NHD
U.K.H.C.D.O NATIONAL HAEMOPHILIA DATABASE

Bleeding Disorder Statistics for Wales

April 2019 to March 2020

A report from the UK National Haemophilia Database

August 2021

The following report is based on people who are registered with the National Haemophilia Database with a Welsh post code (unless otherwise stated), regardless of which Haemophilia Centre they were treated at.

Contents

New Registrations

Table 1 New registrations - Number of new registrations between April 2019 & March 2020, by diagnosis and gender

Coagulation Defect	Male	Female	Total
Haemophilia A	7	0	7
Haemophilia A Carrier		14	14
Acquired Haemophilia A	10	3	13
Haemophilia B	5	0	5
Haemophilia B Carrier	0	6	6
von Willebrand disease	13	15	28
Acquired von Willebrands	2	1	3
F.VII deficiency	3	3	6
F.XI Deficiency	5	8	13
Co-inherited diagnoses	2	2	4
Dysfibrinogenemia	0	2	2
Hypofibrinogenemia	0	1	1
Platelet-type Pseudo von Willebrand Disease	0	1	1
Other platelet defects	8	17	25
Miscellaneous	0	1	1
Unclassified bleeding disorder	3	19	22
Total	**58**	**93**	**151**

Carrier of Haemophilia A includes and Females with FVIII deficiency
Carrier of Haemophilia B includes and Females with FIX deficiency and Factor IX Leyden carriers

Table 1 shows the number of new registrations to the National Haemophilia Database of people with a Welsh postcode.

Table 2 New registrations of Haemophilia A & B between April 2019 & March 2020, by age and disease severity

Coagulation Defect	Age (years)	Number of Patients (factor level iu/dl)			
		< 1	1 - 5	> 5	Total
Haemophilia A	0 : 9	1	1	0	2
	10 : 19	0	0	0	0
	20 : 29	0	1	1	2
	30 : 39	0	0	0	0
	40 : 49	0	0	0	0
	50 : 59	0	0	2	2
	60 : 69	0	0	0	0
	70 +	0	0	1	1
Total		**1**	**2**	**4**	**7**
Haemophilia B	0 : 9	1	0	2	3
	10 : 19	0	0	0	0
	20 : 29	0	0	1	1
	30 : 39	0	0	0	0
	40 : 49	0	0	0	0
	50 : 59	0	0	1	1
	60 : 69	0	0	0	0
	70 +	0	0	0	0
Total		**1**	**0**	**4**	**5**

N.B Age calculated at mid-year, 30/09/2019

Table 2 shows the number of new registrations of people with Haemophilia A and B with a Welsh postcode. This is broken down by age and disease severity as per the ISTH severity classification.

In Register

Table 3 In Register – The total number of people in the register as of 31st March 2020 and the number treated between April 2019 & March 2020

Coagulation Defect	In Register			Treated (n)	Treated %
	Males	Females	Total		
Haemophilia A	297	1	298	158	53.02%
Haemophilia A Carrier		84	84	6	7.14%
Haemophilia A with Liver Transplant	1	0	1	0	0.00%
Acquired Haemophilia A	26	11	37	6	16.22%
Haemophilia B	63	0	63	38	60.32%
Haemophilia B Carrier		26	26	1	3.85%
von Willebrand disease	170	297	467	48	10.28%
Acquired von Willebrand disease	8	1	9	1	11.11%
Probable von Willebrand disease	3	5	8	0	0.00%
Platelet-type Pseudo von Willebrand Disease	3	3	6	0	0.00%
F.V deficiency	1	3	4	0	0.00%
F.VII deficiency	19	22	41	1	2.44%
F.X deficiency	0	1	1	0	0.00%
F.XI Deficiency	46	78	124	1	0.81%
F.XIII Deficiency	0	1	1	1	100.00%
Combined II+VII+IX+X Deficiency	1	0	1	0	0.00%
Co-inherited diagnoses	3	11	14	0	0.00%
Prothrombin Deficiency	1	1	2	0	0.00%
Acquired Prothrombin Deficiency	1	0	1	0	0.00%
Afibrinogenemia	1	0	1	1	100.00%
Dysfibrinogenemia	9	23	32	1	3.13%
Hypofibrinogenemia	5	8	13	1	7.69%
Hypodysfibrinogenemia	1	1	2	0	0.00%
Glanzmanns Thrombasthenia	2	2	4	1	25.00%
Bernard Soulier	0	2	2	0	0.00%
Other platelet defects	59	119	178	8	4.49%
Miscellaneous	3	4	7	2	28.57%
Unclassified bleeding disorder	8	59	67	3	4.48%
Totals	**731**	**763**	**1,494**	**278**	

Table 3 shows the total number of active registrations of people with a Welsh postcode and the number who were issued treatment during 2019/20.

Table 4 In Register – The total number of people in the register as of 31st March 2020, by diagnosis and Health Board

Coagulation Defect	Aneurin Bevan University Health Board	Betsi Cadwaladr University Health Board	Cardiff and Vale University Health Board	Cwm Taf Morgannwg University Health Board	Hywel Dda University Health Board	Powys Teaching Health Board	Swansea Bay University Health Board	Total
Haemophilia A	59	55	55	43	30	8	48	298
Haemophilia A with Liver Transplant	0	0	0	1	0	0	0	1
Acquired Haemophilia A	6	5	8	9	6	1	2	37
Haemophilia A Carrier	15	18	15	5	5	1	25	84
Haemophilia B	18	7	12	15	5	1	5	63
Haemophilia B Carrier	5	1	7	7	1	2	3	26
von Willebrand disease	82	157	81	32	36	59	21	468
Acquired von Willebrand disease	3	1	3	1	0	0	1	9
Probable von Willebrand disease	2	2	2	0	1	1	0	8
Platelet-type Pseudo von Willebrand Disease	0	0	0	1	2	3	0	6
Prothrombin Deficiency	0	0	0	1	1	0	0	2
F.V deficiency	2	0	0	0	1	0	1	4
F.VII deficiency	7	13	5	4	8	1	3	41
F.X deficiency	0	0	1	0	0	0	0	1
F.XI Deficiency	58	11	15	10	10	2	18	124
F.XIII Deficiency	0	1	0	0	0	0	0	1
Combined II+VII+IX+X Deficiency	0	0	1	0	0	0	0	1
Co-inherited diagnoses	2	4	2	0	1	3	2	14
Acquired Prothrombin Deficiency	0	0	1	0	0	0	0	1
Afibrinogenemia	0	0	0	1	0	0	0	1
Dysfibrinogenemia	7	2	17	4	0	0	2	32
Hypofibrinogenemia	2	1	4	3	2	0	1	13
Hypodysfibrinogenemia	0	0	1	0	0	0	1	2
Glanzmanns Thrombasthenia	0	0	2	0	2	0	0	4
Bernard Soulier	0	1	1	0	0	0	0	2
Other platelet defects	26	43	37	37	8	5	22	178
Miscellaneous	2	2	1	2	0	0	0	7
Unclassified bleeding disorder	21	3	17	11	4	1	10	67
Total	**317**	**327**	**288**	**187**	**123**	**88**	**165**	**1,495**

Table 4 shows the number of people registered by health board. People are allocated to a health board based on their home postcode. The source database used for mapping postcodes to health boards is the ONS Postcode Directory (Nov 2020) and is available to download at https://ons.maps.arcgis.com/home/item.html?id=8da1cb5b6daa4d72b8bbef115cf26746.

Table 5 In register – The total number of people with haemophilia A & B in the register as of 31st March 2020, by severity and age group

Coagulation Defect	Age (years)	Number of Patients (factor level iu/dl)			
		< 1	1 - 5	> 5	Total
Haemophilia A	<18 years	31	11	20	**62**
	≥18 years	60	18	157	**235**
Total		**91**	**29**	**177**	**297**
Haemophilia B	<18 years	3	7	7	**17**
	≥18 years	11	16	19	**46**
Total		**14**	**23**	**26**	**63**

N.B Age calculated at mid-year, 30/09/2019

Table 5 shows a more detailed breakdown of active registrations of people with a Welsh postcode with haemophilia A and B. This is broken down by age and disease severity as per the ISTH severity classification.

Table 6 In register – The number of people with other selected bleeding disorders in the register as of 31st March 2020 and the number treated between April 2019 & March 2020, by disease severity

Coagulation Defect	Number of Patients (factor level iu/dl)							
	<5		≥5		N/K		Total	
	In Reg	Treated	In Reg	Treated	In Reg	Treated	In Reg	Treated
F.V deficiency	-	-	4	-	-	-	4	-
F.VII deficiency	1	-	40	1	-	-	41	1
F.X deficiency	-	-	1	-	-	-	1	-
F.XI Deficiency	10	-	114	1	-	-	124	1
Total	**11**	**-**	**159**	**2**	**-**	**-**	**170**	**2**

Coagulation Defect	<2		5 - <10		10 - <15		Total	
	In Reg	Treated	In Reg	Treated	In Reg	Treated	In Reg	Treated
F.XIII Deficiency	1	1	-	-	-	-	1	1
Total	**1**	**1**	**-**	**-**	**-**	**-**	**1**	**1**

Table 6 shows the number of people with other selected bleeding disorders and a Welsh postcode known to the NHD during 2019/20. It is acknowledged that these disorders have no recognised classification of disease severity. However, the table above gives an idea of the range of registered levels.

Table 7 In Register – The total number of people with von Willebrand Disease in the register as of 31st March 2020 and the number treated between April 2019 & March 2020, by disease severity, age group and gender

von Willebrand disease	VWD Activity IU/dl										Total	Treated
	<10	10 - 29	≥30	N/K	Sub Total	<10	10 - 29	≥30	N/K	Sub Total		
	<18 years					≥18 years						
Males												
Type 1	3	9	7	1	**20**	17	31	10	0	**58**	**78**	**4**
Type 2A	3	0	0	0	**3**	10	2	2	0	**14**	**17**	**5**
Type 2B	1	0	0	0	**1**	0	2	1	0	**3**	**4**	**2**
Type 2M	1	0	0	0	**1**	4	3	0	0	**7**	**8**	**1**
Type 2N	0	0	0	0	**0**	0	0	0	0	**0**	**0**	**0**
Type 2 Unspecified	1	1	0	0	**2**	2	1	1	0	**4**	**6**	**1**
Type 3	3				**3**	3				**3**	**6**	**5**
Type Unreported	2	1	0	1	**4**	10	9	13	0	**32**	**36**	**5**
Low VWF	3	0	1	0	**4**	2	1	8	0	**11**	**15**	**1**
										Sub Total Males	**170**	**24**
Females												
Type 1	5	11	3	0	**19**	19	48	37	0	**104**	**123**	**10**
Type 2A	2	1	0	0	**3**	11	10	6	0	**27**	**30**	**5**
Type 2B	0	0	0	0	**0**	0	0	6	1	**7**	**7**	**0**
Type 2M	0	1	0	0	**1**	7	3	5	0	**15**	**16**	**0**
Type 2N	0	0	1	0	**1**	0	0	5	0	**5**	**6**	**1**
Type 2 Unspecified	0	2	1	0	**3**	3	2	0	0	**5**	**8**	**0**
Type 3	0				**0**	1				**1**	**1**	**1**
Type Unreported	2	4	5	0	**11**	11	16	43	3	**73**	**84**	**6**
Low VWF	0	0	2	0	**2**	0	0	20	0	**20**	**22**	**1**
										Sub Total Females	**297**	**24**
										Grand Total - Males and Females	**467**	**48**

Table 7 shows people registered with von Willebrand disease broken down by age, activity level, subtype, gender and treatment. Whilst there is no generally agreed severity classification for VWD, it is reported here in subdivisions from "<10, 10-29 and ≥30" to give some indication of the distribution of severity.

Treatment

Table 8 People with a Welsh postcode, treated between April 2019 & March 2020 by diagnosis all severities, and region which issued the treatment

Coagulation Defect	Region Issuing Treatment	Patients Treated (n)
Haemophilia A	Birmingham & Black Country	1
	Cheshire, Warrington & Wirral	19
	London	2
	Wales	139
Haemophilia A Carrier	Wales	6
Acquired Haemophilia A	Wales	6
Haemophilia B	Birmingham & Black Country	1
	Cheshire, Warrington & Wirral	3
	Leicestershire & Lincolnshire	1
	Wales	33
Haemophilia B Carrier	Wales	1
von Willebrand disease	Birmingham & Black Country	1
	Cheshire, Warrington & Wirral	2
	London	1
	Wales	46
Acquired von Willebrands	Wales	1
F.VII deficiency	Wales	1
F.XI Deficiency	Wales	1
F.XIII Deficiency	Wales	1
Afibrinogenemia	Wales	1
Dysfibrinogenemia	Wales	1
Hypofibrinogenemia	Cheshire, Warrington & Wirral	1
Glanzmann's Thrombasthenia	Wales	1
Other platelet defects	Wales	8
Miscellaneous	Wales	2
Unclassified bleeding disorder	Wales	3
	Grand total	**283**

N.B This table may contain duplicates where a person received treatment from more than one region

Table 8 reports people with a Welsh postcode by the region that issued the treatment. Some people received treatment outside of Wales. More detail on the treatment issued to people with severe haemophilia A and B can be found in tables 12 and 18 respectively.

Table 9 People with a non-Welsh postcode, registered & treated at a Welsh Haemophilia Centre between April 2019 & March 2020, by diagnosis, all severities

Coagulation Defect	Patient's home postcode region	Patients Registered (n)	Patients Treated (n)
Haemophilia A	East of England	1	0
	London	2	1
	Midlands	4	2
	North West	1	0
	South East	3	1
	South West	8	5
	Sub total	**19**	**9**
Haemophilia A Carrier	London	2	0
	South West	2	0
	Sub total	**4**	**0**
Haemophilia B	South East	1	0
	Sub total	**1**	**0**
von Willebrand disease	Midlands	7	0
	North East and Yorkshire	2	0
	North West	2	0
	Scotland	1	0
	South West	2	1
	Sub total	**14**	**1**
F.XI Deficiency	South West	2	0
Co-inherited diagnoses	Midlands	2	1
Hypofibrinogenemia	South West	1	0
Other platelet defects	Scotland	4	0
Other platelet defects	South West	2	0
Unclassified bleeding disorder	South West	2	0
	Grand total	**51**	**11**

Those reported in Table 9 were registered at or issued treatment from a Welsh Haemophilia Centre during 2019/20, however, they have a postcode recorded on the NHD which falls outside of Wales.

Haemophilia A and Factor VIII use

Table 10 Factor VIII issued, by diagnosis

Coagulation Defect	Patients Treated	FVIII (IU)			
		Plasma	Recombinant	Enhanced Half-Life	Total
Haemophilia A	147	372,925	21,151,750	3,548,000	25,072,675
Haemophilia A Carrier	1 - 2	-	76,000	-	76,000
Acquired Haemophilia A	1 - 2	-	150,500	-	150,500
von Willebrand disease	36	932,500	-	-	932,500
Acquired von Willebrand Disease	1 - 2	2,500	-	-	2,500
Total	**183***	**1,307,925**	**21,378,250**	**3,548,000**	**26,234,175**

Includes products containing a combination of VWF and FVIII, which are reported in FVIII units

Table 10 shows the number of people with a Welsh postcode who were issued factor VIII concentrate during 2019/20. Also shown are the number of units issued broken down by diagnosis and product type. No investigational products were reported.

Table 11 Products issued to treat Haemophilia A (including inhibitors)

Manufacturer	Product	Patients (n)	Total Units
Bayer	Kogenate	1 - 2	390,000
BPL	Optivate	1 - 2	372,925
Novo Nordisk	NovoEight	16	3,329,000
	NovoSeven (mg)	3	141
Octapharma	Nuwiq	1 - 2	560,000
Pfizer	ReFacto AF	57	9,192,500
Roche	Hemlibra (mg)	11	19,891
SOBI/Biogen	Elocta	22	3,548,000
Takeda	Advate	56	7,680,250
	FEIBA	1 - 2	174,000
	Desmopressin	7	499

Units in IU unless otherwise stated

Table 11 shows the number of units of products issued to people with a Welsh postcode with Haemophilia A, all severities, including those with inhibitors, broken down by supplier.

Table 12 Factor VIII issued by Haemophilia Centre and Health Board for people with *Severe* Haemophilia A (incl. treatment for inhibitors)

Haemophilia Centre Issuing Treatment	Health Board based on patients postcode	Severe Haemophilia A		
		Patients treated (n)	Total FVIII Units	Mean Usage
Bangor	Betsi Cadwaladr University Health Board	11	4,074,675	370,425
Birmingham (Queen Elizabeth)	Swansea Bay University Health Board	1	243,000	243,000
Cardiff	Aneurin Bevan University Health Board	14	3,597,000	256,929
	Cardiff and Vale University Health Board	11	2,334,000	212,182
	Cwm Taf Morgannwg University Health Board	17	4,146,000	243,882
	Hywel Dda University Health Board	9	1,842,000	204,667
	Powys Teaching Health Board	1	201,500	201,500
	Swansea Bay University Health Board	6	1,192,500	198,750
Hammersmith Hospital, London	Cardiff and Vale University Health Board	1	12,000	12,000
Liverpool (R. I.)	Betsi Cadwaladr University Health Board	7	1,806,000	258,000
Manchester Children's	Betsi Cadwaladr University Health Board	4	591,000	147,750
Swansea	Hywel Dda University Health Board	3	550,000	183,333
	Swansea Bay University Health Board	7	999,750	142,821
The Royal London Hospital	Cardiff and Vale University Health Board	1	27,000	27,000
	Totals	**93**	**21,616,425**	**232,435**

Table 12 reports the number of people with a Welsh postcode with severe haemophilia A treated and the number of units of factor VIII issued during 2019/20. This is broken down by the haemophilia centre which issued the treatment and by health board based on the person's postcode as recorded on the NHD.

Note: Eight people were issued treatment from two different centres.

Table 13 Factor VIII usage by Health Board for people with *Severe Haemophilia A* only (incl. treatment for inhibitors)

Health Board	General Population	Severe Haemophilia A			
		Patients treated (n)	Total FVIII Units (IU)	Mean Usage	FVIII Units Per Capita
Betsi Cadwaladr University Health Board	699,559	21	6,471,675	308,175	9.25
Aneurin Bevan University Health Board	594,164	14	3,597,000	256,929	6.05
Cwm Taf Morgannwg University Health Board	448,639	17	4,146,000	243,882	9.24
Hywel Dda University Health Board	387,284	10	2,392,000	239,200	6.18
Swansea Bay University Health Board	390,308	11	2,435,250	221,386	6.24
Cardiff and Vale University Health Board	500,490	11	2,373,000	215,727	4.74
Powys Teaching Health Board	132,435	1	201,500	201,500	1.52
Wales	**3,152,879**	**85**	**21,616,425**	**1,686,800**	**6.86**

Ranked by mean usage

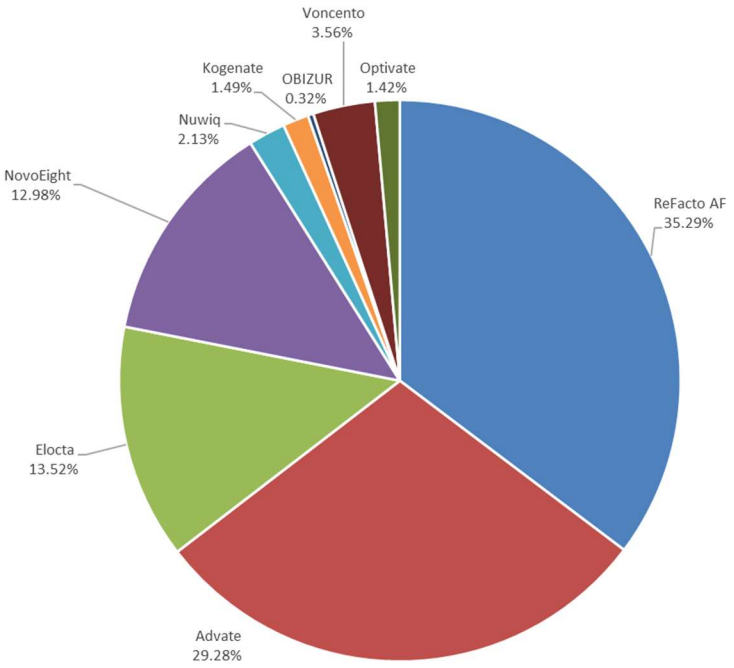
Mid-2019 population estimates, Office for National Statistics. Published by the Welsh Government, under the Open Government Licence v3.0. Updated June 2020

https://statswales.gov.wales/Catalogue/Population-and-Migration/Population/Estimates/Local-Health-Boards/populationestimates-by-lhb-age

Table 13 reports the number of people with severe haemophilia A treated and number of units of factor VIII issued broken down by Health Board and ranked by the mean number of units issued per person. Usage per capita of population is also reported.

Note: This table does not contain duplicates. People are allocated to a Health Board based on their home postcode.

Figure 1 Market share of factor VIII concentrates issued between April 2019 & March 2020



Includes products containing a combination of VWF and FVIII, which are reported in FVIII units

This pie chart and table are arranged in descending order of recombinant products by volume, then descending order of plasma products by volume

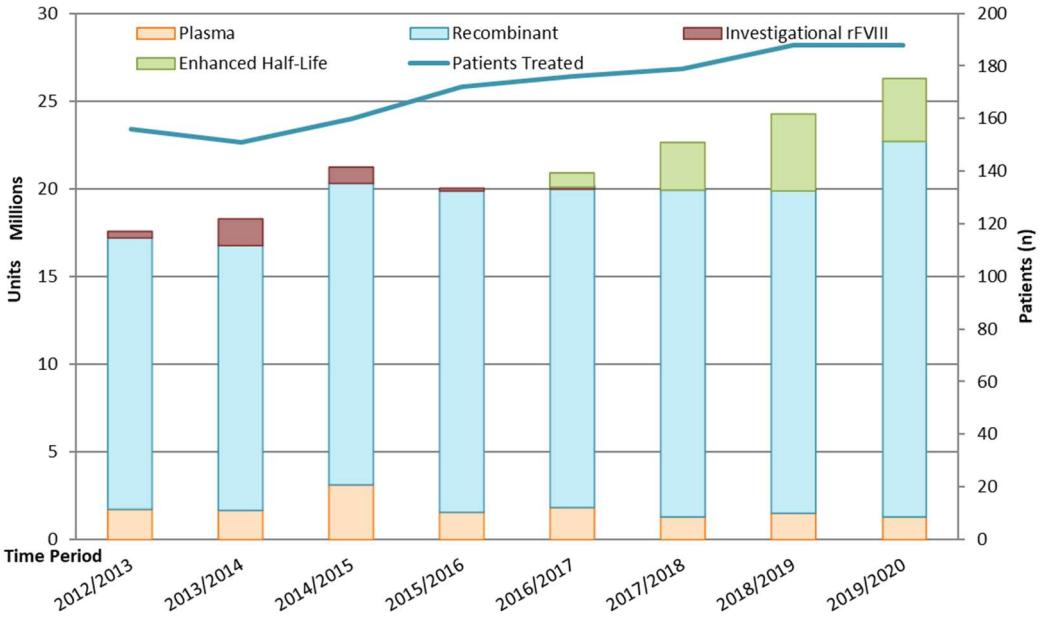
Manufacturer	Product	Patients (n)	Units (IU)
Pfizer	ReFacto AF	58	9,258,500
Takeda	Advate	56	7,680,250
SOBI/Biogen	Elocta	22	3,548,000
Novo Nordisk	NovoEight	18	3,405,000
Octapharma	Nuwiq	1 - 2	560,000
Bayer	Kogenate	1 - 2	390,000
Takeda	OBIZUR	1 - 2	84,500
CSL Behring	Voncento	37	935,000
BPL	Optivate	1 - 2	372,925
	Total	**191***	**26,234,175**

** This is the total excluding numbers which have been suppressed.*

Figure 1 shows the market breakdown of factor VIII concentrates issued for all diagnoses, including people with inhibitors. Also included is a table showing the number of units and number of people issued with these products.

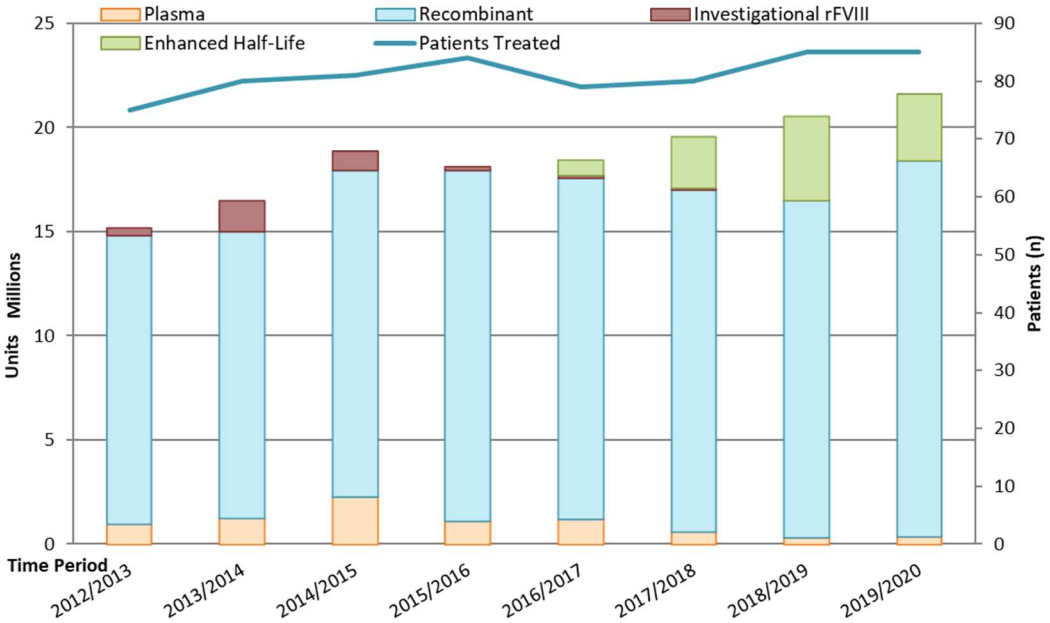
NOTE: The number of people in this table cannot be compared with table 10 as this table includes people treated with more than one product type. The numbers in Table 10 do not contain duplicates.

Figure 2 Factor VIII units issued by financial year between April 2012 & March 2020 – all diagnoses, all severities



Includes products containing a combination of VWF and FVIII, which are reported in FVIII units

Figure 3 Factor VIII units by financial year between April 2012 & March 2020 – *Severe Haemophilia A only*



Includes products containing a combination of VWF and FVIII, which are reported in FVIII units

Figures 2 and 3 give an historical view of the number of factor VIII units issued between 2012/13 and 2019/20 for all diagnoses and for people with severe haemophilia A respectively. The number of people treated is represented by the blue line using a secondary axis.

Table 14 Data table for Figure 2 - Factor VIII units issued by financial year between April 2012 & March 2020 - all diagnoses

Year	Plasma		Recombinant		Investigational rFVIII		Enhanced Half-Life		Total		Patients Treated	
	IU	% difference year on year	IU	% difference year on year	IU	% difference year on year	IU	% difference year on year	IU	% difference year on year	n	% difference year on year
2012/2013	1,692,150	-	15,525,592	-	359,500	-	0		17,577,242	-	156	-
2013/2014	1,642,820	-2.92	15,130,500	-2.54	1,484,500	+312.9	0		18,257,820	+3.9	151	-3.21
2014/2015	3,113,500	+89.5	17,127,000	+13.2	943,170	-36.47	0		21,183,670	+16	160	+6
2015/2016	1,544,000	-50.41	18,262,750	+6.6	176,000	-81.34	0		19,982,750	-5.67	172	+7.5
2016/2017	1,794,475	+16.2	18,155,898	-0.59	134,875	-23.37	794,500	-	20,879,748	+4.5	176	+2.3
2017/2018	1,284,910	-28.40	18,570,750	+2.3	76,020	-43.64	2,668,000	+235.8	22,599,680	+8.2	179	+1.7
2018/2019	1,473,380	+14.7	18,421,250	-0.81	7,875	-89.64	4,351,500	+63.1	24,254,005	+7.3	188	+5
2019/2020	1,307,925	-11.23	21,378,250	+16.1	0	-100.00	3,548,000	-18.46	26,234,175	+8.2	188	-

Table 15 Data table for Figure 3 - Factor VIII units by financial year between April 2012 & March 2020 - *Severe Haemophilia A*

Year	Plasma		Recombinant		Investicational rFVIII		Enhanced Half-Life		Total		Patients Treated	
	IU	% difference year on year	IU	% difference year on year	IU	% difference year on year	IU	% difference year on year	IU	% difference year on year	n	% difference year on year
2012/2013	1,009,150	-	13,810,092	-	359,500	-	0		15,828,392	-	75	-
2013/2014	1,275,000	+26.3	13,724,750	-0.62	1,484,500	+312.9	0		16,274,750	+2.8	80	+6.7
2014/2015	2,291,500	+79.7	15,639,750	+14	943,170	-36.47	0		20,222,750	+24.3	81	+1.3
2015/2016	1,135,000	-50.47	16,808,000	+7.5	176,000	-81.34	0		19,078,000	-5.66	84	+3.7
2016/2017	1,236,475	+8.9	16,306,398	-2.98	134,875	-23.37	768,500	-	19,547,848	+2.5	79	-5.95
2017/2018	647,310	-47.65	16,357,250	+0.3	76,020	-43.64	2,455,000	+219.5	20,106,870	+2.9	80	+1.3
2018/2019	361,880	-44.09	16,114,000	-1.49	7,875	-89.64	4,052,500	+65.1	20,890,260	+3.9	85	+6.3
2019/2020	372,925	+3.1	18,026,500	+11.9	0	-100.00	3,217,000	-20.62	21,989,350	+5.3	85	-

Tables 14 and 15 are the data tables for Figures 2 and 3.

Haemophilia B and Factor IX use

Table 16 Factor IX issued, by diagnosis

Coagulation Defect	Patients Treated	FIX (IU)				
		Plasma	Recombinant	Enhanced Half-Life	Investigational	Total
Haemophilia B	38	-	2,301,750	823,750	-	3,125,500
Haemophilia B Carrier	1	-	22,000	-	-	22,000
Total	**39**	**-**	**2,323,750**	**823,750**	**-**	**3,147,500**

Table 16 shows the number of people with a Welsh postcode who were issued factor IX concentrate during 2019/20. Also shown are the number of units issued broken down by diagnosis and product type.

Table 17 Products issued to treat Haemophilia B (including inhibitors)

Manufacturer	Product	Patients (n)	Total Units
CSL Behring	IDELVION	4	298,000
NovoNordisk	Refixia	1 - 2	117,000
Pfizer	BeneFIX	29	2,297,750
SOBI/Biogen	ALPROLIX	6	408,750
Takeda	RIXUBIS	1 - 2	4,000

Units in IU unless otherwise stated

Table 17 shows the number of units of products issued to people with a Welsh postcode with Haemophilia B, all severities, including those with inhibitors, broken down by supplier.

Table 18 Factor IX issued to people with *Severe* Haemophilia B (incl. treatment for inhibitors), by Haemophilia Centre and Health Board

Haemophilia Centre Issuing Treatment	Health Board based on patients postcode	Severe Haemophilia B		
		Patients treated (n)	Total FIX Units	Mean Usage
Cardiff	Aneurin Bevan University Health Board	4	529,250	132,313
	Cwm Taf Morgannwg University Health Board	5	953,250	190,650
	Hywel Dda University Health Board	1	2,000	2,000
	Swansea Bay University Health Board	1	90,000	90,000
Liverpool (R. I.)	Betsi Cadwaladr University Health Board	1	295,000	295,000
Swansea	Hywel Dda University Health Board	1	2,000	2,000
	Swansea Bay University Health Board	1	135,000	135,000
	Totals	**14**	**2,006,500**	**143,321**

Table 18 reports the number of people with severe haemophilia B treated and the number of units of factor IX issued during 2019/20. This is broken down by the haemophilia centre which issued the treatment and by health board based on the person's postcode as recorded on the NHD.

Note: One person was issued treatment from two different centres.

Table 19 Factor IX usage for people with *Severe Haemophilia B* only (incl. treatment for inhibitors), by Health Board

Health Board	General Population	Severe Haemophilia B			
		Patients treated (n)	Total FIX Units (IU)	Mean Usage	FIX Units Per Capita
Betsi Cadwaladr University Health Board	699,559	1	295,000	295,000	0.42
Cwm Taf Morgannwg University Health Board	448,639	5	953,250	190,650	2.12
Aneurin Bevan University Health Board	594,164	4	529,250	132,313	0.89
Swansea Bay University Health Board	390,308	2	225,000	112,500	0.58
Hywel Dda University Health Board	387,284	1	4,000	4,000	0.01
Cardiff and Vale University Health Board	500,490	0	0	-	0.00
Powys Teaching Health Board	132,435	0	0	-	0.00
Wales	**3,152,879**	**13**	**2,006,500**	**734,463**	**4.02**

Ranked by mean usage

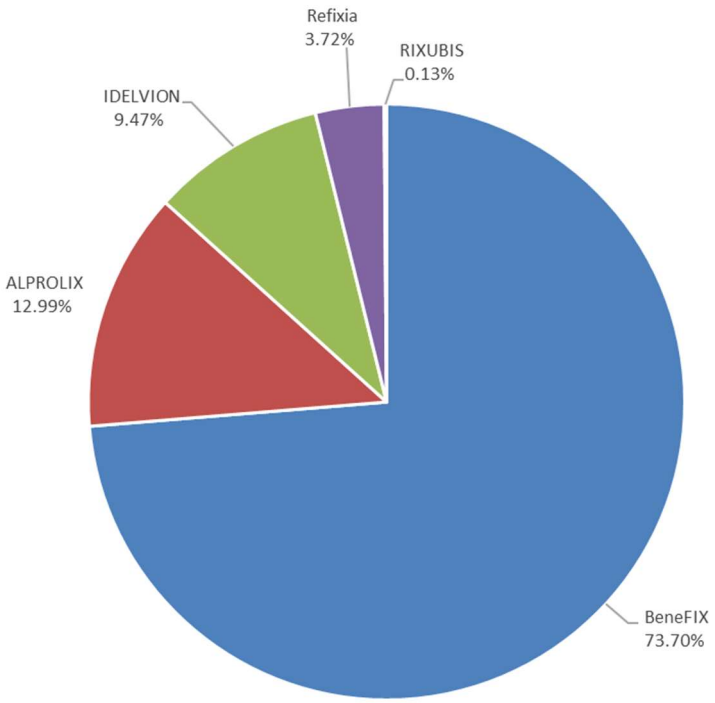
Mid-2019 population estimates, Office for National Statistics. Published by the Welsh Government, under the Open Government Licence v3.0. Updated June 2020

https://statswales.gov.wales/Catalogue/Population-and-Migration/Population/Estimates/Local-Health-Boards/populationestimates-by-lhb-age

Table 19 reports the number of people with severe haemophilia B treated and number of units of factor IX issued broken down by Health Board and ranked by the mean number of units issued per person. Usage per capita of population is also reported.

This table does not contain duplicates. People are allocated to a Health Board based on their home postcode.

Figure 4 Market share of factor IX concentrates issued to people with a Welsh postcode between April 2019 & March 2020



This pie chart and table are arranged in descending order of recombinant products by volume, then descending order of plasma products by volume

Manufacturer	Product	Patients (n)	Units (IU)
Pfizer	BeneFIX	30	2,319,750
SOBI/Biogen	ALPROLIX	6	408,750
CSL Behring	IDELVION	4	298,000
NovoNordisk	Refixia	2	117,000
Takeda	RIXUBIS	1	4,000
	Total	**43**	**3,147,500**

Figure 4 shows the market breakdown of factor IX concentrates issued for all diagnoses, including people with inhibitors. Also included is a table showing the number of units and number of people issued with these products.

NOTE: The number of people in this table cannot be compared with table 16 as this table includes people treated with more than one product type. The numbers in Table 16 do not contain duplicates.

Figure 5 Factor IX units by financial year between April 2012 & March 2020 – all diagnoses, all severities

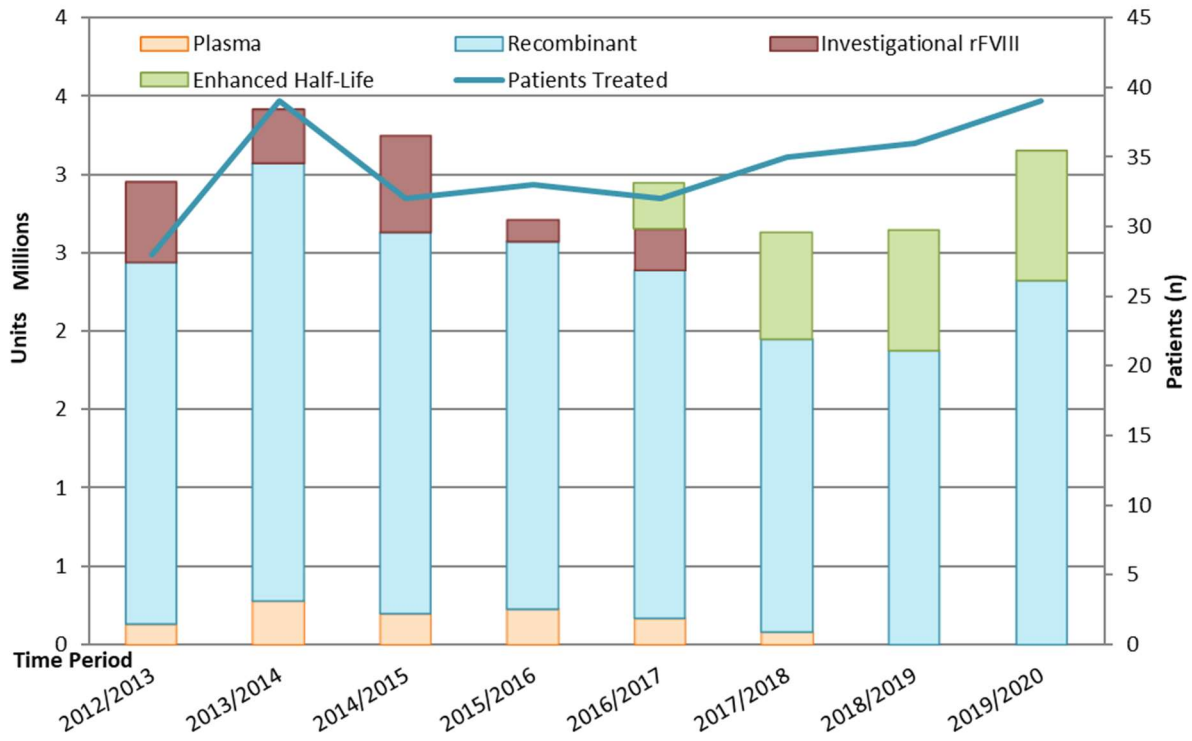


Figure 5 gives an historical view of the number of factor IX units issued between 2012/13 and 2019/20 for all diagnoses. The number of people treated is represented by the blue line using a secondary axis.

Table 20 Data table for figure 5 - Factor IX units issued by financial year between April 2012 & March 2020 – all diagnoses

Year	Plasma		Recombinant		Investigational rFVIII		Enhanced Half-Life		Total		Patients Treated	
	IU	% difference year on year	IU	% difference year on year	IU	% difference year on year	IU	% difference year on year	IU	% difference year on year	n	% difference year on year
2012/2013	133,000	-	2,305,500	-	517,180	-	0		2,955,680	-	28	-
2013/2014	274,000	+106	2,794,770	+21.2	349,016	-32.52	0		3,417,786	+15.6	39	+39.3
2014/2015	198,000	-27.74	2,432,500	-12.96	617,447	+76.9	0		3,247,947	-4.97	32	-17.95
2015/2016	228,000	+15.2	2,343,000	-3.68	136,553	-77.88	0		2,707,553	-16.64	33	+3.1
2016/2017	170,000	-25.44	2,220,000	-5.25	263,692	+93.1	289,015	-	2,942,707	+8.7	32	-3.03
2017/2018	78,000	-54.12	1,870,500	-15.74	-	-	678,500	+134.8	2,627,000	-10.73	35	+9.4
2018/2019	-	-	1,876,000	+0.3	-	-	769,000	+13.3	2,645,000	+0.7	36	+2.9
2019/2020	-	-	2,323,750	+23.9	-	-	823,750	+7.1	3,147,500	+19	39	+8.3

Table 20 is the data table for Figure 5.

Von Willebrand Disease, selected other bleeding disorders and acquired defects

Tables 21 - 23 shows the number of people with Welsh postcodes and reported products issued to treat von Willebrand disease, selected other disorders and acquired bleeding disorders during 2019/20, broken down by supplier.

Table 21 Concentrates issued to treat von Willebrand Disease

Manufacturer	Product	Patients (n)	Total Units
CSL Behring	Voncento	36	932,500
LFB Biomedicaments	Willfact /Wilfactin	1 - 2	20,000
Desmopressin	Desmopressin	13	327.2

Units in IU unless otherwise stated
Products containing VWF as well as FVIII are reported in FVIII units

Table 22 Concentrates issued to treat selected other bleeding disorders

Manufacturer	Product	Patients Treated (n)	F.VII Deficiency mg	F.XI Deficiency IU	F.XIII Deficiency IU
BPL	FXI	1 - 2	-	1,000	-
CSL Behring	Fibrogammin P	1 - 2	-	-	18,750
Novo Nordisk	NovoSeven (mg)	1 - 2	1	-	-

Units in IU unless otherwise stated

Table 23 Concentrates issued to treat Acquired Defects

Manufacturer	Product	Patients Treated (n)	Acquired Haemophilia A IU	Acquired von Willebrands IU
CSL Behring	Voncento	1 - 2	-	2,500
Novo Nordisk	NovoEight	1 - 2	66,000	-
Novo Nordisk	NovoSeven (mg)	1 - 2	35	-
Takeda	OBIZUR	1 - 2	84,500	-
	FEIBA	8	498,000	-

Units in IU unless otherwise stated
Products containing VWF as well as FVIII are reported in FVIII units

Table 24 Inhibitors by disease severity

Coagulation Defect	Severity (iu/dl) / Subtype	In Register *	Inhibitors		
			New n (%)	Ongoing n (%)	Historical n (%)
Haemophilia A	< 1	102	1 (1.0)	11 (10.8)	16 (15.7)
	1 - 5	30	0 (0.0)	0 (0.0)	1 (3.3)
	> 5	184	1 (0.5)	2 (1.1)	10 (5.4)
	Total	**316**	**2 (0.6)**	**13 (4.1)**	**27 (8.5)**
Haemophilia B	< 1	14	0 (0.0)	0 (0.0)	0 (0.0)
	1 - 5	24	0 (0.0)	0 (0.0)	0 (0.0)
	> 5	26	0 (0.0)	0 (0.0)	0 (0.0)
	Total	**64**	**0 (0.0)**	**0 (0.0)**	**0 (0.0)**
von Willebrand disease	Type 3	8	0 (0.0)	1 (12.5)	0 (0.0)
	Others	473	0 (0.0)	0 (0.0)	0 (0.0)
	Total	**467**	**0 (0.0)**	**1 (0.2)**	**0 (0.0)**

** Including people not regularly treated*

Table 24 shows the incidence of new inhibitors during 2019/20, the prevalence of those still considered active and those considered inactive inhibitors for haemophilia A, B and von Willebrand disease, broken down by disease severity.

Those labelled “new” were reported for the first time in the year 2019/20. Those labelled “ongoing” are those reported in previous years which have not been eradicated. Those reported as “historical” are those reported to have been previously eradicated or disappeared and not ongoing.

Table 25 Products issued to people with congenital bleeding disorders reported to have a positive inhibitor during 2019/20

Manufacturer	Product	Patients (n)	Units
Haemophilia A			
BPL	Optivate	1 - 2	184,375
Novo Nordisk	NovoSeven (mg)	3	141
Pfizer	ReFacto AF	1 - 2	790,000
Roche	Hemlibra (mg)	1 - 2	4,440
SOBI/Biogen	Elocta	5	1,057,500
Takeda	Advate	1 - 2	676,000
	FEIBA	1 - 2	174,000
von Willebrand Disease			
CSL Behring	Voncento	1 - 2	8,500

Units in IU unless otherwise stated

Table 25 shows the number of people with a Welsh postcode and an inhibitor newly reported or ongoing during 2019/20 plus reported products issued, broken down by diagnosis and supplier.

Table 26 Adverse Events

Adverse Event	Number of Events
Allergy Event	0
Infection Event	0
Inhibitor Event	0
Intracranial haemorrhage	1
Malignancy Event	1
Neurological Event	1
Other Event	1
Poor Efficacy Event	0
Thrombotic Event	0
Total	**4**

See table 24 for breakdown of inhibitors by disease severity in Haemophilia A, B and von Willebrand disease

Table 27 shows the type and number of adverse events reported in people with a Welsh postcode during 2019/20.

Table 27 Causes of Death

Coagulation Defect	Cause of Death	Severity (factor level iu/dl)				
		< 1	1 - 5	> 5	N/K	Total
Haemophilia A Carrier	Cerebral haemorrhage	0	0	1	0	**1**
Acquired Haemophilia A	Carcinoma	0	1	0	0	**1**
	Haemorrhage (misc)	0	0	1	0	**1**
	Ischaemic Heart Disease	1	0	0	0	**1**
	Unknown	1	2	0	0	**3**
von Willebrand disease	Carcinoma					**1**
	Unknown					**3**
F.XI Deficiency	ARDS					**1**
Other Platelet Defects	Unknown					**1**
Total						**13**

Table 28 shows the causes of death reported in people with a Welsh postcode during 2019/20, broken down by diagnosis and disease severity.