



ATHN Research Report Brief

ATHNdataset — December 31, 2013



Acknowledgements

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

Thank you HTC's!

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.

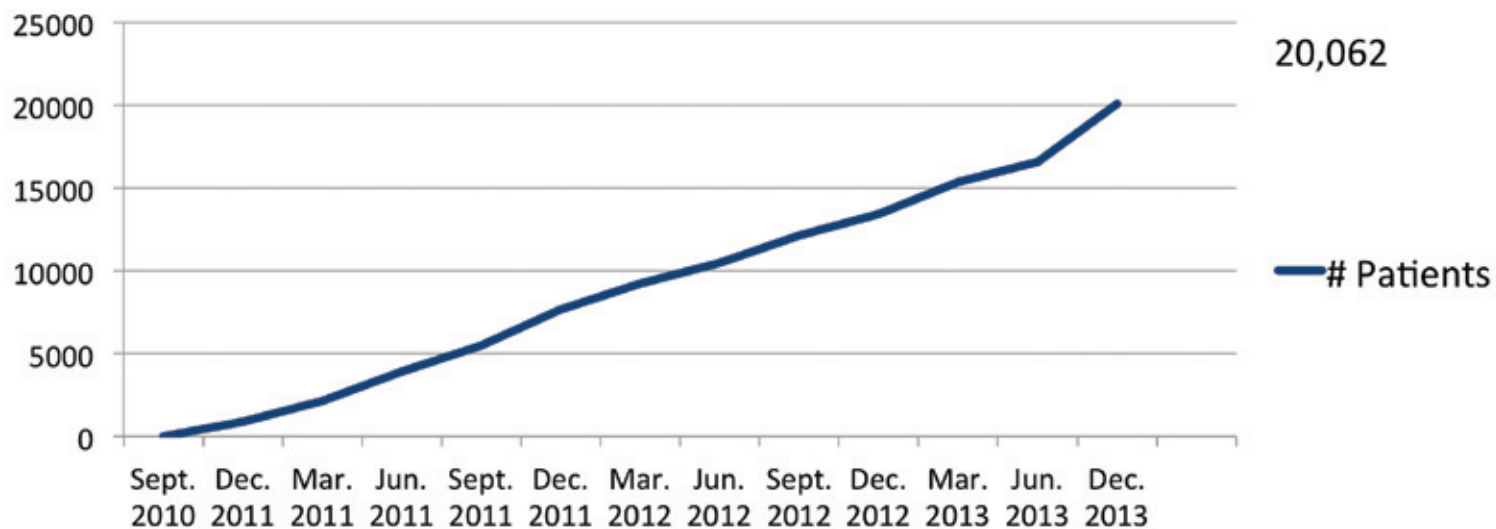
Finally, if you desire to include any extract of the report in any publication of yours, you must obtain prior written consent to such publication and agree to provide appropriate attribution to ATHN and its Affiliate HTCs.

ATHNdataset

ATHNdataset — Growth of the ATHNdataset

ATHNdataset PATIENT ACCRUAL OVER TIME (AS OF DECEMBER 31, 2013)

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 20,062 patients.



ATHNdataset — Demographic Profile

ATHNdataset

ATHNdataset COMPARED TO 2010 HEMOPHILIA DATA SET (AS OF DECEMBER 31, 2013)

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

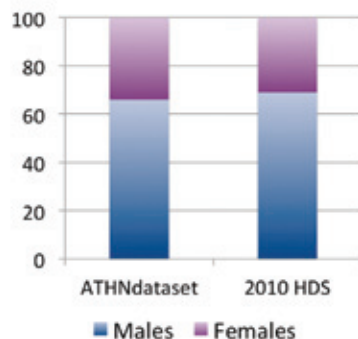
The 2010 Hemophilia Data Set included 32,612 active patients with Factor VIII Deficiency, Factor IX Deficiency, Von Willebrand Disease and other factor deficiencies.

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

GENDER COMPARISON

ATHNdataset Gender	# Patients	% Patients
Male	12,190	66%
Female	6,434	34%
Total	18,624	100%

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

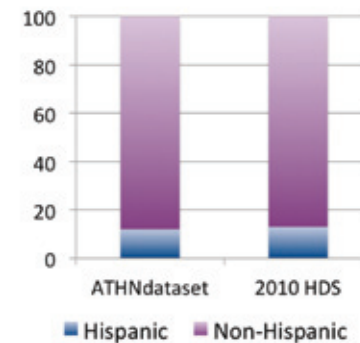


ETHNICITY COMPARISON

ATHNdataset Ethnicity	# Patients	% Patients
Hispanic	2,190	12%
Non Hispanic	16,398	88%
Total	18,588*	100%

* 36 Unknown

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



ATHNdataset — Demographic Profile

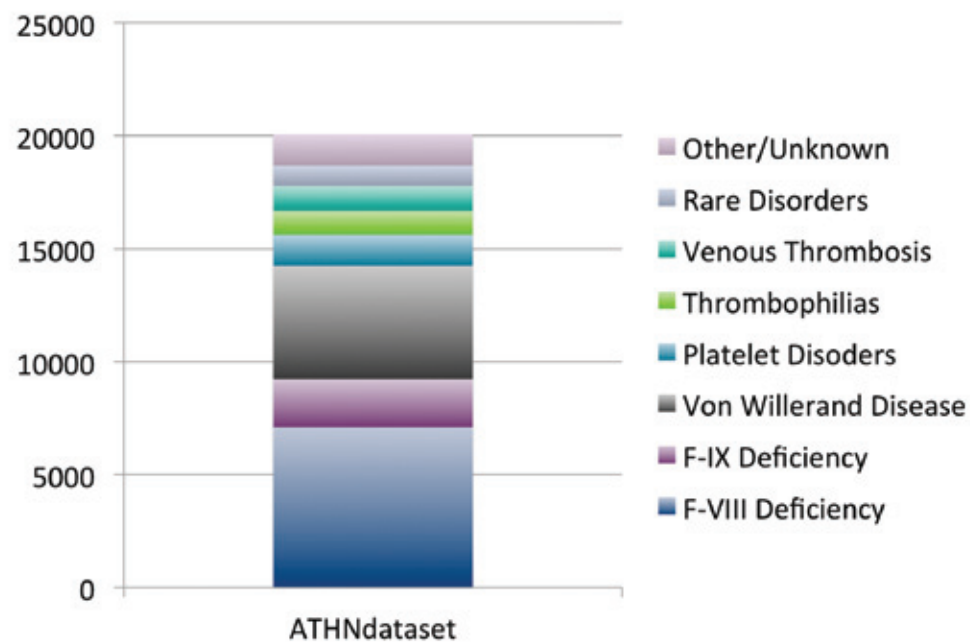
ATHNdataset	AGE AND SEX BY REGION AND TOTAL U.S. (AS OF DECEMBER 31, 2013)								
	REGION								TOTAL
	New England	Mid-Atlantic	Southeast	Great Lakes	Northern States	Great Plains	Mountain States	Western States	US
BY SEX									
Female	932	326	561	3416	379	519	251	625	7009
	34.6%	22.5%	23.6%	48.1%	31.9%	28.0%	19.8%	29.5%	34.9%
Male	1764	1123	1816	3690	810	1336	1017	1497	13053
	65.4%	77.5%	76.4%	51.9%	68.1%	72.0%	80.2%	70.5%	65.1%
Total	2696	1449	2377	7106	1189	1855	1268	2122	20062
	100%	100%	100%	100%	100%	100%	100%	100%	100%
BY AGE									
0-2 years	83	47	85	237	37	117	58	62	726
	3.1%	3.2%	3.6%	3.3%	3.1%	6.3%	4.6%	2.9%	3.6%
3-12 years	722	355	651	1506	245	650	319	647	5095
	26.8%	24.5%	27.4%	21.2%	20.6%	35.0%	25.2%	30.5%	25.4%
13-18 years	573	280	488	1289	167	430	220	502	3949
	21.3%	19.3%	20.5%	18.1%	14.0%	23.2%	17.4%	23.7%	19.7%
19-29 years	594	276	507	1284	266	268	259	450	3904
	22.0%	19.0%	21.3%	18.1%	22.4%	14.4%	20.4%	21.2%	19.5%
30-49 years	415	272	374	1309	292	205	250	275	3392
	15.4%	18.8%	15.7%	18.4%	24.6%	11.1%	19.7%	13.0%	16.9%
50-74 years	268	199	248	1327	164	170	148	171	2695
	9.9%	13.7%	10.4%	18.7%	13.8%	9.2%	11.7%	8.1%	13.4%
75+ years	41	20	24	154	18	15	14	15	301
	1.5%	1.4%	1.0%	2.2%	1.5%	0.8%	1.1%	0.7%	1.5%
Total	2696	1449	2377	7106	1189	1855	1268	2122	20062
	100%	100%	100%	100%	100%	100%	100%	100%	100%

ATHNdataset — Demographic Profile

ATHNdataset	RACE AND ETHNICITY BY REGION AND TOTAL U.S. (AS OF DECEMBER 31, 2013)								
	REGION								TOTAL
	New England	Mid-Atlantic	Southeast	Great Lakes	Northern States	Great Plains	Mountain States	Western States	US
BY RACE									
White	2301	1176	1845	6143	1069	1608	1177	1702	17021
	85.9%	81.2%	77.7%	86.5%	90.0%	87.1%	92.9%	80.4%	85.0%
Black	244	235	478	855	83	191	37	93	2216
	9.1%	16.2%	20.1%	12.0%	7.0%	10.3%	2.9%	4.4%	11.1%
Asian	96	35	34	94	24	37	20	206	546
	3.6%	2.4%	1.4%	1.3%	2.0%	2.0%	1.6%	9.7%	2.7%
Native Hawaiian or Other Pacific Islander	6							75	106
	0.2%							3.5%	0.5%
American Indian or Alaska Native	31	0	14	8	8	6	28	41	136
	1.2%	0.0%	0.6%	0.1%	0.7%	0.3%	2.2%	1.9%	0.7%
Total Known	2678	1448	2376	7104	1188	1847	1267	2117	20025
	100%	100%	100%	100%	100%	100%	100%	100%	100%
Unknown	18	1	1	2	1	8	1	5	37
	0.7%	0.1%	0.0%	0.0%	0.1%	0.4%	0.1%	0.2%	0.2%
Total	2696	1449	2377	7106	1189	1855	1268	2122	20062
	100%	100%	100%	100%	100%	100%	100%	100%	100%
BY ETHNICITY									
Hispanic or Latino	336	85	238	264	94	296	231	811	2355
	12.5%	5.9%	10.0%	3.7%	7.9%	16.0%	18.2%	38.3%	11.8%
Non Hispanic or Latino	2342	1363	2138	6840	1094	1551	1036	1306	17670
	87.5%	94.1%	90.0%	96.3%	92.1%	84.0%	81.8%	61.7%	88.2%
Total Known	2678	1448	2376	7104	1188	1847	1267	2117	20025
	100%	100%	100%	100%	100%	100%	100%	100%	100%
Unknown	18	1	1	2	1	8	1	5	37
	0.7%	0.1%	0.0%	0.0%	0.1%	0.4%	0.1%	0.2%	0.2%
Total	2696	1449	2377	7106	1189	1855	1268	2122	20062
	100%	100%	100%	100%	100%	100%	100%	100%	100%

ATHNdataset — Bleeding and Clotting Disorders Within the Population

ATHNdataset	PATIENT AUTHORIZATIONS BY PRIMARY DIAGNOSIS TOTAL U.S. (AS OF DECEMBER 31, 2013)	
PRIMARY BLEEDING OR CLOTTING DISORDER	TOTAL U.S. ATHNdataset CASES	% ATHNdataset
Factor VIII Deficiency	7098	35.38%
Factor IX Deficiency	2137	10.65%
Von Willebrand Disease	4998	24.91%
Rare Disorders	900	4.49%
Platelet Disorders	1375	6.85%
Thrombophilias	1074	5.35%
Venous Thrombosis	1105	5.51%
Other/Unknown	1375	6.85%
TOTAL	20062	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, 2013)							
DISEASE SEVERITY		REGION							TOTAL
	New England	Mid-Atlantic	Southeast	Great Lakes	Northern States	Great Plains	Mountain States	Western States	US
Mild	255	165	274	356	128	228	176	295	1877
	25.6%	23.2%	23.5%	29.8%	28.0%	29.6%	24.6%	31.3%	27.0%
Moderate	173	127	209	193	77	114	130	142	1165
	17.4%	17.9%	17.9%	16.2%	16.8%	14.8%	18.2%	15.1%	16.8%
Severe	567	419	685	644	252	427	410	506	3910
	57.0%	58.9%	58.6%	54.0%	55.1%	55.5%	57.3%	53.7%	56.2%
TOTAL	995	711	1168	1193	457	769	716	943	6952
	100%	100%	100%	100%	100%	100%	100%	100%	100%
Unknown Severity	42	26	8	31	4	3	11	21	146
	4.1%	3.5%	0.7%	2.5%	0.9%	0.4%	1.5%	2.2%	2.1%
TOTAL	1037	737	1176	1224	461	772	727	964	7098
	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, 2013)						
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		316	153		475
	0.0%	0.0%	0.0%		24.4%	20.4%		6.7%
No HIV	308	1901	1188	1583	979	597	67	6623
	100.0%	100.0%	100.0%	99.7%	75.6%	79.6%	97.1%	93.3%
TOTAL	308	1901	1188	1587	1295	750	69	7098
	100%	100%	100%	100%	100%	100%	100%	100%
HEPATITIS CO-MORBIDITY								
Hepatitis C	0			126	714	415	17	1277
	0.0%			7.9%	55.1%	55.3%	24.6%	18.0%
No Hepatitis C	308	1900	1184	1461	581	335	52	5821
	100.0%	99.9%	99.7%	92.1%	44.9%	44.7%	75.4%	82.0%
TOTAL	308	1901	1188	1587	1295	750	69	7098
	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, 2013)							
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	0	25	23	19	8	8	0	83
		0.0%	5.4%	7.0%	5.4%	2.6%	2.5%	0.0%	4.4%
	Not Prescribed/Unk	62	440	306	335	305	306	40	1794
		100.0%	94.6%	93.0%	94.6%	97.4%	97.5%	100.0%	95.6%
	Total Mild	62	465	329	354	313	314	40	1877
		100.0%	100.0%	100.0%	100.0%	100.0%	100.0%	100.0%	100.0%
Moderate	Prescribed	12	110	71	57	29	8	0	287
		18.8%	36.5%	33.6%	24.4%	14.6%	5.5%	0.0%	24.6%
	Not Prescribed/Unk	52	191	140	177	170	137	11	878
		81.3%	63.5%	66.4%	75.6%	85.4%	94.5%	100.0%	75.4%
	Total Moderate	64	301	211	234	199	145	11	1165
		100.0%	100.0%	100.0%	100.0%	100.0%	100.0%	100.0%	100.0%
Severe	Prescribed	58	777	478	533	274	66		2187
		34.9%	70.2%	75.3%	54.9%	36.5%	24.4%		55.9%
	Not Prescribed/Unk	108	330	157	438	476	205	9	1723
		65.1%	29.8%	24.7%	45.1%	63.5%	75.6%	90.0%	44.1%
	Total Severe	166	1107	635	971	750	271	10	3910
		100.0%	100.0%	100.0%	100.0%	100.0%	100.0%	100.0%	100.0%
Unknown	Prescribed	3	7	0	6	1	1	0	18
		18.8%	25.0%	0.0%	21.4%	3.0%	5.0%	0.0%	12.3%
	Not Prescribed/Unk	13	21	13	22	32	19	8	128
		81.3%	75.0%	100.0%	78.6%	97.0%	95.0%	100.0%	87.7%
	Total Unknown	16	28	13	28	33	20	8	146
		100.0%	100.0%	100.0%	100.0%	100.0%	100.0%	100.0%	100.0%
All Severities	Total All	308	1901	1188	1587	1295	750	69	7098
		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, 2013)								
PAYER CATEGORY		REGION							TOTAL	
	New England	Mid-Atlantic	Southeast	Great Lakes	Northern States	Great Plains	Mountain States	Western States	US	
Medicaid	118	82	91	153	56	53	58	84	695	
	25.3%	24.2%	20.2%	28.7%	23.1%	26.6%	28.0%	16.6%	23.6%	
Medicare	26	50	41	49	31	16	16	25	254	
	5.6%	14.7%	9.1%	9.2%	12.8%	8.0%	7.7%	4.9%	8.6%	
Private	313	195	251	289	136	109	129	186	1608	
	67.0%	57.5%	55.8%	54.1%	56.2%	54.8%	62.3%	36.7%	54.6%	
Uninsured			56		10	11		113	198	
			12.4%		4.1%	5.5%		22.3%	6.7%	
Other	7	11	11	41	9	10		99	190	
	1.5%	3.2%	2.4%	7.7%	3.7%	5.0%		19.5%	6.5%	
TOTAL	467	339	450	534	242	199	207	507	2945	
	100%	100%	100%	100%	100%	100%	100%	100%	100%	
Unknown	570	398	726	690	219	573	520	457	4153	
	55.0%	54.0%	61.7%	56.4%	47.5%	74.2%	71.5%	47.4%	58.5%	
TOTAL	1037	737	1176	1224	461	772	727	964	7098	
	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, 2013)							
DISEASE SEVERITY		REGION							TOTAL
	New England	Mid-Atlantic	Southeast	Great Lakes	Northern States	Great Plains	Mountain States	Western States	US
Mild	63	52	101	186	38	68	54	48	610
	28.3%	29.1%	33.6%	28.5%	24.1%	32.7%	31.0%	26.1%	29.3%
Moderate	63	66	90	330	66	86	62	31	794
	28.3%	36.9%	29.9%	50.5%	41.8%	41.3%	35.6%	16.8%	38.2%
Severe	97	61	110	137	54	54	58	105	676
	43.5%	34.1%	36.5%	21.0%	34.2%	26.0%	33.3%	57.1%	32.5%
TOTAL	223	179	301	653	158	208	174	184	2080
	100%	100%	100%	100%	100%	100%	100%	100%	100%
Unknown Severity	12	7	5	19	2	1	2	9	57
	5.1%	3.8%	1.6%	2.8%	1.3%	0.5%	1.1%	4.7%	2.7%
TOTAL	235	186	306	672	160	209	176	193	2137
	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, 2013)						
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	21	0	68
	0.0%	0.0%	0.0%		10.8%	7.0%	0.0%	3.2%
No HIV	83	538	326	440	372	281	29	2069
	100.0%	100.0%	100.0%	99.5%	89.2%	93.0%	100.0%	96.8%
TOTAL	83	538	326	442	417	302	29	2137
	100%	100%	100%	100%	100%	100%	100%	100%
HEPATITIS C CO-MORBIDITY								
Hepatitis C	0			38	189	137	7	374
	0.0%			8.6%	45.3%	45.4%	24.1%	17.5%
No Hepatitis C	83	537	324	404	228	165	22	1763
	100.0%	99.8%	99.4%	91.4%	54.7%	54.6%	75.9%	82.5%
TOTAL	83	538	326	442	417	302	29	2137
	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, 2013)							
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	0	11						24
		0.0%	7.0%						3.9%
	Not Prescribed/Unk	15	146	101	122	92	99	11	586
		100.0%	93.0%	96.2%	98.4%	96.8%	97.1%	91.7%	96.1%
	Total Mild	15	157	105	124	95	102	12	610
		100.0%	100.0%	100.0%	100.0%	100.0%	100.0%	100.0%	100.0%
Moderate	Prescribed		32	23	12	10		0	84
			15.8%	20.0%	8.3%	6.1%		0.0%	10.6%
	Not Prescribed/Unk	35	171	92	132	155	113	12	710
		94.6%	84.2%	80.0%	91.7%	93.9%	95.8%	100.0%	89.4%
	Total Moderate	37	203	115	144	165	118	12	794
		100.0%	100.0%	100.0%	100.0%	100.0%	100.0%	100.0%	100.0%
Severe	Prescribed	11	110	64	70	46	22		324
		45.8%	65.1%	64.0%	42.7%	32.6%	30.1%		47.9%
	Not Prescribed/Unk	13	59	36	94	95	51		352
		54.2%	34.9%	36.0%	57.3%	67.4%	69.9%		52.1%
	Total Severe	24	169	100	164	141	73		676
		100.0%	100.0%	100.0%	100.0%	100.0%	100.0%		100.0%
Unknown	Prescribed	0	0	1	2	0	1	0	4
		0.0%	0.0%	16.7%	20.0%	0.0%	11.1%	0.0%	7.0%
	Not Prescribed/Unk	7	9	5	8	16	8	0	53
		100.0%	100.0%	83.3%	80.0%	100.0%	88.9%	0.0%	93.0%
	Total Unknown	7	9	6	10	16	9	0	57
		100.0%	100.0%	100.0%	100.0%	100.0%	100.0%	0.0%	100.0%
All Severities	Total All	83	538	326	442	417	302	29	2137
		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, 2013)								
PAYER CATEGORY		REGION							TOTAL	
	New England	Mid-Atlantic	Southeast	Great Lakes	Northern States	Great Plains	Mountain States	Western States	US	
Medicaid	25	21	18	75	16	19		16	195	
	25.5%	27.6%	18.6%	28.4%	19.8%	33.9%		16.0%	24.0%	
Medicare	6	11	8	17	14	3			69	
	6.1%	14.5%	8.2%	6.4%	17.3%	5.4%			8.5%	
Private	66	39	50	118	46	26	26	40	411	
	67.3%	51.3%	51.5%	44.7%	56.8%	46.4%	66.7%	40.0%	50.7%	
Uninsured	0	3	8	13	0	0	1	28	53	
	0.0%	3.9%	8.2%	4.9%	0.0%	0.0%	2.6%	28.0%	6.5%	
Other			13	41		8		11	83	
			13.4%	15.5%		14.3%		11.0%	10.2%	
TOTAL	98	76	97	264	81	56	39	100	811	
	100%	100%	100%	100%	100%	100%	100%	100%	100%	
Unknown	137	110	209	408	79	153	137	93	1326	
	58.3%	59.1%	68.3%	60.7%	49.4%	73.2%	77.8%	48.2%	62.0%	
TOTAL	235	186	306	672	160	209	176	193	2137	
	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, 2013)								
		REGION							TOTAL	
		New England	Mid-Atlantic	Southeast	Great Lakes	Northern States	Great Plains	Mountain States	Western States	US
BY SEX										
Female	564	241	374	1077	214	204	113	356	3143	
	65.4%	61.3%	60.1%	66.1%	61.3%	56.0%	63.8%	59.2%	62.9%	
Male	299	152	248	552	135	160	64	245	1855	
	34.6%	38.7%	39.9%	33.9%	38.7%	44.0%	36.2%	40.8%	37.1%	
Total	863	393	622	1629	349	364	177	601	4998	
	100%	100%	100%	100%	100%	100%	100%	100%	100%	
BY AGE										
0-2 years	16	6	6	19	5				63	
	1.9%	1.5%	1.0%	1.2%	1.4%				1.3%	
3-12 years	264	105	175	416	93	136	50	207	1446	
	30.6%	26.7%	28.1%	25.5%	26.6%	37.4%	28.2%	34.4%	28.9%	
13-18 years	262	99	168	390	76	128	40	192	1355	
	30.4%	25.2%	27.0%	23.9%	21.8%	35.2%	22.6%	31.9%	27.1%	
19-29 years	178	85	135	351	67	53	31	115	1015	
	20.6%	21.6%	21.7%	21.5%	19.2%	14.6%	17.5%	19.1%	20.3%	
30-49 years	79	47	87	243	68	26	27	48	625	
	9.2%	12.0%	14.0%	14.9%	19.5%	7.1%	15.3%	8.0%	12.5%	
50-74 years	58	44	42	191	34	17	22	33	441	
	6.7%	11.2%	6.8%	11.7%	9.7%	4.7%	12.4%	5.5%	8.8%	
75+ years	6	7	9	19	6	0			53	
	0.7%	1.8%	1.4%	1.2%	1.7%	0.0%			1.1%	
Total	863	393	622	1629	349	364	177	601	4998	
	100%	100%	100%	100%	100%	100%	100%	100%	100%	

Von Willebrand Disease — Demographic Profile

VON WILLEBRAND DISEASE	RACE AND ETHNICITY BY REGION AND TOTAL U.S. (AS OF DECEMBER 31, 2013)								
	REGION								TOTAL
	New England	Mid-Atlantic	Southeast	Great Lakes	Northern States	Great Plains	Mountain States	Western States	US
BY RACE									
White	778	330	516	1490	330	329	172	506	4451
	90.2%	84.0%	83.0%	91.5%	94.6%	90.4%	97.2%	84.2%	89.1%
Black	50	52	92	114	12	30	0	10	360
	5.8%	13.2%	14.8%	7.0%	3.4%	8.2%	0.0%	1.7%	7.2%
Asian	14	11	12	23				52	122
	1.6%	2.8%	1.9%	1.4%				8.7%	2.4%
Native Hawaiian or Other Pacific Islander		0	0		0	0	0	27	33
		0.0%	0.0%		0.0%	0.0%	0.0%	4.5%	0.7%
American Indian or Alaska Native	16	0						6	32
	1.9%	0.0%						1.0%	0.6%
Total	863	393	622	1629	349	364	177	601	4998
	100%	100%	100%	100%	100%	100%	100%	100%	100%
BY ETHNICITY									
Hispanic or Latino	100	30	72	76	25	48	25	224	600
	11.6%	7.6%	11.6%	4.7%	7.2%	13.2%	14.1%	37.3%	12.0%
Non Hispanic or Latino	763	363	550	1553	324	316	152	377	4398
	88.4%	92.4%	88.4%	95.3%	92.8%	86.8%	85.9%	62.7%	88.0%
Total	863	393	622	1629	349	364	177	601	4998
	100%	100%	100%	100%	100%	100%	100%	100%	100%

Von Willebrand Disease — Disease Types

VON WILLEBRAND DISEASE		DISEASE TYPES BY REGION AND TOTAL U.S. (AS OF DECEMBER 31, 2013)							
VWD TYPE	REGION								TOTAL
	New England	Mid-Atlantic	Southeast	Great Lakes	Northern States	Great Plains	Mountain States	Western States	US
VWD, Type 1	753	326	448	1387	271	268	114	509	4076
	89.0%	85.8%	74.9%	86.8%	79.2%	77.9%	69.1%	86.6%	83.9%
VWD, Type 2	68	38	115	168	52	61	39	60	601
	8.0%	10.0%	19.2%	10.5%	15.2%	17.7%	23.6%	10.2%	12.4%
VWD, Type 3	25	16	35	43	19	15	12	19	184
	3.0%	4.2%	5.9%	2.7%	5.6%	4.4%	7.3%	3.2%	3.8%
TOTAL w Type Specified	846	380	598	1598	342	344	165	588	4861
	100.0%	100.0%	100.0%	100.0%	100.0%	100.0%	100.0%	100.0%	100.0%
VWD, Type Other/Unknown	17	13	24	31	7	20	12	13	137
	2.0%	3.3%	3.9%	1.9%	2.0%	5.5%	6.8%	2.2%	2.7%
TOTAL	863	393	622	1629	349	364	177	601	4998
	100%	100%	100%	100%	100%	100%	100%	100%	100%

Von Willebrand Disease — HIV and Hepatitis C

VON WILLEBRAND DISEASE		HIV AND HEPATITIS C (HCV) BY AGE GROUP (AS OF DECEMBER 31, 2013)						
AGE								TOTAL
HIV CO-MORBIDITY								
	0-2 years	3-12 years	13-18 years	19-29 years	30-49 years	50-74 years	75+ years	US
HIV	0	0	0				0	8
	0.0%	0.0%	0.0%				0.0%	0.2%
No HIV	63	1446	1355	1014	620	439	53	4990
	100.0%	100.0%	100.0%	99.9%	99.2%	99.5%	100.0%	99.8%
TOTAL	63	1446	1355	1015	625	441	53	4998
	100%	100%	100%	100%	100%	100%	100%	100%
HEPATITIS C CO-MORBIDITY								
Hepatitis C	0	0	0		33	43		85
	0.0%	0.0%	0.0%		5.3%	9.8%		1.7%
No Hepatitis C	63	1446	1355	1011	592	398	48	4913
	100.0%	100.0%	100.0%	99.6%	94.7%	90.2%	90.6%	98.3%
TOTAL	63	1446	1355	1015	625	441	53	4998
	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)	Deficiency of Von Willebrand factor
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)

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)
F-VIII Replacement: Human Plasma Derived Factor VIII Concentrates (*rich in Von Willebrand factor)	Alphanate (Grifols)*, Humate-P (CSL)*, Koate DVI* (Kedrion), Hemophil-M (Baxter), Monoclate-P (CSL), Monarc-M
F-IX Replacement: Recombinant Factor IX Concentrates	BeneFIX (Pfizer) - non-VWD factor containing products
F-IX Replacement: Human Plasma Derived Factor IX Concentrates	AlphaNine SD (Grifols), MonoNine (CSL), Profilnine SD (Grifols), factor IX complex
VWD Treatments: VWF-Containing Factor VIII Concentrates (Human Plasma Derived)	Alphanate (Grifols), Humate-P (CSL), Wilate (Octapharma)
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 by Region

LIST OF PARTICIPATING HTCS BY REGION			
Region	ATHN Affiliate	City	State
I	New England Hemophilia Center/UMASS Memorial Hospital	Worcester	MA
I	Yale Hemophilia Center	New Haven	CT
I	Maine Hemophilia and Thrombosis Center	Scarborough	ME
I	Dartmouth-Hitchcock Comprehensive Hemophilia and Thrombosis Center	Lebanon	NH
I	Rhode Island Hemostasis and Thrombosis Center / Rhode Island Hospital	Providence	RI
I	Vermont Regional Hemophilia Center	Burlington	VT
I	University of Connecticut Health Center	Farmington	CT
I	Massachusetts General Hospital	Boston	MA
I	Boston Hemophilia Center at Boston Children's Hospital	Boston	MA
II	Weill Cornell Medical College	New York	NY
II	Puerto Rico Hemophilia Treatment Center	San Juan	PR
II	Nadeene Brunini Comprehensive Hemophilia Care Center St. Michael's Medical Center	Newark	NJ
II	Mary M. Gooley Hemophilia Center, Inc.	Rochester	NY
II	Hemophilia Center of Western New York, Inc.	Buffalo	NY
II	Albany Regional Hemophilia and vonWillebrand Treatment Center	Albany	NY
II	Long Island Jewish Medical Center Comprehensive Hemophilia Center	New Hyde Park	NY
II	Mount Sinai Regional Comprehensive Hemophilia Treatment Center	New York	NY
II	Newark Beth Israel Medical Center - Hemophilia Center	Newark	NJ
II	SUNY Upstate Medical University - Adult and Pediatric Program	Syracuse	NY
III	Children's National Hemophilia Center	Washington	DC
III	Georgetown University	Washington	DC
III	University of Virginia	Charlottesville	VA

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	Children's Hospital of the King's Daughters, Inc.	Norfolk	VA
III	Hemophilia Center of Central Pennsylvania / Penn State Milton S. Hershey Medical Center	Hershey	PA
III	Lehigh Valley Hospital	Allentown	PA
III	Hemophilia Center of Western Pennsylvania	Pittsburgh	PA
III	West Virginia University School of Medicine Hemophilia Treatment Center	Morgantown	WV
III	Charleston Area Medical Center	Charleston	WV
III	The Johns Hopkins University Hemophilia Treatment Center	Baltimore	MD
III	Penn Comprehensive Hemophilia and Thrombophilia Program / Hospital of the University of Pennsylvania	Philadelphia	PA
III	Children's Hospital of Philadelphia (CHOP)	Philadelphia	PA
IV - North	Wake Forest University Health Sciences	Winston-Salem	NC
IV - North	University of Kentucky Hemophilia Treatment Center	Lexington	KY
IV - North	The Brody School of Medicine at East Carolina University	Greenville	NC
IV - North	Palmetto Health	Columbia	SC
IV - North	East Tennessee Comprehensive Hemophilia Center	Knoxville	TN
IV - North	Vanderbilt University Medical Center	Nashville	TN
IV - North	St. Jude Children's Research Hospital	Memphis	TN
IV - North	Harold R. Roberts Comprehensive Hemophilia Diagnostic and Treatment Center / University of North Carolina at Chapel Hill	Chapel Hill	NC
IV - North	Duke University Health System	Durham	NC
IV - North	University of Louisville Hemophilia Treatment Center	Louisville	KY
IV - South	Nemours Children's Clinic - Jacksonville / The Nemours Foundation	Jacksonville	FL

Definitions — Participating HTC by Region

LIST OF PARTICIPATING HTCS BY REGION			
Region	ATHN Affiliate	City	State
IV - South	University of Miami	Miami	FL
IV - South	Backus Children's Hospital	Savannah	GA
IV - South	Georgia Regents University Adult Hemophilia Treatment Center	Augusta	GA
IV - South	University of Mississippi Medical Center	Jackson	MS
IV - South	St. Joseph's Children's Hospital Pediatric Hemophilia Program	Tampa	FL
IV - South	Georgia Regents University Pediatric Hemophilia Treatment Center	Augusta	GA
IV - South	All Children's Hospital	St. Petersburg	FL
IV - South	Emory / Children's Healthcare of Atlanta Comprehensive Hemophilia Program	Atlanta	GA
IV - South	University of Alabama at Birmingham	Birmingham	AL
V - East	Children's Hospital of Michigan/Hemostasis and Thrombosis Center	Detroit	MI
V - East	Henry Ford Health System Hemophilia and Thrombosis Treatment Center	Detroit	MI
V - East	Northern Regional Bleeding Disorder Center at Munson Medical Center	Traverse City	MI
V - East	West Michigan Pediatric at Bronson	Kalamazoo	MI
V - East	Hemophilia Clinic - West Michigan Cancer Center	Kalamazoo	MI
V - East	Eastern Michigan Hemophilia Treatment Center Hurley Medical Center	Flint	MI
V - East	DeVos Children's Coagulation Disorders Program	Grand Rapids	MI
V - East	Ohio State University Medical Center/Hemophilia Treatment Center	Columbus	OH
V - East	Cincinnati Children's Hospital Medical Center / Hemophilia and Thrombosis Center	Cincinnati	OH
V - East	University of Cincinnati Medical Center Hemophilia Treatment Center	Cincinnati	OH
V - East	Nationwide Children's Hospital	Columbus	OH
V - East	Northwest Ohio Hemophilia Treatment Center at the Toledo Hospital	Toledo	OH
V - East	West Central Ohio Hemophilia Center	Dayton	OH

Definitions — Participating HTC by Region

LIST OF PARTICIPATING HTCS BY REGION			
Region	ATHN Affiliate	City	State
V - East	Indiana Hemophilia and Thrombosis Center	Indianapolis	IN
V - East	Michigan State University Center for Bleeding Disorders & Clotting Disorders	East Lansing	MI
V - East	University Hospitals Case Medical Center	Cleveland	OH
V - East	Children's Hospital Medical Center of Akron	Akron	OH
V - East	Detroit Receiving Hospital and University Medical Center / Comprehensive Center for Bleeding Disorders and Thrombosis	Detroit	MI
V - East	University of Michigan Hemophilia and Coagulation Disorders Program	Ann Arbor	MI
V - West	Northwestern Center for Bleeding Disorders	Chicago	IL
V - West	Stroger Hospital of Cook County (Adults)	Chicago	IL
V - West	Ann and Robrt H. Lurie Children's Memorial Hospital	Chicago	IL
V - West	University of Minnesota Medical Center, Fairview	Minneapolis	MN
V - West	North Dakota Hemophilia and Thrombophilia Treatment Center	Fargo	ND
V - West	Hemophilia Outreach Center	Green Bay	WI
V - West	Gundersen Lutheran Administrative Services, Inc.	LaCrosse	WI
V - West	UWHC Comprehensive Program for Bleeding Disorders	Madison	WI
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 (Pediatrics)	Chicago	IL
V - West	Bleeding and Clotting Disorders Institute	Peoria	IL
Great Plains	Louisiana Center for Bleeding and Clotting Disorders/Tulane University Health Science Center	New Orleans	LA
Great Plains	Arkansas Center for Bleeding Disorders	Little Rock	AR
Great Plains	Oklahoma Center for Bleeding Disorders	Oklahoma City	OK

Definitions — Participating HTC by Region

LIST OF PARTICIPATING HTCS BY REGION			
Region	ATHN Affiliate	City	State
Great Plains	Fort Worth Bleeding Disorders Program	Fort Worth	TX
Great Plains	Texas Children's Hemophilia & Thrombosis Center/Baylor College of Medicine	Houston	TX
Great Plains	Iowa Hemophilia and Thrombosis Center	Iowa City	IA
Great Plains	Children's Mercy Hospital	Kansas City	MO
Great Plains	The John Bouhasin Center for Children with Bleeding Disorders	St. Louis	MO
Great Plains	University of Nebraska Medical Center	Omaha	NE
Great Plains	North Texas Hemophilia and Thrombosis Program - Adult and Pediatric Programs / Center for Cancer & Blood Disorders	Dallas	TX
Great Plains	Children's Medical Center of Dallas	Dallas	TX
VIII	University of Colorado Denver Hemophilia and Thrombosis Center	Aurora	CO
VIII	University of New Mexico, Ted R. Montoya Hemophilia & Thrombosis Program	Albuquerque	NM
VIII	Arizona Hemophilia and Thrombosis Center / University of Arizona Health Sciences Center	Tucson	AZ
VIII	Arizona Hemophilia and Thrombosis Center at Phoenix Children's Hospital	Phoenix	AZ
VIII	Intermountain Hemophilia and Thrombosis Center	Salt Lake City	UT
IX	Childrens Hospital Los Angeles	Los Angeles	CA
IX	University of California, San Diego Medical Center	San Diego	CA
IX	Stanford University	Palo Alto	CA
IX	Kapiolani Medical Center for Women and Children	Honolulu	HI
IX	University of California at Davis Hemophilia Treatment Center	Sacramento	CA

Definitions — Participating HTC by Region

LIST OF PARTICIPATING HTCS BY REGION			
IX	University of California, San Francisco Hemophilia & Thrombosis Center	San Francisco	CA
IX	Orthopaedic Hospital	Los Angeles	CA
IX	Rady Children's Hospital San Diego	San Diego	CA
IX	Center for Comprehensive Care and Diagnosis of Inherited Blood Disorders	Orange	CA
IX	Children's Hospital Research Center Oakland	Oakland	CA
IX	City of Hope	Duarte	CA
IX	Children's Hospital of Central California	Madera	CA
IX	Hemophilia Treatment Center of Nevada	Las Vegas	NV
IX	Children's Hospital of Orange County	Orange	CA
X	Seattle Children's Hospital	Seattle	WA
X	Puget Sound Blood Center & Program	Seattle	WA
X	Oregon Health and Science University	Portland	OR
X	St. Luke's Hemophilia Center	Boise	ID
X	Providence Sacred Heart Medical Center and Children's Hospital	Spokane	WA



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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.*