Agenda

- Pfizer Global Hemophilia Franchise: A *History of Leadership*
- US Clinical Program Update
- IIRs – Key Areas of Interest
- Future Directions in Hemophilia Research
- Product Enhancements
Pfizer: A Brief Overview

- In October 2009, two companies—Pfizer and Wyeth—came together to form the largest biopharmaceuticals company in the world.

- At Pfizer, our mission is to apply science and our global resources to improve health and well-being at every stage of life.
  - Pfizer is comprised of nine diverse health care business units and has a strong global presence.
  - Together with our vaccine and immunology portfolios, Pfizer is the world’s premier biopharmaceutical company.
Hemophilia and the Pfizer Specialty Care Business Unit

- Our Specialty Care Business Unit (SCBU) is focused on high priority disease areas and enhanced biologic capabilities.

- Hemophilia and the advancement of biotherapeutics is a key focal point for the SCBU.
A Heritage of Groundbreaking Technology

*ReFacto is no longer marketed in the U.S.*
Pfizer Provides Ongoing Support that Fosters the Advancement of Hemophilia Treatment
We Work Closely with Partners to Improve Hemophilia Care Worldwide

For nearly a decade, Wyeth (now Pfizer) collaborated with the World Federation of Hemophilia (WFH) to support their efforts in improving hemophilia care around the globe.

Wyeth (now Pfizer) has acted as the sole corporate sponsor of the world-renowned Twinning Program, which connects emerging treatment centers with more-established centers around the world.

- As of June 2010, there are 31 Twinning Partnerships worldwide.
We Provide Access to Hemophilia Medicines to Patients Who Need Them

- Wyeth, now Pfizer, has been the largest single donor of hemophilia products to the WFH.

- Pfizer is currently partnering with other organizations to donate hemophilia products to patients in emerging countries.
We Offer Ongoing Support to Children’s Hemophilia Camping Initiatives

- Globally, we recognize the unique importance of the summer camp experience to children in the bleeding disorders community.

- Pfizer is the exclusive and executive sponsor of NACCHO (North American Camping Conference of Hemophilia Organizations), which is the only conference dedicated to sharing best practices for bleeding disorder summer camps.
We Continually Strive to Advance Our Offerings to the Hemophilia Community

The goal of our ongoing research is aimed at enhancing the convenience of our current treatments and expanding the science of treating hemophilia.

Rapid Reconstitution System
This system is designed to help you spend less time reconstituting and to make infusion more apt to fit into your lifestyle.

- Low diluent volume of only 4 mL
- Prefilled syringe is easy to handle
- Needle-less transfer system eliminates needlestick injuries during reconstitution
- Distinct, clear vial adapter

Pfizer Specialty Care
ADVANCING SCIENCE. IMPROVING LIVES.
Furthering the Science of Hemophilia Treatment through Strategic Relationships

Through strategic relationships, we continue to lead research into emerging technologies aimed at creating additional treatment options for patients with hemophilia and their loved ones.
For over 150 years, Pfizer has been changing the course of diseases and lives through the introduction of new treatments.

By researching novel therapies and strengthening our relationships, we sustain our commitment to the hemophilia community.

Our business has a direct impact on the patients we serve—and with that, comes great responsibility.

We reinforce our pledge to act with integrity in our efforts at enhancing the lives of the hemophilia community — today, tomorrow, and beyond.
US Clinical Program Update

May Orfali, MD
Medical Lead Hematology
Hematology: BeneFIX- Study 400

• Study Design
  • An open-label, randomized, crossover study of on-demand therapy and 2 prophylaxis regimens of BeneFIX (100 IU/kg once weekly and 50 IU/kg twice weekly) in subjects with moderately severe to severe hemophilia B (FIX:C ≤2%)

• Study Rationale
  • To provide data on prophylaxis treatment with BeneFIX for publication and potential hypothesis for larger study to obtain “prophylaxis” indication

Study Status Update:

• Study start (FPFV): May 2007
• Study complete (LPLV): Nov 2010
• Actual enrollment: 50 enrolled, 47 randomized (Enrollment closed)
BeneFIX- Study 400

Dose is investigator’s discretion

Screening → On demand

Time (mo) 4 4 2 4

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50 IU/kg 2x/wk 100 IU/kg weekly 50 IU/kg 2x/wk

On demand

100 IU/kg weekly

Total duration of study is approx. 59 wks/subject
Study Design
- 2-segment, 2-year, open label, multicenter study in moderately severe to severe hemophilia A patients

Study Rationale
- to supplement the label with PK, safety and efficacy data in pediatric patients (under 6 years of age)
- to fulfill a postmarketing commitment to compare to two prophylaxis regimens
- to gain a prophylaxis indication.

Study Status Update
Study start (FPFV): Jan 2008
Study complete (LPLV): July 2013
Actual subjects: 31 (72 planned)
Study design

Two year open-label, multi center study in Hemophilia A subjects that has an observational and active component for the safety assessment.

Primary objective

- Evaluate FVIII inhibitor development, defined as an inhibitor titer of \( \geq 0.6 \) BU using the Nijmegen modification of the Bethesda assay and verified by the central laboratory

Study Status Update

- Study start (FPFV): Feb 2009
- Study complete (LPLV): Mar 2013
- Actual subjects: 6 (50 planned)
**What is an IIR?**

- Investigator-initiated research (IIR) is conducted by an investigator, institution or organization (who is the sponsor of a study) and is designed for the development of specific and defined medical knowledge.
  - The sponsor of a study is the person who takes responsibility for and initiates a clinical investigation. The sponsor may be another pharmaceutical company, a private or academic organization, cooperative group, government agency or an individual (MD, PhD, NP, etc.).

  **Pfizer is never considered the sponsor for IIR studies.**

- Pfizer support may be provided in the form of pure compound, formulated drug, funding, or a combination of these, depending upon the type of research. If other non-monetary services are requested such as laboratory assays, PK analysis, etc., these can also be considered.

- Across the industry, IIRs may also be known as IOPs, IITs, ISS, IIS, ISRs, etc.
IIR Submission

Types of Research Eligible for Support through an IIR:

- **Clinical studies** of approved and unapproved uses, involving approved or unapproved Pfizer drugs
- **In vitro** or **animal studies** which include funding
- **Observational studies**, such as epidemiology studies and certain outcomes research studies where the primary focus is the scientific understanding of disease
- Other types of **independent research on disease states**, including novel diagnostic screening tools and surveys where Pfizer has no direct commercial interest

All IIRs should have compelling scientific merit and not pose a concern for patient safety. When considering IIRs for a given asset program, it should be understood that Pfizer does not own the data and therefore cannot use study results for promotion.
Hemophilia IIR- Key topics of Interest

Key Topics of Interest:

- Epidemiology / burden of disease / Outcome Research
- Patient adherence to prescribed regimen
- Routine prophylaxis and preventative treatment
- Surgical prophylaxis, dosing
- On Demand dosing
Hemophilia IIR-Key Topics of Interest

- Treatment of inhibitors: Immune Tolerance Therapy, inhibitor bypass tx
- Switching experience
- Manage adolescents hemophilia patients (QoL)
- Manage aging hemophilia population
- Basic science: Point of differentiation study
- Clinical monitoring of hemophilia treatment
- Recovery experience (hemophilia B patients)
IIR- How to Apply?

1. Speak with your RMRS directly, or

2. Go to www.pfizer.com/iir

3. click on “submit an IIR request” (blue button on the right side of the screen)

This will direct you through the IIR process

This site also allows you to monitor the status of your research proposals