WINTER 2012

Rocky Mountain Hemophilia & Bleeding Disorders Association

RMHBDA Newsletter

The Rocky Mountain

RMHBDA is a 501(c)(3) nonprofit organization founded in 2000 and is a chartered chapter of the National Hemophilia Foundation.

Our mission is to improve the quality of care and life for persons with inherited bleeding disorders, including hemophilia and von Willebrand Disease through education, peer support, resources, and referral.

In This Issue

Rocky Mountain Hemophilia & Bleeding Disorders Association

2100 Fairway Drive, Suite 107
Bozeman, Montana 59715-5815
406.586.4050

www.rockymountainhemophilia.org

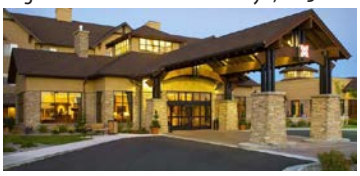
Brad Benne, *Executive Director*
brad.rmhbda@gmail.com
cell 406.600.2554

www.facebook.com/rmhbda

RMHBDA Education Weekend 2013

February 22–24

Registration deadline is February 1, 2013!



Event will be at Hilton Garden Inn, Bozeman, 2023 Commerce Way *and hosted by* Rocky Mountain Hemophilia *and* University of Colorado Hemophilia &Thrombosis Center

It's time for the ninth Annual Education Weekend for people affected by bleeding disorders in Montana and Wyoming! You and your family are invited for a weekend of informative sessions, youth programming for all ages, and an opportunity to connect with others dealing with similar challenges. This education weekend and annual meeting of RMHBDA is designed to bring you education, up-to-date information about life with a bleeding disorder, and connect you with other families in our two-state area. Check-in for the event will be Friday, February 22 from 4-6pm, followed by a chapter welcome and Pizza Party and Program at Old Chicago.

Continued on page 3

Family Camp 2013

June 14 - 16

Each summer, RMHBDA invites affected families living in Montana and Wyoming to attend a weekend retreat. The weekend is packed full of education, bonding, and fun!

Luccock Park Camp, Livingston, MT (Paradise Valley) For more info, visit www.luccock.org/

For the parents and teens, we will have teambuilding programming led by our

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From Our Executive Director

A Courageous Journey

By Brad Benne, Executive Director

Mark Twain once wrote, "Courage is resistance to fear, mastery of fear, not absence of fear." That quote best describes our chapter and how we approached our first walk along with the many other obstacles we face relating to bleeding disorders here in Montana and Wyoming. Before I was involved in a leadership position with our chapter, our Board of Directors and previous Executive Director made a brave decision to participate in the NHF Walk program.

Although each chapter has unique challenges, our greatest challenge begins with location. We encompass all of Montana and Wyoming and many of our members travel great distances to receive adequate medical care and to attend chapter events. For many of us, our closest Hemophilia Treatment Center is in Aurora, Colorado. As you can imagine, due to the lack of access to quality care, families here quickly become a very close knit group with strong voices of support and advocacy for one another. Fortunately, our largest obstacle also brings us closer together.

As a chapter, we experienced some resistance and doubts as we moved forward with planning our walk. We knew very little about organizing a walk, and just like any new endeavor for any organization, big or small, the idea of pulling off such an event successfully, frightened us. Considerable effort had been put into planning previous fundraisers, but results never yielded sustaining results to support and maintain our chapter's financial stability. At one point, our chapter even waivered and canceled our walk seven months before our event. But after our annual meeting, we rallied and restored plans for our first walk because we knew the future of our chapter depended on the walk.

Raising $50,000 and rallying a hundred walkers was a daunting and overwhelming goal for our organization. The support and guidance from NHF staff and other hemophilia chapters who willingly shared their ideas and experiences with us helped our chapter create a plan. With that said, our biggest challenge remained because we are by far one of the smallest and logistically challenged chapters to participate in the walk program. Although we followed the recommendations of the NHF staff, in some cases we had to adjust our game plan. For example, we combined our "Kickoff" and "Call to Action" meetings and included an educational session and free dinner.

We were thrilled to have over a hundred walkers and raise $27,500 as a chapter in our first walk! The walk also helped our chapter by making bleeding disorders more visible in our community. Hopefully, as the walk grows we will continue to spread awareness to every corner of Montana and Wyoming. We

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NHF Walk Turns Five Years Old

Reprinted with permission of NHF

It has been five years since the National Hemophilia Foundation (NHF) launched its Hemophilia Walk program, starting with five walks, held in New York City, Phoenix, Cleveland, Raleigh and Houston. During the first Walk season, about 2,700 walkers, and national and local sponsors raised more than $1 million in support of their chapters. It was a promising beginning.

Since then, the Walk program has been growing at an impressive rate. This year, chapters across the US will host 23 Walks. At press time, 8,000 people had registered to walk, and the chapters had raised $1.3 million.

Brad Benne, executive director of the Rocky Mountain Hemophilia & Bleeding Disorders Association in Bozeman, Montana, says his chapter has experienced challenges similar to the Lone Star Chapter. His chapter encompasses all of Montana and Wyoming; many members travel long distances for fundraising and educational events.

"We are small, but mighty," Benne says. The chapter held its first Walk on September 8, 2012. At first, Benne was somewhat apprehensive about the small chapter's ability to generate enough interest and volunteers to plan and participate in a Walk. Then he realized the potential benefits were too great to ignore. "Our board of directors saw it as another way for our chapter to sustain itself," he says.

The Rocky Mountain chapter has held successful fundraisers before but nothing on the scale of the Walk. "Most of our fundraisers bring in about $2,000," he says. "If our Walk raises $20,000, that will be a huge success for us." The chapter, in fact, raised more than $25,000.

Walks bring more than money to the chapters. They can add members, too. "Walks are a great way for families to get more involved with our chapter," Compton says. Families with newly diagnosed children can feel overwhelmed at educational events, but Walks allow them to participate, she says. In addition, kids with bleeding disorders meet each other and families bond. "New families have gotten to know us through our Walks and then will start coming to our other events," says Compton.

Walks also help make bleeding disorders more visible in the community. "My goal of the Walk is to spread awareness of bleeding disorders to every corner of Montana and Wyoming," Benne says. The reasons are practical as well as aspirational. For many chapter members, the hemophilia treatment center in Denver, Colorado, is an 11-hour drive. "People wind up going to local hospitals and emergency rooms that aren't as knowledgeable as they could be about bleeding disorders," Benne says. "The Walk is a great opportunity for our chapter and community to get their stories out there."

The Walks are supported by National Presenting Sponsor Baxter, Pacesetter Sponsor Bayer HealthCare, Official Sponsors Pfizer Hemophilia and Grifols, and Supporting Sponsor Biogen Idec Hemophilia.

For more information and to find a Walk in your area, go to: hemophilia.org/walk.

2013 Save the Date
September 7
Bozeman

National Hemophilia Foundation and Rocky Mountain Hemophilia and Bleeding Disorders Association Present

HEMOPHILIA walk℠

Every step makes a difference

See many more and larger versions at

www.facebook.com/rmhbda.

From page 1: **Family Camp 2013**

guest, hemophilia leadership expert, Pat Torrey and some time to relax with other families. This is a great opportunity to learn from and share experiences with one another.

We also have many great activities planned for our campers including arts & crafts projects, field games, and educational sessions for children with bleeding disorders and their siblings. Infusion classes will be offered from HTC RN, Sue Geraghty.

Call Brad with any questions at 406.600.2554

We need help organizing!

If you are interested in serving on the Education Weekend committee, please contact Brad at 406.600.2554.

This is your organization!

2013 Mile High Colorado Camp

Save The Date!
July 14–19, 2013

Leadership Pre-Camp Retreat
July 12–14, 2013

Forms will be available in mid-March 2013! Stay Tuned!

The Hemophilia and Thrombosis Center (HTC) is proud to once again sponsor the summer camp program at Rocky Mountain Village.

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RMHBDA Welcomes Dr. Carrie Laborde to Billing's St. Vincent Healthcare

Dr. Carrie Laborde was raised in New Orleans, Louisiana. She attended the University of New Orleans for undergraduate and Louisiana State University Medical School in New Orleans and completed her Pediatric Internship and Residency at Vanderbilt Medical Center in Nashville, Tennessee. Dr. Laborde then completed her Pediatric Hematology and Oncology fellowship at Children's Medical Center, University of Texas Southwestern Medical Center in Dallas, Texas. And now we welcome her to St. Vincent Healthcare at the Children's Cancer and Blood Disorders clinic.

From page 1: **Education Weekend 2013**

On Saturday morning, we will cover topics like Inhibitors, Von Willebrand's Disease Basics, Healthcare Reform, Venipuncture, Hemophilia Basics, and Joint Health.

For our youth, we will have a variety of programming available. Please pack your life-jackets for pool time in the afternoon.

Don't miss your chapter's annual meeting on Saturday for all members; important decisions will be made at this meeting and your input is needed! Lodging and meals will be provided to attending members, so don't hesitate to send your registration today! Don't miss this opportunity with your chapter, industries, HTC staff, accredited speakers, and your family — a special and rewarding weekend for all.

Need assistance to attend Education Weekend?

RMHBDA will provide Patient Assistance applications in all registration packets, please save all gas, food, and travel expense receipts! If you have any questions, please call Brad Benne 406-586-4050.

Tentative Schedule

Friday, February 22

4:00-6:00 PM	Registration & exhibits
6:00-6:15 PM	Welcome
6:15-7:30 PM	Dinner & Program, *Sponsored by CSL Behring*

Saturday, February 23

7:00-8:00 AM	Breakfast
8:00-10:30 AM	Sessions
10:30-12:00 AM	Exhibits/Break
12:00-1:00 PM	Lunch
1:00-3:00 PM	Annual Meeting/BOD Development
3:00-7:00 PM	Bowling/Free Time
6:00 PM	Dinner & Program, *Sponsored by Baxter Healthcare*

Sunday, February 24

7:00-8:00 AM	Breakfast
8:00-11:30 AM	Sessions
11:30 AM	Check out/Good Byes

Volunteers

If you are interested in volunteering to help plan this Education Day please, contact Brad Benne at 406.586.4050.

Bayer Leadership Program

Apply to be an intern through the Bayer Hemophilia Leadership Development Program (BHLDP) and experience a unique opportunity to build foundational leadership skills AND deepen your connection to the hemophilia community!

Bayer is looking for students enrolled full-time in college who are touched by hemophilia and have a strong interest in and commitment to becoming a future leader in the hemophilia community. Interns will travel to Bayer's U.S. headquarters in New Jersey, where they will spend eight weeks participating in activities that aim to help them grow personally and professionally.

Through the program, selected interns will have the opportunity to:

- Participate in formal training on communication skills, effective problem solving, leadership and compliance
- Spend time working with mentors within Bayer to gain insights into the inner workings of the various corporate departments–emphasizing the importance of collaboration
- Work with hemophilia organizations in the area to learn about the work done to support the hemophilia community and how business professionals can support these efforts
- Engage in hands on projects that will build on the skills developed during the program

The program will also include a meeting with healthcare public policy professionals in Washington D.C. where interns will see first-hand how effective advocacy relations impacts legislative decisions.

This is an eight-week, paid internship program (June through August 2013). All housing, travel and related activities will be organized and underwritten by Bayer.

Interns will be selected based on an application form and interview process. Applications are due no later than Friday, February 8, 2013, at 11:59 PM ET.

Novo Nordisk Announces U.S. Results
of Largest Multinational Psychosocial Study of Living with Hemophilia

November 7, 2012 — Orlando, Fla. — U.S. patient and caregiver results derived from the largest multinational psychosocial study of the hemophilia community are being presented at the National Hemophilia Foundation (NHF) 64th Annual Meeting. In total, four posters were accepted from the Hemophilia Experiences, Results and Opportunities (HERO) study, a comprehensive analysis of the experience of living with hemophilia. One of the posters will be featured in an oral presentation at the meeting.

HERO examined the disorder's effect on interpersonal relationships, careers, access to care and quality of life. The HERO initiative aims to improve outcomes in hemophilia by calling for and enabling enhanced psychosocial support based on increased understanding and awareness of the issues.

"We worked with key physicians and advocacy groups to develop a comprehensive study that, for the first time, examines the impact of living with hemophilia on all aspects of a family's life, including interpersonal relationships, access to care, employment, and the quality of life," said Robert Gut, MD, PhD, VP Clinical Development & Medical Affairs Biopharmaceuticals, Novo Nordisk.

In the United States, 189 adults with hemophilia and 190 parents of children with hemophilia participated in the quantitative arm of the HERO research.

Read the complete press release at http://goo.gl/jHV7F

Biogen Idec and Sobi Announce Study Results
Positive Top-Line Results From Phase 3 Study Investigating Long-Lasting Recombinant Factor VIII Fc Fusion Protein In Hemophilia A

- Individualized and weekly prophylactic regimens resulted in low single-digit median annualized bleeding rates
- 98% of bleeding episodes were controlled with one or two injections of rFVIIIFc
- No patients developed inhibitors to rFVIIIFc
- The primary efficacy and safety objectives were met and Biogen Idec plans to submit an application to US FDA in first half 2013

Weston, Mass. & Stockholm, Sweden — Oct. 31, 2012 — Biogen Idec and (Sobi) today announced positive results from A-LONG, a clinical study that evaluated a new long-lasting clotting factor candidate in people with hemophilia A. Hemophilia A is a rare inherited disorder that impairs blood coagulation. Top-line results from A-LONG, a global, multi-center, Phase 3 clinical study of the companies' long-lasting recombinant Factor VIII Fc fusion protein (rFVIIIFc), showed that rFVIIIFc was effective in the control and prevention of bleeding, routine prophylaxis and perioperative management. Recombinant FVIIIFc was generally well-tolerated. Additional analyses of the A-LONG study are ongoing, and the companies anticipate presenting detailed results at a future scientific meeting.

Biogen Idec plans to submit a Biologics License Application (BLA) to the U.S. Food and Drug Administration (FDA) in the first half of 2013. Consistent with guidelines published by theEuropean Medicines Agency (EMA) that require a study in children less than 12 years of age prior to filing, Biogen Idec and Sobi expect to file a Marketing Authorization Application with the EMA upon completion of the ongoing Kids A-LONG study.

Read the complete press release at http://goo.gl/T9j3E

We Love Donations!

RMHBDA is a 501 (c)(3) nonprofit organization which means that contributions are tax deductible; check with your tax professional to determine how this specifically affects you. We appreciate your consideration. An envelope is enclosed for your convenience.

Rocky Mountain Hemophilia

& Bleeding Disorders Association

2013 Calendar of Events & Programs

Tentative

The chapter is still determining exact dates for several programs and events for the community.

January 2013
13–15 NHF National Walk Training
24–26 NACCHO Camp Conference

February 2013
22–24 RMHBDA Education Weekend in Bozeman
27–3/1 NHF Washington Days

March 2013
Hemophilia Awareness Month!

April 2013
17 World Hemophilia Day:
25–27 HFA Annual Symposium

May 2013
14–15 Walk "Call to Action" Meeting (Bozeman & Billings)

June 2013
14–16 RMHBDA Family Camp (Luccock Camp Park, Livingston, MT)

July 2013
12–14 Mile High Summer Camp Leadership Pre-camp Retreat
TBA Minor League Baseball Night: Cheyenne, Missoula &Billings (Pending Funding)
14 – 19 Mile High Summer Camp (Rocky Mountain Village, Empire CO)

August 2013
17–18 Walk Kickoff Event (Bozeman & Billings)

September 2013
TBA Walk for Hemophilia, Bozeman (Pending MSU Football sched & park availability)

October 2013
3–5 NHF Annual Meeting (Anaheim)
11–13 TBA Men's Retreat (West Yellowstone) (Pending Funding)

December 2013:
4–5 RMHBDA Holiday Party (Bozeman & Billings)

From page 1: **From Our Executive Director: A Courageous Journey**

also raised nearly $10,000 in corporate donations, thanks to generous support from the pharmaceutical industry. We did step outside the box by raising $3,000 of sponsorship from local businesses not directly related to the bleeding disorder community. In addition, we were fortunate to have Sara Jestrab, a Montana State University student and NYLI participant, serve as a summer intern. Sara helped us spread the word of bleeding disorders and our walk in local newspapers, radio public service announcements, and Facebook. In addition to a stunningly beautiful fall day in Montana, with spectacular mountain scenery, we created a festive event by incorporated live music, face painting, super heroes, a duck race, and lunch for all walkers and volunteers!

Although we still have a lot of work to do as a chapter, and the year was full of numerous challenges, the rewards were much greater! Through media exposure, we were able to spread awareness of bleeding disorders in our communities. Even the majority of our volunteers and sponsors knew very little about bleeding disorders. The money we raised has created financial stability to expand our programming and replenish our financial assistance program. Our chapter members have always been highly involved with our programs, but the walk created a renewed sense of pride and enthusiasm among our chapter members and volunteers. The energy and hard work our chapter poured into making our first walk a huge success was a defining moment for our chapter - a moment as a member and the director that I am deeply proud to have played a role.

We are excited for our 2nd Annual Walk on September 7, 2013 in Bozeman, Montana. We believe our walk will continue to grow in spreading awareness and raising money to sustain and improve our chapter for years to come.

BAYER HEALTHCARE AND THE HEMOPHILIA COMMUNITY:

Commitment, Leadership *and* Innovation

Bayer HealthCare

Printed in USA KN10001111

From page 3: **2013 Mile High Colorado Camp**

Who Should Attend?

- Children with hemophilia or other bleeding disorders
- Siblings of the above groups

Mile High Colorado Camp is for ages 7–18. We accept 6-year-olds on a case by case basis. Programming is determined by age. Check back with us to learn about the different programs we offer at camp!

Why Attend Camp?

The purpose of camp is to learn about bleeding disorders, develop skills and have fun! Campers will have the opportunity to meet new friends and participate in a variety of traditional camp activities. As always, we have included educational components with the goal of encouraging self-confidence and independence.

Many campers have learned to perform self-infusion, experienced teamwork, and discovered new skills during the week of camp. Staff at the Hemophilia & Thrombosis Center(HTC) and Rocky Mountain Village wants this to be a wonderful experience that creates a wealth of fond memories for your camper.

What Does It Cost?

Each family is required to pay a non-refundable $75.00 deposit. The remainder of the camp cost, approximately $1000.00 per camper, is underwritten by other sources. If you have questions or need additional information, please call Brad Benne at 406.600.2554. Scholarship forms are available. Scholarships will be granted on an individual basis..

Yunnan Paiyao

Reference: A Tooth From The Tiger's Mouth

Yunnan paiyao is the best all-around formula to stop traumatic bleeding and prevent infection. It is a must for your medicine cabinet or first-aid kit. The ingredients that make up *Yunnan paiyao* were a secret, kept by only one family of doctors. The Communist government forced the family to give up the formula, and it became the property of the state, which made it available cheaply in a variety of forms. *Yunnan paiyao* was part of China's aid to Vietnam during the Vietnam War owing to its effectiveness in treating battlefield injuries.

Yunnan paiyao both stops bleeding and removes blood clots. These two seemingly contradictory actions are what make it so suitable for healing wounds. As the bleeding stops, blood congeals in the wound. This congealed blood sometimes blocks normal circulation and prevents the wound from healing properly. *Yunnan paiyao* stops the bleeding but simultaneously encourages normal circulation, removing pus and congealed blood, which can prevent the flesh from being properly nourished. This helps the flesh to regenerate with minimal scarring. *Yunnan paiyao* is a prepackaged "patent remedy" that comes in two forms:

- In a small box containing a vial of white powder. A small red pill (see "The Red Pill") is wrapped in cotton waddling. The powder is easier to apply externally.
- In a box containing blister packs of 16 capsules each with 1 red pill per blister pack. The capsules are easier to take internally, although they can be broken open so the powder inside can be applied directly on the wound.

Yunnan paiyao can be taken internally if there is suspicion of internal hemorrhage or to prevent and stop infection. In these cases, ¼ teaspoon of powder or 2–3 capsules can be taken with warm water twice a day. This can be useful for head injuries where there may be bruising or bleeding of the brain. In these cases, it is very important to stop any bleeding that may be occurring, while preventing the formation of blood clots that may cause problems later. In instances where there is suspicion of concussion of internal bleeding, take *Yunnan paiyao* and go to the hospital immediately.

For bruises and contusions, sprains, and strains, where there is no hemorrhage or concussion, *Yunnan paiyao* is taken with alcohol (vodka or rice wine). In Chinese sports, medicine alcohol is said to "course the channels" by dilating the blood vessels and pushing energy through meridians. This accentuates *Yunnan paiyao's* property of dispersing stagnant blood. In cases of hemorrhage or concussion, *Yunnan paiyao* should not be taken with alcohol.

The Red Pill

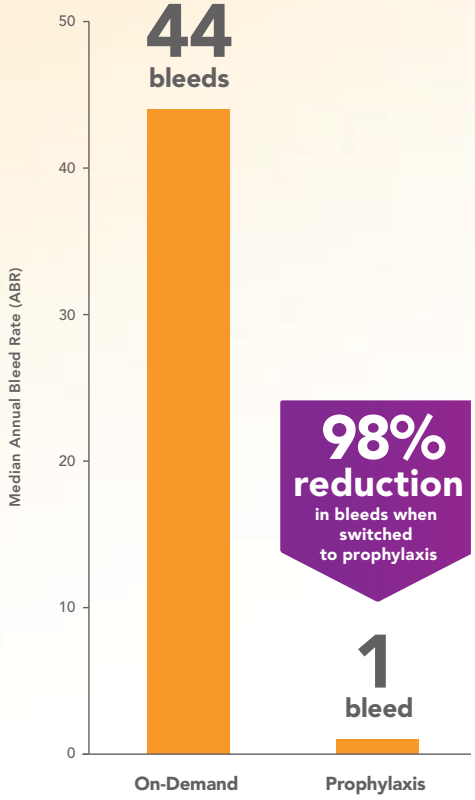
The red pill that accompanies each blister pack of capsules and each vial of powder is to be taken only for serious injuries such as stab or gunshot wounds. For this purpose, it is taken with strong rice wine or vodka. I cannot say that I have used the red pill, but martial arts practitioners in China have reported using both it and the powder to heal these kind of wounds without recourse to antibiotics. I am not recommending that you treat your gunshot or stab wounds yourself, but having access to medicines that could save your life is always useful.

Prophylaxis with ADVATE delivers 44 to 1[1]

ADVATE IS THE **ONLY** RECOMBINANT FACTOR VIII (EIGHT) THAT IS

PROPHYLAXIS WITH ADVATE

THE POWER TO REDUCE YOUR ANNUAL BLEED RATE (ABR)



Significant reduction in ABR[1]

After switching from 6 months of on-demand treatment to 12 months of prophylaxis with ADVATE in 53 previously treated patients with severe or moderately severe hemophilia A:

- **Median ABR of 1** while on either prophylaxis regimen[1]
  - prophylaxis every second day (20-40 IU/kg)
  - prophylaxis every third day (20-80 IU/kg, targeted to maintain FVIII trough levels ≥1%)
- **42% of patients experienced zero bleeds** during 1 year on prophylaxis[1]
- **No subject developed factor VIII inhibitors** or withdrew due to an adverse event (AE)[4]

Indication for ADVATE

ADVATE [Antihemophilic Factor (Recombinant), Plasma/Albumin-Free Method] is a medicine used to replace clotting factor VIII that is missing in people with hemophilia A (also called "classic" hemophilia). ADVATE is used to prevent and control bleeding in people with hemophilia A. Your healthcare provider may give you ADVATE when you have surgery.

ADVATE is not used to treat von Willebrand Disease.

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.

References: 1. ADVATE prescribing information. Westlake Village, CA: Baxter Healthcare Corporation; December 2011. **2.** Helixate FS prescribing information. Kankakee, IL: CSL Behring LLC; August 2009. **3.** Kogenate FS prescribing information. Tarrytown, NY: Bayer Healthcare LLC; March 2011. **4.** Valentino LA, Mamonov V, Hellmann A, et al. A randomized comparison of two prophylaxis regimens and a paired comparison of on-demand and prophylaxis treatments in hemophilia A management. *J Thromb Haemost.* 2012;10(3):359-367. **5.** Maruish ME, ed. *User's Manual for the SF-36v2 Health Survey.* 3rd ed. Lincoln, RI: QualityMetric Incorporated; 2011.

TALK TO YOUR HEALTHCARE PROVIDER TO SEE HOW PROPHYLAXIS

FDA APPROVED FOR PROPHYLAXIS IN BOTH **ADULTS & CHILDREN (0-16 YEARS)**[1-3]

PROPHYLAXIS WITH ADVATE

THE POWER TO IMPROVE YOUR PHYSICAL HEALTH–RELATED QUALITY OF LIFE

Physical Component Score
Improvements in physical functioning, well-being, and energy level.[5]

Bodily Pain
Reduced levels of pain and improvements in the limitations of work due to pain.[5]

Role Physical
Improvements in the ability to perform physical activities without limitations due to health.[5]

Clinically meaningful improvements

After 12 months of prophylactic treatment, physical health–related quality of life improved in patients, mainly due to clinically meaningful improvements in*:

- the amount of pain experienced by a patient and how much pain interferes with normal work
- the impact physical health can have on performing work or other daily activities

*Clinically significant changes were not seen in the physical health–related sub-categories of General Health and Physical Functioning and the mental health–related component score and sub-categories of Mental Health, Role Emotional, Social Functioning, and Vitality.

Detailed Important Risk Information for ADVATE

You should not use ADVATE if you are allergic to mice or hamsters or any ingredients in ADVATE.

You should tell your healthcare provider if you have or have had any medical problems, take any medicines, including prescription and non-prescription medicines and dietary supplements, have any allergies, including allergies to mice or hamsters, are nursing, are pregnant, or have been told that you have inhibitors to factor VIII.

You can have an allergic reaction to ADVATE. Call your healthcare provider right away and stop treatment if you get a rash or hives, itching, tightness of the throat, chest pain or tightness, difficulty breathing, lightheadedness, dizziness, nausea, or fainting.

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

Side effects that have been reported with ADVATE include: cough, sore throat, unusual taste, abdominal pain, diarrhea, nausea/vomiting, headache, fever, dizziness, hot flashes, chills, sweating, joint swelling/aching, itching, hematoma, swelling of legs, runny nose/congestion, and rash.

Call your healthcare provider right away about any side effects that bother you or if your bleeding does not stop after taking ADVATE.

Please see Brief Summary of ADVATE Prescribing Information on the next page.

ADVATE
[Antihemophilic Factor (Recombinant), Plasma/Albumin-Free Method]

There's more to life.

advate.com | 888.4.ADVATE

WITH ADVATE CAN HELP REDUCE YOUR ANNUAL BLEED RATE (ABR)

ADVATE
[Antihemophilic Factor (Recombinant), Plasma/Albumin-Free Method]
Brief Summary of Prescribing Information. Please see package insert for full prescribing information.

INDICATIONS AND USAGE

Control and Prevention of Bleeding Episodes

ADVATE [Antihemophilic Factor (Recombinant), Plasma/Albumin-Free Method] is an Antihemophilic Factor (Recombinant) indicated for control and prevention of bleeding episodes in adults and children (0-16 years) with Hemophilia A.

Perioperative Management

ADVATE is indicated in the perioperative management in adults and children (0-16 years) with Hemophilia A.

Routine Prophylaxis

ADVATE is indicated for routine prophylaxis to prevent or reduce the frequency of bleeding episodes in adults and children (0-16 years) with Hemophilia A.

ADVATE is not indicated for the treatment of von Willebrand disease.

CONTRAINDICATIONS

Known anaphylaxis to mouse or hamster protein or other constituents of the product.

WARNINGS AND PRECAUTIONS

Anaphylaxis and Hypersensitivity Reactions

Allergic-type hypersensitivity reactions, including anaphylaxis, are possible and have been reported with ADVATE. Symptoms have manifested as dizziness, paresthesias, rash, flushing, face swelling, urticaria, dyspnea, and pruritus. [See Patient Counseling Information (17) in full prescribing informaiton]

ADVATE contains trace amounts of mouse immunoglobulin G (MulgG): maximum of 0.1 ng/IU ADVATE and hamster proteins: maximum of 1.5 ng/IU ADVATE. Patients treated with this product may develop hypersensitivity to these non-human mammalian proteins.

Discontinue ADVATE if hypersensitivity symptoms occur and administer appropriate emergency treatment.

Neutralizing Antibodies

Carefully monitor patients treated with AHF products for the development of Factor VIII inhibitors by appropriate clinical observations and laboratory tests. Inhibitors have been reported following administration of ADVATE predominantly in previously untreated patients (PUPs) and previously minimally treated patients (MTPs). If expected plasma Factor VIII activity levels are not attained, or if bleeding is not controlled with an expected dose, perform an assay that measures Factor VIII inhibitor concentration. [See Warnings and Precautions, Monitoring Laboratory Tests]

Monitoring Laboratory Tests

The clinical response to ADVATE may vary. If bleeding is not controlled with the recommended dose, determine the plasma level of Factor VIII and administer a sufficient dose of ADVATE to achieve a satisfactory clinical response. If the patient's plasma Factor VIII level fails to increase as expected or if bleeding is not controlled after the expected dose, suspect the presence of an inhibitor (neutralizing antibodies) and perform appropriate tests as follows:

- Monitor plasma Factor VIII activity levels by the one-stage clotting assay to confirm the adequate Factor VIII levels have been achieved and maintained when clinically indicated. [See Dosage and Administration (2) in full prescribing information]
- Perform the Bethesda assay to determine if Factor VIII inhibitor is present. If expected Factor VIII activity plasma levels are not attained, or if bleeding is not controlled with the expected dose of ADVATE, use Bethesda Units (BU) to titer inhibitors.
  - If the inhibitor titer is less than 10 BU per mL, the administration of additional Antihemophilic Factor concentrate may neutralize the inhibitor and may permit an appropriate hemostatic response.
  - If the inhibitor titer is above 10 BU per mL, adequate hemostasis may not be achieved. The inhibitor titer may rise following ADVATE infusion as a result of an anamnestic response to Factor VIII. The treatment or prevention of bleeding in such patients requires the use of alternative therapeutic approaches and agents.

ADVERSE REACTIONS

The serious adverse drug reactions (ADRs) seen with ADVATE are hypersensitivity reactions and the development of high-titer inhibitors necessitating alternative treatments to Factor VIII.

The most common ADRs observed in clinical trials (frequency ≥ 10% of subjects) were pyrexia, headache, cough, nasopharyngitis, vomiting, arthralgia, and limb injury.

Clinical Trial Experience

Because clinical trials are conducted under widely varying conditions, adverse reaction rates observed in the clinical trials of a drug cannot be directly compared to rates in clinical trials of another drug and may not reflect the rates observed in clinical practice.

ADVATE has been evaluated in five completed studies in previously treated patients (PTPs) and one ongoing study in previously untreated patients (PUPs) with severe to moderately severe Hemophilia A (Factor VIII ≤ 2% of normal). A total of 234 subjects have been treated with ADVATE as of March 2006. Total exposure to ADVATE was 44,926 infusions. The median duration of participation per subject was 370.5 (range: 1 to 1,256) days and the median number of exposure days to ADVATE per subject was 128.0 (range: 1 to 598).[1]

The summary of adverse reactions (ADRs) with a frequency ≥ 5% (defined as adverse events occurring within 24 hours of infusion or any event causally related occurring within study period) is shown in Table 1.

No subject was withdrawn from a study due to an ADR. There were no deaths in any of the clinical studies.

IMMUNOGENICITY

The development of Factor VIII inhibitors with the use of ADVATE was evaluated in clinical studies with pediatric PTPs (< 6 years of age with > 50 Factor VIII exposures) and PTPs (≥ 10 years of age with > 150 Factor VIII exposures). Of 198 subjects who were treated for at least 10 exposure days or on study for a minimum of 120 days, 1 adult developed a low-titer inhibitor (2.0 [BU] in the Bethesda assay) after 26 exposure days. Eight weeks later, the inhibitor was no longer detectable, and *in vivo* recovery was normal at 1 and 3 hours after infusion of another marketed recombinant Factor VIII concentrate. This single event results in a Factor VIII inhibitor frequency in PTPs of 0.51% (95% CI of 0.03 and 2.91% for the risk of any Factor VIII inhibitor development).[1,2] No Factor VIII inhibitors were detected in the 53 treated pediatric PTPs.

In clinical studies that enrolled previously untreated subjects (defined as having had up to 3 exposures to a Factor VIII product at the time of enrollment), 5 (20%) of 25 subjects who received ADVATE developed inhibitors to Factor VIII.[1] Four patients developed high titer (> 5 BU) and one patient developed low-titer inhibitors. Inhibitors were detected at a median of 11 exposure days (range 7 to 13 exposure days) to investigational product.

Immunogenicity also was evaluated by measuring the development of antibodies to heterologous proteins. 182 treated subjects were assessed for anti-Chinese hamster ovary (CHO) cell protein antibodies. Of these patients, 3 showed an upward trend in antibody titer over time and 4 showed repeated but transient elevations of antibodies. 182 treated subjects were assessed for mulgG protein antibodies. Of these, 10 showed an upward trend in anti-mulgG antibody titer over time and 2 showed repeated but transient elevations of antibodies. Four subjects who demonstrated antibody elevations reported isolated events of urticaria, pruritus, rash, and slightly elevated eosinophil counts. All of these subjects had numerous repeat exposures to the study product without recurrence of the events and a causal relationship between the antibody findings and these clinical events has not been established.

Of the 181 subjects who were treated and assessed for the presence of anti-human von Willebrand Factor (VWF) antibodies, none displayed laboratory evidence indicative of a positive serologic response.

Post-Marketing Experience

The following adverse reactions have been identified during post-approval use of ADVATE. Because these reactions are reported voluntarily from a population of uncertain size, it is not always possible to reliably estimate their frequency or establish a causal relationship to drug exposure.

Among patients treated with ADVATE, cases of serious allergic/hypersensitivity reactions including anaphylaxis have been reported and Factor VIII inhibitor formation (observed predominantly in PUPs). Table 2 represents the most frequently reported post-marketing adverse reactions as MedDRA Preferred Terms.

Table 1
Summary of Adverse Reactions (ADRs)[a] with a Frequency ≥ 5% in 234 Treated Subjects[b]

MedDRA[c] System Organ Class	MedDRA Preferred Term	Number of ADRs	Number of Subjects	Percent of Subjects
General disorders and administration site conditions	Pyrexia	78	50	21
Nervous system disorders	Headache	104	49	21
Respiratory, thoracic and mediastinal disorders	Cough	75	44	19
Infections and infestations	Nasopharyngitis	61	40	17
Gastrointestinal disorders	Vomiting	35	27	12
Musculoskeletal and connective tissue disorders	Arthralgia	44	27	12
Injury, poisoning and procedural complications	Limb injury	55	24	10
Infections and infestations	Upper respiratory tract infection	24	20	9
Respiratory, thoracic and mediastinal disorders	Pharyngolaryngeal pain	23	20	9
Respiratory, thoracic and mediastinal disorders	Nasal congestion	24	19	8
Gastrointestinal disorders	Diarrhea	24	18	8
Gastrointestinal disorders	Nausea	21	17	8
General disorders and administration site conditions	Pain	19	17	8
Skin and subcutaneous tissue disorders	Rash	16	13	6
Infections and infestations	Ear infection	16	12	5
Injury, poisoning and procedural complications	Procedural pain	16	12	5
Respiratory, thoracic and mediastinal disorders	Rhinorrhea	15	12	5

[a] ADRs are defined as any Adverse Event that occurred within 24 hours after being infused with investigational product OR all Adverse Events assessed related or possibly related to investigational product OR Adverse Events for which the investigator's or sponsor's opinion of causality was missing or indeterminate.

[b] The ADVATE clinical program included 234 treated subjects from 5 completed studies in PTPs and 1 ongoing study in PUPs as of 27 March 2006.

[c] MedDRA version 8.1 was used.

Table 2
Post-Marketing Experience

Organ System [MedDRA Primary SOC]	Preferred Term
Immune system disorders	Anaphylactic reaction[a] Hypersensitivity[a]
Blood and lymphatic system disorders	Factor VIII inhibition
General disorders and administration site conditions	Injection site reaction Chills Fatigue/Malaise Chest discomfort/pain Less-than-expected therapeutic effect

[a] These reactions have been manifested by dizziness, paresthesias, rash, flushing, face swelling, urticaria, and/or pruritus.

References: 1. Shapiro A, Gruppo R, Pabinger I et al. Integrated analysis of safety and efficacy of a plasma- and albumin-free recombinant factor VIII (rAHF-PFM) from six clinical studies in patients with hemophilia A. Expert Opin Biol Ther 2009 9:273-283. **2.** Tarantino MD, Collins PW, Hay PW et al. Clinical evaluation of an advanced category antihaemophilic factor prepared using a plasma/albumin-free method: pharmacokinetics, efficacy, and safety in previously treated patients with haemophilia A. Haemophilia 2004 10:428-437.

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151 Patient Groups Send Letter to HHS Secretary on Essential Health Benefits Proposal

December 18, 2012

The Honorable Kathleen Sebelius
Secretary of Health and Human Services
The Hubert H. Humphrey Building
200 Independence Avenue, S.W.
Washington, D.C. 20201

Re: Comments on Essential Health Benefits Proposed Rule

Dear Secretary Sebelius:

We, the undersigned, are health advocacy organizations representing millions of patients and their families who are committed to implementation of the Affordable Care Act (ACA). The manner in which the essential health benefits (EHB) are defined will directly impact how well health coverage works or does not work for approximately 23 million patients expected to be enrolled in the exchanges and the millions of enrollees in non-grandfathered individual and small group plans outside of the exchanges. We are writing to thank you for acknowledging our earlier concerns with the December 2011 EHB bulletin, and to comment on the proposed EHB rule issued on November 26, 2012. Specifically, we recognize the changes regarding prescription drug coverage, and now ask you to further consider our views as you finalize the rule in order to provide all patients with meaningful and affordable care and treatment.

Prescription Drug Coverage

We are pleased that you recognize the "one drug per class" minimum requirement was not workable for patients, particularly for those with serious complex chronic health conditions. The proposed language in the rule, "at least the greater of: 1) one drug in every category and class; or 2) the same number of drugs in each category and class as the EHB-benchmark plan" provides patients with greater access to medications. Unfortunately, it will inevitably fail to meet many patients' needs and presents additional difficulties. Nevertheless, in the final rule, we urge you not to go below the proposed standard.

Meeting a Target Number of Drugs: Patients do not respond to a specific number of drugs but rather to specific drugs that best meet their needs as prescribed by their physician. The proposed rule merely requires plans to meet a target number of drugs within a specific class without regard to which drugs are covered. Under the standard described in the proposed rule, plans can choose not to include certain drugs that may have unique and important therapeutic advantages in terms of safety and efficacy, and still meet the requirements of EHB coverage just as long as they include a minimum number of drugs in the class. A system must be in place to review the adequacy and quality of each plan formulary; the quantity of medications must not be the only measure. EHB plans could exclude more effective therapies in some classes, which would violate the patient protections and non-discrimination policies in the law and would not be consistent with "typical" employer plans. A robust formulary is necessary because not all patients respond to medicines in the same way. Physicians may need to change medicines over the course of an illness, patients may become resistant to or suffer adverse side-effects from a particular drug, some may need more than one medication from the same class at the same time, and patients taking multiple medicines need alternatives to avoid harmful interactions. Patients need access to a full range of medicines. If they are not able to access appropriate medications, patients may become ill, impacting healthcare spending in the long run.

State Variation in Drug Coverage: According to analysis conducted by Avalere Health, there exists a wide variation in the total number of drugs included in the state selected benchmark plans. While some states have over 1,000 drugs on their formulary, others have fewer than half of that amount. Although simply judging the quality of a formulary by the number of drugs covered is a poor measure of its adequacy, it is troubling that we see such significant variation across states. This perpetuates the fragmented system of health care in the country. To meet patients' needs, we suggest that plans be required to cover all or substantially all drugs in each class.

Plan for New Drugs: The proposed rule does not discuss how plans must address new drugs that come onto the market during the course of a plan year. The standard described in the proposed rule appears to tie the EHB formulary requirements for 2014 and 2015 to the number of drugs offered by the benchmark plan in 2012 and does not include any requirements for plans to cover drugs approved after 2012. We would suggest that plans be required to update their formularies using methods similar to Medicare Part D and the private insurance market. For example, Part D requires that independent Pharmacy and Therapeutic (P&T) Committees make decisions on coverage of new products within 180 days of their approval. As part of the requirement to review newly approved drugs, patients in EHB plans should be able to remain on older therapies without the fear that their prescriptions will be taken off the formulary when a newer drug is added.

Drug classification system: HHS proposes to use the US Pharmacopeia (USP) system to classify the drugs in EHB formularies, but this system would require changes to be used for this purpose. The USP only updates their drug classification system every three years, which will cause delays in reflecting new medical innovations. USP also does not recognize combination products, which have been shown to improve adherence and have become the standard of care in some areas. The USP system is also very broad, which would allow plans to cover the same number of drugs in a class as the benchmark while exclude groups of drugs needed for patients with certain diagnoses. If changes are not made to the USP, we recommend that HHS consider alternative approaches.

Appeals Process for Drugs not on Formulary: While the proposed rule states that a plan "must have procedures in place that allow an enrollee to request clinically appropriate drugs not covered by the health plan," such a process is not laid out and we are concerned the interests of patients will not be adequately protected. The proposed rule merely states that a plan has to have a process, but does not provide any standards or requirements for an appeal process. We would recommend that the procedures outlined in Medicare Part D, which calls for an expedited, timelimited process with emergency filling of prescriptions be required. Further, we believe that HHS should adopt a standard of guaranteeing access to medically necessary pharmaceuticals through the appeals process.

Read the complete letter on www.rockymountainhemophilia.org

Rocky Mountain Hemophilia
& Bleeding Disorders Association
2100 Fairway Drive, Suite 107
Bozeman, Montana 59715-5815

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