

# ATHN Research Report Brief

ATHNdataset — December 31, 2015



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## Thank You!

ATHN is grateful for the dedicated efforts of the ATHN-Affiliated Hemophilia Treatment Centers and their patients. The dedicated support and generous contributions of the ATHN Industry Consortium partners enable us to further our commitment to a safe, secure national database for improving the quality of care for patients with bleeding disorders.

Founding Visionary Partner



Visionary Partner



Industry Leader



Industry Supporters

CSL Behring  
Pfizer Hemophilia  
Bayer Healthcare  
Emergent BioSolution

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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 DECEMBER 31, 2015)

The ATHNdataset is a HIPAA compliant limited dataset under the stewardship of ATHN.

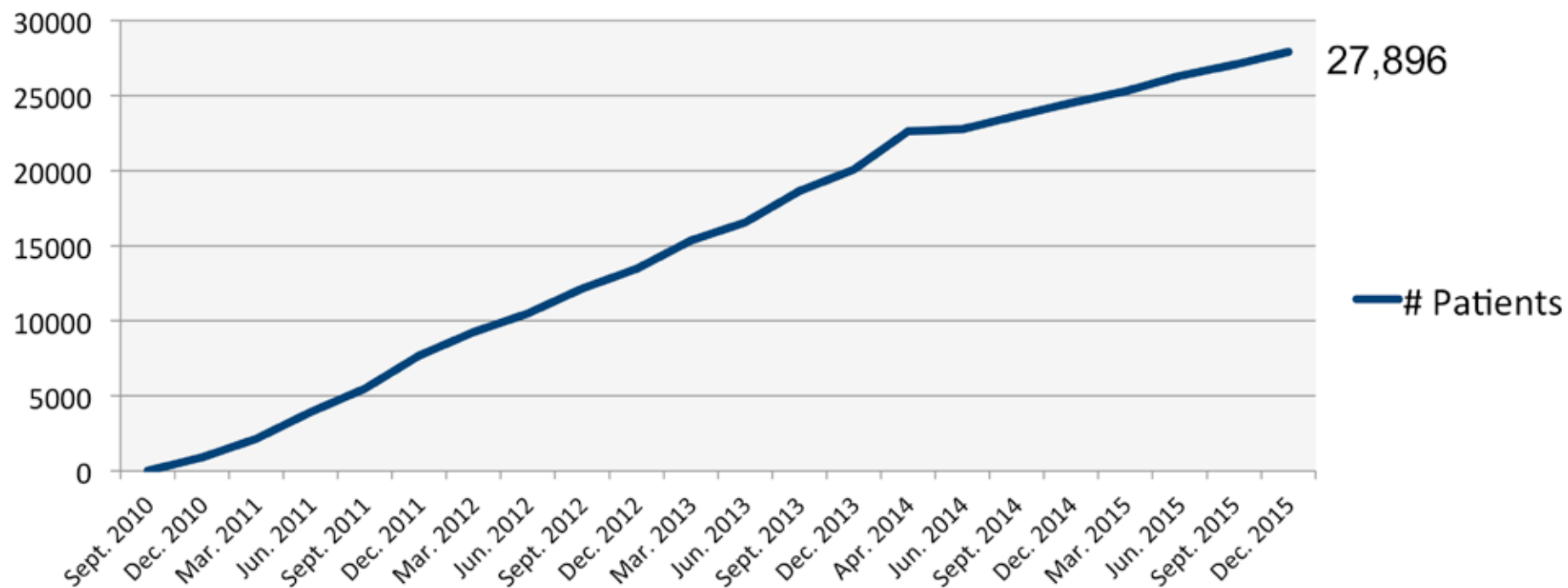
Participating Hemophilia Treatment Centers across the U.S. abstract data from the medical record.

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 December 2010, the ATHNdataset has grown to include 27,896 patients.



# ATHNdataset — Growth of ATHNdataset

ATHNdataset

ATHNdataset (AS OF DECEMBER 31, 2015) COMPARED TO 2010 HEMOPHILIA DATA SET 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.

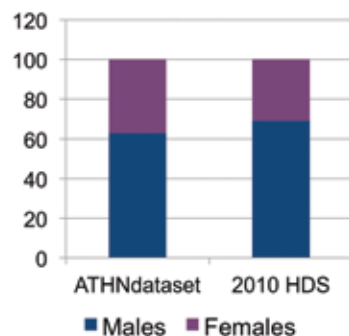
Individual level data are not available through the Hemophilia Data Set.

## GENDER COMPARISON

ATHNdataset Gender	# Patients	% Patients
Male	17,705*	63%
Female	10,191**	37%
<b>Total</b>	<b>27,896</b>	<b>100%</b>

\* 7 male to female \*\* 1 female to male

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

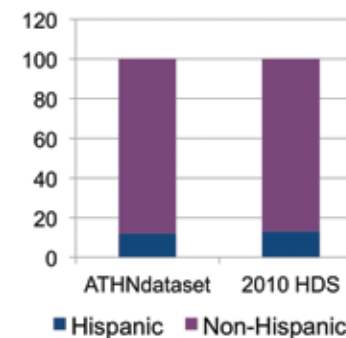


## ETHNICITY COMPARISON

ATHNdataset Ethnicity	# Patients	% Patients
Hispanic	3,516	13%
Non Hispanic	23,994	87%
<b>Total</b>	<b>27,510*</b>	<b>100%</b>

\* 386 Unknown

2010 HDS Ethnicity	# Patients	% Patients
Hispanic	4,289	12%
Non-Hispanic	28,323	88%
<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 DECEMBER 31, 2015)								
	REGION								TOTAL
	New England	Mid-Atlantic	Southeast	Great Lakes	Northern States	Great Plains	Mountain States	Western States	US
<b>BY SEX</b>									
Female	1291	560	866	4626	614	841	523	870	10191
	36.6%	26.3%	25.4%	50.4%	33.2%	29.3%	25.2%	30.6%	36.5%
Male	2240	1573	2547	4546	1238	2033	1551	1977	17705
	63.4%	73.8%	74.6%	49.6%	66.9%	70.7%	74.8%	69.4%	63.5%
<b>Total</b>	<b>3531</b>	<b>2133</b>	<b>3413</b>	<b>9172</b>	<b>1852</b>	<b>2874</b>	<b>2074</b>	<b>2847</b>	<b>27896</b>
	100%	100%	100%	100%	100%	100%	100%	100%	100%
<b>BY AGE</b>									
0-2 years	73	89	104	221	41	110	38	59	735
	2.1%	4.2%	3.1%	2.4%	2.2%	3.8%	1.8%	2.1%	2.6%
3-12 years	884	462	908	1684	403	913	463	822	6539
	25.0%	21.7%	26.6%	18.4%	21.8%	31.8%	22.3%	28.9%	23.4%
13-18 years	732	359	696	1657	271	629	336	635	5315
	20.7%	16.8%	20.4%	18.1%	14.6%	21.9%	16.2%	22.3%	19.1%
19-29 years	781	444	754	1737	394	483	450	667	5710
	22.1%	20.8%	22.1%	18.9%	21.3%	16.8%	21.7%	23.4%	20.5%
30-49 years	598	419	549	1747	437	382	465	410	5007
	16.9%	19.6%	16.1%	19.1%	23.6%	13.3%	22.4%	14.4%	17.9%
50-74 years	406	333	375	1851	268	317	296	230	4076
	11.5%	15.6%	11.0%	20.2%	14.5%	11.0%	14.3%	8.1%	14.6%
75+ years	57	27	27	275	38	40	26	24	514
	1.6%	1.3%	0.8%	3.0%	2.1%	1.4%	1.3%	0.8%	1.8%
<b>Total</b>	<b>3531</b>	<b>2133</b>	<b>3413</b>	<b>9172</b>	<b>1852</b>	<b>2874</b>	<b>2074</b>	<b>2847</b>	<b>27896</b>
	100%	100%	100%	100%	100%	100%	100%	100%	100%

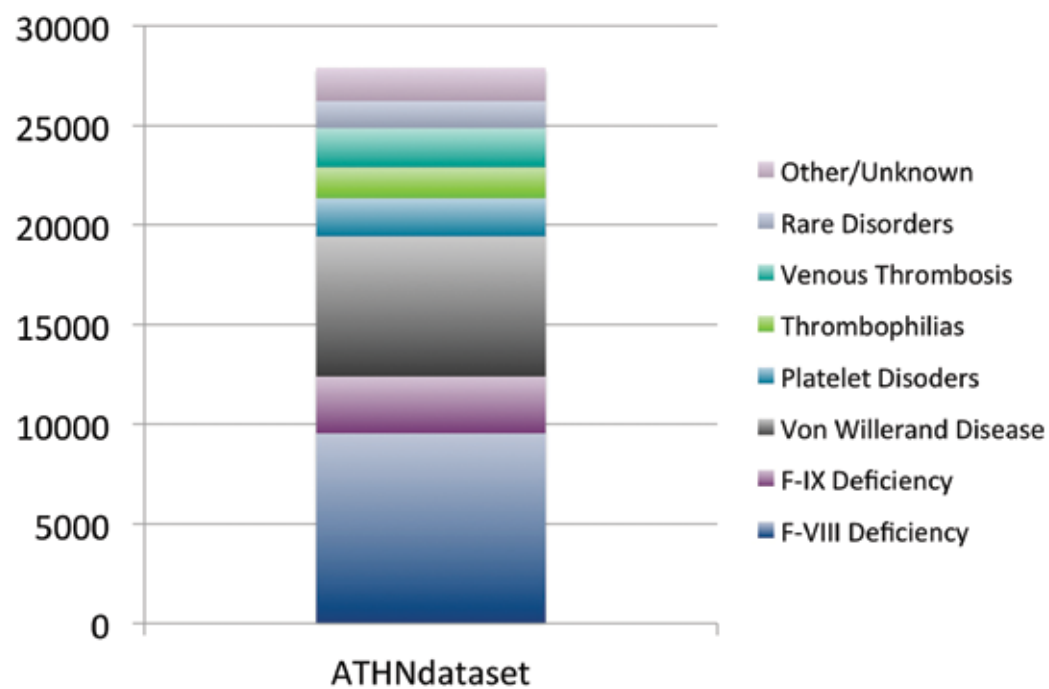
ATHNdataset Age	Mean	Standard Deviation	Median
	27	20	21





# ATHNdataset — Bleeding and Clotting Disorders Within the Population

ATHNdataset	PATIENT AUTHORIZATIONS BY PRIMARY DIAGNOSIS TOTAL U.S. (AS OF DECEMBER 31, 2015)	
PRIMARY BLEEDING OR CLOTTING DISORDER	TOTAL U.S. ATHNdataset CASES	% ATHNdataset
Factor VIII Deficiency	9553	34.25%
Factor IX Deficiency	2846	10.20%
Von Willebrand Disease	7032	25.21%
Rare Disorders	1346	4.83%
Platelet Disorders	1908	6.84%
Thrombophilias	1542	5.53%
Venous Thrombosis	1991	7.14%
Other/Unknown	1678	6.02%
<b>TOTAL</b>	<b>27896</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 DECEMBER 31, 2015)							
DISEASE SEVERITY	REGION								TOTAL
	New England	Mid-Atlantic	Southeast	Great Lakes	Northern States	Great Plains	Mountain States	Western States	US
<b>Mild</b>	347	224	402	475	202	325	334	370	2679
	28.5%	24.6%	24.6%	34.4%	27.9%	28.6%	30.7%	29.5%	28.6%
<b>Moderate</b>	217	162	322	215	122	183	199	203	1623
	17.8%	17.8%	19.7%	15.6%	16.8%	16.1%	18.3%	16.2%	17.4%
<b>Severe</b>	654	525	913	691	401	629	555	682	5050
	53.7%	57.6%	55.8%	50.0%	55.3%	55.3%	51.0%	54.3%	54.0%
<b>TOTAL</b>	<b>1218</b>	<b>911</b>	<b>1637</b>	<b>1381</b>	<b>725</b>	<b>1137</b>	<b>1088</b>	<b>1255</b>	<b>9352</b>
	100%	100%	100%	100%	100%	100%	100%	100%	100%
<b>Unknown Severity</b>	29	66	11	17	12	32	15	19	201
	2.3%	6.8%	0.7%	1.2%	1.6%	2.7%	1.4%	1.5%	2.1%
<b>TOTAL</b>	<b>1247</b>	<b>977</b>	<b>1648</b>	<b>1398</b>	<b>737</b>	<b>1169</b>	<b>1103</b>	<b>1274</b>	<b>9553</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 DECEMBER 31, 2015)						
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		8	364	255		630
	0.0%	0.0%		0.4%	18.9%	22.5%		6.6%
No HIV	308	2391	1552	2133	1561	880	98	8923
	100%	100%	99.9%	99.6%	81.1%	77.5%	98.0%	93.4%
<b>TOTAL</b>	<b>308</b>	<b>2391</b>	<b>1553</b>	<b>2141</b>	<b>1925</b>	<b>1135</b>	<b>100</b>	<b>9553</b>
	100%	100%	100%	100%	100%	100%	100%	100%
HEPATITIS CO-MORBIDITY								
Hepatitis C	0	0		77	923	580	26	1608
	0.0%	0.0%		3.6%	48.0%	51.1%	26.0%	16.8%
No Hepatitis C	308	2391	1551	2064	1002	555	74	7945
	100%	100%	99.9%	96.4%	52.1%	48.9%	74.0%	83.2%
<b>TOTAL</b>	<b>308</b>	<b>2391</b>	<b>1553</b>	<b>2141</b>	<b>1925</b>	<b>1135</b>	<b>100</b>	<b>9553</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 DECEMBER 31, 2015)							
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		24	28	30	21	15	0	119
			4.0%	6.4%	5.8%	4.2%	3.0%	0.0%	4.4%
	Not Prescribed/Unk	60	574	410	485	484	480	67	2560
		98.4%	96.0%	93.6%	94.2%	95.8%	97.0%	100%	95.6%
	<b>Total Mild</b>	<b>61</b>	<b>598</b>	<b>438</b>	<b>515</b>	<b>505</b>	<b>495</b>	<b>67</b>	<b>2679</b>
		100%	100%	100%	100%	100%	100%	100%	100%
Moderate	Prescribed	9	156	120	108	62	31		488
		18.8%	36.6%	44.3%	30.6%	21.0%	14.8%		30.1%
	Not Prescribed/Unk	39	270	151	245	234	179	17	1135
		81.3%	63.4%	55.7%	69.4%	79.1%	85.2%	89.5%	69.9%
	<b>Total Moderate</b>	<b>48</b>	<b>426</b>	<b>271</b>	<b>353</b>	<b>296</b>	<b>210</b>	<b>19</b>	<b>1623</b>
		100%	100%	100%	100%	100%	100%	100%	100%
Severe	Prescribed	71	1030	688	881	579	161		3412
		38.0%	77.4%	83.3%	71.3%	54.1%	41.3%		67.6%
	Not Prescribed/Unk	116	301	138	355	491	229	8	1638
		62.0%	22.6%	16.7%	28.7%	45.9%	58.7%	80.0%	32.4%
	<b>Total Severe</b>	<b>187</b>	<b>1331</b>	<b>826</b>	<b>1236</b>	<b>1070</b>	<b>390</b>	<b>10</b>	<b>5050</b>
		100%	100%	100%	100%	100%	100%	100%	100%
Unknown	Prescribed	0	4	3	4	1	3	0	15
		0.0%	11.1%	16.7%	10.8%	1.9%	7.5%	0.0%	7.5%
	Not Prescribed/Unk	12	32	15	33	53	37	4	186
		100%	88.9%	83.3%	89.2%	98.2%	92.5%	100%	92.5%
	<b>Total Unknown</b>	<b>12</b>	<b>36</b>	<b>18</b>	<b>37</b>	<b>54</b>	<b>40</b>	<b>4</b>	<b>201</b>
		100%	100%	100%	100%	100%	100%	100%	100%
<b>All Severities</b>	<b>Total All</b>	<b>308</b>	<b>2391</b>	<b>1553</b>	<b>2141</b>	<b>1925</b>	<b>1135</b>	<b>100</b>	<b>9553</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 DECEMBER 31, 2015)							
PAYER CATEGORY	REGION								TOTAL
	New England	Mid-Atlantic	Southeast	Great Lakes	Northern States	Great Plains	Mountain States	Western States	US
Medicaid	255	185	537	385	146	328	230	286	2352
	31.0%	31.5%	39.5%	32.0%	25.7%	35.2%	30.6%	26.7%	32.2%
Medicare	58	53	97	93	56	57	46	63	523
	7.1%	9.0%	7.1%	7.7%	9.8%	6.1%	6.1%	5.9%	7.2%
Private	476	328	641	582	349	493	445	343	3657
	57.9%	55.9%	47.1%	48.4%	61.3%	53.0%	59.2%	32.0%	50.1%
Uninsured			6			26		19	62
			0.4%			2.8%		1.8%	0.8%
Other	31	19	79	142	13	27	30	360	701
	3.8%	3.2%	5.8%	11.8%	2.3%	2.9%	4.0%	33.6%	9.6%
<b>TOTAL</b>	<b>822</b>	<b>587</b>	<b>1360</b>	<b>1203</b>	<b>569</b>	<b>931</b>	<b>752</b>	<b>1071</b>	<b>7295</b>
	100%	100%	100%	100%	100%	100%	100%	100%	100%
Unknown	425	390	288	195	168	238	351	203	2258
	34.1%	39.9%	17.5%	14.0%	22.8%	20.4%	31.8%	15.9%	23.6%
<b>TOTAL</b>	<b>1247</b>	<b>977</b>	<b>1648</b>	<b>1398</b>	<b>737</b>	<b>1169</b>	<b>1103</b>	<b>1274</b>	<b>9553</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 DECEMBER 31, 2015)							
DISEASE SEVERITY		REGION							TOTAL
	New England	Mid-Atlantic	Southeast	Great Lakes	Northern States	Great Plains	Mountain States	Western States	US
Mild	81	69	138	219	47	101	83	68	806
	28.5%	27.2%	33.4%	29.3%	21.5%	30.1%	30.5%	26.6%	29.0%
Moderate	85	88	126	384	85	146	104	54	1072
	29.9%	34.7%	30.5%	51.4%	38.8%	43.5%	38.2%	21.1%	38.5%
Severe	118	97	149	144	87	89	85	134	903
	41.6%	38.2%	36.1%	19.3%	39.7%	26.5%	31.3%	52.4%	32.5%
<b>TOTAL</b>	<b>284</b>	<b>254</b>	<b>413</b>	<b>747</b>	<b>219</b>	<b>336</b>	<b>272</b>	<b>256</b>	<b>2781</b>
	100%	100%	100%	100%	100%	100%	100%	100%	100%
Unknown Severity	13	15	6	12	4	5	4	6	65
	4.4%	5.6%	1.4%	1.6%	1.8%	1.5%	1.5%	2.3%	2.3%
<b>TOTAL</b>	<b>297</b>	<b>269</b>	<b>419</b>	<b>759</b>	<b>223</b>	<b>341</b>	<b>276</b>	<b>262</b>	<b>2846</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 DECEMBER 31, 2015)						
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		45	40	0	87
	0.0%	0.0%	0.0%		7.9%	8.9%	0.0%	3.1%
No HIV	97	679	408	592	522	410	51	2759
	100%	100%	100%	99.7%	92.1%	91.1%	100%	96.9%
<b>TOTAL</b>	<b>97</b>	<b>679</b>	<b>408</b>	<b>594</b>	<b>567</b>	<b>450</b>	<b>51</b>	<b>2846</b>
	100%	100%	100%	100%	100%	100%	100%	100%
HEPATITIS C CO-MORBIDITY								
Hepatitis C	0		0	35	216	198	10	460
	0.0%		0.0%	5.9%	38.1%	44.0%	19.6%	16.2%
No Hepatitis C	97	678	408	559	351	252	41	2386
	100%	99.9%	100%	94.1%	61.9%	56.0%	80.4%	83.8%
<b>TOTAL</b>	<b>97</b>	<b>679</b>	<b>408</b>	<b>594</b>	<b>567</b>	<b>450</b>	<b>51</b>	<b>2846</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 DECEMBER 31, 2015)							
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		7	8	11			0	36
			3.7%	6.7%	6.8%			0.0%	4.5%
	Not Prescribed/Unk	15	182	112	152	135	155	19	770
		88.2%	96.3%	93.3%	93.3%	96.4%	98.1%	100%	95.5%
	<b>Total Mild</b>	<b>17</b>	<b>189</b>	<b>120</b>	<b>163</b>	<b>140</b>	<b>158</b>	<b>19</b>	<b>806</b>
		100%	100%	100%	100%	100%	100%	100%	100%
Moderate	Prescribed	6	36	33	39	26	11	0	151
		16.7%	14.0%	20.1%	18.6%	12.4%	6.4%	0.0%	14.1%
	Not Prescribed/Unk	30	222	131	171	183	160	24	921
		83.3%	86.1%	79.9%	81.4%	87.6%	93.6%	100%	85.9%
	<b>Total Moderate</b>	<b>36</b>	<b>258</b>	<b>164</b>	<b>210</b>	<b>209</b>	<b>171</b>	<b>24</b>	<b>1072</b>
		100%	100%	100%	100%	100%	100%	100%	100%
Severe	Prescribed	18	153	94	130	102	50		548
		43.9%	68.9%	79.7%	61.0%	51.5%	48.1%		60.7%
	Not Prescribed/Unk	23	69	24	83	96	54	6	355
		56.1%	31.1%	20.3%	39.0%	48.5%	51.9%	85.7%	39.3%
	<b>Total Severe</b>	<b>41</b>	<b>222</b>	<b>118</b>	<b>213</b>	<b>198</b>	<b>104</b>	<b>7</b>	<b>903</b>
		100%	100%	100%	100%	100%	100%	100%	100%
Unknown	Prescribed	0	0	1	1	0	2	0	4
		0.0%	0.0%	16.7%	12.5%	0.0%	11.8%	0.0%	6.2%
	Not Prescribed/Unk	3	10	5	7	20	15	1	61
		100%	100%	83.3%	87.5%	100%	88.2%	100%	93.8%
	<b>Total Unknown</b>	<b>3</b>	<b>10</b>	<b>6</b>	<b>8</b>	<b>20</b>	<b>17</b>	<b>1</b>	<b>65</b>
		100%	100%	100%	100%	100%	100%	100%	100%
All Severities	<b>Total All</b>	<b>97</b>	<b>679</b>	<b>408</b>	<b>594</b>	<b>567</b>	<b>450</b>	<b>51</b>	<b>2846</b>
		100%	100%	100%	100%	100%	100%	100%	100%

## Factor IX Deficiency — Payer Profile

FACTOR IX DEFICIENCY		PAYER PROFILE BY REGION AND TOTAL U.S. (AS OF DECEMBER 31, 2015)							
PAYER CATEGORY	REGION								TOTAL
	New England	Mid-Atlantic	Southeast	Great Lakes	Northern States	Great Plains	Mountain States	Western States	US
Medicaid	53	37	103	130	35	85	44	50	537
	28.2%	28.9%	30.8%	27.4%	19.9%	36.3%	23.8%	23.0%	27.7%
Medicare	23	10	24	47	18	14	16	23	175
	12.2%	7.8%	7.2%	9.9%	10.2%	6.0%	8.7%	10.6%	9.0%
Private	106	75	187	238	115	128	119	75	1043
	56.4%	58.6%	55.8%	50.1%	65.3%	54.7%	64.3%	34.6%	53.8%
Uninsured				10	0				27
				2.1%	0.0%				1.4%
Other			18	50	8			64	156
			5.4%	10.5%	4.5%			29.5%	8.0%
<b>TOTAL</b>	<b>188</b>	<b>128</b>	<b>335</b>	<b>475</b>	<b>176</b>	<b>234</b>	<b>185</b>	<b>217</b>	<b>1938</b>
	100%	100%	100%	100%	100%	100%	100%	100%	100%
Unknown	109	141	84	284	47	107	91	45	908
	36.7%	52.4%	20.1%	37.4%	21.1%	31.4%	33.0%	17.2%	31.9%
<b>TOTAL</b>	<b>297</b>	<b>269</b>	<b>419</b>	<b>759</b>	<b>223</b>	<b>341</b>	<b>276</b>	<b>262</b>	<b>2846</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 DECEMBER 31, 2015)								
		REGION							TOTAL	
		New England	Mid-Atlantic	Southeast	Great Lakes	Northern States	Great Plains	Mountain States	Western States	US
<b>BY SEX</b>										
Female	754	344	559	1367	322	358	243	476	4423	
	64.0%	60.9%	62.4%	65.1%	62.3%	59.4%	64.6%	59.8%	62.9%	
Male	425	221	337	733	195	245	133	320	2609	
	36.1%	39.1%	37.6%	34.9%	37.7%	40.6%	35.4%	40.2%	37.1%	
<b>Total</b>	<b>1179</b>	<b>565</b>	<b>896</b>	<b>2100</b>	<b>517</b>	<b>603</b>	<b>376</b>	<b>796</b>	<b>7032</b>	
	100%	100%	100%	100%	100%	100%	100%	100%	100%	
<b>BY AGE</b>										
0-2 years	8	8	11	14		10	0		61	
	0.7%	1.4%	1.2%	0.7%		1.7%	0.0%		0.9%	
3-12 years	343	129	236	491	128	188	99	248	1862	
	29.1%	22.8%	26.3%	23.4%	24.8%	31.2%	26.3%	31.2%	26.5%	
13-18 years	342	120	223	512	103	175	82	243	1800	
	29.0%	21.2%	24.9%	24.4%	19.9%	29.0%	21.8%	30.5%	25.6%	
19-29 years	292	137	200	462	108	112	73	187	1571	
	24.8%	24.3%	22.3%	22.0%	20.9%	18.6%	19.4%	23.5%	22.3%	
30-49 years	106	88	135	339	105	62	56	68	959	
	9.0%	15.6%	15.1%	16.1%	20.3%	10.3%	14.9%	8.5%	13.6%	
50-74 years	81	77	80	250	56	52	63	40	699	
	6.9%	13.6%	8.9%	11.9%	10.8%	8.6%	16.8%	5.0%	9.9%	
75+ years	7	6	11	32	12				80	
	0.6%	1.1%	1.2%	1.5%	2.3%				1.1%	
<b>Total</b>	<b>1179</b>	<b>565</b>	<b>896</b>	<b>2100</b>	<b>517</b>	<b>603</b>	<b>376</b>	<b>796</b>	<b>7032</b>	
	100%	100%	100%	100%	100%	100%	100%	100%	100%	

Age (years)	Mean	Standard Deviation	Median
	24	17	18





## Von Willebrand Disease — Disease Types

VON WILLEBRAND DISEASE	VWD TYPES AND SUBTYPES — TOTAL U.S. (AS OF DECEMBER 31, 2015)		
VWD TYPE	TOTAL U.S.	SUBTYPE	% OF TYPE
VWD, Type 1	5642		
	82.8%		
		Type 1	5613
			99.5%
		Type 1C	29
			0.5%
VWD, Type 2	935		
	13.7%		
		Type 2	144
			15.4%
		Type 2A	351
			37.5%
		Type 2B	224
			24.0%
		Type 2M	156
			16.7%
		Type 2N	60
			6.4%
VWD, Type 3	233		
	3.4%		
		Type 3	233
			100%
<b>TOTAL w Type Specified</b>	<b>6810</b>		
	100%		
VWD, Type Other/Unknown	222		
	3.2%		
<b>TOTAL</b>	<b>7032</b>		
	100%		

# Von Willebrand Disease — HIV and Hepatitis C

VON WILLEBRAND DISEASE		HIV AND HEPATITIS C (HCV) BY AGE GROUP (AS OF DECEMBER 31, 2015)						
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		6		0	12
	0.0%	0.0%	0.0%		0.6%		0.0%	0.2%
No HIV	61	1862	1800	1569	953	695	80	7020
	100%	100%	100%	99.9%	99.4%	99.4%	100%	99.8%
<b>TOTAL</b>	<b>61</b>	<b>1862</b>	<b>1800</b>	<b>1571</b>	<b>959</b>	<b>699</b>	<b>80</b>	<b>7032</b>
	100%	100%	100%	100%	100%	100%	100%	100%
HEPATITIS C CO-MORBIDITY								
Hepatitis C	0	0	0	7	37	71	6	121
	0.0%	0.0%	0.0%	0.5%	3.9%	10.2%	7.5%	1.7%
No Hepatitis C	61	1862	1800	1564	922	628	74	6911
	100%	100%	100%	99.6%	96.1%	89.8%	92.5%	98.3%
<b>TOTAL</b>	<b>61</b>	<b>1862</b>	<b>1800</b>	<b>1571</b>	<b>959</b>	<b>699</b>	<b>80</b>	<b>7032</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), Adynovate (Baxalta), Helixate FS (CSL), Kogenate FS (Bayer), NovoEight (NovoNordisk), 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), IXinity (Emergent), 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®, Gammalex®, 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, Harvoni (Gilead), Sovaldi (Gilead), Viekira Pak (AbbieVie)
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 — Participating HTC's by Region

LIST OF PARTICIPATING HTC'S 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/UMass Memorial 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	SUNY Upstate Medical University-Adult Program	Syracuse	NY
II	SUNY Upstate Medical University-Pediatric Program	Syracuse	NY
II	University of Puerto Rico Hemophilia Treatment Center	San Juan	PR
II	UMDNJ-Robert Wood Johnson University Hospital	New Brunswick	NJ
II	Weill Cornell Medical College - New York Presbyterian Hospital	New York	NY

## Definitions — Participating HTC's by Region

LIST OF PARTICIPATING HTC'S 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	University of Tennessee - Memphis	Memphis	TN

## Definitions — Participating HTC by Region

LIST OF PARTICIPATING HTCS BY REGION			
Region	ATHN Affiliate	City	State
IV - North	Vanderbilt University Medical Center	Nashville	TN
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
IV - South	University of South Florida Adult Comprehensive Hemophilia Treatment Center	Tampa	FL
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

## Definitions — Participating HTC's by Region

LIST OF PARTICIPATING HTC'S BY REGION			
Region	ATHN Affiliate	City	State
V - East	Michigan State University Center for Bleeding Disorders & Clotting	East Lansing	MI
V - East	Nationwide Children's Hospital Columbus	Columbus	OH
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

## Definitions — Participating HTC by Region

LIST OF PARTICIPATING HTCS BY REGION			
Region	ATHN Affiliate	City	State
V - West	University of Minnesota Medical Center, Fairview	Minneapolis	MN
V - West	UWHC Comprehensive Program for Bleeding Disorders	Madison	WI
Great Plains	Arkansas Center for Bleeding Disorders	Little Rock	AR
Great Plains	Children's Mercy Hospital - Kansas City	Kansas City	MO
Great Plains	Childrens Hosptial New Orleans	New Orleans	LA
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	South Texas Comprehensive Hemophilia and Thrombophilia Treatment Center	San Antonio	TX
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

## Definitions — Participating HTC's by Region

LIST OF PARTICIPATING HTC'S BY REGION			
Region	ATHN Affiliate	City	State
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
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	Guam Comprehensive Hemophilia Care Program	Guam	GU
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	UCSF Benioff Children's Hospital Oakland	Oakland	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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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.