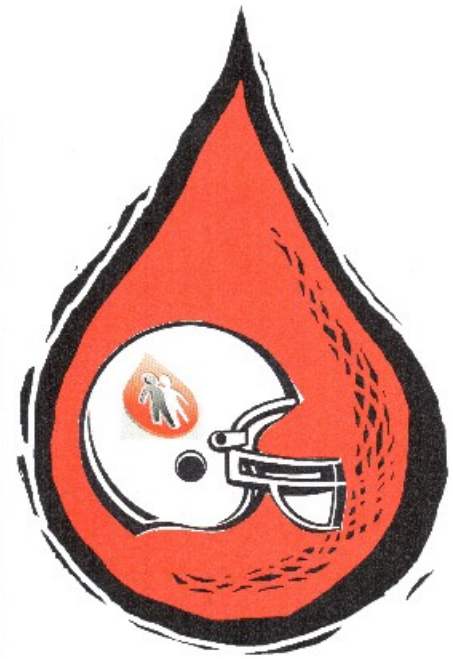

BIG RED FACTOR

2018—Issue IV

Nebraska Chapter News

Thank you all for an absolutely amazing year. 2018 was a full of great events, a lot of time spent together and some new growth for our chapter. We added and dropped some programs, we changed venues for others and we even added our very first second full time person, Misti. We welcomed new families into our community and gathered together to support losses of our blood brothers. We are blessed to be able to serve the bleeding disorder community here in Nebraska through education, advocacy and building life long relationships that help get us through the hard days and help us celebrate the exciting ones and big milestones.

As we look to 2019, I'm excited for change and growth for our chapter. We are exploring new events, new programs and updating things that we've done forever. We are working on new partnerships, new funding options and a greater imprint on our community. Exciting things are going to be happening and we want you on board with us. The ground work for the future of NENHF is being laid now by the staff, the board and by your input and guidance. Thank you for trusting us with your community and for letting us be part of your family.

This newsletter issue is brought to you in partnership with the Nebraska Hemophilia Treatment Center. Look for several articles from your HTC physicians on Hemlibra, Long Acting Factors and Transition Care. Thank you to the HTC for providing these articles and for the Hemophilia Alliance Foundation for funding the printing and mailing of this issue.

Have a Happy and Safe Holiday Season!

See you in 2019!

Inside this issue:

NEBRASKA CHAPTER
NATIONAL HEMOPHILIA FOUNDATION
www.nebraskanhf.org

NEBRASKA CHAPTER
NATIONAL HEMOPHILIA FOUNDATION
www.nebraskanhf.org

Our Mission:

The National Hemophilia Foundation—Nebraska Chapter is dedicated to finding better treatments and cures for inheritable bleeding disorders and to preventing the complications of these disorders through education, advocacy & research.

Staff

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Maureen Grace

Development Manager
Misti Mitchell

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The material in this newsletter is provided for your general information only. The Nebraska Chapter does not give medical advice or engage in the practice of medicine. NHF-NE does not recommend 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.

2019
First Quarter Events

February

16th: Advocacy Workshop

Tentative VWD Education Day

March

Hemophilia Awareness Month

3rd-4th: Nebraska Advocacy Hill Day and Training

27th-29th: Washington Days, Washington DC

Unite for Bleeding Disorders

2019 Save the Dates

April 27-28, 2019
Family Education Weekend
Lincoln, Nebraska

July 14, 2019
Women's Retreat with HOI
Des Moines, IA

September 21, 2019
Unite for Bleeding Disorders Walk
Chalco Hills Recreation Center

A Proud Member of
COMMUNITY HEALTH CHARITIES℠ NEBRASKA

Washington Days

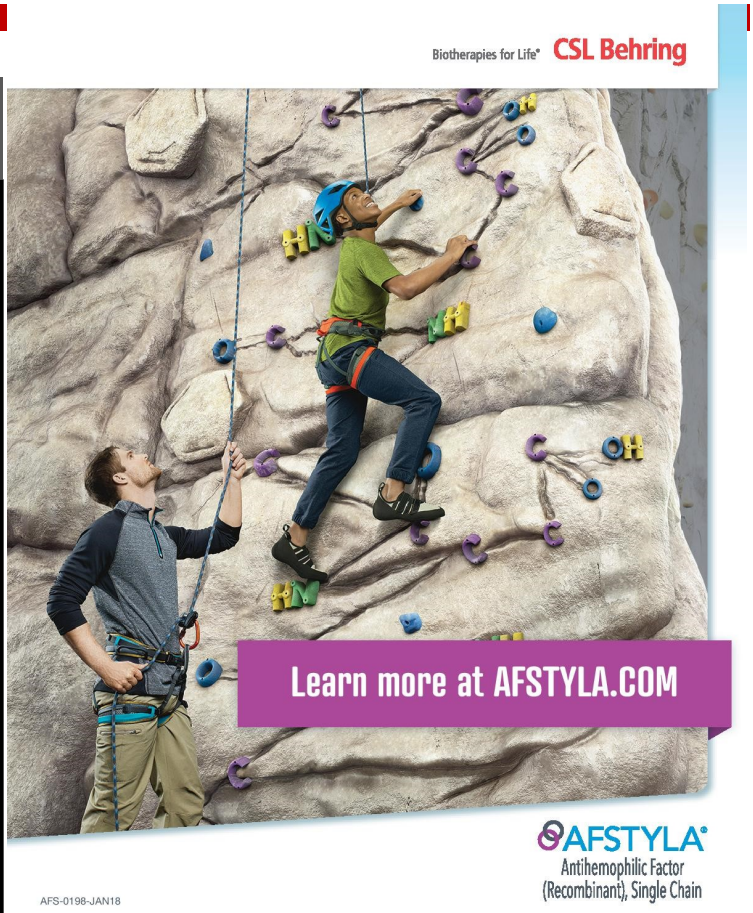
NHF's Washington Days will be held
March 27-29th in Washington DC.
Registration is open through February 8th.

If you would like to apply for travel funds through Nebraska NHF, please complete the application found on our website under Advocacy.

Limited Travel Grants are available to cover the cost of airfare and hotel stay.

Applications Due January 11th

Applicants must be willing to commit to year round advocacy efforts with the Nebraska Chapter to receive travel funds for advocacy.



Harvest Fest

The Nebraska Chapter holds an annual harvest fest gathering for the families of the bleeding disorders community. This year's event took place on Saturday, October 20th. In past years, the event was held at Vala's Pumpkin Patch, this year we decided to try a new location at the Roca Berry Farm in Roca, NE. The venue change proved to be delightful for those in attendance. Roca Farm did not disappoint as it had several unique activities and food vendors that kept adults and children entertained.

Harvest Fest is a fun event for families to enjoy the day and connect with others in the bleeding disorders community. Our sponsors for the event were Novo Nordisk, Shire, Superior Biologics, Bioethics Advantage and Aptevo. Thank you for making this great fall event possible.



SUPERIOR BIOLOGICS
Making a difference in Peoples' lives

EXPERTISE IN:
- Hemophilia A
- Hemophilia B
- Von Willebrand's Disease
- Other Bleeding Disorders

SERVICES INCLUDE:
- Nursing & Pharmacy 24/7
- Infusion Education & Training
- Reimbursement Specialists
- Direct Communication with the People You Know
- Same Day Delivery

Craig MacDonald
Regional Territory Manager
760-485-8281

Vanessa Sidwell
Regional Accounts Manager
816-739-9841

Office: 844.237.3561
Fax: 844.237.3562

WE ARE FAMILY

You and your family are unique.

So is your hemophilia.

Helping you get back to family time.

Experienced ARJ pharmacists and nurses are delivering Nebraskans personalized quality home infusion care every day.

- Last year, we secured over $625,000 in patient resource assistance.
- Most ARJ patients are recommended by friends or family.
- Patients love our Ready Pack® infusion kits for easy self-infusion.

Our pharmacy is conveniently located near you.
(866) 451-8804
arjinfusion.com/nebraska

ARJ Infusion SERVICES
Where Caring is the Ultimate Factor.

Women's Retreat

It's been years since we've hosted a Women's Retreat and we knew it was time to bring it back. Through a grant through the Hemophilia Alliance Foundation, we were able to host a Women's Retreat at the Courtyard Omaha Aksarben Village on November 10th. 15 women gathered to learn about managing stress and improving wellness, self-care, meditation and engaged in conversations while crafting. The attendees ranged from affected, carriers, caregivers, mothers and board members. We talked about managing our every day stressors and how we plan to take time for ourselves and self care. We worked through some guided meditation to center ourselves and to focus on how effective taking even a few minutes to destress can go a really long way.

A special thank you to The Hemophilia Alliance for sponsoring this event and to NCHS for bringing in nail stickers and massage chairs to make this event full of fellowship and relaxation for the ladies in attendance.

PLEASE SAVE THE DATE FOR A JOINT WOMEN'S RETREAT WITH HEMOPHILIA OF IOWA

The weekend of JULY 14th, 2019

The Changing Landscape of the Hemophilia Community

The hemophilia community is incredibly strong, with a rich but heavy history. Very few rare disease groups have the same support, family and tight knit community as the bleeding disorder community does. We are so fortunate to be able to provide year round education, advocacy and support to those in our chapter. However, the culture of the community is changing, and we are evolving along with it. The mission of NE NHF has always been to provide these things to our community and that dedication will not waiver. Some of these programs and events will start to look different over the coming years as we work to ensure the needs of the community are met while balancing the costs of programming with changes in our funding structure.

We have always been fortunate to have most of our funding come fairly easy from our industry partners. The way these companies are now supporting chapters is changing and funding isn't at same level it has been sustained at for years. Their focus is shifting more to direct education and advocacy efforts and funding fewer fundraisers and social events. We knew the change was coming eventually, but it has arrived sooner than we anticipated. It is imperative that we diversify our revenues at a chapter level so that we can continue to offer the same amount of programming and events as we have for years. This is affecting chapters all across the country, not just Nebraska. We are working closely with our National team to create a development plan that will assure that NENHF is financially stable for years to come. Part of this plan is to hire a full time development manager to grow our fundraising efforts beyond industry support and become less financially reliant on industry. We are excited for this growth and what it means for our chapter. Transitioning this position to full time and filling it with a greatly qualified individual will take us a long way.

You may wonder what this will look like for our chapter as we work on growth, change and stability while still serving our mission for the Nebraska bleeding disorder community. We are still incredibly dedicated to providing advocacy on a state and national level and meaningful and relevant education throughout the year through multiple educational events. We just have to be extremely cognizant of our budget so that we can continue these programs as our funding shifts. This does mean that some events that have been the same for years, will start to see some changes. NE NHF loves offering fun, networking events for our families but starting with Harvest Festival this year, you will see some venue changes and we are looking into trying new events in place of the ones we have been seeing for years. We want to ensure that we are offering meaningful programming, reaching all areas of our community and that we are supportive of everyone in Nebraska affected by a bleeding disorder.

Change is scary, but it can also be very refreshing. I truly believe the next couple years will be a little bit of both. That being said, we are very open to input, ideas and assistance from anyone in our chapter who wants to step up or give us new ideas. At the end of the day, this is your life and your community, and we want the chapter to reflect that. The next few years will be ever changing as we find our new stride, but through it all, NENHF is dedicated to remain on mission with our bleeding disorder community and family in the forethought of everything we do.

Thank you for letting us be part of your family and your lives. We love this community and it's our pleasure to serve you. Thank you from myself and our Board of Directors for this amazing ongoing opportunity to be your hemophilia chapter.

Maureen Grace, Executive Director Reprinted from June Newsletter

PING (Parent Information Networking Group)

PING is an opportunity for families to come together and learn information through a short educational piece, then build a network with others in the bleeding disorders community. It is a semi-annual event held in June and December each year. On December 8, we held our last PING event of 2018 at the Lincoln Children's Museum in Lincoln, NE. Families with kids came together for this holiday gathering where they enjoyed a taco bar, gave chapter staff feedback on 2018 and gave ideas for future programming. The museum was closed for this event so the children were able to enjoy the place freely while parents networked.

Thank you to our 2019 Industry Sponsors: Bayer, Bioethics Advantage, CSL Behring, CVS Speciality, NCHS, Novo Nordisk, Shire and Genentech. This is one of our favorite events of the year and it's possible through your support.

Medicaid Expansion

Joe Mickeliunas,
Nebraska Chapter Advisory Board Member

November's midterm election saw Nebraskans vote to pass Initiative 427 which will expand Medicaid under the Affordable Care Act. The initiative has been opposed by Governor Pete Ricketts and GOP state legislators, but Nebraskans supported the referendum that was initiated due to the many obstacles put in place by Ricketts and the legislature that left many Nebraskans below the poverty line uninsured. A study by the Urban Institute shows that 45,000 will gain coverage and the uninsured rate will fall to 9.6% from 12.4% next year.

Initiative 427 will bring in nearly $600 million a year in federal funding. A study of the economic impact of Medicaid expansion conducted by economics professor Allan Jenkins and management professor Ron Konecny of the University of Nebraska at Kearney found that the federal funds will reportedly create and sustain 11,000 jobs and generate $1.3 billion in economic activity across the state.

The vote to pass Initiative 427 is a victory for the bleeding disorders community. NHF has stated that "persons with hemophilia or related bleeding disorders who fall into coverage gaps may continue to lack access to life-saving clotting factor therapies and to a primary care doctor and/or hemophilia treatment center (HTC). As a result, they will be unable to develop or adhere to a recommended treatment plan and may have significantly more bleeds, require hospitalization or suffer other related complications. Such events can significantly burden the health care system and end up costing states more money in the long run." Medicaid expansion will help to close this gap and bring coverage to many who do not have adequate care.

Between now and the 2020 election, we must remain vigilant to see that the will of the people and Medicaid expansion in Nebraska is seen through. Governor Ricketts has not announced how he will implement Initiative 427. To ensure a smooth transition and implementation of Medicaid expansion, contact your local representative or the office of Governor Ricketts and share your personal story. The bleeding disorders community should remain united in making sure all of our members have ample coverage to maintain a happy and healthy life without fear of falling into an insurance gap.

NEBRASKA CHAPTER
NATIONAL HEMOPHILIA FOUNDATION

Nebraska Advocacy Hill Day

NEBRASKA CHAPTER
NATIONAL HEMOPHILIA FOUNDATION
www.nebraskanhf.org

SUNDAY, MARCH 3

Telling Your Story Educational Dinner

Location TBD, OMAHA

6:00 PM- 9:00 PM

TELL YOUR STORY
USE YOUR VOICE
ADVOCATE FOR BLEEDING DISORDERS

MONDAY

MARCH 4, 2018

NEBRASKA STATE CAPITOL
LINCOLN, NEBRASKA
8:30 AM- 1:00 PM

WWW.NEBRASKANHF.ORG

Transition Care with the HTC

James Harper, M.D.
Pediatric Oncology and Hematology
University of Nebraska Medical Center

I wanted to take this opportunity to discuss our plan for transition care between pediatric and adult care for our kids with hemophilia and severe von Willebrand's disease.

Transition between pediatric and adult care for teens and young adults with chronic diseases like sickle cell or cystic fibrosis is often quite difficult and sadly, those severely affected children have the most need to smoothly transition but frequently have a hard time doing so. Hemophilia has an advantage over these other diseases due to the need for the boys to take action and become independent while they are with you.

As some of you recall, we start to have this discussion around the time of your child's 4th birthday and we continue throughout your time with us.

At age 4, I want my patients with severe hemophilia or VWD to start participating in their care by helping their parents get the factor ready. They are too little to self-infuse, but helping to set things up gets the boys connected to the process.

At age 7-8 years, we want them to learn to self-infuse. This is a key skill for boys with severe hemophilia and those with moderate hemophilia who bleed frequently. The time between the start of a bleed and the administration of clotting factor helps to determine how much blood is lost into the bleeding site. This is important in terms of pain, and in terms of damage to local tissues from the bleed. Self-infusion is generally faster than depending on a parent to infuse the factor.

By 10-12 years, boys should be able to help keep track of their factor supply and should be encouraged to do this and to maintain their bleeding diary with only supervision from his parents. The bleeding diary can be completed electronically, or on paper. It is very important in this era of prophylaxis that we can review all the bleeds that occur so we can detect target joints or evidence that dose adjustments may be necessary.

Learning and mastering this skill means that your son will be even more independent. Remember, boys who have "Binder Moms" often grow up to have "Binder Wives". My mother and grandmother insisted that while I was in high school, I learned how to cook, mend my clothes, and do my own laundry. My grandmother told me that she made me do this because she thought I was special and did not want me to become dependent on the first girl that winked at me. Your sons are special and they need to be strong and independent, so make them do their own bleed diary. (It really is important.)

Transition Care, continued.

Talking with their home care reps and their insurance case workers are a big step toward adulthood. I do think that bringing your sons into these discussions as you feel they are ready is an important step towards their independence.

Finally, the need to transition to adult medical care is an important last step. Unlike many centers that have a fixed cut point at which your son moves to the adult team once he reaches a certain age, we do not currently do this. At age 19, either team may see your son. We offer this option in case your son wishes to go to college or trade school and would therefore benefit from maintaining a continuous medical record before moving on to his post-college life.

Adult treatment:
Krishna Gundabolu, MD
Nebraska Medical Center
402.559.5600

Pediatric treatment:
James Harper, MD
Children's Hospital:
402.955.3950

Nebraska Medicine
SERIOUS MEDICINE. EXTRAORDINARY CARE.

Children's
HOSPITAL & MEDICAL CENTER
OMAHA

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.

HEMLIBRA 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 OTHER POSSIBLE SIDE EFFECTS OF HEMLIBRA?

The most common side effects of HEMLIBRA include: redness, tenderness, warmth, or itching at the site of injection; headache; and joint pain. These are not all of the possible side effects of HEMLIBRA.

You may report side effects to the FDA at (800) FDA-1088 or www.fda.gov/medwatch. You may also report side effects to Genentech at (888) 835-2555.

Please see Brief Summary of Medication Guide on the following page for more important safety information, including **Serious Side Effects**.

A ONCE-WEEKLY SUBCUTANEOUS (GIVEN UNDER THE SKIN) INJECTION FOR PEOPLE WITH HEMOPHILIA A WITH FACTOR VIII INHIBITORS

We extend our appreciation to the individuals, families, and healthcare providers who participated in the clinical trials that led to the approval of HEMLIBRA®. We thank you and celebrate with the community who made it a reality.

Discover **HEMLIBRA.com**

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 with hemophilia A with factor VIII inhibitors.

WHAT IS THE MOST IMPORTANT INFORMATION I SHOULD KNOW ABOUT HEMLIBRA?

HEMLIBRA increases the potential for your blood to clot. Discontinue prophylactic use of bypassing agents the day before starting HEMLIBRA prophylaxis. Carefully follow your healthcare provider's instructions regarding when to use an on-demand bypassing agent, and the dose and schedule you should use. HEMLIBRA may cause the following serious side effects when used with 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 signs and symptoms of TMA during or after treatment with HEMLIBRA.
- **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 the signs or symptoms of blood clots during or after treatment with HEMLIBRA.

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.

Medication Guide Brief Summary
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. Discontinue prophylactic use of bypassing agents the day before starting HEMLIBRA prophylaxis. Carefully follow your healthcare provider's instructions regarding when to use an on-demand bypassing agent, and the dose and schedule you should use. HEMLIBRA may cause the following serious side effects when used with 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 HEMLIBRA:
  - 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 with hemophilia A with 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.

Tell your healthcare provider about all the medicines you 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.
- 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.
- 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 before the next scheduled dosing day and then continue with your normal weekly dosing schedule. Do not double your dose to make up for a missed dose.
- HEMLIBRA 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 for more than 7 days at 86°F (30°C) or below.
- 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 HEMLIBRA.

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.

WHAT ARE THE INGREDIENTS IN HEMLIBRA?

Active ingredient: emicizumab

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

Manufactured by: Genentech, Inc., A Member of the Roche Group,
1 DNA Way, South San Francisco, CA 94080-4990
U.S. License No. 1048
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For more information, go to www.HEMLIBRA.com or call 1-866-HEMLIBRA.
This Medication Guide has been approved by the U.S. Food and Drug Administration
Issued: 11/2017

HEMLIBRA™
emicizumab-kxwh
injection for subcutaneous use | 150 mg/mL

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SAVE THE DATE:

FAMILY EDUCATION WEEKEND

APRIL 27-28, 2019
LINCOLN, NE

LONG ACTING (EXTENDED HALF LIFE) FACTORS: MYTH OR REAL?

By Dr. Krishna Gundabolu

Hemophilia A occurs in around 1 in 5000 and Hemophilia B in 1in 30,000 male live births. When triggered by appropriate events, clotting is an important function of blood organ system not only to stop bleeding but also is vital in inflammation (repair process). A clot typically consists of blood cells (Red blood cells, platelets, white blood cells etc. with platelets playing a vital role) along with various clotting factors. Half-life is the amount of time in which the functional activity (level) of these coagulation factors drop down to half of previous level, for example from the time it takes for the level to drop from 100% to 50% or from 80% to 40% etc. Factor VIII and IX have half-lives around 8-12 hours and 18-24 hours respectively. The "peak" level is the factor level one achieves right after the injection and the "trough" level is the level right before the scheduled dose. For patients with severe congenital hemophilia A (VIII) or B (IX) without inhibitor disorder, who are on factor prophylaxis, generally the goal is to prevent a trough level less than 2% so that joint and other bleeding complications could be minimized. Different factor products (both VIII and IX) half-lives vary by the age of a person.

Since primary prophylaxis has become the standard of care for all with severe congenital hemophilia A and B after multiple studies showed improvement in joint health and quality of life, patients take factor infusions 2-3 times per week depending on their individual life styles and activities. Factor IX with longer natural half life has an inherent advantage compared to factor VIII allowing most Hemophilia B patients get away with 2 times a week infusion frequency compared to Hemophilia A patients who may need 3 times a week infusions with standard half-life products.

An ideal Extended half-life (EHL) product should allow a lower frequency of injections and maintain similar efficacy (Annualized bleeding rate), improve quality of life, no additional risk of inhibitor development with least serious adverse effects. Half-life of factors can be enhanced by various techniques including PEGylation (Adding PEG-Poly Ethylene Glycol- chains to factor which protects it from degradation by enzymes and removal by clearance receptors), Fc fusion, albumin fusion (attaching immunoglobulin Fc portion or Albumin allows them to be recycled effectively) or enhancing Von Willebrand factor affinity (for factor VIII products only). By doing so depending on the molecule, the half-life of recombinant factor VIII was increased to around 14-19 hours (1.5 to 2 fold increase) from 8-12 hours and factor IX increased to around 80-110 hours (4-6 fold increase) from 18-24 hours. Therefore the benefit to achieve the above goals is far more pronounced in factor IX-EHL products. There are many factor VIII and IX EHL products, which are currently FDA (Food and Drug Administration) approved for clinical use (listed below) and all of them have shown to be safer with no increased risk of inhibitors compared to the conventional third generation recombinant factors. On the other hand many cost efficiency analysis had show that EHL products could double the cost of therapy compared to the standard half-life products.

LONG ACTING (EXTENDED HALF LIFE) FACTORS: MYTH OR REAL? Cont.

Emicizumab (Hemlibra) is a bi-specific factor IXa and factor X directed antibody (Antibody with two heads binding to factors IXa and X- working like factor VIII at a physiological level), which was originally approved by FDA for patients with Factor VIII inhibitor disorder but approved in October 2018 for Severe Hemophilia A patients without inhibitor. The advantage for this drug is its route and frequency of administration as this is the first drug, which can be administered sub-cutaneous (under skin) with the frequency of administration as little as once every 4 weeks (weekly, twice weekly or four weekly options are available).

We can safely conclude that, In Hemophilia there had been tremendous leaps in positive scientific direction and EHL products certainly made a difference in the quality of life for many patients. Which option is right for you, depends on the discussion between you and your provider based on your individual life style, needs and preferences.

FACTOR VIII- EHL recombinant products:

NAME	MOLECULAR STRUCTURE	FDA approval	Mean half-life
Eloctate®	Fc-Fusion	2014	19 hours
Adynovate®	PEGylated	2015	14-16 hours
Afstyla®	Increased affinity for vWF	2016	14-15 hours
Jivi®	PEGylated	2018	18 hours

Factor IX-EHL recombinant products:

NAME	MOLECULAR STRUCTURE	FDA approval	Mean half-life
Alprolix®	Fc-Fusion	2014	80-90 hours
Idelvion®	Albumin fusion	2016	100-110 hours
Rebinyn®	PEGylated	2017	90-100 hours

This article was brought to you by the physicians at the
Nebraska Hemophilia Treatment Center (HTC).
Please make all medical and treatment decisions in partnership with your treating physician.

A Hemlibra Update from the Nebraska HTC

by James Harper, M.D.

Hemlibra (Emicizumab) has been approved by the FDA for all severe hemophilia patients (not just those with inhibitors).

This drug is called a "bispecific antibody". Antibodies are like fidget spinners. They have three ends. One end binds to the white blood cell and triggers the immune system, the other two ends bind the bad thing and allow it to be killed. The antibody then has two arms that bind to the bacteria or virus and one that binds to the immune system. A bispecific antibody has one binding site for one target chemical and the other binds a second.

Hemlibra binds active factor IX and inactive factor X. The factor IX activates factor X much like it would if bound to factor VIII. This chemical reaction is abnormal in both hemophilia A and B and is the root cause of bleeding in these diseases.

Factor VIII acts much like a cradle that holds activated factor IX and factor X. It allows factor IX to activate large amounts of factor X. This is key as both of these enzymes complete for space on the surface of the blood vessel and without factor VIII, don't produce enough clot to make your bleeding stop.

Hemlibra was first used to treat factor VIII inhibitors. It is not attacked by inhibitor antibodies that attack factor VIII. It was very successful in preventing bleeding. It was found tragically to combine badly with FEIBA at FEIBA's normal doses, resulting in life threatening clots. It is now recommended that if FEIBA has to be used with Hemlibra, that it be done using a lower dose. NovoSeven RT has been shown to be safer and the manufacturer recommends this agent if an acute bleed happens in a patient using this for inhibitor treatment.

Whether Hemlibra or Immune Tolerance should be used first when a new inhibitor patient is found is a matter of debate amongst hemophilia treaters. Hemlibra will bypass factor VIII inhibitors, but will not act to lower the titer of the inhibitor antibody. Immune Tolerance will act to lower the titer over time and may get the titer low enough to be permanently suppressed. It is more expensive and requires daily factor administration.

Hemlibra now has been approved for factor VIII deficient patients who do not have inhibitors. This offers a choice between frequent short acting factor doses and weekly doses of Hemlibra to start and then perhaps fewer (as little as monthly) subcutaneous injections.

In the case of severe bleeding or surgery, it is possible to treat this bleeding either with Hemlibra by itself or with additional doses of factor VIII.

If you are interested in trying this medication,

contact the treatment center and we will discuss it with you.

ADYNOVATE [Antihemophilic Factor (Recombinant), PEGylated] Important Information

What is ADYNOVATE?

- ADYNOVATE is an injectable medicine that is used to help treat and control bleeding in children and adults with hemophilia A (congenital Factor VIII deficiency).
- Your healthcare provider (HCP) may give you ADYNOVATE when you have surgery.
- ADYNOVATE can reduce the number of bleeding episodes when used regularly (prophylaxis).

ADYNOVATE is not used to treat von Willebrand disease.

DETAILED IMPORTANT RISK INFORMATION

Who should not use ADYNOVATE?

Do not use ADYNOVATE if you:

- Are allergic to mice or hamster protein
- Are allergic to any ingredients in ADYNOVATE or ADVATE [Antihemophilic Factor (Recombinant)]

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

What should I tell my HCP before using ADYNOVATE?

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 ADYNOVATE passes into your milk and if it can harm your baby.
- Are or become pregnant. It is not known if ADYNOVATE may harm your unborn baby.
- Have been told that you have inhibitors to factor VIII (because ADYNOVATE may not work for you).

What important information do I need to know about ADYNOVATE?

- You can have an allergic reaction to ADYNOVATE. 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.
- Do not attempt to infuse yourself with ADYNOVATE unless you have been taught by your HCP or hemophilia center.

What else should I know about ADYNOVATE 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 ADYNOVATE 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 ADYNOVATE?

- The common side effects of ADYNOVATE are headache and nausea. These are not all the possible side effects with ADYNOVATE. Tell your HCP about any side effects that bother you or do not go away.

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 ADYNOVATE on the following page and discuss with your HCP.

For full Prescribing Information, visit www.ADYNOVATE.com.

References: 1. ADYNOVATE Prescribing Information. **2.** Mullins ES, Stasyshyn O, Alvarez-Román MT, et al. Extended half-life pegylated, full-length recombinant factor VIII for prophylaxis in children with severe haemophilia A. *Haemophilia*. 2016 Nov 27. doi: 10.1111/hae.13119 [Epub ahead of print]. **3.** Data on file.

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Shire

ADYNOVATE
[Antihemophilic Factor (Recombinant), PEGylated]

Patient Important facts about

ADYNOVATE® [Antihemophilic Factor (Recombinant), PEGylated]

This leaflet summarizes important information about ADYNOVATE. 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 ADYNOVATE. If you have any questions after reading this, ask your healthcare provider.

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

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 ADYNOVATE so that your treatment will work best for you.

What is ADYNOVATE?

ADYNOVATE is an injectable medicine that is used to help treat and control bleeding in children and adults with hemophilia A (congenital Factor VIII deficiency). Your healthcare provider may give you ADYNOVATE when you have surgery. ADYNOVATE can reduce the number of bleeding episodes when used regularly (prophylaxis).

ADYNOVATE is not used to treat von Willebrand disease.

Who should not use ADYNOVATE?

You should not use ADYNOVATE if you:

- Are allergic to mice or hamster protein
- Are allergic to any ingredients in ADYNOVATE or ADVATE® [Antihemophilic Factor (Recombinant)]

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

How should I use ADYNOVATE?

ADYNOVATE is given directly into the bloodstream.

You may infuse ADYNOVATE 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 ADYNOVATE by themselves or with the help of a family member.

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

Reconstituted product (after mixing dry product with wet diluent) must be used within 3 hours and cannot be stored or refrigerated. Discard any ADYNOVATE left in the vial at the end of your infusion as directed by your healthcare professional.

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

How should I use ADYNOVATE? (cont'd)

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

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

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 ADYNOVATE passes into your milk and if it can harm your baby.
- Are pregnant or planning to become pregnant. It is not known if ADYNOVATE may harm your unborn baby.
- Have been told that you have inhibitors to factor VIII (because ADYNOVATE may not work for you).

What are the possible side effects of ADYNOVATE?

You can have an allergic reaction to ADYNOVATE.

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.

The common side effects of ADYNOVATE are headache and nausea. 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 ADYNOVATE. You can ask your healthcare provider for information that is written for healthcare professionals.

What else should I know about ADYNOVATE 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 ADYNOVATE 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 ADYNOVATE for a condition for which it is not prescribed. Do not share ADYNOVATE 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 ADYNOVATE. The FDA-approved product labeling can be found at www.shirecontent.com/PI/PDFs/ADYNOVATE_USA_ENG.pdf or 855-4-ADYNOVATE.

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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Kid's Corner

On the Road Again:
Travel advice to make your trip safe and fun!

Author: Heather Boerner

Planes, trains, cars and vans—there are lots of ways to travel these days! And all the things that keep you healthy and stop bleeds fast at home can come with you on your trip.

Here are some travel tips for making your next trip safe and fun:

Pre-Trip Checkup

Your friendly neighborhood nurse, doctor or physical therapist at your hemophilia treatment center (HTC) may want to see you before you go.

If you like to hop, skip, run and play, a physical therapist might ask a few questions about what kind of exercises you like. He can help you come up with active things to do while you're away. And if there's a pool in your hotel, swimming is great for joints! So listen close and remember your physical therapist's tips, even when you're daydreaming about your trip.

Power Packing

To keep boredom away, traveling long distances means packing things to keep you entertained, like your favorite toys, books, games, music and movies. It also means packing supplies that keep your body strong and help you heal from bleeds.

You can help your parents round up your usual supplies, putting the important stuff in your carry-on bag and extra supplies in your suitcase. Mom and Dad might have to order these supplies before the trip, so remind them, and know where in the carry-on bag your factor and supplies are stored.

Alert! Alert!

Travel can be tricky. That's why it's important to be prepared. If you have a medical alert bracelet, be sure to wear it before leaving home. And make sure Mom and Dad have your travel letter. It's written by your doctor, and describes your bleeding disorder and the treatment you need to keep you healthy. Your parents will probably present this to the airport or other security staff, so all your factor and supplies can come with you on the plane.

Have Fun

Now that you're packed, prepared and prepped, grab your camera and get ready for a super trip!

A Note from Outgoing Board Chair, Dale Gibbs

In 2014, I began my current service as a Chapter board member and at the end of 2018 I will end the past year as board President. This isn't the first time I have been on the board and I have to reflect back that the timelines couldn't be more different.

During the 1990's the Chapter was struggling with finances, membership and conflicting personalities. Since 2014, there have been difficulties but none have been as frustrating to me as those in the 1990's.
Just prior to beginning my board term, the Chapter had joined with NHF and things became better for all of us.

Over the years, the Chapter relied on industry financial support for almost it's entire budget but today, that financial support is a small part of our budget. That is a good thing too because industry financial support is being cut due to federal financial laws and regulations and other things. Because any non-profit organization has to have money, we have become our own fundraisers and very successfully too. This work also brings pride to us and more of a feeling that it is OUR Chapter.

During the 1990's the Chapter had no paid Executive Director and relied on Sharon and Carl Clark for administrative duties. Around 1997, through a small grant, the Chapter was able to hire someone as Director but we were pretty much flying blindly. Since merging with NHF, we have had a few issues such as three directors in two years but compared to before, the Chapter has become stronger with each change. Kristi Harvey-Simi led us from the merger and her skills made the Chapter stronger. Her skills were evident to NHF and she now oversees many of the merged Chapters for NHF, including ours. Maureen Grace, our current Executive Director has done an amazing job through a difficult time for herself and the Chapter by herself. The Chapter owes her an immense thank you for her work!

This past year's Chapter accomplishments have been very positive.

- Misti Mitchell was hired as the Development Manager and is a great addition with her background in non-profit fundraising. She will take us to a new level and free up Maureen for other work. Their combined work will drive us to do more and take the Chapter to another level.
- As a result of a townhall meeting held in 2017, The Chapter has organized committees to involve more members and do more than relying on Maureen and Misti to do everything. Membership is open to all Chapter members and the committees, in addition to doing more work for the Chapter, are designed to be transparent and more responsive to members. Please consider participating in one or more committees.
    - The Governance Committee has had one meeting and is working on making the Chapter events, meetings and operations better.
    - The Advocacy Committee has had two meetings and is working on representing you and the Chapter to the Nebraska Legislature, state agencies responsible for rules and regulations that impact us and our Nebraska Senators and Congressmen. You may have noticed that we have an Advocacy article in every newsletter and at times ask for your assistance for specific advocacy issues.
    - The Events, and Education and Programming Committees have yet to be fully developed and we definitely would like your participation.

In December, we began working on the strategic plan for the Chapter. This will be about a nine month process but when done will give us a direction and road markers to our future. We have been lacking in doing this on a regular basis but now that we feel the Chapter has a strong operation and leadership we want to develop the plan.

The Chapter board has been instrumental in all of our accomplishments this past year and has a vision that will make the Chapter stronger. We would welcome others involvement with us so please consider becoming a member.

I hope you feel good about where the Chapter is and going and urge you to keep in touch with us. The more we are all on the same path, the stronger we are.

Be a Part of Making Tough Decisions

Steve Place

I have lived for 64 years with mild hemophilia and have voluminous experience, from which I came to my own rock-solid conclusions about how someone with a bleeding disorder can participate in sports and other potentially life-changing activities.

One experience was a significant life change at age 10, when I sustained a serious head injury. I felt able to ride my bike with no hands. Sand and a quick moment of unbalance tossed me off my bike, and my head hit the pavement hard.

I got myself up and walked back home with my bike. My sister saw me and screamed. That's when I felt a huge lump on my forehead and knew I was in trouble. Our doctor, who made house calls, wrapped my head with a pressure bandage and told my parents to give me two aspirin every four hours and added that if I made it through the night, I probably would be all right. Aspirin and all, I survived.

All of a sudden, I was prohibited from participating in contact sports. This is tough for a 10-year-old boy. Although I was always the smallest kid in my class and the last to be chosen at sports, it still hurt. Fortunately, my mom and dad and sister were very supportive, and we got through it together. They steered me to other avenues that led me to a happy, healthy, and productive life. I thank God every day for my hemophilia!

I look back and wish I could have accepted at age 10 what I strongly believe about my bleeding disorder today. I went from "Boo-hoo, I can't do certain things!" to "Drop back, punt, and come up with a new plan."

Now that may sound a bit harsh, but the sooner we accept our limitations in life and pursue the best and safest path, the better off our lives and our families' lives will be. Yes, we must think about our families, too; it's not all about the person with hemophilia. Every person with a bleeding disorder has affected and will continue to affect the lives of those we love most. Our bleeds seem to come at the most inconvenient times, for us and for them!

It was traumatic when at age 10, I had to stop doing the things my buddies were doing. But here I am today, happy, married for 40 years with two daughters, successful, and in excellent health. I am physically active in my daily pursuits. I am a professional handyman and sole proprietor, and I work daily with all types of sharp tools, both power and manual. Safety and thinking through a job are paramount. Knee pads and elbow/forearm protection are vitally important. The most dangerous tool in my toolbox is a dull blade.

I treat on demand and prior to some potential bleeding situations. I have 95% mobility in all of my joints. The only time I infuse, apart from surgery, is when I make a mistake.

We all want to be the best parents we can be for our kids. Good, tough prodding and steering today can result in a wonderful life later on for them. I made a very strong, positive personal decision that has guided my life for the last 50-plus years. I decided that I will respect my disorder, but I will not be afraid of it. I will determine what I will do, and what I will not do. I basically took charge of my life.

Stephen is 64 and has been married for 40 years. He has two adult daughters, and works 50 hours a week. He is active in his church, both teaching and leading. He believes that life is great, especially when "I respect my disease, but am not afraid of it." scplace122@comcast.net

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