In this issue

Update

THE HEMOPHILIA HEALTH CARE ACT HB 1705

Pennsylvania Hemophilia Program Medical Directors Strongly Support Legislation to Protect Patient Access to Treatment and Services

All medical directors from the eight state-recognized hemophilia programs in Pennsylvania have voiced their strong support of HB 1705, The Hemophilia Health Care Act. As patients, we appreciate the sensitivity of program staff to issues that affect our treatment and quality of life. HB 1705 will protect our access to quality care, factor replacement therapies and supportive services. Please call your local hemophilia treatment program and thank the medical director for supporting this important legislation that will help to protect patients with bleeding disorders in Pennsylvania. Following are excerpts from their letters to the Pennsylvania General Assembly.

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The Winning Spirit

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Quarterly Newsletter of the National Hemophilia Foundation Delaware Valley Chapter

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From the Executive Director

Over the last several months, the Delaware Valley Chapter and the Western Pennsylvania Chapter of the NHF have combined their resources and energies to help patients affected by bleeding disorders. Our efforts have succeeded in the introduction of The Hemophilia Health Care Act, HB 1705, in the House of Representatives. This important legislation will secure patient access to hemophilia programs, factor replacement therapies and supportive services for all of us..... BUT WE'VE ONLY JUST BEGUN!!!

We need the help of each member of our bleeding disorders community in Pennsylvania to make visits to local legislative offices to let our representatives know that we need their support of HB 1705. Without each of us making that effort, we can't be successful in moving this legislation to passage in the House of Representatives in the fall. It's simple, BUT VERY, VERY IMPORTANT!!

If you can help in this effort, call the DVC office right away! We'll send you out a simple legislative kit that will help you when you make that visit. Your purpose will be to tell your legislator how bleeding disorders has affected your life. You just need to tell your personal story and ask for their support of HB 1705. Very simple.

Before you call us, go to: <legis.state.pa.us> and find out your Representative's name and local office phone number. It's very easy. You can type in your address and locate the information in a flash! Then, make a 15 minute appointment with your representative's local office (near your home) and then call us so we can send you the simple guide for your visit!!

WE NEED YOUR HELP!! WE JUST CAN'T DO THIS WITHOUT YOU!! 215-885-6500!!

Baxter Offers Advate Trial Prescription Program

Baxter is offering an Advate Trial Prescription Program to all Hemophilia Treatment Centers. A patient can receive three free doses of up to 12,000 IU of Advate at no charge. Ask your physician about this free trial.

Bayer Offers Kogenate FS Experience Program

Bayer HealthCare, LLC Biological Products Division, is offering six free doses of up to 20,000 IU of Kogenate FS at no charge. In addition, patients will receive a free, EZ LOG electronic patient diary. Ask your physician about this program.

We Gratefully Acknowledge...the pledge of support from the following manufacturers for 2005.

American Red Cross	Baxter Bioscience	Bayer Corporation	Grifols	Wyeth	Nabi	ZLB Behring
Monarc-M	Recombinate	Kogenate FS	Alpha Nine SD	BeneFixTM	Autoplex T	Monoclate-P
	Hemofil M	Koate-DVI	Alphanate	ReFacto	WinRho	Mononine
	Proplex T	Konyne-80	SD/HT			Helixate FS
	Feiba VH		Profilnine SD		Novo Nordisk	Humate-P
	Bebulin VH				NovoSevenTM	Stimate
	Albumin (Human)					Gammar-P I.V
	Advate					

Calendar

October
15 Wyeth Family Day
21 Oktoberfest!
27 NHF meeting/
San Diego!

November
5 - 6 Inhibitor Summit
24 Happy Thanksgiving!

December
10 Holiday Party!

"Oktoberfest" will be held at the Hilton Hotel on City Avenue **(a NEW LOCATION!)** on Friday, **October 21, 2005**, sponsored by Wyeth! What a great night of food, music, gaming events and a terrific live auction! For an invitation, call the office (215-885-6500!! Help support Bob Romano, Megan McEnroe and their great committee by planning to attend this year!!

Wyeth Family Day will be held on **Saturday, October 15th** at the Wyeth corporate campus in Collegeville, Pennsylvania. This will be a great time to bring the kids and enjoy fabulous food and fun with other families who share your challenges! Meet the folks from Wyeth and hear about ways to navigate the insurance nightmare! Lots of games and prizes for everyone!! Bring grandmom, too! For an invitation, call the DVC office @215-885-6500! Come and join the fun!!

The 57th NHF Annual Meeting will be held on **October 27, 28 and 29, 2005** in San Diego, California. This is the only meeting in the United States completely focused on bleeding disorders.

On **November 5 and 6, 2005**, the Delaware Valley Chapter will host the first "**Inhibitor Summit**" in the United States, right here in Philadelphia! This important meeting will be at the Center City Marriott Courtyard (near City Hall). This event is sponsored by Novo Nordisk and will provide the most up-to-date information to our local patients affected by inhibitors . Patients from the northeast region of the United States are invited and Novo Nordisk will be supporting the costs associated with travel and lodging to help patients attend. If you or your child is affected by this serious condition, call your local hemophilia program nurse to get an invitation and details about this important meeting! The DVC also has invitations. Call us to find out more! 215-885-6500!!

Top; Oktoberfest supporters, below; Kathy Sell (DVC Board member) and registration helpers Oktoberfest 2004

Santa will arrive for the **DVC Annual Holiday Party** on **Saturday, December 10, 2005!** Bring the kids to Jefferson Alumni Hall and experience a great day of fun and holiday gifts for everyone! This has become a favorite Chapter event!! Mark your calendars now and plan to be with all of us!! You won't be disappointed!

Holiday Party December 2004

The following letter was sent to the Delaware Valley Chapter from Shasteen & Scholz, P.C., Attorneys at Law, Lincoln Nebraska (if you have any questions, contact Shasteen & Scholz directly)

E. Virgil Falloon
Shasteen & Scholz, P.C., Attorneys at Law
840 North 48th Street
Lincoln, NE 68504
402-464-0064
URGENT NOTICE RE; HCV LITIGATION

Dear Sir or Madam:
It has come to our attention that a misconception may have developed in the hemophilia community relevant to the Hepatitis C (HCV) litigation.

The misconception is that there is an ongoing class action and that a person does not need to be individually represented by an attorney to have their claim investigated and pursued against the responsible companies.

There is NO class action. The class action was NOT certified by Judge Grady. People with hemophilia infected with hepatitis C will not receive a notice from a court or a judge or any attorney because there is NO class action for them to receive notice of.

Therefore, claims may be presented and pursued on an individual basis only. Those who were infected with HCV from anti-hemophilic factor that are represented by attorneys and will have their claims investigated and presented to the responsible companies for settlement or suit.

Those with hemophilia and hepatitis C who do not have attorneys have no one working for them or on their behalf in the HCV litigation. There will be no professional to prepare and present their claim to the responsible companies. Nevertheless, they too have the right to sue the responsible entities on their own, in pro per. Because the claims are so complex, however, such persons would likely be better off hiring lawyers. We are available to provide a free legal consultation to those who are interested in taking legal action.

If you have any questions, you are welcome to contact me.

Yours very truly,
E. Virgil Falloon

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Pennsylvania Hemophilia Program Medical Directors Strongly

HB 1705

"We are aware that the Council of State Governments (CSG) voted to include a "Standards of Care for Hemophilia" as part of its Suggested State Legislation Program for 2005. We need legislative assistance in Pennsylvania to help us protect the ability of each individual with a bleeding disorder to maintain their access to the medical products and services they need to live long, healthy lives. By doing so, we will also be preserving Pennsylvania's status as a model for all states that are committed to the health and well being of their citizens."

Lloyd E. Barron, II, MD, Director
Lehigh Valley Hospital Hemophilia Center
Allentown, PA

"With rapid changes in health care delivery, we want to make sure that children with bleeding disorders continue to have access to appropriate care, helping to ensure healthy, productive adult lives for all of them. The Hemophilia Treatment Centers in Pennsylvania have a proven record of success in diagnosing and treating patients with bleeding disorders and minimizing complications that can arise from bleeding."

Catherine S. Manno, MD, Medical Director
Comprehensive Hemostasis and Thrombosis Center
Children's Hospital of Philadelphia
Philadelphia, PA

"I believe that legislation would help to maintain the support services necessary for patients with bleeding disorders, allow for diagnosis of those who suffer unknowingly from this disorder and maintain the quality care presently available for my patients in the following ways:

- Protect patients' access to the eight state-recognized hemophilia programs.
- Protect the quality of home pharmacy supportive services.
- Protect patients' access to the special coagulation laboratories associated with hemophilia programs.
- Protect patients' access to all factor therapies (there are no generics).
- Protect women from unnecessary hysterectomies and unexplained bleeding due to an undiagnosed bleeding disorder.

We want to preserve Pennsylvania's status as a model for all states that are committed to the health and well being of their citizens."

Jamie Ellen Siegel, MD, Director
Cardeza Foundation Hemophilia Center
Thomas Jefferson University
Philadelphia, PA

"The climate is changing dramatically in regards to our patients' ability to access appropriate medical treatments. Insurance companies are acutely aware of the cost of treatment and have erected many barriers to therapy. As it is, our staff spends a huge amount of time waiting on the telephone to get information for pre-authorizations, etc. I strongly urge the Pennsylvania legislature to assist us in helping protect the ability of each individual with a bleeding disorder to keep access to the medical products and services they need to live long, healthy lives. By doing so, you will be preserving Pennsylvania's status as a model for all states that are committed to helping the well-being of its citizens."

Terrence P. Cescon, MD, Director
Hemophilia Program
The Reading Hospital and Medical Center
Reading, PA

Support Legislation to Protect Patient Access to Treatment and Services

"Treatment for patients with hemophilia requires prompt access to clotting factor concentrates. Many insurance providers use mail-order pharmacy services that require a week's notice for delivery. Hemophilia Treatment Centers and home care agencies assist the families and/or patients with hemophilia in administration of clotting factor replacement products whenever possible in the home setting. This greatly improves the lives and productivity of patients with hemophilia, allowing children with hemophilia to attend school on a regular basis, their parents to have productive work lives and adults with hemophilia to have productive work and social lives. It is imperative that home nursing services are supported for this disease. If not supported, patients must go to a hospital, which increases the expense and delays in treatment worsen the outcome. Our goal is to have all families and patients independent in home therapy, as this gives them control over their disease and allows for prompt treatment."

Barbara A. Konkle, MD, Director
Penn Comprehensive Hemophilia and Thrombosis Program
Philadelphia, PA

"Over the past 20 years, numerous studies have shown that patients receiving comprehensive care at Hemophilia Treatment Centers (HTCs) have reduced numbers of hospitalizations for bleeding, reduced numbers of days lost from school and work and improved quality of life. Thus, it is essential that legislation insure that individuals with hemophilia and related bleeding disorders are guaranteed unrestricted access to Pennsylvania hemophilia treatment programs for all aspects of their care. To contain costs, some insurance companies place limitations on the types of treatment products they will reimburse. Others limit patients to a single pharmacy. Such limitations impose barriers to good patient care and create unnecessary problems for the patient and Hemophilia Treatment Center. We need legislation in Pennsylvania that will preserve the ability of each and every individual with hemophilia and related bleeding disorders to have access to the treatment products and medical services necessary to maintain optimum health and productivity."

M. Elaine Eyster, MD, Director
Hemophilia Center of Central Pennsylvania
Milton S. Hershey Medical Center
Hershey, PA

"The climate is dramatically changing regarding patients' access to appropriate medical treatments in Pennsylvania due, in large part, to many, new restrictions imposed by insurance companies. The situation has now evolved to the point that a large part of my day is spent advocating for my patients' medical needs at the expense of providing direct clinical care. My time, and that of my staff, should be refocused on the actual medical, educational and social service needs of my center's children and their families. My staff and I are especially challenged by these restrictions."

Frank E. Shafer, MD, Director
Bleeding Disorders Program
St. Christopher's Hospital for Children
Philadelphia, PA

"One of the most critical issues we are dealing with is the ever-increasing threat to the ability of patients to access the full range of medical treatments and services they need to maintain optimum health and a good quality of life. Among the threats are attempts by insurance companies to limit which drugs (factor replacement therapies) they are willing to reimburse. As a result, physicians may be restricted as to which products they may prescribe and therefore some patients may not receive the product that they and their physician believe is in the best interests of their health. In addition, some insurance companies place limitations on where patients may obtain their anti-hemophilic factor and ancillary medical products and services. In some cases, they go as far as limiting patients to a single provider by means of what are sometimes called "sole-source provider contracts." These contracts are extremely problematic because in addition to factor provision, some patients may need specific ancillary services which are not offered by every provider."

Margaret V. Ragni, MD, Director
Hemophilia Center of Western Pennsylvania
Pittsburgh, PA

The National Hemophilia Foundation Voices Its Support of HB 1705

To Members of the Pennsylvania Legislature:

For more than fifty-five years, the National Hemophilia Foundation (NHF) has advocated for the needs of Americans affected by hemophilia and other bleeding disorders. Our efforts are intended to improve the lives and health of these individuals through advocacy, education and research. One of the most critical issues we are dealing with at the national and state levels is the ever-increasing threat to the ability of patients to access the full range of medical treatments and services they need to maintain optimum health and a good quality of life.

Among these threats are attempts by health care payers, whether public or private, to limit which drugs-in this case, anti-hemophilic factor- they are willing to reimburse. As a result, physicians may be restricted as to which products they may prescribe and therefore some patients may not receive the product that they and their physician believe is in the best interest of their health.

The Medical and Scientific Advisory Council of the NHF has determined that these limitations are problematic. In addition, some payers place limitations on where patients may obtain their anti-hemophilia factor and ancillary medical products and services. In some cases, they go as far as limiting patients to a single provider by means of what are sometimes called "sole-source provider contracts." These contracts are extremely problematic because in addition to factor provision, some patients may need specific ancillary services which are not offered by every provider.

The system of health care provision and the rules that govern it vary greatly from state to state. We work closely with a national network of local chapters in order to better understand and address the particular needs and concerns of patients within each state. In a number of states, our chapters have determined that a legislative remedy is needed to correct the current situation and ensure that all patients have access to quality care. We strongly support these chapters in this determination and chosen strategy.

In Pennsylvania, we have two chapters, known respectively as the Delaware Valley Chapter and the Western Pennsylvania Chapter. The Hemophilia Health Care Act (HB 1705), which our Pennsylvania chapters have introduced, is similar to legislation that has been effectively implemented in New Jersey. We believe that this legislation can indeed be an effective remedy in Pennsylvania and in other states as well.

You may also be aware that in November 2004, the Council of State Governments (CSG) voted to include a "Standards of Care for Hemophilia" law as part of its Suggested State Legislation Program for 2005. The Committee that proposed this legislation was chaired by Pennsylvania Representative Chris Ross.

We strongly urge you to assist us in protecting the ability of each individual in Pennsylvania with a bleeding disorder to access the medical products and services they need to live long, healthy lives. By doing so, you will also be preserving Pennsylvania's status as a model for all states that are committed to the health and well being of their citizens.

Sincerely,

Alan J. Kinniburgh, Ph.D.
Chief Executive Officer

Glenn Mones
Vice President for Public Policy

Exclusion of Blood Clotting Factors from the Medicare Part B Competitive Acquisition Program SUPPORT FROM SENATOR RICK SANTORUM (PA) AND REPRESENTATIVE PHIL ENGLISH (PA) PLAYED A KEY ROLE!!

On June 27, 2005, the federal Centers for Medicare and Medicaid Services (CMS) released regulations regarding implementation of the Medicare Part B Competitive Acquisition Program. The National Hemophilia Foundation successfully advocated for the exclusion of blood-clotting factors from this program. If blood-clotting factors were incorporated into the program, access issues would have occurred for Medicare beneficiaries reliant on their life-saving therapy.

The two Pennsylvania state Chapters of NHF, the Delaware Valley Chapter and the Western Pennsylvania Chapter, have been advocating for choice of product and choice of provider for people with bleeding disorders within the Commonwealth of Pennsylvania. The Competitive Acquisition Program would have steered Medicare beneficiaries with hemophilia to selected providers while most likely restricting access to all brands of blood clotting factors. As we have seen, such access restrictions have detrimental effects on patients with bleeding disorders.

In March, both Pennsylvania Chapters met with Senator Santorum's staff as part of NHF's Washington Day activities. Through this effort, Senator Santorum recognized the legitimate access concerns posed by the Competitive Acquisition Program and wrote to CMS to urge the exclusion of blood-clotting factor from the roll out of this program. Representative English, who represents the Erie county area, did the same. Both Senator Santorum and Representative English serve on congressional committees with jurisdiction over the Medicare program, and their efforts proved vital in this success.

The two Pennsylvania chapters of NHF remain consistent in their opposition to private, state and federal insurance programs that will restrict access and choice of brand and provider. The standard of care and quality of life for individuals with bleeding disorders has increased significantly over the years and must not be rolled back. The decision about which brand of factor will work best for the patient is not for an insurer to decide. The physician and patient have the knowledge to decide what is the best course of treatment. In this effort, we will remain vigilant.

We want to sincerely thank Senator Santorum and Representative English for their leadership on this very important issue!!

The Future of Factor

Enhanced factor treatment holds promise to change the daily lives of people with hemophilia and their families.

By Elizabeth Thompson Beckly

Replacing missing blood-clotting proteins to treat and prevent bleeds has made it possible for people to live with hemophilia and has improved the quality of many patients' lives.

Factor treatment is not a cure, however, and it is not without its drawback. As people search for options, the possibility that a cure might one day be found in gene therapy has attracted a lot of attention in recent years.

But less often in the spotlight are the many bright minds and passionate convictions that drive those working to advance what is known already to be effective. They belong to the scientists and hemophilia advocates who maintain a vision for factor that takes it leaps and bounds beyond where it is today, with the hope of making products less interfering and easier to use while maintaining purity and safety.

Many agree that enhanced factor treatment holds a lot of promise to change the daily lives of people with hemophilia and their families, and it's important to develop products that meet their needs.

Safety First

Following the devastation wrought in the 1980s by blood products infected with HIV and hepatitis C, the focus of factor development has been on reducing the potential risk of viral transmission, and rightly so.

Closer attention to factor product safety, with distillation, purification and most recently bioreactor processes, seems to have paid off. Factor products today are much safer, says Bruce Ewenstein, MD, PhD, global medical director for hemophilia therapies for Baxter BioScience in Westlake Village, California. One of the reasons Ewenstein went to Baxter after 17 years treating hemophilia patients in Boston was to work on the "third generation of factor VIII (FVIII) products. He notes that several plasma-derived factor products remain in the market, but that their use is rapidly fading in the US as recombinant products become more popular.

"Attention now can be turned to improving other components of hemophilia with new generations of products," Ewenstein says, "offering a description of where factor research is headed that echoes ideas shared by several others in the hemophilia community."

The two areas of greatest concern - and two of the fronts researchers are pursuing aggressively - are the development of inhibitors, or the immunogenecity of a product, and what Ewenstein says could be called "convenience" regarding the frequency of factor treatment.

"But [that term] doesn't fully capture how important this is to the patient population," he says.

FVIII drugs used to treat hemophilia A, the most common form of the disease, have a half-life of about 10-12 hours in the treatment of moderate or serious bleeds, requiring infusion two or three times a week, or as often as once a day. A product with a longer half-life would result in many fewer infusions.

A better understanding of why some patients generate an inhibitor response to factor concentrate could open several research avenues to better-designed or better-administered products.

Inhibitor Risk Assessment

According to Ewenstein, the first step toward discovering why some people with hemophilia develop inhibitors is to research which genetic or environmental components may be involved. Baxter has launched two large studies, one using genomics to try to identify if there could be specific genes that put one at greater or lower risk for genetic inhibitors, and the other trying to identify certain risks that might be controllable, in terms of environmental issues.

Potential environmental risks might include the age when the person was first treated, whether he or she was treated on-demand or prophylactically and stresses such as major trauma or surgery. "The list could go on and on," Ewenstein says.

"Pinpointing the greatest risks could allow modifications to treatment to help avoid them," he says. "Translated into practice, it could mean taking people at very high risk-whether genetic or environmental-and giving them some sort of immuno-suppressing drug along with factor concentrate. Or it might be possible to reduce the inherent risk of immunogenecity in a particular product," Ewenstein adds, cautioning that he thinks it will take a long time to develop a molecule that is ideal with respect to inhibitor generation.

"I think approaching [inhibitor development] with a combination of risk assessment and immune modulation would take us to that goal faster," he says.

Wyeth Pharmaceuticals is also contributing to the research as to why people develop inhibitors, and the company is working with the Centers for Disease Control and Prevention to find the cause.

"There is a great deal about inhibitors that we, the medical community, don't fully understand," says Jay Feingold, MD, PhD, assistant VP of Global Medical Affairs, Wyeth Pharmaceuticals. "To advance our knowledge, Wyeth is helping to fund a project to be conducted by the Centers for Disease Control and Prevention to collect specific information on the who, what, when, where and why of inhibitor formation. We hope this effort ultimately will help healthcare professionals better manage inhibitors."

Molecule Modification

Another goal is to increase factor's half-life without increasing the immunogenecity. Some approaches are to modify the factor molecule itself, to develop one that is better than what

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The Future of Factor

is found in nature or to deliver it in a way that it is less likely to be cleared by the body.

Baxter is looking at both possibilities for FVIII, although Ewenstein says he could not discuss the specifics of what is in the Baxter pipeline.

He cites as an example, a small molecule with enough FVIII activity to prevent bleeds that could mean more convenient treatment (e.g. switching from taking factor intravenously to subcutaneous administration or intranasal sprays). "One could even dream of a pill taken orally," Ewenstein says.

"A small-molecule replacement for FVIII to at least achieve some FVIII activity would mean a big increase in quality of life," he says, noting it also would address venous access issues.

"A lot of folks have come to see this almost as the equivalent of a gene therapy cure because it would so change the way the drug is administered that it would become much less intrusive," Ewenstein says.

Longer Half-Life

To that end, Bayer Healthcare has been championing a FVIII product that could result in a prolonged interval of a week or more between treatments. Last November, Bayer announced a $100 million deal to develop a new, longer-lasting version of its Kogenate product using a liposome technology developed by Zilip-Pharma.

"If we can change this paradigm, it will be a huge opportunity to help those with hemophilia live the life they choose," says Michael Mathews, vice president of global strategic marketing for coagulation in Bayer's biological products division (Bayer BP). "For the first time in hemophilia A treatment, we have the potential to reduce the frequency of administration or dosing."

Zilip-Pharma's technology uses biological-free liposomes with a polyethylene glycol (PEG) coating that helps avoid detection by the body's immune system. The PEGylated liposomes have a longer half-life and have been used with other approved products.

Zilip-Pharma's multicenter, patient-blinded phase II clinical pilot studies suggest that Kogenate molecules attached to these liposomes can extend the time between bleeds, meaning fewer treatments, particularly in prophylactic regimens, according to Zilip-Pharma founder Robert Taub.

Bayer officials say they do not expect to launch the new Kogenate product commercially for about five years and that it is contingent on continued positive clinical results, regulatory reviews and license approvals.

"Bayer hopes to have an Investigational New Drug application filed with the US Food and Drug Administration (FDA) by the second quarter of this year and to start phase III studies in 2006," says Michael Fournel, senior vice president of research and technology at Bayer BP.

In other research to extend half-life, Maxygen, Inc., based in Redwood City, California, is working with a new factor VIIa (FVIIa) molecule that has shown an improved in vivo half-life compared to alternative products in preclinical animal models. The company says the new molecule, called MAXY-VII, "appears to stimulate more rapid and increased thrombin generation in blood resulting in a significant reduction in blood loss."

Maxygen plans to advance its FVIIa product candidate to eventually treat trauma and intracerebral hemorrhage, as well as hemophilia.

Longer Activation

"There are several ways to try to address the problem of frequent infusions," Fournel says. In addition to attempts to extend half-life, Bayer and others are trying to modify the FVIII molecule directly to alter its pharmacodynamic properties.

One such approach is that of Andrew Gale, PhD, assistant professor at The Scripps Research Institute in La Jolla, California, whose research involves the stabilization of active FVIII. Gale's work is funded in part by Bayer and by a three-year Career Development Award from NHF.

Normally, after injection and activation, FVIII is quickly inactivated by spontaneous dissociation. Gale's concept is to prevent that spontaneous dissociation by modifying some of the molecule's amino acids so it does not degrade as quickly. His method involves introducing a disulfide bond that improves the length of the protein's activity following its activation.

This type of bond, between the sulfur atoms in two cysteine amino acid residues, is present normally in the FVIII protein. Gale has added two new cysteine residues in two specific locations such that they will form a disulfide bond to specifically stabilize the active FVIII.

"The result is, once it is activated, it stays activated a longer time," Gale says. "The hope is since this protein has greater effective activity, less of it would be needed to effect the same benefit."

Working with pure proteins from his laboratory bench, Gale says he has extended activity of this engineered FVIII from a normal half-life of two to five minutes to a half-life greater than two hours for the activated form.

"How this will work in vivo in people or animals, still needs to be determined," he says. Gale hopes to obtain funding to begin studies in mice.

Also unknown is how this longer-activated factor could affect treatment. Would a patient be injected less frequently or injected with less of the protein? Gale notes that there have been shortages of FVIII in the past, keeping some patients from obtaining as much as they might want for ongoing prophylactic treatment, as opposed to treating ongoing bleeding episodes.

"If less is needed, we are les likely to have shortages," Gale says. "People could be treated more often pro-actively rather than reactively."

But he concedes that his research is in the very early stages, a long way from becoming a real therapy. And should it ever reach that point, Gale says he will leave it to the doctors and companies producing factors for treatment to determine what is best for individual patients.

Researchers like Gale and Ewenstein, as well as the pharmaceutical companies working for a cure, contribute to the belief that patients with hemophilia will enjoy an improved quality of life with better factor products in the not too distant future. They all agree that the future looks bright.

Previous article taken from HemAware, a publication of the National Hemophilia Foundation, March/April 2005 edition.