

# ATHN Research Report Brief

ATHNdataset — September 30, 2014



# Acknowledgements

This report would not be possible without the dedicated efforts of the ATHN Affiliated Hemophilia Treatment Centers and their patients.

## Thank you HTCs!

ATHN gratefully acknowledges its Founding Partner Novo Nordisk and its Industry Consortium for its support.

### Industry Visionaries



### Industry Supporters

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**NOTE**

BLACKENED CELLS INDICATE SMALL SIZED CELLS, DEFINED AS 5 CASES OR LESS

This Research Report is prepared using standardized data collected for the ATHNdataset, a HIPAA compliant limited data set under the stewardship of the American Thrombosis and Hemostasis Network (ATHN).

Data collection is a voluntary effort by clinical teams and data managers at federally funded Hemophilia Treatment Centers (HTCs) across the U.S. The project is open to all ATHN Affiliated centers. Patients are asked to opt-in. At the time of reporting, not all patients receiving care at participating centers have been asked to join, and not all have agreed to participate. The ATHNdataset is extracted from the patient's medical record. It is not the official medical record of the patient. Unknown or missing data indicates that the data was not reported to the ATHNdataset during the reporting period.

Participating centers use reasonable efforts to ensure the accuracy of the data. System design with structured data entry, self-audits and HTC re-use of the information in fulfillment of government funded projects further help to drive quality of the data. However, ATHN makes no warranty or representation that the information will be adequate or satisfactory for particular use.

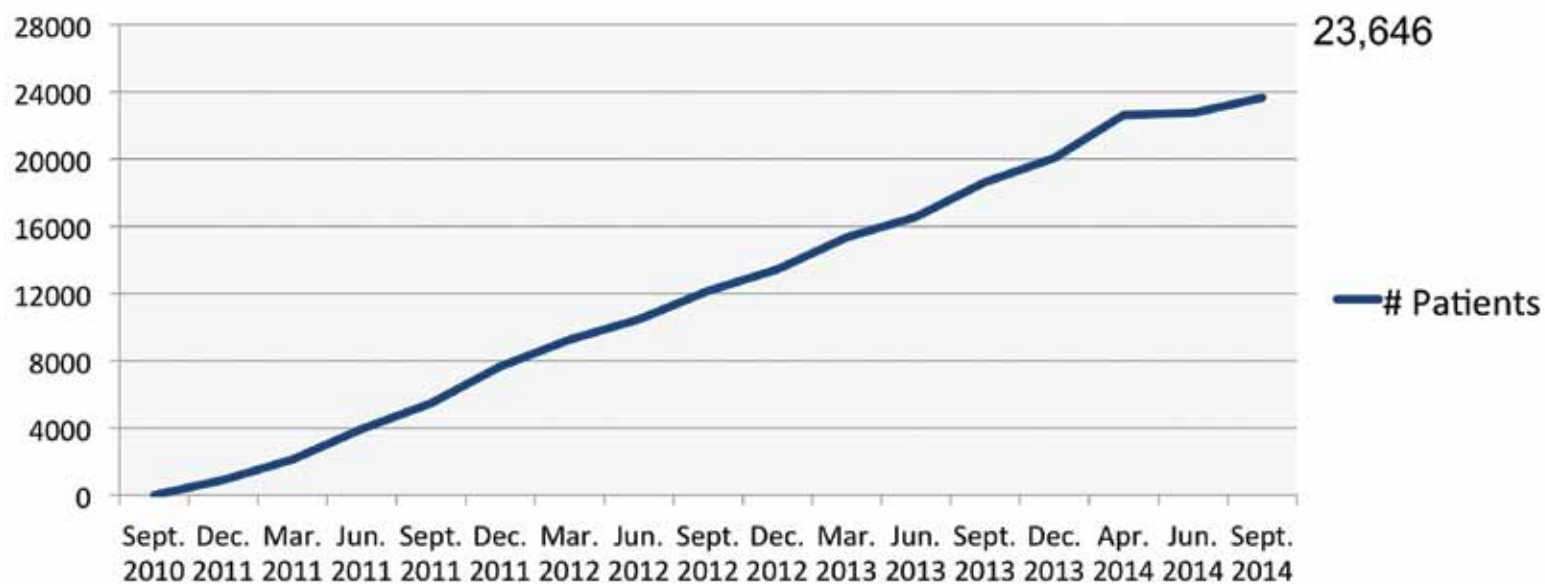
Finally, if you desire to include any extract of the report in any publication of yours, you must obtain prior written consent to such publication and agree to provide appropriate attribution to ATHN and its Affiliate HTCs.

# ATHNdataset

# ATHNdataset — Growth of the ATHNdataset

ATHNdataset PATIENT ACCRUAL OVER TIME (AS OF SEPTEMBER 30, 2014)

|   |
|---|
| The ATHNdataset is a HIPAA compliant limited dataset under the stewardship of ATHN.               |
| Participating Hemophilia Treatment Centers across the U.S. abstract data from the medical record. |
| Patients choose to "opt in" by signing a patient authorization.                                   |
| No special lab tests or clinic visits are required.   |
| Patient identity is protected through the use of a unique system generated identifier.            |
| Since September 2010, the ATHNdataset has grown to include 23,646 patients.                       |



# ATHNdataset — Demographic Profile

ATHNdataset

ATHNdataset COMPARED TO 2010 HEMOPHILIA DATA SET (AS OF SEPTEMBER 30, 2014) BEGINNING IN JANUARY 2014, DATA HAS BEEN COLLECTED USING THE NEW ATHN INFRASTRUCTURE.

The ATHNdataset demographics compare favorably to the aggregate Hemophilia Data Set of active patients under care of Hemophilia Treatment Centers.

The 2010 Hemophilia Data Set included 32,612 active patients with Factor VIII Deficiency, Factor IX Deficiency, Von Willebrand Disease and other factor deficiencies.

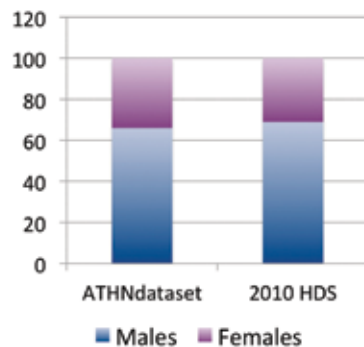
Individual level data are not available through the Hemophilia Data Set.

## GENDER COMPARISON

| ATHNdataset Gender | # Patients    | % Patients  |
|--------------------|---------------|-------------|
| Male               | 15,159        | 64%         |
| Female             | 8,487         | 36%         |
| <b>Total</b>       | <b>23,646</b> | <b>100%</b> |

\* 3 male to female

| 2010 HDS Gender | # Patients    | % Patients  |
|-----------------|---------------|-------------|
| Male            | 22,411        | 69%         |
| Female          | 10,201        | 31%         |
| <b>Total</b>    | <b>32,612</b> | <b>100%</b> |

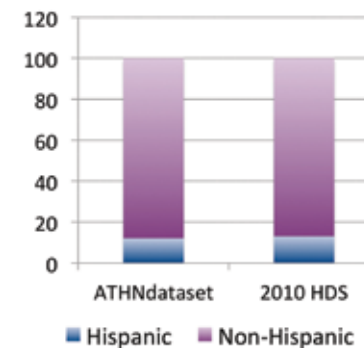


## ETHNICITY COMPARISON

| ATHNdataset Ethnicity | # Patients     | % Patients  |
|-----------------------|----------------|-------------|
| Hispanic              | 2,743          | 12%         |
| Non Hispanic          | 20,558         | 88%         |
| <b>Total</b>          | <b>23,301*</b> | <b>100%</b> |

\* 345 Unknown

| 2010 HDS Ethnicity | # Patients    | % Patients  |
|--------------------|---------------|-------------|
| Hispanic           | 4,289         | 13%         |
| Non-Hispanic       | 28,323        | 87%         |
| <b>Total</b>       | <b>32,612</b> | <b>100%</b> |



# ATHNdataset — Demographic Profile

| ATHNdataset   | AGE AND SEX BY REGION AND TOTAL U.S. (AS OF SEPTEMBER 30, 2014) |              |             |             |                 |              |                 |                |              |
|---------------|---|--------------|-------------|-------------|-----------------|--------------|-----------------|----------------|--------------|
|               | REGION  |              |             |             |                 |              |                 |                | TOTAL        |
|               | New England   | Mid-Atlantic | Southeast   | Great Lakes | Northern States | Great Plains | Mountain States | Western States | US           |
| <b>BY SEX</b> |   |              |             |             |                 |              |                 |                |              |
| Female        | 1100  | 395          | 686         | 4162        | 448             | 647          | 364             | 685            | 8487         |
|               | 35.5%   | 22.9%        | 24.2%       | 49.7%       | 31.9%           | 28.6%        | 23.0%           | 29.0%          | 35.9%        |
| Male          | 1998  | 1330         | 2148        | 4218        | 957             | 1614         | 1220            | 1674           | 15159        |
|               | 64.5%   | 77.1%        | 75.8%       | 50.3%       | 68.1%           | 71.4%        | 77.0%           | 71.0%          | 64.1%        |
| <b>Total</b>  | <b>3098</b>   | <b>1725</b>  | <b>2834</b> | <b>8380</b> | <b>1405</b>     | <b>2261</b>  | <b>1584</b>     | <b>2359</b>    | <b>23646</b> |
|               | 100%  | 100%         | 100%        | 100%        | 100%            | 100%         | 100%            | 100%           | 100%         |
| <b>BY AGE</b> |   |              |             |             |                 |              |                 |                |              |
| 0-2 years     | 83  | 58           | 87          | 248         | 33              | 127          | 50              | 57             | 743          |
|               | 2.7%  | 3.4%         | 3.1%        | 3.0%        | 2.4%            | 5.6%         | 3.2%            | 2.4%           | 3.1%         |
| 3-12 years    | 804   | 384          | 759         | 1678        | 300             | 759          | 384             | 678            | 5746         |
|               | 26.0%   | 22.3%        | 26.8%       | 20.0%       | 21.4%           | 33.6%        | 24.2%           | 28.7%          | 24.3%        |
| 13-18 years   | 654   | 317          | 609         | 1482        | 193             | 525          | 282             | 548            | 4610         |
|               | 21.1%   | 18.4%        | 21.5%       | 17.7%       | 13.7%           | 23.2%        | 17.8%           | 23.2%          | 19.5%        |
| 19-29 years   | 668   | 347          | 600         | 1536        | 319             | 350          | 320             | 533            | 4673         |
|               | 21.6%   | 20.1%        | 21.2%       | 18.3%       | 22.7%           | 15.5%        | 20.2%           | 22.6%          | 19.8%        |
| 30-49 years   | 506   | 341          | 444         | 1585        | 338             | 257          | 328             | 332            | 4131         |
|               | 16.3%   | 19.8%        | 15.7%       | 18.9%       | 24.1%           | 11.4%        | 20.7%           | 14.1%          | 17.5%        |
| 50-74 years   | 337   | 256          | 309         | 1633        | 201             | 223          | 199             | 195            | 3353         |
|               | 10.9%   | 14.8%        | 10.9%       | 19.5%       | 14.3%           | 9.9%         | 12.6%           | 8.3%           | 14.2%        |
| 75+ years     | 46  | 22           | 26          | 218         | 21              | 20           | 21              | 16             | 390          |
|               | 1.5%  | 1.3%         | 0.9%        | 2.6%        | 1.5%            | 0.9%         | 1.3%            | 0.7%           | 1.6%         |
| <b>Total</b>  | <b>3098</b>   | <b>1725</b>  | <b>2834</b> | <b>8380</b> | <b>1405</b>     | <b>2261</b>  | <b>1584</b>     | <b>2359</b>    | <b>23646</b> |
|               | 100%  | 100%         | 100%        | 100%        | 100%            | 100%         | 100%            | 100%           | 100%         |

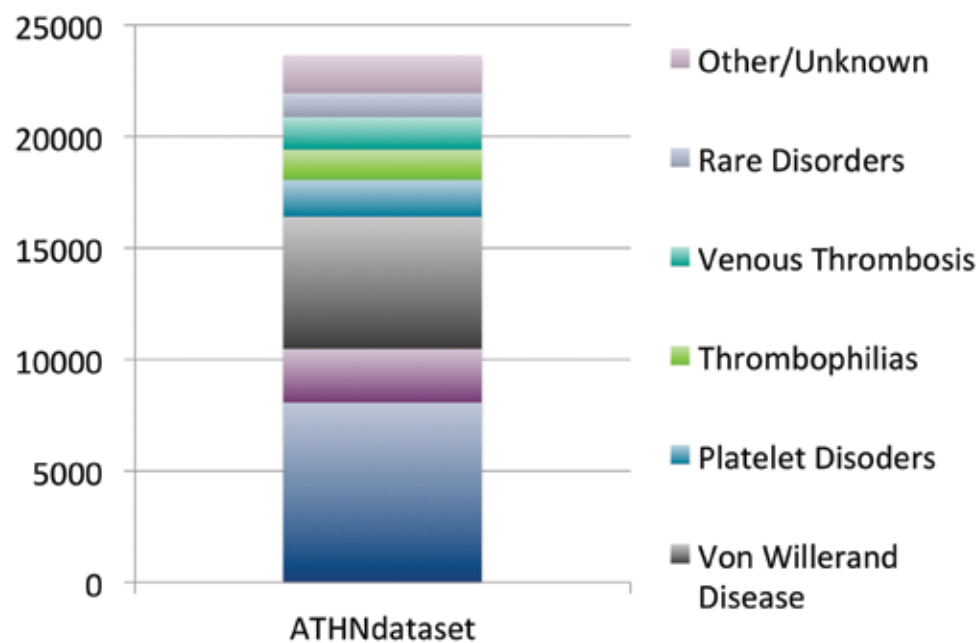
| ATHNdataset Age | Mean | Standard Deviation | Median |
|-----------------|------|--------------------|--------|
|                 | 27   | 20                 | 20     |





# ATHNdataset — Bleeding and Clotting Disorders Within the Population

| ATHNdataset                           | PATIENT AUTHORIZATIONS BY PRIMARY DIAGNOSIS TOTAL U.S. (AS OF SEPTEMBER 30, 2014) |               |
|---------------------------------------|---|---------------|
| PRIMARY BLEEDING OR CLOTTING DISORDER | TOTAL U.S. ATHNdataset CASES  | % ATHNdataset |
| Factor VIII Deficiency                | 8067  | 34.1%         |
| Factor IX Deficiency                  | 2406  | 10.2%         |
| Von Willebrand Disease                | 5917  | 25.0%         |
| Rare Disorders                        | 1067  | 4.5%          |
| Platelet Disorders                    | 1650  | 7.0%          |
| Thrombophilias                        | 1352  | 5.7%          |
| Venous Thrombosis                     | 1479  | 6.3%          |
| Other/Unknown                         | 1708  | 7.2%          |
| <b>TOTAL</b>                          | <b>23646</b>  | <b>100%</b>   |



# Factor VIII Deficiency

## Factor VIII Deficiency — Bleeding Disorder Disease Severity

| FACTOR VIII DEFICIENCY  |             | DISEASE SEVERITY BY REGION AND TOTAL U.S. (AS OF SEPTEMBER 30, 2014) |             |             |                 |              |                 |                |             |  |
|-------------------------|-------------|--|-------------|-------------|-----------------|--------------|-----------------|----------------|-------------|--|
| DISEASE SEVERITY        |             | REGION   |             |             |                 |              |                 |                | TOTAL       |  |
|                         | New England | Mid-Atlantic   | Southeast   | Great Lakes | Northern States | Great Plains | Mountain States | Western States | US          |  |
| <b>Mild</b>             | 278         | 181  | 322         | 365         | 140             | 253          | 235             | 322            | 2096        |  |
|                         | 26.2%       | 22.7%  | 23.5%       | 29.7%       | 26.5%           | 28.1%        | 27.6%           | 29.8%          | 26.8%       |  |
| <b>Moderate</b>         | 180         | 144  | 266         | 199         | 93              | 147          | 156             | 169            | 1354        |  |
|                         | 17.0%       | 18.1%  | 19.4%       | 16.2%       | 17.6%           | 16.3%        | 18.3%           | 15.7%          | 17.3%       |  |
| <b>Severe</b>           | 603         | 473  | 781         | 666         | 296             | 500          | 460             | 589            | 4368        |  |
|                         | 56.8%       | 59.3%  | 57.1%       | 54.2%       | 56.0%           | 55.6%        | 54.1%           | 54.5%          | 55.9%       |  |
| <b>TOTAL</b>            | <b>1061</b> | <b>798</b>   | <b>1369</b> | <b>1230</b> | <b>529</b>      | <b>900</b>   | <b>851</b>      | <b>1080</b>    | <b>7818</b> |  |
|                         | 100%        | 100%   | 100%        | 100%        | 100%            | 100%         | 100%            | 100%           | 100%        |  |
| <b>Unknown Severity</b> | 50          | 44   | 16          | 48          | 18              | 25           | 20              | 28             | 249         |  |
|                         | 4.5%        | 5.2%   | 1.2%        | 3.8%        | 3.3%            | 2.7%         | 2.3%            | 2.5%           | 3.1%        |  |
| <b>TOTAL</b>            | <b>1111</b> | <b>842</b>   | <b>1385</b> | <b>1278</b> | <b>547</b>      | <b>925</b>   | <b>871</b>      | <b>1108</b>    | <b>8067</b> |  |
|                         | 100%        | 100%   | 100%        | 100%        | 100%            | 100%         | 100%            | 100%           | 100%        |  |

## Factor VIII Deficiency — HIV and Hepatitis C

| FACTOR VIII DEFICIENCY |            | HIV AND HEPATITIS C (HCV) BY AGE GROUP (AS OF SEPTEMBER 30, 2014) |             |             |             |             |           |             |
|------------------------|------------|---|-------------|-------------|-------------|-------------|-----------|-------------|
| AGE                    |            |   |             |             |             |             |           | TOTAL       |
| HIV CO-MORBIDITY       |            |   |             |             |             |             |           |             |
|                        | 0-2 years  | 3-12 years  | 13-18 years | 19-29 years | 30-49 years | 50-74 years | 75+ years | US          |
| HIV                    | 0          | 0   | 0           |             | 339         | 192         |           | 538         |
|                        | 0.0%       | 0.0%  | 0.0%        |             | 22.2%       | 21.5%       |           | 6.7%        |
| No HIV                 | 298        | 2113  | 1325        | 1833        | 1185        | 700         | 75        | 7529        |
|                        | 100.0%     | 100.0%  | 100.0%      | 99.7%       | 77.8%       | 78.5%       | 97.4%     | 93.3%       |
| <b>TOTAL</b>           | <b>298</b> | <b>2113</b>   | <b>1325</b> | <b>1838</b> | <b>1524</b> | <b>892</b>  | <b>77</b> | <b>8067</b> |
|                        | 100%       | 100%  | 100%        | 100%        | 100%        | 100%        | 100%      | 100%        |
| HEPATITIS CO-MORBIDITY |            |   |             |             |             |             |           |             |
| Hepatitis C            | 0          | 0   |             | 116         | 833         | 507         | 23        | 1481        |
|                        | 0.0%       | 0.0%  |             | 6.3%        | 54.7%       | 56.8%       | 29.9%     | 18.4%       |
| No Hepatitis C         | 298        | 2113  | 1323        | 1722        | 691         | 385         | 54        | 6586        |
|                        | 100.0%     | 100.0%  | 99.9%       | 93.7%       | 45.3%       | 43.2%       | 70.1%     | 81.6%       |
| <b>TOTAL</b>           | <b>298</b> | <b>2113</b>   | <b>1325</b> | <b>1838</b> | <b>1524</b> | <b>892</b>  | <b>77</b> | <b>8067</b> |
|                        | 100%       | 100%  | 100%        | 100%        | 100%        | 100%        | 100%      | 100%        |

## Factor VIII Deficiency – Continuous Prophylaxis (Prescribed)

| FACTOR VIII DEFICIENCY |                       | PROPHYLAXIS PRESCRIBED FOR PATIENTS BY BLEEDING DISORDER DISEASE SEVERITY AND AGE GROUP<br>(AS OF SEPTEMBER 30, 2014) |             |             |             |             |             |           |             |
|------------------------|-----------------------|---|-------------|-------------|-------------|-------------|-------------|-----------|-------------|
| DISEASE SEVERITY       | PROPHYLAXIS           | AGE   |             |             |             |             |             |           | TOTAL       |
|                        |                       | 0-2 years   | 3-12 years  | 13-18 years | 19-29 years | 30-49 years | 50-74 years | 75+ years | US          |
| Mild                   | Prescribed            |   | 25          | 24          | 22          | 10          | 11          | 0         | 93          |
|                        |                       |   | 5.2%        | 6.7%        | 5.5%        | 2.7%        | 3.0%        | 0.0%      | 4.4%        |
|                        | Not Prescribed/Unk    | 58  | 460         | 334         | 380         | 362         | 359         | 50        | 2003        |
|                        |                       | 98.3%   | 94.9%       | 93.3%       | 94.5%       | 97.3%       | 97.0%       | 100.0%    | 95.6%       |
|                        | <b>Total Mild</b>     | <b>59</b>   | <b>485</b>  | <b>358</b>  | <b>402</b>  | <b>372</b>  | <b>370</b>  | <b>50</b> | <b>2096</b> |
|                        | 100.0%                | 100.0%  | 100.0%      | 100.0%      | 100.0%      | 100.0%      | 100.0%      | 100.0%    |             |
| Moderate               | Prescribed            | 10  | 123         | 87          | 83          | 44          | 14          |           | 362         |
|                        |                       | 18.5%   | 35.2%       | 36.1%       | 27.8%       | 19.3%       | 8.3%        |           | 26.7%       |
|                        | Not Prescribed/Unk    | 44  | 226         | 154         | 216         | 184         | 155         | 13        | 992         |
|                        |                       | 81.5%   | 64.8%       | 63.9%       | 72.2%       | 80.7%       | 91.7%       | 92.9%     | 73.3%       |
|                        | <b>Total Moderate</b> | <b>54</b>   | <b>349</b>  | <b>241</b>  | <b>299</b>  | <b>228</b>  | <b>169</b>  | <b>14</b> | <b>1354</b> |
|                        | 100.0%                | 100.0%  | 100.0%      | 100.0%      | 100.0%      | 100.0%      | 100.0%      | 100.0%    |             |
| Severe                 | Prescribed            | 58  | 849         | 539         | 712         | 395         | 102         |           | 2657        |
|                        |                       | 34.5%   | 69.1%       | 77.4%       | 65.4%       | 45.7%       | 32.6%       |           | 60.8%       |
|                        | Not Prescribed/Unk    | 110   | 380         | 157         | 376         | 470         | 211         | 7         | 1711        |
|                        |                       | 65.5%   | 30.9%       | 22.6%       | 34.6%       | 54.3%       | 67.4%       | 77.8%     | 39.2%       |
|                        | <b>Total Severe</b>   | <b>168</b>  | <b>1229</b> | <b>696</b>  | <b>1088</b> | <b>865</b>  | <b>313</b>  | <b>9</b>  | <b>4368</b> |
|                        | 100.0%                | 100.0%  | 100.0%      | 100.0%      | 100.0%      | 100.0%      | 100.0%      | 100.0%    |             |
| Unknown                | Prescribed            | 2   | 7           | 5           | 8           | 6           | 4           | 0         | 32          |
|                        |                       | 11.8%   | 14.0%       | 16.7%       | 16.3%       | 10.2%       | 10.0%       | 0.0%      | 12.9%       |
|                        | Not Prescribed/Unk    | 15  | 43          | 25          | 41          | 53          | 36          | 4         | 217         |
|                        |                       | 88.2%   | 86.0%       | 83.3%       | 83.7%       | 89.8%       | 90.0%       | 100.0%    | 87.1%       |
|                        | <b>Total Unknown</b>  | <b>17</b>   | <b>50</b>   | <b>30</b>   | <b>49</b>   | <b>59</b>   | <b>40</b>   | <b>4</b>  | <b>249</b>  |
|                        | 100.0%                | 100.0%  | 100.0%      | 100.0%      | 100.0%      | 100.0%      | 100.0%      | 100.0%    |             |
| <b>All Severities</b>  | <b>Total All</b>      | <b>298</b>  | <b>2113</b> | <b>1325</b> | <b>1838</b> | <b>1524</b> | <b>892</b>  | <b>77</b> | <b>8067</b> |
|                        |                       | 100%  | 100%        | 100%        | 100%        | 100%        | 100%        | 100%      | 100%        |

## Factor VIII Deficiency — Payer Profile

| FACTOR VIII DEFICIENCY |             | PAYER PROFILE BY REGION AND TOTAL U.S. (AS OF SEPTEMBER 30, 2014) |             |             |                 |              |                 |                |             |
|------------------------|-------------|---|-------------|-------------|-----------------|--------------|-----------------|----------------|-------------|
| PAYER CATEGORY         | REGION      |   |             |             |                 |              |                 |                | TOTAL       |
|                        | New England | Mid-Atlantic  | Southeast   | Great Lakes | Northern States | Great Plains | Mountain States | Western States | US          |
| Medicaid               | 174         | 118   | 334         | 256         | 92              | 194          | 125             | 139            | 1432        |
|                        | 27.8%       | 31.8%   | 39.6%       | 31.7%       | 25.9%           | 36.3%        | 28.8%           | 20.6%          | 30.8%       |
| Medicare               | 39          | 49  | 70          | 66          | 43              | 26           | 17              | 42             | 352         |
|                        | 6.2%        | 13.2%   | 8.3%        | 8.2%        | 12.1%           | 4.9%         | 3.9%            | 6.2%           | 7.6%        |
| Private                | 396         | 192   | 395         | 381         | 205             | 253          | 280             | 247            | 2349        |
|                        | 63.4%       | 51.8%   | 46.9%       | 47.2%       | 57.8%           | 47.3%        | 64.5%           | 36.5%          | 50.6%       |
| Uninsured              |             |   | 20          |             | 7               | 37           |                 | 29             | 101         |
|                        |             |   | 2.4%        |             | 2.0%            | 6.9%         |                 | 4.3%           | 2.2%        |
| Other                  | 13          | 11  | 24          | 101         | 8               | 25           | 11              | 219            | 412         |
|                        | 2.1%        | 3.0%  | 2.9%        | 12.5%       | 2.3%            | 4.7%         | 2.5%            | 32.4%          | 8.9%        |
| <b>TOTAL</b>           | <b>625</b>  | <b>371</b>  | <b>843</b>  | <b>807</b>  | <b>355</b>      | <b>535</b>   | <b>434</b>      | <b>676</b>     | <b>4646</b> |
|                        | 100%        | 100%  | 100%        | 100%        | 100%            | 100%         | 100%            | 100%           | 100%        |
| Unknown                | 486         | 471   | 542         | 471         | 192             | 390          | 437             | 432            | 3421        |
|                        | 43.7%       | 55.9%   | 39.1%       | 36.9%       | 35.1%           | 42.2%        | 50.2%           | 39.0%          | 42.4%       |
| <b>TOTAL</b>           | <b>1111</b> | <b>842</b>  | <b>1385</b> | <b>1278</b> | <b>547</b>      | <b>925</b>   | <b>871</b>      | <b>1108</b>    | <b>8067</b> |
|                        | 100%        | 100%  | 100%        | 100%        | 100%            | 100%         | 100%            | 100%           | 100%        |





# Factor IX Deficiency

## Factor IX Deficiency — Bleeding Disorder Disease Severity

| FACTOR IX DEFICIENCY |             | DISEASE DISORDER SEVERITY BY REGION AND TOTAL U.S. (AS OF SEPTEMBER 30, 2014) |            |             |                 |              |                 |                |             |
|----------------------|-------------|---|------------|-------------|-----------------|--------------|-----------------|----------------|-------------|
| DISEASE SEVERITY     |             | REGION  |            |             |                 |              |                 |                | TOTAL       |
|                      | New England | Mid-Atlantic  | Southeast  | Great Lakes | Northern States | Great Plains | Mountain States | Western States | US          |
| Mild                 | 69          | 57  | 109        | 181         | 39              | 77           | 66              | 56             | 654         |
|                      | 27.5%       | 26.4%   | 31.8%      | 27.0%       | 22.8%           | 30.6%        | 30.6%           | 26.5%          | 28.1%       |
| Moderate             | 75          | 80  | 108        | 354         | 68              | 111          | 83              | 40             | 919         |
|                      | 29.9%       | 37.0%   | 31.5%      | 52.8%       | 39.8%           | 44.1%        | 38.4%           | 19.0%          | 39.4%       |
| Severe               | 107         | 79  | 126        | 135         | 64              | 64           | 67              | 115            | 757         |
|                      | 42.6%       | 36.6%   | 36.7%      | 20.2%       | 37.4%           | 25.4%        | 31.0%           | 54.5%          | 32.5%       |
| <b>TOTAL</b>         | <b>251</b>  | <b>216</b>  | <b>343</b> | <b>670</b>  | <b>171</b>      | <b>252</b>   | <b>216</b>      | <b>211</b>     | <b>2330</b> |
|                      | 100%        | 100%  | 100%       | 100%        | 100%            | 100%         | 100%            | 100%           | 100%        |
| Unknown Severity     | 12          | 14  | 5          | 24          | 6               | 3            | 4               | 8              | 76          |
|                      | 4.6%        | 6.1%  | 1.4%       | 3.5%        | 3.4%            | 1.2%         | 1.8%            | 3.7%           | 3.2%        |
| <b>TOTAL</b>         | <b>263</b>  | <b>230</b>  | <b>348</b> | <b>694</b>  | <b>177</b>      | <b>255</b>   | <b>220</b>      | <b>219</b>     | <b>2406</b> |
|                      | 100%        | 100%  | 100%       | 100%        | 100%            | 100%         | 100%            | 100%           | 100%        |

## Factor IX Deficiency — HIV and Hepatitis C

| FACTOR IX DEFICIENCY     |           | HIV AND HEPATITIS C (HCV) BY AGE GROUP (AS OF SEPTEMBER 30, 2014) |             |             |             |             |           |             |
|--------------------------|-----------|---|-------------|-------------|-------------|-------------|-----------|-------------|
| AGE                      |           |   |             |             |             |             |           | TOTAL       |
| HIV CO-MORBIDITY         |           |   |             |             |             |             |           |             |
|                          | 0-2 years | 3-12 years  | 13-18 years | 19-29 years | 30-49 years | 50-74 years | 75+ years | US          |
| HIV                      | 0         | 0   | 0           |             | 49          | 25          | 0         | 76          |
|                          | 0.0%      | 0.0%  | 0.0%        |             | 10.1%       | 6.9%        | 0.0%      | 3.2%        |
| No HIV                   | 74        | 598   | 366         | 484         | 438         | 338         | 32        | 2330        |
|                          | 100.0%    | 100.0%  | 100.0%      | 99.6%       | 89.9%       | 93.1%       | 100.0%    | 96.8%       |
| <b>TOTAL</b>             | <b>74</b> | <b>598</b>  | <b>366</b>  | <b>486</b>  | <b>487</b>  | <b>363</b>  | <b>32</b> | <b>2406</b> |
|                          | 100%      | 100%  | 100%        | 100%        | 100%        | 100%        | 100%      | 100%        |
| HEPATITIS C CO-MORBIDITY |           |   |             |             |             |             |           |             |
| Hepatitis C              | 0         |   | 0           | 32          | 216         | 169         | 8         | 426         |
|                          | 0.0%      |   | 0.0%        | 6.6%        | 44.4%       | 46.6%       | 25.0%     | 17.7%       |
| No Hepatitis C           | 74        | 597   | 366         | 454         | 271         | 194         | 24        | 1980        |
|                          | 100.0%    | 99.8%   | 100.0%      | 93.4%       | 55.7%       | 53.4%       | 75.0%     | 82.3%       |
| <b>TOTAL</b>             | <b>74</b> | <b>598</b>  | <b>366</b>  | <b>486</b>  | <b>487</b>  | <b>363</b>  | <b>32</b> | <b>2406</b> |
|                          | 100%      | 100%  | 100%        | 100%        | 100%        | 100%        | 100%      | 100%        |

## Factor IX Deficiency – Continuous Prophylaxis (Prescribed)

| FACTOR IX DEFICIENCY |                       | PROPHYLAXIS PRESCRIBED FOR PATIENTS BY BLEEDING DISORDER DISEASE SEVERITY AND AGE GROUP<br>(AS OF SEPTEMBER 30, 2014) |            |             |             |             |             |           |             |
|----------------------|-----------------------|---|------------|-------------|-------------|-------------|-------------|-----------|-------------|
| DISEASE SEVERITY     | PROPHYLAXIS           | AGE   |            |             |             |             |             |           | TOTAL       |
|                      |                       | 0-2 years   | 3-12 years | 13-18 years | 19-29 years | 30-49 years | 50-74 years | 75+ years | US          |
| Mild                 | Prescribed            |   | 8          |             |             |             |             |           | 29          |
|                      |                       |   | 5.0%       |             |             |             |             |           | 4.4%        |
|                      | Not Prescribed/Unk    | 9   | 151        | 101         | 128         | 105         | 121         | 10        | 625         |
|                      |                       | 90.0%   | 95.0%      | 93.5%       | 96.2%       | 97.2%       | 96.8%       | 90.9%     | 95.6%       |
|                      | <b>Total Mild</b>     | <b>10</b>   | <b>159</b> | <b>108</b>  | <b>133</b>  | <b>108</b>  | <b>125</b>  | <b>11</b> | <b>654</b>  |
|                      |                       | 100.0%  | 100.0%     | 100.0%      | 100.0%      | 100.0%      | 100.0%      | 100.0%    | 100.0%      |
| Moderate             | Prescribed            |   | 28         | 30          | 21          | 15          | 8           | 0         | 104         |
|                      |                       |   | 12.0%      | 20.1%       | 13.1%       | 8.1%        | 5.7%        | 0.0%      | 11.3%       |
|                      | Not Prescribed/Unk    | 35  | 205        | 119         | 139         | 171         | 132         | 14        | 815         |
|                      |                       | 94.6%   | 88.0%      | 79.9%       | 86.9%       | 91.9%       | 94.3%       | 100.0%    | 88.7%       |
|                      | <b>Total Moderate</b> | <b>37</b>   | <b>233</b> | <b>149</b>  | <b>160</b>  | <b>186</b>  | <b>140</b>  | <b>14</b> | <b>919</b>  |
|                      |                       | 100.0%  | 100.0%     | 100.0%      | 100.0%      | 100.0%      | 100.0%      | 100.0%    | 100.0%      |
| Severe               | Prescribed            | 8   | 136        | 71          | 98          | 60          | 29          |           | 403         |
|                      |                       | 30.8%   | 70.8%      | 70.3%       | 54.1%       | 35.3%       | 35.4%       |           | 53.2%       |
|                      | Not Prescribed/Unk    | 18  | 56         | 30          | 83          | 110         | 53          |           | 354         |
|                      |                       | 69.2%   | 29.2%      | 29.7%       | 45.9%       | 64.7%       | 64.6%       |           | 46.8%       |
|                      | <b>Total Severe</b>   | <b>26</b>   | <b>192</b> | <b>101</b>  | <b>181</b>  | <b>170</b>  | <b>82</b>   | <b>5</b>  | <b>757</b>  |
|                      |                       | 100.0%  | 100.0%     | 100.0%      | 100.0%      | 100.0%      | 100.0%      | 100.0%    | 100.0%      |
| Unknown              | Prescribed            | 0   | 0          | 2           | 2           | 0           | 1           | 0         | 5           |
|                      |                       | 0.0%  | 0.0%       | 25.0%       | 16.7%       | 0.0%        | 6.3%        | 0.0%      | 6.6%        |
|                      | Not Prescribed/Unk    | 1   | 14         | 6           | 10          | 23          | 15          | 2         | 71          |
|                      |                       | 100.0%  | 100.0%     | 75.0%       | 83.3%       | 100.0%      | 93.8%       | 100.0%    | 93.4%       |
|                      | <b>Total Unknown</b>  | <b>1</b>  | <b>14</b>  | <b>8</b>    | <b>12</b>   | <b>23</b>   | <b>16</b>   | <b>2</b>  | <b>76</b>   |
|                      |                       | 100.0%  | 100.0%     | 100.0%      | 100.0%      | 100.0%      | 100.0%      | 100.0%    | 100.0%      |
| All Severities       | <b>Total All</b>      | <b>74</b>   | <b>598</b> | <b>366</b>  | <b>486</b>  | <b>487</b>  | <b>363</b>  | <b>32</b> | <b>2406</b> |
|                      |                       | 100%  | 100%       | 100%        | 100%        | 100%        | 100%        | 100%      | 100%        |

## Factor IX Deficiency — Payer Profile

| FACTOR IX DEFICIENCY |             | 3.7 PAYER PROFILE BY REGION AND TOTAL U.S. (AS OF SEPTEMBER 30, 2014) |            |             |                 |              |                 |                |             |  |
|----------------------|-------------|---|------------|-------------|-----------------|--------------|-----------------|----------------|-------------|--|
| PAYER CATEGORY       |             | REGION  |            |             |                 |              |                 |                | TOTAL       |  |
|                      | New England | Mid-Atlantic  | Southeast  | Great Lakes | Northern States | Great Plains | Mountain States | Western States | US          |  |
| Medicaid             | 45          | 23  | 51         | 96          | 27              | 41           | 20              | 18             | 321         |  |
|                      | 32.1%       | 27.7%   | 30.0%      | 29.0%       | 22.7%           | 33.1%        | 19.6%           | 14.1%          | 26.8%       |  |
| Medicare             | 16          | 7   | 16         | 27          | 16              | 6            | 8               | 15             | 111         |  |
|                      | 11.4%       | 8.4%  | 9.4%       | 8.2%        | 13.5%           | 4.8%         | 7.8%            | 11.7%          | 9.3%        |  |
| Private              | 77          | 48  | 92         | 140         | 68              | 72           | 71              | 50             | 618         |  |
|                      | 55.0%       | 57.8%   | 54.1%      | 42.3%       | 57.1%           | 58.1%        | 69.6%           | 39.1%          | 51.6%       |  |
| Uninsured            | 0           |   |            | 18          |                 |              |                 | 9              | 37          |  |
|                      | 0.0%        |   |            | 5.4%        |                 |              |                 | 7.0%           | 3.1%        |  |
| Other                |             |   | 8          | 50          | 7               |              |                 | 36             | 110         |  |
|                      |             |   | 4.7%       | 15.1%       | 5.9%            |              |                 | 28.1%          | 9.2%        |  |
| <b>TOTAL</b>         | <b>140</b>  | <b>83</b>   | <b>170</b> | <b>331</b>  | <b>119</b>      | <b>124</b>   | <b>102</b>      | <b>128</b>     | <b>1197</b> |  |
|                      | 100%        | 100%  | 100%       | 100%        | 100%            | 100%         | 100%            | 100%           | 100%        |  |
| Unknown              | 123         | 147   | 178        | 363         | 58              | 131          | 118             | 91             | 1209        |  |
|                      | 46.8%       | 63.9%   | 51.2%      | 52.3%       | 32.8%           | 51.4%        | 53.6%           | 41.6%          | 50.2%       |  |
| <b>TOTAL</b>         | <b>263</b>  | <b>230</b>  | <b>348</b> | <b>694</b>  | <b>177</b>      | <b>255</b>   | <b>220</b>      | <b>219</b>     | <b>2406</b> |  |
|                      | 100%        | 100%  | 100%       | 100%        | 100%            | 100%         | 100%            | 100%           | 100%        |  |



# Von Willebrand Disease

# Von Willebrand Disease — Demographic Profile

| VON WILLEBRAND DISEASE |             | AGE AND SEX BY REGION AND TOTAL U.S. (AS OF SEPTEMBER 30, 2014) |            |             |                 |              |                 |                |             |
|------------------------|-------------|---|------------|-------------|-----------------|--------------|-----------------|----------------|-------------|
|                        |             | REGION  |            |             |                 |              |                 |                | TOTAL       |
|                        | New England | Mid-Atlantic  | Southeast  | Great Lakes | Northern States | Great Plains | Mountain States | Western States | US          |
| <b>BY SEX</b>          |             |   |            |             |                 |              |                 |                |             |
| Female                 | 659         | 271   | 455        | 1241        | 250             | 280          | 166             | 388            | 3710        |
|                        | 64.3%       | 60.4%   | 61.0%      | 65.4%       | 61.0%           | 58.3%        | 66.1%           | 59.1%          | 62.7%       |
| Male                   | 366         | 178   | 291        | 658         | 160             | 200          | 85              | 269            | 2207        |
|                        | 35.7%       | 39.6%   | 39.0%      | 34.7%       | 39.0%           | 41.7%        | 33.9%           | 40.9%          | 37.3%       |
| <b>Total</b>           | <b>1025</b> | <b>449</b>  | <b>746</b> | <b>1899</b> | <b>410</b>      | <b>480</b>   | <b>251</b>      | <b>657</b>     | <b>5917</b> |
|                        | 100%        | 100%  | 100%       | 100%        | 100%            | 100%         | 100%            | 100%           | 100%        |
| <b>BY AGE</b>          |             |   |            |             |                 |              |                 |                |             |
| 0-2 years              | 10          |   | 6          | 19          |                 | 9            |                 |                | 59          |
|                        | 1.0%        |   | 0.8%       | 1.0%        |                 | 1.9%         |                 |                | 1.0%        |
| 3-12 years             | 318         | 114   | 201        | 465         | 112             | 159          | 64              | 212            | 1645        |
|                        | 31.0%       | 25.4%   | 26.9%      | 24.5%       | 27.3%           | 33.1%        | 25.5%           | 32.3%          | 27.8%       |
| 13-18 years            | 315         | 110   | 203        | 446         | 76              | 161          | 59              | 215            | 1585        |
|                        | 30.7%       | 24.5%   | 27.2%      | 23.5%       | 18.5%           | 33.5%        | 23.5%           | 32.7%          | 26.8%       |
| 19-29 years            | 224         | 104   | 153        | 413         | 91              | 77           | 44              | 133            | 1239        |
|                        | 21.9%       | 23.2%   | 20.5%      | 21.8%       | 22.2%           | 16.0%        | 17.5%           | 20.2%          | 20.9%       |
| 30-49 years            | 86          | 62  | 109        | 308         | 78              | 38           | 37              | 54             | 772         |
|                        | 8.4%        | 13.8%   | 14.6%      | 16.2%       | 19.0%           | 7.9%         | 14.7%           | 8.2%           | 13.0%       |
| 50-74 years            | 66          | 49  | 64         | 222         | 44              | 36           | 39              | 36             | 556         |
|                        | 6.4%        | 10.9%   | 8.6%       | 11.7%       | 10.7%           | 7.5%         | 15.5%           | 5.5%           | 9.4%        |
| 75+ years              | 6           | 6   | 10         | 26          | 6               | 0            |                 |                | 61          |
|                        | 0.6%        | 1.3%  | 1.3%       | 1.4%        | 1.5%            | 0.0%         |                 |                | 1.0%        |
| <b>Total</b>           | <b>1025</b> | <b>449</b>  | <b>746</b> | <b>1899</b> | <b>410</b>      | <b>480</b>   | <b>251</b>      | <b>657</b>     | <b>5917</b> |
|                        | 100%        | 100%  | 100%       | 100%        | 100%            | 100%         | 100%            | 100%           | 100%        |

| Age (years) | Mean | Standard Deviation | Median |
|-------------|------|--------------------|--------|
|             | 23   | 17                 | 18     |





## Von Willebrand Disease — Disease Types

| VON WILLEBRAND DISEASE        |             | DISEASE TYPES BY REGION AND TOTAL U.S. (AS OF SEPTEMBER 30, 2014) |            |             |                 |              |                 |                |             |
|-------------------------------|-------------|---|------------|-------------|-----------------|--------------|-----------------|----------------|-------------|
| VWD TYPE                      | REGION      |   |            |             |                 |              |                 |                | TOTAL       |
|                               | New England | Mid-Atlantic  | Southeast  | Great Lakes | Northern States | Great Plains | Mountain States | Western States | US          |
| VWD, Type 1                   | 890         | 369   | 541        | 1586        | 312             | 358          | 164             | 550            | 4770        |
|                               | 88.6%       | 85.4%   | 76.0%      | 85.4%       | 78.0%           | 78.7%        | 71.3%           | 85.8%          | 83.2%       |
| VWD, Type 2                   | 86          | 43  | 131        | 226         | 67              | 78           | 52              | 69             | 752         |
|                               | 8.6%        | 10.0%   | 18.4%      | 12.2%       | 16.8%           | 17.1%        | 22.6%           | 10.8%          | 13.1%       |
| VWD, Type 3                   | 29          | 20  | 40         | 46          | 21              | 19           | 14              | 22             | 211         |
|                               | 2.9%        | 4.6%  | 5.6%       | 2.5%        | 5.3%            | 4.2%         | 6.1%            | 3.4%           | 3.7%        |
| <b>TOTAL w Type Specified</b> | <b>1005</b> | <b>432</b>  | <b>712</b> | <b>1858</b> | <b>400</b>      | <b>455</b>   | <b>230</b>      | <b>641</b>     | <b>5733</b> |
|                               | 100%        | 100%  | 100%       | 100%        | 100%            | 100%         | 100%            | 100%           | 100%        |
| VWD, Type Other/Unknown       | 20          | 17  | 34         | 41          | 10              | 25           | 21              | 16             | 184         |
|                               | 2.0%        | 3.8%  | 4.6%       | 2.2%        | 2.4%            | 5.2%         | 8.4%            | 2.4%           | 3.1%        |
| <b>TOTAL</b>                  | <b>1025</b> | <b>449</b>  | <b>746</b> | <b>1899</b> | <b>410</b>      | <b>480</b>   | <b>251</b>      | <b>657</b>     | <b>5917</b> |
|                               | 100%        | 100%  | 100%       | 100%        | 100%            | 100%         | 100%            | 100%           | 100%        |

# Von Willebrand Disease — HIV and Hepatitis C

| VON WILLEBRAND DISEASE   |           | HIV AND HEPATITIS C (HCV) BY AGE GROUP (AS OF SEPTEMBER 30, 2014) |             |             |             |             |           |             |
|--------------------------|-----------|---|-------------|-------------|-------------|-------------|-----------|-------------|
| AGE                      |           |   |             |             |             |             |           | TOTAL       |
| HIV CO-MORBIDITY         |           |   |             |             |             |             |           |             |
|                          | 0-2 years | 3-12 years  | 13-18 years | 19-29 years | 30-49 years | 50-74 years | 75+ years | US          |
| HIV                      | 0         | 0   | 0           |             |             |             | 0         | 8           |
|                          | 0.0%      | 0.0%  | 0.0%        |             |             |             | 0.0%      | 0.1%        |
| No HIV                   | 59        | 1645  | 1585        | 1238        | 767         | 554         | 61        | 5909        |
|                          | 100.0%    | 100.0%  | 100.0%      | 99.9%       | 99.4%       | 99.6%       | 100.0%    | 99.9%       |
| <b>TOTAL</b>             | <b>59</b> | <b>1645</b>   | <b>1585</b> | <b>1239</b> | <b>772</b>  | <b>556</b>  | <b>61</b> | <b>5917</b> |
|                          | 100%      | 100%  | 100%        | 100%        | 100%        | 100%        | 100%      | 100%        |
| HEPATITIS C CO-MORBIDITY |           |   |             |             |             |             |           |             |
| Hepatitis C              | 0         | 0   | 0           |             | 36          | 60          | 6         | 106         |
|                          | 0.0%      | 0.0%  | 0.0%        |             | 4.7%        | 10.8%       | 9.8%      | 1.8%        |
| No Hepatitis C           | 59        | 1645  | 1585        | 1235        | 736         | 496         | 55        | 5811        |
|                          | 100.0%    | 100.0%  | 100.0%      | 99.7%       | 95.3%       | 89.2%       | 90.2%     | 98.2%       |
| <b>TOTAL</b>             | <b>59</b> | <b>1645</b>   | <b>1585</b> | <b>1239</b> | <b>772</b>  | <b>556</b>  | <b>61</b> | <b>5917</b> |
|                          | 100%      | 100%  | 100%        | 100%        | 100%        | 100%        | 100%      | 100%        |



# Definitions

# Definitions – Regions

| DEFINITION OF REGIONS  |                                      |
|------------------------|--------------------------------------|
| New England Region     | Regions I and II                     |
| Mid-Atlantic Region    | Region III                           |
| Southeast Region       | Region IV-N and IV-S                 |
| Great Lakes Region     | Region V-E                           |
| Northern States Region | Region V-W                           |
| Great Plains Region    | Region VI and VII                    |
| Mountain States Region | Regions VIII and X                   |
| Western States Region  | Region IX                            |
| Region I               | State equals: MA, CT, ME, VT, NH, RI |
| Region II              | State equals: NY, NJ, PR             |
| Region III             | State equals: PA, DC, VA, DE, WV, MD |
| Region IV-N            | State equals: NC, SC, KY, TN         |
| Region IV-S            | State equals: GA, FL, AL, MS         |
| Region V-E             | State equals: MI, OH, IN             |
| Region V-W             | State equals: WI, IL, MN, ND, SD     |
| Region VI              | State equals: TX, LA, AR, OK         |
| Region VII             | State equals: MO, IA, NE, KS         |
| Region VIII            | State equals: CO, NM, AZ, UT, MT, WY |
| Region IX              | State equals: CA, HI, GU, NV         |
| Region X               | State equals: OR, WA, AK, ID         |

## Definitions – Clinical Content

| DEFINITION OF CLINICAL CONTENT       |  |
|--------------------------------------|--|
| Factor VIII Deficiency               | Hemophilia A   |
| Factor IX Deficiency                 | Hemophilia B, also known as Christmas disease  |
| Factor VIII/IX Disease Severity      |  |
| Factor VIII/IX Deficiency (Mild)     | Condition resulting from a level of clotting activity of >5% of normal activity in the bloodstream (Normal range of factor VIII or IX is 50-200%)  |
| Factor VIII/IX Deficiency (Moderate) | Condition resulting from a level of clotting activity of 1-5% of normal activity in the bloodstream (Normal range of factor VIII or IX is 50-200%)   |
| Factor VIII/IX Deficiency (Severe)   | Condition resulting from a level of clotting activity of <1% of normal activity in the bloodstream (Normal range of factor VIII or IX is 50-200%)  |
| Von Willebrand Disease (VWD)         |  |
| VWD, Type 1                          | Includes subtype 1C  |
| VWD, Type 2                          | Includes subtypes 2, 2A, 2B, 2M, 2N, type 2 unknown subtype  |
| VWD, Type 3                          | No subtypes  |
| VWD, Type Other/Unknown              | Includes acquired VWD  |
| Rare Disorders                       | Includes disorders resulting from deficiency of Factor I (fibrinogen), Factor II, Factor V (not Factor V Leiden), Factor V and VIII, Factor X, Factor XI, Factor XII, Factor XIII, PAI-1, Protein S, Protein C as well as Ehlers-Danlos syndrome and Hereditary Hemorrhagic Telangiectasia (Osler-Weber-Rendu) |
| Platelet Disorders                   | Includes Bernard Soulier; Glanzmann Thrombasthenia; Grey Platelet Syndrome; Hermansky-Pudlak Syndrome; Platelet Function Disorder, Hereditary; Platelet Function Disorder, Acquired; Release Defect; as well as Storage Pool Deficiency  |

# Definitions – Medications

| DEFINITION OF MEDICATIONS  |  |
|--|--|
| F-VIII Replacement: Recombinant Factor VIII Concentrates   | Advate (Baxter), Helixate FS (CSL), Kogenate FS (Bayer), Recombinate (Baxter), ReFacto AF (Pfizer), Xyntha (Pfizer), Eloctate (Biogen)   |
| F-VIII Replacement: Human Plasma Derived Factor VIII Concentrates (*rich in Von Willebrand factor) | Alphanate (Grifols)*, Humate-P (CSL)*, Koate DVI (Kedrion)*, Hemophil (Baxter), Monoclate-P (CSL), Monarc-M (Baxter)   |
| F-IX Replacement: Recombinant Factor IX Concentrates   | BeneFIX (Pfizer) - non-VWD factor containing products, Alprolix (Biogen), Rixubis (Baxter)   |
| F-IX Replacement: Human Plasma Derived Factor IX Concentrates                                      | AlphaNine SD (Grifols), MonoNine (CSL)   |
| VWD Treatments: VWF-Containing Factor VIII Concentrates (Human Plasma Derived)                     | Alphanate (Grifols), Humate-P (CSL), Wilate (Octapharma)   |
| F-XIII Replacement   | Corifact (CSL), Tretten (NovoNordisk)  |
| Bypassing Agents   | NovoSeven RT (Novo Nordisk), FEIBA (Baxter), Autoplex T  |
| Immune Tolerance Induction (ITI)   | Immune modulating drugs may include, but are not limited, to the following: corticosteroids, cyclophosphamide (Cytoxan®), mycophenolate mofetil (CellCept®), mycophenolic acid (Myfortic®), azathioprine (Imuran®, Azasan®), tacrolimus (Prograf®, Hecoria®), sirolimus (Rapamune®), cyclosporine (Sandimmune®), IVIg (Carimune®, Flebogamma®, Gammagard®, Gammaplex®, Hizentra®, Privigen®, Vivaglobin®), and rituximab (Rituxan®). |
| Blood Bank Products  | Cryoprecipitate, Fresh-frozen plasma, Platelets, Packed RBCs or whole blood  |
| Hepatitis C treatment regimens   | Interferon, PEG-interferon, EPO, ribavirin, boceprevir, telaprevir   |
| Desmopressin Formulations  | DDAVP injection, Stimate spray   |



# Definitions — Patient Sample

| DEFINITION OF PATIENT SAMPLE |   |
|------------------------------|---|
| ATHNdataset Population       | The population includes only those individuals who receive care at one of the participating HTCs.<br>Individuals actively "opt in" to the ATHNdataset.<br>Each participant has signed a patient authorization form indicating willingness to share data as part of the ATHNdataset. |
| Small Cell Size              | Cell with 5 or fewer cases  |

## Definitions — Participation HTC by Region

| LIST OF PARTICIPATING HTCS BY REGION |   |               |       |
|--------------------------------------|---|---------------|-------|
| Region                               | ATHN Affiliate  | City          | State |
| I                                    | Boston Hemophilia Center at Boston Children's Hospital                              | Boston        | MA    |
| I                                    | Boston Hemophilia Center- Brigham and Women's Hospital                              | Boston        | MA    |
| I                                    | Dartmouth-Hitchcock Comprehensive Hemophilia and Thrombosis Center                  | Lebanon       | NH    |
| I                                    | Maine Hemophilia and Thrombosis Center  | Scarborough   | ME    |
| I                                    | Massachusetts General Hospital for Children   | Boston        | MA    |
| I                                    | New England Hemophilia Center/UMassMemorial Hospital                                | Worcester     | MA    |
| I                                    | Rhode Island Hospital Hemostasis and Thrombosis Center                              | Providence    | RI    |
| I                                    | University of Connecticut Health Center & Connecticut Children's Medical Center     | Hartford      | CT    |
| I                                    | Vermont Regional Hemophilia Center  | Burlington    | VT    |
| I                                    | Yale Hemophilia Center  | New Haven     | CT    |
| II                                   | Albany Regional Hemophilia & von Willebrand Treatment Center                        | Albany        | NY    |
| II                                   | Hemophilia Center of Western New York, Inc.   | Buffalo       | NY    |
| II                                   | Long Island Jewish Medical Center Comprehensive Hemophilia Center                   | New Hyde Park | NY    |
| II                                   | Mary M. Gooley Hemophilia Center, Inc.  | Rochester     | NY    |
| II                                   | Mount Sinai Regional Comprehensive Hemophilia Treatment Center                      | New York      | NY    |
| II                                   | Nadeene Brunini Comprehensive Hemophilia Care Center / St. Michael's Medical Center | Newark        | NJ    |
| II                                   | Newark Beth Israel Medical Center - Hemophilia Center                               | Newark        | NJ    |
| II                                   | Puerto Rico Hemophilia Treatment Center   | San Juan      | PR    |
| II                                   | SUNY Upstate Medical University-Adult Program                                       | Syracuse      | NY    |
| II                                   | SUNY Upstate Medical University-Pediatric Program                                   | Syracuse      | NY    |
| II                                   | UMDNJ-Robert Wood Johnson University Hospital                                       | New Brunswick | NJ    |
| II                                   | Weill Cornell Medical College - New York Presbyterian Hospital                      | New York      | NY    |

## Definitions — Participation HTC by Region

| LIST OF PARTICIPATING HTCS BY REGION |  |                 |       |
|--------------------------------------|--|-----------------|-------|
| Region                               | ATHN Affiliate   | City            | State |
| III                                  | Central Virginia Center for Coagulation Disorders / Virginia Commonwealth University               | Richmond        | VA    |
| III                                  | Charleston Area Medical Center   | Charleston      | WV    |
| III                                  | Children's Hospital of Philadelphia (CHOP)   | Philadelphia    | PA    |
| III                                  | Children's Hospital of the King's Daughters  | Norfolk         | VA    |
| III                                  | Children's National Hemophilia Center  | Washington      | DC    |
| III                                  | Georgetown University  | Washington      | DC    |
| III                                  | Hemophilia Center of Central Pennsylvania / Penn State Milton S. Hershey Medical Center            | Hershey         | PA    |
| III                                  | Hemophilia Center of Western Pennsylvania  | Pittsburgh      | PA    |
| III                                  | Johns Hopkins University Hemophilia Treatment Center   | Baltimore       | MD    |
| III                                  | Lehigh Valley Hospital   | Bethlehem       | PA    |
| III                                  | Pediatric Hematology Program University of Virginia  | Charlottesville | VA    |
| III                                  | Penn Comprehensive Hemophilia and Thrombophilia Program/Hospital of the University of Pennsylvania | Philadelphia    | PA    |
| III                                  | West Virginia University Medical Center Hemophilia Treatment Center                                | Morgantown      | WV    |
| IV - North                           | Brody School of Medicine at East Carolina University   | Greenville      | NC    |
| IV - North                           | Comprehensive Hemophilia Treatment Center, University of North Carolina at Chapel Hill             | Chapel Hill     | NC    |
| IV - North                           | Duke University Health System  | Durham          | NC    |
| IV - North                           | East Tennessee Comprehensive Hemophilia Center   | Knoxville       | TN    |
| IV - North                           | Palmetto Health Richland   | Columbia        | SC    |
| IV - North                           | St. Jude Children's Research Hospital  | Memphis         | TN    |
| IV - North                           | University of Kentucky Hemophilia Treatment Center   | Lexington       | KY    |
| IV - North                           | University of Louisville Hemophilia Treatment Center   | Louisville      | KY    |
| IV - North                           | Vanderbilt University Medical Center   | Nashville       | TN    |

## Definitions — Participation HTCs by Region

| LIST OF PARTICIPATING HTCS BY REGION |   |                |       |
|--------------------------------------|---|----------------|-------|
| Region                               | ATHN Affiliate  | City           | State |
| IV - North                           | Wake Forest University Health Sciences  | Winston-Salem  | NC    |
| IV - South                           | All Children's Hospital   | St. Petersburg | FL    |
| IV - South                           | Children's Hospital @ Memorial University Medical Center  | Savannah       | GA    |
| IV - South                           | Comprehensive Bleeding Disorders Center at Emory University and Children's Health Care of Atlanta | Atlanta        | GA    |
| IV - South                           | Georgia Regents University - Adult Hemophilia Treatment Center                                    | Augusta        | GA    |
| IV - South                           | Georgia Regents University Pediatric Hemophilia Treatment Center                                  | Augusta        | GA    |
| IV - South                           | Nemours Children's Clinic of Jacksonville / The Nemours Foundation                                | Jacksonville   | FL    |
| IV - South                           | Pediatric Hematology/Oncology of St. Joseph's Children's Hospital - Tampa                         | Tampa          | FL    |
| IV - South                           | University of Alabama Birmingham  | Birmingham     | AL    |
| IV - South                           | University of Miami Comprehensive Hemophilia Treatment Center                                     | Miami          | FL    |
| IV - South                           | University of Mississippi Medical Center  | Jackson        | MS    |
| V - East                             | Akron Children's Hospital HTC   | Akron          | OH    |
| V - East                             | Children's Hospital of Michigan Hemostasis and Thrombosis Center                                  | Detroit        | MI    |
| V - East                             | Cincinnati Children's Hospital Medical Center, Hemophilia & Thrombosis Center                     | Cincinnati     | OH    |
| V - East                             | DeVos Children's Coagulation Disorders Program  | Grand Rapids   | MI    |
| V - East                             | Eastern Michigan Hemophilia Treatment Center Hurley Medical Center                                | Flint          | MI    |
| V - East                             | Hemophilia Clinic - West Michigan Cancer Center   | Kalamazoo      | MI    |
| V - East                             | Henry Ford Health System Bleeding and Thrombosis Treatment Center                                 | Detroit        | MI    |
| V - East                             | Indiana Hemophilia and Thrombosis Center  | Indianapolis   | IN    |
| V - East                             | Karmanos Cancer Center/Detroit Receiving Hospital and University Medical Center                   | Detroit        | MI    |
| V - East                             | Michigan State University Center for Bleeding Disorders & Clotting                                | East Lansing   | MI    |
| V - East                             | Nationwide Children's Hospital Columbus   | Columbus       | OH    |

## Definitions — Participation HTC by Region

| LIST OF PARTICIPATING HTCS BY REGION |   |               |       |
|--------------------------------------|---|---------------|-------|
| Region                               | ATHN Affiliate  | City          | State |
| V - East                             | Northern Regional Bleeding Disorder Center at Munson Medical Center | Traverse City | MI    |
| V - East                             | Northwest Ohio Hemophilia Treatment Center at the Toledo Hospital   | Toledo        | OH    |
| V - East                             | Ohio State University Medical Center Hemophilia Treatment Center    | Columbus      | OH    |
| V - East                             | University Hospitals Health System Cleveland                        | Cleveland     | OH    |
| V - East                             | University of Cincinnati Medical Center Hemophilia Treatment Center | Cincinnati    | OH    |
| V - East                             | University of Michigan Hemophilia and Coagulation Disorders         | Ann Arbor     | MI    |
| V - East                             | West Central Ohio Hemophilia Center - Dayton Children's Hospital    | Dayton        | OH    |
| V - East                             | West Michigan Pediatric at Bronson                                  | Kalamazoo     | MI    |
| V - West                             | Ann & Robert H. Lurie Children's Hospital of Chicago                | Chicago       | IL    |
| V - West                             | Bleeding and Clotting Disorders Institute                           | Peoria        | IL    |
| V - West                             | Children's Hospitals & Clinics of Minnesota                         | Minneapolis   | MN    |
| V - West                             | Comprehensive Center for Bleeding Disorders, Milwaukee              | Wauwatosa     | WI    |
| V - West                             | Gundersen Lutheran Administrative Services, Inc.                    | LaCrosse      | WI    |
| V - West                             | Hemophilia Outreach Center Green Bay                                | Green Bay     | WI    |
| V - West                             | North Dakota Hemophilia & Thrombophilia Treatment Center            | Fargo         | ND    |
| V - West                             | Northwestern Center for Bleeding Disorders                          | Chicago       | IL    |
| V - West                             | Rush University Medical Center                                      | Chicago       | IL    |
| V - West                             | South Dakota Center for Blood Disorders                             | Sioux Falls   | SD    |
| V - West                             | Stroger Hospital of Cook County - Adults                            | Chicago       | IL    |
| V - West                             | Stroger Hospital of Cook County - Pediatrics                        | Chicago       | IL    |
| V - West                             | University of Minnesota Medical Center, Fairview                    | Minneapolis   | MN    |
| V - West                             | UWHC Comprehensive Program for Bleeding Disorders                   | Madison       | WI    |

## Definitions — Participation HTC by Region

| LIST OF PARTICIPATING HTCS BY REGION |  |                |       |
|--------------------------------------|--|----------------|-------|
| Region                               | ATHN Affiliate   | City           | State |
| Great Plains                         | Arkansas Center for Bleeding Disorders   | Little Rock    | AR    |
| Great Plains                         | Children's Mercy Hospital - Kansas City  | Kansas City    | MO    |
| Great Plains                         | Fort Worth Bleeding Disorders Program  | Fort Worth     | TX    |
| Great Plains                         | Gulf States Hemophilia and Thrombophilia Center  | Houston        | TX    |
| Great Plains                         | Hemophilia Treatment Center - Adult Program Saint Louis University                                     | St. Louis      | MO    |
| Great Plains                         | Iowa Hemophilia and Thrombosis Center  | Iowa City      | IA    |
| Great Plains                         | Louisiana Center for Bleeding and Clotting Disorders, Tulane University Health Science Center          | New Orleans    | LA    |
| Great Plains                         | Nebraska Regional Hemophilia Treatment Center  | Omaha          | NE    |
| Great Plains                         | North Texas Comprehensive Hemophilia Treatment Center  | Dallas         | TX    |
| Great Plains                         | North Texas Hemophilia and Thrombosis Program - Pediatric Program / Center for Cancer & Blood Disorder | Dallas         | TX    |
| Great Plains                         | Oklahoma Center for Bleeding & Clotting Disorders  | Oklahoma City  | OK    |
| Great Plains                         | Texas Children's Hemophilia & Thrombosis Center/Baylor College of Medicine                             | Houston        | TX    |
| Great Plains                         | The John Bouhasin Center for Children with Bleeding Disorders  | Saint Louis    | MO    |
| Great Plains                         | Washington University Center in St. Louis - Adult Program  | St Louis       | MO    |
| Great Plains                         | Washington University Center in St. Louis - Pediatric Program  | St Louis       | MO    |
| VIII                                 | Arizona Hemophilia and Thrombosis Center / University of Arizona Health Science Center                 | Tucson         | AZ    |
| VIII                                 | Arizona Hemophilia and Thrombosis Treatment Center at Phoenix Children's Hospital                      | Phoenix        | AZ    |
| VIII                                 | Intermountain Hemophilia and Thrombosis Center   | Salt Lake City | UT    |
| VIII                                 | University of Colorado Denver Hemophilia and Thrombosis Center   | Aurora         | CO    |
| VIII                                 | University of New Mexico Ted R. Montoya Hemophilia & Thrombosis Program                                | Albuquerque    | NM    |
| IX                                   | Center for Comprehensive Care and Diagnosis of Inherited Blood Disorders                               | Orange         | CA    |
| IX                                   | Childrens Hospital Los Angeles   | Los Angeles    | CA    |

## Definitions — Participation HTCs by Region

| LIST OF PARTICIPATING HTCS BY REGION |  |               |       |
|--------------------------------------|--|---------------|-------|
| Region                               | ATHN Affiliate   | City          | State |
| IX                                   | Children's Hospital of Central California                                    | Madera        | CA    |
| IX                                   | Children's Hospital Research Center Oakland                                  | Oakland       | CA    |
| IX                                   | City of Hope Medical Center  | Duarte        | CA    |
| IX                                   | Hemophilia Treatment Center of Nevada  | Las Vegas     | NV    |
| IX                                   | Kapiolani Medical Center for Women and Children                              | Honolulu      | HI    |
| IX                                   | LPCH/Stanford University HTC   | Palo Alto     | CA    |
| IX                                   | Orthopaedic Hospital of Los Angeles  | Los Angeles   | CA    |
| IX                                   | Rady Children's Hospital San Diego   | San Diego     | CA    |
| IX                                   | University of California at Davis Hemophilia Treatment Center                | Sacramento    | CA    |
| IX                                   | University of California, San Diego Hemophilia & Thrombosis Treatment Center | San Diego     | CA    |
| IX                                   | University of California, San Francisco Hemophilia & Thrombosis Center       | San Francisco | CA    |
| X                                    | Alaska Hemophilia Treatment Center   | Anchorage     | AK    |
| X                                    | Providence Sacred Heart Medical Center and Children's Hospital               | Spokane       | WA    |
| X                                    | Puget Sound Blood Center   | Seattle       | WA    |
| X                                    | Seattle Children's Hospital and Regional Medical Center                      | Seattle       | WA    |
| X                                    | St. Luke's Hemophilia Center   | Boise         | ID    |
| X                                    | The Hemophilia Center at Oregon Health & Science University                  | Portland      | OR    |



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**our mission.** *To provide stewardship of a secure national database, adherent to all privacy guidelines, which will be used to support clinical outcomes analysis, research, advocacy and public health reporting in the hemostasis and thrombosis community.*

**our values.** *Improving clinical outcomes and care, facilitating continuity of care, fostering collaboration, maintaining confidentiality, conserving resources through a common infrastructure.*