Glenn Pierce, MD, PhD
World Federation of Hemophilia Board of Directors and Former Senior Vice President Hematology
Biogen, Inc.
Innovation in Hemophilia: From Blood to Genes, and the Unintended Consequences Along the Way

Glenn Pierce MD PhD
World Federation of Hemophilia Board of Directors and Former Senior Vice President, Hematology, Cell and Gene Therapy, Biogen, Inc.
La Jolla, CA USA

Disclosures

• Advisor for BioMarin, Genentech/Roche (hemophilia)
• Boards of WFH and WFH-USA, NHF MASAC, WFH MAB (bleeding disorders)
• Third Rock Ventures Entrepreneur-in-residence and Voyager Therapeutics Board (AAV)
• Born with severe Factor VIII deficiency
Agenda

- What is hemophilia?
- How has treatment evolved over the past 50 years?*
- The present and future: Bioengineered factor and non-factor replacement pipeline
- The future: Gene therapy status
- World view*

* With sociopolitical commentary along the way

Pierce and Pipe, A Cornucopia of Therapies Under Study for Hemophilia, Molecular Therapy (2017), https://doi.org/10.1016/j.ymthe.2017.10.009
What Is Hemophilia?

- Deficiency of Factor VIII or Factor IX, which work together in the clotting factor cascade, to produce a thrombin generated clot
- X-linked, 1:5000 (FVIII) to 1:25000 (FIX) male births
- Absence means little thrombin generation and weak, friable clots leading to prolonged bleeding
- Internal bleeding - joints, muscles, life-threatening intracranial and intraabdominal bleeds

Hemophilia Untreated or Poorly Treated
Judith Graham Pool- Cryoprecipitate discovery

Judith Graham Pool, Ph.D., and Angela E. Shannon, B.S.
Production of High Potency Concentrates of Antihemophilic Globulin
In a Closed Bag System — Assay in Vitro and in Vivo
The Beginning of Modern Treatment

- “Primitive” protein purification in mid-60s to partially purify clotting factors from plasma
- Creation of entire plasma products industry
- “Purified” clotting factor concentrates <<1% FVIII or FIX
- More pure than Cryo, which was more concentrated than FFP
- Improved purification technologies, including MAb columns, led to ~99% pure FVIII and FIX by late 1980s

Conventional recombinant and plasma derived clotting factor concentrates
Impact of Technology on Hemophilia Treatment

Evolution of Products

Therapeutic Value

- **Investigational therapies (2015–)**
  - Gene therapy
  - Novel agents

- **EHL clotting factors (2014–)**
  - Prolonged half-life (FVIII/FIX)

- **Recombinant clotting factors FVIII, FIX, FVIIa (1990s)**
  - Protein engineering era

- **Plasma-derived clotting factors (1969)**
  - Widespread viral contamination

- **Protein engineering era**
  - Widespread viral contamination

- **Recombinant era**
  - EHL clotting factors (2014–)
  - Prolonged half-life (FVIII/FIX)

- **Investigational therapies (2015–)**
  - Gene therapy
  - Novel agents

- **24/7 coverage**
  - More prophylaxis
  - Some prophylaxis
  - On-demand

No Change- Half Century Later

Or 100 years later...
Promises Not Kept

- Plasma-derived product is plentiful and cheap
- Our contaminated plasma-derived concentrates are safe
- Recombinant-derived product is plentiful
- rFactor is very inexpensive to make (true) and sell (not true)
- There is nothing we can do for the 70% untreated (that promise kept until 2015)

We have had a very difficult relationship with the manufacturers of clotting factor replacement therapy for the reasons outlined above

Why Were Extended Half Life Products Developed?

- Conventional clotting factors have short half lives
- Frequent IV infusions required to control bleeding
- Extended half life FVIII and FIX decrease frequency and burden of treatment
- Conventional product prophy regimens don’t prevent crippling

Extension of Half-Life Technologies

PEGylation

Fc-fusion

Albumin-fusion

Half life $t_{1/2}$ is the amount of time it takes for 50% of the dose to disappear from the circulation. Longer is better...

[IG: Immunoglobulin G; PEG: Polyethylene glycol]

---

FVIII and FIX Extended Half Life (EHL) Products

<table>
<thead>
<tr>
<th>Engineered protein</th>
<th>Company</th>
<th>Status</th>
<th>Half-life (hours)*</th>
</tr>
</thead>
<tbody>
<tr>
<td>rFVIII-Fc (Eloctate)</td>
<td>Bioverativ</td>
<td>Licensed 6-14</td>
<td>19</td>
</tr>
<tr>
<td>PEG-FVIII (Adynovate)</td>
<td>Shire</td>
<td>Licensed 12-15</td>
<td>14.3</td>
</tr>
<tr>
<td>GlycoPEG FVIII (N8-GP)</td>
<td>Novo Nordisk</td>
<td>Phase 3 (2017-18)</td>
<td>18.4</td>
</tr>
<tr>
<td>PEG-FVIII (BAY94-9027)</td>
<td>Bayer</td>
<td>Phase 3 (2018)</td>
<td>19</td>
</tr>
<tr>
<td>Single chain FVIII (Afstyla)</td>
<td>CSL</td>
<td>Licensed 6-16</td>
<td>14.2</td>
</tr>
<tr>
<td>FIX</td>
<td></td>
<td></td>
<td></td>
</tr>
<tr>
<td>rFIXFc (Alprolix)</td>
<td>Bioverativ</td>
<td>Licensed 3-14</td>
<td>82.1</td>
</tr>
<tr>
<td>GlycoPEG-FIX (N9-GP, Rebinyn)**</td>
<td>Novo Nordisk</td>
<td>Licensed 4-17 (not prophy)</td>
<td>93</td>
</tr>
<tr>
<td>rIX-FP (Idelvion)</td>
<td>CSL</td>
<td>Licensed 3-16</td>
<td>101.7</td>
</tr>
</tbody>
</table>

Key Findings:
- 3 FVIII EHLs 50% increased half life
- 3 FIX EHLs 4-5x increased half life
- All EHLs effective; schedules interchangeable like Gen1-3 rFVIII/rFIX
- Some have specific assay requirements.

* Conventional FVIII t1/2 is ~12 hours and FIX is ~19-24 hours

**FDA no prophy; EMA no kids
Further Improvements- Bioengineered Pipeline

<table>
<thead>
<tr>
<th>Molecule</th>
<th>Status</th>
<th>Dosing</th>
<th>PK, Efficacy</th>
<th>Company</th>
</tr>
</thead>
<tbody>
<tr>
<td>FIX Fc-XTEN, BIVV001</td>
<td>Preclinical</td>
<td>SQ, ≥1/week</td>
<td>Mice, monkeys</td>
<td>Bioverativ</td>
</tr>
<tr>
<td>FVIIIIFc-VWF-XTEN, BIVV002</td>
<td>Phase 1/2</td>
<td>~1/week</td>
<td>Mice, monkeys</td>
<td>Bioverativ</td>
</tr>
<tr>
<td>VWF-Albumin, CSL626</td>
<td>Preclinical, Ph1/2 H1,18</td>
<td>monkeys</td>
<td></td>
<td>CSL</td>
</tr>
<tr>
<td>rVIIa-FP, CSL689</td>
<td>Phase2/3 On demand, inhibitors</td>
<td>FVIIa levels up to 48 hr</td>
<td>Normal human volunteers, inhibitor pts</td>
<td>CSL</td>
</tr>
</tbody>
</table>

rFVIIIIFc-VWF-XTEN

Novel fusion protein, consisting of:

- **D’D3 domains of VWF** provide protection & stability of VWF while evading half-life limitation of endogenous VWF
- **XTEN polypeptides**, which improve the pharmacokinetic profile and degrade naturally
- **rFVIII fused to dimeric Fc** which maintains thrombin-mediated release of FVIII from VWF like natural FVIII. Once released FVIII will then bind phospholipids and participate in the clotting cascade

• rFVIIIfc-VWF-XTEN
• Entering clinic H2, 2017
• Expected to provide FVIII activity coverage for at least 1 week

Seth Chhabra, Blood 2015 126:2279;

Shutting Down Coagulation: Inhibitors

• TFPI is an endogenous plasma inhibitor of coagulation. It limits the activity of Factor Xa and Factor Vlla/Tissue Factor in the extrinsic pathway.

• Antithrombin is a serpin that inactivates Xa, IXa, XIa, XIIa, Vlla, and IIa

Inhibit the inhibitors
• Anti-TFPI MAb
• Antithrombin siRNA
### Novel Therapies Pipeline

<table>
<thead>
<tr>
<th>Molecule</th>
<th>Status</th>
<th>Dosing</th>
<th>PK, Efficacy</th>
<th>Company</th>
</tr>
</thead>
<tbody>
<tr>
<td>Anti-TFPI, PF-06741086, MAb</td>
<td>Post-Ph1</td>
<td>SQ ?/2 weeks</td>
<td>Stops acute bleeding in mice; no clin data</td>
<td>Pfizer</td>
</tr>
<tr>
<td>Anti-TFPI, NN7415, Concizumab</td>
<td>Ph1 (2010); 2nd Ph1/2 inhibitors</td>
<td>? weekly</td>
<td>?</td>
<td>Novo</td>
</tr>
<tr>
<td>Anti-TFPI, BAY1093884, MAb</td>
<td>Ph1</td>
<td>IV and SQ, dose escalating</td>
<td>Preclin mouse, NHP efficacy; No clinical data</td>
<td>Bayer</td>
</tr>
<tr>
<td>Fitusiran, siRNA</td>
<td>Pre-Ph3</td>
<td>monthly</td>
<td>Clinical data</td>
<td>Alnylam</td>
</tr>
<tr>
<td>Emicizumab, Bispecific MAb</td>
<td>Filed (inhibitors) Ph3 (non-inhib)</td>
<td>Weekly-monthly</td>
<td>Results inhibitor pts filed</td>
<td>Genentech-Chugai-Roche</td>
</tr>
</tbody>
</table>


### Fitusiran

**Investigational RNAi Therapeutic for Treatment of Hemophilia**

**Fitusiran (ALN-AT3)**
- SC-administered small interfering RNA (siRNA) therapeutic targeting antithrombin (AT)
  - Non-biologic, chemically-synthesized, with targeting ligand to specifically deliver to liver—site of AT synthesis
  - Harnesses natural RNA interference (RNAi) mechanism for regulation of plasma AT levels

**Therapeutic hypothesis**
- Hemophilia A and B are bleeding disorders characterized by ineffective clot formation due to insufficient thrombin generation
- Fitusiran is designed to lower AT, with goal of promoting sufficient thrombin generation to restore hemostasis and prevent bleeding
  - Observation of ameliorated bleeding phenotype in patients with co-inheritance of thrombophilic traits in hemophilia
  - Supported by pre-clinical data and emerging Phase 1 clinical results

**One fatal SAE (intracerebral venous thrombosis) occurred after data transfer date. 1 Sep 17 dosing temporarily suspended**
Fitusiran Serious Adverse Event

- Hemophilia A w/o inhibitors
- First dose fitusiran Aug 2015, since Mar 2016 80mg/month
- Patient reported bleed free since Aug 2016
- Aug 2017: exercise induced hip pain, 3 doses FVIII 31-46 IU/kg on 3 separate days; severe headache and vomiting
- R/O spinal meningitis, LP performed under FVIII coverage
- CT: subarachnoid hemorrhage 1 week after hip pain; patient treated w FVIII 2-3x/day
- Patient died day 16 post hip pain
- Central venous sinus thrombosis diagnosed following re-review of CT scans; Recent AT level was 16%

How does FIX/FX bispecific antibody work?

Emicizumab supports the interaction between FIXa and FX, and thereby promotes FX activation and accelerates coagulation.

HAVEN 1 primary endpoint
Randomized comparison of treated bleeds

- Median ABR calculated with negative binomial regression model.
- Median ABR calculated by number of bleeds/duration of efficacy period in days*365.25.
- CI, confidence interval; IQR, interquartile range.
- ABR calculated with negative binomial regression model.
- Median ABR calculated by number of bleeds/duration of efficacy period in days*365.25.

**Primary endpoint**

- **87% reduction** in bleed rate with emicizumab prophylaxis
- **23.3** to **2.9** (12.33 to 43.89 to 1.69 to 5.02)

**Arm A**

- Emicizumab prophylaxis

**Arm B**

- No prophylaxis (episodic BPAs only)

**Patients (%)**

- **0 bleeds**: 5.6%
- **1–3 bleeds**: 22.9%
- **4–10 bleeds**: 44.4%
- **>10 bleeds**: 100%

**Arm A**

- Emicizumab prophylaxis

**Arm B**

- No prophylaxis (episodic BPAs only)

**Primary analysis data cut-off – October 25, 2016**


**HAVEN 1**

**Emicizumab pharmacokinetics**

- **Pharmacokinetic/pharmacodynamic modeling predicted emicizumab concentration ≥45 µg/mL would result in ≥50% of patients achieving zero bleeds**
- **Target met with weekly subcutaneous dosing: mean trough plasma concentrations >50 µg/mL achieved and sustained once steady-state was reached.**

HAVEN 1 safety summary
All emicizumab patients

<table>
<thead>
<tr>
<th>Event</th>
<th>Total (N=103)</th>
</tr>
</thead>
<tbody>
<tr>
<td>Total number of adverse events (AEs), n</td>
<td>198</td>
</tr>
<tr>
<td>Total patients ≥1 AE, n (%)</td>
<td>73 (70.9)</td>
</tr>
<tr>
<td>Serious AE*</td>
<td>9 (8.7)</td>
</tr>
<tr>
<td>Thrombotic microangiopathy (TMA)**</td>
<td>3 (2.9)</td>
</tr>
<tr>
<td>Thrombotic event</td>
<td>2 (1.9)</td>
</tr>
<tr>
<td>Death**</td>
<td>1 (&lt;1)</td>
</tr>
<tr>
<td>AEs leading to withdrawal</td>
<td>2 (1.9)</td>
</tr>
<tr>
<td>Grade ≥3 AE</td>
<td>8 (7.8)</td>
</tr>
<tr>
<td>Related AE</td>
<td>23 (22.3)</td>
</tr>
<tr>
<td>Local injection-site reaction</td>
<td>15 (14.6)</td>
</tr>
</tbody>
</table>

*Third TMA event occurred after primary data cut-off, patient also experienced fatal rectal hemorrhage

Thrombotic events were skin necrosis/superficial thrombophlebitis in one patient, and cavernous sinus thrombosis in a second patient

No patients tested positive for anti-drug antibodies

5/8 patients using aPCC products >100 U/kg for >1 day developed thrombi


HAVEN 1 updated data
Assessment of interaction between emicizumab and aPCC

- TMA/thrombotic events only occurred with aPCC treatment averaging >100 U/kg daily for ≥24 hours
- aPCC contains activated and non-activated coagulation factors, including FII, FVII, FIX, and FX, which can accumulate with repeat dosing
- Risk may be mitigated with careful dosing guidance
- No further events in >350 patients treated in emicizumab development program to date

Summary: the Pipeline Pre-Gene Therapy

• Approved extended half life products gaining market share

• 2 shots on goal for FVIII that lasts at least a week
  • FVIII-Fc-VWF-XTEN entering Phase 1/2

• >2 shots on goal to inhibit the inhibitors of clotting
  • Multiple Phase 1/2 trials for anti-TFPI; Fitusiran entering Phase 3?

• Anti-FIX/FX bispecific antibody, Emicizumab most advanced non-factor replacement therapy
  • Data in adults and children with inhibitors show efficacy

• What does all this mean for conventional FVIII and FIX?

Why Gene Therapy for Hemophilia?

**FVIII/FIX REPLACEMENT THERAPY**
- Replacement products are expensive and demanding treatment
  - 3-4x weekly IV
  - New EHLs still require 2x weekly IV for FVIII, 1x every 1-2 weeks IV for FIX
  - Optimal therapy = high burden
  - Venous access challenging
- Factor levels not consistent
  - Saw tooth response
  - Breakthrough bleeding at trough factor levels
  - Bleeding sequelae including joint damage continue

**GENE THERAPY**
- Single gene disorder
  - Clear cause and effect relationship
- Wide therapeutic window (50-150%)
  - Low levels effective
  - High levels effective
- Efficacy easy to assess
  - Clinical - bleeding
  - Laboratory – Factor levels
- First in-human studies
  - Adeno-associated virus (AAV) vector serotypes AAV 2, 5, 6, 8, 10
  - Liver specific promoters
  - Codon-optimized expression cassettes
  - B-domain-deleted human FVIII
  - R338L (Padua) variant of FIX

---

Why Gene Therapy? Because the System is Broken

**Developed world** exploited by anachronistic goal dosing to 1% troughs...joints still bleeding

Prophy dosing to 1% troughs: >$300K/year FVIII, >$600K/year FIX

10-15% troughs needed for full protection (4-5x increase in dosing regimen)

Low manufacturing costs make problem fixable but no collective will

Solution: non-factor therapies and gene therapy
Gene Therapy: Genes Must Enter Cells

- Viral vectors: AAVs, AdVs, and LVs, etc.
- Non-viral vectors: polymers, liposomes, and CPPs, etc.
- Physical methods: microinjections, and electroporation, etc.


**Adeno-associated Virus (AAV) Gene Transfer**

DNA (gene) → Gene encapsulated in AAV → Gene therapy → AAV releases gene into cell → Gene expresses protein → Secreted protein

Target cell
**Tissue tropism is “relative”**
Pre-existing immunity ~50% for most serotypes
“Non-integrating”


**Gene Therapies Pipeline: FIX Clinical Trials**

<table>
<thead>
<tr>
<th>Molecule</th>
<th>Status</th>
<th>Dosing vg/kg</th>
<th>Efficacy</th>
<th>Company</th>
</tr>
</thead>
<tbody>
<tr>
<td>AAV2-FIX</td>
<td>Closed Ph1/2</td>
<td>6E12, liver delivery</td>
<td>12% for 6 weeks, immune response ID'ed</td>
<td>Avigen-Bayer</td>
</tr>
<tr>
<td>AAV8-FIX</td>
<td>Reopened Ph1/2</td>
<td>2E12 high dose IV</td>
<td>2-6%</td>
<td>UCLondon-St Jude</td>
</tr>
<tr>
<td>AAV8-FIX R338</td>
<td>Closed 2016</td>
<td></td>
<td>0-26%, most lost FIX</td>
<td>Shire</td>
</tr>
<tr>
<td>AAV8*-FIX R338</td>
<td>Ph1/2, SPK-9001</td>
<td>SE11</td>
<td>16-81%</td>
<td>Spark-Pfizer</td>
</tr>
<tr>
<td>AAV5-FIX AMT-060/061 (R338)</td>
<td>Ph1/2</td>
<td>SE12-1E13</td>
<td>5-7%, ABR 59/76% lower</td>
<td>UniQure</td>
</tr>
<tr>
<td>AAVrh10-FIX</td>
<td>Ph1/2, stopped</td>
<td></td>
<td>10-20%; immune response, high LFTs</td>
<td>Dimension</td>
</tr>
<tr>
<td>AAV6-FIX</td>
<td>Ph1/2</td>
<td>Safe harbor in Albumin gene</td>
<td>Trial open, gene editing</td>
<td>Sangamo</td>
</tr>
</tbody>
</table>

*92% AAV8*
## Gene Therapies Pipeline: FVIII Clinical Trials

<table>
<thead>
<tr>
<th>Molecule</th>
<th>Status</th>
<th>Dosing vg/kg</th>
<th>Efficacy</th>
<th>Company</th>
</tr>
</thead>
<tbody>
<tr>
<td>AAV**-FVIII</td>
<td>Ph1/2, SPK-8011</td>
<td>5E11 1E12 in progress</td>
<td>11 and 14% @ 23/12 weeks</td>
<td>Spark</td>
</tr>
<tr>
<td>AAV5-FVIII</td>
<td>Ph1/2, BMN270</td>
<td>6E13</td>
<td>~100%@52weeks</td>
<td>BioMarin</td>
</tr>
<tr>
<td>AAV6-FVIII, SBS25</td>
<td>Ph1/2</td>
<td>?</td>
<td>Trial open</td>
<td>Sangamo</td>
</tr>
<tr>
<td>AAV8-FVIII, GO-8</td>
<td>Ph1/2</td>
<td>FVIII-V3</td>
<td>Trial open in UK</td>
<td>St. Jude/UCLondon</td>
</tr>
</tbody>
</table>

** AAV serotype not disclosed

---

**AAV5-hFVIII-SQ [BMN 270] VECTOR GENOME SCHEMATIC

AAV5 Seroprevalence/Humoral immunity:
21 Screened; 2 were positive by total antibody assay (TAB)
Kinetics of FVIII activity levels in 4 weekly intervals for subjects administered BMN 270 with 6e13 vg/kg

Median/Mean FVIII activity values were utilized within a visit window. Excludes FVIII values within 72 hours of exogenous FVIII administration. X denotes data point greater than 1.5 IQR above 75th percentile, or less than 1.5 IQR below 25th percentile.

ABRs Post BMN 270: Bleeding stopped after FVIII expression >5%

ABR is calculated as [(the number of bleeding episodes/total number of days during the calculation period) x 365.25]. Note that the figure includes only the 6 subjects in Cohort 3 who were receiving prophylactic exogenous FVIII replacement therapy prior to study enrollment.

The annualized Factor VIII usage is calculated as [(the number of infusions of exogenous FVIII replacement therapy/total number of days during the calculation period) x 365.25]. Note that the figure includes only the 6 subjects in Cohort 3 that were receiving prophylactic exogenous FVIII replacement therapy prior to study enrollment.
Sustained mean FIX activity levels for 10 subjects infused with SPK-9001 at dose of 5 x 10¹¹ vg/kg

Source: Spark data as of June 5, 2017; most recent update. Participant 1 80 week data as of June 23, 2017. FIX:C Activity = circulating factor IX activity level
EHL-rFIX – Extended half-life recombinant factor IX

First: 33% at 80 weeks
Second: 43% at 52 weeks
Third: 24% at 52 weeks
Fourth: 44% at 52 weeks
Fifth: 38% at 52 weeks
Sixth: 18% at 43 weeks
Seventh: 16% at 42 weeks
Eighth: 25% at 32 weeks
Ninth: 79% at 28 weeks
Tenth: 30% at 22 weeks

*Participants 7 and 9 experienced asymptomatic, transient elevation in liver enzymes, or decline in FIX activity, potentially indicative of an immune response to the Spark100 vector capsid. Both received a tapering course of corticosteroids (now completed) after which their ALTs returned to baseline, while Factor IX activity levels have remained stable. As of June 5th, neither participant had experienced a bleed nor taken factor concentrate.

**THE CURE**

If You Build It, Will They Come?

- The Cure has existed for 40 Years
- Liver transplants cure hemophilia A and B (with some side effects and a mortality rate)
- Clotting factors start getting made when the new liver plumbing is connected
- No more 1% troughs. No more bleeding. Period.

Weird... Strange... Liberating... Reborn...
Transformational...
Indescribable: from every waking moment of every single day to “just” arthritic pain
This is what it’s like to be cured....
The Innovators’ Dilemma: Disruptive Technologies

- Floppy drives ➔ hard drives ➔ flash drives
- Film ➔ digital photography
- Dumb phones ➔ smart phones
- Hemophilia transitions
  - Cryo/FFP to plasma-derived concentrates
  - Plasma-derived to recombinant factors
  - Conventional to Extended Half Life factors
  - EHL to MAbs and siRNA; peaks/troughs gone
- Gene therapies (The C word)
  - Primitive gene therapy to gene editing
- Capitalism

The Power of Gene Editing

... turn chihuahuas into Great Danes with a just a few quick, targeted gene edits.
The Future of Hemophilia Care

- Non-factor replacements with high troughs
- Extended half-life products
- Gene therapy for factor V and B
- Conventional clotting factors
- By-passing agents for inhibitors

Developing World: No change after 100 years

15% of world uses 70% of product
85% of world uses 30% of product
What’s wrong with this?
Prospective Clotting Factor Donations to WFH

<table>
<thead>
<tr>
<th>Supplier</th>
<th>Number of units/year (MIUs)</th>
<th>Years of donation (incl)</th>
</tr>
</thead>
<tbody>
<tr>
<td>Bioverativ-Sobi*</td>
<td>100</td>
<td>2015</td>
</tr>
<tr>
<td>Green Cross</td>
<td>2</td>
<td>2017</td>
</tr>
<tr>
<td>CSL Behring</td>
<td>2-4</td>
<td>2010</td>
</tr>
<tr>
<td>Grifols</td>
<td>20-30</td>
<td>2014</td>
</tr>
<tr>
<td>Project Recovery (Biotest and Canadian Blood Services)</td>
<td>3-4</td>
<td>2013</td>
</tr>
</tbody>
</table>

*Bioverativ was spun off from Biogen in January 2017

2016:
58 countries
14,000 patients treated
Nearly 800 surgeries
458 children on prophylaxis

KENYA: April 2016: clotting factor for the first time
Technology-and Access-
Enable the Impossible

May, 2017

Thank You

ANY QUESTIONS?

glennfpierce@gmail.com