Bleeding Disorder Statistics for Wales

April 2016 to March 2017

A report from the UK National Haemophilia Database

January 2018

The following report is based on patients 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 patients newly registered between April 2016 & March 2017, by diagnosis and gender

Coagulation Defect	Male	Female	Total
Haemophilia A	7	0	7
Haemophilia A Carrier		10	10
Acquired Haemophilia A	2	2	4
Haemophilia B	2	0	2
Haemophilia B Carrier	0	1	1
von Willebrand disease	3	12	15
Probable von Willebrands disease	0	0	0
Platelet-type Pseudo von Willebrand Disease	2	1	3
Acquired von Willebrands	3	0	3
F.VII deficiency	1	0	1
F.XI Deficiency	3	2	5
Dysfibrinogenemia	2	1	3
Hypofibrinogenemia	2	0	2
Hypodysfibrinogenemia	0	1	1
Co-inherited diagnoses	0	1	1
Acquired Prothrombin Deficiency	1	0	1
Acquired Deficiency (other)	1	0	1
Glanzmanns Thrombasthenia	1	1	2
Platelet defects	5	13	18
Unclassified	0	6	6
Total	**35**	**51**	**86**

Table 2 New Registrations of Haemophilia A & B between April 2016 & March 2017, 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	2	1	2	5
	10 : 19	0	0	1	1
	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	1	1
	70 +	0	0	0	0
	Total	**2**	**1**	**4**	**7**
Haemophilia B	0 : 9	0	2	0	2
	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**	**2**	**0**	**2**

N.B Age calculated at mid-year, 30/09/2016

In Register

Table 3 In Register - The total number of patients in the register as of 31st March 2017 and the number treated between April 2016 & March 2017, by diagnosis

Coagulation Defect	In Register			Treated (n)	Treated %
	Males	Females	Total In Reg		
Haemophilia A	270	1	271	135	49.82%
Acquired Haemophilia A	10	6	16	2	12.50%
Females with VIII deficiency		35	35	3	8.57%
Haemophilia A Carrier		24	24	1	4.17%
Haemophilia B	57	0	57	31	54.39%
Females with IX deficiency		15	15	0	0.00%
Haemophilia B Carrier		1	1	0	0.00%
von Willebrand disease	154	264	418	37	8.85%
Acquired von Willebrands	5	0	5	3	60.00%
Platelet-type Pseudo von Willebrand Disease	3	1	4	0	0.00%
Probable von Willebrands disease	2	2	4	0	0.00%
Prothrombin Deficiency	0	1	1	0	0.00%
Acquired Prothrombin Deficiency	1	0	1	1	100.00%
F.V deficiency	1	3	4	0	0.00%
F.VII deficiency	16	15	31	0	0.00%
F.X deficiency	0	1	1	0	0.00%
F.XI Deficiency	38	53	91	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	10	11	0	0.00%
Acquired Deficiency (other)	1	0	1	0	0.00%
Afibrinogenemia	1	0	1	1	100.00%
Dysfibrinogenemia	8	16	24	0	0.00%
Hypofibrinogenemia	4	5	9	1	11.11%
Hypodysfibrinogenemia	0	1	1	0	0.00%
Glanzmanns Thrombasthenia	1	2	3	0	0.00%
Bernard Soulier	0	2	2	0	0.00%
Platelet defects	36	72	108	1	0.93%
Miscellaneous	3	4	7	0	0.00%
Unclassified	4	16	20	0	0.00%
Totals	**617**	**551**	**1,168**	**217**	

Table 4 In Register - The total number of patients with Haemophilia A & B in the register as of 31st March 2017, by severity and age group

Coagulation Defect	Age Range	Factor level iu/dl			Total
		< 1	1 - 5	> 5	
Haemophilia A	<18 years	25	9	22	**56**
	≥18 years	59	22	134	**215**
	Sub Total	**84**	**31**	**156**	**271**
Haemophilia B	<18 years	1	7	8	**16**
	≥18 years	11	14	16	**41**
	Sub Total	**12**	**21**	**24**	**57**

Table 5 In Register – The total number of patients in the register as of 31st March 2017, 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	55	48	49	24	24	7	**271**
Acquired Haemophilia A	4	2	3	4	0	3	0	**16**
Females with VIII deficiency	13	5	10	4	2	1	0	**35**
Haemophilia A Carrier	10	3	5	3	0	3	0	**24**
Haemophilia B	6	17	7	11	11	4	1	**57**
Females with IX deficiency	2	4	1	4	2	1	1	**15**
Haemophilia B Carrier	1	0	0	0	0	0	0	**1**
von Willebrand disease	29	72	148	68	18	32	51	**418**
Acquired von Willebrands	0	3	0	2	0	0	0	**5**
Platelet-type Pseudo von Willebrand Disease	0	0	0	0	0	2	2	**4**
Probable von Willebrands disease	0	2	1	1	0	0	0	**4**
Prothrombin Deficiency	1	0	0	0	0	0	0	**1**
Acquired Prothrombin Deficiency	0	0	0	1	0	0	0	**1**
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	45	11	7	6	9	0	**91**
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	3	2	2	2	0	0	2	**11**
Acquired Deficiency (other)	1	0	0	0	0	0	0	**1**
Afibrinogenemia	0	0	0	0	1	0	0	**1**
Dysfibrinogenemia	5	2	2	15	0	0	0	**24**
Hypofibrinogenemia	2	2	1	2	1	1	0	**9**
Hypodysfibrinogenemia	1	0	0	0	0	0	0	**1**
Glanzmanns Thrombasthenia	0	0	0	1	0	2	0	**3**
Bernard Soulier	0	0	1	1	0	0	0	**2**
Platelet defects	22	17	23	24	11	6	5	**108**
Miscellaneous	1	1	2	1	1	0	1	**7**
Unclassified	4	5	2	5	2	2	0	**20**
Total	**190**	**241**	**283**	**207**	**79**	**98**	**70**	**1,168**

Table 6 In Register – The total number of patients with Von Willebrand Disease in the register as of 31st March 2017and the number treated between April 2016 & March 2017, 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	10	6	1	**20**	19	30	16	0	**65**	**85**	**4**
Type 2A	2	0	0	0	**2**	8	0	2	0	**10**	**12**	**4**
Type 2B	1	0	0	0	**1**	0	2	0	0	**2**	**3**	**1**
Type 2M	0	0	0	0	**0**	1	2	0	0	**3**	**3**	**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	3				**3**	2				**2**	**5**	**3**
Type Unreported	1	1	2	1	**5**	13	14	13	0	**40**	**45**	**6**
Sub Total Males											**154**	**19**
Females												
Type 1	6	6	2	0	**14**	16	29	56	0	**101**	**115**	**5**
Type 2A	0	1	0	0	**1**	11	5	6	0	**22**	**23**	**3**
Type 2B	0	0	0	0	**0**	0	1	5	0	**6**	**6**	**1**
Type 2M	0	3	2	0	**5**	4	3	3	0	**10**	**15**	**1**
Type 2N	0	0	0	0	**0**	0	0	4	0	**4**	**4**	**0**
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	3	3	3	1	**10**	14	19	49	5	**87**	**97**	**7**
Sub Total Females											**264**	**18**
Grand Total - Males and Females											**418**	**37**

Table 7 In Register – The number of patients with selected rarer bleeding disorders in the register as of 31st March 2017 and the number treated between April 2016 & March 2017, 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	0	0	0	31	0
F.X deficiency	0	0	1	0	0	0	1	0
F.XI Deficiency	2	0	89	0	0	0	91	0
Total	**3**	**-**	**124**	**-**	**-**	**-**	**127**	**-**

Coagulation Defect	<2		≥2		N/K		Total	
	In Reg	Treated	In Reg	Treated	In Reg	Treated	In Reg	Treated
F.XIII Deficiency	1	1	0	0	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	84	1 (1.2)	10 (11.9)	26 (31.0)
	1 - 5	31	0 (0.0)	1 (3.2)	2 (6.5)
	> 5	156	0 (0.0)	0 (0.0)	6 (3.8)
	Total	**271**	**1 (0.4)**	**11 (4.1)**	**34 (12.5)**
Haemophilia B	< 1	12	0 (0.0)	0 (0.0)	0 (0.0)
	1 - 5	21	0 (0.0)	0 (0.0)	0 (0.0)
	> 5	24	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	7	0 (0.0)	1 (14.3)	1 (14.3)
	Others	411	0 (0.0)	0 (0.0)	0 (0.0)
	Total	**418**	**0 (0.0)**	**1 (0.2)**	**1 (0.2)**

** Including patients not regularly treated*

Treatment

Table 9a Patients with a Welsh postcode, treated between April 2016 & March 2017, all severities, by diagnosis and treatment region

Coagulation Defect	Region	Patients Treated (n)
Haemophilia A	Birmingham & Black Country	1
	Cheshire, Warrington & Wirral	17
	London	1
	Wales	**118**
Haemophilia A Carrier	**Wales**	**4**
Acquired Haemophilia A	**Wales**	**2**
Haemophilia B	Birmingham & Black Country	1
	Cheshire, Warrington & Wirral	3
	Wales	**27**
von Willebrand disease	London	1
	Wales	**37**
Acquired von Willebrands	**Wales**	**3**
F.XIII Deficiency	**Wales**	**1**
Acquired Prothrombin Deficiency	**Wales**	**1**
Afibrinogenemia	London	1
	Wales	**1**
Hypofibrinogenemia	Cheshire, Warrington & Wirral	1
Other platelet defects	**Wales**	**1**
	Total	**221**

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

Table 9b Patients with an English postcode, registered & treated at a Welsh Haemophilia Centre between April 2016 & March 2017, all severities, by diagnosis

Coagulation Defect	In Register			Treated (n)
	Males	Females	Total	
Haemophilia A	16	0	16	9
Haemophilia A Carrier	0	2	2	0
Females with VIII deficiency	0	3	3	0
Haemophilia B	2	0	2	1
von Willebrand disease	4	5	9	2
F.XI Deficiency	1	1	2	0
Other platelet defects	0	1	1	0

Haemophilia A and Factor VIII use

Table 10 Factor VIII units issued to patients with a Welsh postcode, by diagnosis

Coagulation Defect	Patients Treated	Plasma FVIII (IU)	Recombinant FVIII (IU)	Total FVIII (IU)
Haemophilia A	127	1,236,475	18,380,909	19,617,384
Haemophilia A Carrier	1	-	1,250	1,250
Females with VIII deficiency	2	-	267,500	267,500
Haemophilia B	1	-	1,000	1,000
von Willebrand disease	10	68,000	-	68,000
Total	**141**	**1,304,475**	**18,650,659**	**19,955,134**

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

Manufacturer	Product	Total Units
Baxter	Advate	5,679,398
	FEIBA	147,000
Bayer	Kogenate	1,296,000
BPL	Optivate	310,475
CSL Behring	Helixate Nexgen	753,500
Grifols	Fanhdi	926,000
Novo Nordisk	NovoSeven (mg)	569
Pfizer	ReFacto AF	9,782,500
SOBI/Biogen	Elocta	793,500
	Investigational Factor VIII	76,011

Units in IU unless otherwise stated

Figure 1 Market share of factor VIII concentrates issued to patients with a Welsh postcode between April 2016 & March 2017

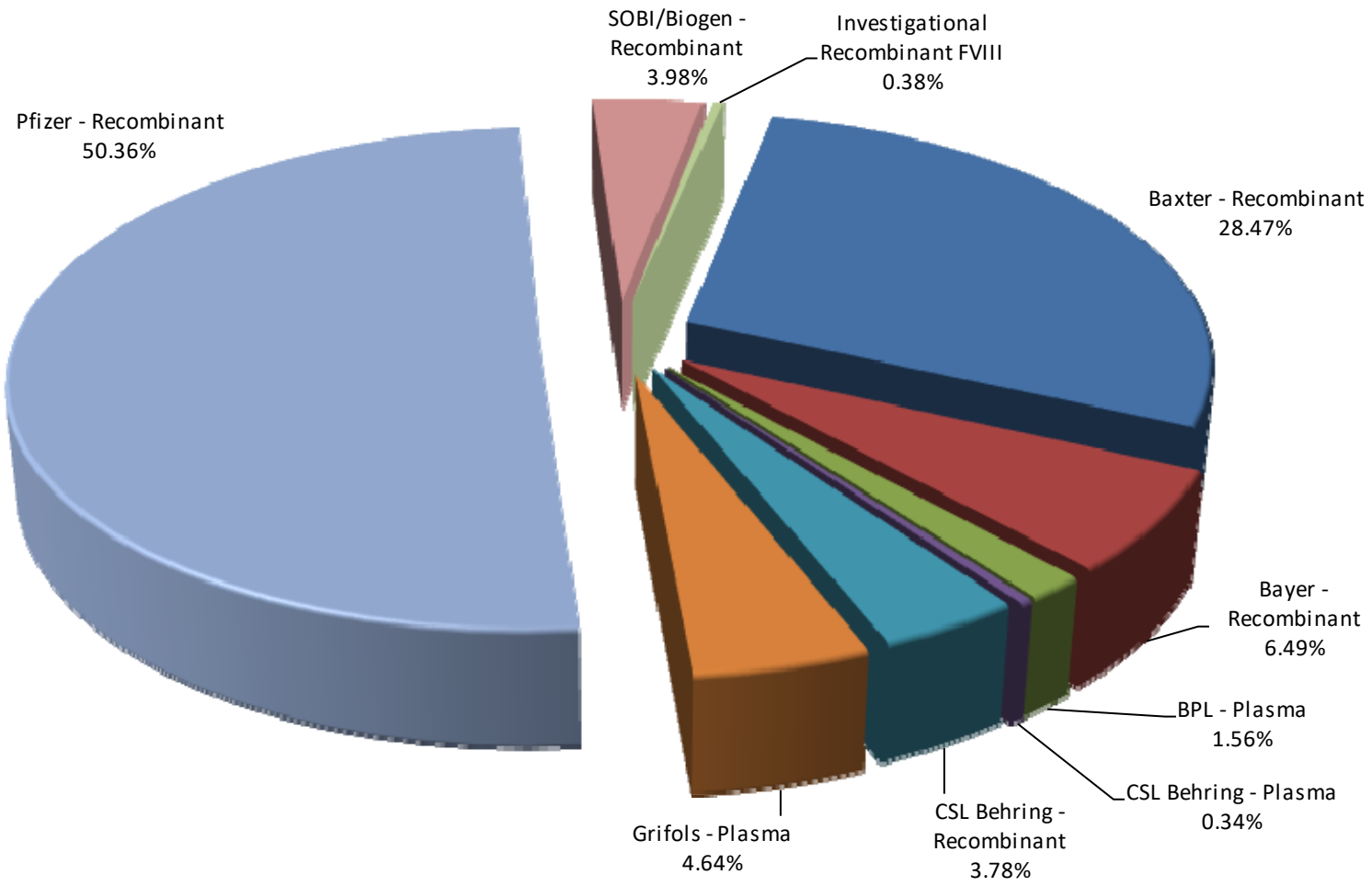

Figure 2 Factor VIII units by financial year between April 2009 & March 2017 – all diagnoses, all severities

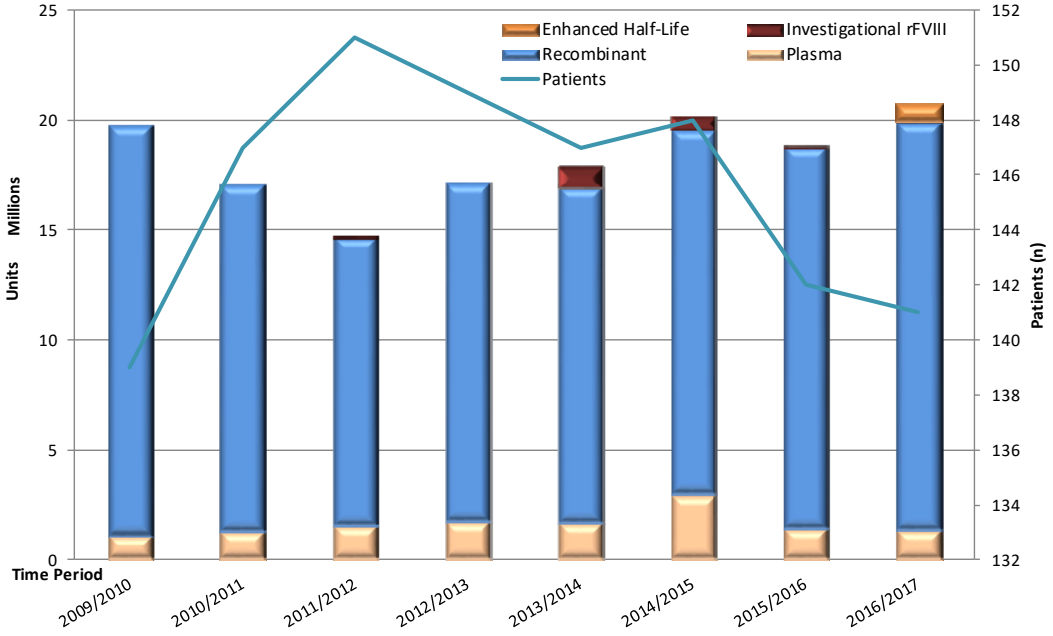

Figure 3 Factor VIII units by financial year between April 2009 & March 2017 – *Severe Haemophilia A only*

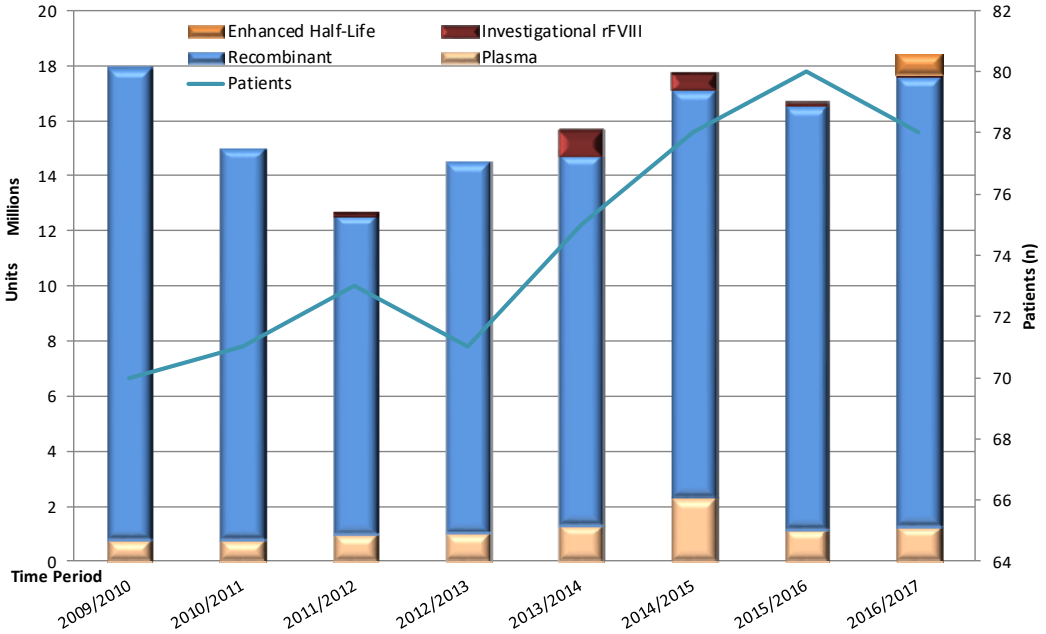

Table 12 Data table related to Figure 2 - Factor VIII units by financial year between April 2009 & March 2017

Year	Plasma		Recombinant		Investigational rFVIII		Enhanced Half-Life	Total		Patients	
	IU	% difference since 2009/10	IU	% difference since 2009/10	IU	% difference since 2011/12	IU	IU	% difference since 2009/10	n	% difference since 2009/10
2009/2010	1,085,410	100.00%	18,686,536	100.00%	0		0	19,771,946	100.00%	139	100.00%
2010/2011	1,272,665	117.25%	15,760,532	84.34%	0		0	17,033,197	86.15%	147	105.76%
2011/2012	1,489,895	137.27%	13,209,238	70.69%	121,000	100.00%	0	14,820,133	74.96%	151	108.63%
2012/2013	1,679,650	154.75%	15,447,092	82.66%	0	0.00%	0	17,126,742	86.62%	149	107.19%
2013/2014	1,629,820	150.16%	15,329,250	82.03%	964,000	796.69%	0	17,923,070	90.65%	147	105.76%
2014/2015	2,929,500	269.90%	16,643,000	89.06%	604,170	499.31%	0	20,176,670	102.05%	148	106.47%
2015/2016	1,349,000	124.28%	17,382,500	93.02%	160,353	132.52%	0	18,891,853	95.55%	142	102.16%
2016/2017	1,304,475	120.18%	18,574,648	99.40%	76,011	62.82%	794,500	20,749,634	104.94%	141	101.44%

Table 13 Data table related to Figure 3 - Factor VIII units by financial year between April 2009 & March 2017 - *Severe Haemophilia A only*

Year	Plasma		Recombinant		Investigational rFVIII		Enhanced Half-Life	Total		Patients	
	IU	% difference since 2009/10	IU	% difference since 2009/10	IU	% difference since 2011/12	IU	IU	% difference since 2009/10	n	% difference since 2009/10
2009/2010	758,000	100.00%	17,216,854	100.00%	0		0	17,974,854	100.00%	70	100.00%
2010/2011	744,560	98.23%	14,260,574	82.83%	0		0	15,005,134	83.48%	71	101.43%
2011/2012	930,000	122.69%	11,679,738	67.84%	121,000	100.00%	0	12,730,738	70.83%	73	104.29%
2012/2013	1,009,150	133.13%	13,490,092	78.35%	0	0.00%	0	14,499,242	80.66%	71	101.43%
2013/2014	1,275,000	168.21%	13,467,000	78.22%	964,000	796.69%	0	15,706,000	87.38%	75	107.14%
2014/2015	2,291,500	302.31%	14,842,500	86.21%	604,170	499.31%	0	17,738,170	98.68%	78	111.43%
2015/2016	1,135,000	149.74%	15,424,250	89.59%	160,353	132.52%	0	16,719,603	93.02%	80	114.29%
2016/2017	1,236,475	163.12%	16,381,000	95.15%	76,011	62.82%	768,500	18,461,986	102.71%	78	111.43%

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
Birmingham & Black Country	1	179,000	179,000
Cheshire, Warrington & Wirral	11	2,607,750	237,068
Wales	66	14,905,736	225,844
	78	**17,692,486**	**226,827**

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	529,278	18	3,613,761	200,764	6.83
Aneurin Bevan Local Health Board	584,133	12	3,385,000	282,083	5.79
Betsi Cadwaladr University Local Health Board	695,822	21	5,813,225	276,820	8.35
Cardiff and Vale University Local Health Board	489,931	11	2,287,250	207,932	4.67
Cwm Taf Local Health Board	298,116	7	1,273,250	181,893	4.27
Hywel Dda Local Health Board	383,710	7	920,000	131,429	2.40
Powys Teaching Local Health Board	132,160	1	400,000	400,000	3.03
Wales	**3,113,150**	**77**	**17,692,486**	**229,773**	**5.68**

** Source: Mid-year population estimate (2016), 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 issued to patients with a Welsh postcode

Coagulation Defect	Patients Treated	Plasma FIX (IU)	Recombinant FIX (IU)	Total FIX (IU)
Haemophilia B	31	170,000	2,980,707	3,150,707

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

Manufacturer	Product	Total Units
CSL Behring	Mononine	170,000
Pfizer	BeneFIX	2,428,000
SOBI/Biogen	ALPROLIX	289,015
	Elocta	1,000
	Investigational Factor IX	263,692

Units in IU unless otherwise stated

**Potentially anomalous use of product in Table 17 is accounted for as follows:*

Elocta: Treated in error - confirmed by Centre

Figure 4 Market share of factor IX concentrates issued to patients with a Welsh postcode between April 2016 & March 2017

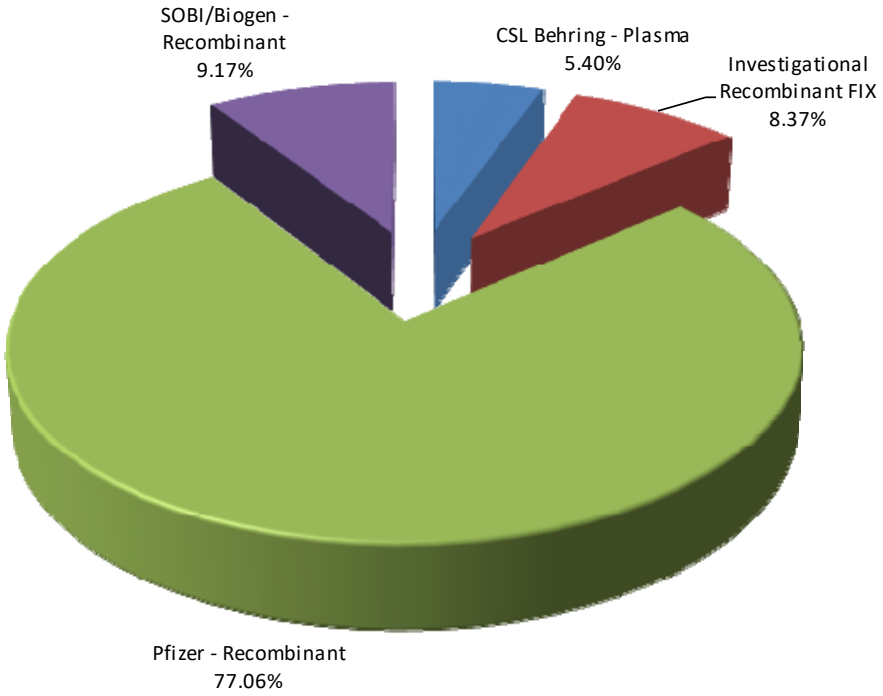

Figure 5 Factor IX units by financial year between April 2009 & March 2017 - all diagnoses, all severities

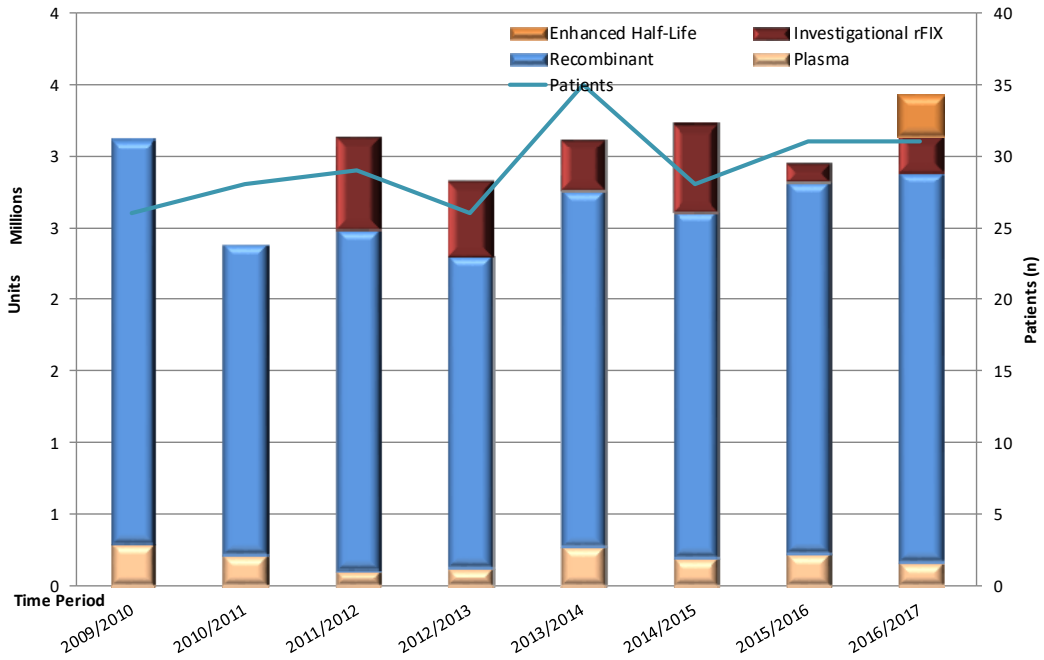

Table 18 Data table related to figure 5 - Factor IX units by financial year between April 2009 & March 2017

Year	Plasma		Recombinant		Investigational rFIX		Enhanced Half-Life	Total		Patients	
	IU	% difference since 2009/10	IU	% difference since 2009/10	IU	% difference since 2011/12	IU	IU	% difference since 2009/10	n	% difference since 2009/10
2009/2010	294,000	100.00%	2,830,000	100.00%	0		0	3,124,000	100.00%	26	100.00%
2010/2011	212,000	72.11%	2,177,000	76.93%	0		0	2,389,000	76.47%	28	107.69%
2011/2012	106,000	36.05%	2,383,500	84.22%	647,689	100.00%	0	3,137,189	100.42%	29	111.54%
2012/2013	133,000	45.24%	2,178,500	76.98%	517,180	79.85%	0	2,828,680	90.55%	26	100.00%
2013/2014	274,000	93.20%	2,490,770	88.01%	349,016	53.89%	0	3,113,786	99.67%	35	134.62%
2014/2015	198,000	67.35%	2,422,000	85.58%	617,447	95.33%	0	3,237,447	103.63%	28	107.69%
2015/2016	228,000	77.55%	2,591,000	91.55%	136,553	21.08%	0	2,955,553	94.61%	31	119.23%
2016/2017	170,000	57.82%	2,717,015	96.01%	263,692	40.71%	289,015	3,439,722	110.11%	31	119.23%

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	1,600,707	160,071

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	529,278	2	217,055	108,528	0.41
Aneurin Bevan Local Health Board	584,133	3	578,000	192,667	0.99
Cwm Taf Local Health Board	298,116	4	610,200	152,550	2.05
Hywel Dda Local Health Board	383,710	1	195,452	195,452	0.51
Wales	**3,113,150**	**10**	**1,600,707**	**160,071**	**0.51**

** Source: Mid-year population estimate (2016), 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 issued to treat von Willebrand Disease

Manufacturer	Product	Total Units
CSL Behring	Voncento	480,000
	Haemate P	68,000

Units in IU unless otherwise stated

Table 22 Concentrates issued to treat Rarer Bleeding Disorders

Manufacturer	Product	F.XIII Deficiency
CSL Behring	Fibrogammin P	15,000

Units in IU unless otherwise stated

Table 23 Concentrates issued to treat Acquired Defects

Manufacturer	Product	Acquired Haemophilia A	Acquired von Willebrands	Acquired Prothrombin Deficiency
Baxter	FEIBA	67,000	-	4,000
CSL Behring	Voncento	-	22,000	-

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**
	Unknown	0	0	1	0	**1**
von Willebrand disease	Unknown	0	1	1	0	**2**
Acquired Haemophilia A	Unknown	0	1	0	0	**1**
Acquired Deficiency (other)	Carcinoma	0	0	1	0	**1**
Other platelet defects	Unknown	0	0	0	1	**1**
		1	**2**	**3**	**1**	**7**

Table 25 Adverse Events

Adverse Event	Number of Events
Allergy Event	1
Infection Event	0
Inhibitor Event	2
Malignancy Event	0
Other Event	0
Poor Efficacy Event	0
Thrombotic Event	0
Total	**3**