Bleeding Disorder Statistics for Wales

April 2015 to March 2016

A report from the UK National Haemophilia Database

June 2017

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

Please note: ISTH classification for severity of haemophilia A and B is used in this report.

	Severe	**Moderate**	**Mild**
IU/ml	< 0.01	0.01 - 0.05	> 0.05
% or IU/dl	< 1	1 - 5	> 50

Contents

New Registrations

Table 1 New Registrations - Number of patients newly registered between April 2015 & March 2016 showing their coagulation defect and gender

Coagulation Defect	Male	Female	Total
Haemophilia A	11	0	11
Haemophilia A Carrier		8	8
Females with VIII deficiency		3	3
Haemophilia B	1	0	1
von Willebrand disease	10	8	18
Probable von Willebrands disease	2	1	3
F.VII deficiency	1	1	2
F.XI Deficiency	5	3	8
Dysfibrinogenemia	1	3	4
Hypofibrinogenemia	2	2	4
Platelet defects	4	11	15
Unclassified	3	5	8
Total	**40**	**45**	**85**

Table 2 New Registrations of Haemophilia A & B between April 2015 & March 2016, 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	0	1	4
	10 : 19	0	0	2	2
	20 : 29	1	0	2	3
	30 : 39	0	0	1	1
	40 : 49	0	0	0	0
	50 : 59	0	0	0	0
	60 : 69	0	0	0	0
	70 +	0	0	1	1
Total		**4**	**0**	**7**	**11**
Haemophilia B	0 : 9	0	1	0	1
	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**	**1**	**0**	**1**

N.B Age calculated at mid-year, 30/09/2015

In Register

Table 3 In Register - The total number of registered patients with all types of bleeding disorder as of 31st March 2016 and the number treated between April 2015 & March 2016

Coagulation Defect	In Register			Treated (n)	Treated %
	Males (n)	Females (n)	Total In Reg		
Haemophilia A	268	1	269	134	49.81%
Acquired Haemophilia A	8	5	13	0	0.00%
Females with VIII deficiency		35	35	3	8.57%
Haemophilia A Carrier		14	14	1	7.14%
Haemophilia B	56	0	56	31	55.36%
Females with IX deficiency		14	14	0	0.00%
von Willebrand disease	152	254	406	47	11.58%
Acquired von Willebrands	2	0	2	0	0.00%
Platelet-type Pseudo von Willebrand Disease	1	0	1	0	0.00%
Probable von Willebrands disease	2	2	4	0	0.00%
F.V deficiency	1	3	4	0	0.00%
F.VII deficiency	15	16	31	1	3.23%
F.X deficiency	0	1	1	0	0.00%
F.XI Deficiency	35	51	86	1	1.16%
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	1	10.00%
Fibrinogen Deficiency	1	1	2	0	0.00%
Afibrinogenemia	2	0	2	2	100.00%
Dysfibrinogenemia	7	15	22	0	0.00%
Hypofibrinogenemia	2	5	7	1	14.29%
Glanzmanns Thrombasthenia	0	1	1	0	0.00%
Bernard Soulier	0	2	2	0	0.00%
Platelet defects	31	60	91	1	1.10%
Miscellaneous	3	4	7	0	0.00%
Unclassified	4	10	14	0	0.00%
Totals	**592**	**504**	**1,096**	**224**	

Table 4 In Register - The total number of patients with Haemophilia A & B currently in the register, by severity and age group

Coagulation Defect	Age Range	Factor level iu/dl			Total
		< 1	1 - 5	> 5	
Haemophilia A	<18 years	22	10	25	**57**
	≥18 years	57	22	133	**212**
Sub Total		**79**	**32**	**158**	**269**
Haemophilia B	<18 years	1	5	7	**13**
	≥18 years	11	15	17	**43**
Sub Total		**12**	**20**	**24**	**56**

Table 5 In Register - The total number of patients currently in the register, by diagnosis and Health Board

Coagulation Defect	Abertawe Bro Morgannwg University	Aneurin Bevan	Betsi Cadwaladr University	Cardiff and Vale University	Cwm Taf	Hywel Dda	Powys Teaching	Total
Haemophilia A	64	53	46	51	24	24	7	**269**
Acquired Haemophilia A	4	2	2	4	0	1	0	**13**
Females with VIII deficiency	12	5	10	4	2	1	1	**35**
Haemophilia A Carrier	4	2	5	3	0	0	0	**14**
Haemophilia B	6	16	7	11	12	3	1	**56**
Females with IX deficiency	2	4	1	4	2	0	1	**14**
von Willebrand disease	26	73	146	63	18	29	51	**406**
Acquired von Willebrands	0	2	0	0	0	0	0	**2**
Platelet-type Pseudo von Willebrand Disease	0	0	0	0	0	0	1	**1**
Probable von Willebrands disease	0	2	1	1	0	0	0	**4**
F.V deficiency	1	2	0	0	0	1	0	**4**
F.VII deficiency	7	2	14	1	0	7	0	**31**
F.X deficiency	0	0	1	0	0	0	0	**1**
F.XI Deficiency	13	43	11	6	6	7	0	**86**
F.XIII Deficiency	0	0	1	0	0	0	0	**1**
Combined II+VII+IX+X Deficiency	0	0	0	1	0	0	0	**1**
Co-inherited diagnoses	2	2	2	2	0	0	2	**10**
Fibrinogen Deficiency	1	0	1	0	0	0	0	**2**
Afibrinogenemia	0	0	1	0	1	0	0	**2**
Dysfibrinogenemia	4	1	1	16	0	0	0	**22**
Hypofibrinogenemia	0	2	1	2	1	1	0	**7**
Glanzmanns Thrombasthenia	0	0	0	0	0	1	0	**1**
Bernard Soulier	0	0	1	1	0	0	0	**2**
Platelet defects	13	17	21	23	11	2	4	**91**
Miscellaneous	1	1	2	1	1	0	1	**7**
Unclassified	3	3	1	3	2	2	0	**14**
Total	**163**	**232**	**276**	**197**	**80**	**79**	**69**	**1,096**

Table 6 In Register - The total number of patients with Von Willebrand Disease currently in the register and the number treated between April 2015 & March 2016, by disease severity, age group and gender

von Willebrand disease	<18 years (VWD Activity iu/dl)					≥18 years (VWD Activity iu/dl)					Total	Treated
	<10	10 - 29	≥30	N/K	Sub Total	<10	10 - 29	≥30	N/K	Sub Total		
Males												
Type 1	3	9	7	1	**20**	16	29	18	0	**63**	**83**	**6**
Type 2A	1	0	0	0	**1**	8	0	3	0	**11**	**12**	**1**
Type 2B	1	0	0	0	**1**	0	1	0	0	**1**	**2**	**1**
Type 2M	0	0	0	0	**0**	2	2	0	0	**4**	**4**	**1**
Type 2N	0	0	0	0	**0**	0	0	0	0	**0**	**0**	**0**
Type 2 Unspecified	0	0	0	0	**0**	1	0	0	0	**1**	**1**	**0**
Type 3	4				**4**	1				**1**	**5**	**5**
Type Unreported	1	1	3	1	**6**	13	14	12	0	**39**	**45**	**7**
Sub Total Males											**152**	**21**
Females												
Type 1	5	5	2	0	**12**	16	27	55	0	**98**	**110**	**10**
Type 2A	0	1	0	0	**1**	11	5	6	0	**22**	**23**	**4**
Type 2B	0	0	0	0	**0**	0	1	5	0	**6**	**6**	**1**
Type 2M	0	3	0	0	**3**	4	3	2	0	**9**	**12**	**0**
Type 2N	0	0	0	0	**0**	0	0	4	0	**4**	**4**	**1**
Type 2 Unspecified	0	0	0	0	**0**	1	1	0	0	**2**	**2**	**0**
Type 3	0				**0**	2				**2**	**2**	**1**
Type Unreported	2	4	3	1	**10**	14	17	49	5	**85**	**95**	**9**
Sub Total Females											**254**	**26**
Grand Total - Males and Females											**406**	**47**

Table 7 In Register - The number of patients with selected rarer bleeding disorders currently registered and the number treated between April 2015 & March 2016, 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	0	0	4	0	0	0	4	0
F.VII deficiency	1	0	30	1	0	0	31	1
F.X deficiency	0	0	1	0	0	0	1	0
F.XI Deficiency	2	0	79	1	5	0	86	1
Total	**3**	**-**	**114**	**2**	**5**	**-**	**122**	**2**

Coagulation Defect	<2		≥2		N/K		Total	
	In Reg	Treated	In Reg	Treated	In Reg	Treated	In Reg	Treated
F.XIII Deficiency	0	0	1	1	0	0	1	1
Total	**-**	**-**	**1**	**1**	**-**	**-**	**1**	**1**

Table 8 Inhibitors by disease severity

Coagulation Defect	Severity (iu/dl) / Subtype	In Register *	Inhibitors		
			Newly Reported n (%)	Ongoing n (%)	Historical n (%)
Haemophilia A	< 1	79	1 (1.3)	8 (10.1)	23 (29.1)
	1 - 5	32	0 (0.0)	1 (3.1)	3 (9.4)
	> 5	158	0 (0.0)	0 (0.0)	8 (5.1)
	Total	**269**	**1 (0.4)**	**9 (3.3)**	**34 (12.6)**
Haemophilia B	< 1	12	0 (0.0)	0 (0.0)	0 (0.0)
	1 - 5	20	0 (0.0)	0 (0.0)	0 (0.0)
	> 5	24	0 (0.0)	0 (0.0)	0 (0.0)
	Total	**56**	**0 (0.0)**	**0 (0.0)**	**0 (0.0)**
von Willebrand disease	Type 3	7	0 (0.0)	1 (14.3)	1 (14.3)
	Others	399	0 (0.0)	0 (0.0)	0 (0.0)
	Total	**406**	**0 (0.0)**	**1 (0.2)**	**1 (0.2)**

** Including patients not regularly treated*

Treatment

Table 9 Patients with a Welsh postcode, treated between April 2015 & March 2016, all severities, by diagnosis and treatment region

Coagulation Defect	Region	Patients Treated (n)
Haemophilia A	Cheshire, Warrington & Wirral	14
	London	3
	Surrey & Sussex	1
	Wales	**119**
	Wessex	1
Haemophilia B	Birmingham & Black Country	1
	Cheshire, Warrington & Wirral	4
	Wales	**26**
von Willebrand disease	Birmingham & Black Country	2
	Cheshire, Warrington & Wirral	1
	London	1
	Wales	**44**
Haemophilia A Carrier	Cheshire, Warrington & Wirral	**1**
	Wales	**3**
F.VII deficiency	**Wales**	**1**
F.XI Deficiency	**Wales**	**1**
F.XIII Deficiency	Cheshire, Warrington & Wirral	**1**
	Wales	**1**
Co-inherited diagnoses	**Wales**	**1**
Afibrinogenemia	Cheshire, Warrington & Wirral	**1**
	Wales	**1**
Hypofibrinogenemia	Cheshire, Warrington & Wirral	**1**
Other platelet defects	**Wales**	**1**
	Total	**230**

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

Haemophilia A and Factor VIII use

Table 10 Factor VIII units used by patients with a Welsh postcode

Coagulation Defect	Patients Treated	Plasma FVIII (IU)	Recombinant FVIII (IU)	Total FVIII (IU)
Haemophilia A	124	1,135,000	17,719,353	18,854,353
Haemophilia A Carrier	1	-	1,000	1,000
Females with VIII deficiency	2	-	25,500	25,500
von Willebrand disease	15	212,000	-	212,000
Co-inherited diagnoses	1	2,000	-	2,000
Total	**143**	**1,349,000**	**17,745,853**	**19,094,853**

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

Manufacturer	Product	Total Units
Baxter	Advate	5,470,500
	FEIBA	1,526,000
Bayer	Kogenate	1,335,000
BPL	Optivate	377,000
CSL Behring	Helixate Nexgen	417,000
Grifols	Fanhdi	758,000
Novo Nordisk	NovoSeven (mg)	1,404
Pfizer	ReFacto AF	10,336,500
	Investigational Factor VIII	160,353

Units in IU unless otherwise stated

Figure 1 Market share of factor VIII concentrates issued to patients with a Welsh postcode between April 2015 & March 2016

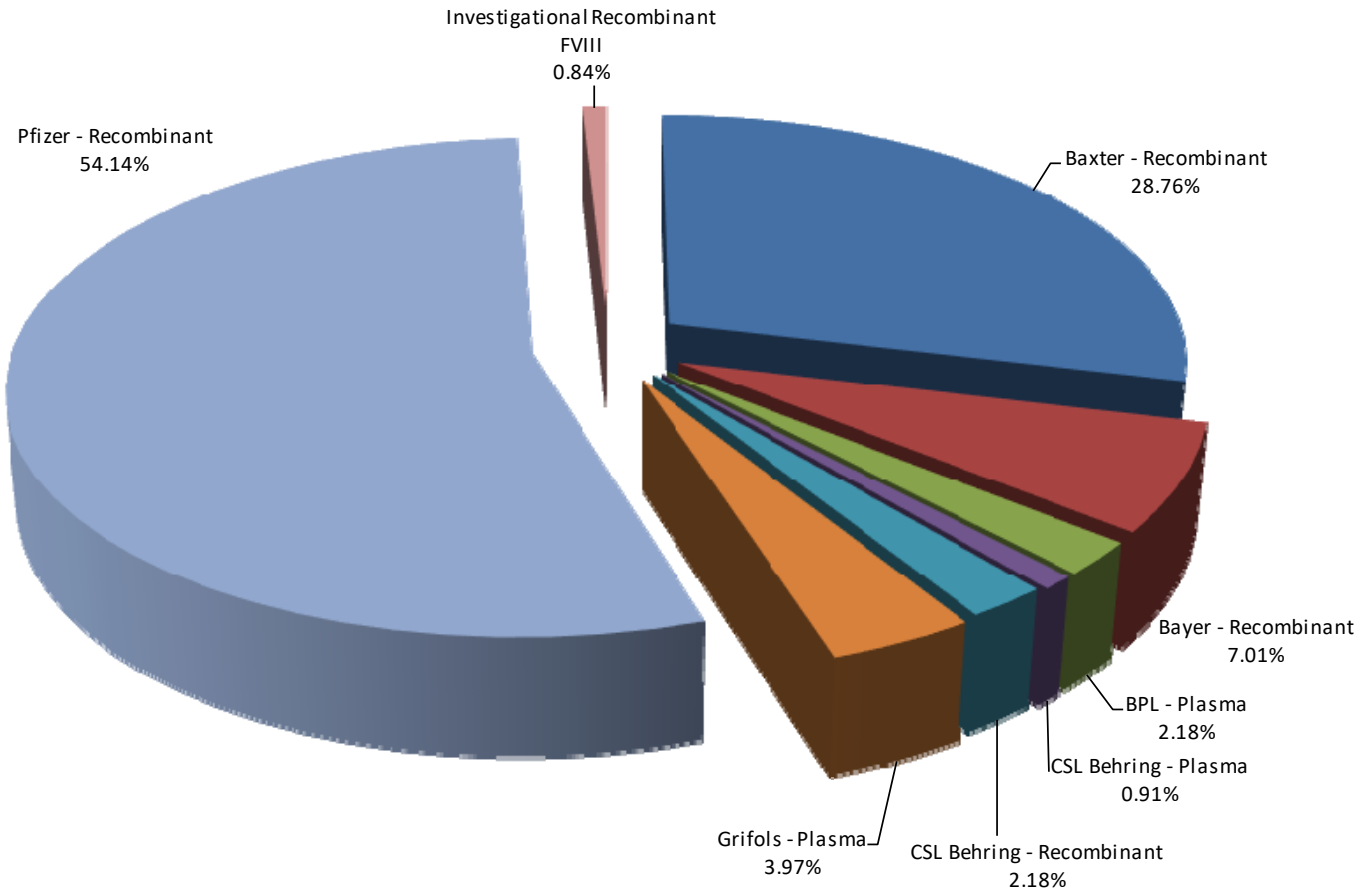

Figure 2 Factor VIII units by financial year between April 2008 & March 2016 - all diagnoses, all severities

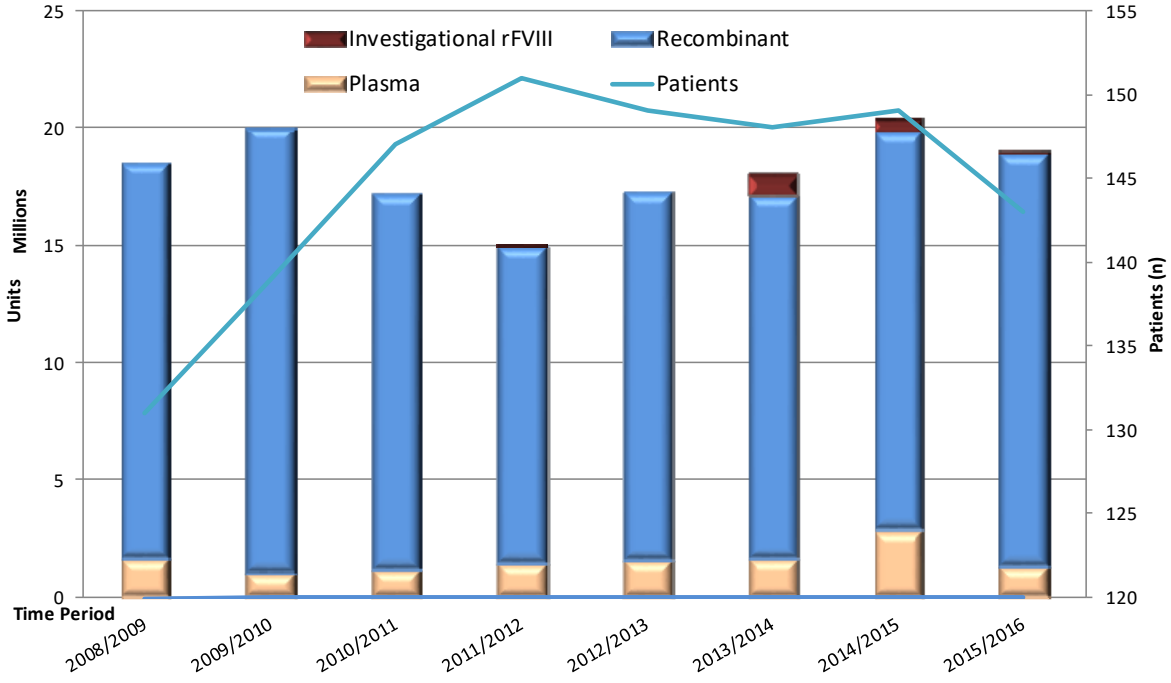

Figure 3 Factor VIII units by financial year between April 2008 & March 2016 - *Severe Haemophilia A only*

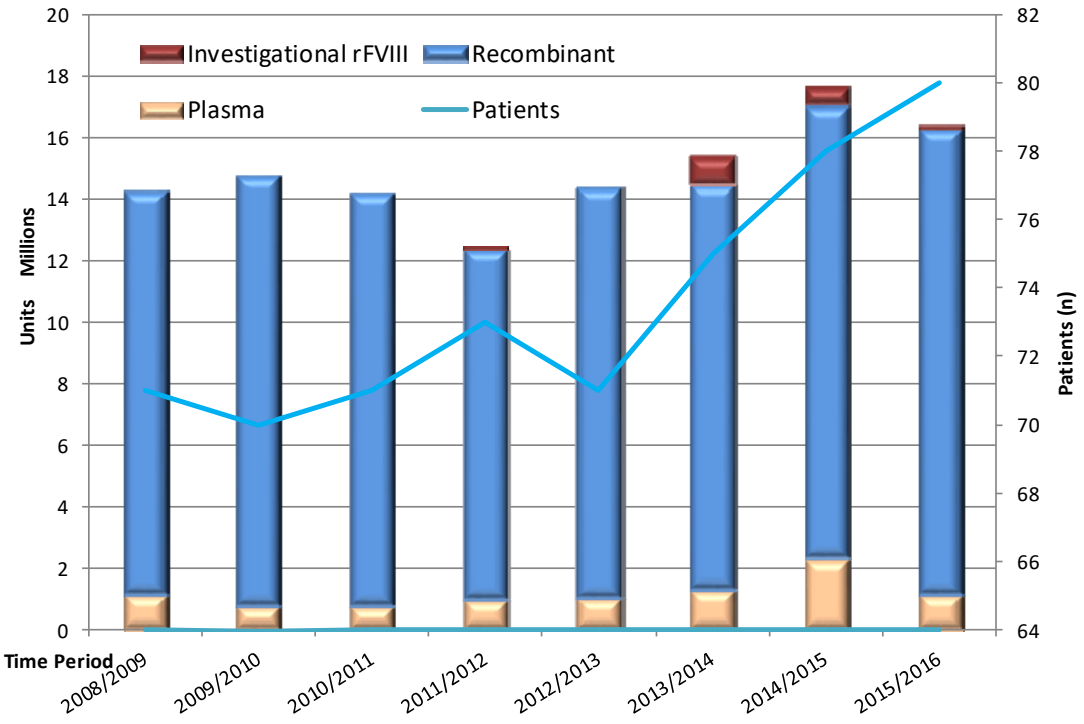

Table 12 Data table related to Figure 2 - Factor VIII units by financial year between April 2008 & March 2016

Year	Plasma		Recombinant		Investigational rFVIII		Total		Patients	
	IU	% difference since 2008/09	IU	% difference since 2008/09	IU	% difference since 2011/12	IU	% difference since 2008/09	n	% difference since 2008/09
2008/2009	1,652,425	100.00%	16,865,842	100.00%	0		18,518,267	100.00%	131	100.00%
2009/2010	1,082,440	65.51%	18,945,286	112.33%	0		20,027,726	108.15%	139	106.11%
2010/2011	1,211,665	73.33%	16,029,282	95.04%	0		17,240,947	93.10%	147	112.21%
2011/2012	1,487,895	90.04%	13,450,238	79.75%	121,000	100.00%	15,059,133	81.32%	151	115.27%
2012/2013	1,591,650	96.32%	15,733,092	93.28%	0	0.00%	17,324,742	93.55%	149	113.74%
2013/2014	1,629,820	98.63%	15,518,750	92.01%	964,000	796.69%	18,112,570	97.81%	148	112.98%
2014/2015	2,929,500	177.28%	16,898,000	100.19%	604,170	499.31%	20,431,670	110.33%	149	113.74%
2015/2016	1,349,000	81.64%	17,585,500	104.27%	160,353	132.52%	19,094,853	103.11%	143	109.16%

Table 13 Data table related to Figure 3 - Factor VIII units by financial year between April 2008 & March 2016 - *Severe Haemophilia A only*

Year	Plasma		Recombinant		Investigational rFVIII		Total		Patients	
	IU	% difference since 2008/09	IU	% difference since 2008/09	IU	% difference since 2011/12	IU	% difference since 2008/09	n	% difference since 2008/09
2008/2009	1,116,000	100.00%	13,117,382	100.00%	0		14,233,382	100.00%	71	100.00%
2009/2010	758,000	67.92%	13,959,604	106.42%	0		14,717,604	103.40%	70	98.59%
2010/2011	744,560	66.72%	13,453,324	102.56%	0		14,197,884	99.75%	71	100.00%
2011/2012	930,000	83.33%	11,434,738	87.17%	121,000	100.00%	12,485,738	87.72%	73	102.82%
2012/2013	1,009,150	90.43%	13,338,592	101.69%	0	0.00%	14,347,742	100.80%	71	100.00%
2013/2014	1,275,000	114.25%	13,152,500	100.27%	964,000	796.69%	15,391,500	108.14%	75	105.63%
2014/2015	2,291,500	205.33%	14,751,000	112.45%	604,170	499.31%	17,646,670	123.98%	78	109.86%
2015/2016	1,135,000	101.70%	15,092,250	115.06%	160,353	132.52%	16,387,603	115.13%	80	112.68%

Table 14 Factor VIII usage by region for *Severe Haemophilia A* patients only (incl. treatment for inhibitors)

Region	Patients treated (n) Severe Haemophilia A	Total FVIII Units Severe Haemophilia A	Mean Usage Severe Haemophilia A
Cheshire, Warrington & Wirral	10	1,983,000	198,300
Wales	67	13,880,603	207,173
Wessex	1	4,000	4,000
	78	**15,867,603**	**203,431**

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

Table 15 Factor VIII usage by Health Board for *Severe Haemophilia A* patients only (incl. treatment for inhibitors)

Health Board	General Population *	Patients treated (n) Severe Haemophilia A	Total FVIII Units Severe Haemophilia A	Mean Usage Severe Haemophilia A	FVIII Units Per Capita Severe Haemophilia A
Abertawe Bro Morgannwg University Local Health Board	525,466	17	3,135,603	184,447	5.97
Aneurin Bevan Local Health Board	581,789	13	3,084,000	237,231	5.30
Betsi Cadwaladr University Local Health Board	694,473	19	4,785,750	251,882	6.89
Cardiff and Vale University Local Health Board	484,752	11	2,040,500	185,500	4.21
Cwm Taf Local Health Board	296,735	9	1,534,250	170,472	5.17
Hywel Dda Local Health Board	383,229	7	936,500	133,786	2.44
Powys Teaching Local Health Board	132,642	1	351,000	351,000	2.65
Wales	**3,099,086**	**77**	**15,867,603**	**206,073**	**5.12**

** Source: Mid-year population estimate (2015), by Welsh health boards, for single year of age and gender*
URL: https://statswales.gov.wales/Catalogue/Population-and-Migration/Population/Estimates/Local-Health-Boards/populationestimates-by-lhb-age

Haemophilia B and Factor IX use

Table 16 Factor IX units used by patients with a Welsh postcode

Coagulation Defect	Patients Treated	Plasma FIX (IU)	Recombinant FIX (IU)	Total FIX (IU)
Haemophilia B	31	228,000	2,731,553	2,959,553

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

Manufacturer	Product	Total Units
CSL Behring	Mononine	228,000
Pfizer	BeneFIX	2,595,000
	Investigational Factor IX	136,553

Units in IU unless otherwise stated

Figure 4 Market share of factor IX concentrates issued to patients with a Welsh postcode between April 2015 & March 2016

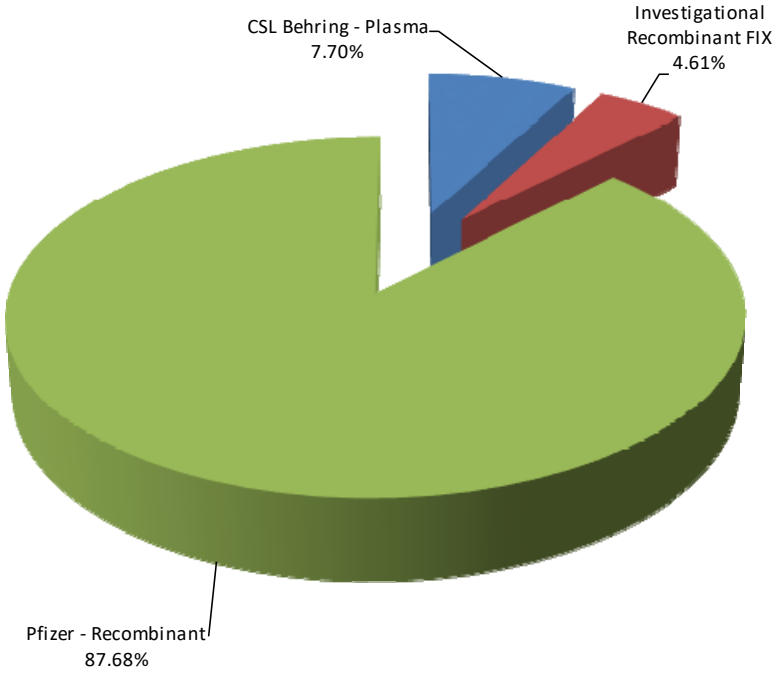

Figure 5 Factor IX units by financial year between April 2008 & March 2016 - all diagnoses, all severities

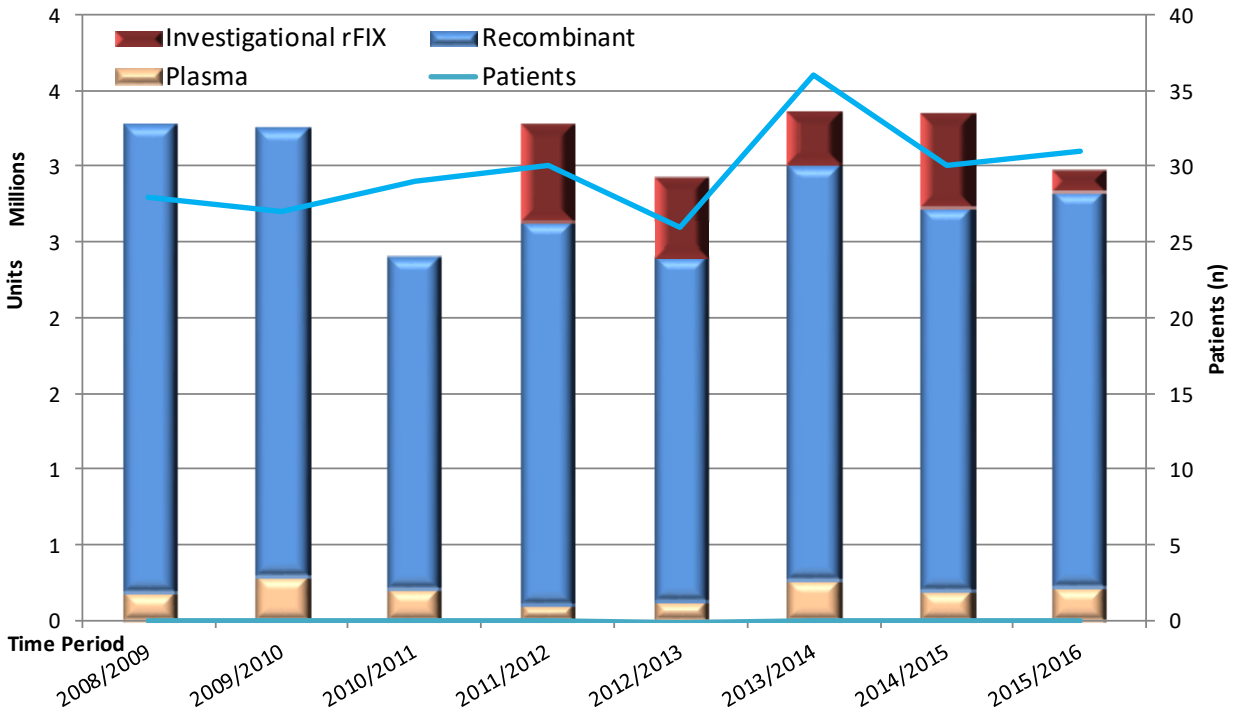

Table 18 Data table related to figure 5 - Factor IX units by financial year between April 2008 & March 2016

Year	Plasma		Recombinant		Investigational rFIX		Total		Patients	
	IU	% difference since 2008/09	IU	% difference since 2008/09	IU	% difference since 2011/12	IU	% difference since 2008/09	n	% difference since 2008/09
2008/2009	185,000	100.00%	3,084,000	100.00%	0		3,269,000	100.00%	28	100.00%
2009/2010	294,000	158.92%	2,951,500	95.70%	0		3,245,500	99.28%	27	96.43%
2010/2011	212,000	114.59%	2,189,000	70.98%	0		2,401,000	73.45%	29	103.57%
2011/2012	106,000	57.30%	2,515,500	81.57%	647,689	100.00%	3,269,189	100.01%	30	107.14%
2012/2013	133,000	71.89%	2,270,000	73.61%	517,180	79.85%	2,920,180	89.33%	26	92.86%
2013/2014	274,000	148.11%	2,731,520	88.57%	349,016	53.89%	3,354,536	102.62%	36	128.57%

Table 19 Factor IX usage by region for *Severe Haemophilia B* patients only (incl. treatment for inhibitors)

Region	Patients treated (n) Severe Haemophilia B	Total FIX Units Severe Haemophilia B	Mean Usage Severe Haemophilia B
Wales	10	1,392,553	139,255

Table 20 Factor IX usage by Health Board for *Severe Haemophilia B* patients only (incl. treatment for inhibitors)

Health Board	General Population *	Patients treated (n) Severe Haemophilia B	Total FIX Units Severe Haemophilia B	Mean Usage Severe Haemophilia B	FIX Units Per Capita Severe Haemophilia B
Abertawe Bro Morgannwg University Local Health Board	525,466	1	5,655	5,655	0.01
Aneurin Bevan Local Health Board	581,789	4	679,000	169,750	1.17
Cwm Taf Local Health Board	296,735	4	645,568	161,392	2.18
Hywel Dda Local Health Board	383,229	1	62,330	62,330	0.16
Wales	**3,099,086**	**10**	**1,392,553**	**139,255**	**0.45**

** Source: Mid-year population estimate (2015), by Welsh health boards, for single year of age and gender*
URL: https://statswales.gov.wales/Catalogue/Population-and-Migration/Population/Estimates/Local-Health-Boards/populationestimates-by-lhb-age

Von Willebrand Disease, Rarer Bleeding Disorders and Acquired Defects

Table 21 Concentrates used to treat von Willebrand Disease

Manufacturer	Product	Total Units
BPL	FVIII 8Y	40,000
CSL Behring	Voncento	205,000
	Haemate P	172,000
Octapharma	Wilate	2,000

Units in IU unless otherwise stated

Table 22 Concentrates used to treat Rarer Bleeding Disorders

Manufacturer	Product	F.VII deficiency	F.XI Deficiency	F.XIII Deficiency
BPL	FXI	-	1,000	-
CSL Behring	Fibrogammin P	-	-	13,750
Novo Nordisk	NovoSeven (mg)	1	-	-

Units in IU unless otherwise stated

Table 23 Concentrates used to treat Acquired Defects

None reported

Deaths and Adverse Events

Table 24 Causes of Death

Diagnosis	Cause of Death	Severity (factor level iu/dl)				Total
		< 1	1 - 5	> 5	N/K	
Haemophilia A	Haemorrhage (misc)	1	0	0	0	**1**
	Infection (Bacterial)	0	0	1	0	**1**
	Liver failure	1	0	0	0	**1**
	Unknown	0	0	2	0	**2**
Haemophilia B	Ischaemic Heart Disease	1	0	0	0	**1**
	Unknown	0	0	1	0	**1**
von Willebrand disease	Ischaemic Heart Disease	0	0	1	0	**1**
F.XI Deficiency	Carcinoma	0	0	1	0	**1**
Dysfibrinogenemia	Unknown	0	0	0	1	**1**
Acquired Haemophilia A	Unknown	0	0	1	0	**1**
		3	**0**	**7**	**1**	**11**

Table 25 Adverse Events

Adverse Event	Number of Events
Allergy Event	0
Infection Event	0
Inhibitor Event	0
Malignancy Event	0
Other Event	0
Poor Efficacy Event	0
Thrombotic Event	0
Total	**0**