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

Bleeding Disorder Statistics for Wales

April 2018 to March 2019

A report from the UK National Haemophilia Database

December 2019

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.

Health board Boundary Changes

Since the last report, there has been a boundary change between Abertawe Bro Morgannwg University Health Board and Cwm Taf University Health Board to create Cwm Taf Morgannwg University Health Board and Swansea Bay University Health Board.

This has affected tables 4, 12, 13, 18 and 19.

Contents

New Registrations

Table 1 New registrations - Number of new registrations between April 2018 & March 2019, by diagnosis and gender

Coagulation Defect	Male	Female	Total
Haemophilia A	8	0	8
Haemophilia A Carrier		3	3
Acquired Haemophilia A	4	4	8
Haemophilia A with Liver Transplant	1	0	1
Haemophilia B Carrier	0	4	4
von Willebrand disease	11	15	26
Probable von Willebrands disease	1	2	3
Acquired von Willebrands	1	0	1
F.VII deficiency	2	1	3
F.XI Deficiency	1	10	11
Dysfibrinogenemia	1	6	7
Hypofibrinogenemia	1	2	3
Prothrombin Deficiency	1	0	1
Platelet defect	6	16	22
Unclassified	1	11	12
Total	**39**	**74**	**113**

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 2018 & March 2019, 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	3	1	1	5
	10 : 19	0	0	0	0
	20 : 29	0	0	1	1
	30 : 39	0	0	0	0
	40 : 49	0	0	2	2
	50 : 59	0	0	0	0
	60 : 69	0	0	0	0
	70 +	0	0	0	0
	Total	**3**	**1**	**4**	**8**
Haemophilia B	0 : 9	0	0	0	0
	10 : 19	0	0	0	0
	20 : 29	0	0	0	0
	30 : 39	0	0	0	0
	40 : 49	0	0	0	0
	50 : 59	0	0	0	0
	60 : 69	0	0	0	0
	70 +	0	0	0	0
	Total	**0**	**0**	**0**	**0**

N.B Age calculated at mid-year, 30/09/2018

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 2019 and the number treated between April 2018 & March 2019

Coagulation Defect	In Register			Treated (n)	Treated %
	Males	Females	Total		
Haemophilia A	295	1	296	155	52.36%
Haemophilia A Carrier		69	69	4	5.80%
Haemophilia A with Liver Transplant	1	0	1	0	0.00%
Acquired Haemophilia A	16	9	25	3	12.00%
Haemophilia B	57	0	57	32	56.14%
Haemophilia B Carrier		20	20	2	10.00%
von Willebrand disease	162	293	455	48	10.55%
Acquired von Willebrands	6	0	6	2	33.33%
Probable von Willebrands disease	3	5	8	0	0.00%
Platelet-type Pseudo von Willebrand Disease	3	2	5	0	0.00%
F.V deficiency	1	3	4	0	0.00%
F.VII deficiency	17	20	37	1	2.70%
F.X deficiency	0	2	2	0	0.00%
F.XI Deficiency	44	72	116	0	0.00%
F.XIII Deficiency	0	1	1	1	100.00%
Combined II+VII+IX+X Deficiency	1	0	1	0	0.00%
Co-inherited diagnoses	1	9	10	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	21	30	1	3.33%
Hypofibrinogenemia	5	7	12	1	8.33%
Hypodysfibrinogenemia	1	1	2	0	0.00%
Glanzmanns Thrombasthenia	2	2	4	0	0.00%
Bernard Soulier	0	2	2	1	50.00%
Platelet Defect	51	105	156	6	3.85%
Miscellaneous	3	3	6	1	16.67%
Unclassified	5	39	44	2	4.55%
Totals	**686**	**687**	**1,373**	**261**	

Table 3 shows the total number of active registrations of people with a Welsh postcode and the number who were issued treatment during 2018/19.

Table 4 In Register – The total number of people in the register as of 31st March 2019, 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	54	53	42	31	7	50	296
Haemophilia A with Liver Transplant	0	0	0	1	0	0	0	1
Acquired Haemophilia A	3	5	6	5	4	1	1	25
Haemophilia A Carrier	10	15	10	5	5	0	24	69
Haemophilia B	17	7	12	13	2	1	5	57
Haemophilia B Carrier	5	1	5	3	1	2	3	20
von Willebrand disease	75	158	80	34	31	56	21	455
Acquired von Willebrands	3	0	2	0	0	0	1	6
Probable von Willebrands disease	2	2	2	0	1	1	0	8
Platelet-type Pseudo von Willebrand Disease	0	0	0	0	2	3	0	5
Prothrombin Deficiency	0	0	0	1	1	0	0	2
F.V deficiency	2	0	0	0	1	0	1	4
F.VII deficiency	6	11	3	4	8	1	4	37
F.X deficiency	0	1	1	0	0	0	0	2
F.XI Deficiency	52	13	13	10	10	2	16	116
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	2	2	0	0	2	2	10
Acquired Prothrombin Deficiency	0	0	1	0	0	0	0	1
Afibrinogenemia	0	0	0	1	0	0	0	1
Dysfibrinogenemia	7	2	16	3	0	0	2	30
Hypofibrinogenemia	2	1	4	3	1	0	1	12
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
Platelet Defect	23	37	33	31	7	4	21	156
Miscellaneous	1	2	1	2	0	0	0	6
Unclassified	13	3	12	9	2	1	4	44
Total	**282**	**316**	**261**	**167**	**109**	**81**	**157**	**1,373**

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 (May 2018) 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 2019, 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	29	12	21	**62**
	≥18 years	59	18	157	**234**
Total		**88**	**30**	**178**	**296**
Haemophilia B	<18 years	1	7	5	**13**
	≥18 years	11	15	18	**44**
Total		**12**	**22**	**23**	**57**

N.B Age calculated at mid-year, 30/09/2018

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 selected rarer bleeding disorders in the register as of 31st March 2019 and the number treated between April 2018 & March 2019, 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	-	36	1	-	-	37	1
F.X deficiency	-	-	2	-	-	-	2	-
F.XI Deficiency	7	-	109	-	-	-	116	-
Total	**8**	**-**	**151**	**1**	**-**	**-**	**159**	**1**

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 selected rarer bleeding disorders and a Welsh postcode known to the NHD during 2018/19. It is acknowledged that these rarer 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 2019 and the number treated between April 2018 & March 2019, 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	4	9	6	1	**20**	15	32	10	0	**57**	**77**	**8**
Type 2A	3	0	0	0	**3**	10	2	2	0	**14**	**17**	**4**
Type 2B	1	0	0	0	**1**	0	2	0	0	**2**	**3**	**1**
Type 2M	0	0	0	0	**0**	4	4	0	0	**8**	**8**	**1**
Type 2N	0	0	0	0	**0**	0	0	0	0	**0**	**0**	**0**
Type 2 Unspecified	0	1	0	0	**1**	1	1	1	0	**3**	**4**	**0**
Type 3	3				**3**	3				**3**	**6**	**5**
Type Unreported	1	1	1	1	**4**	10	10	12	0	**32**	**36**	**8**
Low VWF	1	0	2	0	**3**	0	1	7	0	**8**	**11**	**0**
										Sub Total Males	**162**	**27**
Females												
Type 1	5	10	4	0	**19**	20	45	36	0	**101**	**120**	**8**
Type 2A	1	1	0	0	**2**	11	9	5	0	**25**	**27**	**4**
Type 2B	0	0	0	0	**0**	0	1	5	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	6	0	**6**	**7**	**1**
Type 2 Unspecified	0	2	1	0	**3**	2	2	0	0	**4**	**7**	**0**
Type 3	0				**0**	2				**2**	**2**	**0**
Type Unreported	3	5	4	0	**12**	10	16	45	4	**75**	**87**	**7**
Low VWF	0	0	1	0	**1**	0	0	19	0	**19**	**20**	**1**
										Sub Total Females	**293**	**21**
										Grand Total - Males and Females	**455**	**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 2018 & March 2019 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	21
	London	5
	Wales	133
	Wessex	1
Haemophilia A Carrier	Wales	4
Acquired Haemophilia A	Birmingham & Black Country	1
	Wales	2
Haemophilia B	Birmingham & Black Country	1
	Cheshire, Warrington & Wirral	3
	Wales	28
Haemophilia B Carrier	Wales	2
von Willebrand disease	Cheshire, Warrington & Wirral	3
	London	1
	Wales	46
Acquired von Willebrands	Wales	2
F.VII deficiency	Wales	1
F.XIII Deficiency	Wales	1
Afibrinogenemia	Wales	1
Dysfibrinogenemia	Wales	1
Hypofibrinogenemia	Cheshire, Warrington & Wirral	1
Bernard Soulier	Cheshire, Warrington & Wirral	1
Platelet defect	Cheshire, Warrington & Wirral	1
	Wales	5
Miscellaneous	Wales	1
Unclassified bleeding disorder	Wales	2
	Grand total	**269**

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 region which 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 2018 & March 2019, by diagnosis, all severities

Coagulation Defect	Patient's home postcode region	Patients Registered (n)	Patients Treated (n)
Haemophilia A	North of England	1	0
	Midlands and East of England	4	2
	London	1	0
	South East	2	2
	South West	8	4
	Sub total	**16**	**8**
Haemophilia A Carrier	London	2	0
	South West	2	0
	Sub total	**4**	**0**
Acquired Haemophilia A	Midlands and East of England	1	1
		1	**1**
Haemophilia B	South East	1	0
	Sub total	**1**	**0**
von Willebrand disease	North of England	1	1
	Midlands and East of England	6	0
	South West	3	1
	Scotland	1	0
	Sub total	**11**	**2**
F.XI Deficiency	South West	2	0
Co-inherited diagnoses	Midlands and East of England	1	1
Platelet Defect	South West	1	0
Platelet Defect	Scotland	4	0
Unclassified bleeding disorder	South West	1	0
	Grand total	**42**	**12**

Those reported in Table 9 were registered at or issued treatment from a Welsh Haemophilia Centre during 2018/19, 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	Investigational	Total
Haemophilia A	146	361,880	18,150,500	4,351,500	7,875	22,863,880
Haemophilia A Carrier	3	-	34,250	-	-	34,250
Acquired Haemophilia A	1	-	284,000	-	-	284,000
Acquired von Willebrands	2	20,500	-	-	-	20,500
von Willebrand disease	37	1,089,000	-	-	-	1,089,000
Platelet Defect	1	2,000	-	-	-	2,000
Total	**190**	**1,473,380**	**18,468,750**	**4,351,500**	**7,875**	**24,293,630**

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 2018/19. Also shown are the number of units issued broken down by diagnosis and product type.

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

Manufacturer	Product	Total Units
Bayer	Kogenate	585,500
BPL	Optivate	361,880
CSL Behring	Helixate Nexgen	39,500
Novo Nordisk	NovoEight	3,019,250
	NovoSeven (mg)	924
Octapharma	Nuwiq	1,017,000
Pfizer	ReFacto AF	7,841,500
Roche	Hemlibra (mg)	3,060
SOBI/Biogen	Elocta	4,351,500
Takeda	FEIBA	11,000
	Advate	5,647,750
	Investigational FVIII	7,875

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	10	3,739,380	373,938
Birmingham (Queen Elizabeth)	Swansea Bay University Health Board	1	259,500	259,500
Cardiff	Aneurin Bevan University Health Board	14	3,735,000	266,786
	Cardiff and Vale University Health Board	10	1,914,250	191,425
	Cwm Taf Morgannwg University Health Board	14	3,306,750	236,196
	Hywel Dda University Health Board	7	1,412,000	201,714
	Powys Teaching Health Board	1	368,000	368,000
	Swansea Bay University Health Board	7	698,375	99,768
Hammersmith Hospital, London	Cardiff and Vale University Health Board	1	4,000	4,000
Liverpool Adult's	Betsi Cadwaladr University Health Board	7	2,088,000	298,286
Manchester Children's	Betsi Cadwaladr University Health Board	6	641,500	106,917
Swansea	Hywel Dda University Health Board	3	299,000	99,667
	Swansea Bay University Health Board	5	906,500	181,300
	Totals	**86**	**19,372,255**	**225,259**

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 2018/19. 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: Three 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	Patients treated (n)	Total FVIII Units (IU)	Mean Usage	FVIII Units Per Capita
Powys Teaching Health Board	132,447	1	368,000	368,000	2.78
Betsi Cadwaladr University Health Board	698,369	23	6,468,880	281,256	9.26
Aneurin Bevan University Health Board	591,225	14	3,735,000	266,786	6.32
Cwm Taf Morgannwg University Health Board	445,190	14	3,306,750	236,196	7.43
Cardiff and Vale University Health Board	496,413	10	1,918,250	191,825	3.86
Hywel Dda University Health Board	385,615	9	1,711,000	190,111	4.44
Swansea Bay University Health Board	389,372	12	1,864,375	155,365	4.79
Wales	**3,138,631**	**83**	**19,372,255**	**1,689,538**	**6.17**

Ranked by mean usage

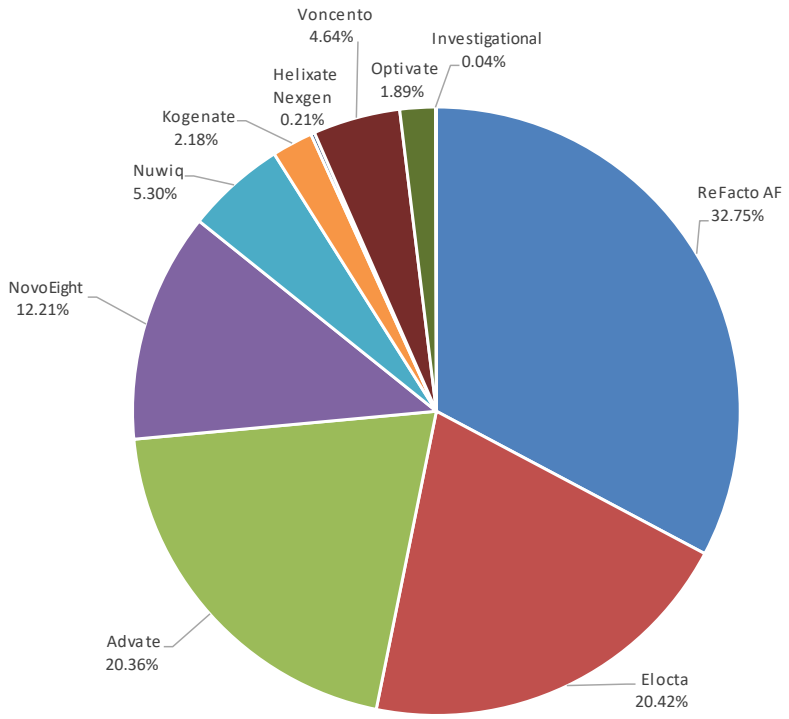
Mid-2018 population estimates, Office for National Statistics. Published by the Welsh Government, under the Open Government Licence v3.0. Updated June 2019

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. Peoples are allocated to a Health Board based on their home postcode.

Figure 1 Market share of factor VIII concentrates issued between April 2018 & March 2019



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

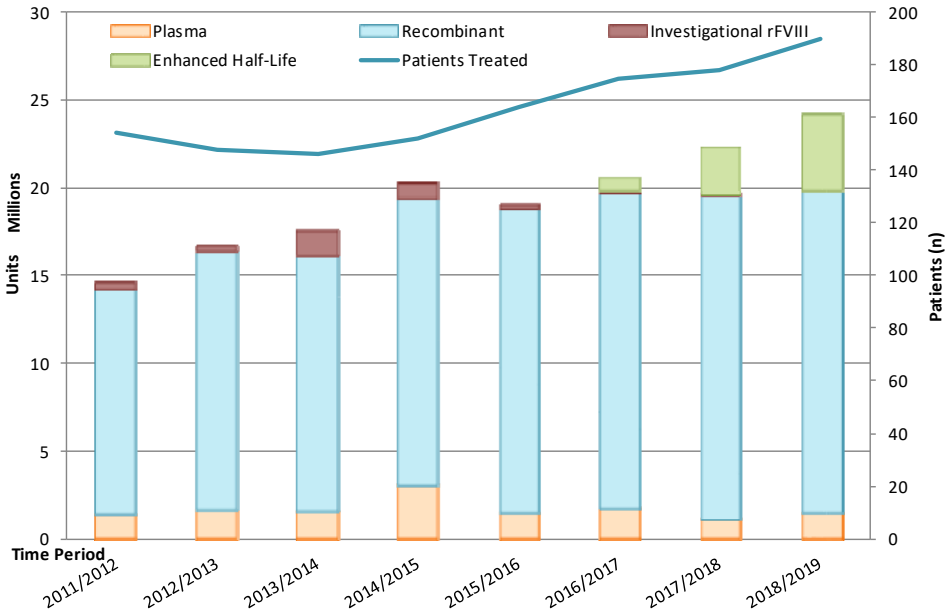
This pie chart and table are arranged in descending order of recombinant products by volume, then descending order of plasma products by volume

Manufacturer	Product	Units (IU)	Patients (n)
Pfizer	ReFacto AF	6,279,500	56
SOBI/Biogen	Elocta	3,914,500	20
Takeda	Advate	3,904,000	56
Novo Nordisk	NovoEight	2,340,250	18
Octapharma	Nuwiq	1,017,000	2
Bayer	Kogenate	417,500	2
CSL Behring	Helixate Nexgen	39,500	2
CSL Behring	Voncento	890,000	40
BPL	Optivate	361,880	1
	Investigational	7,875	1
	Total	**19,172,005**	**198**

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.

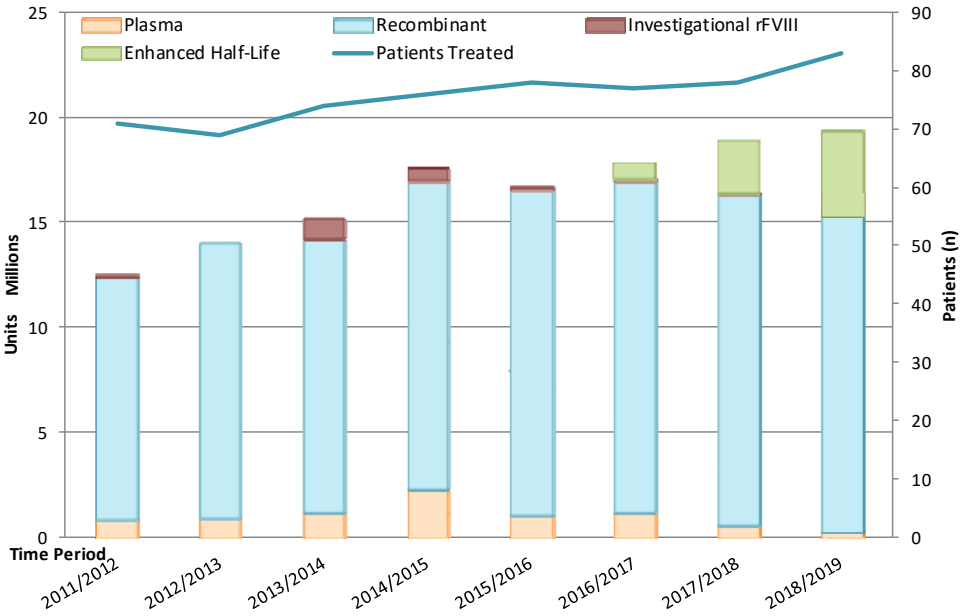
NOTE: The number of people in this table cannot be compared with table 10 as this table includes people treated with more than one product type. The numbers in Table 10 do not contain duplicates.

Figure 2 Factor VIII units issued by financial year between April 2011 & March 2019 – 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 2011 & March 2019 – *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 2011/12 and 2018/19 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 2011 & March 2019 - all diagnoses

Year	Plasma		Recombinant		Investigational rFVIII		Enhanced Half-Life		Total		Patients Treated	
	IU	% difference since 2011/12	IU	% difference since 2011/12	IU	% difference since 2011/12	IU	% difference since 2016/17	IU	% difference since 2011/12	n	% difference since 2011/12
2011/2012	1,450,395	1.00	12,845,488	1.00	409,010	1.00	0		14,704,893	1.00	154	1.00
2012/2013	1,670,650	1.15	14,743,592	1.15	359,500	0.88	0		16,773,742	1.14	148	0.96
2013/2014	1,612,820	1.11	14,528,500	1.13	1,484,500	3.63	0		17,625,820	1.20	146	0.95
2014/2015	3,042,500	2.10	16,373,500	1.27	943,170	2.31	0		20,359,170	1.38	152	0.99
2015/2016	1,522,500	1.05	17,347,250	1.35	176,000	0.43	0		19,045,750	1.30	164	1.06
2016/2017	1,785,975	1.23	18,025,250	1.40	134,875	0.33	794,500	1.00	20,740,600	1.41	175	1.14
2017/2018	1,213,910	0.84	18,417,750	1.43	76,020	0.19	2,668,000	3.36	22,375,680	1.52	178	1.16
2018/2019	1,473,380	1.02	18,468,750	1.44	7,875	0.02	4,351,500	5.48	24,301,505	1.65	190	1.23

Table 15 Data table for Figure 3 - Factor VIII units by financial year between April 2011 & March 2019 - *Severe Haemophilia A*

Year	Plasma		Recombinant		Investigational rFVIII		Enhanced Half-Life		Total		Patients Treated	
	IU	% difference since 2011/12	IU	% difference since 2011/12	IU	% difference since 2011/12	IU	% difference since 2016/17	IU	% difference since 2011/12	n	% difference since 2011/12
2011/2012	930,000	1.00	11,457,738	1.00	121,000	1.00	0		12,508,738	1.00	71	1.00
2012/2013	1,009,150	1.09	12,995,592	1.13	0	0	0		14,004,742	1.12	69	0.97
2013/2014	1,275,000	1.37	12,929,750	1.13	964,000	7.97	0		15,168,750	1.21	74	1.04
2014/2015	2,291,500	2.46	14,622,250	1.28	604,170	4.99	0		17,517,920	1.40	76	1.07
2015/2016	1,135,000	1.22	15,328,000	1.34	176,000	1.45	0		16,639,000	1.33	78	1.10
2016/2017	1,236,475	1.33	15,692,750	1.37	134,875	1.11	768,500	1.00	17,832,600	1.43	77	1.08
2017/2018	647,310	0.70	15,655,000	1.37	76,020	0.63	2,455,000	3.19	18,833,330	1.51	78	1.10
2018/2019	361,880	0.39	14,950,000	1.30	7,875	0.07	4,052,500	5.27	19,372,255	1.55	83	1.17

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	32	-	1,861,500	768,000	-	2,629,500
Haemophilia B Carrier	2	-	10,500	-	-	10,500
Total	**34**	**-**	**1,872,000**	**768,000**	**-**	**2,640,000**

Table 16 shows the number of people with a Welsh postcode who were issued factor IX concentrate during 2018/19. 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	Total Units
CSL Behring	IDELVION	68,000
NovoNordisk	Refixia	121,000
Pfizer	BeneFIX	1,735,500
SOBI/Biogen	ALPROLIX	579,000
Takeda	RIXUBIS	126,000

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 postcide	Severe Haemophilia B		
		Patients treated (n)	Total FIX Units	Mean Usage
Cardiff	Aneurin Bevan University Health Board	3	408,000	136,000
	Cwm Taf Morgannwg University Health Board	4	810,000	202,500
	Hywel Dda University Health Board	1	20,000	20,000
	Swansea Bay University Health Board	1	113,000	113,000
Swansea	Hywel Dda University Health Board	1	7,000	7,000
	Swansea Bay University Health Board	1	124,000	124,000
	Totals	**11**	**1,482,000**	**134,727**

Table 18 reports the number of people with severe haemophilia B treated and the number of units of factor IX issued during 2018/19. 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	Patients treated (n)	Total FIX Units (IU)	Mean Usage	FIX Units Per Capita
Cwm Taf Morgannwg University Health Board	445,190	4	810,000	202,500	1.82
Aneurin Bevan University Health Board	591,225	3	408,000	136,000	0.69
Swansea Bay University Health Board	389,372	2	237,000	118,500	0.61
Hywel Dda University Health Board	385,615	1	27,000	27,000	0.07
Betsi Cadwaladr University Health Board	698,369	0	0	-	0.00
Cardiff and Vale University Health Board	496,413	0	0	-	0.00
Powys Teaching Health Board	132,447	0	0	-	0.00
Wales	**3,138,631**	**10**	**1,482,000**	**484,000**	**3.19**

Ranked by mean usage

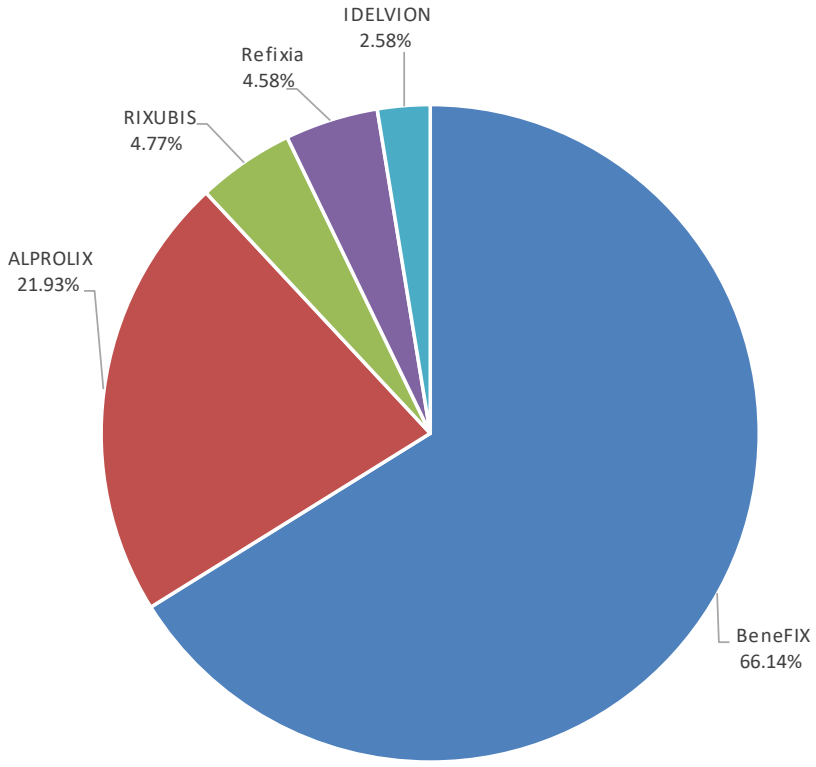
Mid-2018 population estimates, Office for National Statistics. Published by the Welsh Government, under the Open Government Licence v3.0. Updated June 2019

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 2018 & March 2019



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	Units (IU)	Patients (n)
Pfizer	BeneFIX	1,746,000	27
SOBI/Biogen	ALPROLIX	579,000	5
Shire	RIXUBIS	126,000	2
NovoNordisk	Refixia	121,000	2
CSL Behring	IDELVION	68,000	1
	Total	**2,640,000**	**37**

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 2011 & March 2019 - all diagnoses, all severities

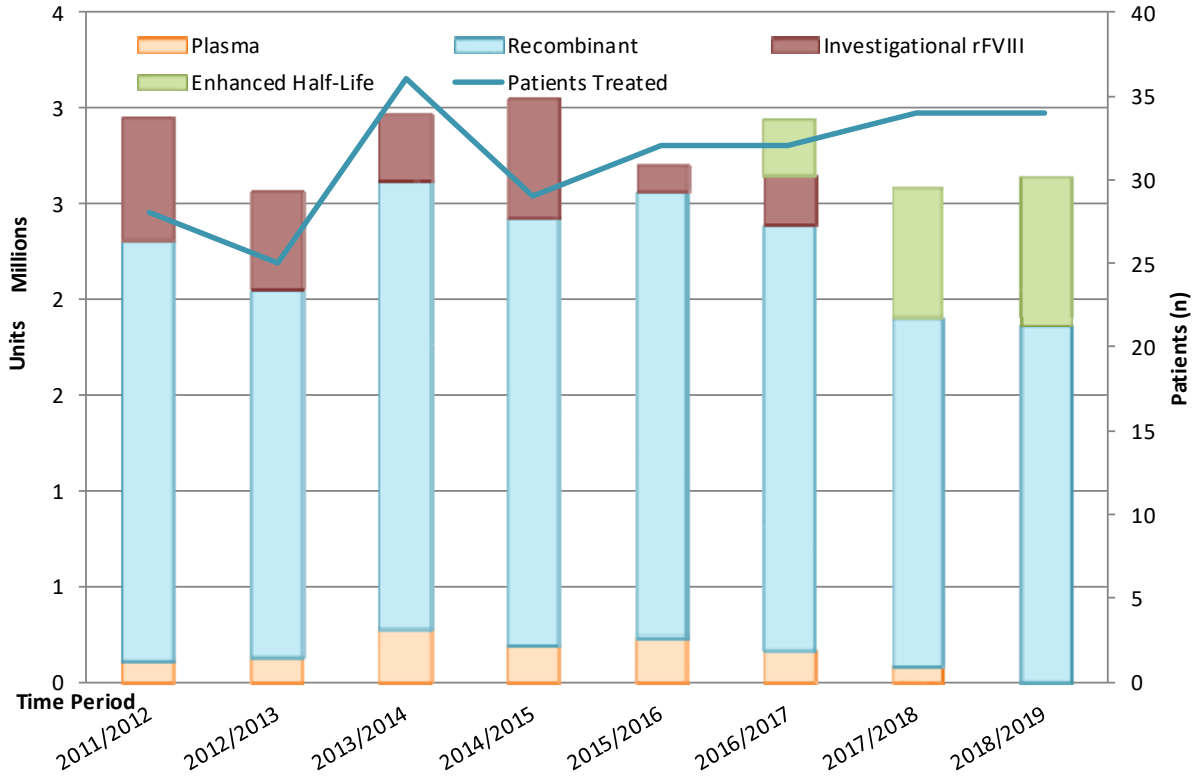


Figure 5 gives an historical view of the number of factor IX units issued between 2011/12 and 2018/19 for all diagnoses. The number of people treated is represented by the blue line using a secondary axis.

Table 20 Data table for figure 5 - Factor IX units issued by financial year between April 2011 & March 2019 – all diagnoses

Year	Plasma		Recombinant		Investigational rFVIII		Enhanced Half-Life		Total		Patients Treated	
	IU	% difference since 2011/12	IU	% difference since 2011/12	IU	% difference since 2011/12	IU	% difference since 2016/17	IU	% difference since 2011/12	n	% difference since 2011/12
2011/2012	106,000	1.00	2,203,500	1.00	647,689	1.00	0		2,957,189	1.00	28	1.00
2012/2013	133,000	1.25	1,915,500	0.87	517,180	0.80	0		2,565,680	0.87	25	0.89
2013/2014	274,000	2.58	2,346,770	1.07	349,016	0.54	0		2,969,786	1.00	36	1.29
2014/2015	198,000	1.87	2,229,500	1.01	617,447	0.95	0		3,044,947	1.03	29	1.04
2015/2016	228,000	2.15	2,338,000	1.06	136,553	0.21	0		2,702,553	0.91	32	1.14
2016/2017	170,000	1.60	2,220,000	1.01	263,692	0.41	289,015	1.00	2,942,707	1.00	32	1.14
2017/2018	78,000	0.74	1,828,500	0.83	0	0.00	678,500	2.35	2,585,000	0.87	34	1.21
2018/2019	0	0.00	1,872,000	0.85	0	0.00	768,000	2.66	2,640,000	0.89	34	1.21

Table 20 is the data table for Figure 5.

Von Willebrand Disease, Rarer 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 rarer disorders and acquired bleeding disorders during 2018/19, broken down by supplier.

Table 21 Concentrates issued to treat von Willebrand Disease

Manufacturer	Product / Patients (n)	Total Units
CSL Behring	Voncento (n= 37)	1,089,000
LFB Biomedicaments	Willfact /Wilfactin (n= 2)	30,000
Desmopressin	Desmopressin (n= 13)	362.5

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

Table 22 Concentrates issued to treat Rarer Bleeding Disorders

Manufacturer	Product / Patients (n)	F.VII Deficiency	F.XIII Deficiency
CSL Behring	Fibrogammin P (n= 1)	-	10,000
Novo Nordisk	NovoSeven (mg) (n= 1)	1	-

Units in IU unless otherwise stated

Table 23 Concentrates issued to treat Acquired Defects

Manufacturer	Product / Patients (n)	Acquired Haemophilia A	Acquired von Willebrands
CSL Behring	Voncento (n= 2)	-	20,500
Pfizer	ReFacto AF (n= 1)	284,000	-
Takeda	FEIBA (n= 3)	324,000	-

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	88	2 (2.3)	12 (13.6)	13 (14.8)
	1 - 5	30	0 (0.0)	0 (0.0)	1 (3.3)
	> 5	178	0 (0.0)	2 (1.1)	7 (3.9)
	Total	**296**	**2 (0.7)**	**14 (4.7)**	**21 (7.1)**
Haemophilia B	< 1	12	0 (0.0)	0 (0.0)	0 (0.0)
	1 - 5	22	0 (0.0)	0 (0.0)	0 (0.0)
	> 5	23	0 (0.0)	0 (0.0)	0 (0.0)
	Total	**57**	**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	447	0 (0.0)	0 (0.0)	0 (0.0)
	Total	**455**	**0 (0.0)**	**1 (0.2)**	**0 (0.0)**

** Including people not regularly treated*

Table 24 shows the incidence of new inhibitors during 2018/19, 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 2018/19. 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 2018/19

Manufacturer	Product / Patients (n)	Units
Haemophilia A		
BPL	Optivate (n= 1)	361,880
CSL Behring	Helixate Nexgen (n= 1)	28,000
Novo Nordisk	NovoSeven (mg) (n= 6)	924
Roche	Hemlibra (mg) (n= 3)	3,060
SOBI/Biogen	Elocta (n= 6)	2,009,000
Takeda	Advate (n= 4)	646,000
	FEIBA (n= 1)	11,000
von Willebrand Disease		
CSL Behring	Voncento (n= 1)	77,000

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 2018/19 plus reported products issued, broken down by diagnosis and supplier.

Table 26 Adverse Events

Adverse Event	Number of Events
Allergy Event	0
Infection Event	1
Inhibitor Event	2
Intracranial haemorrhage	1
Malignancy Event	0
Other Event	1
Poor Efficacy Event	1
Thrombotic Event	0
Total	**6**

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 2018/19.

Table 27 Causes of Death

Coagulation Defect	Cause of Death	Severity (factor level iu/dl)				
		< 1	1 - 5	> 5	N/K	Total
Haemophilia A	Unknown	0	0	2	0	**2**
Haemophilia A Carrier	Unknown	0	0	1	0	**1**
Acquired Haemophilia A	Ischaemic Heart Disease	0	0	1	0	**1**
	Unknown	2	0	0	0	**2**
von Willebrand disease	Carcinoma	0	0	1	0	**1**
	Unknown	0	0	7	0	**7**
F.XI Deficiency	Unknown	0	0	1	0	**1**
	Total	**2**	**0**	**13**	**0**	**15**

Table 28 shows the causes of death reported in people with a Welsh postcode during 2018/19, broken down by diagnosis and disease severity.