



ATHN Research Report Brief

ATHNdataset — September 30, 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



Industry Supporters

CSL BEHRING
BIOGEN IDEC HEMOPHILIA

Table of Contents

ATHNdataset

Growth of the ATHNdataset	6
Patient Accrual Over Time	6
ATHNdataset Compared to 2010 Hemophilia Data Set	7
Demographic Profile	8
Age and Sex By Region and Total US	8
Race and Ethnicity By Region and Total US	9
Bleeding and Clotting Disorders Within the Population	10
Patient Authorizations By Primary Diagnosis Total US	10

Factor VIII Deficiency

Bleeding Disorder Disease Severity	12
By Region and Total US	12
HIV and Hepatitis C	13
By Age Group	13
Continuous Prophylaxis (Prescribed)	14
By Bleeding Disorder Disease Severity and Age Group	14
Payer Profile	15
By Region and Total US	15

Factor IX Deficiency

Bleeding Disorder Disease Severity	18
By Region and Total US	18
HIV and Hepatitis C	19
By Age Group	19
Continuous Prophylaxis (Prescribed)	20
By Bleeding Disorder Disease Severity and Age Group	20
Payer Profile	21
By Region and Total US	21

Von Willebrand Disease

Demographic Profile	24
Age and Sex By Region and Total US.....	24
Race and Ethnicity By Region and Total US.....	25
Disease Types	26
Types By Region and Total US.....	26
HIV and Hepatitis C	27
By Age Group.....	27

Definitions

Definition of Regions	30
Definition of Clinical Content	31
Definition of Medications	32
Definition of Patient Sample	33

NOTE

BLACKENED CELLS INDICATE SMALL SIZED CELLS,
DEFINED AS 5 CASES OR LESS

This Research Report is prepared using standardized data collected for the ATHNdataset, a HIPAA compliant limited data set under the stewardship of the American Thrombosis and Hemostasis Network (ATHN).

Data collection is a voluntary effort by clinical teams and data managers at federally funded Hemophilia Treatment Centers (HTCs) across the U.S. The project is open to all ATHN Affiliated centers. Patients are asked to opt-in. At the time of reporting, not all patients receiving care at participating centers have been asked to join, and not all have agreed to participate. The ATHNdataset is extracted from the patient's medical record. It is not the official medical record of the patient. Unknown or missing data indicates that the data was not reported to the ATHNdataset during the reporting period.

Participating centers use reasonable efforts to ensure the accuracy of the data. System design with structured data entry, self-audits and HTC re-use of the information in fulfillment of government funded projects further help to drive quality of the data. However, ATHN makes no warranty or representation that the information will be adequate or satisfactory for particular use.

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

ATHNdataset

ATHNdataset — Growth of the ATHNdataset

ATHNdataset

PATIENT ACCRUAL OVER TIME (AS OF SEPTEMBER 30, 2013)

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

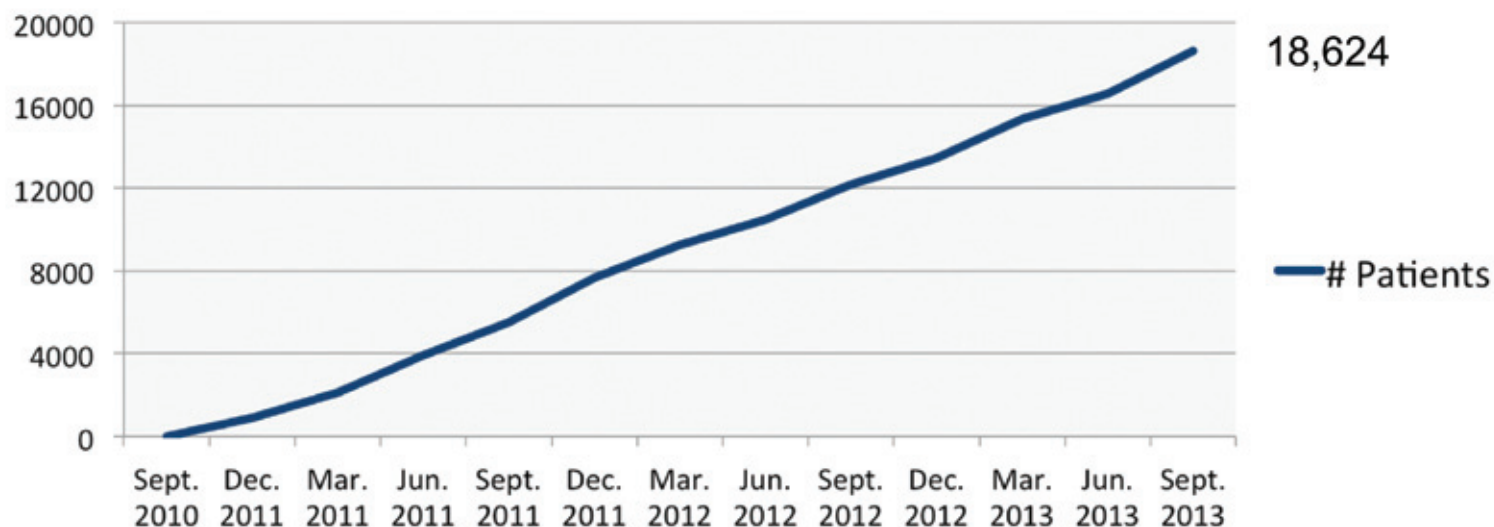
Participating Hemophilia Treatment Centers across the U.S. abstract data from the medical record.

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 18,624 patients.



ATHNdataset — Demographic Profile

ATHNdataset

ATHNdataset COMPARED TO 2010 HEMOPHILIA DATA SET (AS OF SEPTEMBER 30, 2013)

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

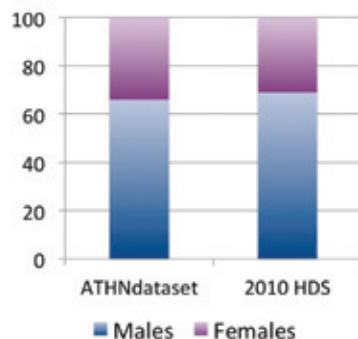
The 2010 Hemophilia Data Set included 32,612 active patients with Factor VIII Deficiency, Factor IX Deficiency, Von Willebrand Disease and other factor deficiencies.

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%

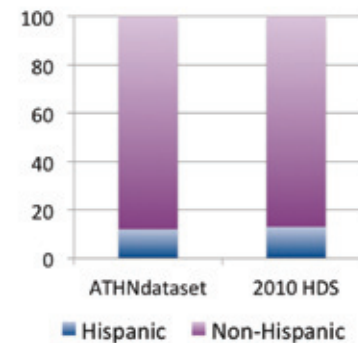


ETHNICITY COMPARISON

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

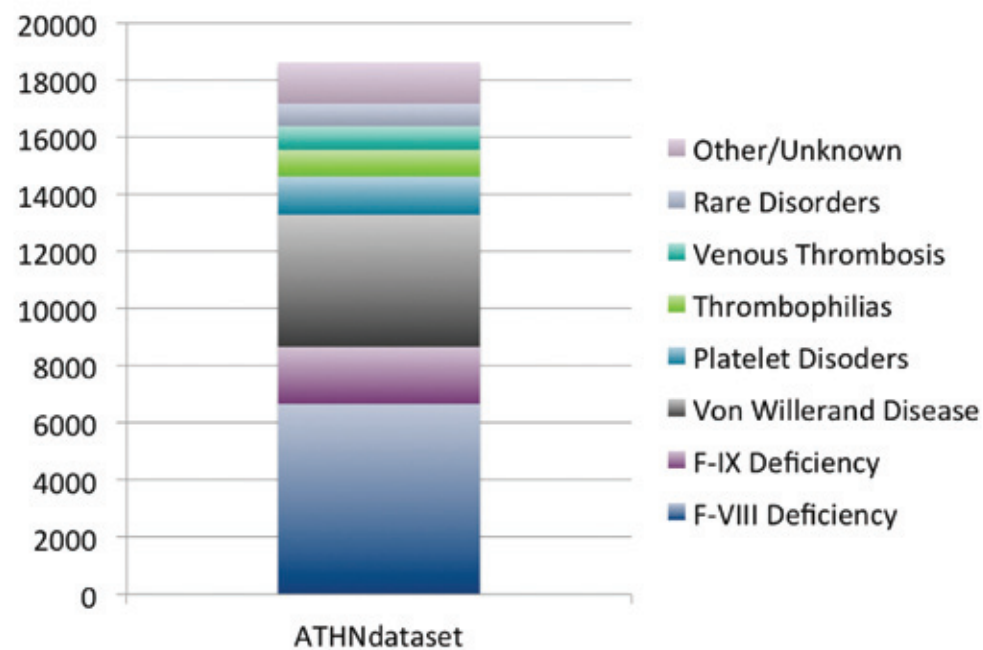
ATHNdataset	AGE AND SEX BY REGION AND TOTAL U.S. (AS OF SEPTEMBER 30, 2013)								
	REGION								TOTAL
	New England	Mid-Atlantic	Southeast	Great Lakes	Northern States	Great Plains	Mountain States	Western States	US
BY SEX									
Female	845	294	526	3212	335	444	199	579	6434
	34.0%	22.1%	23.5%	47.8%	30.8%	26.8%	18.1%	28.9%	34.5%
Male	1641	1038	1712	3508	754	1212	899	1426	12190
	66.0%	77.9%	76.5%	52.2%	69.2%	73.2%	81.9%	71.1%	65.5%
Total	2486	1332	2238	6720	1089	1656	1098	2005	18624
	100%	100%	100%	100%	100%	100%	100%	100%	100%
BY AGE									
0-2 years	81	42	83	232	34	117	49	55	693
	3.3%	3.2%	3.7%	3.5%	3.1%	7.1%	4.5%	2.7%	3.7%
3-12 years	670	346	618	1468	227	598	287	611	4825
	27.0%	26.0%	27.6%	21.8%	20.8%	36.1%	26.1%	30.5%	25.9%
13-18 years	543	274	465	1209	154	397	199	471	3712
	21.8%	20.6%	20.8%	18.0%	14.1%	24.0%	18.1%	23.5%	19.9%
19-29 years	541	244	464	1200	250	242	229	425	3595
	21.8%	18.3%	20.7%	17.9%	23.0%	14.6%	20.9%	21.2%	19.3%
30-49 years	381	247	356	1240	259	169	200	261	3113
	15.3%	18.5%	15.9%	18.5%	23.8%	10.2%	18.2%	13.0%	16.7%
50-74 years	238	162	231	1235	149	122	122	167	2426
	9.6%	12.2%	10.3%	18.4%	13.7%	7.4%	11.1%	8.3%	13.0%
75+ years	32	17	21	136	16	11	12	15	260
	1.3%	1.3%	0.9%	2.0%	1.5%	0.7%	1.1%	0.7%	1.4%
Total	2486	1332	2238	6720	1089	1656	1098	2005	18624
	100%	100%	100%	100%	100%	100%	100%	100%	100%

ATHNdataset — Demographic Profile

ATHNdataset	RACE AND ETHNICITY BY REGION AND TOTAL U.S. (AS OF SEPTEMBER 30, 2013)								
	REGION								TOTAL
	New England	Mid-Atlantic	Southeast	Great Lakes	Northern States	Great Plains	Mountain States	Western States	US
BY RACE									
White	2114	1071	1744	5809	978	1431	1015	1607	15769
	85.7%	80.4%	78.0%	86.5%	89.9%	86.8%	92.5%	80.4%	84.8%
Black	225	224	446	811	80	171	35	88	2080
	9.1%	16.8%	19.9%	12.1%	7.4%	10.4%	3.2%	4.4%	11.2%
Asian	94	34	29	88	21	35	17	191	509
	3.8%	2.6%	1.3%	1.3%	1.9%	2.1%	1.5%	9.6%	2.7%
Native Hawaiian or Other Pacific Islander	6							75	102
	0.2%							3.8%	0.5%
American Indian or Alaska Native	29		14	6	6	6	27	39	128
	1.2%		0.6%	0.1%	0.6%	0.4%	2.5%	2.0%	0.7%
Total Known	2468	1332	2237	6718	1088	1648	1097	2000	18588
	100%	100%	100%	100%	100%	100%	100%	100%	100%
Unknown	18	0	1	2	1	8	1	5	36
	0.7%	0.0%	0.0%	0.0%	0.1%	0.5%	0.1%	0.2%	0.2%
Total	2486	1332	2238	6720	1089	1656	1098	2005	18624
	100%	100%	100%	100%	100%	100%	100%	100%	100%
BY ETHNICITY									
Hispanic or Latino	309	78	221	250	84	281	214	753	2190
	12.5%	5.9%	9.9%	3.7%	7.7%	17.1%	19.5%	37.7%	11.8%
Non Hispanic or Latino	2159	1254	2016	6468	1004	1367	883	1247	16398
	87.5%	94.1%	90.1%	96.3%	92.3%	82.9%	80.5%	62.4%	88.2%
Total Known	2468	1332	2237	6718	1088	1648	1097	2000	18588
	100%	100%	100%	100%	100%	100%	100%	100%	100%
Unknown	18	0	1	2	1	8	1	5	36
	0.7%	0.0%	0.0%	0.0%	0.1%	0.5%	0.1%	0.2%	0.2%
Total	2486	1332	2238	6720	1089	1656	1098	2005	18624
	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 SEPTEMBER 30, 2013)	
PRIMARY BLEEDING OR CLOTTING DISORDER	TOTAL U.S. ATHNdataset CASES	% ATHNdataset
Factor VIII Deficiency	6669	35.81%
Factor IX Deficiency	1995	10.71%
Von Willebrand Disease	4625	24.83%
Rare Disorders	783	4.20%
Platelet Disorders	1330	7.14%
Thrombophilias	938	5.04%
Venous Thrombosis	835	4.48%
Other/Unknown	1449	7.78%
TOTAL	18624	100%



Factor VIII Deficiency

Factor VIII Deficiency — Bleeding Disorder Disease Severity

FACTOR VIII DEFICIENCY		DISEASE SEVERITY BY REGION AND TOTAL U.S. (AS OF SEPTEMBER 30, 2013)							
DISEASE SEVERITY		REGION							TOTAL
	New England	Mid-Atlantic	Southeast	Great Lakes	Northern States	Great Plains	Mountain States	Western States	US
Mild	236	159	250	347	116	209	149	282	1748
	24.7%	23.6%	22.9%	30.0%	26.9%	28.9%	23.2%	31.2%	26.6%
Moderate	168	119	194	183	73	110	112	131	1090
	17.6%	17.7%	17.8%	15.8%	16.9%	15.2%	17.4%	14.5%	16.6%
Severe	553	395	647	627	242	405	382	492	3743
	57.8%	58.7%	59.3%	54.2%	56.1%	55.9%	59.4%	54.4%	56.9%
TOTAL	957	673	1091	1157	431	724	643	905	6581
	100%	100%	100%	100%	100%	100%	100%	100%	100%
Unknown Severity	17	13	5	31	1	3	4	14	88
	1.7%	1.9%	0.5%	2.6%	0.2%	0.4%	0.6%	1.5%	1.3%
TOTAL	974	686	1096	1188	432	727	647	919	6669
	100%	100%	100%	100%	100%	100%	100%	100%	100%

Factor VIII Deficiency — HIV and Hepatitis C

FACTOR VIII DEFICIENCY		HIV AND HEPATITIS C (HCV) BY AGE GROUP (AS OF SEPTEMBER 30, 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		305	137		447
	0.0%	0.0%	0.0%		25.3%	20.0%		6.7%
No HIV	279	1829	1107	1501	900	547	59	6222
	100.0%	100.0%	100.0%	99.7%	74.7%	80.0%	98.3%	93.3%
TOTAL	279	1829	1107	1505	1205	684	60	6669
	100%	100%	100%	100%	100%	100%	100%	100%
HEPATITIS CO-MORBIDITY								
Hepatitis C	0			126	675	375	16	1196
	0.0%			8.4%	56.0%	54.8%	26.7%	17.9%
No Hepatitis C	279	1828	1104	1379	530	309	44	5473
	100.0%	99.9%	99.7%	91.6%	44.0%	45.2%	73.3%	82.1%
TOTAL	279	1829	1107	1505	1205	684	60	6669
	100%	100%	100%	100%	100%	100%	100%	100%

Factor VIII Deficiency — Continuous Prophylaxis (Prescribed)

FACTOR VIII DEFICIENCY		PROPHYLAXIS PRESCRIBED FOR PATIENTS BY BLEEDING DISORDER DISEASE SEVERITY AND AGE GROUP (AS OF SEPTEMBER 30, 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		22	23	14	8	8	0	76
			5.1%	7.5%	4.1%	2.8%	2.7%	0.0%	4.3%
	Not Prescribed/Unk	55	411	282	325	280	283	36	1672
		98.2%	94.9%	92.5%	95.9%	97.2%	97.3%	100.0%	95.7%
	Total Mild	56	433	305	339	288	291	36	1748
		100.0%	100.0%	100.0%	100.0%	100.0%	100.0%	100.0%	100.0%
Moderate	Prescribed	12	102	63	52	27	8	0	264
		22.6%	34.9%	33.0%	23.5%	14.2%	6.0%	0.0%	24.2%
	Not Prescribed/Unk	41	190	128	169	163	125	10	826
		77.4%	65.1%	67.0%	76.5%	85.8%	94.0%	100.0%	75.8%
	Total Moderate	53	292	191	221	190	133	10	1090
		100.0%	100.0%	100.0%	100.0%	100.0%	100.0%	100.0%	100.0%
Severe	Prescribed	55	740	442	483	238	58		2017
		34.4%	68.1%	73.2%	52.1%	33.8%	23.1%		53.9%
	Not Prescribed/Unk	105	346	162	444	467	193	9	1726
		65.6%	31.9%	26.8%	47.9%	66.2%	76.9%	90.0%	46.1%
	Total Severe	160	1086	604	927	705	251	10	3743
		100.0%	100.0%	100.0%	100.0%	100.0%	100.0%	100.0%	100.0%
Unknown	Prescribed			0		0	0	0	11
				0.0%		0.0%	0.0%	0.0%	12.5%
	Not Prescribed/Unk	8	13	7	14	22	9		77
		80.0%	72.2%	100.0%	77.8%	100.0%	100.0%		87.5%
	Total Unknown	10	18	7	18	22	9		88
		100.0%	100.0%	100.0%	100.0%	100.0%	100.0%		100.0%
All Severities	Total All	279	1829	1107	1505	1205	684	60	6669
		100%	100%	100%	100%	100%	100%	100%	100%

Factor VIII Deficiency — Payer Profile

FACTOR VIII DEFICIENCY		PAYER PROFILE BY REGION AND TOTAL U.S. (AS OF SEPTEMBER 30, 2013)							
PAYER CATEGORY	REGION								TOTAL
	New England	Mid-Atlantic	Southeast	Great Lakes	Northern States	Great Plains	Mountain States	Western States	US
Medicaid	114	81	83	141	51	38	55	74	637
	25.1%	24.5%	20.4%	27.9%	22.9%	21.7%	29.7%	16.3%	23.3%
Medicare	26	50	25	44	29	15	14	22	235
	5.7%	15.1%	6.1%	8.7%	13.0%	8.6%	7.6%	4.9%	8.6%
Private	304	187	239	278	123	96	112	175	1504
	67.0%	56.5%	58.7%	55.0%	55.2%	54.9%	60.5%	38.6%	55.0%
Uninsured			50		10	17		101	186
			12.3%		4.5%	9.7%		22.3%	6.8%
Other	7	12	10	40	10	9		81	171
	1.5%	3.6%	2.5%	7.9%	4.5%	5.1%		17.9%	6.3%
TOTAL	454	331	407	505	223	175	185	453	2733
	100%	100%	100%	100%	100%	100%	100%	100%	100%
Unknown	520	355	689	683	209	552	462	466	3936
	53.4%	51.7%	62.9%	57.5%	48.4%	75.9%	71.4%	50.7%	59.0%
TOTAL	974	686	1096	1188	432	727	647	919	6669
	100%	100%	100%	100%	100%	100%	100%	100%	100%

Factor IX Deficiency

Factor IX Deficiency — Bleeding Disorder Disease Severity

FACTOR IX DEFICIENCY		DISEASE DISORDER SEVERITY BY REGION AND TOTAL U.S. (AS OF SEPTEMBER 30, 2013)							
DISEASE SEVERITY		REGION							TOTAL
	New England	Mid-Atlantic	Southeast	Great Lakes	Northern States	Great Plains	Mountain States	Western States	US
Mild	62	47	97	176	35	59	47	43	566
	28.6%	28.5%	34.0%	28.1%	24.1%	33.5%	30.5%	23.8%	29.0%
Moderate	61	63	84	319	57	66	54	34	738
	28.1%	38.2%	29.5%	51.0%	39.3%	37.5%	35.1%	18.8%	37.9%
Severe	94	55	104	131	53	51	53	104	645
	43.3%	33.3%	36.5%	20.9%	36.6%	29.0%	34.4%	57.5%	33.1%
TOTAL	217	165	285	626	145	176	154	181	1949
	100%	100%	100%	100%	100%	100%	100%	100%	100%
Unknown Severity	4	4	2	19	8	1	1	7	46
	1.8%	2.4%	0.7%	2.9%	5.2%	0.6%	0.6%	3.7%	2.3%
TOTAL	221	169	287	645	153	177	155	188	1995
	100%	100%	100%	100%	100%	100%	100%	100%	100%

Factor IX Deficiency — HIV and Hepatitis C

FACTOR IX DEFICIENCY		HIV AND HEPATITIS C (HCV) BY AGE GROUP (AS OF SEPTEMBER 30, 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		41	20	0	63
	0.0%	0.0%	0.0%		10.5%	7.2%	0.0%	3.2%
No HIV	76	504	317	400	350	259	26	1932
	100.0%	100.0%	100.0%	99.5%	89.5%	92.8%	100.0%	96.8%
TOTAL	76	504	317	402	391	279	26	1995
	100%	100%	100%	100%	100%	100%	100%	100%
HEPATITIS C CO-MORBIDITY								
Hepatitis C	0	0		36	175	128	7	347
	0.0%	0.0%		9.0%	44.8%	45.9%	26.9%	17.4%
No Hepatitis C	76	504	316	366	216	151	19	1648
	100.0%	100.0%	99.7%	91.0%	55.2%	54.1%	73.1%	82.6%
TOTAL	76	504	317	402	391	279	26	1995
	100%	100%	100%	100%	100%	100%	100%	100%

Factor IX Deficiency – Continuous Prophylaxis (Prescribed)

FACTOR IX DEFICIENCY		PROPHYLAXIS PRESCRIBED FOR PATIENTS BY BLEEDING DISORDER DISEASE SEVERITY AND AGE GROUP (AS OF SEPTEMBER 30, 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	9						20
		0.0%	6.3%						3.5%
	Not Prescribed/Unk	17	135	97	108	87	92	10	546
		100.0%	93.8%	96.0%	98.2%	97.8%	97.9%	90.9%	96.5%
	Total Mild	17	144	101	110	89	94	11	566
		100.0%	100.0%	100.0%	100.0%	100.0%	100.0%	100.0%	100.0%
Moderate	Prescribed		28	22	10	10		0	76
			15.1%	19.1%	7.4%	6.6%		0.0%	10.3%
	Not Prescribed/Unk	30	158	93	125	142	104	10	662
		93.8%	84.9%	80.9%	92.6%	93.4%	96.3%	100.0%	89.7%
	Total Moderate	32	186	115	135	152	108	10	738
		100.0%	100.0%	100.0%	100.0%	100.0%	100.0%	100.0%	100.0%
Severe	Prescribed	8	104	58	61	37	20		289
		34.8%	62.3%	61.7%	40.4%	27.8%	27.8%		44.8%
	Not Prescribed/Unk	15	63	36	90	96	52		356
		65.2%	37.7%	38.3%	59.6%	72.2%	72.2%		55.2%
	Total Severe	23	167	94	151	133	72		645
		100.0%	100.0%	100.0%	100.0%	100.0%	100.0%		100.0%
Unknown	Prescribed	0	0			0	0	0	
		0.0%	0.0%			0.0%	0.0%	0.0%	
	Not Prescribed/Unk		7	6		17		0	43
			100.0%	85.7%		100.0%		0.0%	93.5%
	Total Unknown		7	7	6	17		0	46
			100.0%	100.0%	100.0%	100.0%		0.0%	100.0%
All Severities	Total All	76	504	317	402	391	279	26	1995
		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 SEPTEMBER 30, 2013)								
PAYER CATEGORY		REGION							TOTAL	
	New England	Mid-Atlantic	Southeast	Great Lakes	Northern States	Great Plains	Mountain States	Western States	US	
Medicaid	22	22	15	70	14	14		11	173	
	23.4%	29.3%	18.5%	28.1%	17.7%	28.6%		12.2%	23.1%	
Medicare	6	11		16	13			6	65	
	6.4%	14.7%		6.4%	16.5%			6.7%	8.7%	
Private	65	38	46	112	46	24	20	38	389	
	69.1%	50.7%	56.8%	45.0%	58.2%	49.0%	60.6%	42.2%	51.9%	
Uninsured	0			12	0			24	49	
	0.0%			4.8%	0.0%			26.7%	6.5%	
Other			10	39	6			11	74	
			12.3%	15.7%	7.6%			12.2%	9.9%	
TOTAL	94	75	81	249	79	49	33	90	750	
	100%	100%	100%	100%	100%	100%	100%	100%	100%	
Unknown	127	94	206	396	74	128	122	98	1245	
	57.5%	55.6%	71.8%	61.4%	48.4%	72.3%	78.7%	52.1%	62.4%	
TOTAL	221	169	287	645	153	177	155	188	1995	
	100%	100%	100%	100%	100%	100%	100%	100%	100%	

Von Willebrand Disease

Von Willebrand Disease — Demographic Profile

VON WILLEBRAND DISEASE		AGE AND SEX BY REGION AND TOTAL U.S. (AS OF SEPTEMBER 30, 2013)							
		REGION							TOTAL
	New England	Mid-Atlantic	Southeast	Great Lakes	Northern States	Great Plains	Mountain States	Western States	US
BY SEX									
Female	532	217	356	1027	191	177	86	319	2905
	66.0%	60.8%	60.0%	66.2%	61.6%	56.0%	62.3%	57.7%	62.8%
Male	274	140	237	525	119	139	52	234	1720
	34.0%	39.2%	40.0%	33.8%	38.4%	44.0%	37.7%	42.3%	37.2%
Total	806	357	593	1552	310	316	138	553	4625
	100%	100%	100%	100%	100%	100%	100%	100%	100%
BY AGE									
0-2 years	16		11	19					61
	2.0%		1.9%	1.2%					1.3%
3-12 years	243	102	164	399	84	116	38	184	1330
	30.1%	28.6%	27.7%	25.7%	27.1%	36.7%	27.5%	33.3%	28.8%
13-18 years	259	95	169	372	70	111	30	183	1289
	32.1%	26.6%	28.5%	24.0%	22.6%	35.1%	21.7%	33.1%	27.9%
19-29 years	157	74	120	336	62	48	26	101	924
	19.5%	20.7%	20.2%	21.6%	20.0%	15.2%	18.8%	18.3%	20.0%
30-49 years	74	45	82	236	55	24	18	47	581
	9.2%	12.6%	13.8%	15.2%	17.7%	7.6%	13.0%	8.5%	12.6%
50-74 years	51	30	38	175	29	15	22	33	393
	6.3%	8.4%	6.4%	11.3%	9.4%	4.7%	15.9%	6.0%	8.5%
75+ years	6	6	9	15	6	0			47
	0.7%	1.7%	1.5%	1.0%	1.9%	0.0%			1.0%
Total	806	357	593	1552	310	316	138	553	4625
	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 SEPTEMBER 30, 2013)								
	REGION								TOTAL
	New England	Mid-Atlantic	Southeast	Great Lakes	Northern States	Great Plains	Mountain States	Western States	US
BY RACE									
White	724	297	484	1417	293	286	133	464	4098
	89.8%	83.2%	81.6%	91.3%	94.5%	90.5%	96.4%	83.9%	88.6%
Black	48	48	95	111	12	25	0	8	347
	6.0%	13.4%	16.0%	7.2%	3.9%	7.9%	0.0%	1.4%	7.5%
Asian	14	11	12	23				49	118
	1.7%	3.1%	2.0%	1.5%				8.9%	2.6%
Native Hawaiian or Other Pacific Islander		0	0		0	0	0	27	33
		0.0%	0.0%		0.0%	0.0%	0.0%	4.9%	0.7%
American Indian or Alaska Native	15			0					29
	1.9%			0.0%					0.6%
Total	806	357	593	1552	310	316	138	553	4625
	100%	100%	100%	100%	100%	100%	100%	100%	100%
BY ETHNICITY									
Hispanic or Latino	84	27	66	69	19	47	17	201	530
	10.4%	7.6%	11.1%	4.4%	6.1%	14.9%	12.3%	36.3%	11.5%
Non Hispanic or Latino	722	330	527	1483	291	269	121	352	4095
	89.6%	92.4%	88.9%	95.6%	93.9%	85.1%	87.7%	63.7%	88.5%
Total	806	357	593	1552	310	316	138	553	4625
	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 SEPTEMBER 30, 2013)							
VWD TYPE	REGION								TOTAL
	New England	Mid-Atlantic	Southeast	Great Lakes	Northern States	Great Plains	Mountain States	Western States	US
VWD, Type 1	708	295	427	1319	246	233	84	472	3784
	89.6%	86.0%	74.7%	86.6%	80.9%	77.9%	65.1%	86.6%	84.0%
VWD, Type 2	58	35	110	162	42	51	33	54	545
	7.3%	10.2%	19.2%	10.6%	13.8%	17.1%	25.6%	9.9%	12.1%
VWD, Type 3	24	13	35	42	16	15	12	19	176
	3.0%	3.8%	6.1%	2.8%	5.3%	5.0%	9.3%	3.5%	3.9%
TOTAL w Type Specified	790	343	572	1523	304	299	129	545	4505
	100%	100%	100%	100%	100%	100%	100%	100%	100%
VWD, Type Other/Unknown	16	14	21	29	6	17	9	8	120
	2.0%	3.9%	3.5%	1.9%	1.9%	5.4%	6.5%	1.4%	2.6%
TOTAL	806	357	593	1552	310	316	138	553	4625
	100%	100%	100%	100%	100%	100%	100%	100%	100%

Von Willebrand Disease — HIV and Hepatitis C

VON WILLEBRAND DISEASE		HIV AND HEPATITIS C (HCV) BY AGE GROUP (AS OF SEPTEMBER 30, 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	61	1330	1289	923	576	391	47	4617
	100.0%	100.0%	100.0%	99.9%	99.1%	99.5%	100.0%	99.8%
TOTAL	61	1330	1289	924	581	393	47	4625
	100%	100%	100%	100%	100%	100%	100%	100%
HEPATITIS C CO-MORBIDITY								
Hepatitis C	0	0	0		32	40		80
	0.0%	0.0%	0.0%		5.5%	10.2%		1.7%
No Hepatitis C	61	1330	1289	920	549	353	43	4545
	100.0%	100.0%	100.0%	99.6%	94.5%	89.8%	91.5%	98.3%
TOTAL	61	1330	1289	924	581	393	47	4625
	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
Region IX	State equals: CA, HI, GU, NV
Region X	State equals: OR, WA, AK, ID, WA

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 <5% 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)

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)
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



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.*