US ON SEPTEMBER 12, 2020. ANY CITY, ANY TIME

We can't be together in-person, but we can still UNITE and walk for the NOHF and all those with bleeding disorders.

Register today: www.nohf.org





IT'S ALMOST TIME TO WALK!

We appreciate all of you during this time of uncertainty. While everyone is navigating their new "normal", we want to make sure your safety is our top priority. With that said, it is with a heavy heart the decision was made that our Unite for Bleeding Disorders walk will be virtual this year. Our requests for emergency assistance have increased during the pandemic and your help is needed more than ever.

What does this mean? Although we won't be able to be together at the Akron Zoo this year, we still plan on walking TOGETHER on Saturday, September 12. YOU choose the time and location: your neighborhood, a local park, your backyard. Walk with your team OR individually. You get to choose how you walk! Be sure to take plenty of pictures and videos, upload them and use the hashtag #NOHFUnite2020. Let's "Unite, Even If Apart" this year.

Walkers raising \$25 or more will receive an official Walk t-shirt and a \$10 gift card to Panera. Use this for an individual lunch or pool your cards together and have a celebratory lunch for your whole team! Special thank you to our Exclusive Local Presenting and Lunch sponsor, Akron Children's Hospital. Our deep appreciation for their continued support of this event. Thanks to all our 2020 sponsors: Novo Nordisk, Premier Bank, CSL Behring and Biomarin.

Visit our website, www.nohf.org, to register as an individual or a team. Questions? Contact Dawn, dawn@nohf.org or 216.834.0051











What is HEMLIBRA?

HEMLIBRA is a prescription medicine used for routine prophylaxis to prevent or reduce the frequency of bleeding episodes in adults and children, ages newborn and older, with hemophilia A with or without factor VIII inhibitors.

What is the most important information I should know about HEMLIBRA?

HEMLIBRA increases the potential for your blood to clot. Carefully follow your healthcare provider's instructions regarding when to use an on-demand bypassing agent or factor VIII, and the dose and schedule to use for breakthrough bleed treatment. HEMLIBRA may cause serious side effects when used with activated prothrombin complex concentrate (aPCC; FEIBA*), including thrombotic microangiopathy (TMA), and blood clots (thrombotic events). If aPCC (FEIBA*) is needed, talk to your healthcare provider in case you feel you need more than 100 U/kg of aPCC (FEIBA*) total.

Please see Brief Summary of Medication Guide on following page for Important Safety Information, including Serious Side Effects.

Fall 2020 Page 5

IEML**I**BRA.

emicizumab-kxwh | 100

Medication Guide HEMLIBRA® (hem-lee-bruh) (emicizumab-kxwh)

injection, for subcutaneous use

What is the most important information I should know about HEMLIBRA?

HEMLIBRA increases the potential for your blood to clot. Carefully follow your healthcare provider's instructions regarding when to use an on-demand bypassing agent or factor VIII (FVIII) and the recommended dose and schedule to use for breakthrough bleed treatment.

HEMLIBRA may cause the following serious side effects when used with activated prothrombin complex concentrate (aPCC; FEIBA*), including:

- Thrombotic microangiopathy (TMA). This is a condition involving blood clots and injury to small blood vessels that may cause harm to your kidneys, brain, and other organs. Get medical help right away if you have any of the following signs or symptoms during or after treatment with HEMLIBRA:
 - confusion
 - weakness
 swelling of arms and legs

 - yellowing of skin and eyes
- stomach (abdomen) or back pain nausea or vomiting
- feeling sick decreased urination
- Blood clots (thrombotic events). Blood clots may form in blood vessels in your arm, leg, lung, or head. Get medical help right away if you have any of these signs or symptoms of blood clots during or after treatment with HEMUBRA:
 - swelling in arms or legs pain or redness in your
 - arms or legs

 - shortness of breath chest pain or tightness

 - fast heart rate
- cough up blood
 feel faint
- headache
- numbness in your face
 eye pain or swelling
 trouble seeing

If aPCC (FEIBA*) is needed, talk to your healthcare provider in case you feel you need more than 100 U/kg of aPCC (FEIBA*) total.

See "What are the possible side effects of HEMLIBRA?" for more information about side effects.

What is HEMLIBRA?

HEMLIBRA is a prescription medicine used for routine prophylaxis to prevent or reduce the frequency of bleeding episodes in adults and children, ages newborn and older, with hemophilia A with or without factor VIII inhibitors.

Hemophilia A is a bleeding condition people can be born with where a missing or faulty blood clotting factor (factor VIII) prevents blood from clotting normally.

HEMLIBRA is a therapeutic antibody that bridges clotting factors to help your blood clot.

Before using HEMLIBRA, tell your healthcare provider about all of your medical conditions, including if you:

- are pregnant or plan to become pregnant. It is not known if HEMLIBRA may harm your unborn baby. Females who are able to become pregnant should use birth control (contraception) during treatment with HEMLIBRA, are breastfeeding or plan to breastfeed. It is not known if HEMLIBRA passes into your breast milk.

althcare provider about all the medicines yo take, including prescription medicines, over-the-counter medicines, vitamins, or herbal supplements. Keep a list of them to show your healthcare provider and pharmacist when you get a new medicine.

How should I use HEMLIBRA?

See the detailed "Instructions for Use" that comes with your HEMLIBRA for information on how to prepare and inject a dose of HEMLIBRA, and how to properly throw away (dispose of) used needles and syringes.

- Use HEMLIBRA exactly as prescribed by your healthcare provider.
- Stop (discontinue) prophylactic use of bypassing agents the day before starting HEMLIBRA prophylaxis. You may continue prophylactic use of FVIII for the first week of HEMLIBRA prophylaxis.
- HEMLIBRA is given as an injection under your skin (subcutaneous injection) by you or a caregiver.

- Your healthcare provider should show you or your caregiver how to prepare, measure, and inject your dose of HEMLIBRA before you inject yourself for the first time.
- Do not attempt to inject yourself or another person unless you have been taught how to do so by a healthcare provider.
- Your healthcare provider will prescribe your dose based on your weight. If your weight changes, tell your healthcare provider. You will receive HEMLIBRA 1 time a week for the first four
- weeks. Then you will receive a maintenance dose as prescribed by your healthcare provider. If you miss a dose of HEMLIBRA on your scheduled day, you
- should give the dose as soon as you remember. You must give the missed dose as soon as possible before the next scheduled dose, and then continue with your normal dosing schedule. Do not give two doses on the same day to make up for a missed dose.
- MISBRA may interfere with laboratory tests that measure how well your blood is clotting and may cause a false reading. Talk to your healthcare provider about how this may affect your care.

What are the possible side effects of HEMLIBRA?

See "What is the most important information I should know about HEMLIBRA?"

The most common side effects of HEMLIBRA include:

- redness, tenderness, warmth, or itching at the site of injection
- headache
- joint pain

These are not all of the possible side effects of HEMLIBRA.

Call your doctor for medical advice about side effects. You may report side effects to FDA at 1-800-FDA-1088.

How should I store HEMLIBRA?

- Store HEMLIBRA in the refrigerator at 36°F to 46°F (2°C to 8°C). Do not freeze.
 - Store HEMLIBRA in the original carton to protect the vials
 - from light. Do not shake HEMLIBRA
 - If needed, unopened vials of HEMLIBRA can be stored out of the refrigerator and then returned to the refrigerator. HEMLIBRA should not be stored out of the refrigerator to more than a total of 7 days or at a temperature greater than 86°F (30°C). After HEMLIBRA is transferred from the vial to the syringe, HEMLIBRA should be used right away.

 Throw away (dispose of) any unused HEMLIBRA left in the vial.

Keep HEMLIBRA and all medicines out of the reach of children.

General information about the safe and effective use of

Medicines are sometimes prescribed for purposes other than those listed in a Medication Guide. Do not use HEMLIBRA for a condition for which it was not prescribed. Do not give HEMLIBRA to other people, even if they have the same symptoms that you have. It may harm them. You can ask your pharmacist or healthcare provider for information about HEMLIBRA that is written for health professionals.

That are the ingredients in HEMLIBRA?

Active ingredient: emicizumab-lowh

Inactive ingredients: L-arginine, L-histidine, poloxamer 188, and L-aspartic acid.

Manufactured by: Generatech, Inc., A Member of the Roche Group,
1 DNA Way, South San Francisco, CA 94050-4990
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For more information, go to wewsHEMUBRA com or call 1-866-HEMUBRA.
This Medication Guide has been approved by the U.S. Food and Drug Administration.
Revised: 10/2018



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NOHF 30th Annual Golf Outing

SHAKER HEIGHTS COUNTRY CLUB

MONDAY, JULY 20, 2020

It was another picture-perfect day for the NOHF's 30th and FINAL annual Golf Outing benefitting the Matteo Memorial Fund in memory of Ron and Tim Matteo.

Golfers came out in full force swinging their clubs at the Shaker Heights Country Club. We had raffles galore, and for those golfers who wanted to brag, a putting contest!

Thanks to all our golfers, volunteers and sponsors for their support over all 30 years, we truly appreciate you.

Special thanks to our 2020 sponsors: Akron Children's Hospital, Bayer, CSL Behring, Novo Nordisk and Medexus.



Calendar

September Factor One Source Bingo Night 12th Unite Walk 17th CVS Program "Healthy Joints" 25th-27th October Virtual Family Camp (Kids with a bleeding disorder ages 4-10 and their familieis) 1st Cottrill's Wreath Making Activity 15th Virtual Black & Blue Ball November 28th **Pumpkin Carving** Contest 13th - 14th Virtual Annual Meeting

Visit the NEW website for times and details! www.nohf.org



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
- 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



Novo Nordisk Inc., 800 Scudders Mill Road, Plainsboro, New Jersey 08536 U.S.A.

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In hemophilia with inhibitors,

Bleeds happen:

Take control with NovoSeven® RT

AFTER 30 YEARS OHROLLING BLEET

Controlling bleeds, whenever they happen

 Proven effective to treat hemophilia A or B with inhibitors, at home and in the hospital

Safety supported by clinical trial data

Low rate (0.2%) of blood clots^a

Speed when it's needed

Fast to mix, fast to infuse, and fast to control bleeds^b

NovoSeven® RT—committed to your experience

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

aFor people with hemophilia A or B with inhibitors.

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

Compassionate use, also known as expanded access, began enrolling in 1988; FDA approval received in 1999.

Visit NovoSevenRT.com today to learn more

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
- 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

Please see Brief Summary of Prescribing Information on the following pages.



NOVOSEVEN® RT Coagulation Factor VIIa (Recombinant)

Rx only

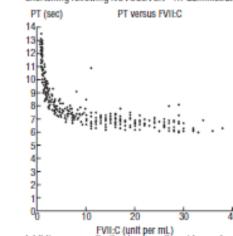
BRIEF SUMMARY. Please consult package insert for full prescribing information.

WARNING: THROMBOSIS: Serious arterial and venous thrombotic events following administration of NOVOSEVEN® RT have been reported. [See Warnings and Precautions] Discuss the risks and explain the signs and symptoms of thrombotic and thromboembolic events to patients who will receive NOVOSEVEN® RT. [See Warnings and Precautions] Monitor patients for signs or symptoms of activation of the coagulation system and for thrombosis. [See Warnings and Precautions]

INDICATIONS AND USAGE: NOVOSEVEN® RT, Coagulation Factor VIIa (Recombinant), is indicated for: Treatment of bleeding episodes and peri-operative management in adults and children with hemophilia A or B with inhibitors, congenital Factor VII (FVII) deficiency, and Glanzmann's thrombasthenia with refractoriness to platelet transfusions, with or without antibodies to platelets; Treatment of bleeding episodes and peri-operative management in adults with acquired hemophilia.

CONTRAINDICATIONS: None known.

WARNINGS AND PRECAUTIONS: Thrombosis: Serious arterial and venous thrombotic events have been reported in clinical trials and postmarketing surveillance. Patients with congenital hemophilia receiving concomitant treatment with aPCCs (activated prothrombin complex concentrates), older patients particularly with acquired hemophilia and receiving other hemostatic agents, or patients with a history of cardiac, vascular disease or predisposed to thrombotic events may have an increased risk of developing thrombotic events [See Adverse Reactions and Drug Interactions J. Monitor patients who receive NOVOSEVEN® RT for development of signs or symptoms of activation of the coagulation system or thrombosis. When there is laboratory confirmation of intravascular coagulation or presence of clinical thrombosis, reduce the dose of NOVOSEVEN® RT or stop the treatment, depending on the patient's condition. Hypersensitivity Reactions: Hypersensitivity reactions, including anaphylaxis, can occur with NOVOSEVEN® RT. Patients with a known hypersensitivity to mouse, hamster, or bovine proteins may be at a higher risk of hypersensitivity reactions. Discontinue infusion and administer appropriate treatment when hypersensitivity reactions occur. Antibody Formation in Factor VII Deficient Patients: Factor VII deficient patients should be monitored for prothrombin time (PT) and factor VII coagulant activity before and after administration of NOVOSEVEN® RT. If the factor VIIa activity fails to reach the expected level, or prothrombin time is not corrected, or bleeding is not controlled after treatment with the recommended doses, antibody formation may be suspected and analysis for antibodies should be performed. Laboratory Tests: Laboratory coagulation parameters (PT/INR, aPTT, FVII:C) have shown no direct correlation to achieving hemostasis. Assays of prothrombin time (PT/INR), activated partial thromboplastin time (aPTT), and plasma FVII clotting activity (FVII:C), may give different results with different reagents. Treatment with NOVOSEVEN® has been shown to produce the following characteristics: PT: As shown below, in patients with hemophilia A/B with inhibitors, the PT shortened to about a 7-second plateau at a FVII:C level of approximately 5 units per mL. For FVII:C levels > 5 units per mL, there is no further change in PT. The clinical relevance of prothrombin time shortening following NOVOSEVEN® RT administration is unknown.



INR: NOVOSEVEN® has demonstrated the ability normalize INR. However, INR values have not been shown to directly predict bleeding outcomes, nor has it been possible to demonstrate the impact of NOVOSEVEN® on bleeding times/volume in models of clinically-induced bleeding in healthy volunteers who had received Warfarin, when laboratory parameters (PT/INR, aPTT, thromboelastogram) have normalized. aPTT: While administration of NOVOSEVEN® shortens the 40 prolonged aPTT in hemophilia A/B patients with

inhibitors, normalization has usually not been observed in doses shown to induce clinical improvement. Data indicate that clinical improvement was associated with a shortening of aPTT of 15 to 20 seconds. FVIIa:C: FVIIa:C levels were measured two hours after NOVOSEVEN® administration of 35 micrograms per kg body weight and 90 micrograms per kg body weight following two days of dosing at two hour intervals. Average steady state levels were 11 and 28 units per mL for the two dose levels, respectively.

ADVERSE REACTIONS: The most common and serious adverse reactions in clinical trials are thrombotic events. Thrombotic adverse reactions following the administration of NOVOSEVEN® in clinical trials occurred in 4% of patients with acquired hemophilia and 0.2% of bleeding episodes in patients with congenital hemophilia. Clinical Trials Experience: Because clinical studies are conducted under widely varying conditions, adverse reaction rates observed in the clinical trials of a drug product cannot be directly compared to rates in clinical trials of another drug, and may not reflect rates observed in practice. Adverse reactions outlined below have been reported from clinical trials and data collected in registries. Hemophilia A or B Patients with Inhibitors: In two studies for hemophilia A or B patients with inhibitors treated for bleeding episodes (N-298), adverse reactions were reported in ≥2% of the patients that were treated with NOVOSEVEN® for 1,939 bleeding episodes (see Table 3 below).

Table 3: Adverse Reactions Reported in ≥2% of the 298 Patients with Hemophilia A or B with Inhibitors

Body System Reactions	# of adverse reactions (n=1,939 treatments)	# of patients (n=298 patients)
Body as a whole Fever	16	13
Platelets, Bleeding, and Clotting Fibrinogen plasma decreased	10	5
Cardiovascular Hypertension	9	6

Serious adverse reactions included thrombosis, pain, thrombophlebitis deep, pulmonary embolism, decreased therapeutic response, cerebrovascular disorder, angina pectoris, DIC, anaphylactic shock and abnormal hepatic function. The serious adverse reactions of DIC and therapeutic response decreased had a fatal outcome. In two clinical trials evaluating safety and efficacy of NOVOSEVEN® administration in the perioperative setting in hemophilia A or B patients with inhibitors (N=51), the following serious adverse reactions were reported: acute post-operative hemarthrosis (n=1), internal jugular thrombosis adverse reaction (n=1), decreased therapeutic response (n=4). Immunogenicity: There have been no confirmed reports of inhibitory antibodies against NOVOSEVEN® or FVII in patients with congenital hemophilia A or B with alloantibodies. The incidence of antibody formation is dependent on the sensitivity and specificity of the assay. Additionally, the observed incidence of antibody (including neutralizing antibody) positivity in an assay may be influenced by several factors including assay methodology, sample handling, timing of sample collection, concomitant medications, and underlying disease. For these reasons, comparison of the incidence of antibodies to NOVOSEVEN® RT with the incidence of antibodies to other products may be misleading. Congenital Factor VII Deficiency: Data collected from the compassionate/emergency use programs, the published literature, a pharmacokinetics study, and the Hemophilia and Thrombosis Research Society (HTRS) registry showed that 75 patients with Factor VII deficiency had received NOVOSEVEN®: 70 patients for 124 bleeding episodes, surgeries, or prophylaxis; 5 patients in the pharmacokinetics trial. The following adverse reactions were reported: intracranial hypertension (n=1), IgG antibody against rFVIIa and FVII (n=1), localized phlebitis (n=1). Immunogenicity: In 75 patients with factor FVII deficiency treated with NOVOSEVEN® RT, one patient developed IgG antibody against rFVIIa and FVII. Patients with factor VII deficiency treated with NOVOSEVEN® RT should be monitored for factor VII antibodies. The incidence of antibody formation is dependent on the sensitivity and specificity of the assay. Additionally, the observed incidence of antibody (including neutralizing antibody) positivity in an assay may be influenced by several factors including assay methodology, sample handling, timing of sample collection, concomitant medications, and underlying disease. For these reasons, comparison of the incidence of antibodies to NOVOSEVEN® RT with the incidence of antibodies to other products may be misleading. <u>Acquired Hemophilia</u>: Data collected from four compassionate use programs, the HTRS registry, and the published literature showed that 139 patients with acquired hemophilia received NOVOSEVEN® for 204 bleeding episodes, surgeries and traumatic injuries. Of these 139 patients, 6 patients experienced 8 serious adverse reactions. Serious adverse reactions included shock (n=1), cerebrovascular accident (n=1) and thromboembolic events (n=6) which included cerebral artery occlusion, cerebral ischemia, angina pectoris, myocardial infarction, pulmonary embolism and deep vein thrombosis. Three of the serious adverse reactions had a fatal outcome. Glanzmann's Thrombasthenia: Data collected from the Glanzmann's Thrombasthenia Registry (GTR) and the HTRS registry showed that 140 patients with Glanzmann's thrombasthenia received NOVOSEVEN® RT for 518 bleeding episodes, surgeries or traumatic injuries. The following adverse reactions were reported: deep vein thrombosis (n=1), headache (n=2), fever (n=2), nausea (n=1), and dyspnea (n=1). Post marketing Experience: Adverse reactions reported during post marketing period were similar in nature to those observed during clinical trials and include reports of thromboembolic adverse events.

DRUG INTERACTIONS: Avoid simultaneous use of activated prothrombin complex concentrates. Do not mix NOVOSEVEN® RT with infusion solutions. Thrombosis may occur if NOVOSEVEN® RT is administered concomitantly with Coagulation Factor XIII. [See Warnings and Precautions]

USE IN SPECIFIC POPULATIONS: Pregnancy: Risk Summary: There are no adequate and well-controlled studies using NOVOSEVEN® RT in pregnant women to determine whether there is a drug-associated risk. Treatment of rats and rabbits with NOVOSEVEN® in reproduction studies has been associated with mortality at doses up to 6 mg per kg body weight and 5 mg per kg body weight respectively. At 6 mg per kg body weight in rats, the abortion rate was 0 out of 25 litters; in rabbits at 5 mg per kg body weight, the abortion rate was 2 out of 25 litters. Twenty-three out of 25 temale rats given 6 mg per kg body weight of NOVOSEVEN® gave birth successfully, however, two of the 23 litters died during the early period of lactation. No evidence of teratogenicity was observed after dosing with NOVOSEVEN®. In the U.S. general population, the estimated background risk of major birth defect and miscarriage in clinically recognized pregnancies is 2-4% and 15-20%, respectively. Lactation: <u>Bisk Summary</u>: There is no information regarding the presence of NOVOSEVEN® RT in human milk, the effect on the breastfed infant, and the effects on milk production. The developmental and health benefits of breastfeeding should be considered along with the mother's clinical need for NOVOSEVEN® RT and any potential adverse effects on the breastfed infant from NOVOSEVEN® RT or from the underlying maternal condition. Pediatric Use: Clinical trials enrolling pediatric patients were conducted with dosing determined according to body weight and not according to age. Hemophilia A or B with Inhibitors; During the investigational phase of product development NOVOSEVEN® was used in 16 children aged 0 to <2 years for 151 bleeding episodes, 27 children aged 2 to <6 years for 140 bleeding episodes, 43 children aged 6 to <12 for 375 bleeding episodes and 30 children aged 12 to 16 years for 446 bleeding episodes. In a double-blind, randomized comparison trial of two dose levels of NOVOSEVEN® in the treatment of joint, muscle and mucocutaneous hemorrhages in hemophilia A and B patients with and without inhibitors 20 children aged 0 to <12 and 8 children aged 12 to 16 were treated with NOVOSEVEN® in doses of 35 or 70 micrograms per kg dose. Treatment was assessed as effective (definite relief of pain/tenderness as reported by the patient and/or a measurable decrease of the size of the hemorrhage and/or arrest of bleeding within 8 hours [rated as excellent = 51%], within 8-14 hours [rated as effective = 18%] or after 14 hours [rated as partially effective = 25%]) in 94% of the patients. NOVOSEVEN® was used in two trials in surgery. In a dose comparison 22 children aged 0 to 16 years were treated with NOVOSEVEN®. Effective intraoperative hemostasis (defined as bleeding that had stopped completely or had decreased substantially [rated as effective = 86%] or bleeding that was reduced but continued [rated as partially effective = 9%]) was achieved in 21/22 (95%) patients. Effective hemostasis was achieved in 10/10 (100%) patients in the 90 mcg/kg dose group and 10/12 (83%) in the 35 mcg/kg dose group at 48 hours; effective hemostasis was achieved in 10/10 (100%) in the 90 mcg/kg dose group and 9/12 (75%) in the 35 mcg/kg dose group at 5 days. In the surgery trial comparing bolus (BI) and continuous infusion (CI) 6 children aged 10 to 15 years participated, 3 in each group. Both regimens were 100% effective (defined as bleeding has stopped completely, or decreased substantially) intra-operatively, through the first 24 hours and at day 5. At the end of the study period (Postoperative day 10 or discontinuation of therapy) hemostasis in two patients in the BI group was rated effective and hemostasis in one patient was rated as ineffective (defined as bleeding is the same or has worsened). Hemostasis in all three patients in the CI group was rated as effective. Adverse drug reactions in pediatric patients were similar to those previously reported in clinical trials with NOVOSEVEN®, including one thrombotic event in a 4 year old with internal jugular vein thrombosis after port-a-cath placement which resolved. Congenital Factor VII deficiency: In published literature, compassionate use trials and registries on use of NOVOSEVEN® in congenital Factor VII deficiency, NOVOSEVEN® was used in 24 children aged 0 to <12 years and 7 children aged 12 to 16 years for 38 bleeding episodes, 16 surgeries and 8 prophylaxis regimens. Treatment was effective in 95% of bleeding episodes (5% not rated) and 100% of surgeries. No thrombotic events were reported. A seven-month old exposed to NOVOSEVEN® and various plasma products developed antibodies against FVII and rFVIIa [see Adverse Reactions and Overdosage]. Glanzmann's Thrombasthenia: In the Glanzmann's Thrombasthenia Registry, NOVOSEVEN® was used in 43 children aged 0 to 12 years for 157 bleeding episodes and in 15 children aged 0 to 12 years for 19 surgical procedures. NOVOSEVEN® was also used in 8 children aged >12 to 16 years for 17 bleeding episodes and in 3 children aged >12 to 16 years for 3 surgical procedures. Efficacy of regimens including NOVOSEVEN® was evaluated by independent adjudicators as 93.6% and 100% for bleeding episodes in children aged 0 to 12 years and >12 to 16 years, respectively. Efficacy in surgical procedures was evaluated as 100% for all surgical procedures in children aged 0 to 16 years. No adverse reactions were reported in Glanzmann's thrombasthenia children. Geriatric Use: Clinical studies of NOVOSEVEN® RT in congenital factor deficiencies and Glanzmann's thrombasthenia did not include sufficient numbers of subjects aged 65 and over to determine whether they respond differently from younger subjects.

OVERDOSAGE: Dose limiting toxicities of NOVOSEVEN® RT have not been investigated in clinical trials. The following are examples of accidental overdose. One newborn female with congenital factor VII deficiency was administered an overdose of NOVOSEVEN® (single dose: 800 micrograms per kg body weight). Following additional administration of NOVOSEVEN® and various plasma products, antibodies against rFVIIa were detected, but no thrombotic complications were reported. One Factor VII deficient male (83 years of age, 111.1 kg) received two doses of 324 micrograms per kg body weight (10-20 times the recommended dose) and experienced a thrombotic event (occipital stroke). One hemophilia B patient (16

years of age, 68 kg) received a single dose of 352 micrograms per kg body weight and one hemophilia A patient (2 years of age, 14.6 kg) received doses ranging from 246 micrograms per kg body weight to 986 micrograms per kg body weight on five consecutive days. There were no reported complications in either case.

More detailed information is available upon request.

For information contact: Novo Nordisk Inc. 800 Scudders Mill Road Plainsboro, NJ 08536, USA 1-877-NOVO-777 www.NOVOSEVENRT.com

Manufactured by: Novo Nordisk A/S 2880 Bagsvaerd, Denmark License Number: 1261

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NovoSeven®RT Coagulation Factor VIIa (Recombinant)





Recognizing annual cumulative donors of \$250 or more.

Platinum: \$5,000+

Special recognition in printed report to the community, in our newsletter and on website. Two NOHF lapel pins AND either two jackets, polo shirts or hats. Four tickets to the Black and Blue Ball. Invitation for four to exclusive annual donor recognition dinner.

Diamond: \$2,500 - \$4,999

Special recognition in printed report to the community, in our newsletter and on website. NOHF lapel pin AND jacket, polo shirt or hat. Four tickets to the Black and Blue Ball. Invitation for two to exclusive annual donor recognition dinner.

Gold: \$1,000 - \$2,499

Special recognition in printed report to the community, in our newsletter and on website.

NOHF lapel pin AND polo shirt or hat. Two tickets to the Black and Blue Ball.

Silver: \$500 - \$999

Special recognition in printed report to the community, in our newsletter and on website.

NOHF lapel pin AND polo shirt or hat.

Bronze: \$250 - \$499Special recognition in printed report to the

community, in our newsletter. NOHF lapel pin



The 340B Factor Program

Specialty Pharmacy & Homecare Service



Exceptional comprehensive care for all bleeding disorders.



For more information, call JD at 330-543-3513 or toll-free 1-800-262-0333, ext. 33513 akronchildrens.org/departments/Hemostasis-and-Thrombosis-Center





A Trusted Partner in Your Circle of Care

- Non-profit pharmacy dedicated to respond to the needs of persons affected by bleeding disorders
- A 340B covered entity offering among the lowest prices for factor available
- Governed by a volunteer board of directors from the bleeding disorders community
- Returns all excess revenues to the community for support of patient services and HTC programs

For more information, please call 800-996-2575



Exploring the science behind gene therapy research

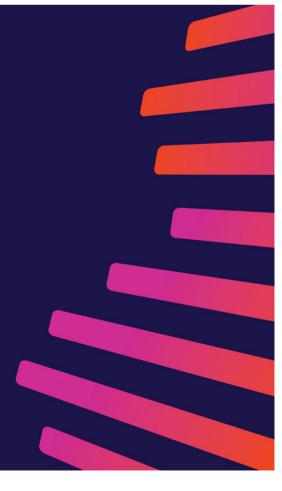
Gene therapy research has the potential to bring an entirely new option to people with specific genetic conditions. Many gene therapies are in clinical trials to evaluate the possible risks and benefits for a range of conditions, including hemophilia. HemDifferently is here with gene therapy education, providing accurate information on the basics and beyond.

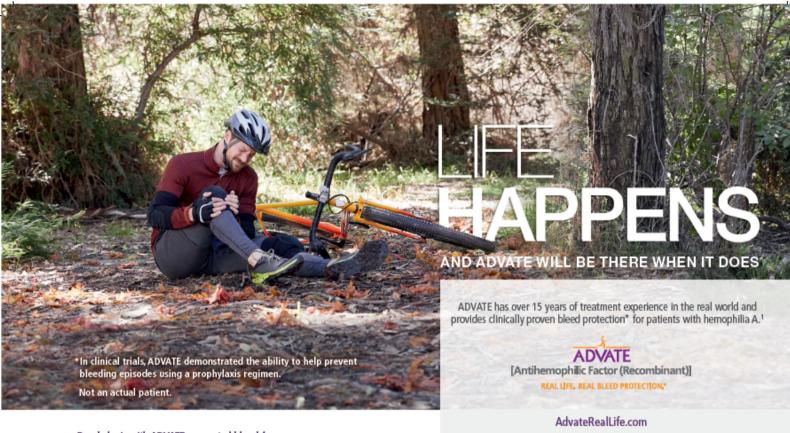
What questions do you have? Get them answered. Explore gene the rapy at ${\bf HemDifferently.com}$

No gene therapies for hemophilia have been approved for use or determined to be safe or effective.

BIOMARIN

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Prophylaxis with ADVATE prevented bleeds1

The ability of ADVATE to treat or prevent bleeds was evaluated in a clinical study using a standard prophylaxis, pharmacokinetic driven prophylaxis, and on-demand treatment. 53 previously treated patients (PTPs) with severe to moderately severe hemophilia A were analyzed. For the first 6 months of the study, patients received on-demand treatment. For the following 12 months of the study, patients received either standard prophylaxis every 48 hours or a pharmacokinetic-driven prophylaxis every 72 hours. The primary goal of the study was to compare annual bleeding rates between those receiving prophylaxis treatment and those receiving treatment on-demand. The number of bleeds per year for the 2 prophylaxis regimens were comparable.

- Those patients experienced a median of 1 overall bleed per year on either prophylaxis treatment vs 44 overall bleeds per year with on-demand treatment.[†] This represented a 98% reduction in overall bleeds per year.
- . Zero bleeds were reported in 42% of patients (22 out of 53 patients) during 12 months on prophylaxis

[†]Median is the middle number in a group of numbers arranged from lowest to highest.

ADVATE Important Information What is ADVATE?

- ADVATE is a medicine used to replace clotting factor (factor VIII or antihemophilic factor) that is missing in people with hemophilia A (also called "classic" hemophilia).
- ADVATE is used to prevent and control bleeding in adults and children (0-16 years) with hemophilia A. Your healthcare provider (HCP) may give you ADVATE when you have surgery.
- ADVATE can reduce the number of bleeding episodes in adults and children (0-16 years) when used regularly (prophylaxis).

ADVATE is not used to treat von Willebrand disease.

DETAILED IMPORTANT RISK INFORMATION Who should not use ADVATE?

Do not use ADVATE if you:

- · Are allergic to mice or hamsters.
- Are allergic to any ingredients in ADVATE.

Tell your HCP if you are pregnant or breastfeeding because ADVATE may not be right for you.

What should I tell my HCP before using ADVATE?

Tell your HCP if you:

- · Have or have had any medical problems.
- Take any medicines, including prescription and non-prescription medicines, such as over-the-counter medicines, supplements or herbal remedies.
- · Have any allergies, including allergies to mice or hamsters.
- Are breastfeeding. It is not known if ADVATE passes into your milk and if it can harm your baby.

Reference: 1. ADVATE Prescribing information.

What should I tell my HCP before using ADVATE? (continued)

- Are or become pregnant. It is not known if ADVATE may harm your unborn baby.
- Have been told that you have inhibitors to factor VIII (because ADVATE may not work for you).

What important information do I need to know about ADVATE?

- You can have an allergic reaction to ADVATE. Call your HCP right away and stop treatment if you get a rash or hives, itching, tightness of the throat, chest pain or tightness, difficulty breathing, lightheadedness, dizziness, nausea or fainting.
- Do not attempt to infuse yourself with ADVATE unless you have been taught by your HCP or hemophilia center.

What else should I know about ADVATE and Hemophilia A?

 Your body may form inhibitors to factor VIII. An inhibitor is part of the body's normal defense system. If you form inhibitors, it may stop ADVATE from working properly. Talk with your HCP to make sure you are carefully monitored with blood tests for the development of inhibitors to factor VIII.

What are possible side effects of ADVATE?

 Side effects that have been reported with ADVATE include: cough, headache, joint swelling/aching, sore throat, fever, itching, unusual taste, dizziness, hematoma, abdominal pain, hot flashes, swelling of legs, diarrhea, chills, runny nose/ congestion, nausea/vomiting, sweating, and rash. Tell your HCP about any side effects that bother you or do not go away or if your bleeding does not stop after taking ADVATE.

You are encouraged to report negative side effects of prescription drugs to the FDA. Visit www.fda.gov/medwatch, or call 1-800-FDA-1088.

Please see Important Facts about ADVATE on the following page and discuss with your HCP.

For Full Prescribing Information, visit www.ADVATE.com.

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Important facts about

ADVATE [Antihemophilic Factor (Recombinant)]

This leaflet summarizes important information about ADVATE. Please read it carefully before using this medicine. This information does not take the place of talking with your healthcare provider, and it does not include all of the important information about ADVATE. If you have any questions after reading this, ask your healthcare provider.

What is the most important information I need to know about ADVATE?

Do not attempt to do an infusion to yourself unless you have been taught how by your healthcare provider or hemophilia center.

You must carefully follow your healthcare provider's instructions regarding the dose and schedule for infusing ADVATE so that your treatment will work best for you.

What is ADVATE?

ADVATE is a medicine used to replace clotting factor (factor VIII or antihemophilic factor) that is missing in people with hemophilia A (also called *classic" hemophilia). The product does not contain plasma or albumin. Hemophilia A is an inherited bleeding disorder that prevents blood from clotting normally.

ADVATE is used to prevent and control bleeding in adults and children (0-16 years) with hemophilia A.

Your healthcare provider may give you ADVATE when you have surgery. ADVATE can reduce the number of bleeding episodes in adults and children (0-16 years) when used regularly (prophylaxis).

ADVATE is not used to treat von Willebrand disease.

Who should not use ADVATE?

You should not use ADVATE if you:

- · Are allergic to mice or hamsters.
- Are allergic to any ingredients in ADVATE.

Tell your healthcare provider if you are pregnant or breastfeeding because ADVATE may not be right for you.

How should I use ADVATE?

ADVATE is given directly into the bloodstream.

You may infuse ADVATE at a hemophilia treatment center, at your healthcare provider's office or in your home. You should be trained on how to do infusions by your healthcare provider or hemophilia treatment center. Many people with hemophilia A learn to infuse their ADVATE by themselves or with the help of a family member.

Your healthcare provider will tell you how much ADVATE to use based on your weight, the severity of your hemophilia A, and where you are bleeding.

You may have to have blood tests done after getting ADVATE to be sure that your blood level of factor VIII is high enough to clot your blood.

Call your healthcare provider right away if your bleeding does not stop after taking ADVATE.

What should I tell my healthcare provider before I use ADVATE?

You should tell your healthcare provider if you:

- · Have or have had any medical problems.
- Take any medicines, including prescription and non-prescription medicines, such as over-the-counter medicines, supplements or herbal remedies.
- · Have any allergies, including allergies to mice or hamsters.
- Are breastfeeding. It is not known if ADVATE passes into your milk and if it can harm your baby.
- Are pregnant or planning to become pregnant. It is not known if ADVATE may harm your unborn baby.
- Have been told that you have inhibitors to factor VIII (because ADVATE may not work for you).

What are the possible side effects of ADVATE?

You can have an allergic reaction to ADVATE.

Call your healthcare provider right away and stop treatment if you get a rash or hives, itching, tightness of the throat, chest pain or tightness, difficulty breathing, lightheadedness, dizziness, nausea or fainting.

Side effects that have been reported with ADVATE include:

cough headache joint swelling/aching sore throat fever itching unusual taste dizziness hematoma abdominal pain hot flashes swelling of legs diarrhea chills runny nose/congestion nausea/vomiting sweating rash

Tell your healthcare provider about any side effects that bother you or do not go away

These are not all the possible side effects with ADVATE. You can ask your healthcare provider for information that is written for healthcare professionals.

What else should I know about ADVATE and Hemophilia A?

Your body may form inhibitors to factor VIII. An inhibitor is part of the body's normal defense system. If you form inhibitors, it may stop ADVATE from working properly. Consult with your healthcare provider to make sure you are carefully monitored with blood tests for the development of inhibitors to factor VIII.

Medicines are sometimes prescribed for purposes other than those listed here. Do not use ADVATE for a condition for which it is not prescribed. Do not share ADVATE with other people, even if they have the same symptoms that you have.

The risk information provided here is not comprehensive. To learn more, talk with your health care provider or pharmacist about ADVATE. The FDA-approved product labeling can be found at www.ADVATE.com or 1-877-825-3327.

You are encouraged to report negative side effects of prescription drugs to the FDA. Visit www.fda.gov/medwatch, or call 1-800-FDA-1088.

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Patented: see https://www.takeda.com/en-us/patents/

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US-ADV-0030v1.0 02/20





Covid-19 may keep us home, but we can still have some fun!

Join us for a special one-hour VIRTUAL celebration of the NOHF's 15th annual Black and Blue Ball.

Grab your device of choice, don your best black and blue attire, and join us for a good cause.

Tickets include: Link to LIVE zoom event, *"Gala Box" shipped directly to your home. Gala Box contains: gift card for wine or beverage of your choice, appetizers, and surprise extras.

*Registered couples receive one Gala Box

Evening entertainment includes: an online silent auction, lively interactive "Heads or Tails" game, special guest speaker, and our Fund-a-Need live auction hosted by Bob Hale!

It's been a challenging year, your support is greatly appreciated.

Ticket Prices: Individual - \$75 Couple - \$125 *Table of 8 - \$750

*Table host will receive an upgraded Gala Box name listed in printed in evening program.

To purchase tickets visit our website: www.nohf.org Questions? Contact Dawn Evans: dawn@nohf.org 216.834.0051

OBDC Advocacy Days



In response to the COVID-19 pandemic, the **Ohio Bleeding Disorders Council** moved its statehouse day to a virtual format and teamed up with the various local chapters to host regional events. Through our six virtual events we have been able to connect legislators with clinicians, advocates, and families to discuss important legislation as well as the impact of COVID-19 on patients with a bleeding disorder. In addition to sharing personal stories and discussing the work of NOHF and other local chapters, we discussed the following priority bills with lawmakers during each event—

- House Bill 469: This bill, sponsored by State Representatives Susan Manchester (R-Waynesfield) and Thomas West (D-Canton) would prohibit health plans and PBM's from implementing copay accumulator adjustor programs. Under these programs, copay assistance cards provided by drug manufacturers or other organizations are not counted towards a patient's deductible. As a result, patients must still pay out of pocket after exhausting the copay assistance. For patients with a bleeding disorder, these programs represent an unfair practice that creates a significant cost burden.
- House Bill 412: Sponsored by State Representatives Randi

Clites (D-Ravenna) and Tim Ginter (R-Salem), HB 412 would establish the Ohio Rare Disease Advisory Council. This council would be comprised of experts, clinicians, and patients who would provide ongoing recommendations to lawmakers regarding policies to assist individuals with a rare disease. Additionally, the bill would require the Ohio Department of Health to publish data on the incidence of rare disease in Ohio. This information will further assist policymakers and advocates.

• House Bill 418: Sponsored by State Representatives Randi Clites (D-Ravenna) and Sara Carruthers (D-Hamilton), this bill would prohibit health plans from changing patient copays or moving drugs off of a formulary during a plan year. These types of 'non-medical switches' create barriers for patients who require prescription drugs to manage a bleeding disorder or other medical condition. Like HB 469, HB 418 is meant to protect patients from actions that dramatically impact their lives through increased costs or changes to drug access.

We are greatly appreciative of the support of Ohio's various HTC's and local chapters in this effort and we look forward to seeing everyone again next year for statehouse day!

DEDICATION and **PERSONAL SUPPORT**

Your Pfizer Patient Affairs Liaison is a professional dedicated to serving you and the hemophilia community by connecting patients and caregivers with Pfizer Hemophilia tools and resources. We are committed to continuing Pfizer's more than 20 years of listening to the hemophilia community and working to meet its needs.



Michael Sager Western PA, Western NY, North & Central OH 330-413-9755 michael.sager@pfizer.com

"I'm proud to be a dedicated advocate for the rare disease community."





MY WORK IS GUIDED BY:

Compassion—Listening to your needs and addressing questions and concerns you may have **Commitment**—Educating you about Pfizer's tools and resources, designed to help you access treatment and more **Connection**—Connecting you with hemophilia advocacy groups and programs

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GEARS FOR GOOD – NORTHERN OHIO RIDE

Covid-19 couldn't stop our riders this year! The beautiful day brought our riders out in support of Team Resilience and the NOHF's annual Gears for Good, Northern Ohio ride. After grabbing water and snacks, our more "adventurous" riders started their day at 7:00a for a 50-mile ride through the beautiful Ohio & Erie Canalway towpath. The rest of Team Resilience finished their 5-40 mile rides with smiles on their faces. The event ended with everyone receiving a boxed lunch celebrating their accomplishment.



Our Gears for Good ride will take place in June 2021 and we hope you will join us for this family-friendly event raising money for the NOHF's emergency assistance fund helping those in our community. Funds raised nationally go directly to the Hemophilia Federation of America's Helping Hands program, which provides urgent funding for basic living expenses such as housing, transportation and utility bills for families living with bleeding disorders.

Special thanks to our 2020 sponsors: Akron Children's Hospital, Bayer, Sanofi Genzyme, CSL Behring, Novo Nordisk, and Cigna.













A planned gift allows you to make charitable gifts, continue to meet your current income needs, and take advantage of current tax incentives.

Have you considered your legacy?

If you have already included NOHF in your plans, please let us know so that you can be included as a charter member of the 1954 Society.

YOUR PLANNED GIVING OPTIONS

GIFT FROM YOUR WILL

Through a provision in your written and executive will, you can make a gift in the form of case, securities, real estate or personal property. There are many type of bequests. Choose the one that best fits your needs and intentions.

BENEFICIARY GIFTS

Naming NOHF as a beneficiary of a qualified retirement plan asset such as a 401(k), 403(b), IRA, or other donor-advised funds, will accomplish a charitable goal while realizing significant tax savings.

GIFTS THAT PAY YOU

You can turn underperforming assets (stock, case in a savings account, CD, Savings bond, etc.) into a gift NOHF that provides income to you. Your Charitable Gift Annuity will give you quarterly, fixed payments for life and tax benefits too.

CHARITABLE IRA ROLLOVER

If you are 70 1/2 or older, you can make a gift directly from your IRA to NOHF. While there is no charitable deduction for a rollover gift, you do avoid the income tax on the donated portion of your required miminum distribution.

CONTACT US

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6505 Market Street Boardman, OH 44512 (330) 746-9522

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