Sangre de Oro, Inc.
PO Box 51494
Albuquerque New Mexico

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Credits

Volume 18 • Fall 2013

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The Bear Facts

Hemophilia Walk of New Mexico

By Jean Cole

Although it was not my first Hemophilia Walk, April 20, 2013, was my first Hemophilia Walk where I actually walked. Let me explain: my first Walk (in Pennsylvania) was spent running around setting up tables, tents, and booths for the event.

My team was small in number and missed me as they walked the path. The next year, my team shrank and both of my team members spent Walk day getting things set up and manning a booth.

Both years, I could hear the teams as they walked. Some were shouting cheers or singing songs learned in camp, some were challenging other teams to keep up, some were walking arm-in-arm or hand-in-hand, and others were simply enjoying the leisurely pace and the cool, sunny morning.

Although I stayed on the sideline, I could not have created better or more fun days than I had on these two days—until this year, with SDO's inaugural Walk.

My team was still small in number but we were big on pride and enthusiasm! Walking the path was an experience like no other. There were many walkers and a few dogs. The joyous sound of the Walkers voices and laughter was heard throughout the park. The smiles on the faces of the youths were plentiful. Even though my team and I were not familiar with many of the Walkers, we felt right at home and shared in the excitement of the morning. Seeing the kids, and some adults, getting their faces painted; watching the kids climb in and out of the ambulance; seeing a few people dancing to the music played by the DJ; it's heart-warming. The pictures tell the story.... after seeing them, I find myself ready to experience it all over again next year!

We have to remember that we aren't always doing this for ourselves. We are walking and raising money for all those who have suffered with bleeding disorders in the past, live with them today, and who will be born with them in the future. The money we raise will ensure that SDO can continue efforts in meeting our mission and providing our bleeding disorders community with educational programs and other resources to improve the lives of our members.

2014 Hemophilia Walk Facts

by Jean Cole

Sangre de Oro extends a HUGE Thank You to each of you for your incredible participation with Hemophilia Walk 2013!

With your help, we raised $34,780!!! A phenomenal amount for our inaugural Walk!

The SDO Walk Committee is excited to share with you that we will again partner with NFH for Hemophilia Walk 2014!!

We are working on the details so be sure to check your e-mail for updates! In the meantime...

Date: 26 April 2014

Location: Tiguex Park

2014 Hemophilia Walk Chair: Joe MacDonald

Walk Committee: Lori Long, Carnie Abajian, and Jean Cole

Are you interested in helping with Walk planning or volunteering to help out on Walk day? Have suggestions or comments on how to make Hemophilia Walk 2014 better than our 2013 Walk? Please send us an e-mail. We would love to hear from you!!

Sign up for the Walk on-line by visiting www.hemophilia.org/walk. Everyone is welcome to participate!

Sign up a team. Choose a team name. Recruit team members from your circle of friends, family, co-workers, and neighbors.

Help us make our event a success. Volunteers are appreciated before and/or during the Walk. Are you unable to do the Walk but still want to participate? Enlist as a volunteer.

CONTACT

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Sangre de Oro, Inc.

Calendar of Events

October

3–5	NHF Annual Meeting in Anaheim, CA
18–20	**Patient and Family Education Weekend in Albuquerque, NM**

November

8	SDO Annual Planning Meeting

December

TBD	Holiday Party (Tentative)

March

	National Hemophilia Awareness Month
27–29	HFA Symposium in Tampa, FL

April

24–26	Region VIII Meeting in Tucson, AZ
15–17	NHF Chapter Training, Las Vegas, NV
17	World Hemophilia Day
26	**Hemophilia Walk of New Mexico at Tiguex Park in Albuquerque**

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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Inhibitor Education Summits 2013

By Charity Marquez

The National Hemophilia Foundation (NHF) hosts Inhibitor Education Summits each summer. This year, families affected by inhibitors gathered in Seattle, Washington and Nashville, Tennessee to learn from clinicians who specialize in inhibitors and to connect with families from across the country who are living with inhibitors.

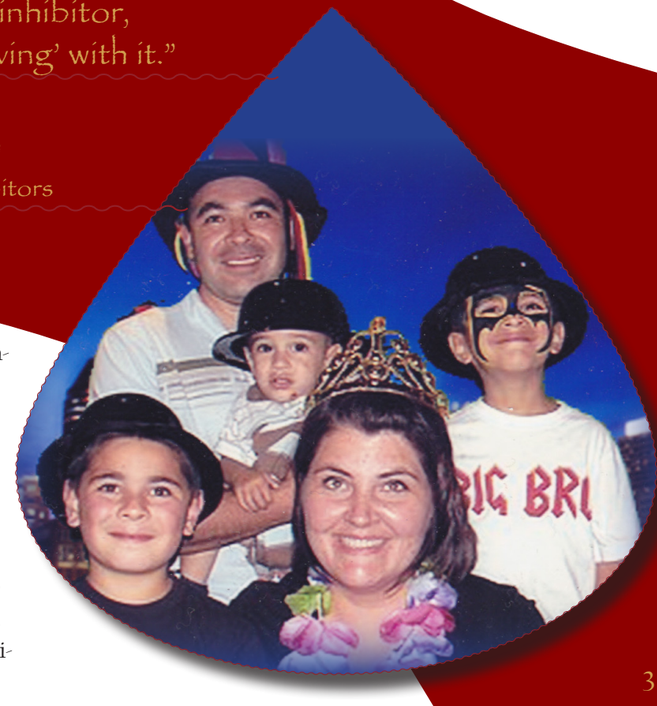
Information is broken down into four tracks. Track 1 is for those just beginning to learn about inhibitors, Track 2 is for those with prior experience with inhibitors, Track 3 is geared toward teenagers and young adults living with inhibitors and for their siblings, and Track 4 is for those adult men living with hemophilia and inhibitors.

Breakout sessions within the tracks focus on a wide range of information. Some of the choices this year were sessions on immune tolerance; sessions designed to teach about the challenges with specific joints, such as the elbows, ankles, or knees; a session about prophylactic treatment with bypassing agents; and a new session for this year about spirituality and its role in dealing with an inhibitor. Participants can pick and choose the topics and sessions that will be of most benefit to them.

Childcare is also provided, along with fun activities for younger children. This year, the kids participated in music therapy and learned about the components of blood. They made a snack with Cheerios, raisins, marshmallows, and pretzels. They were also offered the opportunity to participate in a martial arts session along with the entire family.

To end the conference, there was a dinner and family fun night where the children could dance and play with one another before leaving to go home. In addition to education, the families also got the chance to connect with one another and meet other families dealing with struggles similar to their own. They are encouraged to share their stories and information, and many families keep in touch with one another long after the summit is over.

> "After some years of trying to 'treat' the inhibitor, we finally started 'living' with it."
>
> – Mother of a child with hemophilia with inhibitors



Novo Nordisk is helping people with inhibitors realize their dreams.

changing possibilities in hemophilia™

Novo Nordisk offers financial, educational, and community support programs to people with hemophilia A or B with inhibitors so they can live more normal lives.

Find out more about how you can change your possibilities by calling **1-877-668-6777** today!

For more information, please visit **ChangingPossibilities-US.com**.

novo nordisk®

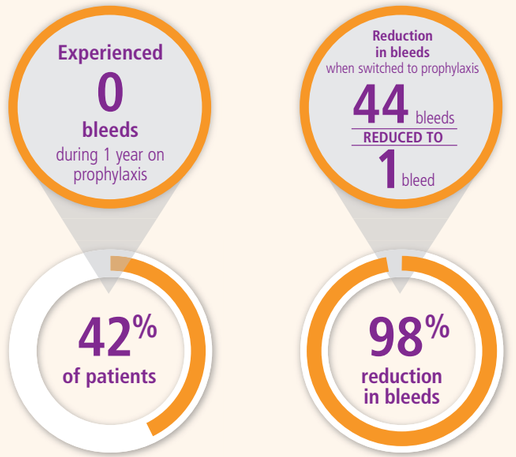
Baxter

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

Going Through Changes?

By Ken Wagg

"My insurance sent me a letter saying I have to use a different pharmacy."

During the past couple of months there have been many changes in pharmacy providers for the bleeding disorder patients of New Mexico.

Change Is Hard: Do Your Research

- Call the insurance company and the Pharmacy Benefit Management (PBM) company and ask the following questions:
  - *Can I continue to use the pharmacy I have always used?*
  - *What are the financial risks if I stay or if I move to the provider I have been directed to use?*
  - *Is there any appeal process for this decision?*
- Call your current pharmacy provider and discuss the information with them. Ask whether they know of any options you haven't considered.
- Call the pharmacy you have been directed to use and ask questions about their services:
  - *How to I get factor?*
  - *If I need factor in an emergency, how do I get it?*
  - *If I need a nurse to administer the factor, how do I get these services?*
- Call your Hemophilia Treatment Center and discuss the information with them. If you are moving to another pharmacy, your physician will need to call a new prescription into the pharmacy in order for you to receive services from them.

"A different pharmacy called and told me I have to get my factor from them from now on."

"My current pharmacy said I do not have to change."

Lastly, do not delay setting up your services with a new pharmacy if you do have to move to them.

"I met some people from a different pharmacy and they told me I could get my factor from them now."

Processing prescriptions is complicated, and if you need your factor in an emergency, you don't want to have any delays. In addition, the insurance company may have a lengthy approval process that adds to the delay.

James Hamilton Memorial Scholarship Fund

In order to be considered, the applicant must provide evidence of the following by submitting pertinent copies of personal records to the scholarship committee chair or designee.

Scholarship Eligibility Criteria

- Be a person with hemophilia
- Be a New Mexico resident
- Be a U.S. citizen or an eligible non-citizen

Other Requirements

- Provide a valid social security number
- Provide proof of application for student aid (FASFA)
- Demonstrate financial need, as evidenced by the FASFA form
- Provide evidence of application to two additional sources of financial aid assistance
- Provide a copy of a high school diploma or GED
- Provide evidence of enrollment or acceptance for enrollment as a regular student working toward a degree or certificate in an eligible program
- Submit completed scholarship application form to Sangre de Oro, Inc.
- Submit a letter of reference from someone, other than a family member, who knows you
- Have a personal interview by the scholarship committee or chairperson
- Must meet deadline for semester of request

Scholarship Renewal Requirements

- Provide evidence of satisfactory academic progress by maintaining a minimum 2.5 GPA out of a possible 4.0 to be considered for scholarship renewal. Official transcript showing final, current grades must be submitted when requesting a renewal of this scholarship. **If your GPA falls below a 2.5, you can reapply the following semester!**

Application Deadlines

Fall semester deadline: August 1st

Spring semester deadline: December 1st

2013 Hemophilia

Camp Sangre Valiente

for our children, affected and non-affected, to have the freedom to experience life without the constraints of their illness. It is always a joy to watch the friendships build and the personal growth at camp. Life changing experiences happen at camp."

And what do the campers have to say about their camp experience?

"The ropes course was awesome."

"My kids LOVE your camp and they are still talking about it. Thank you for all your hard work. We are looking forward to next year."

"I liked camp because I learned to infuse myself and then was able to get my port removed."

"I liked the water slide the best!"

"My friends that I made are Chris and Miguel. I cannot remember all the names but I made a lot of friends."

"I thought the camp was a learning experience. I liked meeting new friends and having fun. The friends I made were Alic, Eli, Mark, and many others."

"Hemophilia camp was epic."



9th Annual

by Cazandra MacDonald

Great memories are made at summer camp. Most people remember horseback riding, camping out under the stars, swimming, and making great friends. What most people do not experience at summer camp is learning how to infuse.

Our fantastic Hemophilia Treatment Center staff ensures that our campers are safe and well cared for. Claudia Mackaron, one of our fantastic nurses, helps to head up the medical clinic at camp. She shared some thoughts upon returning from camp. A big thank you to Claudia and the team for taking care of our boys!

"We had 7 new boys that were ages 6–7 who were learning for the first time to self-infuse. Out of the seven, four successfully self-infused and were awarded the bravery stick! The remaining three were so close and extremely brave. I know next year they will get the bravery stick. They just need some more practice and a dose of self-confidence. I am so proud of all the kids that were so diligent in taking care of themselves.

We had a new experience this year. Two older siblings were very interested in learning to infuse. One of our expert counselors, Alfonso, accepted the challenge to teach them and they practiced infusing on him! It was exciting to see their enthusiasm and successful experiences. Their brothers watched as they were learning and now know that they have someone else in their families that can assist if they have difficulty or need a break.

The campers had their bumps and scrapes but kept going on in fear of being left out of the many fun and action-packed activities. We learned about head bleeds and had a fun experiment of blowing up heads made out of paper mache using water and balloons to demonstrate what a head bleed was all about. Another activity that our medical team taught was the subject of sports and the need for prophylaxis. We shared with the campers the guidelines from NHF regarding safe versus unsafe sports for bleeding disorders.

The siblings also experienced the steps of infusing using plastic gloves with a sponge and water balloon looking like a vein. They practiced aseptic technique in preparing to infuse along with the poke using a real butterfly needle. The younger campers had a fun time poking and pretending they were infusing another person and seeing the red fluid (red-dyed water) coming into the syringe.

Camp is a wonderful place

Walk of New Mexico

We Will Walk the Extra Mile!

By Joe MacDonald

I thought that I had done my part. I organized a Walk team and have been going about the business of reaching my goal. I struggled to shrug off the idea that there was something I was not doing to ensure the success of our Walk. As I continued the business of an average day, I finished all of my commitments in time to pick up my youngest son from school.

As I was walking into the school, the idea hit me. I said to myself, "Self, have you asked your son's school to form a team on his behalf?" I grew a little more excited and began to ponder the possibilities of asking my youngest son's school, but what about my oldest son? Voila! There are two more teams. I didn't even have to try really hard to increase the success of the Walk. Two new teams. Two new fundraising goals. Wow. It really isn't that difficult!

What are you doing to make your goal a reality? How are you looking beyond your goal to something bigger?

I would love to hear from those leaders who are going the extra mile to ensure the success of our fundraising efforts. Remember, every dollar raised is used to empower a family. Together, we can make a difference in our world.

Peace,

Joe

About Inhibitor Family Camp

There are currently 50+ camps across the country that serve the bleeding disorders community; however, none address the unique needs of those living with inhibitors.

Until now...

This program offers a full weekend of education, support, and fun designed specifically for children with hemophilia and active inhibitors. Because we understand the value of having a tight-knit unit of support behind these children, Inhibitor Family Camp is also geared towards the entire immediate family.

Registration is now open for the Victory Junction, North Carolina Camp, October 17-20.

For more information visit www.comphealthed.com.

DON'T MISS OUT—MARK YOUR CALENDAR NOW!

NHF's 65th Annual Meeting
October 3–5, 2013
Anaheim, CA

NHF's 66th Annual Meeting
September 18–20, 2014
Washington, DC

PSI

PSI, Inc.

Patient Services Incorporated (PSI), a 501(c)(3) non-profit, charitable organization, is the ground-breaking premium and co-payment assistance foundation for the chronically ill.

If you would like more information about the financial help available from PSI, please call 1-800-366-7741.

First Time's A Charm

By Hanukkah "Rea" Watson

We knew this day would come soon. Our 7-year-old, Benjamin, seemed just as excited as he was nearly 3 years ago when he overheard a few of his older buddies talking about their camp experiences. The icing on the cake was seeing and hearing about all that went on at camp during a camp presentation hosted by SDO at a Patient and Family Education Weekend.

Benjamin's eyes lit up with excitement as fun-filled photos flashed across the projector screen. It seemed like he could not wait to go, even at age 3. "Ben," I said, "you just have a few more years before you can go. It will be time to go before you know it!"

Time flew and the day to send our little man off to camp was here. My husband and I knew in the back of our minds that this was not any ordinary summer camp. This was a rite of passage for little ones and teens with a bleeding disorder to get a taste of independence and have the opportunity to start taking part in their healthcare. After all, I couldn't be expected to follow him around his entire life to give infusions, right?

We had accepted that this would probably be quite a life changing event for our Benjamin. For years, my husband and I were the ones infusing him and managing his healthcare. We were the ones advocating for his quality of life and instructing others of his do's and don'ts. We had come to the enlightening realization that it was time! Time for hemophilia camp! Time for Ben to come into his own.

Ben had expressed interest in self-infusing for a few years, but as protective parents we didn't really think he could stomach actually infusing himself.

After nearly 5 years of experiencing a pain-free Broviac, that required absolutely no pokes, the Broviac malfunctioned. The next step was to have a port inserted when Ben was about 6 years old. As frightened as I was to have to learn to put on a brave face and stick a needle into our son, I did what any mom would do ... I wept privately, and his dad and I both encouraged him on how much more independent he would become by having a port. It was not easy.

For months, we coaxed Ben into his dad's lap to help ease his anxiety and mine. Then, one day, for the first time, he vocalized his bravery and asked to not sit in dad's lap. For the first time he had spoken up for his own independence. Ben had a second port in nearly a year's time and in that year, he has miraculously become his own advocate. As a first grader, he lets his teachers and classmates know his do's and dont's.

You would think that with all of this behind us I would be gung ho and ready for my little champ to spread his wings for the first time and leave for camp. Absolutely not! I had to put on my brave face again and accept that I was more afraid for myself than I was for him. There was never a question as to the level of care he would receive. We knew the medical staff was experienced, and the counselors were trained, but the fear of him leaving for the first time was very real. We had to let Ben go and experience a week without Mom and Dad.

After returning with a face filled with sheer joy and amazement, Ben assured us that his week was overflowing with fun and games as much as it was about a week of freedom. We now can say that not even hemophilia, with all its bumps, bruises and tears, will hold our son back.

Experimental Hemophilia B Gene Therapy Tricks the Immune System

In July, researchers at The Children's Hospital of Philadelphia (CHOP) published the results of a study in which they used bioengineered decoys as a ruse to foil the immune system. This allowed for the successful delivery of gene therapy in mice with hemophilia B, or FIX deficiency. The lead author of the study was Katherine High, MD, director of CHOP's Center for Cellular and Molecular Therapeutics.

High and her team used capsids, the protein shell that encapsulates a virus, as the decoy. They also used adeno-associated viruses (AAVs) as delivery vehicles, or vectors, to carry the actual genetic material that triggers the production of factor IX. AAVs have recently become the "vector of choice" for researchers because they can deliver the genetic material into living cells to sustain therapeutic effect without causing disease. In addition, they can be targeted to liver cells, which manufacture FIX.

However, one drawback in using injected AAVs is the body's natural immune response. In some cases, antibodies are released, neutralizing the AAVs. To remedy that, investigators injected both empty capsids and genetically altered AAVs into the mice. The empty capsids effectively drew antibodies to them and away from the gene therapy.

"This decoy strategy could be individualized to patients and could greatly expand the population of patients who may benefit from gene therapy," said High. "Right now, 30 to 60 percent of adult patients develop antibodies that block the ability of an intravenously infused vector to reach the target cells in the liver. This approach holds the promise of overcoming this roadblock--pre-existing antibodies--and allowing successful intravenous gene therapy in virtually all adult patients."

This technique also proved effective in follow-up studies performed in rhesus macaque monkeys. The therapy initiated higher levels of factor IX production, with no adverse events reported. Although additional studies, including clinical trials in humans, will be necessary before such a therapy becomes a reality, the authors are encouraged by these preliminary findings.

"Our results, which held up over a range of doses, suggest that in clinical studies, it will be feasible to adjust the ratio of empty capsids to gene vector doses, depending on an individual's pre-existing level of neutralizing antibodies. That means we could personalize gene therapy to make it more efficient for each patient," concluded High. "This work should make it possible to bring effective gene therapy to most adults with severe hemophilia B. Each patient would receive a personalized final formulation that contains just the right amount of empty capsid to neutralize any pre-existing antibody, and allow the gene-expressing vector to reach the liver."

The study, "Overcoming Pre-existing Humoral Immunity to AAV Using Capsid Decoys," was published in the July 2013 online issue of Science Translational Medicine.

Source: ScienceDaily, July 17, 2013

Do you know about the blog "Infusing Love: A Mom's View?" The HFA is hosting this new blog written by mothers of children with a bleeding disorder. New Mexico is represented by Cazandra MacDonald who will be a guest blogger. Visit www.hemophiliafed.org and check out the "News & Stories" tab.

HFA Symposium is an annual community-centered educational event. Symposium draws approximately 400+ patients and parents, 100+ exhibitors, 20+ speakers, and over 50 community volunteers from almost every state across the country!

HFA awards over 100 travel scholarships to first time attendees each year for families who need financial assistance. The national meeting is a place where community members come together to share information and build a network of support in a relaxed environment.

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

The National Hemophilia Foundation (NHF) is looking forward to seeing you at its 65th Annual Meeting, "United in Progress," October 3–5, 2013, in Anaheim, California. Our yearly gathering is an event individuals and families look forward to all year long. Annual Meeting "regulars" know that during this three-day conference, they will have an abundance of educational sessions to attend, fun social activities to look forward to, and downtime to relax and unwind. Our chapter staff, industry partners, and healthcare providers also anticipate reuniting with each other and with individuals and families with bleeding disorders.

This year NHF is providing 44 educational sessions to entice you:

- Genotyping Initiative Update
- Gene Therapy Clinical Trials
- Can We Talk? Moms, Daughters, and Bleeding Disorders
- Athletes in Actions; Safe Sports Training
- Liver Disease Interventions and Hepatitis C Updates
- Ask the Experts in Espanol
- Von Willebrand Factor Deficiency: Challenges for Women and Girls
- Mind/Body Connection: De-Stress with Alternative Therapies.

And from toddlers to teens, we have programs planned for each age group. The popular Babycare/Activity Program for Kids and Teens fills up fast, so be sure to register your children early.

So if California is calling your name, you'd better listen! It won't be long before we'll be heading West for a coastal connection at NHF's 65th Annual Meeting, October 3–5, 2013. Hope to see you there!

Global Genes

by Cazandra Campos-MacDonald

www.globalgenes.org

The Global Genes Project, run by Team RARE (which stands for Raising Rare disease Awareness, Research and Education) is one of the leading rare and genetic disease patient advocacy organizations in the world. The non-profit organization is led by Team RARE and promotes the needs of the rare and genetic disease community under a unifying symbol of hope: the Blue Denim Genes Ribbon™. What began as a grassroots movement in 2009 with a few rare disease parent advocates and foundations has grown to over 500 global organizations.

Rare and genetic diseases affect 1 in 10 Americans, 30 million people in the United States, and 300 million people globally. Over 7,000 distinct rare diseases exist and approximately 80 percent are caused by faulty genes. The National Institutes of Health estimates that 50% of people affected by rare diseases are children.

Webinar Series

Global Genes | RARE Project, in partnership with Shire and Bayer, will be hosting a series of educational webinars geared towards educating patient advocates and caregivers on a variety of topics. Please join subject matter experts in discussions about pressing issues that affect rare disease patient advocates, regardless of disease. There is a lot to be learned and many benefits to creating a platform for sharing best practices.

This RARE Educational Webcast Series is an important resource for those looking to become more involved and empowered by exploring topics related to rare disease advocacy.

- Webinar #1: Importance of Newborn Screening Awareness
- Webinar #2: Understanding Drug Development
- Webinar #3: Essential Health Benefits
- Webinar #4: Understanding Rare Disease Registries

Visit http://globalgenes.org/webinars/ to view webinars.

Rare diseases (disorders) affect all family members. The Global Genes Project hosts an up-to-date blog with stories from parents who are raising children with a rare condition as well as individuals who share their struggles with rare diseases.

To read the blog visit http://globalgenes.org/blog/. To become a guest blogger visit http://globalgenes.org/category/guest-bloggers/.

Remember that hope begins with you.

Back to School

by Cazandra Campos-MacDonald

Ah, back to school. The smell of new pencils and backpacks, new socks and shoes, spiral notebooks and crayons. It is time for the kids to get back into a routine. One of the most important parts of returning to school is meeting with the school nurse and staff at your child's school. Even if your child is a teenager or going to college, it is important for school personnel to know about your child's bleeding disorder. Here are a few reminders as you begin the school year.

- For elementary-aged children, schedule a meeting with the teacher, physical education teacher, nurse, counselor and principal. These are key personnel that will help educate the other staff members caring for your child each day.
- Keep your meeting brief and positive. Often times school personnel have never experienced a child with a bleeding disorder. Encourage the caregivers and let them know that you all are a team helping each other. In time they will feel more comfortable if you are readily available to help them understand the condition.
- Develop a brief presentation for your child's school meeting. If you need help, call the Hemophilia Treatment Center at (505) 272-4461 and ask for Elaine, Claudia, or Brie. They will help you pick out the key components to share regarding your child's bleeding disorder. Included in this issue is a sample "Instruction Sheet" that a mother in the community developed for her son's school meetings. It is a snapshot of what to look for. Feel free to use this as a guide to help you develop your own sheet. It is not comprehensive. There is quite a bit more that may be added, but it is a good stating place. Also visit www.livingwithhemophilia.com and click "On the Go" for a great article on Back to School, as well as a sample tip sheet to take to your school meeting.
- Leave educational materials for school personnel to read. You don't want to leave an entire book, but perhaps a few pamphlets (or a list of web sites). If you need help obtaining materials for your school staff, please contact the HTC or the chapter.
- Middle and high school students should meet with the school nurse on their own. Help your children to understand that even if they do not want the entire student body to know about their bleeding disorder, it is very important for someone to know about their condition. This is the age where children begin to fully take responsibility for their bleeding disorder, and it is crucial. Let them take the lead.

For more information on Back to School issues visit:

- *http://www.hemaware.org/story/back-school-bleeding-disorder*
- *https://www.livingwithhemophilia.com/webapp/on-the-go/articles/tips-for-back-to-school.jsp*

2013 HFA Symposium

by Charity Marquez and Lori Long

The Marquez and Long-Holderried clans ended up at HFA this year and had a wonderful time! Here are some of the things we enjoyed at HFA:

HFA is the place to be if you want to be "In the Know" about the issues that affect the people/families with hemophilia

- Learning. Lots of learning!
- Heated conversations between community members and providers regarding inhibitors and inhibitor development.
- Passionate discussion about genotyping.
- Great place to learn the most recent information regarding the subjects that impact our community and our families most and discuss them amongst ourselves and with hemophilia leaders.
- Opportunity to learn about new resources available, i.e. Hope for Hemophilia, resources for women with bleeding disorders, microhealth, bleeding log apps.
- Opportunity for our kids to talk to older bleeders and express how they feel about their disorder.

Fun

- Chance for people living with hemophilia to talk with others and not feel alone. There are others with similar struggles.
- Hugs! Lots of hugs!
- Chance to hang out with our blood brothers and sisters!
- Chance to "compare notes" on things that work and don't
- A night out for the family at the ball game: balloons, cotton candy, new ball caps!
- Kids get to play with other kids who are "in the same boat"
- Our kids making new friends.
- Our kids doing activities that teach them about dealing with their bleeding disorder.

Opportunities to get assistance to attend

- HFA scholarship to attend (check www.hemophiliafed.org)!
- Chapter scholarships to attend (e-mail sdo@sangredeoro.org)!

The 10th Annual Caliente Classic Golf Tournament

Title Sponsor: Distribution Management Corporation (DMC)

By Joe MacDonald

Many thanks to our generous sponsors:

- AA Auto and Air Conditioning
- Aerolynx Aviation
- AJ Gallagher
- Auctoris
- Aviall
- Bank of the West
- Baxter BioScience
- Bayer HealthCare
- Southwest CornerStone
- Coram
- CSL Behring
- DMC
- Enterprise Holdings
- Koch Construction
- Linton and Associates
- Majestic Forms
- Novo Nordisk
- One Stop Aviation
- Pepsi Beverages Co.
- Pfizer
- REDW
- Ricci Porch & Company
- Select Staffing
- Southwest Airlines
- The Noel Company
- The Payroll Company
- US Bank
- Weck's

We had our tournament in honor of the Cordova family. Loretta was able to attend some of the festivities, and it was wonderful to see her!

Congratulations to our winners, **Steve Hernandez of AA Auto and Air Conditioning (playing for DMC), Stan Hockerson, Rick Brklacich, and Matt Wisely**! Thanks to the winning team for generously donating their winnings to support camp!

While we had many challenges putting together the tournament this year. We also had a lot of help, especially from Dawn Bodenner at DMC. The Caliente Classic plays an important role for the Sangre De Oro Chapter. Without the funds raised from this event, many of the members of our bleeding disorders community would not be able to attend summer camp. This camp is the "life blood" (so to speak) to those who live with bleeding disorders. It is at camp that many of our children meet and form lifelong relationships with those who share the experience of having a bleeding disorder. Children learn that they are not alone and that they have a resource to help them manage and treat their respective bleeding disorders.

Many of the people who participated in the event, either as players or volunteers are actually members of our community. I send my profound appreciation to these mighty men and women who continue to give back to their community. Fundraising efforts proved more than just a financial means to an end. The golf tournament brought us together.

I have participated in this event for approximately 5 years now, and I can honestly say that each year I am very humbled when I see the amazing men and women who give of their time and energy to support our community. There is a connection to something that is greater and bigger than who we are alone. I am grateful to all of the volunteers who dealt with my endless e-mails and constant changing of the schedule. Thank you for sharing your time to make my sons' lives a little easier.

Special thanks to DMC, our Title Sponsor, for the past 8 years, for making a difference in so many lives!

Instructions for _____
(Name of student)

_____ has _____. He/she is infused with _____.
(student) *(condition/severity)* *(product/days infused)*

1. **Minor cuts/scrapes:** _____ should be treated in the same manner as any other child at all times.
(student)
Clean the area and cover with a band-aids. **He/she will not bleed more than anyone else,** only longer.

2. **Mouth bleeds**: Please call _____ . Remember that when blood and saliva mix, it may look worse
(caregiver)
than the actual injury usually is.

3. **Head injuries**: As it would be for any other child, a head injury is serious. A normal bump on the head is one thing, but a fall that would make you gasp is extremely important for us to know about, especially if blood or a lump/bruise is evident. **Call** [*caregiver*] **immediately.**

   We will ask you questions such as "What was he doing? How far did he fall? How big is the bump (e.g., the size of a nickel, quarter)?" Use a pen to mark the size of the bump if it seems to be growing larger.

4. **Joint bleeds**: If he/she is limping or not using an arm or hand, touch the joint. If it is warm to the touch, a joint bleed may be occurring. **Call** _____ **immediately.**
(caregiver)

Bleeding disorders sound very overwhelming, but they are part of our everyday life. If you are in doubt, please call _____.

Important Phone Numbers (for staff to reach caregivers):

The SDO Board

Executive Board Members

Lori Long, President

Joe MacDonald, Vice President

Jose Guillen, Secretary

Eric Marquez, Treasurer

Loretta Cordova, Executive Director

Board Members

Chuck Boberschmidt

Johanna Chappelle

Robert Farias

If you wish participate on the board, please call 341-9321 or contact us at sdo@sangredeoro.org.

The 2013 Family Education Weekend

By Lori Long

Our 2013 Patient and Family Education Weekend will be held at the beautiful Embassy Suites in Albuquerque.

You can register to attend on our web site at www.sangredeoro.org. **Online registration must be completed no later than October 8th to guarantee a room.**

Be sure to also download and fill out the registration forms for your children if they will be attending. You can turn them in when you check in for the weekend. We will have fun activities and programming for them!

Check-in starts Friday, October 18, at 4 p.m. The event runs through Sunday, October 20. We will have some great presenters and speakers and opportunities for visiting with old friends.

We hope to see you there!

October 18–20, 2013

Where: Embassy Suites, 1000 Woodward Place NE, Albuquerque, NM 87102

What: A chance to learn about bleeding disorders and catch up with old friends

FDA Grants Orphan Drug Status to Alnylam's Hemophilia Therapy

In August, Alnylam Pharmaceuticals, Inc., announced that the US Food and Drug Administration (FDA) had granted Orphan Drug Designation to ALN-AT3 for the treatment of hemophilia A. The company, based in Cambridge, Massachusetts, is developing ALN-AT3, a subcutaneously administered (injection just under the skin) RNAi therapy that targets antithrombin (AT) as a way to treat hemophilia A or B (with or without inhibitors) and other rare bleeding disorders. AT is a small plasma protein molecule that inactivates factor Xa and thrombin, which are needed for blood clotting.

ALN-AT3 incorporates Alnylam's proprietary gene-silencing technology called RNAi, or RNA interference. Discovered by scientists in the late 1990s, RNAi is a natural process in which cells turn off, or silence, the activity of specific genes. ALN-AT3 silences certain genes associated with AT generation, "switching off" the protein's production.

At the XXIV Congress of the International Society on Thrombosis and Haemostasis, June 29-July 4, in Amsterdam, Alnylam shared preclinical data from animal trials. The studies revealed that ALN-AT3 improved thrombin generation in mice and nonhuman primates.

Alnylam plans to file an investigational new drug application for ALN-AT3 in late 2013. It will initiate a Phase I clinical trial in humans in early 2014.

"We are very pleased that the FDA has granted Orphan Drug Designation for ALN-AT3 now for both the treatment of hemophilia A and hemophilia B. As a subcutaneously delivered RNAi therapeutic, we believe it represents an innovative approach for the management of hemophilia and has great potential to make a meaningful impact in the treatment of this often debilitating bleeding disorder," said Saraswathy (Sara) Nochur, PhD, Senior Vice President, Regulatory Affairs and Quality Assurance at Alnylam. "ALN-AT3 is a key program in our 'Alnylam 5x15' product development and commercialization strategy, and we look forward to advancing this promising RNAi therapeutic into the clinic in the months to come."

Sources: The Wall Street Journal, August 14, 2013, and Alnylam news release dated August 20, 2013

President's Corner

Dear Community,

Many of you have already heard that our beloved Executive Director, Loretta Cordova, has been diagnosed with cancer. While she and her doctors work toward regaining her health, she will be on a leave of absence. If you would like to send a card, please contact us.

While Loretta is on leave, the board will be conducting her duties. To that end, we have a new e-mail address. Our phone number will remain the same (505.341.9321). Our new e-mail is sdo@sangredeoro.org. Please contact us anytime!

On a happier note, we had a fabulous inaugural Hemophila Walk of New Mexico! We made $34,780! Thanks to everyone who participated! We had a great time, and we're hoping to have an even bigger Walk in 2014! Please see the information on page 1 and join us for the 2014 Hemophilia Walk of New Mexico. Have a team! Volunteer! Donate! Every penny we earn will remain in New Mexico to support our blood brothers and sisters here.

In August, we had our 10th Annual Caliente Classic Golf Tournament. Our wonderful Past President, Johanna Chappelle, was our Golf Chair, and she hit it out of the park! We had a very successful tournament and raised enough funds to pay for our 2014 Sangre Valiente Camp.

We had another successful camp in June. We had our youngest kiddo infuse himself for the first time at the age of 6! He was so excited about it, he asked to have his port removed at his very next clinic visit!

NHF will have its 65th Annual Meeting October 3-5 in Anaheim, CA. Several individuals from New Mexico will be attending. If you have never attended this event, watch their web site for opportunities to apply for scholarships. NHF's 66th Annual Meeting will be September 18–20, 2014 in Washington, DC.

HFA will have its Symposium March 27–29, 2014 in Tampa, FL. HFA also sponsors patients to attend, especially first-time attendees, so watch their web site as well. 2014 will be HFA's 20-year anniversary, so this Symposium promises to be even more amazing than usual!

October 18–20, we will have our 2013 Patient and Family Education Weekend. We hope to see you all there for some learning, networking, and fun!

Have a safe and happy holiday season, and watch for updates on our Walk!

Land Softly,

Lori Long
President