

ATHN Research Report Brief

ATHNdataset — December 31, 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



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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, 2014)

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

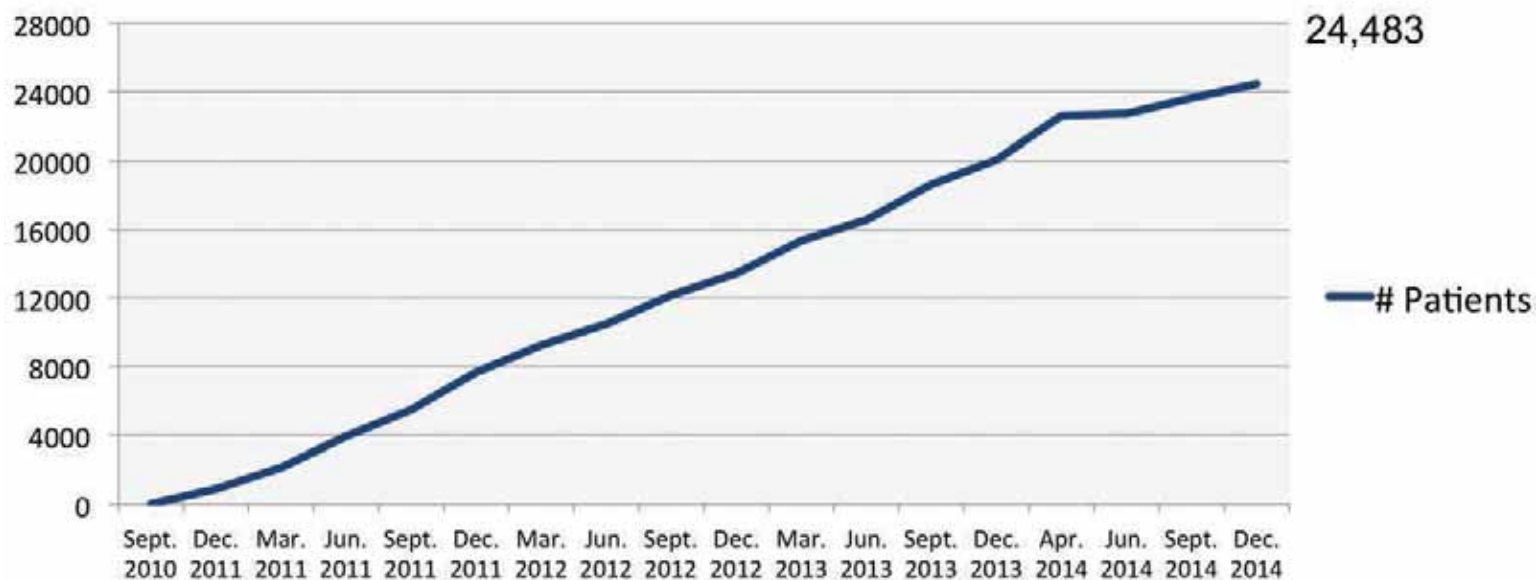
Participating Hemophilia Treatment Centers across the U.S. abstract data from the medical record.

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 24,483 patients.



ATHNdataset — Growth of ATHNdataset

ATHNdataset

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

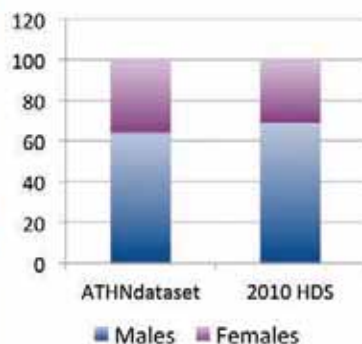
Individual level data are not available through the Hemophilia Data Set.

GENDER COMPARISON

ATHNdataset Gender	# Patients	% Patients
Male	15,660	64%
Female	8,823	36%
Total	24,483	100%

* 3 male to female

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

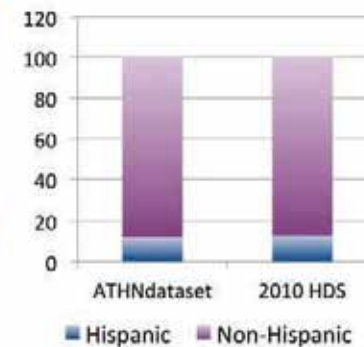


ETHNICITY COMPARISON

ATHNdataset Ethnicity	# Patients	% Patients
Hispanic	2,904	12%
Non Hispanic	21,251	88%
Total	24,155*	100%

* 328 unknown

2010 HDS Ethnicity	# Patients	% Patients
Hispanic	4,289	12%
Non-Hispanic	28,323	88%
Total	32,612	100%



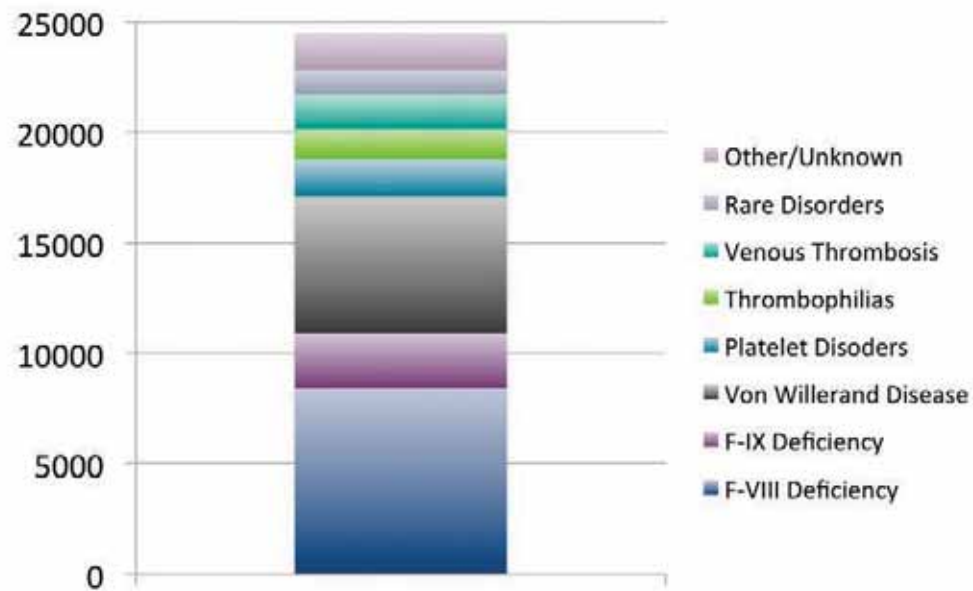
ATHNdataset — Demographic Profile

ATHNdataset	AGE AND SEX BY REGION AND TOTAL U.S. (AS OF DECEMBER 31, 2014)								
	REGION								TOTAL
	New England	Mid-Atlantic	Southeast	Great Lakes	Northern States	Great Plains	Mountain States	Western States	US
BY SEX									
Female	1134	412	729	4224	484	685	414	741	8823
	35.8%	22.9%	24.7%	49.9%	32.1%	28.7%	24.0%	29.9%	36.0%
Male	2032	1385	2227	4244	1023	1701	1312	1736	15660
	64.2%	77.1%	75.3%	50.1%	67.9%	71.3%	76.0%	70.1%	64.0%
Total	3166	1797	2956	8468	1507	2386	1726	2477	24483
	100%	100%	100%	100%	100%	100%	100%	100%	100%
BY AGE									
0-2 years	79	65	91	249	37	124	52	67	764
	2.5%	3.6%	3.1%	2.9%	2.5%	5.2%	3.0%	2.7%	3.1%
3-12 years	837	402	782	1663	334	792	398	718	5926
	26.4%	22.4%	26.5%	19.6%	22.2%	33.2%	23.1%	29.0%	24.2%
13-18 years	666	318	621	1517	205	548	304	577	4756
	21.0%	17.7%	21.0%	17.9%	13.6%	23.0%	17.6%	23.3%	19.4%
19-29 years	670	357	643	1569	335	378	347	550	4849
	21.2%	19.9%	21.8%	18.5%	22.2%	15.8%	20.1%	22.2%	19.8%
30-49 years	521	357	471	1587	362	273	379	344	4294
	16.5%	19.9%	15.9%	18.7%	24.0%	11.4%	22.0%	13.9%	17.5%
50-74 years	347	274	322	1658	209	249	223	205	3487
	11.0%	15.3%	10.9%	19.6%	13.9%	10.4%	12.9%	8.3%	14.2%
75+ years	46	24	26	225	25	22	23	16	407
	1.5%	1.3%	0.9%	2.7%	1.7%	0.9%	1.3%	0.7%	1.7%
Total	3166	1797	2956	8468	1507	2386	1726	2477	24483
	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 DECEMBER 31, 2014)	
PRIMARY BLEEDING OR CLOTTING DISORDER	TOTAL U.S. ATHNdataset CASES	% ATHNdataset
Factor VIII Deficiency	8396	34.29%
Factor IX Deficiency	2513	10.26%
Von Willebrand Disease	6187	25.27%
Rare Disorders	1129	4.61%
Platelet Disorders	1656	6.76%
Thrombophilias	1358	5.55%
Venous Thrombosis	1584	6.47%
Other/Unknown	1660	6.78%
TOTAL	24483	100.00%



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, 2014)							
DISEASE SEVERITY	REGION								TOTAL
	New England	Mid-Atlantic	Southeast	Great Lakes	Northern States	Great Plains	Mountain States	Western States	US
Mild	296	184	338	384	154	263	255	334	2208
	26.8%	22.7%	23.7%	30.5%	26.7%	27.4%	27.7%	30.2%	27.0%
Moderate	186	149	278	205	100	155	169	172	1414
	16.8%	18.4%	19.5%	16.3%	17.4%	16.2%	18.4%	15.6%	17.3%
Severe	623	478	809	672	322	542	496	599	4541
	56.4%	58.9%	56.8%	53.3%	55.9%	56.5%	53.9%	54.2%	55.6%
TOTAL	1105	811	1425	1261	576	960	920	1105	8163
	100%	100%	100%	100%	100%	100%	100%	100%	100%
Unknown Severity	34	47	15	51	19	24	16	27	233
	3.0%	5.5%	1.0%	3.9%	3.2%	2.4%	1.7%	2.4%	2.8%
TOTAL	1139	858	1440	1312	595	984	936	1132	8396
	100%	100%	100%	100%	100%	100%	100%	100%	100%

Factor VIII Deficiency — HIV and Hepatitis C

FACTOR VIII DEFICIENCY		2 HIV AND HEPATITIS C (HCV) BY AGE GROUP (AS OF DECEMBER 31, 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		346	201		554
	0.0%	0.0%	0.0%		21.6%	20.9%		6.6%
No HIV	300	2190	1355	1906	1254	759	78	7842
	100.0%	100.0%	100.0%	99.7%	78.4%	79.1%	97.5%	93.4%
TOTAL	300	2190	1355	1911	1600	960	80	8396
	100%	100%	100%	100%	100%	100%	100%	100%
HEPATITIS CO-MORBIDITY								
Hepatitis C	0	0		110	853	529	23	1516
	0.0%	0.0%		5.8%	53.3%	55.1%	28.8%	18.1%
No Hepatitis C	300	2190	1354	1801	747	431	57	6880
	100.0%	100.0%	99.9%	94.2%	46.7%	44.9%	71.3%	81.9%
TOTAL	300	2190	1355	1911	1600	960	80	8396
	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, 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	25	11	11	0	97
			4.9%	6.4%	6.0%	2.8%	2.7%	0.0%	4.4%
	Not Prescribed/Unk	55	482	351	392	378	400	53	2111
		98.2%	95.1%	93.6%	94.0%	97.2%	97.3%	100.0%	95.6%
	Total Mild	56	507	375	417	389	411	53	2208
		100.0%	100.0%	100.0%	100.0%	100.0%	100.0%	100.0%	100.0%
Moderate	Prescribed	10	127	93	90	46	17	0	383
		18.5%	34.4%	38.8%	28.5%	19.2%	9.3%	0.0%	27.1%
	Not Prescribed/Unk	44	242	147	226	194	166	12	1031
		81.5%	65.6%	61.3%	71.5%	80.8%	90.7%	100.0%	72.9%
	Total Moderate	54	369	240	316	240	183	12	1414
		100.0%	100.0%	100.0%	100.0%	100.0%	100.0%	100.0%	100.0%
Severe	Prescribed	63	912	564	743	434	115		2834
		35.4%	71.6%	79.6%	65.6%	47.7%	35.0%		62.4%
	Not Prescribed/Unk	115	361	145	389	476	214	7	1707
		64.6%	28.4%	20.5%	34.4%	52.3%	65.1%	70.0%	37.6%
	Total Severe	178	1273	709	1132	910	329	10	4541
		100.0%	100.0%	100.0%	100.0%	100.0%	100.0%	100.0%	100.0%
Unknown	Prescribed	0	6	6	6	5	5	0	28
		0.0%	14.6%	19.4%	13.0%	8.2%	13.5%	0.0%	12.0%
	Not Prescribed/Unk	12	35	25	40	56	32	5	205
		100.0%	85.4%	80.7%	87.0%	91.8%	86.5%	100.0%	88.0%
	Total Unknown	12	41	31	46	61	37	5	233
		100.0%	100.0%	100.0%	100.0%	100.0%	100.0%	100.0%	100.0%
All Severities	Total All	300	2190	1355	1911	1600	960	80	8396
		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, 2014)								
PAYER CATEGORY		REGION							TOTAL	
	New England	Mid-Atlantic	Southeast	Great Lakes	Northern States	Great Plains	Mountain States	Western States	US	
Medicaid	187	133	354	298	118	255	153	174	1672	
	28.0%	33.5%	39.4%	33.7%	26.9%	37.8%	28.6%	22.2%	31.7%	
Medicare	40	48	75	80	44	31	28	48	394	
	6.0%	12.1%	8.3%	9.0%	10.0%	4.6%	5.2%	6.1%	7.5%	
Private	417	203	428	389	264	331	337	275	2644	
	62.5%	51.1%	47.6%	44.0%	60.1%	49.0%	63.0%	35.1%	50.1%	
Uninsured			11		7	28		29	83	
			1.2%		1.6%	4.2%		3.7%	1.6%	
Other	20	11	31	117	6	30	15	257	487	
	3.0%	2.8%	3.5%	13.2%	1.4%	4.4%	2.8%	32.8%	9.2%	
TOTAL	667	397	899	885	439	675	535	783	5280	
	100%	100%	100%	100%	100%	100%	100%	100%	100%	
Unknown	472	461	541	427	156	309	401	349	3116	
	41.4%	53.7%	37.6%	32.6%	26.2%	31.4%	42.8%	30.8%	37.1%	
TOTAL	1139	858	1440	1312	595	984	936	1132	8396	
	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, 2014)							
DISEASE SEVERITY		REGION							TOTAL
	New England	Mid-Atlantic	Southeast	Great Lakes	Northern States	Great Plains	Mountain States	Western States	US
Mild	70	62	113	186	42	80	69	59	681
	27.3%	27.3%	31.5%	27.2%	22.7%	29.9%	30.4%	26.3%	28.0%
Moderate	75	81	114	364	71	119	86	43	953
	29.3%	35.7%	31.8%	53.2%	38.4%	44.4%	37.9%	19.2%	39.2%
Severe	111	84	132	134	72	69	72	122	796
	43.4%	37.0%	36.8%	19.6%	38.9%	25.8%	31.7%	54.5%	32.8%
TOTAL	256	227	359	684	185	268	227	224	2430
	100%	100%	100%	100%	100%	100%	100%	100%	100%
Unknown Severity	12	14	9	24	6	4	4	10	83
	4.5%	5.8%	2.5%	3.4%	3.1%	1.5%	1.7%	4.3%	3.3%
TOTAL	268	241	368	708	191	272	231	234	2513
	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, 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		50	28	0	80
	0.0%	0.0%	0.0%		9.7%	7.4%	0.0%	3.2%
No HIV	83	624	376	503	465	349	33	2433
	100.0%	100.0%	100.0%	99.6%	90.3%	92.6%	100.0%	96.8%
TOTAL	83	624	376	505	515	377	33	2513
	100%	100%	100%	100%	100%	100%	100%	100%
HEPATITIS C CO-MORBIDITY								
Hepatitis C	0		0	34	221	169	7	432
	0.0%		0.0%	6.7%	42.9%	44.8%	21.2%	17.2%
No Hepatitis C	83	623	376	471	294	208	26	2081
	100.0%	99.8%	100.0%	93.3%	57.1%	55.2%	78.8%	82.8%
TOTAL	83	624	376	505	515	377	33	2513
	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, 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		7	7					27
			4.3%	6.3%					4.0%
	Not Prescribed/Unk	9	157	104	134	116	123	11	654
		90.0%	95.7%	93.7%	96.4%	97.5%	97.6%	91.7%	96.0%
	Total Mild	10	164	111	139	119	126	12	681
		100.0%	100.0%	100.0%	100.0%	100.0%	100.0%	100.0%	100.0%
Moderate	Prescribed		28	28	24	16	8	0	107
			11.5%	18.2%	14.7%	8.3%	5.5%	0.0%	11.2%
	Not Prescribed/Unk	38	215	126	139	176	138	14	846
		92.7%	88.5%	81.8%	85.3%	91.7%	94.5%	100.0%	88.8%
	Total Moderate	41	243	154	163	192	146	14	953
		100.0%	100.0%	100.0%	100.0%	100.0%	100.0%	100.0%	100.0%
Severe	Prescribed	7	141	72	109	69	35		434
		24.1%	69.5%	70.6%	57.1%	38.8%	39.8%		54.5%
	Not Prescribed/Unk	22	62	30	82	109	53		362
		75.9%	30.5%	29.4%	42.9%	61.2%	60.2%		45.5%
	Total Severe	29	203	102	191	178	88	5	796
		100.0%	100.0%	100.0%	100.0%	100.0%	100.0%	100.0%	100.0%
Unknown	Prescribed	0	1	2	2	0	1	0	6
		0.0%	7.1%	22.2%	16.7%	0.0%	5.9%	0.0%	7.2%
	Not Prescribed/Unk	3	13	7	10	26	16	2	77
		100.0%	92.9%	77.8%	83.3%	100.0%	94.1%	100.0%	92.8%
	Total Unknown	3	14	9	12	26	17	2	83
		100.0%	100.0%	100.0%	100.0%	100.0%	100.0%	100.0%	100.0%
All Severities	Total All	83	624	376	505	515	377	33	2513
		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, 2014)								
PAYER CATEGORY		REGION							TOTAL	
	New England	Mid-Atlantic	Southeast	Great Lakes	Northern States	Great Plains	Mountain States	Western States	US	
Medicaid	49	20	56	100	31	51	26	27	360	
	32.7%	24.1%	29.2%	30.8%	21.4%	30.5%	20.3%	16.8%	26.6%	
Medicare	13	7	21	25	16	9	10	19	120	
	8.7%	8.4%	10.9%	7.7%	11.0%	5.4%	7.8%	11.8%	8.9%	
Private	84	50	104	142	91	98	89	61	719	
	56.0%	60.2%	54.2%	43.7%	62.8%	58.7%	69.5%	37.9%	53.2%	
Uninsured	0			10	0			8	29	
	0.0%			3.1%	0.0%			5.0%	2.1%	
Other			9	48	7			46	123	
			4.7%	14.8%	4.8%			28.6%	9.1%	
TOTAL	150	83	192	325	145	167	128	161	1351	
	100%	100%	100%	100%	100%	100%	100%	100%	100%	
Unknown	118	158	176	383	46	105	103	73	1162	
	44.0%	65.6%	47.8%	54.1%	24.1%	38.6%	44.6%	31.2%	46.2%	
TOTAL	268	241	368	708	191	272	231	234	2513	
	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, 2014)								
		REGION							TOTAL	
		New England	Mid-Atlantic	Southeast	Great Lakes	Northern States	Great Plains	Mountain States	Western States	US
BY SEX										
Female	678	277	471	1286	268	299	190	413	3882	
	64.0%	59.4%	61.4%	65.5%	61.2%	58.5%	66.4%	59.4%	62.7%	
Male	381	189	296	679	170	212	96	282	2305	
	36.0%	40.6%	38.6%	34.6%	38.8%	41.5%	33.6%	40.6%	37.3%	
Total	1059	466	767	1965	438	511	286	695	6187	
	100%	100%	100%	100%	100%	100%	100%	100%	100%	
BY AGE										
0-2 years	11		9	18		9			65	
	1.0%		1.2%	0.9%		1.8%			1.1%	
3-12 years	330	119	198	477	122	173	73	226	1718	
	31.2%	25.5%	25.8%	24.3%	27.9%	33.9%	25.5%	32.5%	27.8%	
13-18 years	324	108	207	473	74	162	67	226	1641	
	30.6%	23.2%	27.0%	24.1%	16.9%	31.7%	23.4%	32.5%	26.5%	
19-29 years	229	103	160	425	96	85	47	143	1288	
	21.6%	22.1%	20.9%	21.6%	21.9%	16.6%	16.4%	20.6%	20.8%	
30-49 years	88	69	116	316	90	41	46	58	824	
	8.3%	14.8%	15.1%	16.1%	20.6%	8.0%	16.1%	8.4%	13.3%	
50-74 years	71	56	67	228	44	39	45	35	585	
	6.7%	12.0%	8.7%	11.6%	10.1%	7.6%	15.7%	5.0%	9.5%	
75+ years	6	7	10	28	7				66	
	0.6%	1.5%	1.3%	1.4%	1.6%				1.1%	
Total	1059	466	767	1965	438	511	286	695	6187	
	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 DECEMBER 31, 2014)							
VWD TYPE	REGION								TOTAL
	New England	Mid-Atlantic	Southeast	Great Lakes	Northern States	Great Plains	Mountain States	Western States	US
VWD, Type 1	910	379	556	1647	338	377	191	583	4981
	88.2%	84.2%	76.1%	85.6%	79.0%	78.7%	72.1%	86.1%	83.2%
VWD, Type 2	89	50	134	231	69	81	59	73	786
	8.6%	11.1%	18.3%	12.0%	16.1%	16.9%	22.3%	10.8%	13.1%
VWD, Type 3	33	21	41	46	21	21	15	21	219
	3.2%	4.7%	5.6%	2.4%	4.9%	4.4%	5.7%	3.1%	3.7%
TOTAL w Type Specified	1032	450	731	1924	428	479	265	677	5986
	100%	100%	100%	100%	100%	100%	100%	100%	100%
VWD, Type Other/Unknown	27	16	36	41	10	32	21	18	201
	2.6%	3.4%	4.7%	2.1%	2.3%	6.3%	7.3%	2.6%	3.2%
TOTAL	1059	466	767	1965	438	511	286	695	6187
	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 (DECEMBER 31, 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		6		0	10
	0.0%	0.0%	0.0%		0.7%		0.0%	0.2%
No HIV	65	1718	1641	1287	818	582	66	6177
	100.0%	100.0%	100.0%	99.9%	99.3%	99.5%	100.0%	99.8%
TOTAL	65	1718	1641	1288	824	585	66	6187
	100%	100%	100%	100%	100%	100%	100%	100%
HEPATITIS C CO-MORBIDITY								
Hepatitis C	0	0	0		36	61		107
	0.0%	0.0%	0.0%		4.4%	10.4%		1.7%
No Hepatitis C	65	1718	1641	1283	788	524	61	6080
	100.0%	100.0%	100.0%	99.6%	95.6%	89.6%	92.4%	98.3%
TOTAL	65	1718	1641	1288	824	585	66	6187
	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 — 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	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 — 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	Vanderbilt University Medical Center	Nashville	TN

Definitions — Participating HTC 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
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
V - East	Michigan State University Center for Bleeding Disorders & Clotting	East Lansing	MI

Definitions — Participating HTC by Region

LIST OF PARTICIPATING HTCS BY REGION			
Region	ATHN Affiliate	City	State
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
V - West	University of Minnesota Medical Center, Fairview	Minneapolis	MN

Definitions — Participating HTC by Region

LIST OF PARTICIPATING HTCS BY REGION			
Region	ATHN Affiliate	City	State
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	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

Definitions — Participating HTCs by Region

LIST OF PARTICIPATING HTCS BY REGION			
Region	ATHN Affiliate	City	State
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	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

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.*