ATHN Strategies 2015-2017

ATHN as Network & Infrastructure
- Expanding capabilities to create partnership opportunities with other entities and, or countries for tools, services and studies.

ATHN as Coordinating Center
- Technical assistance

ATHN as Research Facilitator
- Evolving into a resource for investigator initiated research

Securing Data
Advancing Knowledge
Transforming Care
International Prophylaxis Study Group (IPSG) Survey: An Example

• **Goal:** To identify, in countries where long-term prophylaxis is available, the regimens used in adults with severe hemophilia and in all patients with hemophilia and inhibitors

• **Participants:** Australia, New Zealand, Canada, Europe, US, UK

• **Coordinating Center:** SickKids, Toronto, Canada

• **PI:** Victor Blanchette, MD

• **US Coordinator:** Barbara Konkle, MD
ATHN: Coordinated US Participation and Provided Technical Assistance

ATHN Queries Developed in Clinical Manager

International Electronic Survey
US Participating Sites

40 (33%) of 120 HTCs contacted as of October 20, 2014

- 6 adult only centers
- 1,657 patients
- 1,498 adults (≥18y)
  - 127 with inhibitors
  - 1,371 without inhibitors
IPSG Survey: US Findings

Distribution of adults with severe Hemophilia A and no inhibitors

Total: 1121
On Proph: 651 (58%)

18-30 yo: 603
Prophylaxis: 404 (67%)

31-40 yo: 271
Prophylaxis: 139 (51%)

41-50 yo: 123
Prophylaxis: 60 (49%)

51-70 yo: 115
Prophylaxis: 44 (38%)

>70 yo: 9
Prophylaxis: 4 (44%)
**IPSG Survey: US Findings**

Prophylaxis regimens used in adults with severe Hemophilia A and no inhibitors

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<th>Every 3 days</th>
<th>3/week</th>
<th>2/week</th>
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Total 20 (3%) | 105 (16%) | 78 (12%) | 250 (39%) | 132 (20%) | 20 (3%) | 44 (7%) | 649 (100%)
IPSG Survey: US Findings

Adults with severe Hemophilia B and no inhibitors on prophylaxis

• 121 of 250 (48%) on prophylaxis.

• The percentage of patients on prophylaxis decreased with age, from 53% (18-30y) to 25% (≥ 70y).

• The most common regimens were 2 times a week (47%) and once weekly (26%).
IPSG Survey: US Findings

Children and adolescents with inhibitors

Total 159

On ITI
88

Prophy
26 (30%)

No Prophy
62 (70%)

Not on ITI
58

Prophy
23 (40%)

No Prophy
35 (60%)
IPSG Survey: US Findings

Adults with inhibitors

Total 127

On ITI
19

Prophy
8 (42%)
No Prophy
11 (58%)

Not on ITI
104

Prophy
15 (14%)
No Prophy
89 (86%)
Future Direction

IPSG Survey

- Results to be reviewed by IPSG Study Group in Nov. 2014
- Publication of international results
- US has rights to use its own data

Other Studies

- Serves as model for how the US network can work with ATHN to make it easier to participate in international studies...and perhaps in the future, lead them.