Spring/Summer 2019

KHF Hemosphere

21st Annual Family Day at the Louisville Zoo

The annual Family Day at the Louisville Zoo was well attended as it is every year. One hundred and seventy adults and children from all across the state enjoyed a warm and sunny Saturday with us on May 18th. After touring the ever-expanding number of animal exhibits nestled in a lovely and slightly hilly terrain, our guests gathered at the Hillside Gazebo to enjoy a satisfying picnic lunch and chat with exhibitors and friends. In the meantime, we entertained the little ones with a variety of popular carnival games that offered many fun and cool prizes.

Of course, the "duck pond" is most popular, because each duck picked up resulted in a prize and a happy child. The small animal presentation by the zoo handlers also attracted a crowd of smiling and adoring children, who delighted in touching and petting a couple of little exotic animals.

The culmination of the event was the drawing of door prizes which elicited oohs and aahs from the winners. We thank our volunteers and exhibitors who helped make this wonderful day possible. Exhibitors were Bayer HealthCare, CSL Behring, CVS Caremark, First Choice Home Infusion, Genentech, Grifols, InTouch Pharmacy, Matrix Health, Novo Nordisk, Octapharma, Pfizer, Specialty Care Rx, and Takeda.

Gene Therapy with Transplanted Cells Shows Promise in Hemophilia A Mice

Researchers from the University of California (UC) Davis have been investigating whether two genetically engineered cell types could be customized for long-term therapeutic benefit in individuals with hemophilia A. To this end, Aijun Wang, PhD co-director of the surgical bioengineering laboratory at UC Davis and his team treated hemophilia A mice subjects with this unique gene therapy.

Through an intramuscular injection, the mice subjects received human-sourced endothelial colony-forming cells (ECFCs) and placenta-derived mesenchymal stromal cells (PMSCs). ECFCs can multiply and take on the characteristics of endothelial cells, which typically line the walls of blood vessels in organs such as the heart, liver and intestines and are known to produce FVIII. PMSCs are, according to the authors, "well established as a stem cell therapy product for a wide variety of diseases and conditions."

The ECFCs and PMSCs were specifically engineered with a version of the FVIII gene, along with repurposed retroviruses called lentiviral vectors. These vectors act as vehicles, carrying the customized genetic material to elicit the production of FVIII. One advantage in using lentiviruses is that most patients do not generate antibodies to this type of vector, avoiding an immune response that would otherwise render the treatment ineffective. Another benefit of using lentiviral vectors is their large size, enabling them to deliver greater concentrations of the FVIII gene to produce a more optimal therapeutic effect.

While a drawback to using transplanted cells can be a very brief period of viability – in this case only short-term FVIII production – the outcomes here were promising. The results showed that the co-transplant of PMSCs and ECFCs resulted in the most optimal and enduring transplants, yielding a functional FVIII inside the mice's bodies over at least six months. Investigators also observed that the more successful transplants were performed a few days after birth, versus those performed in adult mice. Following therapy, neonatal hemophilia A mouse models with less than 1% of FVIII saw a two-thirds reduction in blood loss in response to a cut.

"This work demonstrated that co-transplantation of ECFCs with PMSCs at the neonatal age is a potential strategy to achieve stable, long-term engraftment, and thus holds great promise for cell-based treatment of hemophilia A," concluded the authors.

The study, "Potential Long-Term Treatment of Hemophilia A by Neonatal Co-Transplantation of Cord Blood-Derived Endothelial Colony-Forming Cells and Placental Mesenchymal Stromal Cells," was published in the journal Stem Cell Research & Therapy.

Source: Hemophilia News Today, January 28, 2019

From Food to Factor:

The Road to New Therapies in Hemophilia

Wendy Owens

This is the remainder of an article started in the last newsletter.

TO COVER OR NOT TO COVER?

FDA approval of a new medication or therapy does not mean that it will be covered by all health plans. Health insurance companies and PBMs are the gatekeepers for a drug's availability to patients. Let's take PBMs as an example. PBMs are massive, multibillion-dollar companies that manage drug costs for their clients. PBMs' drug formularies include FDA-approved medications and therapies available only by prescription. The drugs on a PBM's formulary, or list, are covered by insurance plans that offer prescription drug benefits. To make this list, an FDA-approved medication or therapy must be evaluated for clinical appropriateness first—how safe it is and how well it works—and cost second.

This evaluation process uses a combination of data on a drug's effectiveness and treatment value to reach a decision about whether to put that drug on a formulary. "It's a clinical-first process that relies on the recommendations of an independent group of physicians and pharmacists before cost considerations," Luddy says. "All medications are subject to this process."

Before the drug reaches a formulary, a panel of independent experts called a pharmacy and therapeutics (P&T) committee looks at the clinical appropriateness of a new drug. The P&T committee reviews drugs for all conditions, including hemophilia. P&T committees can include nurses, doctors, pharmacists, and other clinical experts. The P&T committee for the PBM CVS Caremark, for example, is composed of 22 independent healthcare providers, including practicing pharmacists and physicians.[3] The P&T committee reviews safety data, clinical trial results, doctor recommendations, and FDA-approved prescribing information developed by the pharmaceutical company making the drug.

The P&T committee is not interested in the cost of a new drug. Instead, the committee's goal is to determine whether a new drug is safe for use, and whether it performs better than other drugs currently available to treat the same condition. Each decision by a P&T committee for each drug is either an "include," "exclude," or "optional for inclusion" in a formulary. According to Express Scripts, 15% of the drugs on its formulary receive an "include" designation from the P&T committee, with the other 85% being "optional for inclusion." Fewer than 1% of drugs available in the US receive an "exclude" designation from the Express Scripts P&T committee.[4]

SHOW ME THE MONEY

If a P&T committee advises an insurance company or PBM to add a drug to its formulary as "include" or "optional for inclusion," then it's the job of the insurer or PBM and the pharmaceutical company providing the drug to arrive at a price. According to various studies, the average cost to manufacturers for developing a new drug is $648 million to $2.7 billion, a cost that often depends on what disease or disorder the drug will treat.[5] So how do insurers and pharmaceutical companies, who would like to recover the research and development cost of the drug as well as earn a profit, agree on what a drug should cost insurers?

The answer is simple. Pharmaceutical companies price a drug for as much as the market is willing to pay for it. These companies do their homework and look at the price of drugs similar to theirs. They talk to doctors like your hematologist to see if they would prescribe the company's drug, and they look at how long a person would need to take the drug. A drug's uniqueness is considered in relation to other drugs on the market to treat the same condition. Drugs with added benefits that may have a big impact on a patient population and lower healthcare cost overall can be priced higher.

For example, if a new drug has the potential (or has proven through clinical trials) to change the current practice of medicine used to treat the conditions the drug targets, it could be more expensive, like the hepatitis C treatment Sovaldi,® which is a cure. A drug also is special if it can prevent the need for certain medical treatments or the necessity for surgeries or other procedures. Drugs that can cut down on expensive surgeries, hospital trips, and doctor visits for patients are often priced higher because of the savings

cont. on page 6

Event News

Call to Action

2019 Kentucky Unite Walk

We are so excited to inform you that the 6th Annual Kentucky Unite for Bleeding Disorders Walk will be held on Saturday, October 26 at E.P. "Tom" Sawyer State Park here in Louisville. This is a new venue for our Walk in a very popular park on the eastern edge of Jefferson County, easily accessible from all interstates. With this end of October date, we will once again have a Halloween theme with prizes for best costumes, trick or treat candies, "ghoulish" music, "scary" games, pumpkin painting, door prizes, and more.

We issued a "Call to Action" during the Family Day at the Louisville Zoo and invited our attendees to make a commitment to participate in this year's Walk as Team Captains and start forming and registering teams or as individual Walkers. Special door prizes were awarded among those who committed. You should have received a postcard recently inviting you to get started and register. As before, we will present awards to the top 3 Teams and top 3 Fundraisers and prizes and medals to these winning teams. Incentive prizes will also be awarded for amounts of $250 and more raised.

Time is going by quickly, so please go to **www.uniteforbleedingdisorders.org/event/KY19** to register your Team and instruct your Team members to register as well and start fund-raising. Moneys raised help support important services, which KHF provides to its Kentucky Bleeding Disorders Community, such as scholarships, summer camp, advocacy, and child safety items. Moreover, the Walk gives visibility to families affected by bleeding disorders and increases awareness in the larger community of what life with a bleeding disorder entails for affected men, women, and children.

Play A Round for a Cure Golf Scramble

For its 30th anniversary, the 2019 KHF Golf Scramble fundraiser was held on Monday, June 24th at a beautiful new venue, Glen Oaks Country Club in Prospect. Our fifteen teams of golfers enjoyed eighteen fun, yet challenging holes of golf followed by a scrumptious dinner, silent auction, and awarding of well-deserved prizes in the clubhouse. Of course, we like to pamper our players and provided them with tasty box lunches and snacks for the course, not to mention a goodie bag and commemorative gift to take home. The robust rain shower that developed in the afternoon offered a good soaking but did not dampen the spirits of our enthusiastic players and dedicated volunteers. We thank them all for being good sports and persevering through the rain. The BMR Partners/St. Matthews Specialty Pharmacy team won 1st place with a score of 50. The Bayer HealthCare team achieved 2nd place with a score of 56, and the HEMA Biologics team came in 3rd with a score of 58. The Putting Contest was won by Sam Browning of Louisville, and the $430 "Ball Drop" win went to Steve Chaput, also of Louisville. We thank all donors, volunteers, golf committee members and committee Chair, William Black, as well as our event, team, and player sponsors. They were CSL Behring, Gold Level; Bayer HealthCare, Novo Nordisk, and Takeda, Silver Level; BMR Partners-St. Matthews Specialty Pharmacy, Diplomat Specialty Infusion Group, Genentech KDS, Octapharma, Republic Bank & Trust Company, Genentech Employees, LTC (R) & Mrs. Tharp: "In Memory of Gary Bandy," Glen Hitt, Sr. and Friends, and Heritage Biologics.

Event News

In Memory

April 1, 2019 – June 30, 2019

Theodore Bradley "Ted" Forcht
- William Chris & Christie Aaron
- Guy Adams
- John & Liliana Arceri
- Susan R. Arnold
- Bank of Columbia
- Ann & Brian Beloin
- Eli & Mary Lynne Capilouto
- CKQ Broadcasting
- Cumberland Valley Insurance Management, Inc.
- Paul D. Dole
- First Financial Credit Employees
- First Southern National Bank
- Karen Hackney
- Brenda Hamblin
- Hometown Bank
- Pat Huff
- Thomas L. Jensen, P.S.C.
- Bill & Jenny Storms, Johnny Wheels
- John Bill Keck
- The Staff of Kentucky Association of Health Care Facilities
- Kentucky Bankers Association
- Robert & Dolores Mackey
- Marco Island Center for the Arts
- Robert & Nancy Mitchell
- Dr. Steve & Kim Morton and Family
- Rebecca Myers
- Michael A. Noftsger
- Donnie & Brenda Patrick
- V.R. & M.A. Phipps
- Greg & Suzanne Razmus
- Republican Party of Kentucky
- Enoch & Phyllis Roberts
- Leslie W. & Daniel Sanford
- Jo Scheid & the Rodes Family
- Rodney & Laurie Shockley
- Family of Edwin L. Smith
- Steve & Betty Surmont and Owens Inc.
- Melvin & Margie Tate
- Thomas Consulting
- Mike Weiler, DesigNation Inc.
- Jean M. Wells

Betty Lou Mattingly
- James Ray & Parkwood Baptist Church

Beth McDonald
- Louise Hardaway

Gone from our sight but never our memories; gone from our touch but never our hearts…

From Food to Factor:

The Road to New Therapies in Hemophilia

Wendy Owens

cont. from page 6

they offer customers and insurers on these types of medical expenses. Drug companies also issue higher prices for drugs that can extend or even save lives. To help their chances of getting a drug on an insurance company's or PBM's drug formulary, pharmaceutical companies may negotiate drug rebates to ensure that the drug ends up as a "preferred drug" on a formulary. Preferred drugs cost insurers and patients less, so patients are more likely to choose them. Another way drug manufacturers balance revenue generation is by pricing drugs low at first, and then increasing the price at steady intervals.

WHAT? MORE OBSTACLES TO NEW DRUGS?

So to recap, here are the hurdles a new factor therapy or novel therapy must clear to reach you, the patient: (1) the treatment must receive FDA approval; (2) a P&T committee must give the drug a thumbs-up to "include," or thumbs-sideways for "optional for inclusion" on a formulary; (3) an insurer or PBM and the drug company must negotiate a price to be paid for the new drug. Now you can get that drug, right? Well, not exactly. You can face your own obstacles to receiving a new therapy, and these may be put in place by the same thumbs-up or thumbs-sideways decision group—the P&T committee.

CVS Caremark says, "The physician always makes the ultimate prescribing determination as to the most appropriate course of therapy."[6] But beware. It is a P&T committee that reviews and approves all utilization management (UM) criteria for a drug. UM criteria is a set of techniques used by insurers to manage costs. UM criteria may include prior authorization, step therapy, and quantity limits outside of FDA-approved labeling, which specifies dosing sizes and frequency.[7] What this means is that, after discussing pros, cons, and options of new therapies with you, your hematologist makes the prescribing determination, but you still might not have immediate access to a new therapy.

Prior authorization requires your doctor to tell your health insurer why you need a specific medication, and the health insurer has to agree that you need it. If your health insurer approves the requested authorization, the approval may be valid for only a set period of time and, when that time is up, may require reapproval. Prior authorization is one way insurers and PBMs try to keep costs down. Unfortunately, having to get prior authorization when a therapy is first prescribed, and reapproval at whatever time interval is required, can slow your access to treatment.[8] Find out whether a new therapy requires prior authorization before deciding to switch. Be prepared by having enough of your old product on hand to cover any delay in getting the new therapy.

Another barrier you may face is step therapy, sometimes called "fail first." Step therapy requires a patient to try and fail on typically less expensive therapies in a stepwise process before he or she can receive a new, more expensive therapy or another drug not on the formulary. According to Blue Cross Blue Shield of Michigan, for example, medications that require the use of step therapy can include those with serious side effects and those that can be misused or abused.[9] The try-and-fail process is another way for insurers to keep drug costs down. This tactic is not uncommon for hemophilia patients, despite the serious impact it can have on bleed management, joint health, and quality of life. Check with your health insurance plan to find out if you must receive prior authorization or undergo step therapy to get the new treatment you want to use.

HOW MUCH "CHANGE" WILL A CHANGE COST YOU?

Let's say you now can switch to a new therapy because you and your hematologist have agreed that switching to this therapy is a good option for you, your insurance covers it, and no other barriers exist to your using it. How much will you pay for this new therapy? What you pay is dictated by the prescription drug coverage portion of your health insurance plan. It's critical that you review your health insurance plan information every year at open enrollment. The plan you select must meet both your health needs and your budget. You can check which drugs are available on your plan's formulary at open enrollment, or at any

From Food to Factor:

The Road to New Therapies in Hemophilia

Wendy Owens

time during the year, by calling your health insurer or visiting its website.

Check to see how much you will pay for a new therapy before filling your prescription. Most health plans use a cost-sharing formula for drugs, in which drugs are placed into different cost-sharing levels, called tiers. Generally, drug formularies are broken down into four to six cost-sharing tiers:

- Tier 1: You may pay a $10–$20 copay for drugs that normally are very low-cost and mostly generics (there are no generics to treat hemophilia).
- Tier 2: You may pay about a $40 copay for higher-cost generic drugs and low-cost brand-name drugs.
- Tier 3: You may pay about a $60 copay for brand-name drugs for which there are no generics.
- Tier 4: You may pay a $100-plus copay or a coinsurance payment of 10%–40% of the cost of a drug for highest-cost drugs or specialty drugs (drugs on this tier are usually biologic drugs, like therapies used to treat hemophilia).

It's important to determine on which tier a new hemophilia therapy appears. Part of your decision to switch treatment should be your out-of-pocket cost for the new therapy. Beyond knowing how much you'll pay for a new therapy, watch out: you may have to pay full price for a new drug until you meet your plan's deductible.

RAISING THE BAR ON QUALITY OF LIFE

It's a very exciting time in hemophilia treatment. New therapies could be incremental steps up in your quality of life or have positive, life-changing impacts. You have now, and will have in the future, no shortage of treatment options. And this means you'll have decisions to make. But your healthcare plan can limit or restrict some of those options. Do your homework, talk to your hematologist, and verify access to the therapy of choice and its associated costs.

And please avoid anyone who suggests you use only remedies found in a grocery store to treat your hemophilia!

3. Formulary Development and Management at CVS Caremark, https://www.caremark.com/portal/asset/ FormDevMgmt.pdf (accessed Mar. 5, 2018).
4. "How We Build a Formulary," Express Scripts. Available at lab.express-scripts.com (accessed Mar. 21, 2018).
5. Mathew Herper, "The Cost of Developing Drugs Is Insane. That Paper That Says Otherwise Is Insanely Bad," Forbes, Oct. 17, 2017. Available at www.forbes.com (accessed Mar. 21, 2018).
6. Formulary Development and Management at CVS Caremark (accessed Mar. 10, 2018).
7. As part of a drug approval by the FDA, the drug's manufacturer must specify dosing sizes and frequency. Treatments are prescribed based on these specifications, but if a doctor wants different dosing for a patient, prior authorization may be needed.
8. Wendy Owens, "Could Cost Controls Prevent Access to Your Factor Brand?" PEN's Insurance Pulse, Sept. 2015.
9. "Prior Authorization and Step Therapy Coverage Criteria," Blue Cross Blue Shield of Michigan, June 2018. Available at www.bcbsm.com (accessed June 5, 2018).

Do The Five

Follow these steps to prevent or reduce complications of bleeding disorders

1. **Get an annual comprehensive checkup at a hemophilia treatment center.**
2. **Get vaccinated – Hepatitis A and B are preventable.**
3. **Treat bleeds early and adequately.**
4. **Exercise to protect your joints.**
5. **Get tested regularly for blood-borne infections.**

To find out more about the National Prevention Program developed by the National Hemophilia Foundation in collaboration with the Centers for Disease Control and Prevention (CDC), click on www.hemophilia.org or call toll-free 800-42-HANDI.

KHF does not give medical advice or engage in the practice of medicine. KHF under no circumstances recommends particular treatments for specific individuals and in all cases recommends that you consult your physician or local treatment center before pursuing any course of treatment.

Upcoming Events

Camp Discovery
Sunday, July 28 – Thursday, August 1

Summer Family Event
Saturday, August 24

Kentucky Unite for Bleeding Disorders Walk
Saturday, October 26

KENTUCKY HEMOPHILIA FOUNDATION
1850 Taylor Avenue #2
Louisville, KY 40213-1594

Non Profit Org.
U.S. Postage
PAID
Louisville, KY
Permit No. 883

SOLE POWER

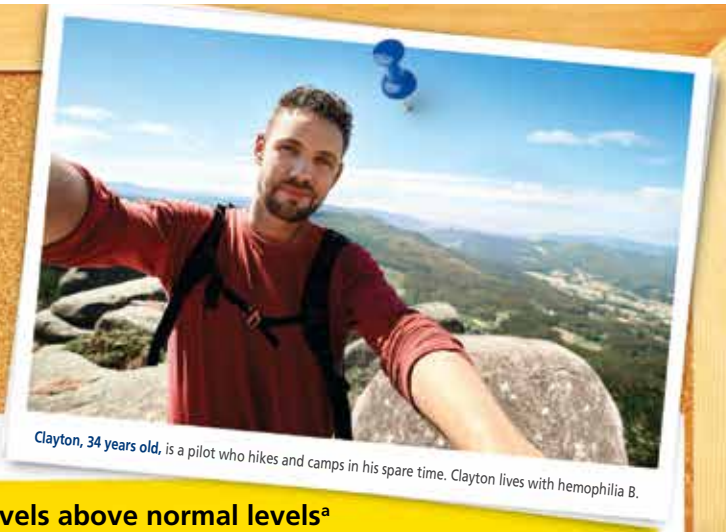
Walk

KHF

Saturday, October 26, 2019
New Location!

In hemophilia B

TAKE CONTROL TO A HIGH LEVEL WITH REBINYN®



Clayton, 34 years old, is a pilot who hikes and camps in his spare time. Clayton lives with hemophilia B.

Rebinyn® elevates factor levels above normal levels[a]

+94% Factor IX (FIX) levels achieved immediately after an infusion[b]

17% FIX levels sustained after 7 days[a]

With a single dose of Rebinyn® 40 IU/kg in adults with ≤2% FIX levels[a]

[a]In two phase 3 studies, factor levels were evaluated for 1 week after the first dose of Rebinyn® 40 IU/kg. The average levels after 7 days were 16.8% in 6 adults, 14.6% in 3 adolescents, 10.9% in 13 children ages 7 to 12 years, and 8.4% in 12 children up to age 6 years.

[b]Based upon a 2.34% increase in factor levels per IU/kg infused in adults.

Image of hemophilia B patient shown is for illustrative purposes only.

INDICATIONS AND USAGE

What is Rebinyn® Coagulation Factor IX (Recombinant), GlycoPEGylated?

Rebinyn® is an injectable medicine used to replace clotting Factor IX that is missing in patients with hemophilia B. Rebinyn® is used to treat and control bleeding in people with hemophilia B. Your healthcare provider may give you Rebinyn® when you have surgery. Rebinyn® is not used for routine prophylaxis or for immune tolerance therapy.

IMPORTANT SAFETY INFORMATION

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

- **Do not attempt to do an infusion yourself unless you have been taught how by your healthcare provider or hemophilia treatment center.** Carefully follow your healthcare provider's instructions regarding the dose and schedule for infusing Rebinyn®.

Who should not use Rebinyn®?

Do not use Rebinyn® if you:

- are allergic to Factor IX or any of the other ingredients of Rebinyn®.
- are allergic to hamster proteins.

What should I tell my health care provider before using Rebinyn®?

Tell your health care provider if you:

- have or have had any medical conditions.
- take any medicines, including non-prescription medicines and dietary supplements.
- are nursing, pregnant, or plan to become pregnant.
- have been told you have inhibitors to Factor IX.

How should I use Rebinyn®?

- Rebinyn® is given as an infusion into the vein.
- **Call your healthcare provider right away if your bleeding does not stop after taking Rebinyn®.**
- Do not stop using Rebinyn® without consulting your healthcare provider.

What are the possible side effects of Rebinyn®?

- **Common side effects include** swelling, pain, rash or redness at the location of the infusion, and itching.
- **Call your healthcare provider right away or get emergency treatment right away if you get any of the following signs of an allergic reaction:** hives, chest tightness, wheezing, difficulty breathing, and/or swelling of the face.
- **Tell your healthcare provider about any side effect that bothers you or that does not go away.**
- Animals given repeat doses of Rebinyn® showed Polyethylene Glycol (PEG) inside cells lining blood vessels in the choroid plexus, which makes the fluid that cushions the brain. The potential human implications of these animal tests are unknown.

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

Rebinyn® is a prescription medication.

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.

Learn more at rebinyn.com

novo nordisk®

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

rebinyn®
Coagulation Factor IX (Recombinant), GlycoPEGylated

rebinyn®

Coagulation Factor IX (Recombinant), GlycoPEGylated

Brief Summary Information about:
REBINYN® Coagulation Factor IX (Recombinant), GlycoPEGylated

Rx Only

This information is not comprehensive.

- Talk to your healthcare provider or pharmacist
- Visit www.novo-pi.com/REBINYN.pdf to obtain FDA-approved product labeling
- Call 1-844-REB-INYN

Read the Patient Product Information and the Instructions For Use that come with REBINYN® before you start taking this medicine and each time you get a refill. There may be new information.

This Patient Product Information does not take the place of talking with your healthcare provider about your medical condition or treatment. If you have questions about REBINYN® after reading this information, ask your healthcare provider.

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

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

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

What is REBINYN®?

REBINYN® is an injectable medicine used to replace clotting Factor IX that is missing in patients with hemophilia B. Hemophilia B is an inherited bleeding disorder in all age groups that prevents blood from clotting normally.

REBINYN® is used to treat and control bleeding in people with hemophilia B.

Your healthcare provider may give you REBINYN® when you have surgery.

REBINYN® is not used for routine prophylaxis or for immune tolerance therapy.

Who should not use REBINYN®?

You should not use REBINYN® if you

- are allergic to Factor IX or any of the other ingredients of REBINYN®
- if you are allergic to hamster proteins

If you are not sure, talk to your healthcare provider before using this medicine.

Tell your healthcare provider if you are pregnant or nursing because REBINYN® might not be right for you.

What should I tell my healthcare provider before I use REBINYN®?

You should tell your healthcare provider if you

- Have or have had any medical conditions.
- Take any medicines, including non-prescription medicines and dietary supplements.
- Are nursing.
- Are pregnant or planning to become pregnant.
- Have been told that you have inhibitors to Factor IX.

How should I use REBINYN®?

Treatment with REBINYN® should be started by a healthcare provider who is experienced in the care of patients with hemophilia B.

REBINYN® is given as an infusion into the vein.

You may infuse REBINYN® 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 hemophilia treatment center or healthcare provider. Many people with hemophilia B learn to infuse the medicine by themselves or with the help of a family member.

Your healthcare provider will tell you how much REBINYN® to use based on your weight, the severity of your hemophilia B, and where you are bleeding. Your dose will be calculated in international units, IU.

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

If your bleeding is not adequately controlled, it could be due to the development of Factor IX inhibitors. This should be checked by your healthcare provider. You might need a higher dose of REBINYN® or even a different product to control bleeding. Do not increase the total dose of REBINYN® to control your bleeding without consulting your healthcare provider.

Use in children

REBINYN® can be used in children. Your healthcare provider will decide the dose of REBINYN® you will receive.

If you forget to use REBINYN®

If you forget a dose, infuse the missed dose when you discover the mistake. Do not infuse a double dose to make up for a forgotten dose. Proceed with the next infusions as scheduled and continue as advised by your healthcare provider.

If you stop using REBINYN®

Do not stop using REBINYN® without consulting your healthcare provider.

If you have any further questions on the use of this product, ask your healthcare provider.

What if I take too much REBINYN®?

Always take REBINYN® exactly as your healthcare provider has told you. You should check with your healthcare provider if you are not sure. If you infuse more REBINYN® than recommended, tell your healthcare provider as soon as possible.

What are the possible side effects of REBINYN®?

Common Side Effects Include:

- swelling, pain, rash or redness at the location of infusion
- itching

Other Possible Side Effects:

You could have an allergic reaction to coagulation Factor IX products. **Call your healthcare provider right away or get emergency treatment right away if you get any of the following signs of an allergic reaction:** hives, chest tightness, wheezing, difficulty breathing, and/or swelling of the face.

Your body can also make antibodies called "inhibitors" against REBINYN®, which may stop REBINYN® from working properly. Your healthcare provider may need to test your blood for inhibitors from time to time.

You may be at an increased risk of forming blood clots in your body, especially if you have risk factors for developing blood clots. Call your healthcare provider if you have chest pain, difficulty breathing, leg tenderness or swelling.

Animals given repeat doses of REBINYN® showed Polyethylene Glycol (PEG) inside cells lining blood vessels in the choroid plexus, which makes the fluid that cushions the brain. The potential human implications of these animal tests are unknown.

These are not all of the possible side effects from REBINYN®. Ask your healthcare provider for more information. You are encouraged to report side effects to FDA at 1-800-FDA-1088.

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

What are the REBINYN® dosage strengths?

REBINYN® comes in three different dosage strengths. The actual number of international units (IU) of Factor IX in the vial will be imprinted on the label and on the box. The three different strengths are as follows:

Cap Color Indicator	Nominal Strength
Red	500 IU per vial
Green	1000 IU per vial
Yellow	2000 IU per vial

Always check the actual dosage strength printed on the label to make sure you are using the strength prescribed by your healthcare provider.

How should I store REBINYN®?

Prior to Reconstitution (mixing the dry powder in the vial with the diluent):

Store in original package in order to protect from light. Do not freeze REBINYN®.

REBINYN® vials can be stored in the refrigerator (36-46°F [2°C-8°C]) for up to 24 months until the expiration date, or at room temperature (up to 86°F [30°C]) for a single period not more than 6 months.

If you choose to store REBINYN® at room temperature:

- Note the date that the product is removed from refrigeration on the box.
- The total time of storage at room temperature should not be more than 6 months. Do not return the product to the refrigerator.
- Do not use after 6 months from this date or the expiration date listed on the vial, whichever is earlier.

Do not use this medicine after the expiration date which is on the outer carton and the vial. The expiration date refers to the last day of that month.

After Reconstitution:

The reconstituted (the final product once the powder is mixed with the diluent) REBINYN® should appear clear without visible particles.

The reconstituted REBINYN® should be used immediately.

If you cannot use the reconstituted REBINYN® immediately, it should be used within 4 hours when stored at or below 86°F (30°C). Store the reconstituted product in the vial.

Keep this medicine out of the sight and out of reach of children.

What else should I know about REBINYN® and hemophilia B?

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

More detailed information is available upon request.

Available by prescription only.

For more information about REBINYN®, please call Novo Nordisk at 1-844-REB-INYN.

Revised: 11/2017

REBINYN® is a trademark of Novo Nordisk A/S.

For Patent Information, refer to: http://novonordisk-us.com/patients/products/product-patents.html

Manufactured by:
Novo Nordisk A/S
Novo Allé, DK-2880 Bagsværd, Denmark

For information about REBINYN® contact:
Novo Nordisk Inc.
800 Scudders Mill Road
Plainsboro, NJ 08536, USA

USA17BIO03951 12/2017

novo nordisk®

KHF Summer Family Event

You're Invited!

It's a lot of fun packed into one day,
Saturday, August 24th. It all starts at
8:30 a.m. at the Hyatt Regency in Louisville, KY
and ends with a cruise on the Mary M. Miller Steamboat.

Printed in USA 02/19 PP-JIV-US-0467-1

KHF Event Calendar

Summer Family Event
(Annual Education Meeting, Post-Meeting Social Activity, & Walk Kick-Off Lunch)
Saturday, August 24, 2019
Hyatt Regency • Louisville, KY

Aug. 24

Oct. 26

Kentucky Unite for Bleeding Disorders Walk
Saturday, October 26, 2019
E.P. "Tom" Sawyer State Park
Louisville, KY

New Location!

Poinsettia Fundraiser
November/December 2019

Nov/ Dec

Dec. 1

Year-End Family Event
Sunday, December 1, 2019
Holy Trinity Clifton Campus • Louisville, KY

Vegasville Fundraiser
February 22, 2020
The Olmstead • Louisville, KY

Dec. 1

Kentucky Unite for Bleeding Disorders Walk

Our Walk will be here before you know it, so please go to www.uniteforbleedingdisorders.org/event/KY19 to register your team and instruct your team members to register as well and start fund-raising. The theme this year will be Halloween — so start planning your costumes too.