

Factor Nine News

The Coalition for Hemophilia B Winter 2012



Topics in Hemophilia

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Steven's Story Living with Hemophilia B

by Matt Sclafani

Steven Sclafani is my son; he's also a boy with severe Factor IX Deficiency, Hemophilia B, also known as Christmas Disease. Hemophilia is a very rare genetic bleeding disorder in which affected individuals have insufficient levels of a blood protein called factor IX, which is a clotting factor. Severe hemophiliacs can easily bruise and are susceptible to prolonged bleeding episodes. Bleeds can occur into places such as a muscle, joint or organ. Bleeds can be painful and debilitating as well as life threatening.

Steven's Hemophilia diagnosis was made five days after birth, his first muscle bleed at twenty-two days old. Our family was completely devastated, what kind of existence will our child have, how can he live a normal life with hemophilia? My wife and I knew very little about this rare disease and what we thought we knew wasn't very encouraging.

After Steven's diagnosis, a North Shore Hospital Hematologist directed us to the Hemophilia Treatment Center (HTC) at



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Meet Inspiration Biopharmaceuticals

We understand hemophilia

At Inspiration, we have made it our personal mission to make a difference in future treatment options available for all people with hemophilia. The founding families of our company have been intimately impacted by hemophilia, as both families have sons who have hemophilia.

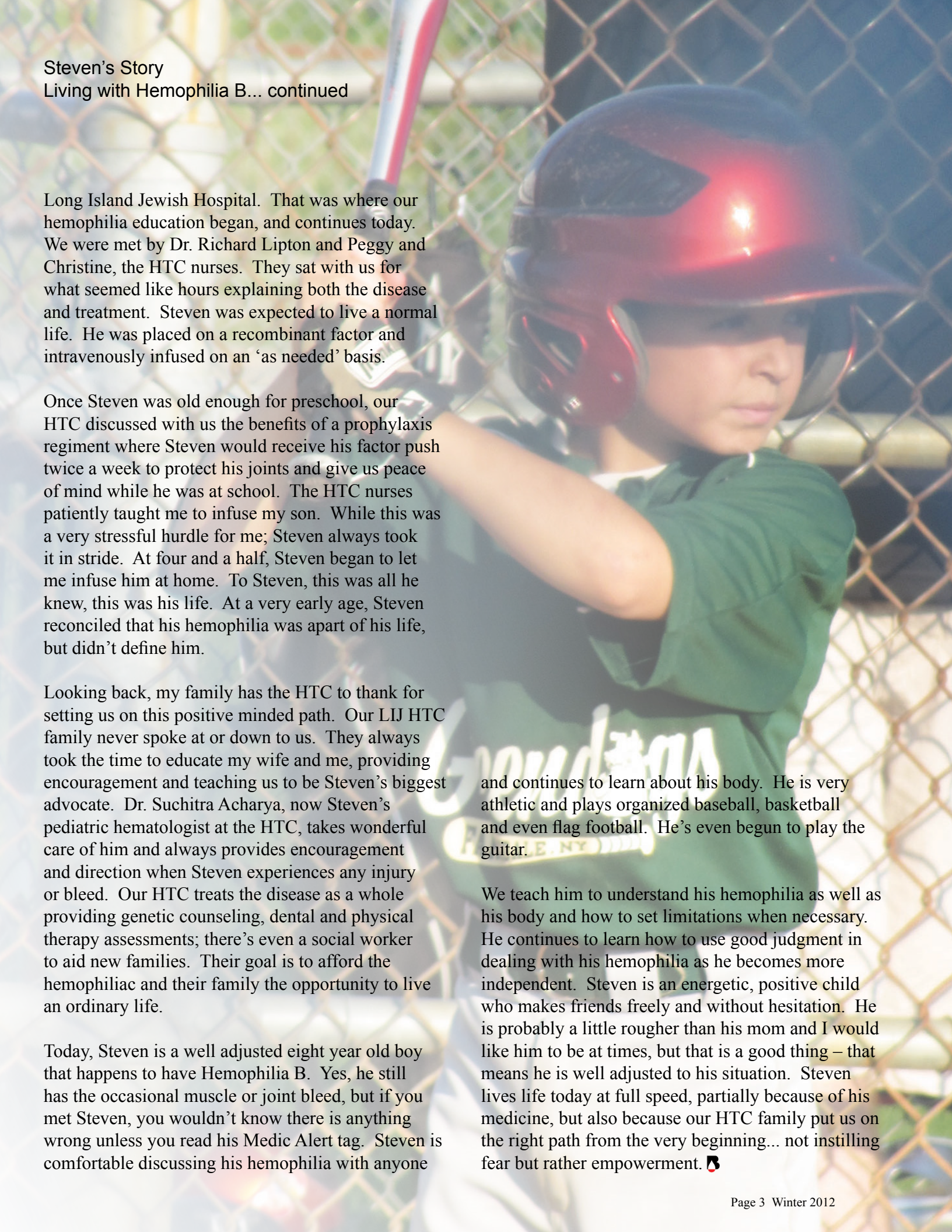
We are committed to:

- Broadening access to care worldwide
- Improving treatment options for people with inhibitor complications

For more information, visit our website at www.inspirationbio.com

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Steven's Story

Living with Hemophilia B... continued

Long Island Jewish Hospital. That was where our hemophilia education began, and continues today. We were met by Dr. Richard Lipton and Peggy and Christine, the HTC nurses. They sat with us for what seemed like hours explaining both the disease and treatment. Steven was expected to live a normal life. He was placed on a recombinant factor and intravenously infused on an 'as needed' basis.

Once Steven was old enough for preschool, our HTC discussed with us the benefits of a prophylaxis regiment where Steven would receive his factor push twice a week to protect his joints and give us peace of mind while he was at school. The HTC nurses patiently taught me to infuse my son. While this was a very stressful hurdle for me; Steven always took it in stride. At four and a half, Steven began to let me infuse him at home. To Steven, this was all he knew, this was his life. At a very early age, Steven reconciled that his hemophilia was apart of his life, but didn't define him.

Looking back, my family has the HTC to thank for setting us on this positive minded path. Our LIJ HTC family never spoke at or down to us. They always took the time to educate my wife and me, providing encouragement and teaching us to be Steven's biggest advocate. Dr. Suchitra Acharya, now Steven's pediatric hematologist at the HTC, takes wonderful care of him and always provides encouragement and direction when Steven experiences any injury or bleed. Our HTC treats the disease as a whole providing genetic counseling, dental and physical therapy assessments; there's even a social worker to aid new families. Their goal is to afford the hemophiliac and their family the opportunity to live an ordinary life.

Today, Steven is a well adjusted eight year old boy that happens to have Hemophilia B. Yes, he still has the occasional muscle or joint bleed, but if you met Steven, you wouldn't know there is anything wrong unless you read his Medic Alert tag. Steven is comfortable discussing his hemophilia with anyone

and continues to learn about his body. He is very athletic and plays organized baseball, basketball and even flag football. He's even begun to play the guitar.

We teach him to understand his hemophilia as well as his body and how to set limitations when necessary. He continues to learn how to use good judgment in dealing with his hemophilia as he becomes more independent. Steven is an energetic, positive child who makes friends freely and without hesitation. He is probably a little rougher than his mom and I would like him to be at times, but that is a good thing – that means he is well adjusted to his situation. Steven lives life today at full speed, partially because of his medicine, but also because our HTC family put us on the right path from the very beginning... not instilling fear but rather empowerment. 🐾

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Available in the following potencies and color coded assay ranges

Potency	Diluent Size
500 IU FIX Range	10 mL
1000 IU FIX Range	10 mL
1500 IU FIX Range	10 mL



For more information: Grifols Inc.
Customer Service: 888 325 8579 Fax: 323 441 7968

A903-0911

Mike O'Connor - Swimmer


When Mike O'Connor was born in September of 1990, his parents weren't thinking about him being an athlete. They were more concerned with protecting their boy, who has severe Hemophilia B. And yet, they found the best way to protect him was to help him become an athlete.

Michael's swim career began on a local YMCA team when he was ten years old, and culminated this weekend in his Amherst College Varsity Swim team's second place finish at the prestigious and very competitive NESCAC Championships. (See article below).

Swimming has given Michael a relatively safe way to become strong. As a consequence, joint

bleeding has not been an issue for years, although he has had some nasty muscle bleeds.

With his graduation from college, Michael's competitive career comes to a close, but swimming will always be a part of his life, whether through coaching young swimmers, or speaking to parents and children about his career as an athlete with Hemophilia.

Mike hopes to one day start a summer swim camp to get children with Hemophilia started on their own paths to becoming athletes. This fall he is off to the University of Texas, Jackson School of Geology, on a Fellowship to study Hydrogeology. 



Left to Right:
Tim White '12, Ryan Lichtenfels '12, Ben McBratney '12,
Bart Federak '12, Mike O'Connor '12.

Contact: Justin Long WILLIAMSTOWN, Mass. – The Amherst College men's swimming and diving team finished the NESCAC Championships in second place, setting a new program record with an impressive 1,959 points during the three-day meet. Williams took the team title with 2,011 points, marking the smallest margin of victory in the championship's history. The Lord Jeffs earned 344.5 more points than they did a year ago, but the Ephs once again capitalized on their depth to pick up the victory in their own pool. Amherst began the day in a 52.5-point hole, but Ryan Lichtenfels '12 helped the Jeffs regain the lead by winning the 1,650-yard freestyle for the fourth time in as many years with an NCAA 'B' time of 15:43.96. Fellow senior co-captain Tim White was close behind in second (15:47.20, NCAA 'B'), as four Amherst swimmers placed in the top six with 'B' cuts. The Jeffs padded

their lead by placing three in the top eight of the 200-yard backstroke, with Conor Deveney '15 leading the way by finishing second and just missing out on a school record and NCAA 'A' cut (1:49.57). Amherst bumped its lead to 75 points when Connor Sholtis '15 came in as the runner-up in the 100-yard freestyle (45.53, NCAA 'B'), while Tyler Hampton '15 finished sixth in the event (46.82).

The Ephs took the lead for good by featuring five of the eight best times in the 200-yard breaststroke, as the Jeffs trailed 1,776 to 1,761 with three events to go. Leading Amherst in the 200 breast were seniors Bart Federak (6th, 2:06.60) and Mike O'Connor (7th, 2:06.87), with Federak earning a 'B' cut. Williams put the meet out of reach with a strong showing in the 200-yard butterfly. Amherst's Tyler Bulakul '14 (1:51.47, NCAA 'B') and Michael Rochford '15

(1:52.09, NCAA 'B') placed second and third, respectively, but the Ephs increased their lead to 90 points heading into the final diving event. Amherst's young divers helped narrow the deficit by wrapping up an impressive weekend on the 3-meter board. Colin White '14 finished second with a score of 504.70 and Mark Idleman '15 took fifth (443.75), as the two Lord Jeffs matched their placement from Friday's 1-meter dive. This is the second consecutive year White finished as the runner-up in both diving events. The Jeffs closed the weekend by winning both the 'A' and 'B' heats of the 400-yard freestyle relay, but it wasn't enough to catch the Ephs. Beating out the field with an NCAA provisional time of 3:02.89 in the final heat were Ben McBratney '12, Lichtenfels, Hampton and Sholtis, marking the third win McBratney was a part of during the meet.

Patient Assistance Program

CSL Behring

Product(s): Helixate-FS, Humate-P, Monoclate-P, Mononine

Website: www.cslbehring.com

Reimbursement #: 1-888-415-2167

1. Assurance Program

Product Requirement: Yes, must be using a CSL Behring product

Income Requirement: None

How it Works: Once enrolled in the Program, you will begin earning an Award Certificate for every three consecutive months of product use. Each Certificate is worth a complimentary, one-month supply of product up to the maximum amount redeemable. You can redeem up to a year's worth of product (representing 12 Certificates or 3 years of continuous product usage) or just one month's worth of product - it all depends on your needs and the length of your insurance gap. Note: There is both a quarterly and a yearly maximum for each of their products and goes as follows:

Quarterly maximum: Helixate / Monoclate = 36,000 units.

Mononine / Humate = 18,000 units.

Yearly maximum: Helixate / Monoclate = 144,000 units.

Mononine / Humate = 72,000 units.

2. Patient Assistance Program

Product Requirement: Yes, must be using a CSL Behring product

Income Requirement: Yes.

How it Works: For those without health insurance, CSL Behring is willing to donate an unspecified amount of product to those in need. It is entirely supply-based.

Patient Assistance Program

GRIFOLS

Product(s): Alphanate, Alphanine-SD
Website: www.grifolspatientcare.com
Reimbursement #: 1-888-474-3657

1. Grifols Assurance Program (GAP)

Product Requirement: Yes, must be using a Grifols product

Income Requirement: None

How it Works: Helps protect a patient with insurance against gaps & breaks in their insurance. After three months of continuous use of a Grifols product, one may redeem up to 100,000 IUs of factor at no cost each year. Must be without insurance at time of redemption.

2. Grifols Patient Assistance

Product Restrictions: None

Income Restrictions: 250% of federal poverty limit

How it Works: A maximum of 100,000 units of factor may be redeemed on this program. It is for those who are without insurance. Grifols will work with you to attain adequate health insurance during this time.

Patient Assistance Program



Product(s): NovoSeven, NovoSeven-RT
Website: <http://www.novosevenrt.com>
click on tab “Resources” in left vertical column
Reimbursement #: 1-877-668-6777

1. SevenAssist Program

Product requirement: Must be using NovoSeven
Income requirement: Yes
How it works: A program for those who have lost their insurance coverage. You will need to download and complete 2 forms to enroll. One is for you to fill out and the other is for your doctor.

2. SevenStart Program

Product requirement: Must have an inhibitor
Income requirement: None
How it works: A support program to assist those with inhibitors, a trial program to see if NovoSeven RT is the right treatment for you. You will need to download and complete 2 forms to enroll in SevenSTART. One is for you to fill out and the other is for your doctor.

Novo Nordisk will also assist in your insurance woes and are actively participating with PSI to issue medical grants and scholarships.

Patient Assistance Program



Product(s): BeneFIX, Xyntha
Website: www.hemophiliavillage.com/programs_insurance.asp
HELPLine 1-888-327-7787

1. RSVP (Reimbursement Solutions, Verification, and Payment):

Product requirement: You do not need to be using a Pfizer factor product to qualify for assistance, though if taking advantage of the program, you will receive Pfizer product.

Income requirement: Yes, please call number for details.

How it works: The program provides assistance for those both insured and uninsured. For the insured, RSVP offers reimbursement support services, appeals process information, alternate funding assistance & hardship assistance. With reimbursement support services, patients will get help from experts with understanding their coverage. RSVP can help investigate any underpaid or denied claims, find additional, alternate or supplemental funding and if eligible, provide Pfizer medicines without cost. For those who are uninsured, RSVP may be able to provide eligible patients with their medication at no cost. To qualify, a patient must have no prescription coverage, meet specific income guidelines adjusted by family size and reside in the United States, Puerto Rico or US Virgin Islands. Patients must also be under the treatment of a physician licensed in the United States.



Coagulation Factor IX (Recombinant)
Room Temperature Storage

Rx only

Brief Summary

See package insert for full Prescribing Information. This product's label may have been updated. For further product information and current package insert, please visit www.Pfizer.com or call our medical communications department toll-free at 1-800-934-5556.

Please read this Patient Information carefully before using BeneFix and each time you get a refill. There may be new information. This brief summary does not take the place of talking with your doctor about your medical problems or your treatment.

What is BeneFix?

BeneFix is an injectable medicine that is used to help control and prevent bleeding in people with hemophilia B. Hemophilia B is also called congenital factor IX deficiency or Christmas disease.

BeneFix is **NOT** used to treat hemophilia A.

What should I tell my doctor before using BeneFix?

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

Tell your doctor about all of your medical conditions, including if you:

- are pregnant or planning to become pregnant. It is not known if BeneFix may harm your unborn baby.
- are breastfeeding. It is not known if BeneFix passes into the milk and if it can harm your baby.

How should I infuse BeneFix?

The initial administrations of BeneFix should be administered under proper medical supervision, where proper medical care for severe allergic reactions could be provided.

See the step-by-step instructions for infusing in the complete patient labeling.

You should always follow the specific instructions given by your doctor. If you are unsure of the procedures, please call your doctor or pharmacist before using.

Call your doctor right away if bleeding is not controlled after using BeneFix.

Your doctor will prescribe the dose that you should take.

Your doctor may need to test your blood from time to time.

BeneFix should not be administered by continuous infusion.

What if I take too much BeneFix?

Call your doctor if you take too much BeneFix.

What are the possible side effects of BeneFix?

Allergic reactions may occur with BeneFix. Call your doctor or get emergency treatment right away if you have any of the following symptoms:

- wheezing
- difficulty breathing
- chest tightness
- turning blue (look at lips and gums)
- fast heartbeat
- swelling of the face
- faintness
- rash
- hives

Your body can also make antibodies, called "inhibitors," against BeneFix, which may stop BeneFix from working properly.

Some common side effects of BeneFix are nausea, injection site reaction, injection site pain, headache, dizziness and rash.

BeneFix may increase the risk of thromboembolism (abnormal blood clots) in your body if you have risk factors for developing blood clots, including an indwelling venous catheter through which BeneFix is given by continuous infusion. There have been reports of severe blood clotting events, including life-threatening blood clots in critically ill neonates, while receiving continuous-infusion BeneFix through a central venous catheter. The safety and efficacy of BeneFix administration by continuous infusion have not been established.

These are not all the possible side effects of BeneFix.

Tell your doctor about any side effect that bothers you or that does not go away.

How should I store BeneFix?

DO NOT FREEZE BeneFix. BeneFix kit can be stored at room temperature (below 86°F) or under refrigeration. Store the diluent syringe at 36° to 86°F (2° to 30°C). Throw away any unused BeneFix and diluent after the expiration date indicated on the label.

Freezing should be avoided to prevent damage to the pre-filled diluent syringe.

Different storage conditions are described below.

**Product labeled for Room Temperature Storage
Store at 2° to 30°C (36° to 86°F).**

If you have the product kit labeled for room temperature storage, it can be stored at room temperature (below 30°C or 86°F) or in the refrigerator (2° to 8°C or 36° to 46°F).

**Product labeled for Refrigerator Storage
Continuous refrigeration**

[2° to 8°C (36° to 46°F)]

If you have the product labeled for storage in the refrigerator (2° to 8°C or 36° to 46°F) and you have not taken the kit out of the refrigerator, then the expiration date printed on the package still applies. You can store the product at room temperature (below 30°C or 86°F) for up to 6 months or until it has reached its expiration date, whichever comes first.

If you have taken the product kit labeled for storage in the refrigerator out of the refrigerator and stored it at room temperature (below 30°C or 86°F), then use the product within 6 months from the time you took the product out of the refrigerator or until it has reached its expiration date, whichever comes first. If you cannot remember when you took it out of the refrigerator, then subtract one year (12 months) from the date that is printed on the end flap of the carton package. The date you get is your new expiration date. Throw away any product that has gone over the new expiration date.

BeneFix does not contain a preservative. After reconstituting BeneFix, you can store it at room temperature for up to 3 hours. If you have not used it in 3 hours, throw it away.

Do not use BeneFix if the reconstituted solution is not clear and colorless.

What else should I know about BeneFix?

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

If you would like more information, talk to your doctor. You can ask your doctor for information about BeneFix that was written for healthcare professionals.

This brief summary is based on BeneFix® [Coagulation Factor IX (Recombinant)] Prescribing Information LAB-0464-8.0, revised November 2011.



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COMING SOON 3000 IU



More units. Single vial. Same 5-mL diluent.

14+ YEARS*
**EXPERIENCE
MATTERS**

 **BeneFix**[®]
Coagulation Factor IX (Recombinant)
Room Temperature Storage

*BeneFix was approved February 11, 1997.

What Is BeneFix?

BeneFix[®] Coagulation Factor IX (Recombinant) is an injectable medicine that is used to help control and prevent bleeding in people with hemophilia B. Hemophilia B is also called congenital factor IX deficiency or Christmas disease.

BeneFix is **NOT** used to treat hemophilia A.

Important Safety Information for BeneFix

- BeneFix is contraindicated in patients who have manifested life-threatening, immediate hypersensitivity reactions, including anaphylaxis, to the product or its components, including hamster protein.
- Call your health care provider right away if your bleeding is not controlled after using BeneFix.
- Allergic reactions may occur with BeneFix. Call your health care provider or get emergency treatment right away if you have any of the following symptoms: wheezing, difficulty breathing, chest tightness, your lips and gums turning blue, fast heartbeat, facial swelling, faintness, rash or hives.
- Your body can make antibodies, called "inhibitors," which may interfere with the effectiveness of BeneFix.
- If you have risk factors for developing blood clots, such as a venous catheter through which BeneFix is given by continuous infusion, BeneFix may increase the risk of abnormal blood clots. The safety and efficacy of BeneFix administration by continuous infusion have not been established.
- Some common side effects of BeneFix are nausea, injection site reaction, injection site pain, headache, dizziness and rash.

Please see brief summary of full Prescribing Information for BeneFix on reverse side.

Wyeth[®]

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Printed in USA/November 2011



For Hemophilia B patients who
need an alternative to their
recombinant factor IX therapy



Mononine® Is The One to Choose

- Established Recovery Rates
- Proven Protection
- Demonstrated Safety
- A WHO Standard for Purity and Potency

Important Safety Information

Mononine® is contraindicated in patients with known hypersensitivity to mouse protein.

The following adverse reactions may be observed after administration: headache, fever, chills, flushing, nausea, vomiting, tingling, lethargy, hives, stinging or burning at the infusion site, or other manifestations of allergic reactions, including anaphylaxis.

Mononine® is derived from human plasma. As with all plasma-derived products, the risk of transmission of infectious agents, including viruses and, theoretically, the Creutzfeldt-Jakob disease (CJD) agent, cannot be completely eliminated.

Please see brief summary of prescribing information on adjacent page.

Mononine®
MONOCLONAL ANTIBODY PURIFIED
Coagulation Factor IX (Human)



FACTOR FORWARD

We are Biogen Idec Hemophilia,

and we're exploring ways to make clotting factors last longer.

Backed by more than 30 years of biotechnology leadership and meaningful therapies with global impact, Biogen Idec Hemophilia is blazing a new trail of scientific discovery toward long-lasting clotting factors. We're using forward-thinking technology to advance hemophilia research.

Biogen Idec Hemophilia is:

- A world-class team of scientists, clinicians, thought leaders, and visionaries with extensive experience in hemophilia
- Motivated by 1 goal: to make progress for the hemophilia community
- Driven to deliver one of the most anticipated therapeutic milestones in hemophilia—long-lasting clotting factors
- Passionate about R&D, which is led by Glenn Pierce, MD, PhD. Dr Pierce has dedicated more than 20 years to biotechnology R&D and served as President of the National Hemophilia Foundation
- Creating therapies, programs, and resources with the potential to change lives

Eric Lowe
with his father



changing possibilities in hemophilia®

Dealing with a
bleeding disorder?
We've got your back.

Eric Lowe had the support of his family when he went through double knee replacement surgery.

Now, that family is a lot bigger. Eric connects with others and shares his insights as a member of The Changing Possibilities Coalition—a unique hemophilia community created by Novo Nordisk.

Help change what's possible.
Join The Coalition today at
JointheCPcoalition.com.



Changing Possibilities in Hemophilia®

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U.S. Hemophilia Patient Programs

Patient Assistance: RSVP (Reimbursement Solutions, Verification, and Payment HELPLine) is a reimbursement support services and patient assistance program designed to help uninsured and under-insured patients gain access to Pfizer Hemophilia products. To learn more, patients can call 1-888-327-RSVP (7787) or visit www.hemophiliavillage.com/RSVP

Factor Savings Card Program

- Provides financial assistance to cover part of the cost of treatment for eligible patients who have high out-of-pocket costs but do not meet the eligibility requirements for hardship assistance
- Savings/rebate card with a value of up to \$500 over 12 months to supplement out-of-pocket costs
 - Patients who are prescribed Pfizer Hemophilia products may qualify for this card
 - Patients must also be covered by commercial insurance - no members of government-funded programs are eligible
 - Benefit covers co-pay, co-insurance and deductible costs
 - Number of prescriptions this can be applied to depends on the patient's benefit design
- Patients can obtain the card from their doctor or by calling Pfizer directly at 1-855-PFZ-HEMO
- For more information on the card, patients can visit <https://www.hemophiliavillage.com/resources-support/patient-support/savings-card/default.aspx>

Scholarship Program "The Soozie Courter Sharing a Brighter Tomorrow Hemophilia Scholarship Program: provides tuition assistance from Pfizer Hemophilia. To be eligible for the scholarship, students must have either hemophilia A or hemophilia B and be a high school senior, have a high school diploma or general education development (GED) credential, or be currently enrolled in an accredited junior college, college (as an undergraduate or graduate student), or vocational school. Applicants do not need to use Pfizer Hemophilia products to be considered.

HemophiliaVillage.com: a website resource that has information about treatment options, patients' stories about living with hemophilia, tips for staying healthy and active, and more. By registering for Hemophilia Village, patients can receive a quarterly newsletter *The Villager*, which provides current, topical information about the issues and stories that affect people with hemophilia. Besides this newsletter, patients can receive regular updates on Pfizer Hemophilia products and financial and educational resources available through Pfizer Hemophilia. To register for Hemophilia Village, patients can visit <https://www.hemophiliavillage.com/your-community/about-hemophilia-village/default.aspx>

Our Hemophilia Community: a Facebook page through which Pfizer Hemophilia keeps patients current on resources available to them, communicates general health tips, and up to date information on Pfizer Hemophilia products. Like us on Facebook at <https://www.facebook.com/#!/ourhemophiliacommunity>

Next Steps for Living: a website resource developed by NHF and in collaboration with Pfizer Hemophilia. This website provides educational support for every stage of a hemophilia patient's life, from birth to adulthood. Patients can visit this website resource at <http://www.stepsforliving.hemophilia.org>

Save the Date!



The Coalition for Hemophilia B Family Meetings coming to Texas and Ohio!

Details will be available soon!

We look forward to seeing you!



Reminder

Please remember to mail in your surveys! Your input is very valuable to us!

COMING SOON!
The Coalition for Hemophilia B
will soon be on FACEBOOK!



The Factor Nine Group moderated by Jill Lathrop is located on Facebook - search Hemophilia B Group

For back issues of **Factor Nine Newsletter** or for more information on research, please call or write to:
Kim Phelan; 825 Third Avenue, Suite 226; New York, New York 10022; Telephone (212) 520-8272
Telefax (212)520-8501; E-mail: hemob@ix.netcom.com Website: www.coalitionforhemophiliab.org