



February, 2008

Miguel: Successful Treatment Through Comprehensive Care

Miguel is an engaging 11-year-old boy who attended hemophilia camp for the first time this year. Last year CCBD staff tried to help him enroll in camp, but his care was so complicated that each of the three Midwest hemophilia camps declined to admit him.

Miguel has severe hemophilia B. Most boys with this disorder receive recombinant factor IX concentrates twice a week to prevent bleeding into joints and other critical sites, which allows them to lead a fairly normal life. However, shortly after he received his first treatments with factor IX concentrate, Miguel experienced an adverse reaction and developed a high titer inhibitor that inactivates factor IX replacement treatment, making his hemophilia-related hemorrhages very difficult to treat.

Miguel's treatment became even more complicated because his inhibitor also causes life-threatening allergic anaphylactic reactions to factor IX products. He has suffered numerous bleeds requiring hospitalizations throughout his young life and has also experienced severe pain and chronic joint arthritis due to the many bleeds into his joints. Normal childhood activities like running or jumping cause joint bleeds



for Miguel—he was averaging at least one bleed a month. He couldn't participate in physical education at school and was putting on weight because of his repeated bleeds, joint pain, and restricted activities.

Because of his life-threatening allergic reactions, Miguel's inhibitor could not be eradicated with high doses of factor IX alone (which is the usual approach to immune tolerance therapy for most inhibitor patients). Miguel had previously tried this therapy plus Rituximab, a monoclonal antibody that kills

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Miguel (Continued from cover)

B-cells (white cells that make antibodies). Dr. Gill had hoped that by knocking out his B-cells in addition to giving high daily doses of factor IX infusions, he would be successfully tolerized and able to be treated with factor IX. However, within three weeks, Miguel's inhibitor returned. Once again he developed severe allergic reactions to factor IX.

Miguel's care was also extraordinarily expensive. In order to treat his bleeding episodes, Miguel required recombinant activated factor VII (NovoSeven), a product that can bypass the inhibited step in the clotting cascade so that a clot can form at the bleed site. A typical bleed costs about \$20,000 to treat. In 2006 Miguel's medical costs, including hospitalizations, exceeded \$2 million. In late 2006 his mother was notified that her employer was changing the company insurance plan from an unlimited lifetime amount to a \$1 million lifetime limit. His father works for a small company and Miguel's medical costs would have led to the cancellation of his company's plan. Although both of Miguel's parents work, they could not pay for his medical care without insurance coverage. They also were not eligible for the state high risk insurance option.

Because of her concern for his ongoing quality of life and the issues related to his medical costs and insurance, Dr. Gill proposed an intense immune suppressive treatment to Miguel's parents in late 2006 designed to eradicate the inhibitor and allow

Miguel's hemophilia to be successfully treated with factor IX concentrates. Because of the anaphylactoid nature of his inhibitor, the new treatment designed for Miguel included desensitization to circumvent the anaphylaxis followed by the initiation of standard high tolerization doses of factor IX. He also received the standard course of Rituximab, but this time a course of cytoxan (a strong cytotoxic chemotherapy drug) was given in order to try to inactivate his T-cells; Rituxan was continued on an every-other-monthly basis. In addition, because of the significant immunosuppression anticipated with this treatment, Miguel was started on regular replacement doses of IVIG to help him fight off any new infections. This treatment plan was coordinated by the CCBD comprehensive care team. The nurses (Jodi Haar and Brigit Cornale), social worker (Jane Volkmann) and financial counselor (David Linney) supported Miguel and his family through this very difficult therapy. We are happy to report that at each of his evaluations over the past 9 months there has been no sign of his inhibitor. He is now able to be treated with regular doses of factor IX to prevent bleeding.

When asked about his quality of life now, Angelica, Miguel's mother, says that his life is close to normal. He is no longer in pain and has not had a bleed since November 2006. Miguel is now able to go to camp and is happy to be doing all the normal things that children do.

ON THE WEB

The CCBD webpage is hosted on the BloodCenter of Wisconsin site: www.bcw.edu.

You can go to the main page and follow the tabs for :

BLOOD PRODUCTS & MEDICAL SERVICES and then **COMPREHENSIVE CENTER FOR BLEEDING DISORDERS**.

(Or you can type "**CCBD**" in the search field at the top right corner.)

Insurance Management Begins With Your Clinic Visits

David Linney

At your clinic visit, you are evaluated through a physical exam and laboratory testing. Based on your evaluation, an individual treatment plan is made. This plan is key for you to receive ongoing prescriptions and to assure the best management of emergencies and surgeries.

Insurance coverage for all of your bleeding disorder medical care also begins with your clinic visits. Your

“clinic” treatment plan is key for your insurance company to continue to provide optimal coverage for your very expensive bleeding disorders care. CCBD often has to work with health insurance and drug insurance providers to communicate critical, up-to-date information about your evaluations and prescriptions.

So please be aware how important your clinic visits are—give CCBD a call to schedule your next appointment.

Primary Care Physicians

The Comprehensive Center for Bleeding Disorders (CCBD) staff provides medical management to patients with bleeding and clotting disorders. In order to provide the best medical care in this highly specialized area of hematology, our staff focuses on staying aware of the most current research and medical practices for patients with bleeding and clotting disorders. As a result, our staff may not be aware of the most effective treatment for diseases, illnesses, or conditions that are not within their specialty. To receive the best care for medical problems

not related to hematology, it is very important to have a primary care physician. A primary care physician is able to provide recommendations related to numerous health conditions as well as coordinate care provided by all the specialties involved in your care. If you do not have a primary care physician, CCBD strongly encourages you to find one. If you need help finding a primary care physician who has experience with bleeding or clotting disorders, contact CCBD. We may be able to help you find a physician in your area.

SATURDAY SEMINAR – Spring 2008

Von Willebrand Disease, Platelet Function Defect and Other Bleeding Disorders

Presented by Joan Cox Gill, M.D.
**Director of the Comprehensive Center
for Bleeding Disorders**

Date: TBA
9:00-11:30 a.m.
Blood Research Institute
8733 Watertown Plank Road, Milwaukee

9:00-9:30 Registration & Refreshments

9:30-10:30 Presentation

10:30-11:30 Questions

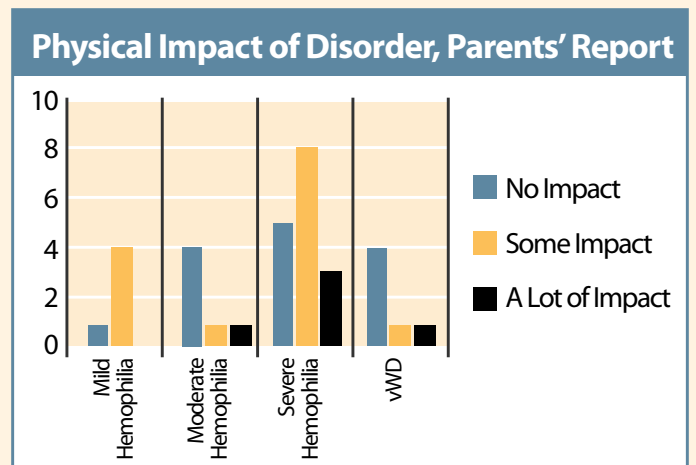
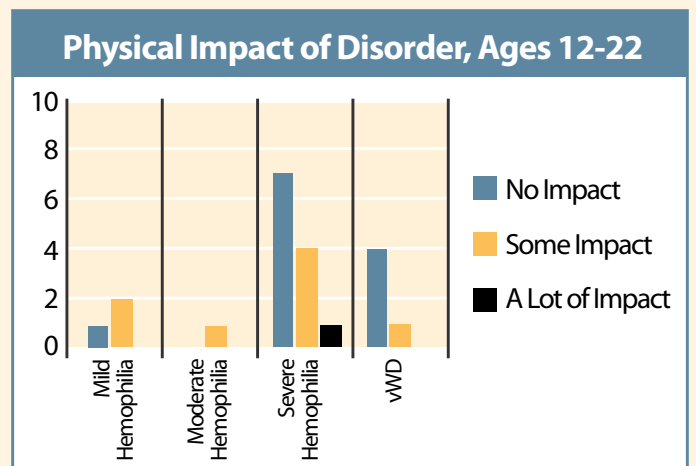
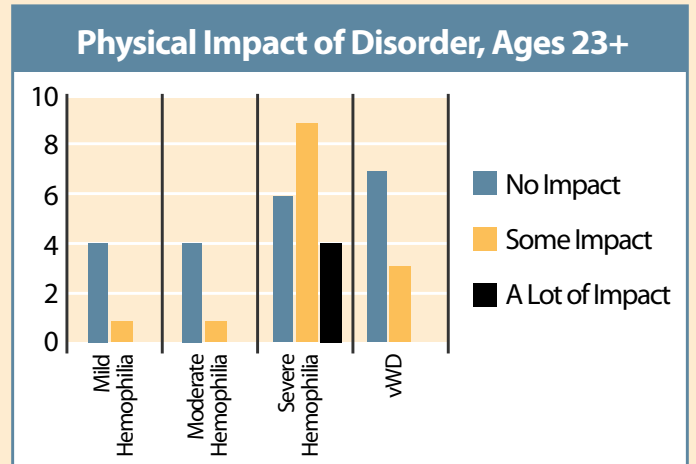
To pre-register or to get additional information about the seminar, please call Jane Volkmann at (414) 937-6575 or toll free at (888) 312-2223.

We look forward to seeing you there!
Refreshments will be provided courtesy of Great Lakes Hemophilia Foundation.

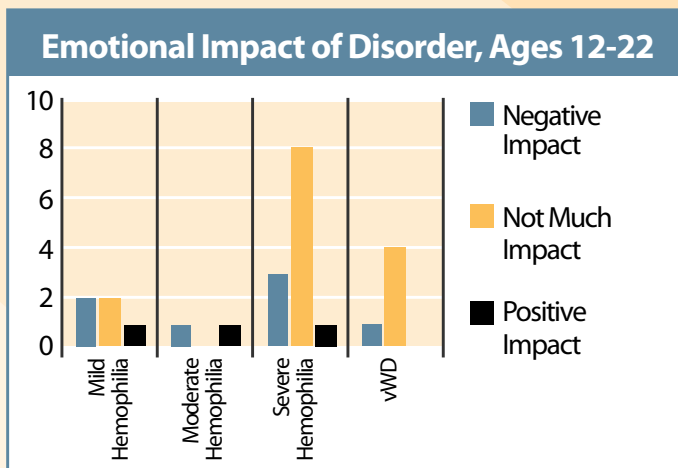
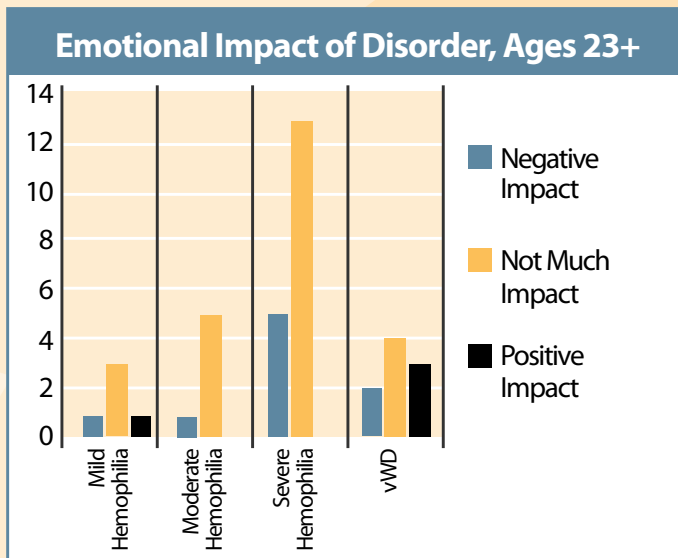
Results of 2007 Patient Questionnaire

Thank you to everyone who participated in the 2007 Patient Questionnaire through interviews with the CCBD social worker. Your answers gave us important information about how to improve both your medical care and your access to medical care. Based on the comments of the 259 new patients and the 115 previously diagnosed patients who participated in the survey, we have made changes to better meet your needs. Below are some of the findings from the 2007 survey:

- Of the 115 previously diagnosed individuals (or their parents) who participated in the questionnaire, 113 (98%) reported that they were very satisfied with the medical care they were receiving through CCBD for their bleeding or clotting disorder. The remaining 2 (2%) reported moderate satisfaction. However, in addition to being quite satisfied with their medical care, a number of patients had very useful suggestions about changes we might make to improve our services. Some of the suggestions were: speed up the clinic process (this was the most popular suggestion); have more hours, days of the week, and locations available for clinics; and give more advance notice of clinic appointments. There were a couple of concerns about medical staff empathy and nurse follow-through which were addressed immediately, but the other suggestions require additional planning and effort on our part. We currently do have limited availability of clinic space and hours, but are hoping to add more clinic times and perhaps experiment with an outreach clinic to see if that helps remove some barriers for patients who live far away from the Center. This may help with the length of clinic, but we will also be working as a team to improve our efficiency over this next year. On the positive side, patients reported that our scheduling process has improved and is much more patient friendly. People liked our clinic's relocation to the 2nd floor of Children's Hospital (from the 8th floor), and some patients felt that the length of clinic had improved. In addition, many patients reported that their phone calls were answered quickly and courteously by CCBD staff. One patient commented that he felt "fortunate to have this resource [CCBD] available".
- Most (98%) of the 115 previously diagnosed patients or parents indicated that they felt they had received enough information regarding their diagnosis. The two respondents who didn't were both adults with a clotting disorder and they were subsequently given more information.
- The next three charts represent the number of patients from different age and diagnosis categories reporting the amount of physical impact their bleeding disorder has on their daily activities. Clearly, older patients with more serious diagnoses and families with newly diagnosed children experience the most severe physical disruptions.

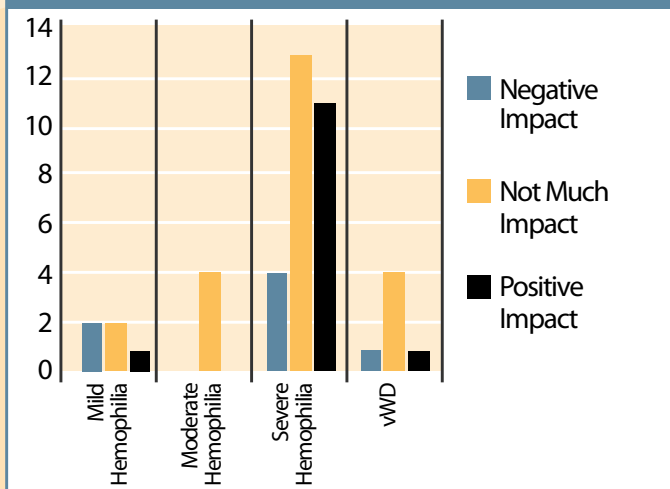


- The following charts represent the number of patients reporting that their disorder has a significant emotional impact. Most patients report either no emotional impact or, especially in the case of parents with a child who has a severe diagnosis, even a positive impact, but there are certainly patients across all categories who do report some negative impact, particularly older patients.



- Given the physical impact of their diagnosis on our patients ages 23 and up, it is important to note that only 20 % of this age group report being unemployed (mostly due to disability). Forty-four percent are employed full time, 23% part time, and 13% are retired.
- A major barrier to medical care is adequate health insurance. Ten percent of our patients report that they have insufficient insurance. Some of the problems our

Emotional Impact of Disorder, Parents' Report



patients are encountering are large out-of-pocket costs, including large co-pays for costly factor product and other medications as well as for physician visits and clinic appointments; reaching lifetime caps; and insurance lapses associated with life events such as changing or losing jobs, reaching adulthood, or divorce (please note that CCBD's financial counselor is available to help with these issues).

- A majority of the 12-22 age group reported weekly exercise: 52% said they exercised 4-7 times per week, 28% 1-3 times per week, and 5% not at all. For the 23-and-up age group, 49% reported exercising 4-7 times per week and 24% said they exercised 1-3 times per week, while 27% did not exercise. This year the respondents again reported a much wider range of activities than in previous years: walking, swimming, lifting weights, working out at a gym, basketball, biking, tennis, volleyball, racquetball, gardening, using an elliptical machine, belly dancing, yoga, Pilates, and soccer.
- An important issue for young people with bleeding disorders is choosing an occupation that is not physically demanding and that will provide adequate insurance to cover the very expensive factor products used to treat their disorder. This year when the 12-22 year olds were asked about their career plans, more than half had already chosen a career (last year about one third had chosen). Some of the choices mentioned this year were forestry, DNR, medicine, hematology/radiology, business manager, and architect.
- Most of these young people are doing their own infusions after certification by CCBD (14 or 93% of the 15 young people with a severe or moderate diagnosis), although 5 members of this group, or 33%, were still relying on a parent to complete

Results of 2007 Patient Questionnaire (continued from p. 5)

the required calendar documentation of their bleeds and infusions.

- We also asked families with young children if they felt they had sufficient contact with other families in the bleeding disorder community. Fourteen percent indicated sufficient contact, 17% would like more and 69% did not have much contact but were fine with that. For those families seeking more contact we provided mentoring families if they chose (we linked two families this past year) as well as information about Great Lakes Hemophilia Foundation, the support and advocacy organization for people with bleeding disorders.
- Our survey again included patients with clotting disorders as well as carriers. Their satisfaction responses are included with the bleeding disorder patients.
- Of the 259 new patients we surveyed, 145 (56%) were referred by a specialist (55 by an ENT specialist, 13 by a neurologist, 11 by an OB/GYN specialist, 9 by other hematologists, 5 by a cardiologist, 4 by a GI specialist, 2 by adolescent medicine specialists, and one by an orthopedic surgeon). Eleven were referred by the preoperative clinic at Children's Hospital of Wisconsin, one by the hospital's ICU department, one was a social services referral, and the rest were unspecified/unknown). Seventy-three (28%) were referred by their primary medical provider, and 30 (12%) came to us because of a family member, usually a family member who had previously been diagnosed by us.

The information we gather from the patient survey is essential in helping us structure our clinic and other processes to best meet the needs of our patients. For 2008 we will be giving patients an opportunity to respond anonymously to questions about barriers to attending comprehensive clinic, as well as satisfaction feedback and suggestions for improvement. We really appreciate the time and thought you put into answering our questions this past year. Your suggestions for improvements and any other feedback about our services are always welcome. You can call Jane Volkmann at (414) 937-6575 or toll free at (888) 312-2223 anytime throughout the year with additional comments or with questions about the survey.

Ask CCBD

Do you have a general question that you would like to address to a member of the CCBD staff, including one of the hematologists who works with our patients? To have your question considered, please visit our page on the BloodCenter of Wisconsin website (www.bcw.edu) and click on "Ask CCBD." We will pick at least one question to answer in every issue of CCBD Connections.

Great Lakes Hemophilia Foundation

All That Jazz Gala

March 15, 2008 at the Hilton Milwaukee City Center
Mark your calendars! Our coming Gala promises to be an action packed night. We welcome Patti Genko as our night's emcee in cooperation with our media partner Smooth Jazz 106.9. We will recognize Audrey Nuskiwicz for her continued dedication to the Hemophilia Treatment Centre in Green Bay. Check the website for updates on our Voice Auction and other fun!

Individual ticket: \$100
Table of 8: \$1,500
Table of 10: \$1,800

Other Upcoming Events:

March 1, 2008 Transition Ignition (Tentative)

This is a one-day workshop for teens and their parents to jumpstart teen responsibility for their own hemophilia care under parental and HTC guidance.

May 19, 2008 - Teeing Up for Charity at The Bog in Saukville, WI

September 27, 2008 - Walk with the Animals at the Milwaukee County Zoo

To register for programs or for more information, call 414-257-0200 or 888-797-4543

HAVE YOU MOVED?

Please complete the form below and return to us at the Comprehensive Center for Bleeding Disorders, PO Box 2178, Milwaukee, WI 53201-2178. It is important that we keep our mailing lists current so that you can be sure to receive current medical information along with announcements regarding our special medical programs.

Patient Name: _____

DOB: _____

New Address: _____

City, State, Zip Code: _____

New Phone Number: _____

New Dentist or Primary Doctor: _____

Office Phone Number: _____

Is this the address of patient's:

MOTHER FATHER BOTH

HAVE YOU RECENTLY TURNED 18 YEARS OLD?

Check the appropriate boxes telling us how we may contact you and who we may speak with regarding your medical care and return it to us at the Comprehensive Center for Bleeding Disorders, PO Box 2178, Milwaukee, WI 53201-2178. Because you are legally an adult, CCBD cannot speak to anyone but you regarding your medical care without your authorization.

I authorize CCBD staff to:

Contact me at my work phone number:

(Detailed messages will not be left)

Leave a detailed message on my home phone/voicemail:

I authorize CCBD staff to speak or leave information with person(s) in my home as follows:

Name / Relationship to Patient

Name / Relationship to Patient

Patient Name: _____

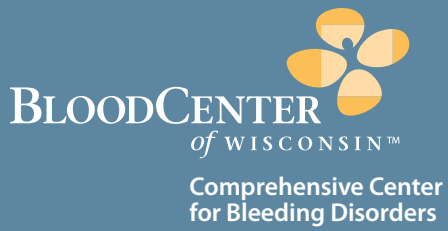
DOB: _____

Patient Signature _____

Date: _____

WE WOULD LIKE YOUR INPUT

Remember, this is your newsletter. We welcome any requests you might have for future articles. Just give us a call and let us know of your ideas or suggestions. Or, just give us a call and let us know if you feel we are providing you with an informative newsletter.



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by providing life-saving solutions grounded in
unparalleled medical and scientific expertise.***
