Bleeding Disorder Statistics for Wales

April 2014 to March 2015

A report from the National Haemophilia Database

April 2016

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.

Contents

New Registrations

Table 1 **New Registrations - Number of patients newly registered between April 2014 & March 2015 showing their coagulation defect and gender**

Coagulation Defect	Male	Female	Total
Haemophilia A	6	0	6
Acquired Haemophilia A	4	2	6
Haemophilia A Carrier		2	2
von Willebrand disease	2	4	6
Acquired von Willebrands	1	0	1
F.V deficiency	0	1	1
F.VII deficiency	1	1	2
F.XI Deficiency	2	2	4
Co-inherited diagnoses	0	1	1
Afibrinogenemia	1	0	1
Dysfibrinogenemia	1	4	5
Hypofibrinogenemia	0	2	2
Platelet defects	0	5	5
Unclassified	1	1	2
Total	**19**	**25**	**44**

Table 2 **New Registrations of Haemophilia A & B between April 2014 & March 2015, 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	0	2	4
	10 : 19	0	0	1	1
	20 : 29	0	0	0	0
	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	0	0
Total		**2**	**0**	**4**	**6**
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, 30/09/2014

In Register

Table 3 **In Register – The total number of registered patients with all types of bleeding disorder as of 31st March 2015 and the number treated between April 2014 & March 2015**

Coagulation Defect	In Register			Treated (n)	Treated %
	Males (n)	Females (n)	Total In Reg		
Haemophilia A	255	1	256	141	55.08%
Acquired Haemophilia A	10	6	16	4	25.00%
Females with VIII deficiency		36	36	4	11.11%
Haemophilia A Carrier		6	6	0	0.00%
Haemophilia B	57	0	57	31	54.39%
Females with IX deficiency		14	14	0	0.00%
von Willebrand disease	146	247	393	39	9.92%
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	0	1	1	0	0.00%
F.V deficiency	1	3	4	0	0.00%
F.VII deficiency	14	14	28	2	7.14%
F.X deficiency	0	1	1	0	0.00%
F.XI Deficiency	32	46	78	2	2.56%
F.XIII Deficiency	0	1	1	1	100.00%
Combined II+VII+IX+X Deficiency	1	0	1	0	0.00%
Co-inherited diagnoses	2	10	12	3	25.00%
Fibrinogen Deficiency	3	4	7	0	0.00%
Afibrinogenemia	2	0	2	2	100.00%
Dysfibrinogenemia	6	12	18	1	5.56%
Hypofibrinogenemia	0	3	3	0	0.00%
Glanzmanns Thrombasthenia	0	1	1	0	0.00%
Bernard Soulier	0	2	2	0	0.00%
Platelet defects	27	50	77	1	1.30%
Miscellaneous	3	5	8	1	12.50%
Unclassified	1	5	6	1	16.67%
Totals	**563**	**468**	**1,031**	**233**	

Table 4 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	62	52	43	49	20	22	8	**256**
Acquired Haemophilia A	6	2	3	4	1	0	0	**16**
Females with VIII deficiency	14	3	11	4	2	1	1	**36**
Haemophilia A Carrier	0	1	3	2	0	0	0	**6**
Haemophilia B	6	16	8	11	12	3	1	**57**
Females with IX deficiency	2	4	1	4	2	0	1	**14**
von Willebrand disease	26	66	150	59	16	27	49	**393**
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	0	1	0	0	0	0	**1**
F.V deficiency	1	2	0	0	0	1	0	**4**
F.VII deficiency	5	2	13	1	0	7	0	**28**
F.X deficiency	0	0	1	0	0	0	0	**1**
F.XI Deficiency	13	39	11	4	4	7	0	**78**
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	3	2	1	0	2	**12**
Fibrinogen Deficiency	1	1	1	4	0	0	0	**7**
Afibrinogenemia	0	0	1	0	1	0	0	**2**
Dysfibrinogenemia	3	1	1	13	0	0	0	**18**
Hypofibrinogenemia	0	1	0	0	1	1	0	**3**
Glanzmanns Thrombasthenia	0	0	0	0	0	1	0	**1**
Bernard Soulier	0	0	1	1	0	0	0	**2**
Platelet defects	10	13	17	22	9	3	3	**77**
Miscellaneous	1	2	2	1	1	0	1	**8**
Unclassified	1	1	0	1	2	1	0	**6**
Total	**153**	**210**	**272**	**183**	**72**	**74**	**67**	**1,031**

Table 5 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	24	8	26	**58**
	≥18 years	57	17	124	**198**
Sub Total		**81**	**25**	**150**	**256**
Haemophilia B	<18 years	2	4	7	**13**
	≥18 years	12	13	19	**44**
Sub Total		**14**	**17**	**26**	**57**

Table 6 In Register – The total number of patients with Von Willebrand Disease currently in the register and the number treated between April 2014 & March 2015, by disease severity, age group and gender

von Willebrand disease	<18 years (VWD Activity iu/dl)				≥18 years (VWD Activity iu/dl)				Total	Treated
	<30	≥30	N/K	Sub Total	<30	≥30	N/K	Sub Total		
	Males									
Type 1	8	7	1	**16**	43	20	1	**64**	**80**	**4**
Type 2A	0	0	0	**0**	6	3	0	**9**	**9**	**1**
Type 2B	1	0	0	**1**	1	0	0	**1**	**2**	**1**
Type 2M	0	0	0	**0**	4	0	0	**4**	**4**	**2**
Type 2N	0	0	0	**0**	0	0	0	**0**	**0**	**0**
Type 2 Unspecified	0	0	0	**0**	1	0	0	**1**	**1**	**0**
Type 3	4		0	**4**	1		0	**1**	**5**	**4**
Type Unreported	1	4	1	**6**	21	17	1	**39**	**45**	**8**
Sub Total				**27**				**119**	**146**	**20**
	Females									
Type 1	11	2	0	**13**	40	57	0	**97**	**110**	**8**
Type 2A	1	0	0	**1**	12	8	0	**20**	**21**	**4**
Type 2B	0	0	0	**0**	1	5	0	**6**	**6**	**0**
Type 2M	1	0	0	**1**	6	3	0	**9**	**10**	**1**
Type 2N	0	0	0	**0**	0	3	0	**3**	**3**	**0**
Type 2 Unspecified	0	0	0	**0**	2	0	0	**2**	**2**	**0**
Type 3	0		0	**0**	2		0	**2**	**2**	**1**
Type Unreported	7	4	1	**12**	29	48	4	**81**	**93**	**5**
Sub Total				**27**				**220**	**247**	**19**
Grand Total				**54**				**339**	**393**	**39**

Table 7 **In Register – The number of patients with selected rarer bleeding disorders currently registered and the number treated between April 2014 & March 2015, 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	27	2	0	0	28	2
F.X deficiency	0	0	1	0	0	0	1	0
F.XI Deficiency	2	0	76	2	0	0	78	2
Total	**3**	**-**	**108**	**4**	**-**	**-**	**111**	**4**

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 / Subtype	In Register *	Inhibitors		
			Newly Reported n (%)	Ongoing n (%)	Historical n (%)
Haemophilia A	≤ 1 iu/dl	81	1 (1.2)	10 (12.3)	23 (28.4)
	>1 and <5 iu/dl	25	0 (0.0)	1 (4.0)	2 (8.0)
	≥ 5 iu/dl	150	0 (0.0)	4 (2.7)	9 (6.0)
	Total	**256**	**1 (0.4)**	**15 (5.9)**	**34 (13.3)**
Haemophilia B	≤ 1 iu/dl	14	0 (0.0)	0 (0.0)	0 (0.0)
	>1 and <5 iu/dl	17	0 (0.0)	0 (0.0)	0 (0.0)
	≥ 5 iu/dl	26	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	1 (14.3)	1 (14.3)	1 (14.3)
	Others	386	0 (0.0)	0 (0.0)	0 (0.0)
	Total	**393**	**1 (0.3)**	**1 (0.3)**	**1 (0.3)**

** Including patients not regularly treated*

Treatment

Treatment by Region and Health Board

Table 9 **Patients with a Welsh postcode, treated between April 2014 & March 2015, all severities, by diagnosis and treatment region**

Coagulation Defect	Region	Patients Treated (n)
Haemophilia A	Cheshire, Warrington & Wirral	17
	London	5
	Wales	**126**
Haemophilia B	Cheshire, Warrington & Wirral	3
	Wales	**28**
von Willebrand disease	Cheshire, Warrington & Wirral	2
	London	1
	Wales	**37**
Haemophilia A Carrier	**Wales**	**4**
F.VII deficiency	Cheshire, Warrington & Wirral	1
	Wales	**1**
F.XI Deficiency	Bristol, North Somerset & South Gloucestershire	**1**
F.XI Deficiency	**Wales**	**1**
F.XIII Deficiency	Cheshire, Warrington & Wirral	**1**
Co-inherited diagnoses	**Wales**	**3**
Afibrinogenemia	Cheshire, Warrington & Wirral	1
	Wales	**1**
Dysfibrinogenemia	**Wales**	**1**
Acquired Haemophilia A	**Wales**	**4**
Platelet defects	**Wales**	**1**
Miscellaneous	**Wales**	**1**
Unclassified bleeding disorder	**Wales**	**1**
	Total	**241**

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

Tables 9 & 10 show the number of patients with a Welsh postcode treated and the Region which issued the treatment.

Table 10 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	12	3,067,250	255,604
Wales	68	14,691,670	216,054
London	3	36,750	12,250
	83	**17,795,670**	**214,406**

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

Table 11 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	517,981	18	3,296,920	183,162	6.36
Aneurin Bevan Local Health Board	577,077	15	3,161,000	210,733	5.48
Betsi Cadwaladr University Local Health Board	688,417	19	5,005,000	263,421	7.27
Cardiff and Vale University Local Health Board	472,121	11	3,825,000	347,727	8.10
Cwm Taf Local Health Board	293,224	9	1,404,750	156,083	4.79
Hywel Dda Local Health Board	381,867	5	618,000	123,600	1.62
Powys Teaching Local Health Board	133,071	1	485,000	485,000	3.64
Wales	**3,063,758**	**78**	**17,795,670**	**228,150**	**5.81**

** Source: Office for National Statistics © Crown Copyright 2014*
Mid-2011 Population Estimates; estimated resident population; revised in light of the 2011 Census.

Table 12 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	13	2,053,447	157,957

Table 13 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 FVIII Units Severe Haemophilia B	Mean Usage Severe Haemophilia B	FVIII Units Per Capita Severe Haemophilia B
Abertawe Bro Morgannwg University Local Health Board	517,981	2	150,732	75,366	0.29
Aneurin Bevan Local Health Board	577,077	5	710,000	142,000	1.23
Cardiff and Vale University Local Health Board	472,121	1	213,000	213,000	0.45
Cwm Taf Local Health Board	293,224	4	803,244	200,811	2.74
Hywel Dda Local Health Board	381,867	1	176,471	176,471	0.46
Wales	**3,063,758**	**13**	**2,053,447**	**157,957**	**0.67**

** Source: Office for National Statistics © Crown Copyright 2014*
Mid-2011 Population Estimates; estimated resident population; revised in light of the 2011 Census

Haemophilia A and Factor VIII use

Table 14 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	123	2,291,500	17,437,670	19,729,170
Females with VIII deficiency	3	-	46,000	46,000
von Willebrand disease	20	636,000	-	636,000
F.VII deficiency	1	2,000	-	2,000
Total	**147**	**2,929,500**	**17,483,670**	**20,413,170**

Table 15 Products used to treat Haemophilia A (including inhibitors)

Manufacturer	Product	Total Units
Baxter	Advate	4,518,750
	FEIBA	811,000
Bayer	Kogenate	1,331,250
BPL	Optivate	255,000
CSL Behring	Helixate Nexgen	1,565,750
Grifols	Fanhdi	2,036,500
Novo Nordisk	NovoSeven (mg)	860
Pfizer	ReFacto AF	9,417,750
	Investigational Factor VIII	604,170

Units in IU unless otherwise stated

Potentially anomalous product use is accounted for as follows: -

BPL Optivate was administered to a single patient undergoing immune tolerance induction.

Figure 1 **Market share of factor VIII concentrates known to have been issued to patients with a Welsh postcode between April 2014 & March 2015**

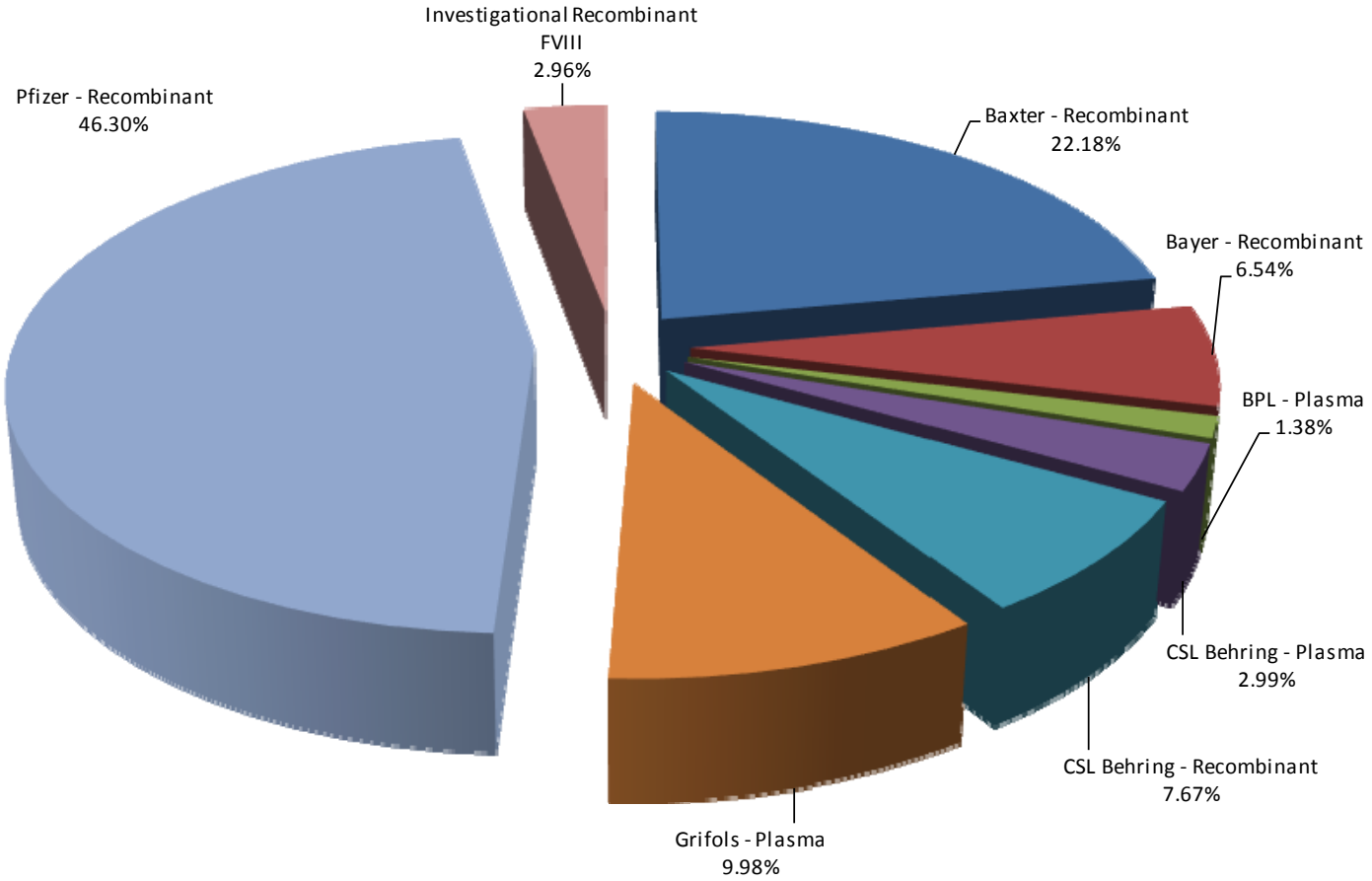

Figure 2 **Factor VIII units by financial year between April 2009 & March 2015 – all diagnoses, all severities**

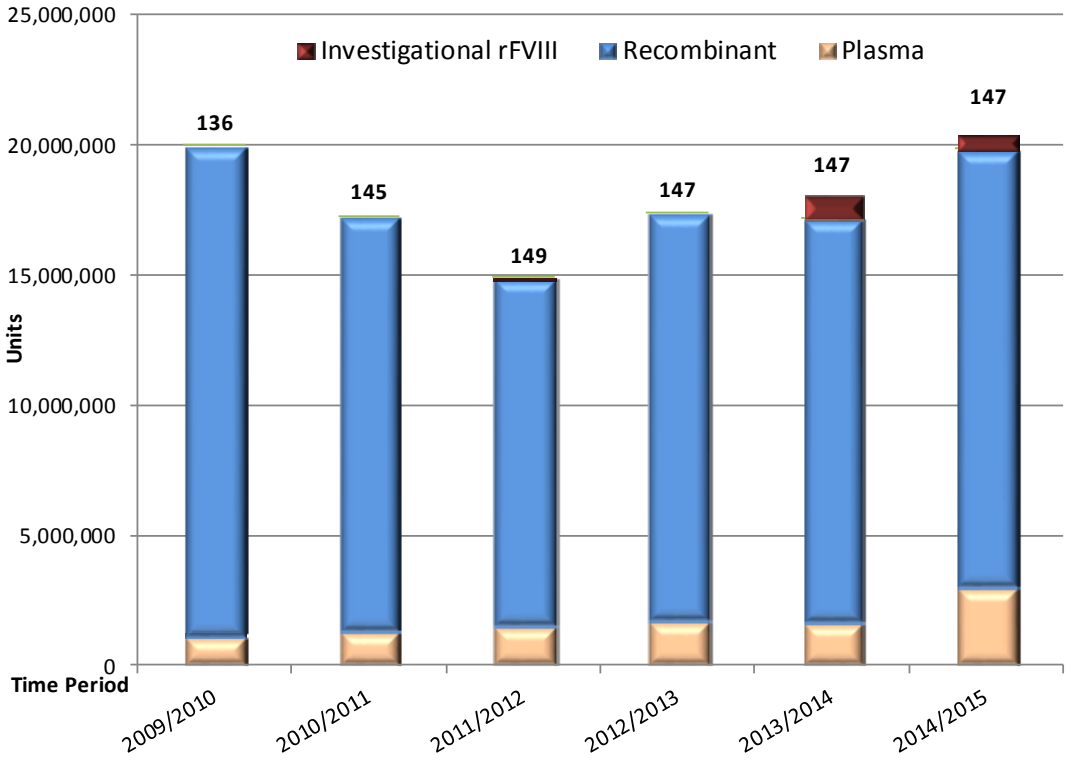

Figure 3 **Factor VIII units by financial year between April 2009 & March 2015 – *Severe Haemophilia A only***

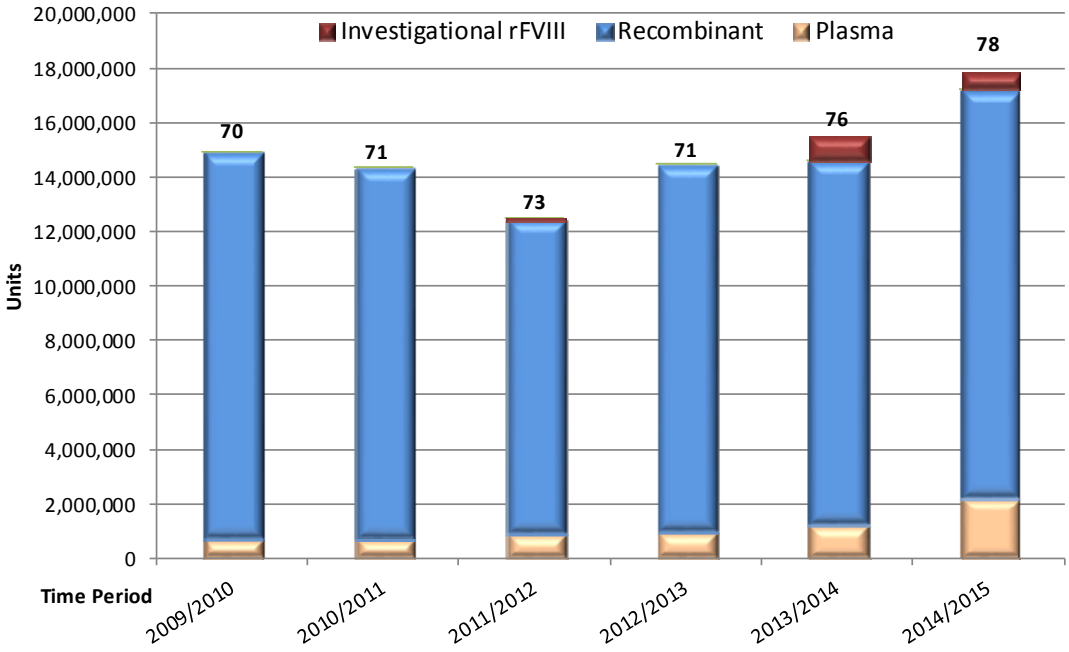

Table 16 Data table related to Figure 2 - Factor VIII units by financial year between April 2009 & March 2015

Year	Plasma		Recombinant		Investigational rFVIII		Total		Patients	
	IU	% difference since 2009/10	IU	% difference since 2009/10	IU	% difference since 2011/12	IU	% difference since 2009/10	n	% difference since 2009/10
2009/2010	1,085,410	100.00%	18,884,786	100.00%	0		19,970,196	100.00%	136	100.00%
2010/2011	1,272,665	117.25%	15,935,282	84.38%	0		17,207,947	86.17%	145	106.62%
2011/2012	1,489,895	137.27%	13,381,238	70.86%	121,000	100.00%	14,992,133	75.07%	149	109.56%
2012/2013	1,684,650	155.21%	15,660,092	82.92%	0	0.00%	17,344,742	86.85%	147	108.09%
2013/2014	1,651,820	152.18%	15,468,750	81.91%	964,000	796.69%	18,084,570	90.56%	147	108.09%
2014/2015	2,929,500	269.90%	16,879,500	89.38%	604,170	499.31%	20,413,170	102.22%	147	108.09%

Table 17 Data table related to Figure 3 - Factor VIII units by financial year between April 2009 & March 2015 – *Severe Haemophilia A only*

Year	Plasma		Recombinant		Investigational rFVIII		Total		Patients	
	IU	% difference since 2009/10	IU	% difference since 2009/10	IU	% difference since 2011/12	IU	% difference since 2009/10	n	% difference since 2009/10
2009/2010	758,000	100.00%	14,111,604	100.00%	0		14,869,604	100.00%	70	100.00%
2010/2011	744,560	98.23%	13,569,324	96.16%	0		14,313,884	96.26%	71	101.43%
2011/2012	930,000	122.69%	11,556,738	81.90%	121,000	100.00%	12,607,738	84.79%	73	104.29%
2012/2013	1,009,150	133.13%	13,459,092	95.38%	0	0.00%	14,468,242	97.30%	71	101.43%
2013/2014	1,275,000	168.21%	13,335,500	94.50%	964,000	796.69%	15,574,500	104.74%	76	108.57%
2014/2015	2,291,500	302.31%	14,900,000	105.59%	604,170	499.31%	17,795,670	119.68%	78	111.43%

Haemophilia B and Factor IX use

Table 18 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	198,000	3,242,447	3,440,447

Table 19 Products used to treat Haemophilia B (including inhibitors)

Manufacturer	Product	Total Units
CSL Behring	Mononine	198,000
Pfizer	BeneFIX	2,625,000
	Investigational Factor IX	617,447

Units in IU unless otherwise stated

Figure 4 **Market share of factor IX concentrates known to have been issued to patients with a Welsh postcode between April 2014 & March 2015**

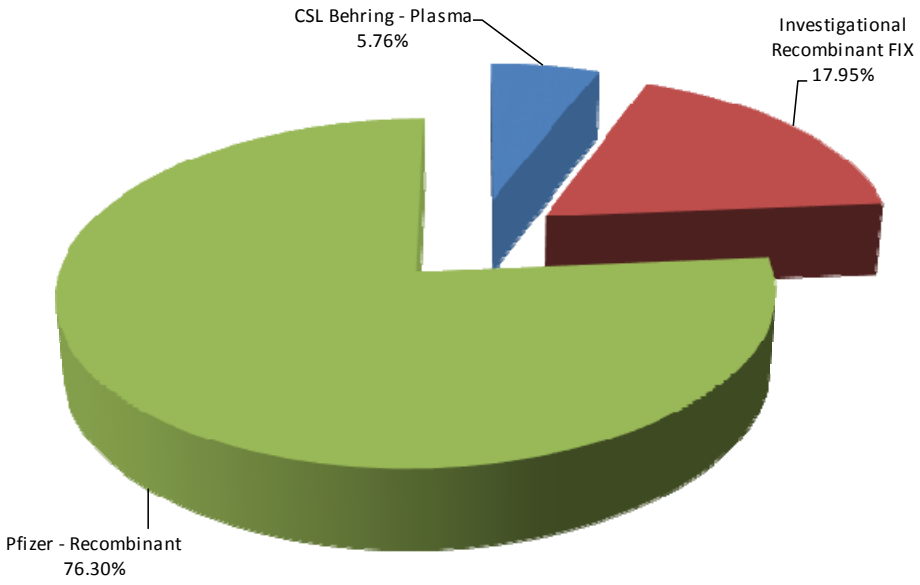

Figure 5 **Factor IX units by financial year between April 2009 & March 2015 – all diagnoses, all severities**

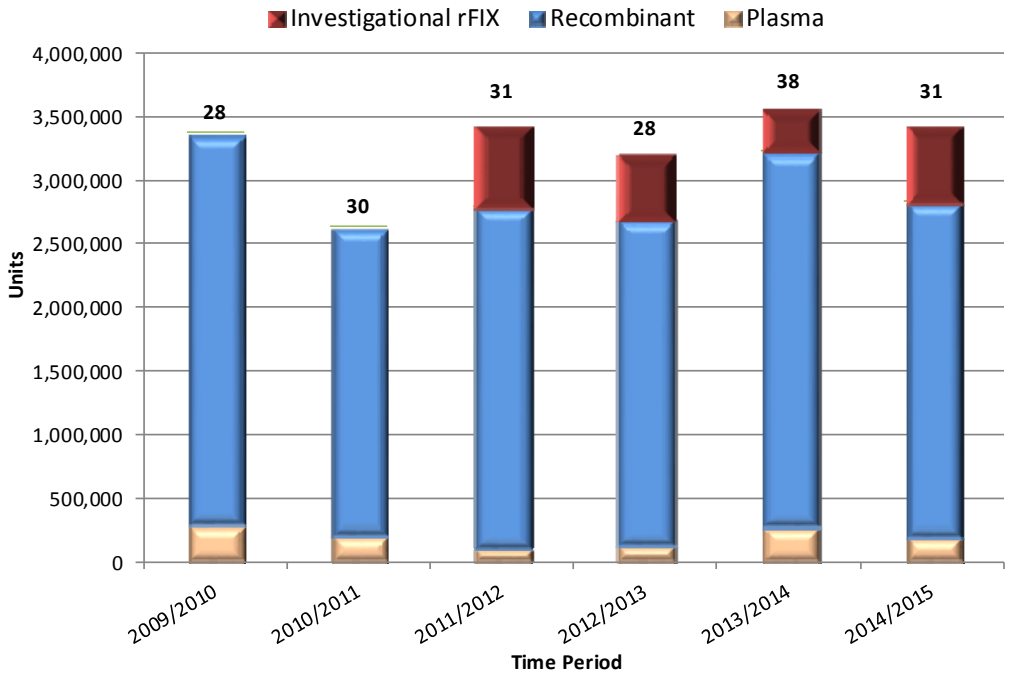

Table 20 Data table related to figure 5 - Factor IX units by financial year between April 2009 & March 2015

Year	Plasma		Recombinant		Investigational rFIX		Total		Patients	
	IU	% difference since 2009/10	IU	% difference since 2009/10	IU	% difference since 2011/12	IU	% difference since 2009/10	n	% difference since 2009/10
2009/2010	294,000	100.00%	3,071,500	100.00%	0		3,365,500	100.00%	28	100.00%
2010/2011	212,000	72.11%	2,413,000	78.56%	0		2,625,000	78.00%	30	107.14%
2011/2012	106,000	36.05%	2,679,500	87.24%	647,689	100.00%	3,433,189	102.01%	31	110.71%
2012/2013	133,000	45.24%	2,562,000	83.41%	517,180	79.85%	3,212,180	95.44%	28	100.00%
2013/2014	274,000	93.20%	2,947,520	95.96%	349,016	53.89%	3,570,536	106.09%	38	135.71%
2014/2015	198,000	67.35%	2,625,000	85.46%	617,447	95.33%	3,440,447	102.23%	31	110.71%

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	27,000
CSL Behring	Voncento	133,000
	Haemate P	609,000

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	-	8,000	-
CSL Behring	Fibrogammin P	-	-	11,250
	Haemate P	2,000	-	-
Novo Nordisk	NovoSeven (mg)	23	-	-
	Octaplas (units)	-	1,200	-

Units in IU unless otherwise stated

Table 23 Concentrates used to treat Acquired Defects

Manufacturer	Product	Acquired Haemophilia A
		Units
Baxter	FEIBA	74,000

Deaths and Adverse Events

Table 24 Causes of Death

Diagnosis	Cause of Death	Severity (factor level iu/dl)				Total
		≤1	>1 and <5	≥5	N/K	
Haemophilia B	Unknown	1	0	0	0	**1**
Females with VIII deficiency	Unknown	0	0	1	0	**1**
Acquired Haemophilia A	Unknown	0	0	1	2	**3**
		1	**0**	**2**	**2**	**5**

Table 25 Adverse Events

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

See Table 8 'Inhibitors by disease severity' for more information