SUMMER 2013

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

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www.rockymountainhemophilia.org

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cell 406.600.2554

www.facebook.com/rmhbda

2nd Annual MT & WY Walk for Hemophilia!

September 7
Bogert Park, Bozeman

Registration 9 AM; Walk 10 AM

To be eligible for a special drawing prize, register as a Team Captain by July 15!

Visit **www.hemophilia.org/walk** to create or join a walk team ; click on the "MT" link, then click on your preference: "Register," "Donate," "Create a team," or "Join a Team." We've raised over $14,000 in corporate sponsorship as of June 27!

Can't make it, we understand! But you can still participate and contribute by hosting a "mini walk," BBQ, small party, or event in your community, or search out "virtual walkers" who can't attend as well! Tell them they can, "**sleep in, save gas, get a t-shirt**," and make a big difference for your family and families throughout Montana and Wyoming. Also, call Brad Benne to find out how you can help! Your support is appreciated, this is your organization!

Sue Geraghty Retires

RMHBDA wants to say an emotional "Thank you, and we will miss you" to Sue Geraghty, whom many of us love as a friend and caregiver. On behalf of our chapter, I want to thank Sue for her tireless service and commitment to our community and families in Montana and Wyoming. Best wishes to you Sue, you are always welcome at any RMHBDA gathering!

Please help us congratulate and say goodbye to Sue. Her last day at the Hemophilia & Thrombosis Center (HTC) was May 31st We invite all patients and families to a thank you/farewell open house on July 12at the HTC beginning at 4:00 PM

Family Camp 2013: Family Fun & Bonding

University of Colorado Anschutz Medical Campus

Our annual Family Camp would not be possible without our generous program funders: CVS Caremark, Baxter, Biogen Idec, CSL Behring, Grifols, Novo Nordisk, Pfi er, and Walgreens Infusion Services.

RMHBDA Family camp was held June 14–16,2013at Luccock Park Camp near Livingston, Montana at the base of the Absaroka Mountains. Sixteen families attended with 21youth and 36adults. Thank you to all who attended.

Adults and youth ages 11-17participated in a powerfully engaging program that inspires adults and kids to believe in their own ability to function at their optimal level and challenge them to grow, presented by Pfi er.

Children ages 2–10enjoyed numerous arts and crafts projects, games, and building forts in the woods, supported by Pat Torrey and Amanda.

Thank you to the camp committee and our chapter volunteers: Sue Scott, Andrea & Leroy Stafford, Bailey, Lisa Maxwell, Becky Ybarra, Sherry McLendon, Ryan Smith, Steve Petty, Kent Pointer, Melana Neutgens, Beth Lambe, Dr. Marilyn Manco-Johnson and Susan Benne! Your good work and valuable time made family camp a wonderful experience for everyone!

MT & WY Hemophilia Walk Kickoff

Rocky Mountain Hemophilia & Bleeding Disorders Association

You Are Invited to the Kickoff Celebration/Baxter Facts First Bleeding Disorders Education Seminars

We will have food and quality education provided by Baxter. And of course, loads of helpful information on how to make your walk team make the last two weeks of fundraising really count! We can't wait for you to join us.

Billings Aug 19 • Bozeman Aug 20 • Helena Aug 21 • Missoula Aug 22
Please RSVP: Brad Benne, brad.rmhbda@gmail.com or 406.586.4050

Baxter Facts First Bleeding Disorders Education Seminar: **Managing Pain**

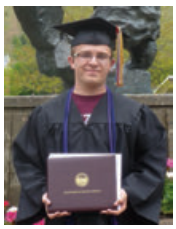
Understanding How To Assess & Manage Pain Effectively

The Facts First program is an interactive, educational series that covers a wide variety of topics affecting the hemophilia community. Facts First seminars bring together hemophilia experts, caregivers, and those living with hemophilia to address issues and questions in an open, conversational setting.

Saturday, September 7, 2013, 9:00 AM – 11:00AM
Bogert Park, 325S. Church Street, Bozeman, MT 59715

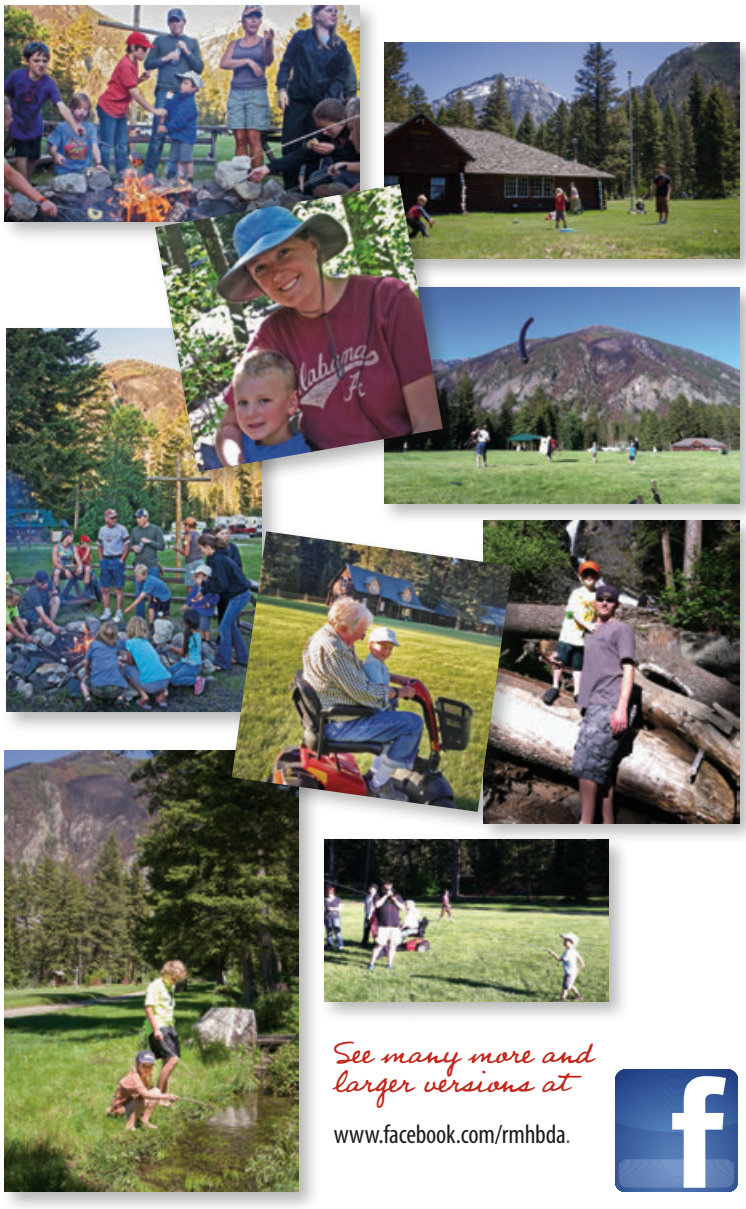
Visit www.hemophilia.org/walk for more information and to register and raise critical funds for our chapter of the National Hemophilia Foundation.

Congrats Grads!



Congratulations to Sean Jeffrey, Sara Jestrab and Dylan Maxwell on their recent college and high school graduations! RMHBDA was founded 13years ago, and Dylan was around my son John's age. These three might be the first graduates of the small group that started this remarkable chapter. I hope my boys are half as impressive and friendly as Sean, Sara, and Dylan. On behalf of my family and the RMHBDA family, we wish Sean, Sara, and Dylan great success and happiness. They all happily accept words of wisdom, well-wishes, and donations to their cause.

Family Camp Photos



See many more and larger versions at
www.facebook.com/rmhbda.

Ladies Only: RMHBDA's "Women's Escape"

Friday –Sunday
November 8–10, 2013
Chico Hot Springs, Pray, MT

We need to organize! Please contact Brad if you are interested in attending and planning our program. Ladies, please leave the boys at home.

Our Women's Escape committee includes: Jessica Amende, Christy Savage, Heidi Hart, Jane Robertson, and Sara Jestrab. Thank you for your time and support in planning this event. **All of your expenses at Chico Hot Springs will be covered**. If you need fuel assistance, please talk with Brad.

Please RSVP: Brad Benne, brad.rmhbda@gmail.com, (406) 586-4050

May Education Series Was A Great Success!

Sponsored by Pfi er Hemophilia

Jackie Kiebler of Pfi er Hemophilia presented "Education for Caregivers" in Billings and Bozeman. Participants shared numerous stories, lessons and shared many "teachable moments" as we move forward in dealing with bleeding disorders in our families. Jackie shared valuable information and her extensive experiences as a medical professional with our chapter. Thank you Jackie and Dan for giving us this educational opportunity.

Wednesday, May 1, 2013at Bin 119in Billings

Sponsored by Baxter

We were fortunate to receive quality advocacy training and knowledge in regards to important legislative issues that affect Montana and Wyoming. Kim Isenberg of Baxter shared a wealth of knowledge and experience with our chapter in Helena, Billings and Bozeman. Thank you Kim and Ryan for making this program a possibility. Join our chapter and bring your voice to fig t for your family and friends affected with bleeding disorders.

Tuesday, May 14, 2013in Montana's Rib and Chop House in Billings.

Please Join Us Next Year!

BAYER HEALTHCARE AND THE HEMOPHILIA COMMUNITY:

Commitment, Leadership *and* Innovation

Bayer HealthCare

Printed in USA KN10001111

Hemophilia Federation of America

RMHBDA is a new member of Hemophilia Federation of America; these are some of their programs.

Helping Hands

Helping Hands™ Hemophilia Federation of America

The financial burden of managing a bleeding disorder is enormous. Costs associated with a hemophilia and other bleeding disorders can affect the entire family's budget. There are several resources that can help during difficult situations.

Asking for Help Is the First Step

It is important to consult with multiple resources to help resolve your financial needs.

- Reach out to your local hemophilia organization — Some local chapters may have funds available to help families with immediate financial needs.
- Contact the nearest Hemophilia Treatment Center (HTC) — The social worker at the Hemophilia Treatment Center can help you explore the options available and may be able to aid you throughout the application process.
- Find out if there are local organizations that provide the services you need — Depending on your needs, there may be local agencies or organizations that help with a specific need.

HFA Resources Include:

- Helping Hands Program — aids families with emergency/urgent funding to assist in crisis situations such as housing, transportation, and utility bills.
- Items Reimbursement — reimburses community members for durable medical equipment and items.

Blood Brotherhood

BLOOD Brotherhood Hemophilia Federation of America

Blood Brotherhood is a national program for adult men, living with hemophilia or von Willebrand disease. It is designed to be a multi-avenue outreach, to provide education and support, promote good health, and establish a sense of community for adult men.

How Can I Participate?

Stay Tuned for the first ever "RMHBDA and Snake River Hemophilia Association Blood Brotherhood event!

Join the Blood Brotherhood Private Online Forum

HFA is proud to sponsor a secure site for adult men with bleeding disorders to meet other Blood Brothers from across the country. In this private format, men with bleeding disorders can share their experiences and learn from each other. This forum offers an opportunity for men to share their experiences and support each other.

Participate in an Upcoming National Webinar or Online Event

Connect with other men during one of the Blood Brotherhood's quarterly webinars or online events focused on issues that are important to men like you! Our upcoming webinars are listed below. For more info, visit www.hemophiliafed.org/programs/blood-brotherhood/

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CHOICE Project

It's your CHOICE, it's your voice.

CHOICE (Community Having Opportunity to Influen e Care Equity) is a project driven by Hemophilia Federation of America (HFA) and supported by the Centers for Disease Control and Prevention (CDC). CHOICE collects information through an online and paper-based survey. This survey collects information regarding health experiences of people who have a doctor-diagnosed bleeding disorder and do not receive care at a federally-funded hemophilia treatment center (HTC). HFA's goal for the CHOICE Project is to put the survey results to work to improve the lives of those in the bleeding disorders community. For more information visit http://www.hemophiliafed.org/programs/choice/

NATIONAL HEMOPHILIA FOUNDATION

National Hemophilia Foundation

Steps for Living

STEPS FOR LIVING
Education for all life stages.

Steps for Living is your one-stop resource for information on bleeding disorders for kids, adolescents, parents and health educators to promote healthy living for the whole family. Newly diagnosed? You are not alone. We have tons of practical resources for you. Trying to figu e out how to help your child make the shift from high school to college? We can help.

Created by parents, patients, and health care professionals from the bleeding disorders community, the Steps for Living website provides information and resources to help you and your family adjust to life with a bleeding disorder as your child grows and matures. Steps for Living was designed to be a practical tool kit to help families deal with the daily challenges of living with a bleeding disorder. There is information and activities for all age groups. We hope you find it useful and helpful.

If you would like more in-depth information about anything on our Web site, please feel free to contact National Hemophilia Foundation's HANDI resource center. The Information Specialists at HANDI are there to answer your questions, and always protect your privacy. HANDI staff members are available Monday through Friday, 9 AM to 5:30PM EST, to answer your requests. Requests can be made by phone to 800-42-HANDI (800-424-2634),email handi@hemophilia.org, or fax (212)328-3799.See more at: http://www.stepsforliving.hemophilia.org/#sthash.IVQjvvo6.dpuf

NHF Annual Meeting

NHF's 65TH ANNUAL MEETING
United in Progress
ANAHEIM, CA ■ OCTOBER 3-5

In an effort to go "green," NHF is requesting that all attendees register online. We will provide $50 off for online registration. Save an additional 15%on your registration fees by registering **online** before Wednesday, July 3, 2013.Note that discounts do not apply to Babycare/Activity Program for Kids/Teens registration. If you cannot register online, please call 800.424.2634 ext. 4 to obtain a paper registration. Online discounts will not apply. There will be no paper registration onsite. All attendees who wish to register onsite will do so electronically.

Registration Fees • Prices include $50 discount

Consumer $125	Babycare/Activity Program for Kids & Teens $75per child
Physician $450*	Nurse $450*
Pharmacist $450	Social Worker $300*
Physical Therapist $300*	Industry $550*
NHF Chapter Staff $150	

**CME/CEU credits are included in the price where applicable*

Victory for Women

Victory for Women (V4W) is NHF's health initiative to address the critical issues faced by women with bleeding disorders.

VICTORY FOR WOMEN
WITH BLOOD DISORDERS
WWW.VICTORYFORWOMEN.ORG

- To increase awareness of women's bleeding disorders so that girls and women receive early, accurate diagnoses, leading to better health outcomes and
- To provide women affected by bleeding disorders with the education, support, skills and resources they need to advocate for their healthcare, financial and social support needs.

Current V4W Projects

Getting the Word Out

- Educating healthcare professionals, girls and women by providing funding to NHF chapters for local projects
- Piloting social marketing efforts to reach women via the web
- Implementing a targeted program to improve the likelihood that campus healthcare providers will recognize the signs and symptoms of bleeding disorders in college women, and refer appropriately for care

Providing Resources and Support

- Grants to NHF chapters for the development of educational programs, as well as providing opportunities for skill building and empowerment for girls and women affected by bleeding disorders
- Outreach efforts to inform affected community members about V4W, and encourage women to get involved with their local chapter
- Encourage collaborative efforts among those in the bleeding disorders community to increase opportunities for girls and women to receive relevant information and support

Additional V4W Activities

- Training for community members and chapter staff on issues relevant to women with bleeding disorders
- Scholarships for women with bleeding disorders pursuing a certifi ate or degree in post-secondary education
- Participation at health professional conferences
- Development of Web site with information for healthcare providers, those who have been diagnosed, and girls and women who are symptomatic but not yet receiving care
- Active participation in NHF's Annual Meeting, organizing a number of sessions on topics of importance to women with bleeding disorders

For more information about V4W, please contact:
Patrice Flax, MS, MSW, NHF Manager of Education,
734.890.2504,pflax@hemophilia.o g,
or contact your local chapter.

What RMHBDA Means to Us...

Dear RMHBDA,

Thank you so much for helping us be able to attend clinic in tough times. The support means the world to us. Bless you all for helping people in need.

Sincerely,
RMHBDA Family

NHF Advocacy Priorities

Please contact your state senators and members of congress to voice your opinion in regards to important legislative issues that will affect your community. Your voice and willingness to fig t for what you believe **does** make a difference!

There Are Two Federal Hemophilia Programs Critically Important To Our Community

Issue 1: HR 460 – The Patients' Access to Treatment Act

Many insurance plans have formularies, which is a list of drugs that are covered by the plan. In addition, plans create separate tiers to set co-payments: generics (Tier I), name brand (Tier II), and off-formulary, brand drugs (Tier III), specialty tier (Tier IV). Insurers set fl t co-pays (i.e. $10/$20/$50) for drugs in the first three tiers. Copayments for specialty tier drugs (Tier IV) are often set at 25–33%of the cost of the drug. Clotting factor is typically placed on the specialty tier. The yearly cost for clotting factor can be $300,000 per year for a person with severe hemophilia, and can exceed $1million for a person that develops an inhibitor. People with bleeding disorders simply cannot afford to pay this cost!

Insurers require higher patient cost-sharing to reduce use and incentivize patients to choose lower-cost generic alternatives. But, there are no generics for factor!

This bill will increase access to life-saving drugs by removing the burden of excessive cost-sharing, benefiting people with bleeding disorders, and others with high-cost chronic conditions, such as leukemia and lymphoma, multiple sclerosis, rheumatoid and psoriatic arthritis, lupus, primary immunodeficien y diseases, and Crohn's disease.

Issue 2: HRSA Maternal & Child Health Bureau

$4.9 million in funding provides: critical, multi-disciplinary services not usually covered by insurance, such as PT assessment, case management and social work services. Reduced funding for the hemophilia program at HRSA could result in staffin cuts

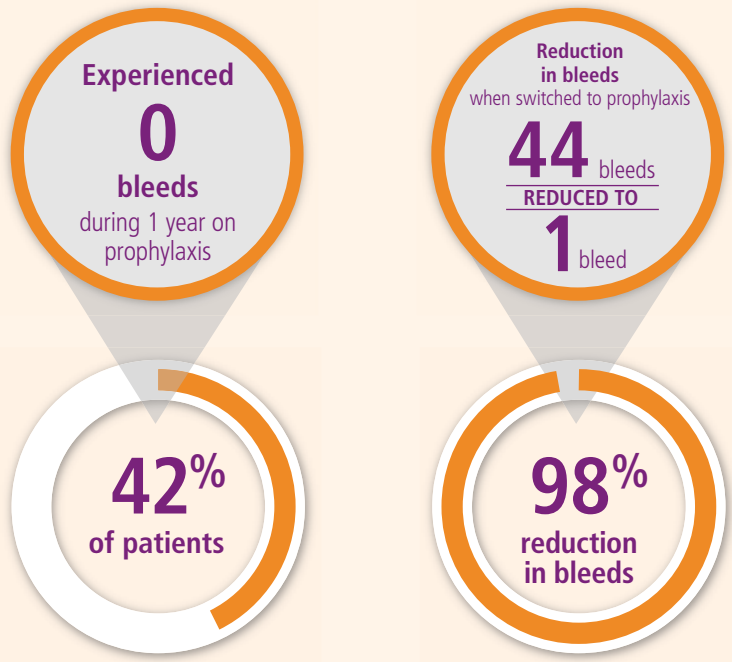
Continued on page 11

UNLOCKING SELF-POTENTIAL

PROPHYLAXIS WITH ADVATE REDUCED BLEEDS IN A CLINICAL STUDY[1,a]

ADVATE is the only recombinant factor VIII (eight) that is FDA approved for prophylaxis in both adults & children (0-16 years)[1]

Significant reduction in median annual bleed rate (ABR) with prophylaxis treatment compared with on-demand treatment[1,a]



- 0 bleeds experienced by 42% of patients during 1 year on prophylaxis[1,a]
- 98% reduction in median annual bleed rate (ABR) from 44 to 1 when switched from on-demand to prophylaxis[1,a]
- 97% reduction in joint bleeds from 38.7 to 1 after switching from on-demand to prophylaxis[1,a]
- No subject developed factor VIII inhibitors or withdrew due to an adverse event (AE)[2,a]

[a]In a clinical study, 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.

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.

References:

1. ADVATE Prescribing Information. Westlake Village, CA: Baxter Healthcare Corporation; July 2012. **2.** 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. **3.** PROLabels: patient-reported outcomes & drug marketing authorizations. ProLabels Web site. http://www.mapi-prolabels.org. Accessed June 5, 2012. **4.** Maruish ME, ed. *User's Manual for the SF-36v2 Health Survey*. 3rd ed. Lincoln, RI: QualityMetric Incorporated; 2011.

Baxter

AND PROVIDED CLINICALLY MEANINGFUL IMPROVEMENTS[b] IN PHYSICAL HEALTH-RELATED QUALITY OF LIFE[1]

ADVATE is the only recombinant factor VIII with physical health-related quality of life results[3,c]

Overall improvement in physical functioning, well-being, general health, and/or energy level, based on Physical Component Score.[4]

Reduced levels of pain and improvements in the limitations of work due to pain.[4]

BODILY PAIN

Improvements in the ability to perform work or other daily activities.[4]

ROLE PHYSICAL

PHYSICAL FUNCTIONING

Changes not observed in limitations to a range of minor and major physical activities.[4]

GENERAL HEALTH

Changes not observed in the views and expectations of overall health.[4]

[b]Clinically meaningful changes were not seen in the mental health-related component score and sub-categories of Mental Health, Role Emotional, Social Functioning, and Vitality.[1]

[c]Based on a list of 29 other drugs and biologics approved with health-related quality of life data in the labeling, as of June 2012.

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

ADVATE is not used to treat von Willebrand Disease.

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

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.

Ask your healthcare provider if prophylaxis with ADVATE is right for you.

ADVATE
[Antihemophilic Factor (Recombinant), Plasma/Albumin-Free Method]
There's more to life.

www.advate.com | 888.4.ADVATE

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 information]*

ADVATE contains trace amounts of mouse immunoglobulin G (MuIgG): 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 (5.3) in full prescribing information]*

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 all Adverse Events that occurred (a) within 24 hours after being infused with investigational product or (b) all Adverse Events assessed related or possibly related to investigational product or (c) 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.

To enroll in the confidential, industry-wide Patient Notification System, call 1-888-873-2838.

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From page 7: **NHF Advocacy Priorities**

at HTCs, jeopardizing the quality of care for people with bleeding disorders.

CDC Division of Blood Disorders

CDC provides about $4 million to HTCs for research and surveillance (decreased from by 3 million over last four years) to prevent joint disease and monitor the safety of blood supply. Reductions to CDC's hemophilia program would jeopardize activities to monitor the safety of the blood supply, improve treatment and prevention strategies, and ensure access to specialized care.

Sean Jeff ey and Brad had the opportunity to briefly meet with Montana's new congressman Steve Daines and Jon Tester. One benefit of living in states that don't have a lot of population, is your VOICE and OPINION are can be extremely powerful! We all share different political opinions, however HR 460 is co-sponsored by a Democratic and Republican, and in my opinion is a "fairness" issue. I don't know any family that can afford $50,000 plus out of pocket for their medicine. I ask you, please contact your representatives, and ask your representative in congress to co-sponsor HR 460 and ask your Senators to please consider introducing companion legislation.

Contact Information

Montana

Senator Max **Baucus** (202)224-4515

Senator Jon **Tester** (202)228-6673

Representative Steve **Daines** (202)225-3211

Wyoming

Senator John **Barrasso** (202)224-6441

Senator Mike **Enzi** (202)224-3424

Representative Cynthia **Lummis** (202)225-2311

Baxter Receives FDA Approval for Rixubis

Baxter

June 27,2013— **FDA approves first recombinant coagulation factor IX that is specifi ally indicated for routine use in preventing bleeding episodes (prophylaxis) .**

The U.S. Food and Drug Administration yesterday approved Rixubis [Coagulation Factor IX (Recombinant)] for use in people with hemophilia B who are 16years of age and older. Rixubis is indicated for the control and prevention of bleeding episodes, perioperative (period extending from the time of hospitalization for surgery to the time of discharge) management, and routine use to prevent or reduce the frequency of bleeding episodes (prophylaxis). The efficacy of Rixubis was evaluated in a multicenter study in which a total of 73male patients between 12and 65 years of age received Rixubis for routine prophylaxis or as needed in response to symptoms of bleeding (on-demand). Overall, patients in the prophylaxis study had a 75percent lower annual bleeding rate when compared to patients who have historically received on-demand treatment. An additional study in a pediatric population is currently ongoing.

Read the full press release at http://www.fda.gov/NewsEvents/Newsroom/PressAnnouncements/ucm358918.htm

NHPCC Patient Survey

The NHPCC (coordinating center from ATHN) will be conducting needs assessments of centers and of patients. They are requesting pilot patients that may be willing to participate in the pilot. Pretest Steps:

1. *Identify an HTC willing to provide a pretest sample of patients.*
2. *HTC will:*
   a. *Recruit patients willing to participate in a confidential telephone interview to provide feedback on the survey questionnaire design. Approximately 10 patients should be identified representing a mix of a) adult bleeding disorder patients and parents of children who are bleeding disorder patients, and b) hemophilia and VWD patients.*
   b. *Provide Allen Cheadle at Group Health (Cheadle.a@ghc.org) with the names, mailing addresses, and phone numbers of the persons recruited.*
3. *Group Health will contact each participant to schedule a telephone interview. Once scheduled, a copy of the questionnaire will be mailed to them with instructions not to open it until the phone interview begins.*
4. *The participant will open the questionnaire envelope at the start of the phone call and walk through the instrument with the interviewer, providing feedback on the design (e.g., is the format easy to follow, are the questions/ response options understandable?). It is expected each interview will take approximately 30 minutes.*

The interviewer will take notes based only on participant comments about the survey design. No actual data will be gathered. Because this is a pretest and no patient data will be gathered, it does not require IRB approval. Because the patient is not providing responses to the questions, they may receive a mailed questionnaire in September and participate in the actual survey.

The interviews are confide tial meaning that although the interviewer knows who the person being interviewed is and is aware of the interviewee's comments, there is an explicit acknowledgement that the interviewer will not expose the interviewee's identity or link any of their comments with their name. All information communicated between those two persons will be held in confiden e.

Each participant in the pretest will receive $25in the form of cash or a gift card.

FDA Accepts BLA for Biogen's ELOCTATE

biogen idec HEMOPHILIA

Last month, Biogenic Idec announced that the US Food and Drug Administration (FDA) had accepted its Biologics License Application (BLA) for the marketing approval of ELOCTATE™ a recombinant factor VIII (FVIII) Fc fusion protein for the treatment of hemophilia A. According to a Biogen press release posted on May 13th,2013,ELOCTATE is the first hemophilia A product candidate in a new class of long-lasting clotting factor therapies. "ELOCTATE has the potential to improve adherence by reducing the number of intravenous injections needed to prevent bleeds, which is an important need for people with hemophilia A," said Glenn Pierce, MD, PhD, senior vice president of Global Medical Affairs and chief medical officer of Biogen Idec's hemophilia therapeutic area. "For those people currently on preventative—or prophylactic—treatment, ELOCTATE provides the potential to reduce the number of intravenous injections by 50 to 100per year." The ELOCTATE BLA was based on results from "A-LONG," the largest registrational phase 3 clinical study in hemophilia A to date. In the A-LONG study, patients who injected ELOCTATE weekly or twice weekly had fewer bleeds. This is in contrast to the current prophylactic regimen for patients with severe hemophilia A, who typically infuse three times per week or every other day to maintain a suffici t level of FVIII in their bloodstream to prevent bleeds. *Source:* Biogen press release dated May 13,2013

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Spencer & Kaylee Straub

** new members*

2013 Calendar

July 2013

12–14 Mile High Summer Camp Leadership Pre-camp Retreat

14– 19 Mile High Summer Camp (Rocky Mountain Village, Empire CO)

August 2013

19–22 Walk Kickoff Event
19 Billings
20 Bozeman
21 Helena
22 Missoula

September 2013

7 Walk for Hemophilia, Bogert Park, Bozeman

October 2013

3–5 NHF Annual Meeting (Anaheim)

12 Blood Brotherhood Event (Tentative)

November 2013

8–10 Women's Retreat at Chico Hot Springs

Donations & Membership

Donations

- *In honor of* Gino Rotellini

Welcome New Members

- Jerome & Kim Hugs
- Chris & Dawn Hunter

Board of Directors

Lisa Maxwell *President,* Great Falls, MT
Chris Graham *Vice President,* Billings, MT
Ben Kuss *Treasurer,* Bozeman, MT
Jane Robertson *Secretary,* Cody, WY
Sara Jestrab Bozeman, MT
Spencer Straub Cheyenne, WY

We Need A New Board Member

Someone willing to raise awareness and support for our chapter. If you are interested, visit http://www.rockymountainhemophilia.org/html/board