

# ATHN Research Report Brief

ATHNdataset — March 31, 2016



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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



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CSL Behring  
Pfizer Hemophilia  
Bayer Healthcare  
Emergent BioSolution

Industry Associate

Genentech

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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 MARCH 31, 2016)

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

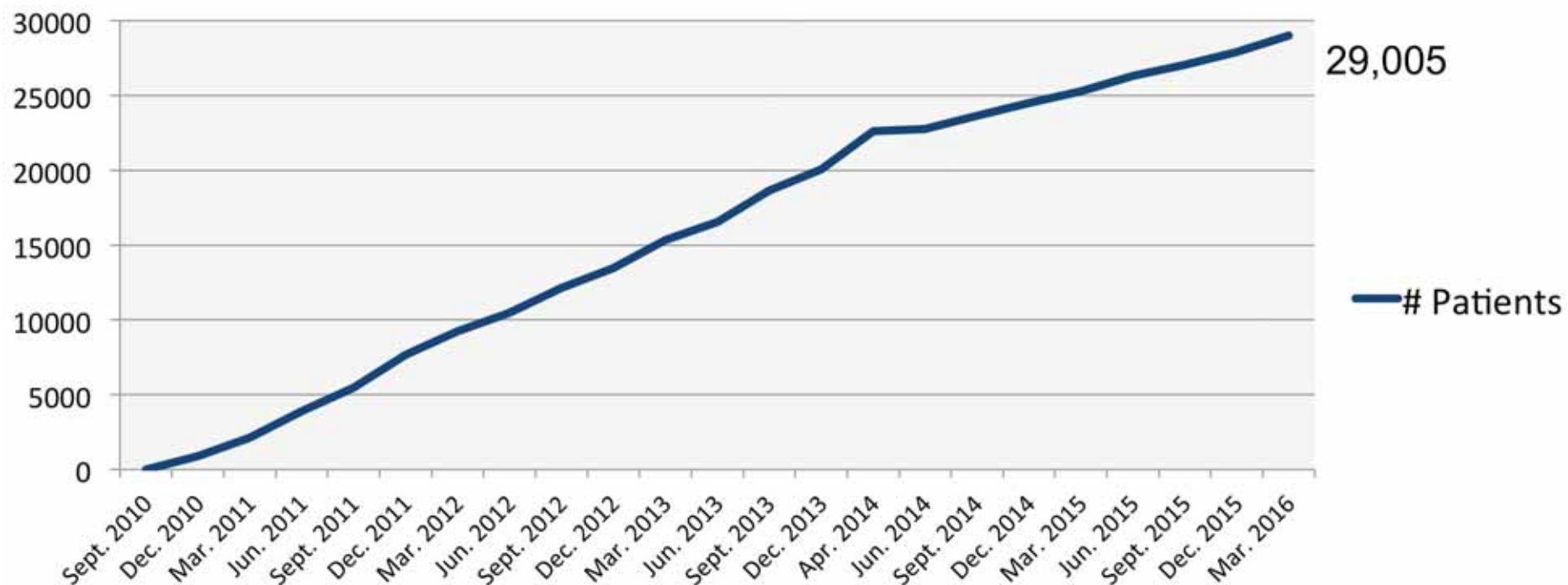
Participating Hemophilia Treatment Centers across the U.S. abstract data from the medical record.

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 29,005 patients.



# ATHNdataset — Growth of ATHNdataset

ATHNdataset

ATHNdataset (AS OF MARCH 31, 2016) 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.

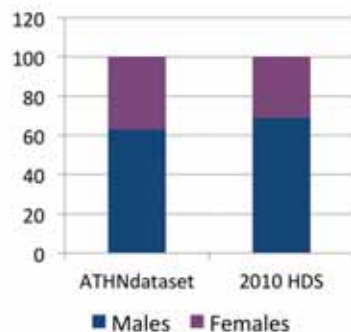
Individual level data are not available through the Hemophilia Data Set.

## GENDER COMPARISON

ATHNdataset Gender	# Patients	% Patients
Male	18,169*	63%
Female	10,836**	37%
<b>Total</b>	<b>29,005</b>	<b>100%</b>

\* 5 male to female \*\* 3 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>

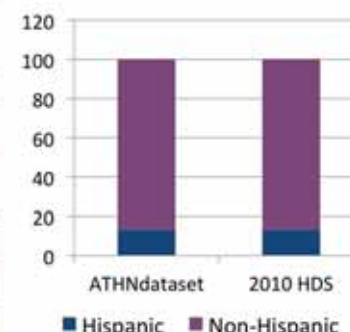


## ETHNICITY COMPARISON

ATHNdataset Ethnicity	# Patients	% Patients
Hispanic	3,646	13%
Non Hispanic	24,928	87%
<b>Total</b>	<b>28,574*</b>	<b>100%</b>

\* 431 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 MARCH 31, 2016)								
	REGION								TOTAL
	New England	Mid-Atlantic	Southeast	Great Lakes	Northern States	Great Plains	Mountain States	Western States	US
<b>BY SEX</b>									
Female	1339	627	930	4810	721	917	582	910	10836
	36.9%	27.9%	26.4%	51.1%	35.9%	30.1%	26.6%	31.1%	37.4%
Male	2291	1622	2595	4610	1288	2133	1610	2020	18169
	63.1%	72.1%	73.6%	48.9%	64.1%	69.9%	73.5%	68.9%	62.6%
<b>Total</b>	<b>3630</b>	<b>2249</b>	<b>3525</b>	<b>9420</b>	<b>2009</b>	<b>3050</b>	<b>2192</b>	<b>2930</b>	<b>29005</b>
	100%	100%	100%	100%	100%	100%	100%	100%	100%
<b>BY AGE</b>									
0-2 years	67	100	108	241	46	121	44	65	792
	1.9%	4.5%	3.1%	2.6%	2.3%	4.0%	2.0%	2.2%	2.7%
3-12 years	905	476	914	1731	420	940	471	858	6715
	24.9%	21.2%	25.9%	18.4%	20.9%	30.8%	21.5%	29.3%	23.2%
13-18 years	755	378	702	1695	294	669	336	667	5496
	20.8%	16.8%	19.9%	18.0%	14.6%	21.9%	15.3%	22.8%	18.9%
19-29 years	829	469	782	1806	430	516	488	662	5982
	22.8%	20.9%	22.2%	19.2%	21.4%	16.9%	22.3%	22.6%	20.6%
30-49 years	606	446	598	1781	486	421	504	420	5262
	16.7%	19.8%	17.0%	18.9%	24.2%	13.8%	23.0%	14.3%	18.1%
50-74 years	413	353	393	1890	291	338	321	236	4235
	11.4%	15.7%	11.2%	20.1%	14.5%	11.1%	14.6%	8.1%	14.6%
75+ years	55	27	28	276	42	45	28	22	523
	1.5%	1.2%	0.8%	2.9%	2.1%	1.5%	1.3%	0.8%	1.8%
<b>Total</b>	<b>3630</b>	<b>2249</b>	<b>3525</b>	<b>9420</b>	<b>2009</b>	<b>3050</b>	<b>2192</b>	<b>2930</b>	<b>29005</b>
	100%	100%	100%	100%	100%	100%	100%	100%	100%

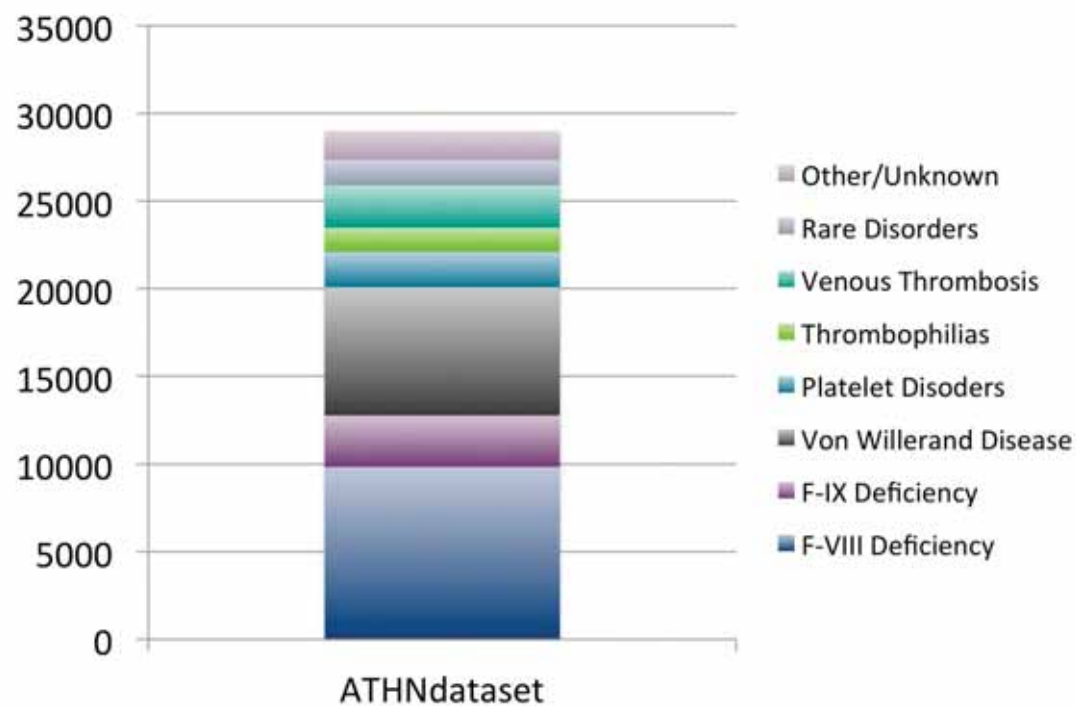
ATHNdataset Age	Mean	Standard Deviation	Median
	27	20	21





# ATHNdataset — Bleeding and Clotting Disorders Within the Population

ATHNdataset	PATIENT AUTHORIZATIONS BY PRIMARY DIAGNOSIS TOTAL U.S. (AS OF MARCH 31, 2016)	
PRIMARY BLEEDING OR CLOTTING DISORDER	TOTAL U.S. ATHNdataset CASES	% ATHNdataset
Factor VIII Deficiency	9835	33.91%
Factor IX Deficiency	2933	10.11%
Von Willebrand Disease	7317	25.23%
Rare Disorders	1452	5.01%
Platelet Disorders	2012	6.94%
Thrombophilias	1379	4.75%
Venous Thrombosis	2419	8.34%
Other/Unknown	1658	5.72%
<b>TOTAL</b>	<b>29005</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 MARCH 31, 2016)							
DISEASE SEVERITY	REGION								TOTAL
	New England	Mid-Atlantic	Southeast	Great Lakes	Northern States	Great Plains	Mountain States	Western States	US
Mild	378	251	424	495	210	349	352	386	2845
	30.2%	25.0%	25.2%	35.4%	28.1%	29.3%	31.2%	30.3%	29.4%
Moderate	230	183	332	220	126	189	208	213	1701
	18.4%	18.2%	19.8%	15.7%	16.9%	15.9%	18.4%	16.7%	17.6%
Severe	644	572	925	684	411	653	568	675	5132
	51.4%	56.9%	55.0%	48.9%	55.0%	54.8%	50.4%	53.0%	53.0%
<b>TOTAL</b>	<b>1252</b>	<b>1006</b>	<b>1681</b>	<b>1399</b>	<b>747</b>	<b>1191</b>	<b>1128</b>	<b>1274</b>	<b>9678</b>
	100%	100%	100%	100%	100%	100%	100%	100%	100%
Unknown Severity	24	13	9	29	12	37	13	20	157
	1.9%	1.3%	0.5%	2.0%	1.6%	3.0%	1.1%	1.6%	1.6%
<b>TOTAL</b>	<b>1276</b>	<b>1019</b>	<b>1690</b>	<b>1428</b>	<b>759</b>	<b>1228</b>	<b>1141</b>	<b>1294</b>	<b>9835</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 MARCH 31, 2016)						
AGE								TOTAL
<b>HIV CO-MORBIDITY</b>								
	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	365	270		647
	0.0%	0.0%		0.4%	18.3%	22.8%		6.6%
No HIV	331	2416	1577	2221	1626	914	103	9188
	100.0%	100.0%	99.9%	99.6%	81.7%	77.2%	97.2%	93.4%
<b>TOTAL</b>	<b>331</b>	<b>2416</b>	<b>1578</b>	<b>2229</b>	<b>1991</b>	<b>1184</b>	<b>106</b>	<b>9835</b>
	100%	100%	100%	100%	100%	100%	100%	100%
<b>HEPATITIS CO-MORBIDITY</b>								
Hepatitis C	0	0		72	890	557	27	1549
	0.0%	0.0%		3.2%	44.7%	47.0%	25.5%	15.7%
No Hepatitis C	331	2416	1575	2157	1101	627	79	8286
	100.0%	100.0%	99.8%	96.8%	55.3%	53.0%	74.5%	84.3%
<b>TOTAL</b>	<b>331</b>	<b>2416</b>	<b>1578</b>	<b>2229</b>	<b>1991</b>	<b>1184</b>	<b>106</b>	<b>9835</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 MARCH 31, 2016)							
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		32	36	28	28	19	0	145
			5.0%	8.1%	5.0%	5.2%	3.6%	0.0%	5.1%
	Not Prescribed/Unk	62	605	408	528	515	512	70	2700
		96.9%	95.0%	91.9%	95.0%	94.8%	96.4%	100%	94.9%
	<b>Total Mild</b>	<b>64</b>	<b>637</b>	<b>444</b>	<b>556</b>	<b>543</b>	<b>531</b>	<b>70</b>	<b>2845</b>
		100%	100%	100%	100%	100%	100%	100%	100%
Moderate	Prescribed	10	166	123	113	69	31		514
		15.6%	38.9%	43.3%	30.5%	22.0%	14.0%		30.2%
	Not Prescribed/Unk	54	261	161	257	244	191	19	1187
		84.4%	61.1%	56.7%	69.5%	78.0%	86.0%	90.5%	69.8%
	<b>Total Moderate</b>	<b>64</b>	<b>427</b>	<b>284</b>	<b>370</b>	<b>313</b>	<b>222</b>	<b>21</b>	<b>1701</b>
		100%	100%	100%	100%	100%	100%	100%	100%
Severe	Prescribed	71	1044	709	926	610	171		3533
		36.0%	79.2%	85.0%	72.9%	55.7%	42.2%		68.8%
	Not Prescribed/Unk	126	275	125	344	485	234	10	1599
		64.0%	20.9%	15.0%	27.1%	44.3%	57.8%	83.3%	31.2%
	<b>Total Severe</b>	<b>197</b>	<b>1319</b>	<b>834</b>	<b>1270</b>	<b>1095</b>	<b>405</b>	<b>12</b>	<b>5132</b>
		100%	100%	100%	100%	100%	100%	100%	100%
Unknown	Prescribed	0	2	3	6	2	1	0	14
		0.0%	6.1%	18.8%	18.2%	5.0%	3.9%	0.0%	8.9%
	Not Prescribed/Unk	6	31	13	27	38	25	3	143
		100%	93.9%	81.3%	81.8%	95.0%	96.2%	100%	91.1%
	<b>Total Unknown</b>	<b>6</b>	<b>33</b>	<b>16</b>	<b>33</b>	<b>40</b>	<b>26</b>	<b>3</b>	<b>157</b>
		100%	100%	100%	100%	100%	100%	100%	100%
<b>All Severities</b>	<b>Total All</b>	<b>331</b>	<b>2416</b>	<b>1578</b>	<b>2229</b>	<b>1991</b>	<b>1184</b>	<b>106</b>	<b>9835</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 MARCH 31, 2016)								
PAYER CATEGORY		REGION							TOTAL	
	New England	Mid-Atlantic	Southeast	Great Lakes	Northern States	Great Plains	Mountain States	Western States	US	
Medicaid	255	180	566	386	169	349	239	281	2425	
	29.1%	28.2%	39.7%	31.0%	27.1%	35.4%	29.3%	26.0%	31.5%	
Medicare	60	70	104	90	63	55	49	56	547	
	6.9%	11.0%	7.3%	7.2%	10.1%	5.6%	6.0%	5.2%	7.1%	
Private	524	367	672	629	379	533	496	358	3958	
	59.9%	57.5%	47.1%	50.4%	60.8%	54.1%	60.8%	33.1%	51.4%	
Uninsured		0				23		15	51	
		0.0%				2.3%		1.4%	0.7%	
Other	35	21	80	139	9	26	31	373	714	
	4.0%	3.3%	5.6%	11.2%	1.5%	2.6%	3.8%	34.4%	9.3%	
<b>TOTAL</b>	<b>875</b>	<b>638</b>	<b>1427</b>	<b>1247</b>	<b>623</b>	<b>986</b>	<b>816</b>	<b>1083</b>	<b>7695</b>	
	100%	100%	100%	100%	100%	100%	100%	100%	100%	
Unknown	401	381	263	181	136	242	325	211	2140	
	31.4%	37.4%	15.6%	12.7%	17.9%	19.7%	28.5%	16.3%	21.8%	
<b>TOTAL</b>	<b>1276</b>	<b>1019</b>	<b>1690</b>	<b>1428</b>	<b>759</b>	<b>1228</b>	<b>1141</b>	<b>1294</b>	<b>9835</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 MARCH 31, 2016)							
DISEASE SEVERITY		REGION							TOTAL
	New England	Mid-Atlantic	Southeast	Great Lakes	Northern States	Great Plains	Mountain States	Western States	US
Mild	87	76	146	226	49	106	91	71	852
	29.2%	27.8%	34.9%	29.8%	21.1%	30.1%	31.1%	27.3%	29.5%
Moderate	89	93	130	386	90	151	115	54	1108
	29.9%	34.1%	31.1%	50.9%	38.8%	42.9%	39.3%	20.8%	38.4%
Severe	122	104	142	146	93	95	87	135	924
	40.9%	38.1%	34.0%	19.3%	40.1%	27.0%	29.7%	51.9%	32.0%
<b>TOTAL</b>	<b>298</b>	<b>273</b>	<b>418</b>	<b>758</b>	<b>232</b>	<b>352</b>	<b>293</b>	<b>260</b>	<b>2884</b>
	100%	100%	100%	100%	100%	100%	100%	100%	100%
Unknown Severity	10	3	7	12	7	4	1	5	49
	3.3%	1.1%	1.7%	1.6%	2.9%	1.1%	0.3%	1.9%	1.7%
<b>TOTAL</b>	<b>308</b>	<b>276</b>	<b>425</b>	<b>770</b>	<b>239</b>	<b>356</b>	<b>294</b>	<b>265</b>	<b>2933</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 MARCH 31, 2016)						
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%		7.6%	8.5%	0.0%	3.0%
No HIV	101	681	428	601	549	431	55	2846
	100.0%	100.0%	100.0%	99.7%	92.4%	91.5%	100.0%	97.0%
<b>TOTAL</b>	<b>101</b>	<b>681</b>	<b>428</b>	<b>603</b>	<b>594</b>	<b>471</b>	<b>55</b>	<b>2933</b>
	100%	100%	100%	100%	100%	100%	100%	100%
HEPATITIS C CO-MORBIDITY								
Hepatitis C	0	0	0	32	199	192	11	434
	0.0%	0.0%	0.0%	5.3%	33.5%	40.8%	20.0%	14.8%
No Hepatitis C	101	681	428	571	395	279	44	2499
	100.0%	100.0%	100.0%	94.7%	66.5%	59.2%	80.0%	85.2%
<b>TOTAL</b>	<b>101</b>	<b>681</b>	<b>428</b>	<b>603</b>	<b>594</b>	<b>471</b>	<b>55</b>	<b>2933</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 MARCH 31, 2016)							
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		12	10	13	6		0	44
			5.9%	8.5%	7.7%	3.9%		0.0%	5.2%
	Not Prescribed/Unk	16	190	108	155	148	169	22	808
		94.1%	94.1%	91.5%	92.3%	96.1%	98.8%	100%	94.8%
	<b>Total Mild</b>	<b>17</b>	<b>202</b>	<b>118</b>	<b>168</b>	<b>154</b>	<b>171</b>	<b>22</b>	<b>852</b>
		100%	100%	100%	100%	100%	100%	100%	100%
Moderate	Prescribed		33	38	43	28	15	0	162
			13.1%	21.6%	19.9%	12.8%	8.1%	0.0%	14.6%
	Not Prescribed/Unk	30	219	138	173	191	170	25	946
		85.7%	86.9%	78.4%	80.1%	87.2%	91.9%	100%	85.4%
	<b>Total Moderate</b>	<b>35</b>	<b>252</b>	<b>176</b>	<b>216</b>	<b>219</b>	<b>185</b>	<b>25</b>	<b>1108</b>
		100%	100%	100%	100%	100%	100%	100%	100%
Severe	Prescribed	19	158	100	138	106	52		574
		40.4%	72.2%	78.1%	66.0%	51.5%	48.2%		62.1%
	Not Prescribed/Unk	28	61	28	71	100	56	6	350
		59.6%	27.9%	21.9%	34.0%	48.5%	51.9%	85.7%	37.9%
	<b>Total Severe</b>	<b>47</b>	<b>219</b>	<b>128</b>	<b>209</b>	<b>206</b>	<b>108</b>	<b>7</b>	<b>924</b>
		100%	100%	100%	100%	100%	100%	100%	100%
Unknown	Prescribed	0	0	1	0	0	0	0	1
		0.0%	0.0%	16.7%	0.0%	0.0%	0.0%	0.0%	2.0%
	Not Prescribed/Unk	2	8	5	10	15	7	1	48
		100%	100%	83.3%	100%	100%	100%	100%	98.0%
	<b>Total Unknown</b>	<b>2</b>	<b>8</b>	<b>6</b>	<b>10</b>	<b>15</b>	<b>7</b>	<b>1</b>	<b>49</b>
		100%	100%	100%	100%	100%	100%	100%	100%
All Severities	<b>Total All</b>	<b>101</b>	<b>681</b>	<b>428</b>	<b>603</b>	<b>594</b>	<b>471</b>	<b>55</b>	<b>2933</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 MARCH 31, 2016)							
PAYER CATEGORY	REGION								TOTAL
	New England	Mid-Atlantic	Southeast	Great Lakes	Northern States	Great Plains	Mountain States	Western States	US
Medicaid	56	34	105	129	45	91	53	54	567
	27.9%	25.0%	30.4%	26.7%	23.9%	35.1%	25.4%	24.8%	27.8%
Medicare	20	11	25	41	14	13	20	22	166
	10.0%	8.1%	7.2%	8.5%	7.5%	5.0%	9.6%	10.1%	8.1%
Private	117	86	196	252	122	150	130	78	1131
	58.2%	63.2%	56.7%	52.2%	64.9%	57.9%	62.2%	35.8%	55.4%
Uninsured				7	0				18
				1.5%	0.0%				0.9%
Other	7		19	54	7			60	158
	3.5%		5.5%	11.2%	3.7%			27.5%	7.7%
<b>TOTAL</b>	<b>201</b>	<b>136</b>	<b>346</b>	<b>483</b>	<b>188</b>	<b>259</b>	<b>209</b>	<b>218</b>	<b>2040</b>
	100%	100%	100%	100%	100%	100%	100%	100%	100%
Unknown	107	140	79	287	51	97	85	47	893
	34.7%	50.7%	18.6%	37.3%	21.3%	27.3%	28.9%	17.7%	30.4%
<b>TOTAL</b>	<b>308</b>	<b>276</b>	<b>425</b>	<b>770</b>	<b>239</b>	<b>356</b>	<b>294</b>	<b>265</b>	<b>2933</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 MARCH 31, 2016)								
		REGION							TOTAL	
		New England	Mid-Atlantic	Southeast	Great Lakes	Northern States	Great Plains	Mountain States	Western States	US
<b>BY SEX</b>										
Female	798	364	586	1396	341	382	263	490	4620	
	63.7%	61.9%	63.0%	65.5%	62.8%	59.6%	65.6%	59.1%	63.1%	
Male	454	224	344	737	202	259	138	339	2697	
	36.3%	38.1%	37.0%	34.6%	37.2%	40.4%	34.4%	40.9%	36.9%	
<b>Total</b>	<b>1252</b>	<b>588</b>	<b>930</b>	<b>2133</b>	<b>543</b>	<b>641</b>	<b>401</b>	<b>829</b>	<b>7317</b>	
	100%	100%	100%	100%	100%	100%	100%	100%	100%	
<b>BY AGE</b>										
0-2 years		9	13	23		9		6	71	
		1.5%	1.4%	1.1%		1.4%		0.7%	1.0%	
3-12 years	366	128	234	485	131	190	102	267	1903	
	29.2%	21.8%	25.2%	22.7%	24.1%	29.6%	25.4%	32.2%	26.0%	
13-18 years	347	124	232	527	113	192	84	251	1870	
	27.7%	21.1%	25.0%	24.7%	20.8%	30.0%	21.0%	30.3%	25.6%	
19-29 years	321	144	210	475	114	121	76	191	1652	
	25.6%	24.5%	22.6%	22.3%	21.0%	18.9%	19.0%	23.0%	22.6%	
30-49 years	117	95	145	339	110	66	65	68	1005	
	9.4%	16.2%	15.6%	15.9%	20.3%	10.3%	16.2%	8.2%	13.7%	
50-74 years	87	83	85	251	58	57	70	42	733	
	7.0%	14.1%	9.1%	11.8%	10.7%	8.9%	17.5%	5.1%	10.0%	
75+ years	9		11	33	12	6			83	
	0.7%		1.2%	1.5%	2.2%	0.9%			1.1%	
<b>Total</b>	<b>1252</b>	<b>588</b>	<b>930</b>	<b>2133</b>	<b>543</b>	<b>641</b>	<b>401</b>	<b>829</b>	<b>7317</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 MARCH 31, 2016)		
VWD TYPE	TOTAL U.S.	SUBTYPE	% OF TYPE
VWD, Type 1	5857		
	82.7%		
		Type 1	5828
			99.5%
		Type 1C	29
			0.5%
VWD, Type 2	985		
	13.9%		
		Type 2	148
			15.0%
		Type 2A	370
			37.6%
		Type 2B	236
			24.0%
		Type 2M	166
			16.9%
		Type 2N	65
			6.6%
VWD, Type 3	237		
	3.3%		
		Type 3	237
			100.0%
<b>TOTAL w Type Specified</b>	<b>7079</b>		
	100%		
VWD, Type Other/Unknown	238		
	3.3%		
<b>TOTAL</b>	<b>7317</b>		
	100%		

# Von Willebrand Disease — HIV and Hepatitis C

VON WILLEBRAND DISEASE		HIV AND HEPATITIS C (HCV) BY AGE GROUP (AS OF MARCH 31, 2016)						
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	71	1903	1870	1650	999	729	83	7305
	100.0%	100.0%	100.0%	99.9%	99.4%	99.5%	100.0%	99.8%
<b>TOTAL</b>	<b>71</b>	<b>1903</b>	<b>1870</b>	<b>1652</b>	<b>1005</b>	<b>733</b>	<b>83</b>	<b>7317</b>
	100%	100%	100%	100%	100%	100%	100%	100%
HEPATITIS C CO-MORBIDITY								
Hepatitis C	0	0	0	6	34	71		116
	0.0%	0.0%	0.0%	0.4%	3.4%	9.7%		1.6%
No Hepatitis C	71	1903	1870	1646	971	662	78	7201
	100.0%	100.0%	100.0%	99.6%	96.6%	90.3%	94.0%	98.4%
<b>TOTAL</b>	<b>71</b>	<b>1903</b>	<b>1870</b>	<b>1652</b>	<b>1005</b>	<b>733</b>	<b>83</b>	<b>7317</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), NUWIQ (Octapharma)
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®, Privilgen®, 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 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	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 by Region

LIST OF PARTICIPATING HTCS 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	Bloodworks Northwest 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.