NHF News Briefs

May 18 – May 24, 2018

This report includes selected news items from the past week on issues of concern to the bleeding disorders community. It is designed to help keep NHF national and local leadership and staff informed of the latest information from the news media. It will be distributed by email on Thursday of each week, covering important news items from the previous seven days. Subjects covered will include hemophilia, other bleeding disorders, gene therapy, hepatitis, HIV/AIDS, and others.

May 21, 2018
Prnewswire.com

Head-To-Head Pharmacokinetic Study Demonstrated Greater Factor IX Activity with Rebinyn® Versus rFIXFc in People with Hemophilia B

A new head-to-head pharmacokinetic study showed that adults with hemophilia B who received a single dose (50 IU/kg) of Rebinyn® [Coagulation Factor IX (Recombinant), GlycoPEGylated] achieved high factor IX activity for longer than those treated with rFIXFc [Coagulation Factor IX (Recombinant), Fc Fusion Protein]. Factor IX activity observed with Rebinyn® was 4.39 times greater than rFIXFc and increased over time, providing six-times greater activity at seven days than rFIXFc at the same dose. These pharmacokinetic findings from the paradigm™7 trial were presented today at the WFH 2018 World Congress in Glasgow, UK.[1]

"Until now, there has been no direct pharmacokinetic comparison of these two extended half-life therapies," said Mindy Simpson, M.D., a hematologist/oncologist at Rush University Medical Center in Chicago and an investigator in the paradigm™7 trial comparing Rebinyn® and rFIXFc. "The study results provide clinicians a better understanding of the pharmacokinetic differences between these hemophilia B treatments."

Thirty minutes after administration, factor IX levels were twice as high in adults treated with Rebinyn® versus those who received rFIXFc. Rebinyn® also prolonged factor IX activity in the body much longer than rFIXFc, achieving an average half-life of 103.2 hours vs. 84.9 hours, respectively.[1]

"Rebinyn® is a prime example of our deep, ongoing commitment to the rare disease community," said Pia D' Urbano, Corporate Vice President, Biopharmaceuticals, Novo Nordisk Inc. "We are proud to offer people living with hemophilia B an extended half-life treatment that can help them reach and maintain high factor IX levels for a long period of time."

About hemophilia B

Hemophilia is a chronic, inherited bleeding disorder that primarily affects males. People with hemophilia B have congenital factor IX deficiency with missing or reduced levels of clotting factor IX, a key protein needed for proper blood clotting.[2]

In the U.S., it is estimated that 5,000 of 21,000 people with hemophilia have been diagnosed with hemophilia B. Of these, approximately 27% have severe hemophilia, 37% have moderate hemophilia and 35% have mild hemophilia, defined by FIX activity levels.[3]

About the paradigm™7 trial

Paradigm™7 was a multicenter, open-label, randomized, crossover, pharmacokinetic trial in previously treated adult males with congenital hemophilia B (factor IX activity ≤2%), conducted in three countries (U.S., Germany, Switzerland). Patients received single injections (50 IU/kg) of Rebinyn® and rFIXFc with ≥21 days between doses. The primary endpoint was dose-normalized area under the factor IX activity-time curve.

About Rebinyn®

Rebinyn® [Coagulation Factor IX (Recombinant), GlycoPEGylated] is an extended half-life factor IX molecule for on demand treatment of bleeding and perioperative management in patients with hemophilia B. It is not indicated for routine prophylaxis or immune tolerance induction.[4] The review of

Rebinyn® was based on the paradigm™ program; in these completed phase 3 trials, 115 previously treated patients had a total of more than 8,800 exposure days for up to 2.7 years of treatment with Rebinyn®.

For more information about Rebinyn®, visit www.Rebinyn.com.

Indications and Usage

What is Rebinyn® Coagulation Factor IX (Recombinant), GlycoPEGylated?
Rebinyn® is an injectable medicine used to replace clotting Factor IX that is missing in patients with hemophilia B. Rebinyn® is used to treat and control bleeding in people with hemophilia B. Your healthcare provider may give you Rebinyn® when you have surgery. Rebinyn® is not used for routine prophylaxis or for immune tolerance therapy.

Important Safety Information

What is the most important information I need to know about Rebinyn®?

- **Do not attempt to do an infusion yourself unless you have been taught how by your healthcare provider or hemophilia treatment center.** Carefully follow your healthcare provider's instructions regarding the dose and schedule for infusing Rebinyn®.

Who should not use Rebinyn®?
Do not use Rebinyn® if you:

- are allergic to Factor IX or any of the other ingredients of Rebinyn®.
- are allergic to hamster proteins.

What should I tell my health care provider before using Rebinyn®?
Tell your health care provider if you:

- have or have had any medical conditions.
- take any medicines, including non-prescription medicines and dietary supplements.
- are nursing, pregnant, or plan to become pregnant.
- have been told you have inhibitors to Factor IX.

Important Safety Information (cont'd)

How should I use Rebinyn®?
- Rebinyn® is given as an infusion into the vein.
- **Call your healthcare provider right away if your bleeding does not stop after taking Rebinyn®.**
- Do not stop using Rebinyn® without consulting your healthcare provider.

What are the possible side effects of Rebinyn®?
- Common side effects include swelling, pain, rash or redness at the location of the infusion, and itching.
- Call your healthcare provider right away or get emergency treatment right away if you get any of the following signs of an allergic reaction: hives, chest tightness, wheezing, difficulty breathing, and/or swelling of the face.
- Tell your healthcare provider about any side effect that bothers you or that does not go away.
- Animals given repeat doses of Rebinyn® showed Polyethylene Glycol (PEG) inside cells lining blood vessels in the choroid plexus, which makes the fluid that cushions the brain. The potential human implications of these animal tests are unknown.

About Novo Nordisk
Novo Nordisk, a global healthcare company, has been committed to discovering and developing innovative medicines to help people living with diabetes lead longer, healthier lives for 95 years. This heritage has given us experience and capabilities that also enable us to help people defeat other serious diseases including obesity, hemophilia and growth disorders. We remain steadfast in our conviction that the formula for success is to stay focused, think long term and do

business in a financially, socially and environmentally responsible way. With U.S. headquarters in New Jersey and production and research facilities in four states, Novo Nordisk employs nearly 6,000 people throughout the country. For more information, visit novonordisk.us, Facebook and Twitter.

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2. Srivastava A, Brewer AK, Mauser-Bunschoten EP et al. Guidelines for the management of hemophilia. Hemophilia. 2013;19(1):e1-47.
3. Community counts: The HTC Population Profile. Centers for Disease Control and Prevention website. https://www.cdc.gov/ncbddd/hemophilia/communitycounts/documents/htc-population-profile-report-5-rev2_508compliant.pdf. Accessed May 4, 2018.
4. Rebinyn® Prescribing Information. May 31, 2017. https://www.rebinynpro.com.

May 21, 2018
Globenewswire.com

NovoEight® Maintains Potency when Stored at 40ºC(104°F) Offering People with Hemophilia A Increased Flexibility in Their Daily Lives

A new long term stability study has shown that hemophilia A treatment NovoEight® (turoctocog alfa) remains potent after three months exposure to temperatures of 40ºC(104°F).[1] The results were presented today at the WFH 2018 World Congress in Glasgow, UK.

Treatment storage issues, especially the need for refrigeration, have been shown to be a common problem reported by people with hemophilia A.[2] A portability study recently published in Patient Preference and Adherence found that most people are restricted in their personal activities, particularly travel and sports, due to temperature storage issues.[2] If efficacy and safety are unaffected, storage flexibility was shown to be a strong driver of product choice.[2]

"Establishing the stability of NovoEight® up to 40°C(104°F) is part of our commitment to helping people with hemophilia A to reduce the burden that this condition places on their lives," said Mads Krogsgaard Thomsen, executive vice president and chief science officer, Novo Nordisk. "The recent data demonstrate the reliable nature of NovoEight® as a portable and convenient treatment option, which could help patients enjoy their daily lives without concern about the storage of their treatment."

The storage conditions for NovoEight® allows for it to be kept at temperatures of up to 40ºC(104°F) for three months.[3]

About the study

This was a long-term stability study comparing the potency of NovoEight® stored at 5ºC(41°F) for 30 months to NovoEight® stored at 5ºC(41°F) for 27 months, then followed by storage at 40°C(104°F) +2°C/75% relative humidity (RH) +5% RH for three months. The potency of NovoEight® was maintained at high storage temperature and humidity conditions.

The effect of repeated temperature changes was tested by storage at 5°C(41°F) +3°C/ambient humidity/darkness for 24 months, followed by cycling between 5°C(41°F) and 40°C(104°F)/75% RH up to 10 times; the product was then stored at 40°C(104°F)/75% RH for three months (resulting in a total of four months at 40°C(104°F)), and returned to 5°C(41°F) to attain 30 months shelf life.[1]

The potency of NovoEight® was maintained at high storage temperature and humidity conditions. Up to 10 cycles of temperature changes and storage at 40°C did not affect potency.[1]

About NovoEight®

NovoEight® is a B-domain truncated recombinant human coagulation factor VIII for the treatment and prevention of bleeding in patients with hemophilia A.[3]

About hemophilia A

Hemophilia is a chronic, inherited bleeding disorder that primarily affects males. People with hemophilia A are either missing or have a malfunctioning factor VIII protein, which is needed for proper blood clotting. People with hemophilia A have a tendency to bleed longer than most or to bleed internally into joints, muscles or organs because they are missing this clotting factor. To manage the

disease and stop bleeding, people with hemophilia A must replace the missing factor VIII protein, which is accomplished by intravenous injection of the clotting factor.[4]

Globally, it is estimated that 150,000 people have been diagnosed with hemophilia A.[5] The disease is severely underdiagnosed in developing countries.

References

1. Turoctocog alfa is stable during storage at 40°C and multiple sequences of temperature cycling, poster #52. Presented at the WFH 2018 World Congress, Glasgow, UK, 21 May 2018.
2. Tischer B, Marino R, Napolitano M. Patient preferences in the treatment of hemophilia A: impact of storage conditions on product choice. Patient Preference and Adherence. 2018;12:431-441
3. NovoEight® Summary of Product Characteristics, April 2018
4. Srivastava A, Brewer AK, Mauser-Bunschoten EP, et al. Guidelines for the management of hemophilia. Haemophilia. 2013;19:e1-47
5. World Federation of Hemophilia. Report on the Annual Global Survey 2016. Published October 2017. Available online. Last accessed April 2018.

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Prnewswire.com

coreHEM Publishes Core Outcome Set for Hemophilia Clinical Trials; Prepares to Launch Next Phase of Work

The coreHEM initiative has announced the publication of a core outcome set for clinical trials of gene therapy in hemophilia. The outcomes were developed and agreed upon by a multi-stakeholder group, through an intensive research and formal consensus process. Participating experts and stakeholders included patients, clinicians, payers, health technology assessment groups, regulators, life sciences companies and others. coreHEM has produced the first set of guidelines recommending a specific, minimum set of outcomes to include in hemophilia gene therapy clinical trials. This core set will ensure that patient perspectives on critical outcomes are included in pivotal trials, allow fair comparisons between alternative treatments, and allow more accurate assessments of the value of these therapies. The coreHEM project was jointly led by the Green Park Collaborative (GPC), the National Hemophilia Foundation (NHF), and McMaster University.

The final report, "coreHEM: Developing Comparative Effectiveness Outcomes for Gene Therapy in Hemophilia," details the methods, results, and impact identified by the initiative, as well as the final core outcome set, and preliminary work on measurements and instruments identified for those outcomes. Research from the coreHEM initiative was published today in *Haemophilia*. "Core outcome set for gene therapy in haemophilia: Results of the coreHEM multistakeholder project." (Iorio A, Skinner MW, Clearfield E,et al.; for the coreHEM panel. Core outcome set for gene therapy in haemophilia: Results of the coreHEM multistakeholder project. Haemophilia. 2018;00:1–6. https://doi.org/10.1111/hae.13504)

The recommended core set includes:

- frequency of bleeds,
- clotting factor activity level,
- duration of expression [of clotting factor gene],
- chronic pain,
- utilization of healthcare system (direct costs),
- mental health status.

"The core outcomes set developed through this work has been positively received by all of the participating stakeholders, and we hope that the participating life sciences companies will include these in future trials. We believe that the consensus methods developed for this project may be applicable to a large number of clinical conditions, with similarly positive results," said Sean R. Tunis MD, one of the three principal investigators for this initiative.

Based on feedback from coreHEM's participants, and to ensure that the core set is of optimal value to users, the project team will now develop a consensus set of recommended measures and instruments to be used for consistent and comparable assessment of the recommended core outcomes for pivotal and post-approval trials of gene therapies in hemophilia. This second phase of work will continue to be led by GPC, NHF, and McMaster University, and is expected to launch shortly.

About coreHEM
coreHEM is a multi-stakeholder partnership run by the Green Park Collaborative, in partnership the National Hemophilia Foundation and McMaster University. coreHEM was funded by a grant from the National Hemophilia Foundation and with support from the following life science industry companies and academic gene therapy groups: Bayer AG, BioMarin Pharmaceutical Inc, Pfizer Inc, Shire Plc, Spark Therapeutics, St. Jude Children's Research Hospital, and uniQure B.V. These sponsoring companies fully participated in the project. Additional conference support was provided by Alnylam Pharmaceuticals, Novo Nordisk, and Roche Genentech.

About the Green Park Collaborative
The Green Park Collaborative (GPC) is a major initiative of the Center for Medical Technology Policy, an independent 501(c)(3) non-profit organization dedicated to improving the quality, relevance, and efficiency of clinical research. GPC is a multi-stakeholder forum for developing condition- and technology-specific study design recommendations to guide the creation of evidence needed to inform both clinical and payment decisions.

About the National Hemophilia Foundation
The National Hemophilia Foundation (NHF) is a 501(c)(3) non-profit organization dedicated to finding better treatments and cures for inheritable bleeding disorders and to preventing the complications of these disorders through education, advocacy and research. NHF's programs and initiatives are made possible through the generosity of individuals, corporations and foundations, as well as through a cooperative agreement with the Centers for Disease Control and Prevention (CDC).

About McMaster University
McMaster University in Hamilton, Canada is one of the world's top 100 universities. This medical-doctoral, research-intensive institution has established an international reputation for evidence-based medicine, knowledge-translation research, health informatics, and problem-based learning.

May 22, 2018
Fiercebiotech.com

Spark Hemophilia B Gene Therapy Clears Test En Route to Pfizer-Sponsored Phase 3

Spark Therapeutics has presented data on hemophilia B patients who received a version of its gene therapy manufactured using a modified process. Early data on the patients suggest the new batch of therapies is at least as effective as its predecessor, clearing away a potential pitfall on Spark's path to market.

Philadelphia-based Spark switched to the new vector manufacturing process last year in anticipation of producing the gene therapy, SPK-9001, at commercial scale. The mid-development switch created a risk that the gene therapy Spark and partner Pfizer moved into phase 3 would be less effective than the one that shone in phase 1/2. Data shared this week allay that fear.

Presenting at the World Federation of Hemophilia World Congress, Spark provided data on the first three patients to receive the gene therapy made under the new process. Each of these patients had 12 weeks of follow up, the length of time needed for factor IX activity levels to reach steady state.

Factor IX activity levels in the three patients ranged from 38.1% to 54.5%. Going into the trial, the patients had factor IX activity levels of less than 2%. The readout links the latest gene therapy to big increases in factor IX activity that are in line with those seen in patients treated with product made under the old process. Factor IX levels in these 10 patients range from 14.3% to 76.8%.

The factor IX activity of all 13 patients with 12 weeks of follow up is well above the levels needed to improve outcomes. Across all 15 patients—two of whom lack 12 weeks of follow up—the rate of annualized bleeding is down 98%. The annualized factor IX infusion rate is down 99%. People who used to suffer multiple bleeds a year and take repeated infusions are largely free of these burdens.

Spark is now working with Pfizer to generate the phase 3 data needed to validate the potential of the gene therapy and bring it to market. The next steps are for Spark to transfer the clinical program to Pfizer—something it expects to happen this summer—and provide it with study drug for the phase 3.

If all goes to plan, Pfizer will ace the phase 3 and the phase 1/2 will continue to show the long-term efficacy of the one-shot treatment. That will give Pfizer and Spark a shot at disrupting the existing hemophilia B market while holding off rival gene therapies from companies including uniQure.

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Prnewswire.com

Bayer Awards Grants to Patient Caregiver and Hemophilia Researchers

As part of the esteemed Bayer Hemophilia Awards Program (BHAP), Bayer awarded grants to researchers from the U.S., and Italy, and a caregiver from Colombia, during the World Federation of Hemophilia 2018 World Congress in Glasgow, Scotland. BHAP is the largest program of its kind that funds clinical research and education to support the next generation of care and treatment for people living with hemophilia.

An independent Grants Review and Awards Committee selected the following recipients among 55 entries from around the world:

- Clay Cohen, MD, Baylor College of Medicine, U.S., received the Fellowship Project Award for his proposed research to determine Factor VIII and Factor IX activity in a new endothelial cell-based model of coagulation
- Alessio Branchini, MD, University of Ferrara, Italy, received the Early Career Investigator Award for his proposed research to investigate how engineered therapeutic proteins with enhanced half-life or activity could boost hemophilia B treatment
- Jenny Margarita Palma Romero, Vihonco IPS, Colombia, received the Caregiver Award for her proposed project that seeks to develop training for healthcare professionals to improve the lives of people with hemophilia in Colombia

"I am honored to be a 2018 BHAP award recipient. This grant will help me build a solid foundation as an investigator in the basic mechanisms of pediatric hemostasis-thrombosis and enable me to focus on my goal of advancing the basic understanding of bleeding disorders," said Dr. Cohen. "Continued support of healthcare providers with an interest in hemophilia care is essential to addressing unmet needs and enhancing disease management strategies for this chronic condition."

In BHAP's 16-year history, Bayer has awarded more than 290 grants exceeding $35 million to clinicians and caregivers from 33 countries and contributed to over 400 publications, poster presentations and other scientific communications by awardees.

"We are proud to recognize the work of Dr. Cohen, Dr. Branchini and Ms. Romero and the promise their efforts hold to better our understanding of hemophilia," said Alexandra Vlajnic, M.D., Vice President of U.S. Medical Affairs at Bayer. "Through these grants, Bayer is demonstrating our commitment to the overall health and well-being of patients. Whether enabling scientific advances or training around first-time infusions, we're proud to support both scientific research and caregiver education."

Bayer: Science for A Better Life
Bayer is a global enterprise with core competencies in the Life Science fields of health care and agriculture. Its products and services are designed to benefit people and improve their quality of life. At the same time, the Group aims to create value through innovation, growth and high earning power. Bayer is committed to the principles of sustainable development and to its social and ethical responsibilities as a corporate citizen. In fiscal 2017, the Group employed around 99,800 people and

had sales of EUR 35.0 billion. Capital expenditures amounted to EUR 2.4 billion, R&D expenses to EUR 4.5 billion. For more information, go to www.bayer.us.

Forward-Looking Statements

This release may contain forward-looking statements based on current assumptions and forecasts made by Bayer Group or subgroup management. Various known and unknown risks, uncertainties and other factors could lead to material differences between the actual future results, financial situation, development or performance of the company and the estimates given here. These factors include those discussed in Bayer's public reports which are available on the Bayer website at www.bayer.com. The company assumes no liability whatsoever to update these forward-looking statements or to conform them to future events or developments.

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May 22, 2018
Endpts.com

FDA Chief Gottlieb Is Building a Regulatory Speedway to Accelerate Gene Therapy Development

By Amber Tong

In a rallying cry for gene therapy, FDA Commissioner Scott Gottlieb says he's determined to clear the pathway for drug developers in a move to accelerate the first wave of gene therapies pointed to the market.

The first therapeutic area to benefit from new surrogate endpoints will be hemophilia, Gottlieb said — immediately ringing a bell for companies like Spark Therapeutics, Pfizer, BioMarin and uniQure, which are developing cures for both versions of the bleeding disorder. Under the yet-to-be-announced guidelines, factor production may in some cases be sufficient as a measure of benefit.

Gottlieb discussed the FDA's policy plans for gene therapy Tuesday at the annual board meeting of the Alliance for Regenerative Medicine. Quoting an MIT study that predicts 40 FDA-approved gene therapy products by the end of 2022, he acknowledged both the "breathtaking" pace of progress and his agency's role in facilitating it all.

"FDA has more than 500 active investigational new drug applications involving gene therapy products," Gottlieb said. "We've received more than one hundred such applications last year alone. This shows the intensity of scientific work going on in this field."

To speed things along, Gottlieb suggested, certain gene therapies may qualify for the regenerative medicine advanced therapy (RMAT) designation — a status established by the 21st Century Cures Act that confers all the benefits of fast track and breakthrough designations. Developers may also eventually apply for accelerated approval, where the FDA would be willing to accept more uncertainty in exchange for promising therapies in "devastating diseases." Longterm effectiveness — or even traditional measurements, such as the demonstration of a reduction in bleeding rates in hemophilia — could come in postmarket follow-ups.

"The use of registries and real-world evidence are likely to play an increasingly important role in this respect," the commissioner said. "Part of our goal is to move toward a system that allows more real-time surveillance of safety questions after new products are approved."

But that still leaves the inherent problems in developing and commercializing gene therapies to be solved.

When you compare reviews of cell and gene therapies from those of traditional drugs, Gottlieb pointed out, you see that the breakdown of clinical versus product issues is almost completed inverted. For these therapies, clinical efficacy is often established early, thus taking up only 20% of the review, while reviewers often devote 80% of the process to work out manufacturing and quality concerns.

Gottlieb spotlighted two manufacturing-related issues hindering the development of gene therapy. The inefficient process of producing gene therapy vectors — the lentiviruses and adeno-associated viruses that delivers the "correct" copies of genes to patients — makes it prohibitively expensive. Furthermore,

the conventional pharma paradigm, which separates early-stage pilot manufacturing from the commercial process, means some treatments would be caught up, or even abandoned, in the transition.

The FDA is trying to help on that front, through an initiative to improve the yield of cell lines and by "actively pursuing new investments" in continuous manufacturing (as opposed to batch manufacturing) platforms.

With a field that's moving ahead rapidly and a technology that's going to "transform medicine and human health," the FDA is keen to address any challenges in manufacturing and clinical development, Gottlieb said.

May 23, 2018
Prnewswire.com

Bayer Presents Preliminary Insights from Global Patient Survey at World Federation of Hemophilia 2018 World Congress

Survey results to inform treatment management strategies through life transitions for people living with hemophilia A

Today, Bayer presented preliminary insights from the U.S. segment of the global HemACTIVE patient survey during a satellite symposium at the World Federation of Hemophilia 2018 World Congress in Glasgow, Scotland. These initial survey results showed that among U.S. adults living with hemophilia:

- Nine out of 10 aspire to be active or very active[1], including participating in outdoor activities[2]
- Two out of three are not able to participate in activities they would like to[3]
- Three out of four make adjustments to their activities because of the condition[4], due to fear of bleeds, pain, and joint damage[5]
- One out of two adjust their treatment, such as infuse more often[6], to allow them to be more active

"Although significant progress has been made over the last two decades, people living with hemophilia are still experiencing physical and emotional burdens that can limit their ability to be active," says Mark Skinner, principal investigator of the HemACTIVE survey and past president of the World Federation of Hemophilia. "Our hope is that this survey will help us better recognize how hemophilia A impacts quality of life so physicians can better tailor care to enable patients to fully participate in the things they enjoy."

HemACTIVE is a global research survey in which Bayer polled 330 people living with hemophilia A between the ages of 18-65 years and parents of children ages 2-18 years old living with the disease from the U.S., Germany, France and Italy. Presented today were initial results from the U.S. segment of the survey that questioned American adults ages 18-31. The survey aims to identify how hemophilia A affects the daily lives of people living with this chronic disease and to better understand the role of treatment. Final results of the HemACTIVE survey are anticipated in August 2018.

"Over the past 25 years, Bayer has been committed to understanding and adapting to the evolving needs of people living with hemophilia A," said Aleksandra Vlajnic, M.D., Vice President of Medical Affairs Hematology, Bayer U.S. "We look forward to working with our partners in the hemophilia community to further analyze and apply these survey results and to explore new ways to help patients with hemophilia A."

About Hemophilia A

Hemophilia has an estimated frequency of 1 in 5,000 male live births and affects approximately 400,000 people around the world, including an estimated 20,000 in the U.S. today.[7] It is a largely inherited disorder in which one of the proteins needed to form blood clots is missing or reduced. In hemophilia A, the most common type of hemophilia, blood clotting is impaired as a result of a lack or defect of coagulation Factor VIII (FVIII). Patients therefore repeatedly experience bleeds in muscles, joints or other tissues, which can result in chronic joint damage. External injuries, even if they are

trivial, can have serious consequences if not treated appropriately, as the blood clots more slowly than in healthy individuals.

Hemophilia treatment has advanced considerably over the past decades with life expectancy for people with hemophilia significantly increasing from about 11.4 years in 1920 to a potentially normal life span today.[8] Today's research aims to reduce burden of treatment and improve the quality of life of people with hemophilia.

Bayer: Science for A Better Life

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1. HemACTIVE, Table 19, A9. How active do you wish you could be in your daily life?/does your child wish they could be in their daily life?
2. HemACTIVE, Table 28, A13C. Which of the following activities do you/does your child wish you/they could do?
3. HemACTIVE, Table 36, B3. To what extent do you agree or disagree with the following statements? I am/My child is not able to participate in activities I/they would like to do because of my/their hemophilia
4. HemACTIVE, Table 30, A15. How many days in a typical week do you make adjustments to your/does your child make adjustments to their activity level because of hemophilia?
5. HemACTIVE, Table 32, A17. Why do you make adjustments to your/does your child make adjustments to their activity level because of hemophilia?
6. HemACTIVE, Table 77, C15. Below are several statements about hemophilia products. For each two pairs of statements, please select the one point on the scale that best describes the way you feel about your/child's hemophilia products.
7. Fast Facts (2015, July 15). Retrieved October 19, 2017, from: https://www.hemophilia.org/About-Us/Fast-Facts
8. Hemophilia and Aging (2014). Retrieved October 19, 2017 from: https://www.hemophilia.org/sites/default/files/document/files/Nurses-Guide-Chapter-17-Aging.pdf