A Longitudinal, Observational Study of Previously Treated Hemophilia Patients (PTPs) Switching Factor Preparations

ATHN 2 Breakfast Breakout Session
October 30, 2015
Agenda:

Welcome!

• Introductions
• Study Update
• Recruitment
• Good Documentation Practices in Clinical Research
• Feedback and Questions
Current Timeline

- **Jun 2015**: Protocol Approved by SC
- **Jul 2015**: Initial IRB Approval
- **Sept 2015**: First Patient In
- **Oct 2015**: Study Actively enrolling at Multiple Sites
- **2016-2020**: Accrual
- **2021**: Study Completed
Study Update: Recruitment

Study Population

The study will enroll approximately 600 patients with hemophilia and are receiving care from one of the HTCs into:

• Arm A (Prospective): patients who are switching factor replacement products
  – will be followed prospectively for up to 1 year

• Arm B (Retrospective): patients who have switched factor replacement products previously (within the past 50 weeks at the time of enrollment).
  – will be assessed retrospectively and/or followed prospectively for up to 1 year
Study Update: Recruitment
As of 10/15/15

• Site Participation:
  – 15 Sites have agreed to participate, 3 are open
    • Western Pennsylvania, Pittsburgh, PA, Dr. Ragni
    • St. Josephs, Tampa FL, Dr. Cockrell
    • Boston Children’s Hospital, Boston, MA, Dr. Croteau

  – More Sites to be recruited (~10) for up to 30 sites
    • Each site should enroll at least 5 subjects

• Accrual to Date
  – Arm A – 1 Subject!
  – Arm B – first subject pending
Study Update: Version 2.0

• Amendment issued September 4, 2015
  – Adds baseline inhibitor screen (Sample stored, or result)
  – Clarifications regarding Arms
  – Updates to Schedule of Assessments (next slide)
  – Other minor clarifications

• Study Manager
  – Forms reflect Version 2.0

• Manual of Operations available
  – Includes tools and forms
# Schedule of Assessments: Version 2.0

<table>
<thead>
<tr>
<th>STUDY PROCEDURES</th>
<th>SCREEN</th>
<th>CYCLE 1</th>
<th>SUBSEQUENT CYCLES (2 – 7)</th>
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<td>Product-specific Form: as applicable</td>
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10/23/201
What Study Procedures are not “Standard Care?”

• Stored sample for baseline inhibitor assay, if required
• Inhibitor assay at 10 exposure days
• Q3 monthly phone follow up
• Product Specific Modules / Questionnaires
Screening

• Identify patients planning to switch soon (ideally within 1 month)

• Screening performed prior to seeing patient for Consent to determine Eligibility (because the screening would be necessary to switch factors anyway)

• A Screening ‘Visit’ may not be needed with subject/family.
  – No tests to determine eligibility are required by protocol, other than Inhibitor test to establish baseline, prior to switch
Study Update: Recruitment

Eligibility Questions

• Timing of Baseline Inhibitor Test in relation to Switch
  – Clarified by Arm in Version 2.0, Section 4.1

• Number of Exposures
  – Must not be treatment naïve/PUP
  – Must be 2 years old
  – Must have had at least 50 Exposure Days with previous Factor
Study Update: Recruitment

All recruitment materials must be approved by your IRB prior to use

• Recruitment brochure
  – Available in English and Spanish
  – Brochures to be sent in bulk to sites
  – Sites may add HTC and Contact Information to back panel

• Recruitment script
  – To contact potential subjects by phone.
  – Modify with local site preferences, as appropriate, or use as is

• Powerpoint slides
  – To present at local and regional meetings
Substudies: Product-Specific Modules

• ATHN working with Industry Partners for selected factor products to collect post-approval data
  – Will be limited to questions about factor use, packaging, patient preferences and similar ‘non-clinical’ data

• Substudies are voluntary for subjects
  – Add-on questionnaires to be administered to subjects receiving specific factor products

• Substudy IRB approval may or may not be required by your institution
Substudies: Product-Specific Modules

• Several sponsors in play
  – Contracts and budgets pending

• Update on current strategy
  – Possible addition of a validated Adherence Tool
Feedback on Study Logistics

Open for Discussion
Administration and Project Plans

• Site Administration at your HTC
• Future Plans
Site Administration: Payments

• Schedule: Annual Payments for 2015-2016
• ATHN will send HTC a Payment Report
  – # Patients Completed
  – # Inhibitor Report Forms Completed
  – # Product Specific Modules Completed
• HTC will review and submit as Invoice
• ATHN will pay HTC based on amounts negotiated
• Contact Cynthia Branch  cbranch@athn.org
  – ATHN National Contract Manager
Contract / Budget

Questions and Feedback
Currently in Development

- Manual of Operations Updates as needed
- Requests?
Future Plans

• Factors presently in pipeline
  – Each becomes eligible as a study product as it achieves FDA approval
• Not every manufacturer will opt for a product-specific module
• Ideally we will catch future factors mostly or entirely in Arm A (prospective)
If your HTC wants to participate in ATHN 2

- Contact Dr. Neufeld or any member of the ATHN 2 Steering Committee to express interest
- Submit Current Protocol and Informed Consent Template to your IRB, or to Western/Copernicus (WIRB)
- Work with ATHN on Contract Execution
  - Cynthia Branch, National Contracts Manager, ATHN
- Participate in Site Initiation Webinar
  - Ellen McCarthy, Clinical Research Project Manager, ATHN
Other Questions?

Open for Discussion
Thank You!
Good Documentation Practices for Clinical Research
Securing Data.
Advancing Knowledge.
Transforming Care.