SPRING 2014

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

Washington Days

"I traveled to Washington Days this year for the first time and as a "newer" diagnosee of our two daughters and myself, I often referred to myself as the rookie of the group when I began to talk about my experiences over the last couple of years living with Von Willebrand's Disease (VWD). I was paired with two lovely gentlemen from the Alaska Hemophilia Foundation for the day as we visited/lobbied with three legislators from the State of Alaska and three legislators from the State of Wyoming. We had a handful of others that joined in for some of the discussions that included several staffers from the NHF office and even one of our RMH own, Lisa M. I was often asked about VWD because it is not nearly as common in the discussion groups and there seemed to be increased curiosity from our nations leaders about a disease that they had never heard of. We all spoke of our personal stories; which included the present and past trials and tribulations and of the huge costs of prescriptions that we all face daily. The House Bill HR 460 we were lobbying for was asking for increased funding for research as well as some much needed adjustments in the prescription tiers that could lower our prescription costs for factor concentrates. One of the additional items we lobbied for with our Senators directly was to ask them to introduce a Senate bill that would be a companion bill to HR 460. The more support the merrier! My trip highlight was that at the very end of the day at the last appointment, I walked in to his office and advised that our group was there to meet with Senator Barrasso from Wyoming; the staffer then advised me that he would be meeting with me directly in his conference room! I was pretty excited at that point!

Continued on page 6

Blood Brotherhood

Hemophilia Federation of America – Rocky Mountain & Snake River Hemophilia Association's Men's Retreat

February 21–23, 2014 — Chico Hot Springs, Pray, MT

Our first annual men's retreat included a discussion with John Beck, LCPC regarding stress management. Some of the topics we discussed were the effects of stress on the brain, methods to diffuse stress. Some

feedback from the eight men that attended the retreat included: "These events foster connection with to others in the same situation" and "Great program, hit something for very diverse needs of our group".

We also enjoyed a dogsled trip in the Absaroka Mountains, relaxing in the hot springs, amazing food and a quality bonding experience. We hope to grow our group for our next event! Please consider joining us, or if you have some ideas for topics to discuss at a Men's retreat. We are also always looking for unique destinations and activities to include in future men's programs.

Rocky Mountain Hemophilia & Bleeding Disorders Association

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Designed by & masthead photo courtesy of Fifth Street Design, Bozeman, Montana.

From Our Executive Director

Welcome to the 2014 Rocky Mountain Bleeding Disorders Walk!

By Brad Benne, Executive Director

The RMHBDA Bleeding Disorders Walk is about the power of community. Coming together for a cause empowers us. We are much stronger when we stand together.

Have you registered yet for the 2014 Rocky Mountain Bleeding Disorders Walk? We've got an exciting new fundraising incentive for you! We're excited to partner with Turnkey Promotions to reveal the 2014 RMHBDA Hemophilia Walk Incentive Program. This year, you can win amazing prizes starting at the $250 fundraising level. The more your raise, the bigger the prize! Stay tuned for prize details!

Of course, the Walk is also an amazing way to raise money to fund education, advocacy and research leading to better treatments and a cure.

Raising money through the walk isn't hard to do. Friends and family want to help; all you have to do is ask them. Register at www.hemophilia.org/walk *today to start; just click on MT and register your team. As an added incentive to get you started now, raise $100 online before May 2, 2014 and get a hat showing you took that first step and are participating in a great cause. You can do even more by forming a team. The more who participate, tshe more fun we have and the more we raise; come together with your friends and family and join in on the excitement today. Help get us off to a good start. Join the RMHBDA Walk now!*

Be sure to call, text, or email everyone you know and ask for support. Ask 10 people today for a donation — you will be well on your way. Or better yet, ask them to join you at the Walk and start a team — get them to register today to get started!

Thanks again for joining us!

Family Camp 2014

June 20- 22, 2014 | Camp on the Boulder | McLeod, Montana

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 at Camp on the Boulder, in McLeod, Montana. For more information, visit www.camponthebolder.org

For the parents and teens, we will have teambuilding programming led by our 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 our HTC. Call Brad with questions 406.586.4050

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.

Now donate at **www.rmhbda.org** with PayPal.

Rocky Mountain Hemophilia

& Bleeding Disorders Association

MOTIVATED VIBRANT POWERFUL!

CoRe CONVERSATIONS

A series of live presentations and webinars designed to enrich, educate, and support the hemophilia community.

Topics Include:

Deciding if You're Camp-Ready | APRIL 15 | 7PM ET (4PM PT)

The decision to send your child to camp for the first time can be stressful; we'll discuss the proposed benefits and perceived fears.

Mapping Your Future | JUNE 17 | 8PM ET (5PM PT)

Finishing high school marks a new beginning: we'll examine some tips on choices and decision-making, from selecting a college or vocational program, to defining your career path.

Setting Educational Expectations | AUG 19 | 7PM ET (4PM PT)

We'll explore how to establish clear goals and routines for school-age children with bleeding disorders.

Braving Change | OCT 21 | 8PM ET (5PM PT)

We'll review resources and possible approaches to informed decision-making during times when change may impact your life.

The Art of Transition | DEC 9 | 7PM ET (4PM PT)

We'll consider the importance of facilitating transitions in our lives, and some tools to do so proactively.

Navigating the Financial Aid + Scholarship Process | Contact your CoRe Manager about a live presentation.

We'll discuss the different financial support resources available to the hemophilia community.

Understanding the Value of Genotyping | Contact your CoRe Manager about a live presentation.

Learn how genotyping can bring invaluable information that may one day lead to greater understanding of hemophilia.

For more information, contact Becky Ybarra at becky.ybarra@biogenidec.com | 801-913-8204

Connect with us BiogenIdecHemophilia.com & /BiogenIdecHemophiliaCoRes

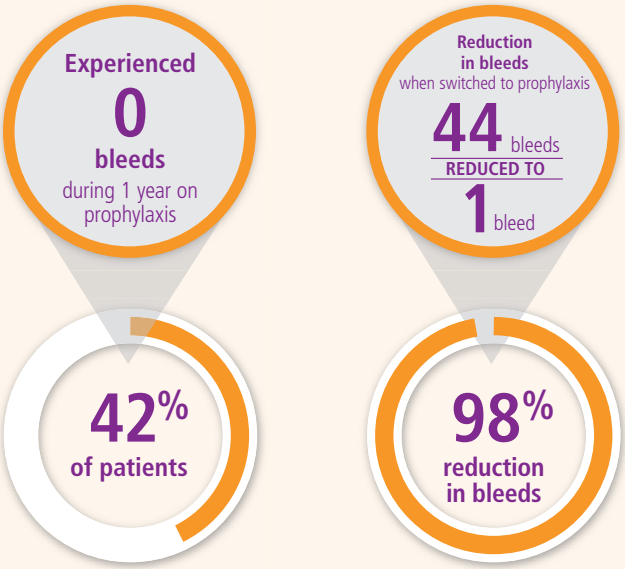
biogen idec HEMOPHILIA

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.

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

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.

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.

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.

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 muIgG protein antibodies. Of these, 10 showed an upward trend in anti-muIgG 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 1: **Washington Days**

The Senator then came in the room, called me out by name and we sat and had a very direct and warm conversation between us regarding my personal story, the issues with medical needs in my hometown, high prescription costs and then asked me what he could do for me. After a long day of meeting with health care advisors, I actually got to meet with my Senator. It was a triumphant moment for me as a woman, mother and citizen of the great State of Wyoming. The trip was everything that I had hoped for and I would love to go back again next year to continue to lobby for issues that are so near and dear to my heart. Go advocacy!"

— **Jodi Rudell**, Cheyenne, WY

"Once again I had the privilege to attend Washington Days and be an advocate for our Chapter representing Montana. My partner, Jodi Rudell from Wyoming was an outstanding advocate! We were paired up with the great State of Alaska to visit our Congressman and Senators. Amazing how each of our states is so similar in the obstacles we as consumers and chapters face. One of our talking points focused on continued funding for the HTCs. With all 3 states facing lower population and massive acreage with long drives (Alaska plane trips) we had no problem explaining why these specialty doctors are so important. Also on our list to present was the Specialty Tier Drug pricing. Even though we live with these dollar amounts every day, Staffers and Congressmen are still amazed at what it costs. I believe it's important to have our voices heard and look forward to making some noise in our own state."

— **Lisa Maxwell**, Great Falls, MT

RMHBDA 2014 Program & Event Calendar

As of March 31, 2014. The chapter is still determining exact dates for several programs and events for the community.

April

4-6 RMHBDA Education Weekend in Helena

17 World Hemophilia Day

24-26 Mountain States Hemophilia Network Annual Meeting, Tucson, AZ

29-30 Pfizer Education Event/ Walk Call to Action:

- 29 Bozeman, 6 pm (Johnny Carinos),
- 30 Billings, 6 pm (Bin 119)

May

13-15 Biogen Idec Education Event/ Walk Call to Action:

- 13 Bozeman
- 14 Billings
- 15 Cody

June

20-22 RMHBDA Family Camp, Camp on the Boulder, McLeod, MT

July

11-13 Mile High Summer Camp Leadership Pre-camp Retreat

13-18 Mile High Summer Camp, Rocky Mountain Village, Empire, CO

August

11-14 Baxter Facts First/ Walk Call to Action:

- 11 Billings
- 12 Bozeman
- 13 Helena
- 14 Kalispell

September

6 RMHBDA Walk for Bleeding Disorders, Billings

18-21 NHF Annual Meeting, Washington, D.C.

TBD CSL Behring "Getting In the Game"

December 2014

TBD Baxter Facts First

Having issues with co-pays or gaps in coverage for your **hemophilia A** treatment??

We may be able to help.

Bayer offers a range of programs that can help you **navigate insurance questions about your hemophilia A** treatment. If you're having issues with co-pays or gaps in coverage, we may be able to offer assistance. Speak with one of our case specialists to find out more.

Call **1-800-288-8374** and press 1 to speak to a trained **insurance specialist!**

Industry News

Bayer Reports Progress for Longer-Acting rFVIII Product — FDA Grants Breakthrough

Last month, Bayer HealthCare announced results from a clinical study that evaluated the safety and efficacy of BAY 94-9027, the company's investigational long-acting, site-specific PEGylated recombinant factor VIII therapy. Known as PROTECT VIII (Prophylaxis in hemophilia A patients via directly PEGylated long-acting rFVIII), the international, multicenter trial included 134 previously-treated adults and adolescents with severe hemophilia A.

Patients in PROTECT VIII selected either on-demand or prophylactic treatment at enrollment. All subjects in the three prophylaxis arms began treatment with the site-specific PEGylated recombinant human factor VIII twice weekly. After a 10-week period, subjects experiencing more than one bleed during this assessment period stayed on two infusions per week at a higher dose. All other subjects were randomized to either every five- or seven-day treatment for six months.

In the study, the site-specific PEGylated factor VIII helped protect against bleeds when used prophylactically every seven days, every five days and twice weekly. The compound was also effective for treatment of acute and breakthrough bleeds, with 91% of events resolving with one or two infusions.

In addition, 88% of patients met the pre-defined criterion of bleeding control in the 10-week initial assessment period and qualified for randomization. All patients receiving infusion every five days remained in this treatment arm. 44% of patients in the every five-day treatment arm experienced no bleeds. A median annualized bleeding rate (ABR) of 1.9 was observed in this treatment arm. 74% of the patients receiving infusion every seven days remained in their treatment arm. 37% experienced no bleeds. A median ABR of 3.9 (including noncompleters) was observed in this treatment arm. The 13 patients who remained in the two times per week treatment arm, because of their high bleeding rate during the assessment period, reduced their median ABR from 17.4 to 4.1. This was in contrast to patients who received on-demand treatment and had a much higher median ABR of 23. Source: Bayer news release dated February 18, 2014

CSL Behring

www.MYSourceCSL.com assistance program and the phone number is 800-676-4266. The program is designed to alleviate copays and coinsurance for private pay patients on Humate or Helixate. The copay assistance limit is $12,000 yearly.

Pfizer

If you are using a nonparticipating provider, you can still take advantage of the Factor Savings Card Program, if eligible, on your next order of factor. Save up to $5,000 on co-pays.

Biogen Idec Is Awarding Up to $50,000 in College Scholarships!

Biogen Idec is proud to enable students with hemophilia to pursue their passions through a wide range of academic scholarships, including awards for vocational/technical schools, community colleges and four-year colleges and universities. The deadline to apply for the 2014-2015 Biogen Idec Hemophilia Scholarship Program is June 12, 2014.

Baxter Presents Data at EAHAD from Long-term Outcomes Registry Reinforcing Prophylaxis Treatment Experience with Advate

(Brussels, BELGIUM)- Baxter International Inc. today presented clinical data on Baxter's leading recombinant factor VIII treatment, including interim data from the first year of observation from the AHEAD (ADVATE Haemophilia A Database) study, a four-year outcomes registry of hemophilia A patients treated with ADVATE [Antihemophilic Factor (Recombinant) Plasma/Albumin-Free Method]. These data were presented during the European Association for Haemophilia and Allied Disorders (EAHAD) meeting in Brussels, Belgium. These real-world data, which support the clinical experience of prophylaxis treatment with ADVATE, found that the majority of patients (55.3%) on prophylaxis had fewer than two bleeding episodes per year, with a median annual bleed rate (ABR) of 1.1. Of these patients, 51 percent experienced no bleeds during one year of treatment. (For more information please visit www.baxterhemophilia.com)

The Education Advantage scholarship program is an example of Baxter's commitment to giving back to the community. Since the program began four years ago, 157 Education Advantage scholarships that include 80 renewals have been awarded, totaling $870,600 to people with hemophilia A. In 2014, the comprehensive scholarship program is open to people with hemophilia A or B (factor VIII or IX deficiency), including those with inhibitors (factor VIII or IX deficiency), regardless of which brand of factor treatment they use.

Education Advantage scholarship program applications will be available as of February 21, 2014 at www.baxternava.com, www.scholarshipamerica.org/baxteredge, or by calling Scholarship America at 877-544-3018, or by emailing baxter@scholarshipamerica.org

Applicants can apply online, download the application and mail it in, or request a paper application. Completed applications are due to Scholarship America by April 15, 2014.

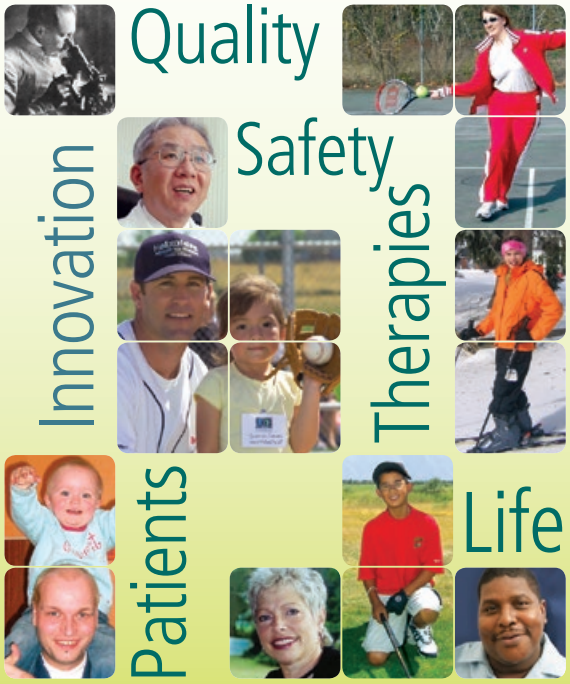
Novo Nordisk Hemophilia Drug Excels in Phase 3

Novo Nordisk announced the completion of pathfinderT2, the first Phase 3 trial with long-acting recombinant factor VIII, N8-GP (turoctocog alfa pegol) for hemophilia A patients. PathfinderT2 is a multi-national trial evaluating safety and efficacy of N8-GP, when administered for prophylaxis and on-demand treatment in patients with hemophilia A who are 12 years or older.

In the trial, 175 patients were treated with a prophylactic regimen of 50 U/kg every fourth day and 11 patients received on-demand treatment, when bleedings occurred. Patients were treated for up to 21 months, resulting in median annualized bleeding rates of 1.3 and 30.9 episodes for patients treated prophylactically and on-demand, respectively.

The pharmacokinetic data documented a single dose half-life of 18.4 hours and a mean trough level of 8% measured immediately before next dose for patients on prophylaxis treatment. N8-GP appeared to have a safe profile and to be well tolerated. Among the 186 patients in the trial, one patient who responded well to prophylactic treatment throughout the trial developed an FVIII inhibitor. This is in line with expectations in a population of previously treated hemophilia A patients.

Novo Nordisk is expecting the three remaining trials in the pathfinderT program to be finalized within the next 12 months. These trials investigate N8-GP as a treatment for pediatric patients, surgical procedures and as once-weekly prophylactic treatment.



At CSL Behring

Innovation leads the way

Committed to making a difference in patients' lives

As the industry leader in coagulation therapies, CSL Behring offers the most extensive portfolio of coagulation products for patients with factor deficiencies, including FI, FVIII, FIX, FXIII, and von Willebrand factor. And we continue to broaden our efforts with a number of recombinant factor therapies in development, including rFVIII, rFVIIa, rFIX, and rVWF.

For more information about our factor products for hemophilia, von Willebrand disease, and other rare bleeding disorders, or to learn about our innovative patient programs, please visit www.cslbehring.com or call consumer affairs at 1-888-508-6978.

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World Hemophilia Day — April 17, 2014

Speak out. Create change

In 2014, World Hemophilia Day will focus on encouraging the global bleeding disorders community to speak out. Create change. This year we are making a particular effort to reach out to young members of the community so as to promote their participation and to develop strong leadership. We want our young members to feel inspired to assume key roles in building and supporting the bleeding disorders community.

Connect with your global online community on www.facebook.com/wfhemophilia and www.twitter.com/wfhemophilia, so that we can all come together to build a stronger community.

World Hemophilia Day 2014 Resources

Access the available resources to support your World Hemophilia Day activities:
www.wfh.org/en/news--events/events/world-hemophilia-day-2014-resources

Give a Gift of Membership

To give the gift of membership to mark World Hemophilia Day:
www.wfh.org/en/page.aspx?pid=1392

Donation

If you would like to make a donation to support the World Federation of Hemophilia:
www.wfh.org/en/page.aspx?pid=1387

Save the Date! Mile High Colorado Camp

Mile High Colorado Camp — **July 20-25, 2014**

Leadership Pre-Camp Retreat — **July 18-20, 2014**

The Hemophilia and Thrombosis Center (HTC) is proud to once again sponsor the summer camp program at **Rocky Mountain Village**. Camp forms will be available in mid-March! Stay Tuned!

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 soon 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 want 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 deposit. The remainder of the camp cost, approximately $1,000 per camper, is underwritten by other sources. Scholarships will be granted on an individual basis. Scholarship forms are available. If you have questions or need scholarship forms or additional information, please call Brad Benne at 406.600.2554.

Help Send A Child To Camp!

This summer make a dream come true. Your contribution will send a youth to Hemophilia summer camp at Mile High Camp in Colorado. Your support makes a lasting difference in the lives of children with a bleeding disorder.

NATIONAL HEMOPHILIA FOUNDATION
www.hemophilia.org

Co-Sponsor H.R. 460, The Patients' Access to Treatments Act of 2013, to Ensure Access to Live-Saving Therapies

About Health Insurance, Specialty Tiers and Hemophilia

- Most private health insurers charge fixed co-payments for different categories, or tiers, of medications: generics (Tier I), name brands (Tier II), and nonpreferred brand medications (Tier III). For example, co-pays might be set at $10/$20/$50, respectively, for medications in the three tiers.
- Some commercial insurers have established a fourth, or specialty, tier that includes biologics and other drugs requiring special administration. Tier IV drugs typically require exorbitant patient cost-sharing. Patients must pay a percentage of the cost of these drugs, from 25% to 33% or more in coinsurance, rather than a fixed co-payment.
- Treatments for hemophilia, known as clotting factor therapies, are frequently placed in the specialty tier. The yearly cost for clotting factor can be as high as $300,000 per year for a person with severe hemophilia and can exceed $1 million for a person who develops an inhibitor. People with bleeding disorders simply cannot afford to pay 25% or more of this cost.
- The intent of requiring higher patient cost-sharing for drugs and biologics is to reduce reliance on these expensive drugs and incentivize patients to choose lower-cost generic alternatives. However, there are no generic alternatives to clotting factor therapies.
- Placing drugs in a specialty tier makes these medically necessary treatments unaffordable for most Americans. People with bleeding disorders who cannot afford specialty tier pricing may delay or go without treatment, resulting in disability and other complications that can lead to increased long-term healthcare costs.

H.R. 460 The Patients' Access to Treatments Act of 2013

- The Patients' Access to Treatments Act **of 2013** would prevent private health insurance plans from requiring higher cost-sharing (co-payments and coinsurance) for medications in the specialty drug tier (typically Tier IV) than what is charged for drugs in a nonpreferred brand drug tier (typically Tier III).
- 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 immunodeficiency diseases and Crohn's disease.
- An analysis by Avalere found that implementation of this bill would increase access to these life-saving drugs while only minimally increasing premiums by approximately $3 per year for plans with specialty tiers, absent any other changes to the plan's benefit design.
- H.R. 460 was introduced by Reps. David McKinley (R-WV) and Lois Capps (D-CA). It currently has 87 bipartisan co-sponsors.

Please co-sponsor H.R. 460 to enable patient access to treatments, reduce disability and limit healthcare costs.

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