

# 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

CSL BEHRING  
 BIOGEN Idec  
 PFIZER

# Table of Contents

<b>ATHNdataset</b>	
<b>Growth of the ATHNdataset</b>	<b>Page #</b>
Patient Accrual Over Time	<b>6</b>
ATHNdataset Compared to 2010 Hemophilia Data Set	<b>7</b>
<b>Demographic Profile</b>	
Age and Sex By Region and Total US	<b>8</b>
Race and Ethnicity By Region and Total US	<b>9</b>
<b>Bleeding and Clotting Disorders Within the Population</b>	
Patient Authorizations By Primary Diagnosis Total US	<b>10</b>
<b>FACTOR VIII DEFICIENCY</b>	
<b>Bleeding Disorder Disease Severity</b>	
By Region and Total US	<b>12</b>
<b>HIV and Hepatitis C</b>	
By Age Group	<b>13</b>
<b>Continuous Prophylaxis (Prescribed)</b>	
By Bleeding Disorder Disease Severity and Age Group	<b>14</b>
<b>Payer Profile</b>	
By Region and Total US	<b>15</b>
<b>FACTOR IX DEFICIENCY</b>	
<b>Bleeding Disorder Disease Severity</b>	
By Region and Total US	<b>18</b>
<b>HIV and Hepatitis C</b>	
By Age Group	<b>19</b>
<b>Continuous Prophylaxis (Prescribed)</b>	
By Bleeding Disorder Disease Severity and Age Group	<b>20</b>
<b>Payer Profile</b>	
By Region and Total US	<b>21</b>

<b>VON WILLEBRAND DISEASE</b>	
<b>Demographic Profile</b>	
Age and Sex By Region and Total US	<b>24</b>
Race and Ethnicity By Region and Total US	<b>25</b>
<b>Disease Types</b>	
VWD Types By Region and Total US	<b>26</b>
<b>HIV and Hepatitis C</b>	
By Age Group	<b>27</b>
<b>DEFINITIONS</b>	
<b>Definition of Regions</b>	<b>30</b>
<b>Definition of Clinical Content</b>	<b>31</b>
<b>Definition of Medications</b>	<b>32</b>
<b>Definition of Patient Sample</b>	<b>33</b>
<b>Participating HTCs by Region</b>	<b>34-39</b>

**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.

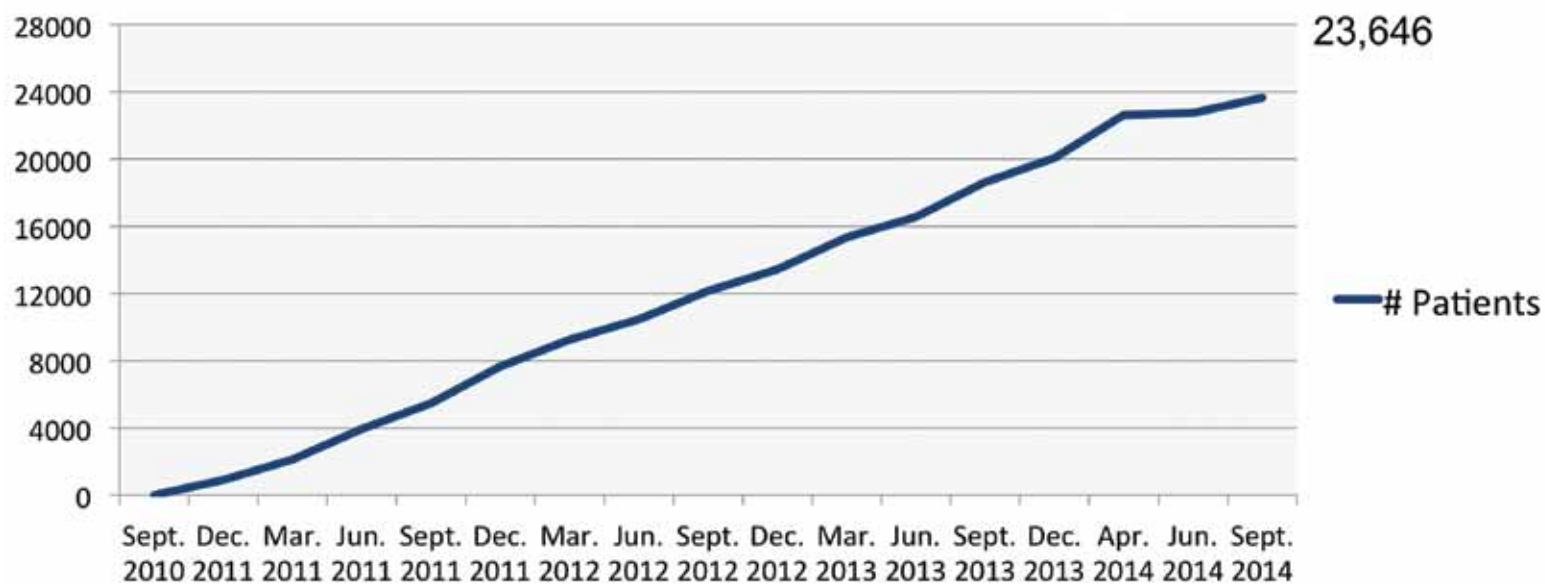
Finally, if you desire to include any extract of the report in any publication of yours, you must obtain prior written consent to such publication and agree to provide appropriate attribution to ATHN and its Affiliate HTCs.

# 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.

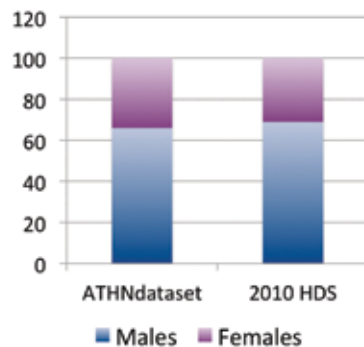
Individual level data are not available through the Hemophilia Data Set.

## 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>

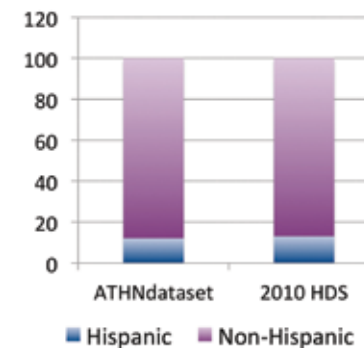


## ETHNICITY COMPARISON

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%

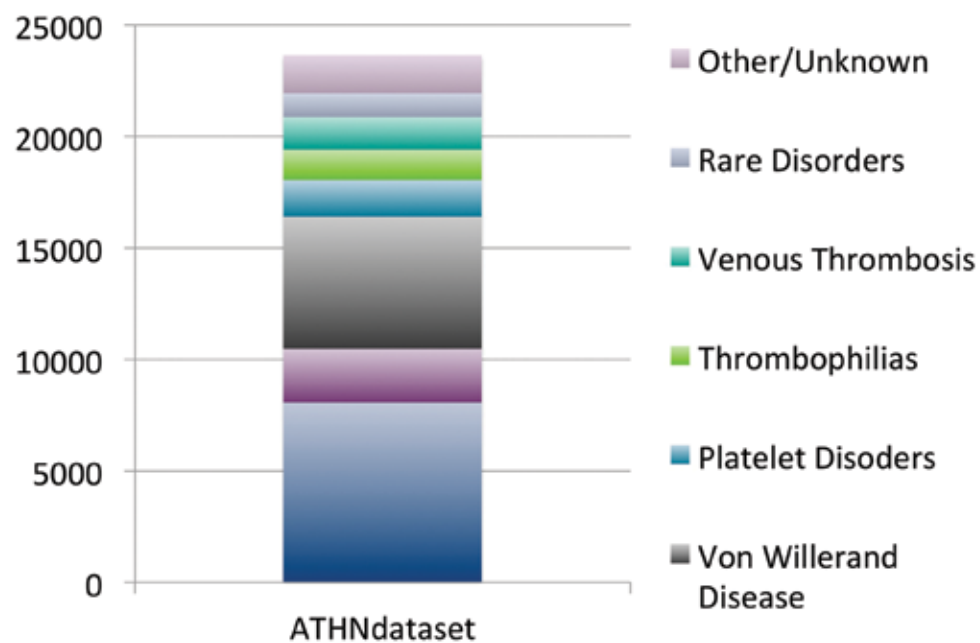
ATHNdataset Age	Mean	Standard Deviation	Median
	27	20	20





# 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 (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 (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. Individuals actively "opt in" to the ATHNdataset. 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



**american thrombosis & hemostasis network**  
72 Treasure Lane, Riverwoods, IL 60015  
Phone: 800-360-2846  
Visit: [www.athn.org](http://www.athn.org)

ATHN is a 501(c)(3) tax exempt organization.

**our vision.** *To advance and improve the care of individuals affected by bleeding and thrombotic disorders.*

**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.*