Factor IX Summer Family Meetings  By: Wayne Cook

This summer, we held our Factor Nine Family meetings in the mid-western part of the United States. Our first stop was FAMOHI0 in Columbus, Ohio on August 1, 2008. The meeting was wonderful! I had the chance to speak to a group of about 25 individuals. We spoke on various topics ranging from insurance issues to joint replacement. We held our family round table discussion where everyone introduces themselves and share a little bit about their lives and challenges they face. This has always been very popular with our groups, because it gives individuals a chance to meet and interact with other members of the Factor IX community and support each other. We also played the game that was initiated at our annual symposium earlier this year, “Are You Smarter Than Your Hemophilia.” The group had a good laugh and learned some new things that they didn’t know about Factor IX. I had a wonderful time in Ohio and I look forward to returning next year.

The weekend of August 8-10, 2008 was in Lincoln, Nebraska. This year marked my third year attending a wonderful event put on by Carl and Sharon Clark and the Nebraska Hemophilia Chapter. Every time I attend their meeting, I feel as if I am a part of the Nebraska family. Their meeting was well attended by over one hundred individuals. They had wonderful speakers speaking on various topics from blood safety and drug interactions, to a gentleman called “THE SCARY GUY” who spoke on the topic of prejudice in the disabilities community. He was a great motivational speaker. Our breakout session was well attended this year by a group of about 15 individuals with Factor IX. Some topics discussed were joint replacement.

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Factor IX Summer Family Meetings  continued from cover

and continuous infusion, insurance issues, and transitions from the ‘tween years to teenagers and youth to adulthood.

The Hemophilia of Indiana held its Annual Meeting in Indianapolis during the weekend of August 22 - 24, 2008. This was my second year attending their annual meeting, and it seems to be getting better each year. Last year, we had an impressive turn out of Factor IX families and individuals, but this year was even better. I had the opportunity to sit down, meet people from different cultures, and speak with them on their issues. I spoke to the group about current research. We had an insightful discussion with some of our older individuals about other health topics. New parents interacted with our group’s older Factor IX gentlemen and discussed how things were from years past. We spoke about transitions; and what to do when children enter adolescents and do some of the wild and crazy things that they do. Towards the end of our meeting, we played the “Are You Smarter Than Your Hemophilia” game with lot of laughs. We closed with an awesome group photo. All in all, we had a great session with positive feedback from the people in the group.

The Coalition for Hemophilia B will be in Cedar Rapids, Iowa during the weekend of October 24 -28, 2008. There, we will attend the Iowa Annual Meeting and hold a Factor Nine Family meeting. We hope to see all of our Iowa members!

Industry News

CLS Limited

CSL Limited, the parent company of CSL Behring, has signed an agreement to acquire Talecris Biotherapeutics, one of the world’s major suppliers of plasma-derived protein therapies for serious and rare conditions. CSL plans to combine Talecris’ business into CSL Behring. Peter Turner, President of CSL Behring will head the business.

This transaction will benefit patients and customers in a number of ways, which include better assurance of product supply through the most robust plasma collections capabilities, greater efficiency, enhanced capacity by removing bottlenecks and through superior growth, consumer and physician choice through a broad product portfolio and enhanced R&D pipeline, overall operating efficiencies that will facilitate strong investment in safety, quality, state-of-the-art manufacturing, R&D and Plasma collection to better meet customer needs.

CSL Behring remains strongly committed to providing high-quality therapies that save and improve the lives of people around the world and will continue to support patient organizations and activities at responsible levels that benefit patient communities.

Novo Nordisk

Novo Nordisk and Neose Technologies, Inc. have announced the completion of the initial phase I clinical trial for NN7128 (GlycoPEGylated factor VIIa), a longer-acting version of NovoSeven®, Novo Nordisk’s existing recombinant factor VIIa (rfVIIa) product. NN7128 is being developed under a license agreement with Neose, a clinical-stage biopharmaceutical company based in Horsham, PA.

To extend the half-life of the new drug, Novo Nordisk’s (rfVIIa) protein is attached with polyethylene glycol (PEG). The process, known as pegylation, prolongs the drug’s presence in the body. Neose’s proprietary GlycoPEGylation technology serves this purpose. The goal of the trial was to measure the pharmacokinetics and safety of NN7128 in 30 healthy subjects. The companies reported that a “significant” prolongation of the drug’s half-life was noted, and that single doses of NN7128 were well tolerated.

“We are pleased to report the successful completion of this Phase I study and to have demonstrated a prolonged half-life of NN7128,” said Soren Bjorn, Corporate Vice President, Biopharm Research at Novo Nordisk. Novo Nordisk is currently continuing analysis of their data. We will keep you updated in upcoming issues of Factor Nine News.
Biovitrum AB of Sweden and Syntonix Pharmaceuticals, Inc. have started a Phase I/IIa clinical study of a new longer-acting factor IX concentrate. The product is a recombinant factor IX molecule attached to the Fc portion of a human antibody molecule. An antibody molecule is shaped like a Y, and the Fc portion is the base of the Y. The Fc portion is the same in all human antibodies. The upper arms of the Y, called Fab fragments, vary from antibody to antibody and are the parts that bind to bacteria, viruses and foreign proteins. Using genetic engineering, a recombinant version of only the Fc portion can be produced.

In the last few years researchers have determined that the Fc portion of antibodies binds to receptors called FcRn on the endothelial cells that line the inside of blood vessels. Interaction between Fc and FcRn is apparently responsible for regulating the half-life of antibodies in the bloodstream. The idea behind the new product is that by hooking the factor IX molecule onto the Fc portion of an antibody molecule, the half-life of the new factor IX molecule (FIXFc) would be increased to be more like that of an antibody molecule.

The half-life is the amount of time for a dose of factor IX or any other substance to decrease to half of its original level in the bloodstream. The average half-life of factor IX is about one day, while that of antibodies is about 20 days. The half-life of FIXFc in animals has been shown to be three to four times longer than that of the currently-available recombinant Factor IX. The half-life in humans is not yet known – that will be determined in the clinical study. However, if it is much longer than one day, the frequency of dosing for prophylactic and other types of treatments should be reduced significantly.

To be permitted to start the clinical study in humans, Biovitrum and Syntonix have already shown that FIXFc appears to be safe and effective in animals. However, until it is actually tested in a clinical study, the safety and effectiveness in humans is unknown. Phase I of a clinical study is mainly to test the safety of a new product, while Phase II is the dose finding portion of a study in which the amount of a product needed to have the desired clinical effect is determined. In this case, Phase I and the first part of Phase II have been combined for a Phase I/IIa study in 14 volunteers with severe hemophilia B. They will look at the pharmacokinetics of the new product and watch for any safety-related issues.

If the results of the Phase I/IIa study are satisfactory, a Phase IIb study is usually conducted to verify the necessary dose in a larger number of patients. Then a Phase III study is performed to look at routine use of the product in a number of patients over a longer period of time. After successful completion of a Phase III study, the companies can apply to the FDA for licensure of the product. Pharmaceutical companies are not allowed to announce estimated dates for licensure of products. However, if there are no major problems uncovered in the clinical studies, FIXFc could be on the market in three to four years, based on usual experience.

Syntonix has developed other recombinant protein products using their SynFusion™ technology to attach proteins to Fc molecules. Some of the other products are inhaled and absorbed via FcRn receptors on the epithelial cells that line the lungs. That might also be possible eventually for FIXFc, but the initial product is for intravenous injection.

Both companies have interesting histories. Syntonix is a small biotech company in Massachusetts that was acquired last year by Biogen Idec. Biogen was one of the original recombinant technology companies and was involved in the initial development of recombinant factor VIII products for hemophilia A. Biovitrum is a remnant of KabiVitrum, one of the original European plasma fractionators. KabiVitrum produced one of the early plasma-derived factor IX products. Biovitrum now manufactures all of the recombinant factor VIII used by Wyeth to produce ReFacto and Xyntha for treatment of hemophilia A. As we have seen in recent issues, along with an increase in the number of patients being treated prophylactically, there are several innovative new factor IX products being developed. If even a few of these are successful, hemophilia B treatment could be entering a new era of improved quality of life for many patients.
A new program provided by The Center for Biomedical Continuing Education (CBCE) and supported by an educational grant from Novo Nordisk, has been designed to connect, inspire, educate, and empower adult patients with hemophilia with inhibitors and their primary caregivers (spouses, parents, and significant others).

Diagnosis and management of hemophilia with inhibitors can be overwhelming; even day-to-day life can often be a challenge. Many people feel they are “on their own” when living with hemophilia with inhibitors. The Adult Inhibitor Summit was created to provide an opportunity for patients and their caregivers to connect with others, discuss common experiences, and share support in the management of this difficult condition.

Currently scheduled Adult Summits:

- Adult Inhibitor Summit II
  November 7-9, 2008
  Chicago, Illinois

- Adult Inhibitor Summit III
  November 21-23, 2008
  Dallas, Texas

For more information or to register, please call CBCE at 888-706-6867 or send an e-mail to: inhibitorsummits@thecbce.com

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**In Memory of Margaret W. Hilgartner, MD**

Margaret W. Hilgartner, MD, Harold Weil Professor of Pediatric Hematology, passed away on August 19, 2008 at the age of 84.

She received a Master’s degree in Zoology from Duke University in 1951, and M.D. degree in 1955. She then moved to New York for a residency followed by fellowship training in Pediatric Hematology at New York Hospital with Drs. Carl Smith and Irving Schulman. It was during this time that she became interested in the newborn with coagulation problems and in the clinical care of children with hemophilia. She developed the first classification of hemophilic arthropathy with Dr. William Arnold at the Hospital for Special Surgery. With her colleagues Dr. Lou Aledort and Dick Lipton, she introduced self-infusion and comprehensive care to patients of all ages in New York City.

Her major research contributions were in the areas of complications of transfusion/infusion therapy, autoimmune responses, treatment of inhibitors, and the infectious complications of hepatitis and HIV. She was particularly interested in the effect of HIV virus on the growth and development of boys with hemophilia. She served as the Director of the Hemophilia Comprehensive Treatment Center from 1970 – 1995, and was the Division Chief of Pediatric Hematology/Oncology from 1978 – 1992. She also served the Children’s Blood Foundation as President from 1992-1995 and until she became ill, as an emeritus board member and medical advisor.

Dr. Hilgartner will be remembered for her inspirational leadership; her prodigious contribution to hemophilia and other pediatric hematological diseases and for the zest with which she embraced life.
It's your life. Wyeth is committed to making it better.

Wyeth lifelines™

An interactive, educational program for people living with hemophilia that includes:

- A bimonthly newsletter with important topics such as reimbursement issues, product updates, and tips on staying healthy.

- Educational materials with information about the manufacturing of recombinant products, frequently asked questions about hemophilia, and more.

- A patient feedback forum to create a true dialogue with the hemophilia community.

Convenient online registration at www.hemophiliavillage.com and via the Hemophilia Hotline (1.888.999.2349).

For more information about products, services, and programs we offer to the hemophilia community, visit our Web site at www.hemophiliavillage.com or call 1.888.999.2349.
In Mayfield, New York on Saturday August 23, 2008, the 7th Annual Adirondack Spintacular was termed “Spectacular!” by all involved! This event, whose purpose was to raise hemophilia and organ donation awareness, attracted over 350 participants from NY, MA, FL, VA and other locales. Proceeds from this event benefit the Lawrence Madeiros Memorial Scholarship Fund, awarded annually to graduating high school seniors living with a chronic disorder and continuing their educations at an accredited college or university.

Whether they laced up running shoes, filled bike tires with air, or just kicked back in a stroller, participants at the Spintacular were there to cycle, walk or run a 5K, one, six or twelve miles along a scenic route in the Adirondack Mountains and around the Great Sacandaga Lake. Positudes, Inc. and the Center for Donation and Transplant in Albany, NY were co-sponsors of the event. Following the Spintacular was a family style barbecue, silent auction, and numerous children’s activities including face painting, games, and an ever-popular bounce house.

“The riders..., the walkers..., the cheering fans..., what a fantastic day! We were fortunate to have two of the six scholarship recipients in attendance at the event; Jessica Abel and Michael O’Connor. Every year we give $6000 in scholarships to high school seniors with chronic disorders so that they may continue their educations. Thanks again and I look forward to seeing everyone next year!”

~ Carol Madeiros

Lawrence Madeiros Scholarship 2008 Winners

Jessica P. Abel, Niskayuna, NY SUNY College to study Teaching (Severe Autoimmune Neutropenia)
“I am looking forward to living my dreams and doing my best to make the world a better place. I am not going to let my blood disorder get in my way!”

Conor W. Coyne, Pittsfield, MA University of Vermont to study Pre-Med and Computer Sciences (Hemophilia A)
“Typically seen as limiting, hemophilia has actually opened more doors for me than it could ever close. . . I wouldn’t change a thing.”

Lauren L. Mancuso, Berwyn, PA University of Pittsburgh to study Nursing (Antiphospholipid Antibody Syndrome)
“The long-term dream is to become a nurse, travel the world helping the less fortunate countries gain medical care and knowledge . . .”

Adam C. Millard, Cypress, TX Texas A & M to study Genetics pursuant to medical school (Hemophilia A)
“One day, I hope to cure hemophilia, which will let me give back to the bleeding disorder community.”

Michael T. O’Connor
Cold Spring Harbor, NY Amherst College - Major undecided. (Hemophilia B)
“I have been lucky to live my life just as anyone else would, to even be recruited to one of the best NCAA Division III swim teams in the country - as an athlete.”

Lina E. Smith, New Hartford, CT Williams College to study Management and Psychology (Ulcerative Colitis)
“Having dealt with medical problems, I have benefited so much from my doctors and nurses that I hope to find a career in the health care field.”
As with all plasma-derived products, the risk of transmission of infectious agents, including viruses, and theoretically, the Creutzfeldt-Jakob disease (CJD) agent, cannot be completely eliminated.

**AlphaNine® SD**
Coagulation Factor IX (Human)

**Sample Program**

Conveniently provided in the following range of sizes

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<thead>
<tr>
<th>Size</th>
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Please contact your healthcare provider or Hemophilia Treatment Center to determine if you are eligible for the program.

Patients currently using AlphaNine® SD and/or have sampled AlphaNine® SD in the past are ineligible for the program. Subject to availability.

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Kirstin Duggan and John Samuel Wieber were married on August 31, 2008 at Clover Forest Plantation in Goochland, Virginia. Kirstin and Sam met at Radford University and now reside in Centreville, Virginia. Kirstin works as a Staffing Manager for Ajilon Finance. Sam works for a Budweiser distributor and is also the head volleyball coach for Centreville High School. Best wishes to the newlyweds from the Coalition for Hemophilia B!

Kirstin and Sam's parents, Nina and Ed Duggan

**March 20, 2009**
The Coalition for Hemophilia B
2nd Annual Fundraising Dinner
New York City
Honoring Dr. Christopher Walsh of Mt. Sinai Hospital, New York

**March 21, 2009**
The Coalition for Hemophilia B
3rd Annual Symposium
New York City

DETAILS TO FOLLOW!

**Save the Date!**

**Wedding Congratulations!**

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Autumn 2008 Factor Nine Family Meeting

Visit the Coalition for Hemophilia B Booth!
In conjunction with
National Hemophilia Foundation Conference
Hyatt Regency Denver at the Colorado Convention Center 800-223-1234
650 15th Street; Denver, Colorado

Factor Nine Family Breakfast Meeting
Saturday, November 15, 2008
8:00 - 9:30 am
Colorado Convention Center, Room 113; Street Level
303-228-8000
700 14th Street; Denver, Colorado
(Across the street from the Hyatt Regency)

Please join us in Denver for Breakfast on Saturday Morning!
We are pleased to present our fun new game called “Are you Smarter than Your Hemophilia” introduced at our New York Symposium this past March. Join our relaxed, open forum created to help people with Hemophilia B and their families gain support, share concerns, stories, and information. Take this opportunity to see your friends and meet new ones!

We look forward to seeing you!