UK Bleeding Disorder Statistics for April 2016 to March 2017

A report from the UK National Haemophilia Database

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Important Note: Throughout this report, Haemophilia A includes Carriers of Haemophilia A and Females with FVIII deficiency.

Haemophilia B includes Carriers of Haemophilia B, Females with FIX deficiency, patients with FIX Leyden and FIX Leyden carriers.

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Haemophilia A

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Table 1 Patients with congenital Haemophilia A (including carriers) registered and treated April 2016 - March 2017

Haemophilia A	Age Range	Factor VIII level (IU/dl)																	
		< 1			1 - 5			>5 & <40			≥ 40			Unknown			Total		
		M	F	Total	M	F	Total	M	F	Total	M	F	Total	M	F	Total	M	F	Total
Total in Register (including low-level carriers)	<18 years	694	-	694	192	4	196	600	97	697	27	131	158	-	5	5	1,513	237	1,750
	≥18 years	1,237	3	1,240	639	2	641	2,456	554	3,010	156	1,041	1,197	1	54	55	4,489	1,654	6,143
	Total	**1,931**	**3**	**1,934**	**831**	**6**	**837**	**3,056**	**651**	**3,707**	**183**	**1,172**	**1,355**	**1**	**59**	**60**	**6,002**	**1,891**	**7,893**
New Registrations * (including low-level carriers)	<18 years	51	-	51	14	-	14	55	17	72	7	28	35	-	-	-	127	45	172
	≥18 years	11	1	12	5	-	5	46	32	78	13	124	137	1	8	9	76	165	241
	Total	**62**	**1**	**63**	**19**	**-**	**19**	**101**	**49**	**150**	**20**	**152**	**172**	**1**	**8**	**9**	**203**	**210**	**413**
Treated in year** (including low-level carriers)	<18 years	661	-	661	147	3	150	151	10	161	6	4	10	-	-	-	965	17	982
	≥18 years	1,200	1	1,201	420	2	422	582	51	633	20	23	43	1	1	2	2,223	78	2,301
	Total	**1,861**	**1**	**1,862**	**567**	**5**	**572**	**733**	**61**	**794**	**26**	**27**	**53**	**1**	**1**	**2**	**3,188**	**95**	**3,283**
Treated with concentrate in year** (including low-level carriers)	<18 years	650	-	650	144	3	147	124	7	131	3	1	4	-	-	-	921	11	932
	≥18 years	1,146	1	1,147	401	2	403	480	26	506	16	7	23	1	1	2	2,044	37	2,081
	Total	**1,796**	**1**	**1,797**	**545**	**5**	**550**	**604**	**33**	**637**	**19**	**8**	**27**	**1**	**1**	**2**	**2,965**	**48**	**3,013**
Treated with Desmopressin in year** (including low-level carriers)	<18 years	1	-	1	2	-	2	40	4	44	3	3	6	-	-	-	46	7	53
	≥18 years	-	-	-	12	-	12	118	25	143	5	17	22	-	-	-	135	42	177
	Total	**1**	**-**	**1**	**14**	**-**	**14**	**158**	**29**	**187**	**8**	**20**	**28**	**-**	**-**	**-**	**181**	**49**	**230**
Treated with FEIBA® in year** (including low-level carriers)	<18 years	17	-	17	-	-	-	-	-	-	-	-	-	-	-	-	17	-	17
	≥18 years	51	-	51	12	-	12	9	-	9	-	-	-	-	-	-	72	-	72
	Total	**68**	**-**	**68**	**12**	**-**	**12**	**9**	**-**	**9**	**-**	**-**	**-**	**-**	**-**	**-**	**89**	**-**	**89**
Treated with NovoSeven® in year** (including low-level carriers)	<18 years	41	-	41	4	-	4	2	-	2	-	-	-	-	-	-	47	-	47
	≥18 years	29	-	29	6	-	6	5	-	5	-	-	-	-	-	-	40	-	40
	Total	**70**	**-**	**70**	**10**	**-**	**10**	**7**	**-**	**7**	**-**	**-**	**-**	**-**	**-**	**-**	**87**	**-**	**87**

** New registrations are a subset of the 'In Register' numbers*
*** Treated includes patients 'In Register' and 'New Registrations'*

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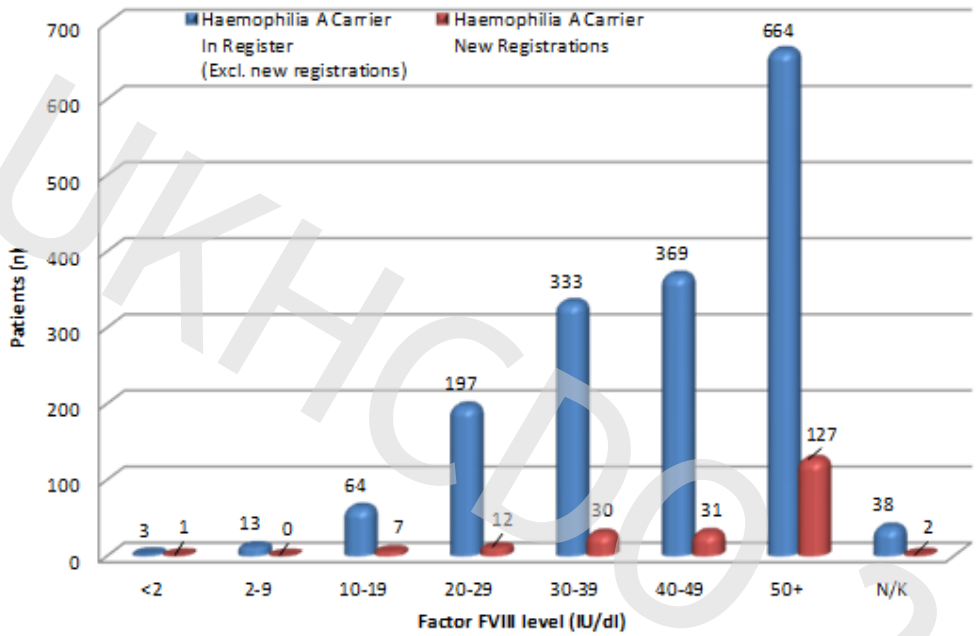
Figure 1 Carriers of Haemophilia A currently registered and newly registered, by baseline factor VIII level



N.B: Includes: Carrier of Haemophilia A and Females with FVIII deficiency

Diagnosis	Factor VIII level (IU/dl)								Grand Total
	<2	2-9	10-19	20-29	30-39	40-49	50+	N/K	
Haemophilia A Carrier In Register (Excl. new registrations)	3	13	64	197	333	369	664	38	1,681
Haemophilia A Carrier New Registrations	1	0	7	12	30	31	127	2	210
Total	**4**	**13**	**71**	**209**	**363**	**400**	**791**	**40**	**1,891**

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Table 2 New registrations of Haemophilia A (including carriers) between April 2016 & March 2017, by age at mid-year, gender and severity

Coagulation Defect	Age (years)	Number of Patients (Factor VIII level (IU/dl))																	
		< 1			1 - 5			> 5 & < 40			≥ 40			Unknown			Total		
		Male	Female	Total	Male	Female	Total	Male	Female	Total	Male	Female	Total	Male	Female	Total	Male	Female	Total
Haemophilia A	0 - < 2	39	0	39	10	0	10	20	2	22	1	6	7	0	0	0	70	8	78
	2 : 4	4	0	4	1	0	1	9	4	13	2	4	6	0	0	0	16	8	24
	5 : 9	3	0	3	2	0	2	12	2	14	2	9	11	0	0	0	19	11	30
	10 : 19	7	0	7	1	0	1	16	10	26	2	14	16	0	0	0	26	24	50
	20 : 29	7	1	8	3	0	3	10	10	20	0	34	34	0	2	2	20	47	67
	30 : 39	2	0	2	2	0	2	9	10	19	1	39	40	0	4	4	14	53	67
	40 : 49	0	0	0	0	0	0	4	7	11	3	14	17	0	2	2	7	23	30
	50 : 59	0	0	0	0	0	0	5	1	6	2	17	19	0	0	0	7	18	25
	60 : 69	0	0	0	0	0	0	6	0	6	4	6	10	1	0	1	11	6	17
	70 +	0	0	0	0	0	0	10	3	13	3	9	12	0	0	0	13	12	25
	Total	**62**	**1**	**63**	**19**	**0**	**19**	**101**	**49**	**150**	**20**	**152**	**172**	**1**	**8**	**9**	**203**	**210**	**413**

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Table 3 New registrations of patients with Severe Haemophilia A aged over 2 years old, and subsequent treatment by year

Haemophilia A	Registration year									Total 2008/2017
	2008/09	2009/10	2010/11	2011/12	2012/13	2013/14	2014/15	2015/16	2016/17	
New registrations per year	18	14	13	16	13	22	22	26	23	167
Treated in each year										
2008/09	18	1								19
2009/10	17	10								27
2010/11	16	11	12							39
2011/12	15	11	11	15						52
2012/13	13	11	9	15	12					60
2013/14	14	10	10	13	11	21				79
2014/15	13	10	10	13	11	21	21			99
2015/16	13	11	8	11	10	20	21	25		119
2016/17	12	10	7	11	10	20	19	21	23	133
No treatment records	0	1	1	0	1	0	0	0	0	3

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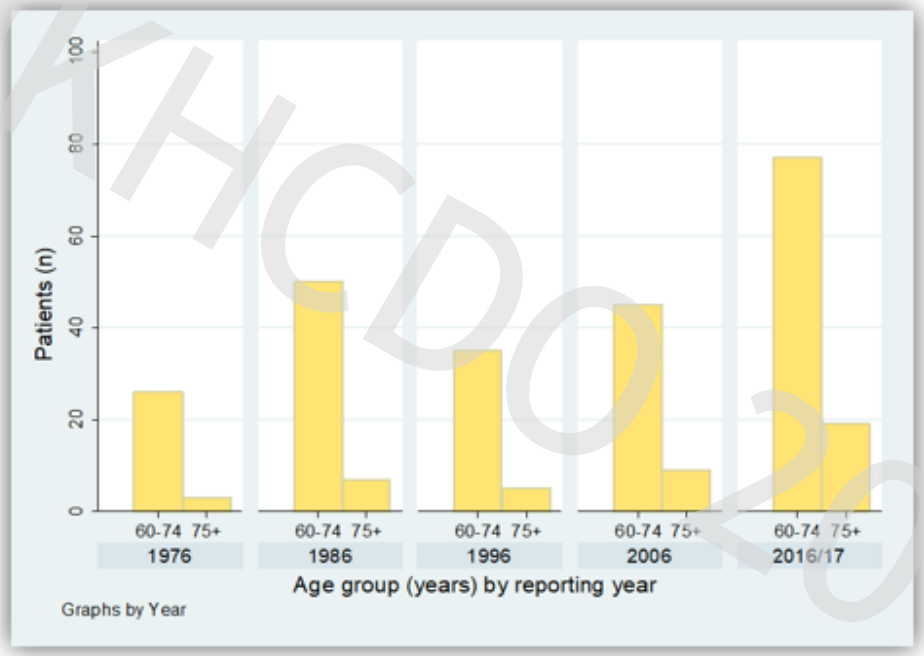
Figure 2a Trend in the number of patients with Severe Haemophilia A aged 60 years and above, 1976 - 2016/17 (including HIV +ve pts)



N.B. Carriers included in figures 2a - 2c

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Figure 2b Trend in the number of patients with Moderate Haemophilia A aged 60 years and above, 1976 - 2016/17 (including HIV +ve pts)

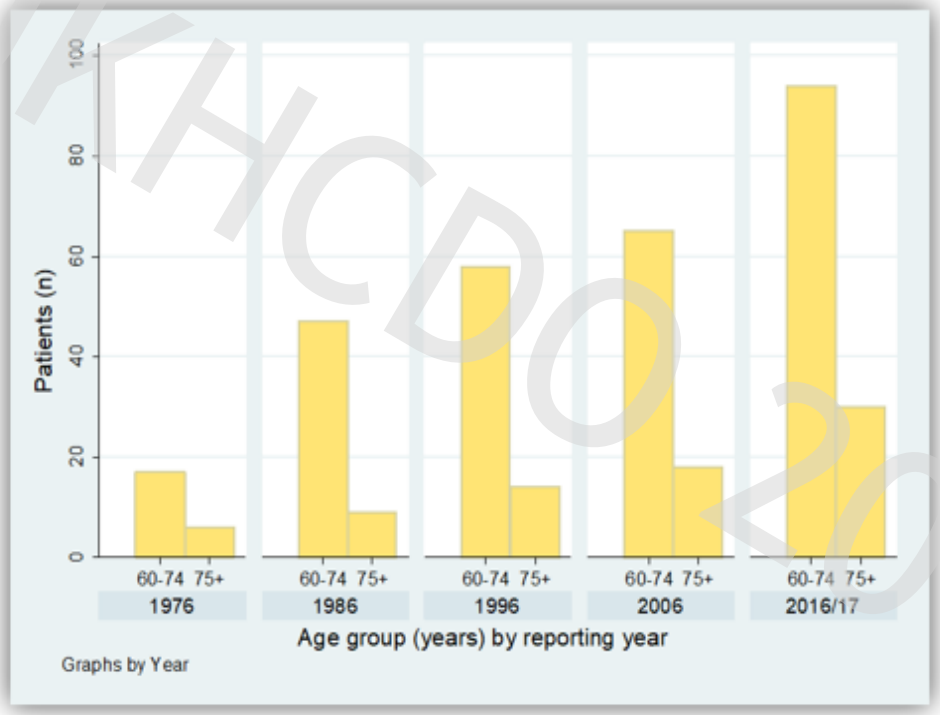

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Figure 2c Trend in the number of patients with Mild Haemophilia A aged 60 years and above, 1976 - 2016/17 (including HIV +ve pts)

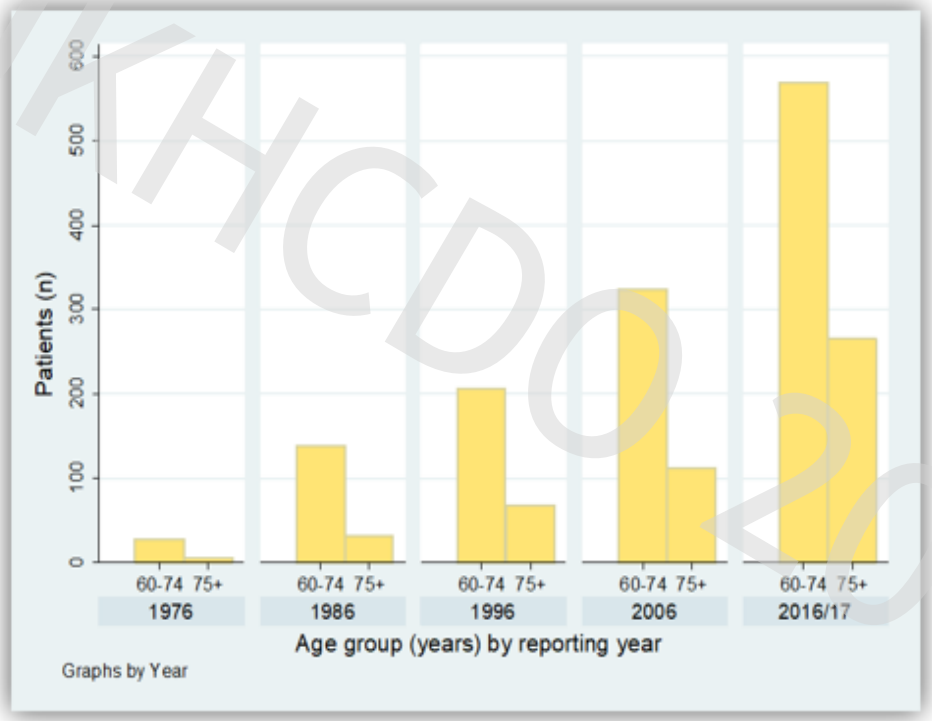

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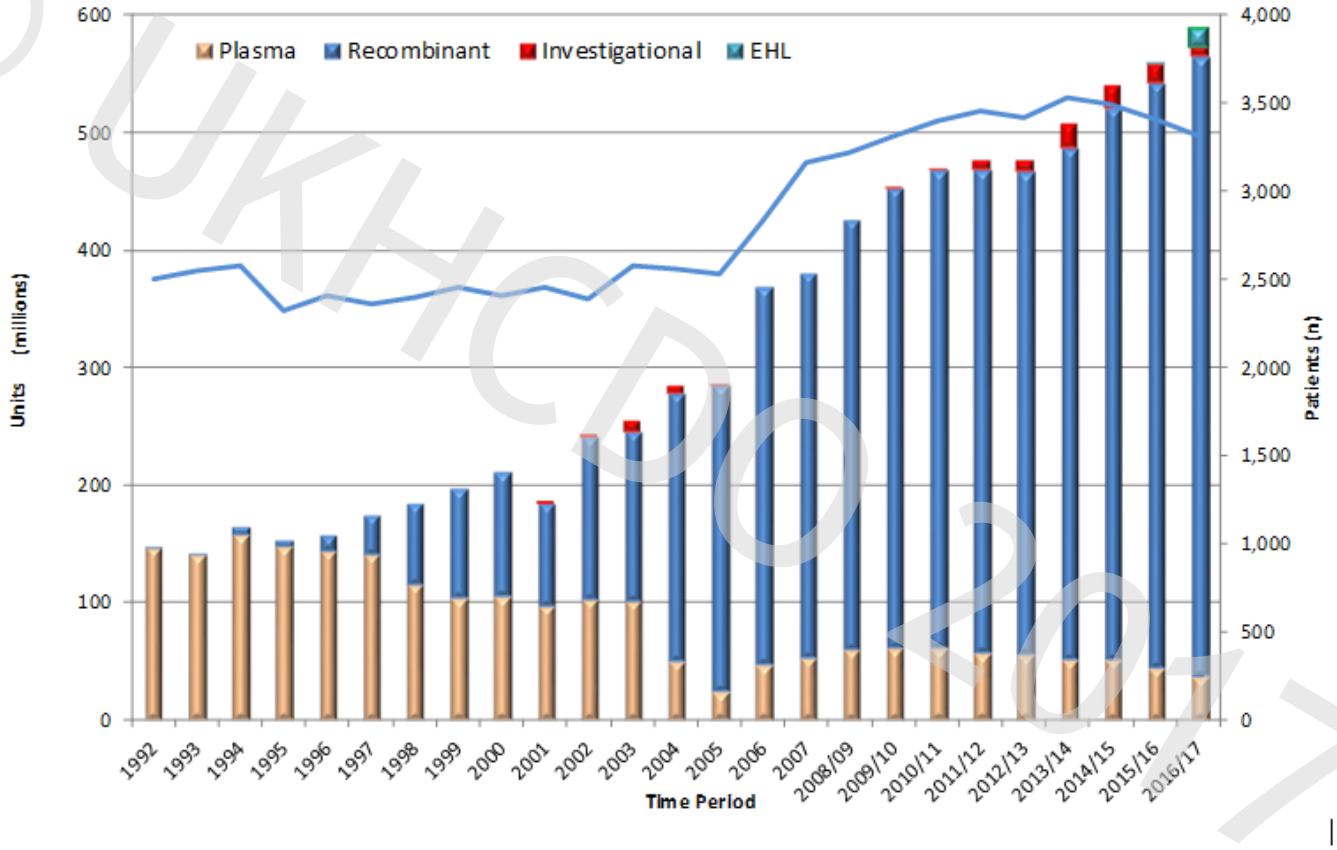
Figure 3 Factor VIII units used by UK Haemophilia Centres 1992 - 2016/17 all diagnoses



N.B: Data for St Thomas' were not submitted 1996-2006.

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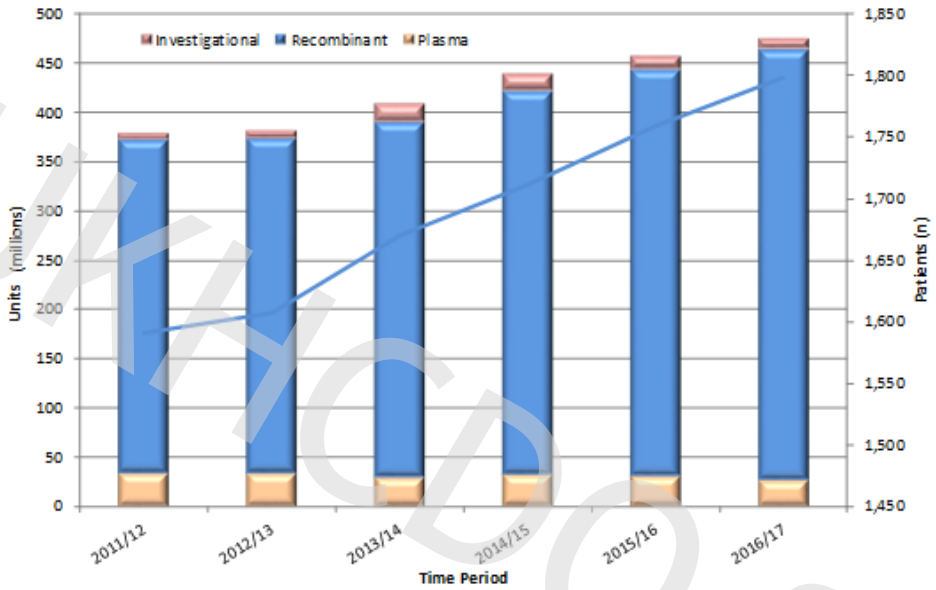
Figure 4 Factor VIII units used by UK Haemophilia Centres to treat Severe Haemophilia A - 2011/12 - 2016/17



Year	Plasma		Recombinant (excluding Investigational)		Investigational rFVIII		Total		Patients	
	IU (millions)	% difference since 2011/12	IU (millions)	% difference since 2011/12	IU (millions)	% difference since 2011/12	IU (millions)	% difference since 2011/12	n	% difference since 2011/12
2011/12	34,524	100.00	338,254	100.00	6,643	100.00	379,421	100.00	1,591	100.00
2012/13	34,072	98.69	338,960	100.21	8,200	123.44	381,232	100.48	1,607	101.01
2013/14	30,780	89.15	358,758	106.06	18,825	283.40	408,364	107.63	1,670	104.97
2014/15	32,932	95.39	388,512	114.86	17,703	266.50	439,147	115.74	1,711	107.54
2015/16	31,363	90.84	411,307	121.60	14,360	216.17	457,030	120.45	1,759	110.56
2016/17	27,916	80.86	435,819	128.84	9,678	145.70	473,414	124.77	1,798	113.01

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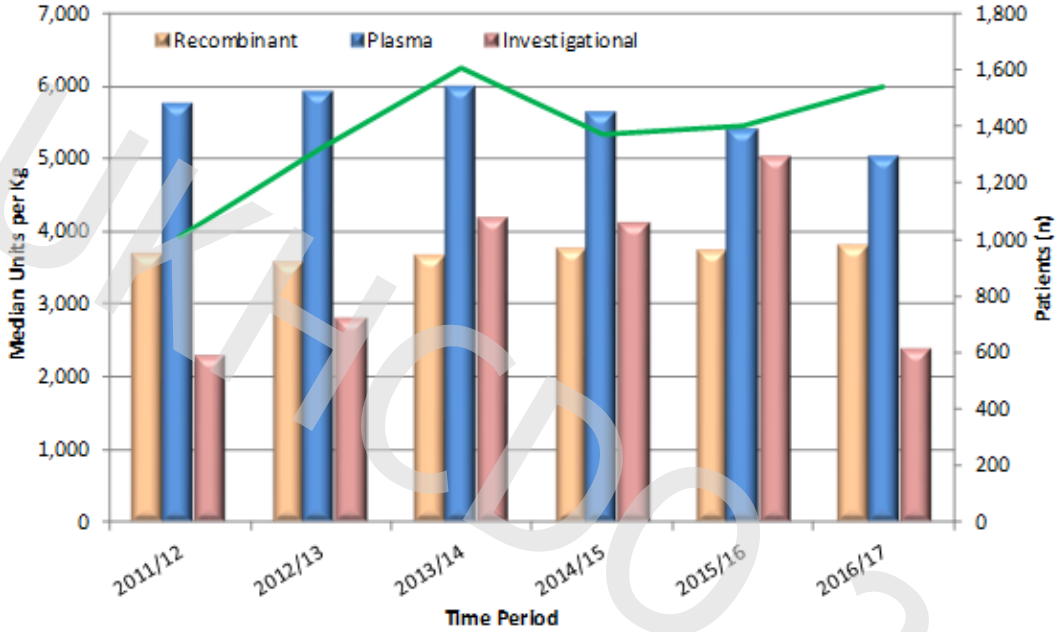
Figure 5 Factor VIII units per kilogram used by UK Haemophilia Centres to treat Severe Haemophilia A - 2011/12 - 2016/17



Year	Plasma		Recombinant (excluding investigational)		Investigational rFVIII		Total		Patients with a known weight	
	Median IU/Kg	% difference since 2011/12	Median IU/Kg	% difference since 2011/12	Median IU/Kg	% difference since 2011/12	Median IU/Kg	% difference since 2011/12	n	% difference since 2011/12
2011/12	5,768	100.00	3,692	100.00	2,308	100.00	3,867	100.00	1,009	100.00
2012/13	5,939	102.96	3,577	96.89	2,814	121.92	3,715	96.07	1,323	131.12
2013/14	6,000	104.02	3,680	99.67	4,185	181.33	3,871	100.10	1,606	159.17
2014/15	5,644	97.85	3,771	102.14	4,133	179.07	3,933	101.71	1,369	135.68
2015/16	5,411	93.81	3,746	101.46	5,038	218.28	3,870	100.08	1,404	139.15
2016/17	5,051	87.57	3,821	103.49	2,393	103.68	3,949	102.12	1,540	152.63

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Table 4 Treatment intensity of patients with Severe Haemophilia A, with no inhibitor - 2011/12 - 2016/17

Treatment Period	FVIII Units		Patients (n)		Treatment Intensity (Units/Pt)		Change in treatment intensity since 2011/12 (%)	
	<18 Years	≥18 years	<18 Years	≥18 years	<18 Years	≥18 years	<18 Years	≥18 years
2011/12	100,308,938	241,236,309	565	951	177,538	253,666	100.0	100.0
2012/13	96,316,420	236,407,243	552	944	174,486	250,431	98.3	98.7
2013/14	103,213,817	261,444,314	578	1,001	178,571	261,183	100.6	103.0
2014/15	107,628,003	284,539,478	571	1,042	188,490	273,071	106.2	107.6
2015/16	106,809,190	299,558,834	573	1,082	186,403	276,857	105.0	109.1
2016/17	112,875,983	318,137,100	571	1,124	197,681	283,040	111.3	111.6

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Table 5 Treatment intensity of patients with Moderate Haemophilia A, with no inhibitor - 2011/12 - 2016/17

Treatment Period	FVIII Units		Patients (n)		Treatment Intensity (Units/Pt)		Change in treatment intensity since 2011/12 (%)	
	<18 Years	≥18 years	<18 Years	≥18 years	<18 Years	≥18 years	<18 Years	≥18 years
2011/12	11,497,191	46,108,773	154	399	74,657	115,561	100.0	100.0
2012/13	11,961,548	45,099,101	150	373	79,744	120,909	106.8	104.6
2013/14	14,078,000	47,377,909	161	386	87,441	122,741	117.1	106.2
2014/15	16,128,950	49,283,041	157	388	102,732	127,018	137.6	109.9
2015/16	15,848,750	54,252,983	145	403	109,302	134,623	146.4	116.5
2016/17	16,990,470	57,507,930	143	393	118,814	146,331	159.1	126.6

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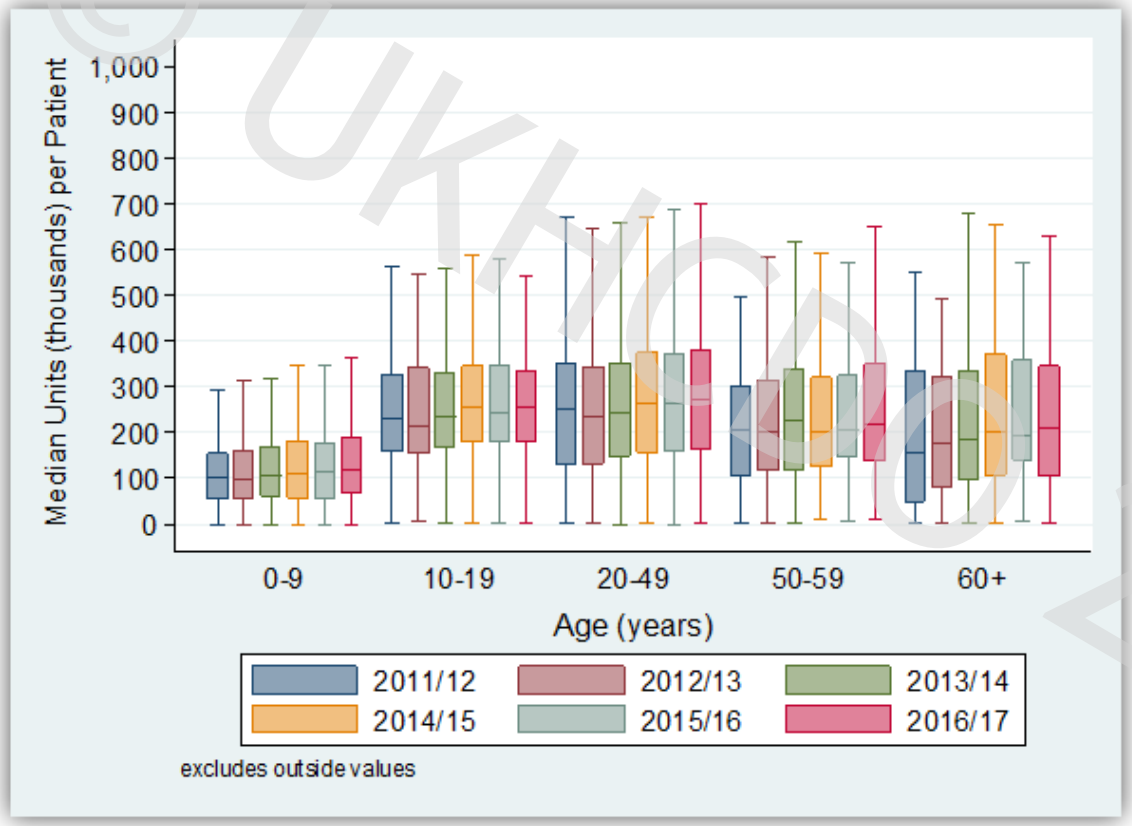
Figure 6 Median usage of factor VIII: UK patients with severe Haemophilia A without inhibitors broken down by age, 2011/12 - 2016/17



Data table for Figure 6

Age Range (years)	Change in median units from 2011/12 to 2016/17 (%)
0-9	18.3
10-19	11.2
20-29	3.7
30-39	15.7
40-49	9.9
50-59	5.8
60+	34.6

N.B. figure based on total usage, not units/kg

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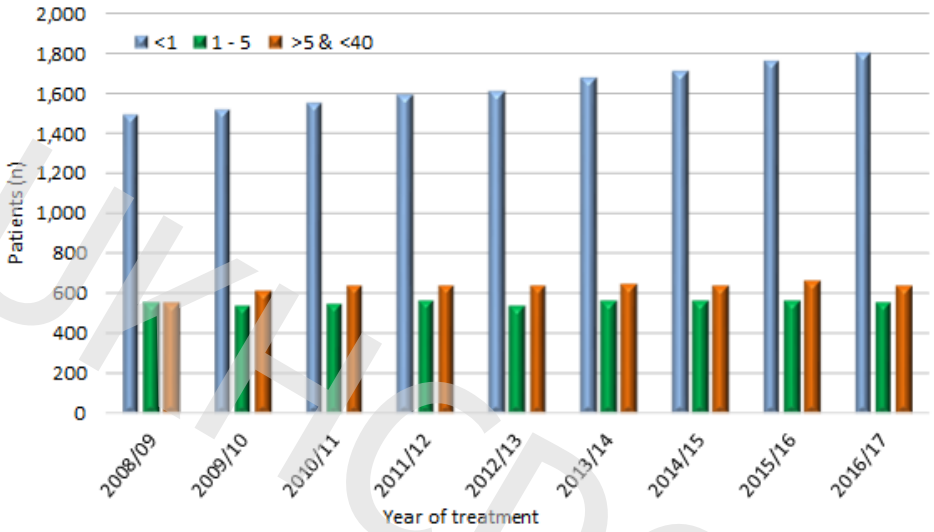
Figure 7 Patients with Severe / Moderate / Mild Haemophilia A treated with FVIII by UK Haemophilia Centres - 2008/09 - 2016/17



Treatment Year	Haemophilia A <1		Haemophilia A 1 - 5		Haemophilia A >5 & <40		Haemophilia A ≥ 40	
	n	% difference since 2008/09	n	% difference since 2008/09	n	% difference since 2008/09	n	% difference since 2008/09
2008/09	1490	100.0	552	100.0	554	100.0	22	100.0
2009/10	1513	101.5	535	96.9	609	109.9	27	122.7
2010/11	1549	104.0	543	98.4	634	114.4	26	118.2
2011/12	1591	106.8	560	101.4	637	115.0	20	90.9
2012/13	1607	107.9	534	96.7	635	114.6	19	86.4
2013/14	1670	112.1	563	102.0	642	115.9	19	86.4
2014/15	1711	114.8	561	101.6	638	115.2	21	95.5
2015/16	1759	118.1	558	101.1	663	119.7	21	95.5
2016/17	1798	120.7	550	99.6	637	115.0	27	122.7

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Table 6 Factor VIII usage by region for patients with Severe Haemophilia A (incl. treatment for inhibitors)

Region	General Population	Patients treated (n) Severe Haemophilia A	Total FVIII Units (IU) Severe Haemophilia A	Mean Usage Severe Haemophilia A	FVIII Units Per Capita Severe Haemophilia A
Yorkshire and the Humber	5,425,741	124	41,164,500	331,972	7.59
London	8,787,892	310	94,567,407	305,056	10.76
East of England	6,130,542	128	37,671,121	294,306	6.14
North East	2,636,848	61	17,871,831	292,981	6.78
South East	9,026,297	292	80,399,971	275,342	8.91
South West	5,515,953	133	34,824,090	261,835	6.31
North West	7,219,623	166	36,388,750	219,209	5.04
East Midlands	4,724,437	145	29,966,297	206,664	6.34
West Midlands	5,800,734	153	29,183,165	190,740	5.03
England	**45,760,739**	**1,512**	**402,037,132**	**265,898**	**8.79**
Northern Ireland	1,862,100	70	14,485,000	206,929	7.78
Scotland East	3,021,290	80	23,293,750	291,172	7.71
Scotland West	2,351,710	58	15,882,250	273,832	6.75
Wales	3,113,200	77	17,692,486	229,773	5.68
United Kingdom	**65,648,100**	**1,797**	**473,390,618**	**263,434**	**7.21**

English regions ranked by mean usage

Source accreditation:

Population Estimates for UK, England and Wales, Scotland and Northern Ireland: Mid-2016: Office for National Statistics licensed under the Open Government Licence v.3.0. © Crown copyright 2017

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NOTE: Patients are allocated to each region based on their home postcode, rather than where they were treated

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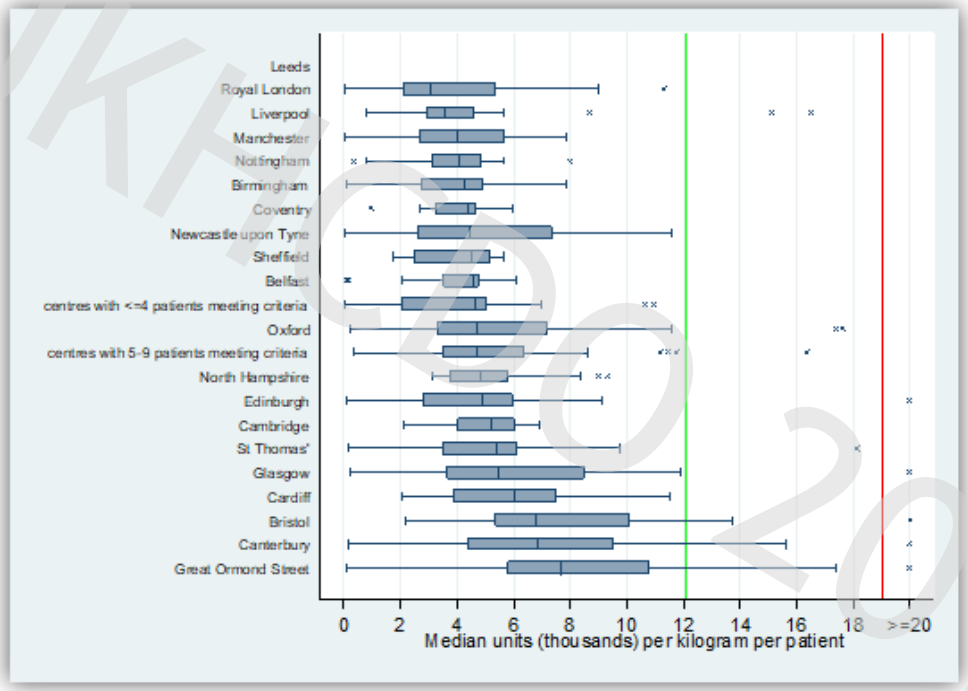
*Figure 8a Annual FVIII usage **(IU/Kg/Pt)** 2016/17 in patients with Severe Haemophilia A **aged under 18 years** with no current inhibitor, by centre, ranked by median usage*



N.B: The 95th percentile of units = 2,069 IU per kilogram

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Data table for Figures 8a & b

Haemophilia Centre	Patients treated	Patients treated with weight reported	Total Units	Median Units	Median Units/Kg
Belfast	17	17	2,179,000	137,500	4,560
Birmingham	41	38	6,202,750	141,750	4,242
Bristol	25	21	5,731,500	189,000	6,750
Cambridge	21	17	3,735,250	129,000	5,206
Canterbury	14	14	3,264,750	181,750	6,887
Cardiff	12	10	2,651,000	204,750	6,048
Coventry	10	10	1,433,000	143,125	4,389
Edinburgh	10	9	1,920,000	88,625	4,915
Glasgow	25	25	5,797,250	170,000	5,468
Great Ormond Street	81	81	22,018,103	246,250	7,672
Leeds	24	0	5,904,000	218,375	-
Liverpool	22	21	3,400,250	140,250	3,571
Manchester	50	49	7,451,750	128,000	3,986
Newcastle upon Tyne	19	19	4,452,000	220,000	4,484
North Hampshire	18	17	3,055,250	172,500	4,843
Nottingham	16	16	2,039,250	105,875	4,090
Oxford	32	32	6,893,000	177,875	4,684
Royal London	17	14	2,487,047	135,000	3,067
Sheffield	15	15	2,666,750	148,500	4,525
St Thomas'	23	19	3,984,250	159,000	5,403
Centres with <=4 patients meeting criteria	25	18	4,362,718	163,500	4,639
Centres with 5-9 patients meeting criteria	54	46	11,247,115	187,000	4,734

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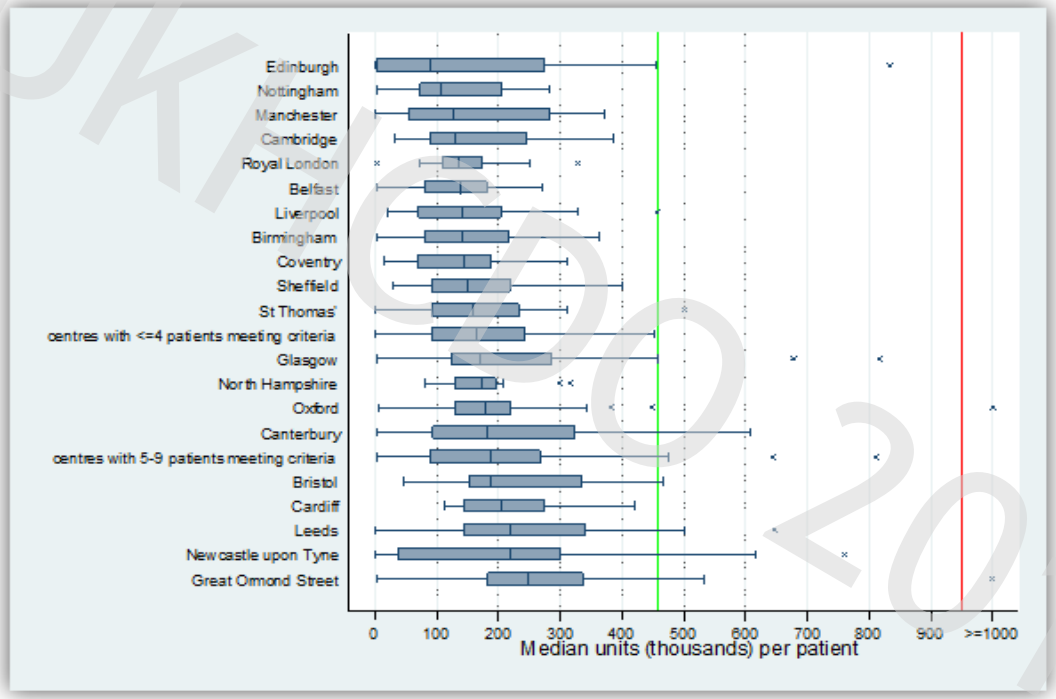
*Figure 8b Annual FVIII usage **(IU/Pt)** 2016/17 in patients with Severe Haemophilia A **aged under 18 years** with no current inhibitor, by centre, ranked by median usage per patient*



N.B: The 95th percentile of units = 457,500 IU

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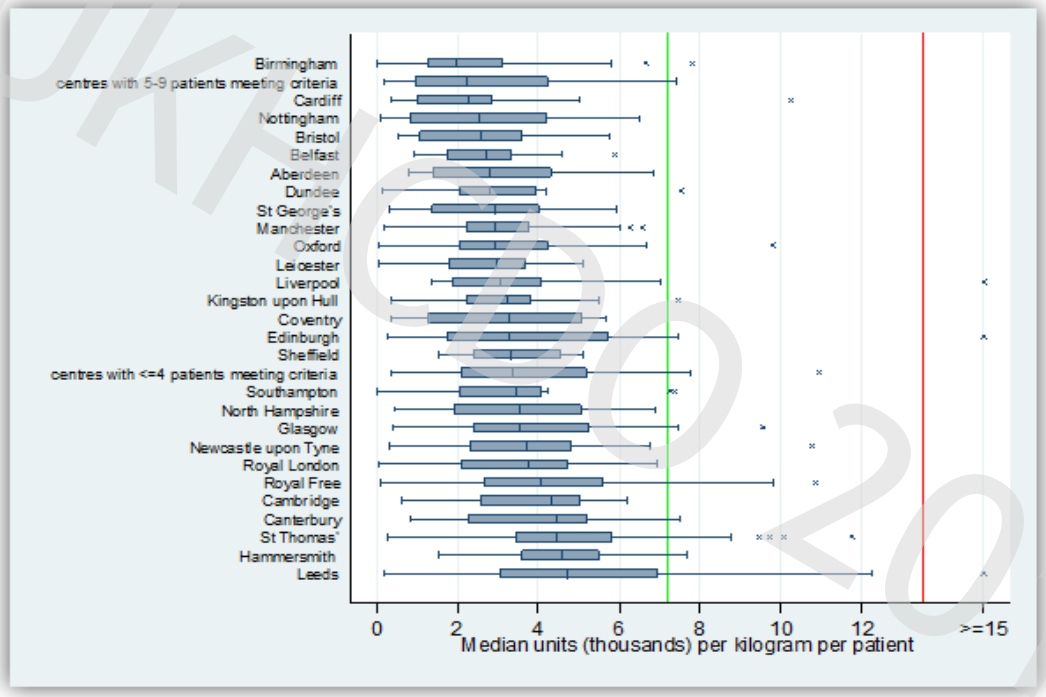
*Figure 9a Annual FVIII usage **(IU/Kg/Pt)** 2016/17 in patients with Severe Haemophilia A **aged 18 years or more** with no current inhibitor, by centre, ranked by median usage*



N.B: The 95th percentile of units = 7,203 IU kilogram

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Data table for Figures 9a & b

Haemophilia Centre	Patients treated	Patients treated with weight reported	Total Units	Median Units	Median Units/Kg
Aberdeen	12	8	3,457,500	283,750	2,777
Belfast	44	43	9,557,000	212,000	2,743
Birmingham	69	50	14,150,000	180,000	1,978
Bristol	30	24	6,252,000	187,000	2,564
Cambridge	31	26	9,054,250	272,500	4,332
Canterbury	22	18	7,702,000	349,000	4,437
Cardiff	34	24	6,560,761	168,250	2,269
Coventry	11	11	2,834,700	242,000	3,270
Dundee	13	13	3,389,500	240,000	2,818
Edinburgh	21	20	6,615,500	254,000	3,279
Glasgow	39	39	11,667,250	297,000	3,562
Hammersmith	27	22	8,771,000	338,000	4,606
Kingston upon Hull	16	13	4,335,750	244,000	3,266
Leeds	33	25	15,971,500	382,000	4,722
Leicester	20	20	4,644,661	237,000	2,989
Liverpool	31	22	8,904,000	240,000	3,056
Manchester	55	50	13,383,750	234,000	2,938
Newcastle upon Tyne	41	38	12,456,000	300,000	3,738
North Hampshire	50	43	13,894,800	256,000	3,538
Nottingham	28	27	5,789,586	177,000	2,526
Oxford	94	91	23,339,250	255,250	2,948
Royal Free	132	127	43,508,158	334,000	4,056
Royal London	44	30	11,385,574	280,170	3,742
Sheffield	19	8	6,730,000	308,000	3,333
Southampton	16	13	3,845,500	244,000	3,450
St George's	26	23	5,886,360	242,000	2,930
St Thomas'	103	76	38,518,750	360,000	4,450
Centres with <=4 patients meeting criteria	32	20	9,053,000	259,500	3,383
Centres with 5-9 patients meeting criteria	31	21	6,479,000	144,000	2,231

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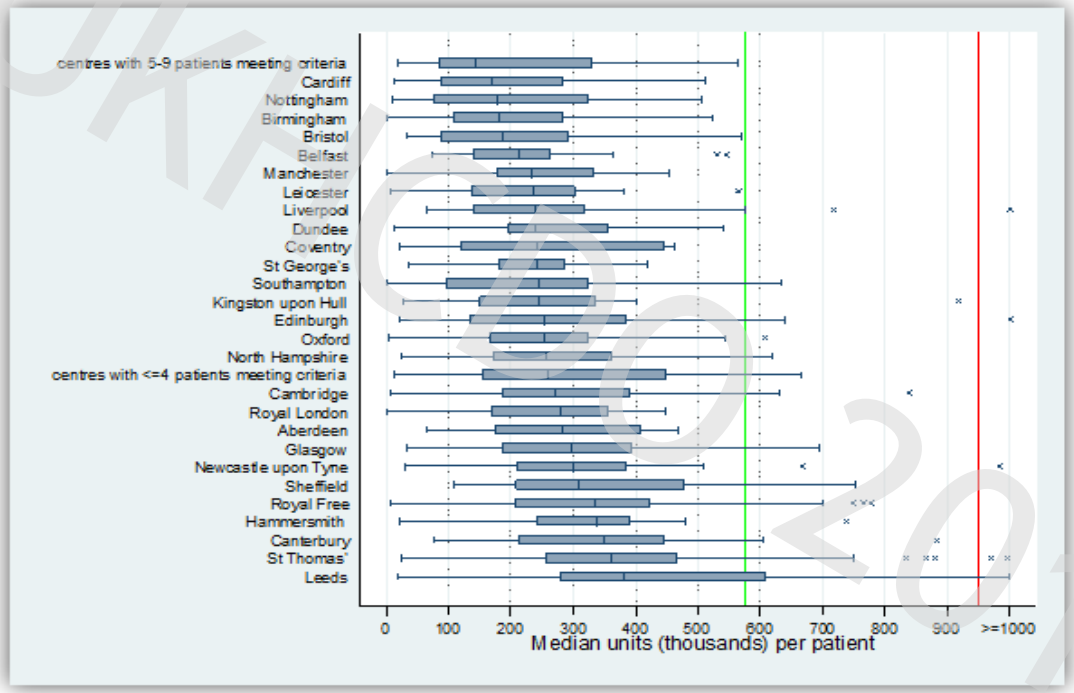
*Figure 9b Annual FVIII usage **(IU/Pt)** 2016/17 in patients with Severe Haemophilia A **aged 18 years or more** with no current inhibitor, by centre, ranked by median usage per patient*



N.B: The 95th percentile of units = 575,000 IU

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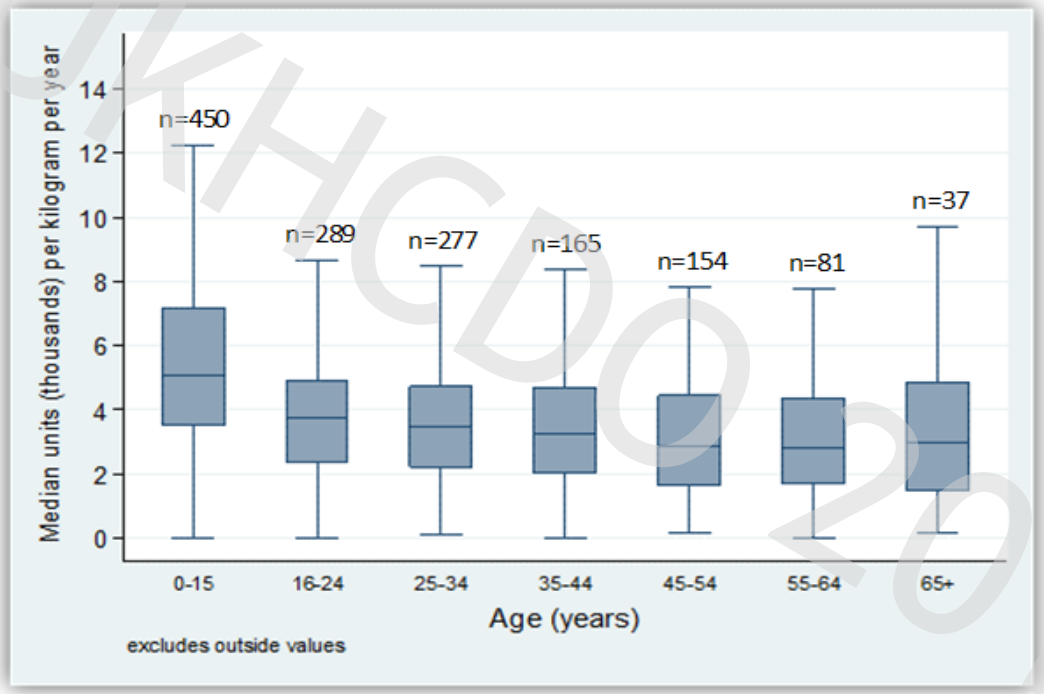
Figure 10 Median factor VIII units used per kilogram body weight per year in patients with Severe Haemophilia A without inhibitors by age, 2016/17



Weight data are missing for 263/1696

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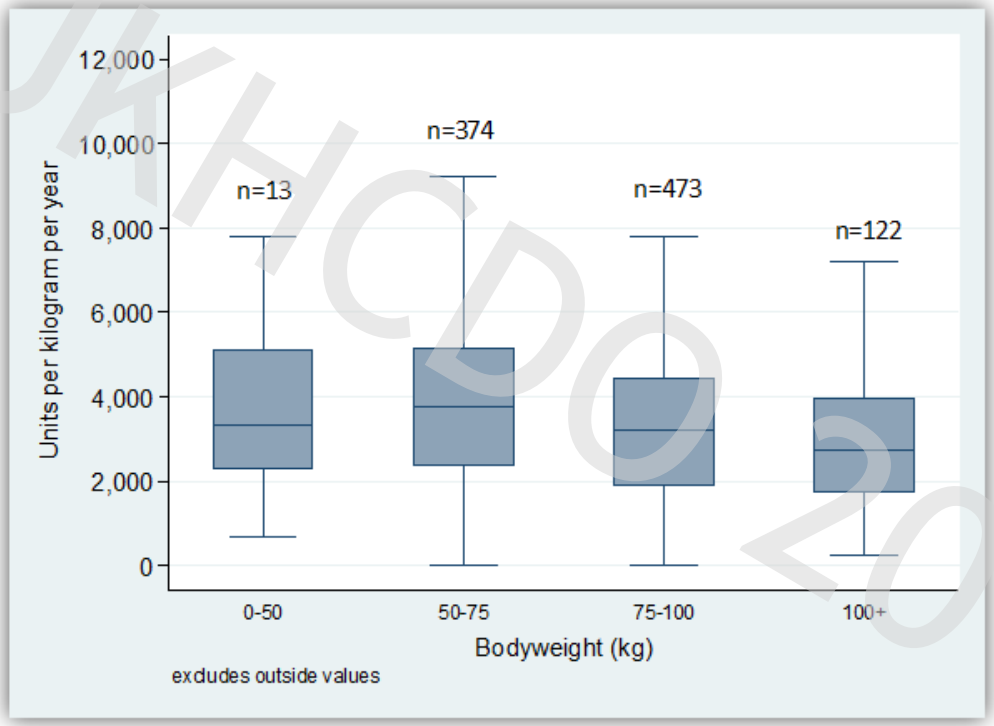
Figure 11 Median factor VIII units used (IU/kg) in patients with Severe Haemophilia A without inhibitors aged 16 and over, by bodyweight 2016/17



Weight data are missing for 213/982

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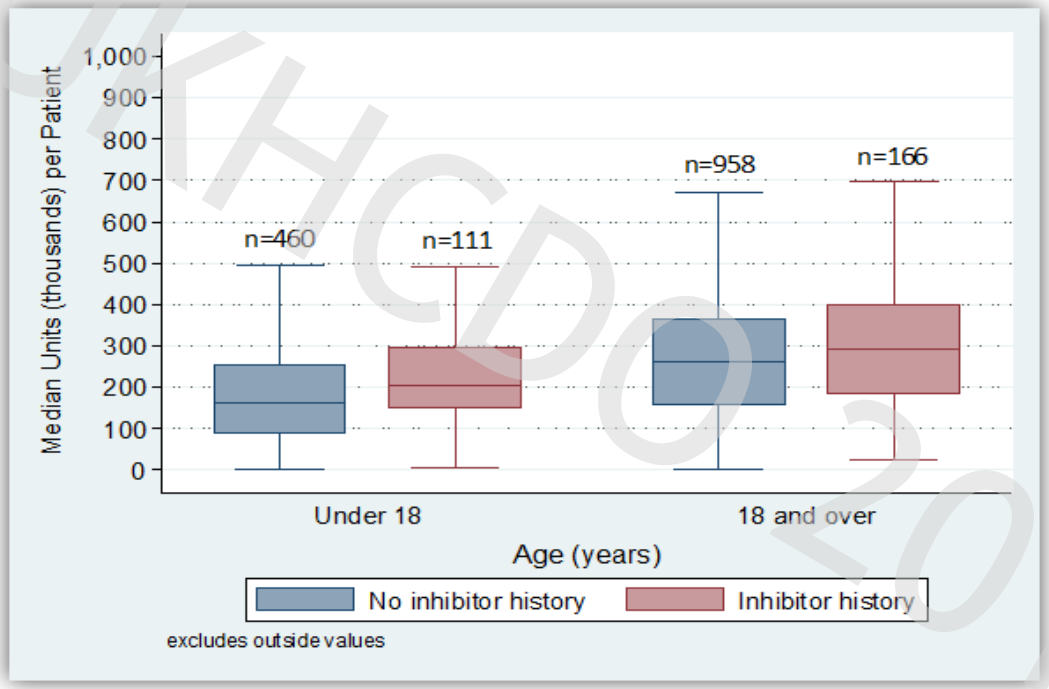
Figure 12 Patients with Severe Haemophilia A with no current inhibitor between April 2016 & March 2017: median usage by inhibitor history



Whitney U test: Under 18 years: p<0.005 (significant); 18 years and over: p=0.03 (significant)

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Table 7 Products used to treat Haemophilia A (including inhibitors) between April 2016 & March 2017

Manufacturer	Product	Total Units	
		2015/16	2016/17
Baxalta	Advate	164,292,360	175,084,058
	FEIBA	36,176,573	35,104,000
Bayer	Kogenate	21,489,750	19,542,086
Biotest	Haemoctin	340,000	336,000
BPL	FVIII 8Y	256,620	221,510
	Optivate	4,715,460	4,500,425
CSL Behring	Haemate P	202,000	79,500
	Helixate Nexgen	27,913,250	24,401,450
	Riastap (g)	-	3
	Voncento	-	5,000
Grifols	Alphanate	142,000	114,000
	Fanhdi	25,281,600	19,616,500
LFB Biomedicaments	Willfact / Wilfactin	-	2,000
Novo Nordisk	NovoSeven (mg)	23,124	19,700
Octapharma	Nuwiq	5,218,500	6,013,280
	Octanate	3,572,500	5,086,500
	Octaplex	-	3,000
Pfizer	BeneFIX	1,000	4,000
	ReFacto AF	276,868,501	284,693,866
SOBI/Biogen	Elocta	285,250	15,257,316
	Desmopressin	27,667	23,767
Various Manufacturers	Investigational Factor VIII	16,147,178	10,573,777

Units in IU unless otherwise stated

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Table 8 Factor VIII units used by UK Haemophilia Centres between April 2016 and March 2017, broken down by diagnosis

Coagulation Defect	Patients Treated	Plasma FVIII (IU)	Recombinant FVIII (IU)	Investigational FVIII (IU)	Enhanced Half-Life FVIII (IU)	Total FVIII (IU)
Haemophilia A	3,015	29,959,435	509,734,740	10,573,777	15,257,316	565,525,268
Acquired Haemophilia A	5	4,000	162,500	-	-	166,500
Haemophilia B*	1	-	-	-	1,000	1,000
von Willebrand disease	755	19,805,754	132,500	-	-	19,938,254
Acquired von Willebrand Disease	36	2,299,000	-	-	-	2,299,000
Platelet-type Pseudo von Willebrand Disease	2	4,000	-	-	-	4,000
Probable von Willebrand disease	2	4,000	-	-	-	4,000
F.VII deficiency*	1	-	90,000	-	-	90,000
Combined V+VIII Deficiency	8	-	50,000	-	-	50,000
Co-inherited diagnoses	15	399,100	1,397,750	-	-	1,796,850
Miscellaneous*	6	132,500	-	-	-	132,500
Unclassified bleeding disorder	1	500	-		-	500
Total	**3,847**	**52,608,289**	**511,567,490**	**10,573,777**	**15,258,316**	**590,007,872**

Products containing VWF as well as FVIII are reported in FVIII units

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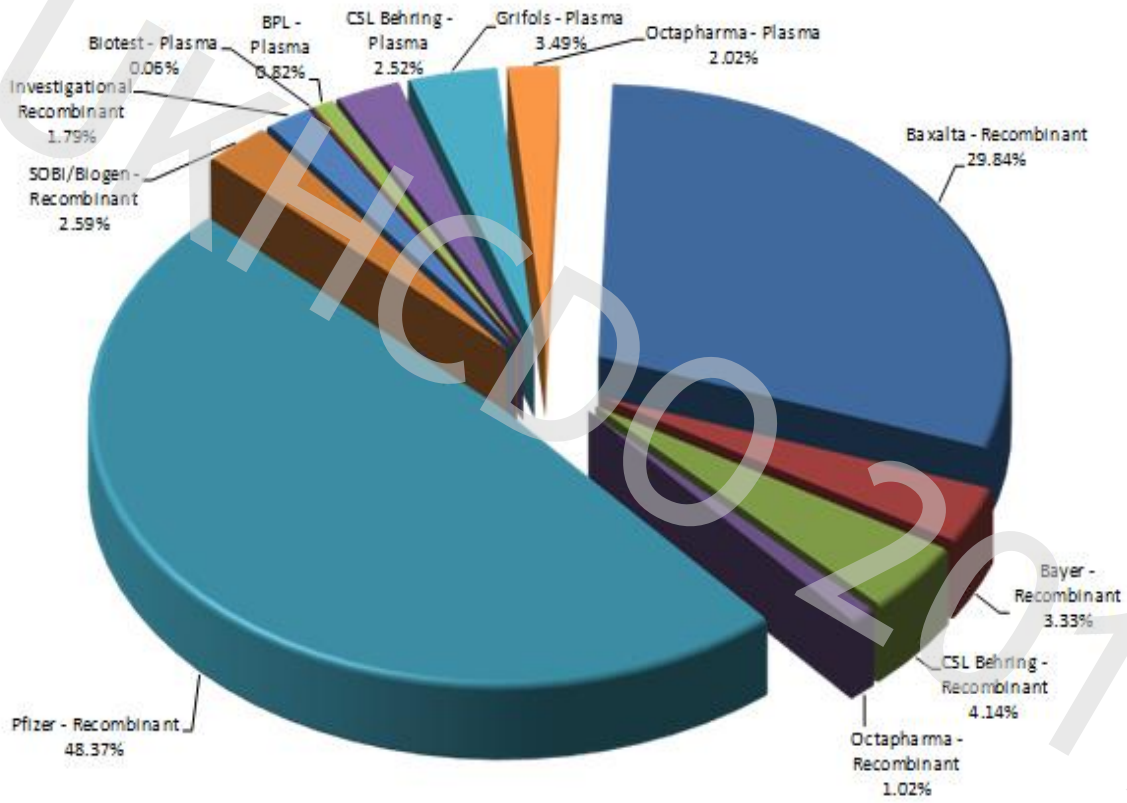
Figure 13 Market Share of factor VIII concentrates issued by UK Haemophilia Centres: April 2016 & March 2017



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Haemophilia B

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Table 9 Patients with congenital Haemophilia B (including carriers) registered and treated April 2016 - March 2017

Haemophilia B	Age Range	Factor IX level (IU/dl)																	
		< 1			1 - 5			>5 & <40			≥ 40			Unknown			Total		
		M	F	Total	M	F	Total	M	F	Total	M	F	Total	M	F	Total	M	F	Total
Total In Register (including deficient females and carriers)	<18years	117	-	117	83	-	83	98	51	149	4	32	36	-	1	1	302	84	386
	≥18years	243	1	244	256	5	261	380	217	597	36	217	253	-	5	5	915	445	1,360
	Total	**360**	**1**	**361**	**339**	**5**	**344**	**478**	**268**	**746**	**40**	**249**	**289**	**-**	**6**	**6**	**1,217**	**529**	**1,746**
New Registrations * (including deficient females and carriers)	<18years	7	-	7	7	-	7	15	12	27	-	10	10	-	-	-	29	22	51
	≥18years	1	-	1	3	-	3	3	15	18	1	28	29	-	1	1	8	44	52
	Total	**8**	**-**	**8**	**10**	**-**	**10**	**18**	**27**	**45**	**1**	**38**	**39**	**-**	**1**	**1**	**37**	**66**	**103**
Treated in year** (including deficient females and carriers)	<18years	110	-	110	46	-	46	32	5	37	-	-	-	-	-	-	188	5	193
	≥18years	225	-	225	136	-	136	94	28	122	3	10	13	-	-	-	458	38	496
	Total	**335**	**-**	**335**	**182**	**-**	**182**	**126**	**33**	**159**	**3**	**10**	**13**	**-**	**-**	**-**	**646**	**43**	**689**
Treated with FIX concentrate in year** (including deficient females and carriers)	<18years	106	-	106	46	-	46	32	5	37	-	-	-	-	-	-	184	5	189
	≥18years	217	-	217	136	-	136	94	27	121	3	10	13	-	-	-	450	37	487
	Total	**323**	**-**	**323**	**182**	**-**	**182**	**126**	**32**	**158**	**3**	**10**	**13**	**-**	**-**	**-**	**634**	**42**	**676**

** New registrations are a subset of the 'In Register' numbers*
*** Treated includes patients 'In Register' and 'New Registrations'*

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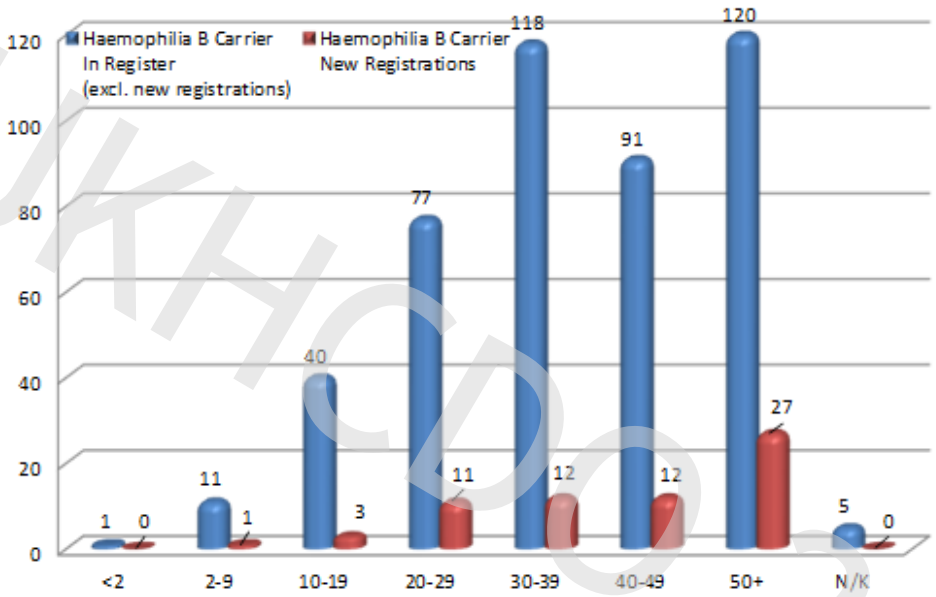
Figure 14 Carriers of Haemophilia B currently registered, by baseline factor IX level



Diagnosis	Factor IX level (IU/dl)								Grand Total
	<2	2-9	10-19	20-29	30-39	40-49	50+	N/K	
Haemophilia B Carrier In Register (excl. new registrations)	1	11	40	77	118	91	120	5	463
Haemophilia B Carrier New Registrations	0	1	3	11	12	12	27	0	66
Total	1	12	43	88	130	103	147	5	529

N.B: Includes: Carrier of Haemophilia B and Females with FIX deficiency

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Table 10 New registrations of Haemophilia B between April 2016 & March 2017, by age at mid-year, gender and severity

Coagulation Defect	Age (years)	Number of Patients (Factor IX level (IU/dl))																	
		< 1			1 - 5			> 5 & < 40			≥ 40			Unknown			Total		
		Male	Female	Total	Male	Female	Total	Male	Female	Total	Male	Female	Total	Male	Female	Total	Male	Female	Total
Haemophilia B	0 - < 2	6	0	6	3	0	3	4	2	6	0	1	1	0	0	0	13	3	16
	2 : 4	0	0	0	3	0	3	3	3	6	0	1	1	0	0	0	6	4	10
	5 : 9	1	0	1	0	0	0	3	3	6	0	5	5	0	0	0	4	8	12
	10 : 19	0	0	0	1	0	1	6	4	10	0	3	3	0	0	0	7	7	14
	20 : 29	1	0	1	2	0	2	0	4	4	0	6	6	0	1	1	3	11	14
	30 : 39	0	0	0	0	0	0	1	5	6	0	12	12	0	0	0	1	17	18
	40 : 49	0	0	0	0	0	0	0	0	0	0	6	6	0	0	0	0	6	6
	50 : 59	0	0	0	1	0	1	1	3	4	1	4	5	0	0	0	3	7	10
	60 : 69	0	0	0	0	0	0	0	2	2	0	0	0	0	0	0	0	2	2
	70 +	0	0	0	0	0	0	0	1	1	0	0	0	0	0	0	0	1	1
	Total	**8**	**0**	**8**	**10**	**0**	**10**	**18**	**27**	**45**	**1**	**38**	**39**	**0**	**1**	**1**	**37**	**66**	**103**

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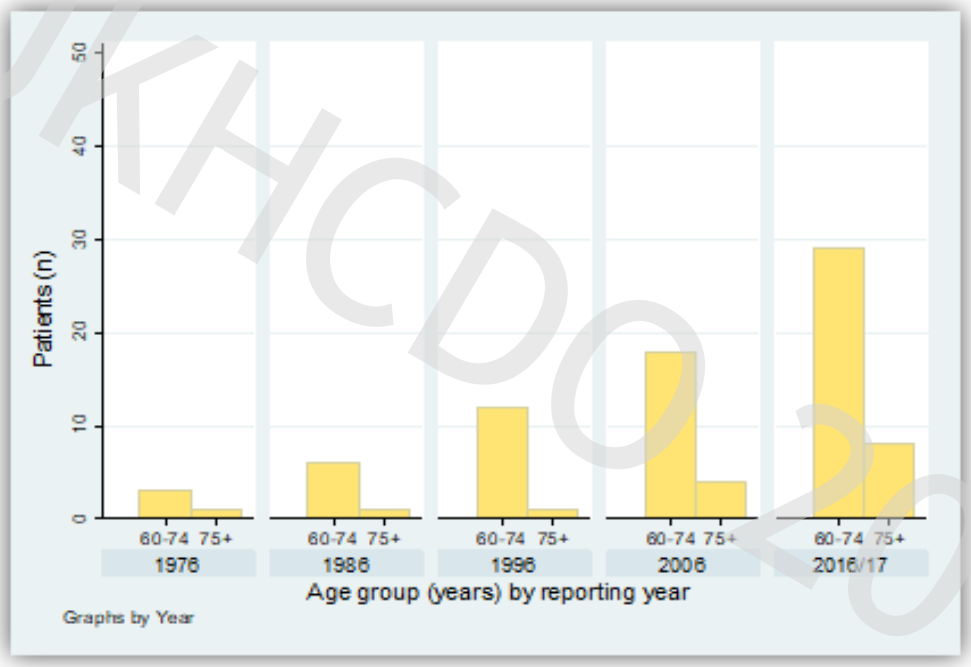
Figure 15a Trend in numbers of Severe Haemophilia B patients aged 60 years and above, 1976 - 2016/17 (including HIV +ve pts)



N.B. Carriers are included in Figures 15a - 15c

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Figure 15b Trend in numbers of Moderate Haemophilia B patients aged 60 years and above, 1976 - 2016/17 (including HIV +ve pts)

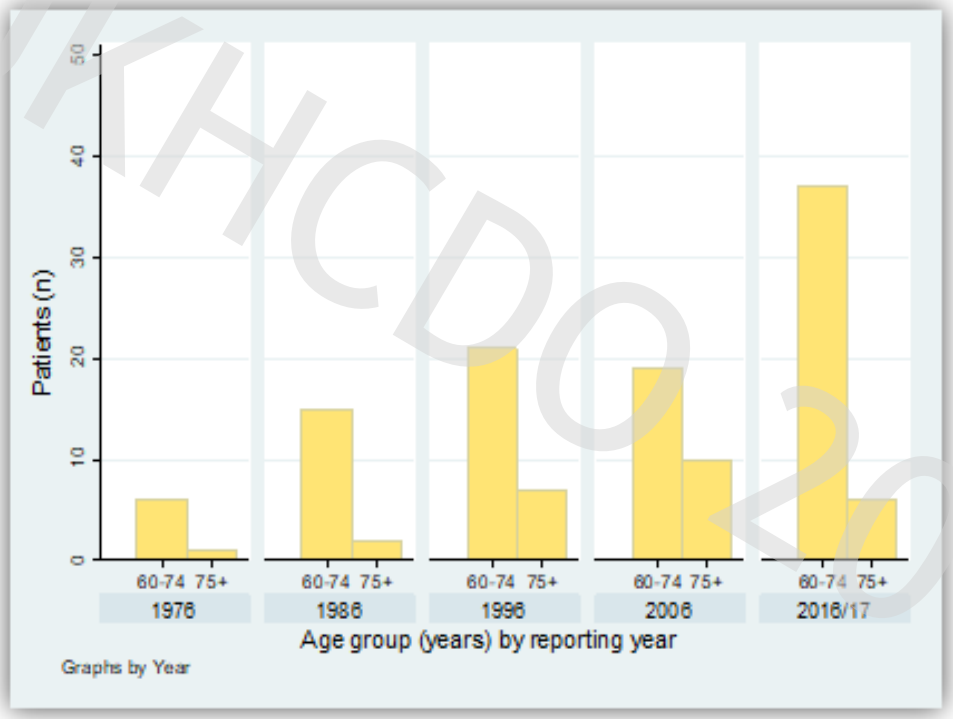

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Figure 15c Trend in numbers of Mild Haemophilia B patients aged 60 years and above, 1976 - 2016/17 (including HIV +ve pts)

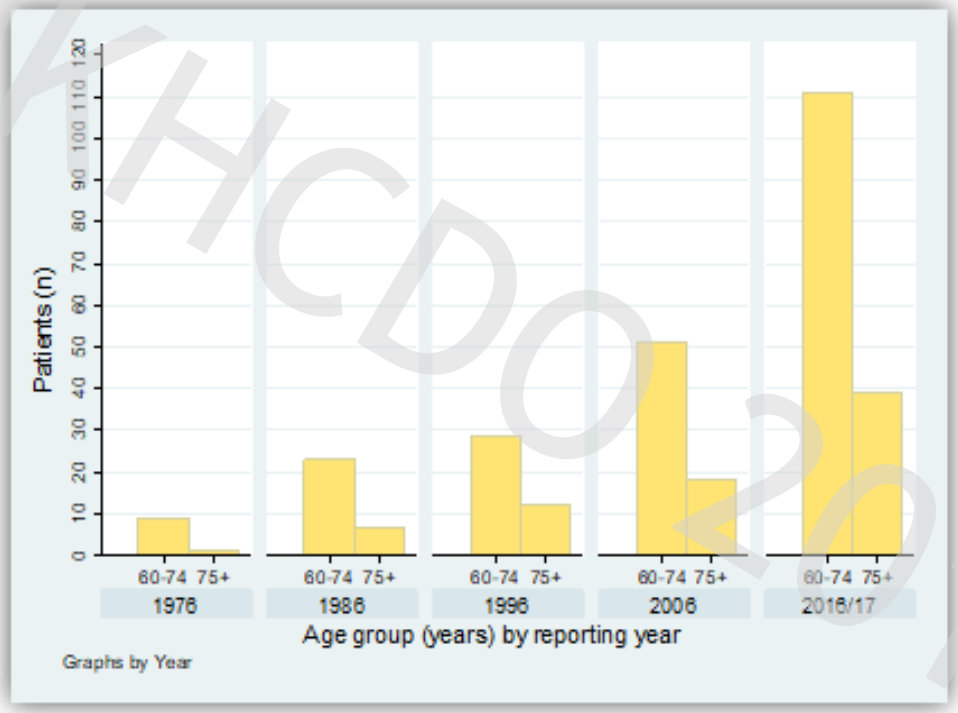

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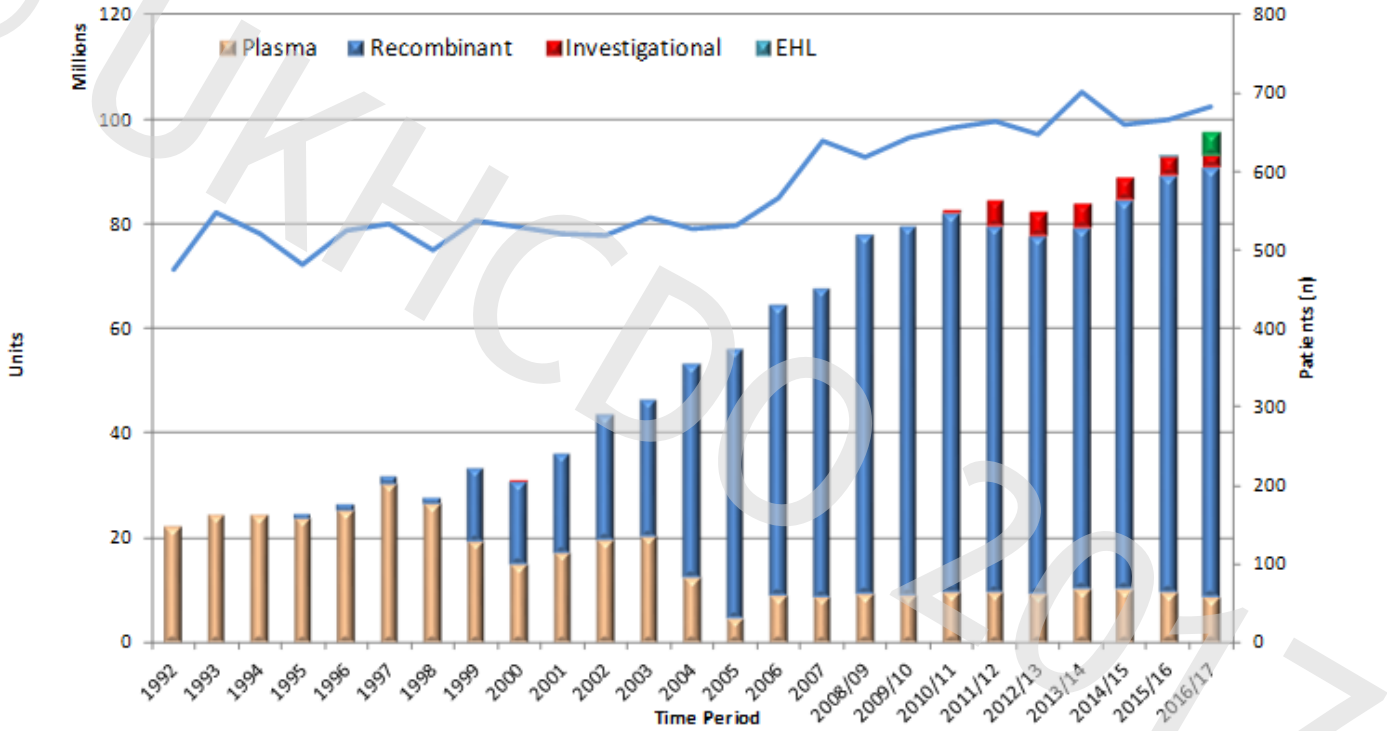
Figure 16 Factor IX units used by UK Haemophilia Centres 1992 - 2016/17 all diagnoses



N.B: Data for St Thomas' were not submitted 1996-2006

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Table 11 Factor IX usage by region for patients with Severe Haemophilia B (incl. treatment for inhibitors)

Region	General Population	Patients treated (n) Severe Haemophilia B	Total FIX Units (IU) Severe Haemophilia B	Mean Usage Severe Haemophilia B	FIX Units Per Capita Severe Haemophilia B
East Midlands	4,724,437	29	5,929,000	204,448	1.25
East of England	6,130,542	37	6,699,183	181,059	1.09
London	8,787,892	56	14,055,078	250,984	1.60
North East	2,636,848	11	2,483,500	225,773	0.94
North West	7,219,623	27	5,809,500	215,167	0.80
South East	9,026,297	55	10,850,018	197,273	1.20
South West	5,515,953	14	3,534,500	252,464	0.64
West Midlands	5,800,734	27	5,473,680	202,729	0.94
Yorkshire and the Humber	5,425,741	24	6,085,070	253,545	1.12
England	**45,760,739**	**280**	**60,919,529**	**217,570**	**1.33**
Northern Ireland	1,862,100	8	2,067,500	258,438	1.11
Scotland East	3,021,290	12	2,300,000	191,667	0.76
Scotland West	2,351,710	12	1,515,903	126,325	0.64
Wales	3,113,200	10	1,600,707	160,071	0.51
United Kingdom	**65,648,100**	**322**	**68,403,639**	**212,434**	**1.04**

Source accreditation:

Population Estimates for UK, England and Wales, Scotland and Northern Ireland: Mid-2016: Office for National Statistics licensed under the Open Government Licence v.3.0. © Crown copyright 2017

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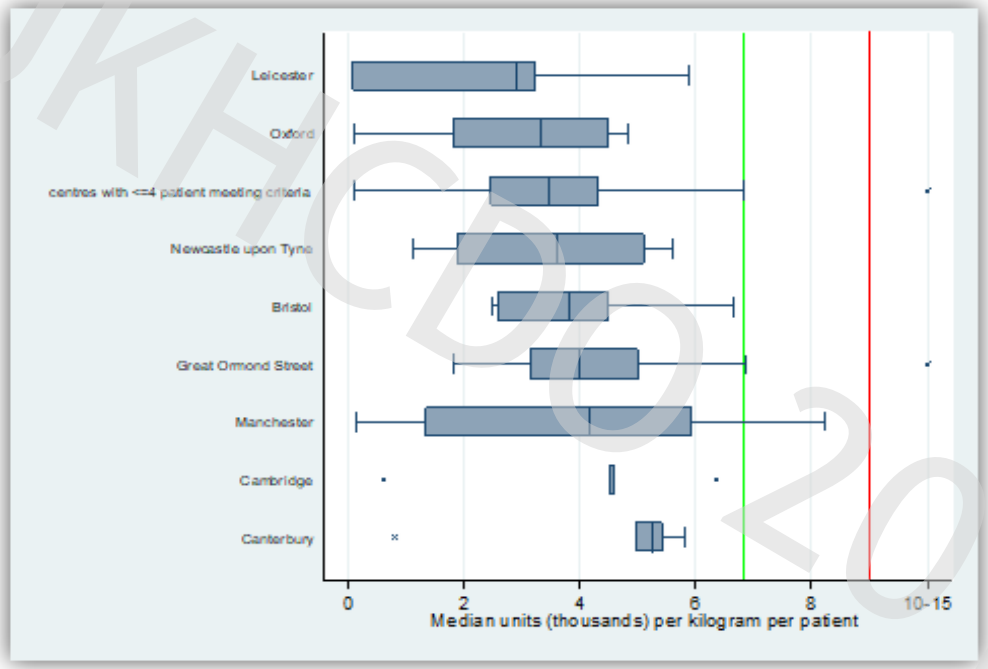
*Figure 17a Annual FIX usage **(IU/Kg/Pt)** 2016/17 in patients with Severe Haemophilia B **aged under 18 years** with no current inhibitor, by centre, ranked by median usage*



N.B: The 95th percentile of units = 6,843 IU per kilogram

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Data table for Figures 17a & b

Haemophilia Centre	Patients treated	Patients treated with weight reported	Total Units	Median Units	Median Units/Kg
Bristol	5	5	580,000	88,500	3,815
Cambridge	7	5	835,500	113,000	4,519
Canterbury	5	5	986,000	203,000	5,256
Great Ormond Street	21	21	2,911,092	120,000	3,995
Leicester	5	5	609,000	51,000	2,914
Manchester	11	11	1,675,500	154,000	4,170
Newcastle upon Tyne	5	5	509,500	105,000	3,602
Oxford	8	8	1,164,500	123,750	3,323
Centres with <=4 patients meeting criteria	39	34	5,177,500	102,000	3,480

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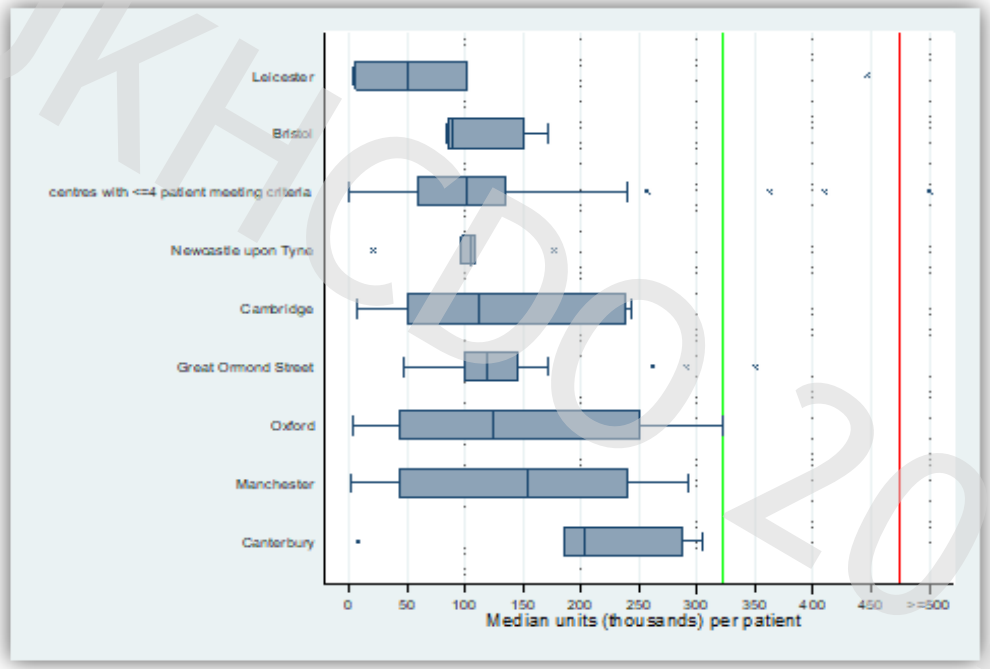
*Figure 17b Annual FIX usage **(IU/Pt)** 2016/17 in Severe Haemophilia B patients **aged under 18 years** with no current inhibitor, by centre, ranked by median usage*



N.B: The 95th percentile of units = 323,000 IU

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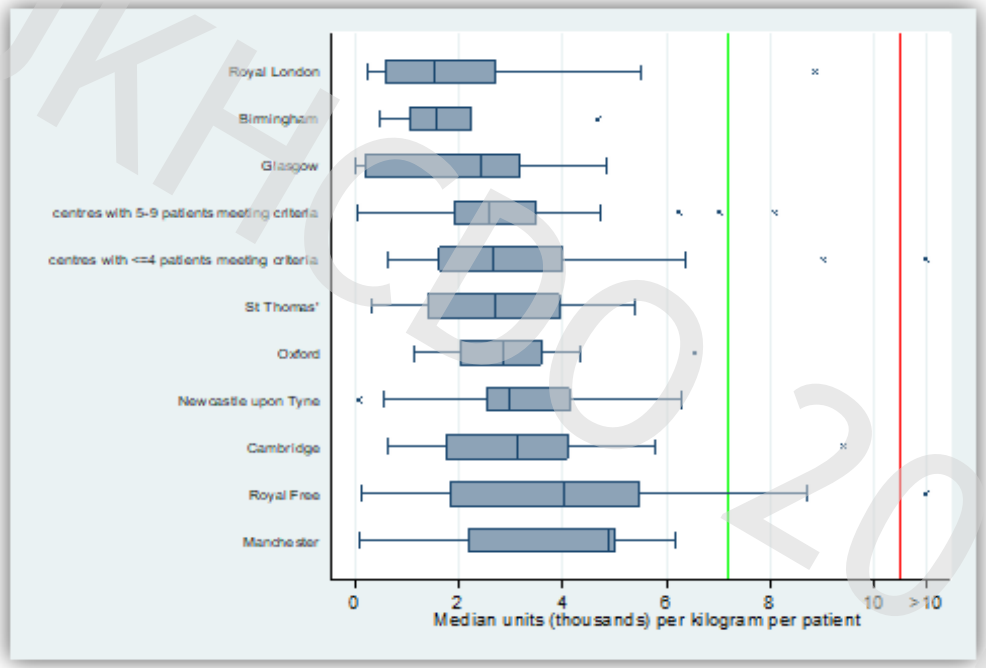
*Figure 18a Annual FIX usage **(IU/Kg/Pt)** 2016/17 in Severe Haemophilia B patients **aged 18 years or more** with no current inhibitor, by centre, ranked by median usage*



N.B: The 95th percentile of units = 7,204 IU per kilogram

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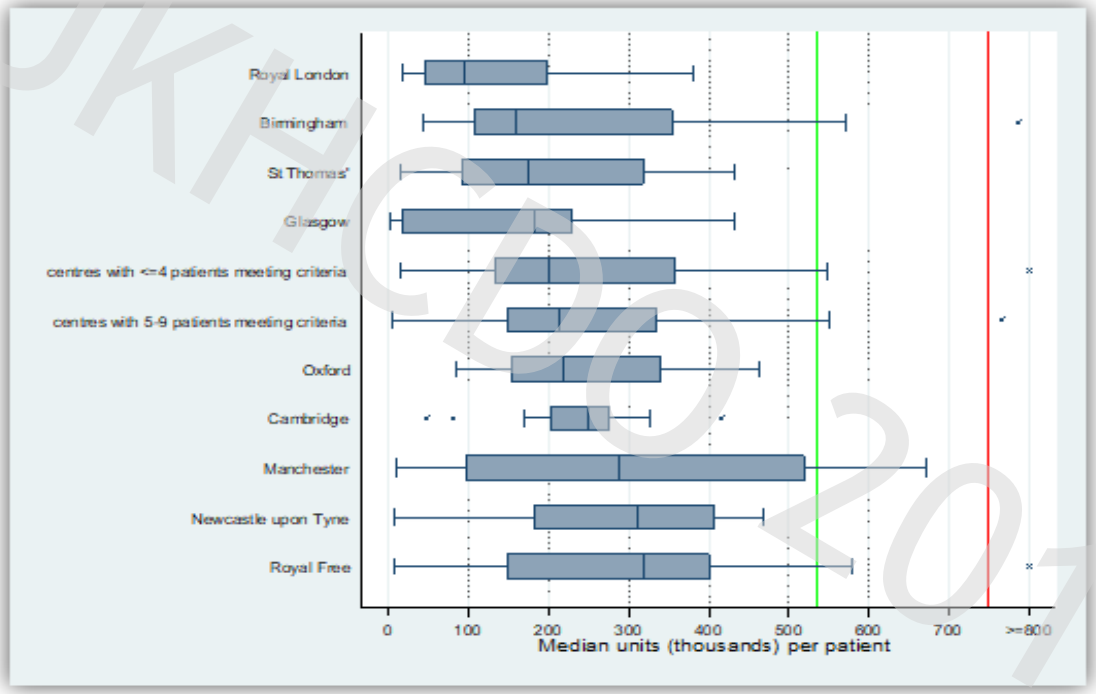
*Figure 18b Annual FIX usage **(IU/Pt)** 2016/17 in Severe Haemophilia B patients **aged 18 years or more** with no current inhibitor, by centre, ranked by median usage*



N.B: The 95th percentile of units = 535,000 IU

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Data table for Figures 18a & b

Haemophilia Centre	Patients treated	Patients treated with weight reported	Total Units	Median Units	Median Units/Kg
Birmingham	10	5	2,554,000	159,000	1,586
Cambridge	13	12	3,084,000	249,000	3,149
Glasgow	11	11	1,798,750	182,750	2,433
Manchester	12	9	3,742,500	288,000	4,872
Newcastle upon Tyne	12	12	3,390,500	312,000	2,992
Oxford	15	13	3,751,930	220,000	2,859
Royal Free	33	33	10,016,000	318,000	4,041
Royal London	14	10	1,940,090	94,950	1,550
St Thomas'	17	15	3,632,500	175,000	2,692
Centres with <=4 patients meeting criteria	39	28	9,998,585	200,000	2,665
Centres with 5-9 patients meeting criteria	41	32	10,157,192	214,200	2,572

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Table 12 Products used to treat Haemophilia B (including inhibitors) between April 2016 and March 2017

Manufacturer	Product	Total Units	
		2015/16	2016/17
Baxalta	FEIBA	2,204,000	1,652,000
	RIXUBIS	-	223,000
BPL	Replenine	2,686,610	2,548,450
CSL Behring	IDELVION	-	1,030,500
	Mononine	540,000	362,000
Grifols	Alphanine	6,338,600	5,876,000
Novo Nordisk	NovoSeven (mg)	8,295	10,391
Pfizer	BeneFIX	79,040,670	77,093,592
SOBI/Biogen	ALPROLIX	54,000	3,303,013
	Elocta	-	1,000
Various Manufacturers	Investigational Factor IX	4,361,406	2,420,676

Units in IU unless otherwise stated

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Table 13 Factor IX units issued by UK Haemophilia Centres between April 2016 and March 2017, broken down by diagnosis

Coagulation Defect	Patients Treated	Plasma FIX (IU)	Recombinant FIX (IU)	Investigational FIX (IU)	Enhanced Half-Life FIX (IU)	Total FIX (IU)
Haemophilia A*	2	-	4,000	-	-	4,000
Haemophilia B	676	8,786,450	77,316,592	2,420,676	4,333,513	92,857,231
von Willebrand disease	2	-	16,000	-	-	16,000
Co-inherited diagnoses	1	-	196,000	-	-	196,000
Unclassified bleeding disorder	1	-	1,500		-	1,500
Total	**682**	**8,786,450**	**77,534,092**	**2,420,676**	**4,333,513**	**93,074,731**

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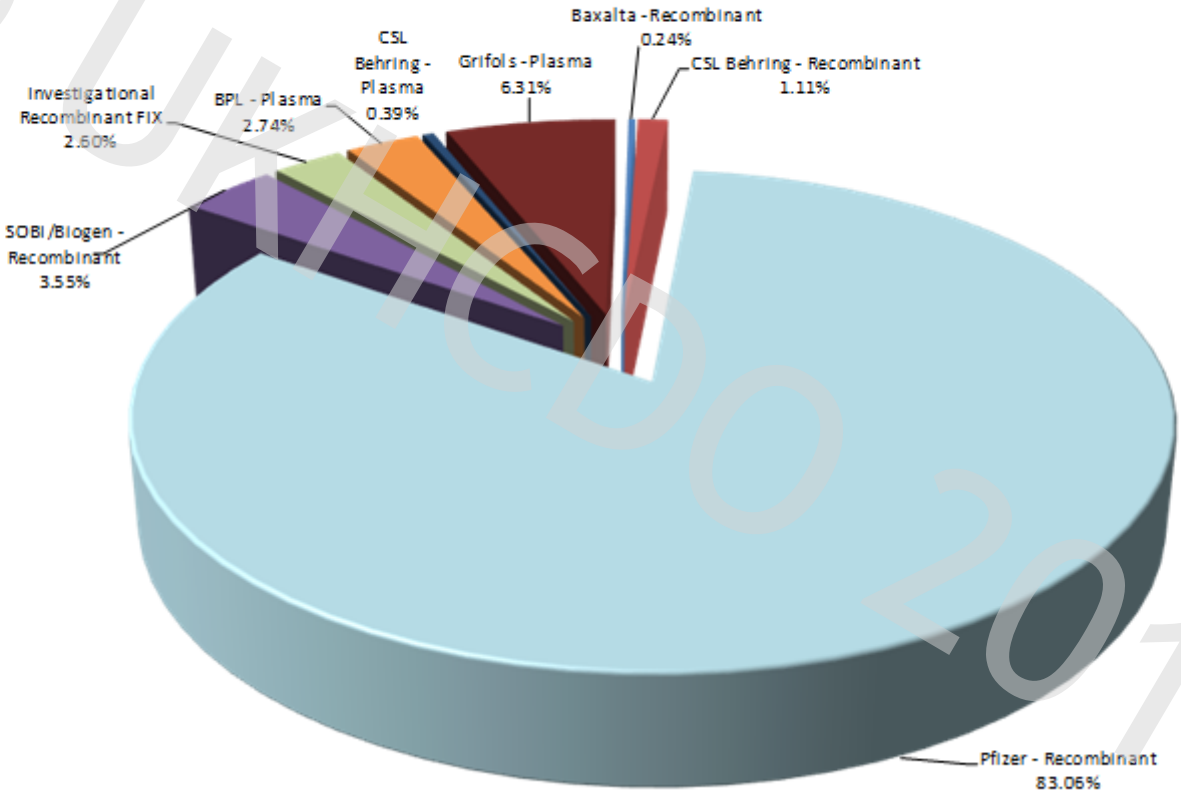
Figure 19 Market Share of factor IX concentrates issued by UK Haemophilia Centres during 2016/17



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Von Willebrand Disease

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Table 14 Patients with von Willebrand Disease registered and treated April 2016 - March 2017

von Willebrand disease	<18 years (VWD Activity IU/dl)					≥18 years (VWD Activity IU/dl)					Total	Treated with Concentrate	Treated with Desmpressin
	<10	10 - 29	≥30	N/K	Sub Total	<10	10 - 29	≥30	N/K	Sub Total			
Males													
Type 1	22	159	282	12	**475**	84	333	636	94	**1,147**	**1,622**	**68**	**35**
Type 2A	33	32	7	0	**72**	94	72	36	3	**205**	**277**	**62**	**6**
Type 2B	5	10	3	0	**18**	12	34	15	2	**63**	**81**	**17**	**1**
Type 2M	20	6	4	0	**30**	23	27	11	3	**64**	**94**	**14**	**1**
Type 2N	0	0	3	0	**3**	3	2	23	4	**32**	**35**	**5**	**2**
Type 2 Unspecified	4	6	4	0	**14**	8	13	8	1	**30**	**44**	**4**	**2**
Type 3	27				**27**	58				**58**	**85**	**58**	**1**
Type Unreported	60	137	267	7	**471**	167	340	613	58	**1,178**	**1,649**	**99**	**38**
										Sub Total Males	**3,887**	**327**	**86**
Females													
Type 1	27	103	218	3	**351**	123	548	1,714	196	**2,581**	**2,932**	**103**	**76**
Type 2A	27	27	10	0	**64**	112	99	67	6	**284**	**348**	**67**	**9**
Type 2B	6	7	9	0	**22**	14	43	39	1	**97**	**119**	**24**	**0**
Type 2M	8	18	9	0	**35**	48	61	19	6	**134**	**169**	**25**	**3**
Type 2N	0	1	2	0	**3**	5	7	52	4	**68**	**71**	**9**	**0**
Type 2 Unspecified	4	1	0	0	**5**	17	23	18	2	**60**	**65**	**6**	**4**
Type 3	18				**18**	51				**51**	**69**	**48**	**0**
Type Unreported	58	144	231	10	**443**	218	611	1,594	156	**2,579**	**3,022**	**155**	**117**
										Sub Total Females	**6,795**	**437**	**209**
										Grand Total - Males and Females	**10,682**	**764**	**295**

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Table 15 New Registrations of von Willebrand Disease between April 2016 & March 2017, by age at mid-year, severity, and gender

von Willebrand disease	<18 years (VWD Activity IU/dl)					≥18 years (VWD Activity IU/dl)					Total
	<10	10 - 29	≥30	N/K	Sub Total	<10	10 - 29	≥30	N/K	Sub Total	
Males											
Type 1	0	10	20	0	**30**	0	6	10	0	**16**	**46**
Type 2A	4	0	0	0	**4**	0	1	0	0	**1**	**5**
Type 2B	0	0	0	0	**0**	0	1	0	0	**1**	**1**
Type 2M	1	0	0	0	**1**	0	0	1	0	**1**	**2**
Type 2N	0	0	1	0	**1**	0	0	3	0	**3**	**4**
Type Unreported	9	27	46	0	**82**	6	12	17	1	**36**	**118**
										Sub Total Males	**176**
Females											
Type 1	2	7	12	0	**21**	2	22	39	0	**63**	**84**
Type 2A	1	5	0	0	**6**	1	3	3	0	**7**	**13**
Type 2B	2	1	0	0	**3**	0	0	1	0	**1**	**4**
Type 2M	0	1	2	0	**3**	0	2	0	0	**2**	**5**
Type 2N	0	0	1	0	**1**	0	0	2	0	**2**	**3**
Type 2 Unspecified	0	0	0	0	**0**	1	0	0	0	**1**	**1**
Type 3	1				**1**	0				**0**	**1**
Type Unreported	9	24	34	1	**68**	10	40	64	1	**115**	**183**
										Sub Total Females	**294**
										Grand Total - Males and Females	**470**

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Table 16 Concentrates used to treat von Willebrand Disease (including inhibitors) between April 2016 & March 2017

Manufacturer	Product	Total Units	
		2015/16	2016/17
Baxalta	Advate	14,500	35,000
Bayer	Kogenate	-	90,500
CSL Behring	Haemate P	7,625,150	4,387,950
	Helixate Nexgen	-	1,500
	Voncento	5,353,350	9,118,900
Grifols	Alphanate	780,000	873,000
	Fanhdi	4,000	8,000
LFB Biomedicaments	Willfact /Wilfactin	555,000	1,027,000
Novo Nordisk	NovoSeven (mg)	1,956	1,087
Octapharma	Wilate	5,710,000	5,417,904
Pfizer	Benefix	-	16,000
	ReFacto AF	29,000	5,500
	Desmopressin	-	14,043
	Investigational rVWF		*

**Anonymised for confidentiality purposes*
Units in IU unless otherwise stated
Products containing VWF and FVIII are reported in FVIII units

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Inhibitors: Congenital and Acquired

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Table 17 Inhibitors by Disease Severity - Haemophilia A, Haemophilia B & von Willebrand Disease

Coagulation Defect	Severity / Subtype	In Register *	Inhibitors				
			Newly Reported n (%)	Ongoing n (%)	Current n (%)	Historical n (%)	Ever n (%)
Haemophilia A	< 1 IU/dl	1,934	25 (1.3)	143 (7.4)	168 (8.7)	284 (14.7)	452 (23.4)
	1 - 5 IU/dl	837	3 (0.4)	28 (3.3)	31 (3.7)	51 (6.1)	82 (9.8)
	>5 - <40 IU/dl	3,707	2 (0.1)	16 (0.4)	18 (0.5)	40 (1.1)	58 (1.6)
	40+	1,355	0 (0.0)	0 (0.0)	0 (0.0)	0 (0.0)	0 (0.0)
	Unknown	60	0 (0.0)	0 (0.0)	0 (0.0)	0 (0.0)	0 (0.0)
	Total	**7,893**	**30 (0.4)**	**187 (2.4)**	**217 (2.7)**	**375 (4.8)**	**592 (7.5)**
Haemophilia B	< 1 IU/dl	361	0 (0.0)	12 (3.3)	12 (3.3)	5 (1.4)	17 (4.7)
	1 - 5 IU/dl	344	0 (0.0)	0 (0.0)	0 (0.0)	0 (0.0)	0 (0.0)
	>5 - <40 IU/dl	746	0 (0.0)	0 (0.0)	0 (0.0)	0 (0.0)	0 (0.0)
	40+	289	0 (0.0)	0 (0.0)	0 (0.0)	0 (0.0)	0 (0.0)
	Unknown	6	0 (0.0)	0 (0.0)	0 (0.0)	0 (0.0)	0 (0.0)
	Total	**1,746**	**0 (0.0)**	**12 (0.7)**	**12 (0.7)**	**5 (0.3)**	**17 (1.0)**
von Willebrand disease	Type 1	4,554	0 (0.0)	0 (0.0)	0 (0.0)	1 (0.0)	1 (0.0)
	Type 3	154	0 (0.0)	5 (3.2)	5 (3.2)	2 (1.3)	7 (4.5)
	Others	5,974	0 (0.0)	0 (0.0)	0 (0.0)	0 (0.0)	0 (0.0)
	Total	**10,682**	**0 (0.0)**	**5 (0.0)**	**5 (0.0)**	**3 (0.0)**	**8 (0.1)**

** Including patients not regularly treated*

New=newly reported in year
Ongoing=inhibitor in year but not new in year
Current=Sum of New + Ongoing
Historical=history of inhibitor, but not current
Ever = Historical + Current

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Table 18 Annual concentrate usage by patients with congenital bleeding disorders who were inhibitor positive at any time during 2016/17

Manufacturer	Product	Haemophilia A		Haemophilia B		von Willebrand Disease		F.VII deficiency		Co-inherited diagnoses	
		Units (IU)	Patients (n)	Units (IU)	Patients (n)	Units (IU)	Patients (n)	Units (IU)	Patients (n)	Units (IU)	Patients (n)
Baxalta	Advate	14,663,500	37	-	-	10,000	1	-	-	-	-
	FEIBA	35,104,000	89	1,652,000	4	-	-	-	-	1,216,000	3
Bayer	Kogenate	2,460,000	6	-	-	90,500	1	-	-	-	-
BPL	FVIII 8Y	221,510	1	-	-	-	-	-	-	-	-
	Optivate	4,174,425	4	-	-	-	-	-	-	-	-
CSL Behring	Haemate P	-	-	-	-	171,500	2	-	-	-	-
	Helixate Nexgen	1,646,000	7	-	-	-	-	-	-	-	-
	Voncento	-	-	-	-	256,000	2	-	-	-	-
Grifols	Fanhdi	6,778,000	16	-	-	-	-	-	-	-	-
Novo Nordisk	NovoSeven (mg)	19,251	83	10,391	10	1,087	3	980	1	-	-
Octapharma	Nuwiq	497,250	6	-	-	-	-	-	-	-	-
	Octanate	3,989,500	7	-	-	-	-	-	-	-	-
Pfizer	ReFacto AF	10,157,250	39	-	-	-	-	-	-	-	-
SOBI/Biogen	Elocta	1,080,500	4	-	-	-	-	-	-	-	-
	Desmopressin	252	5	-	-	-	-	-	-	450	1
Various Manufacturers	Investigational FVII	40	1	-	-	-	-	-	-	-	-
	Investigational FVIII mimetic	*	*	-	-	-	-	-	-	-	-
	Investigational FVIII	17,100	9	-	-	-	-	-	-	-	-

Units in IU unless otherwise stated

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Table 19 Concentrates used to treat Acquired Inhibitors

Manufacturer	Product	Acquired Haemophilia A	Acquired von Willebrands	Acquired Prothrombin Deficiency	Acquired Deficiency - Other
Baxalta	Advate	16,000	-	-	-
	FEIBA	7,088,000	-	4,000	-
	OBIZUR	48,000	-	-	-
Bayer	FXI	-	-	-	7,470
CSL Behring	Haemate P	-	254,000	-	-
	Riastap (g)	-	6	-	-
	Voncento	4,000	636,000	-	-
IDEC Pharmaceuticals	Rituximab	4,208	-	-	-
LFB Biomedicaments	Willfact /Wilfactin	-	306,000	-	-
Novo Nordisk	NovoSeven (mg)	4,155	6	-	-
Octapharma	Octaplex	1,500	-	-	6,500
	Wilate	-	1,409,000	-	-
Pfizer	ReFacto AF	98,500	-	-	-
	Desmopressin	30	122	-	-

Units in IU unless otherwise stated

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Table 20 FEIBA® usage: breakdown by diagnosis

Coagulation Defect	Number of Patients	Total (u)
Haemophilia A	89	35,104,000
Haemophilia B	4	1,652,000
Acquired Haemophilia A	89	7,088,000
Acquired Prothrombin Deficiency	1	4,000
Co-inherited diagnoses	3	1,216,000
	186	**45,064,000**

Table 21 NovoSeven® usage: breakdown by diagnosis

Coagulation Defect	Number of Patients	Total (mg)
Haemophilia A	87	19,666
Haemophilia B	10	10,391
von Willebrand disease	3	1,087
Acquired Haemophilia A	26	4,155
Acquired von Willebrand disease	1	6
F.V deficiency	1	6
F.VII deficiency	56	2,776
Co-inherited diagnoses	2	94
Bernard Soulier	13	394
Glanzmann's Thrombasthenia	44	3,229
Platelet defects	4	182
Unclassified bleeding disorder	3	22
	250	**42,008**

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Rarer Bleeding Disorders

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Table 22 Patients with rarer types of bleeding disorders registered and treated April 2016 & March 2017

Coagulation Defect	Number of Patients in Register			Treated with			% Treated
	Total	Males	Females	Any Product	Concen-trate	Desmo-pressin	
Probable von Willebrand disease	198	54	144	8	2	6	4.0
Platelet-type Pseudo von Willebrand Disease	27	13	14	2	2	0	7.4
F.V deficiency	205	81	124	4	1	0	2.0
F.VII deficiency	1,270	618	652	59	57	1	4.6
F.X deficiency	256	111	145	39	25	0	15.2
F.XI Deficiency	3,020	1,265	1,755	75	60	1	2.5
F.XIII Deficiency	69	39	30	55	55	0	79.7
Combined II+VII+IX+X Deficiency	3	1	2	0	0	0	0.0
Combined V+VIII Deficiency	28	13	15	10	8	3	35.7
Co-inherited diagnoses	300	143	157	30	19	11	10.0
Prothrombin Deficiency	14	5	9	5	4	0	35.7
Afibrinogenemia	14	8	6	11	10	0	78.6
Dysfibrinogenemia	490	190	300	20	20	0	4.1
Hypofibrinogenemia	134	56	78	8	7	1	6.0
Hypodysfibrinogenemia	28	13	15	3	3	0	10.7
Fibrinogen Deficiency	20	10	10	3	3	0	15.0
Acquired Haemophilia A	509	264	245	102	98	1	20.0
Acquired Haemophilia B	1	1	0	0	0	0	0.0
Acquired von Willebrands	130	72	58	41	39	3	31.5
Acquired Prothrombin Deficiency	6	4	2	1	1	0	16.7
Acquired F.XIII Deficiency	1	0	1	0	0	0	0.0
Acquired F.V Deficiency	4	0	4	1	0	0	25.0
Acquired Deficiency (other)	9	8	1	2	2	0	22.2
Glanzmann's Thrombasthenia	134	58	76	47	44	2	35.1
Bernard Soulier	87	43	44	15	13	1	17.2
Other platelet defects	2,426	752	1,674	112	5	87	4.6
Miscellaneous	258	68	190	13	6	6	5.0
Unclassified bleeding disorder	467	63	404	26	7	18	5.6
Haemophilia A with Liver Transplant	10	10	0	0	0	0	0.0
Haemophilia B with Liver Transplant	3	3	0	0	0	0	0.0
Total	10,121	3,966	6,155	692	491	141	6.8

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Table 23 Patients with selected rarer bleeding disorders registered and treated 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	46	2	159	2	0	0	205	4
F.VII deficiency	129	27	1140	32	1	0	1270	59
F.X deficiency	42	30	214	9	0	0	256	39
F.XI Deficiency	233	22	2785	53	2	0	3020	75
Total	**450**	**81**	**4,298**	**96**	**3**	**-**	**4,751**	**177**

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Table 24 New registrations of rarer bleeding disorders between April 2016 & March 2017 showing their coagulation defect and gender

Coagulation Defect	Male	Female	Total	↑ / ↓ from 2015/16
Haemophilia A with Liver Transplant	3	0	3	↓
Platelet-type Pseudo von Willebrand Disease	4	4	8	↑
Probable von Willebrand disease	6	14	20	↓
F.V deficiency	9	9	18	↑
F.VII deficiency	76	77	153	↑
F.X deficiency	12	10	22	↑
F.XI Deficiency	111	161	272	↑
F.XIII Deficiency	2	1	3	↑
Combined V+VIII Deficiency	1	2	3	N/A
Co-inherited diagnoses	12	11	23	↓
Afibrinogenemia	1	2	3	↓
Dysfibrinogenemia	30	44	74	↓
Hypofibrinogenemia	11	11	22	↓
Hypodysfibrinogenemia	13	15	28	N/A
Fibrinogen Deficiency	1	1	2	↑
Prothrombin Deficiency	1	0	1	↓
Acquired Haemophilia A	70	49	119	↑
Acquired F.V deficiency	0	1	1	↓
Acquired Prothrombin Deficiency	1	0	1	↓
Acquired von Willebrand disease	15	6	21	↓
Acquired Deficiency (other)	3	0	3	N/A
Glanzmann's Thrombasthenia	5	6	11	↑
Bernard Soulier	1	2	3	↓
Other platelet defects	79	200	279	↓
Miscellaneous	4	15	19	↓
Unclassified bleeding disorder	10	109	119	↑
Total	**481**	**750**	**1,231**	**↑**

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Table 25 Concentrates used to treat Rarer Bleeding Disorders between April 2016 & March 2017

Manufacturer	Product	F.V deficiency		F.VII deficiency		F.X Leyden		F.XI Deficiency		F.XIII Deficiency	
		Pts treated	Units	Pts treated	Units	Pts treated	Units	Pts treated	Units	Pts treated	Units
Baxter	FVII	-	-	2	104,200	-	-	-	-	-	-
BPL	COAGADEX	-	-	-	-	12	351,250	-	-	-	-
	FX	-	-	-	-	8	142,420	-	-	-	-
	FXI	-	-	-	-	-	-	59	242,367	-	-
CSL Behring	Beriplex	-	-	-	-	15	750,250	-	-	-	-
	Fibrogammin P	-	-	-	-	-	-	-	-	54	781,000
	FXIII	-	-	-	-	-	-	-	-	1	5,000
LFB Biomedicaments	Hemoleven	-	-	-	-	-	-	1	4,000	-	-
Novo Nordisk	NovoSeven (mg)	1	6	56	2,776	-	-	-	-	-	-
	NovoThirteen	-	-	-	-	-	-	-	-	1	32,500
Octapharma	Octaplas (units)	3	815	1	8	2	17,100	8	44	-	-
	Octaplex	-	-	-	-	17	934,900	-	-	-	-
Pfizer	Benefix	-	-	-	-	-	-	-	-	-	-
	ReFacto AF	-	-	1	90,000	-	-	-	-	-	-
	Desmopressin	-	-	1	25.5	-	-	1	15	-	-

Units in IU unless otherwise stated

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Adverse Events

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Table 26 Adverse Events reported between April 2016 & March 2017

Adverse Event	Number of Patients	Number of Events
Allergy Event	4	4
Infection Event	0	0
Inhibitor Event	31	31
Intracranial Haemorrhage	5	5
Malignancy Event	24	24
Other Event	2	2
Poor Efficacy Event	1	1
Thrombotic Event	8	9
Total	**75**	**76**

See table 17 for breakdown of inhibitors by disease severity in Haemophilia A, B and von Willebrand disease

N.B. one of the new inhibitors developed in a patient with a rarer bleeding disorder, so is excluded from Table 17.

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Morbidity and Mortality

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Table 27 Causes of death in patients with Haemophilia A, Haemophilia B between April 2016 & March 2017

Coagulation Defect	Cause of Death	Severity (factor level IU/dl)					Total
		< 1	1 - 5	>5 & <40	≥ 40	Unknown	
Haemophilia A	Carcinoma	2	1	0	1	1	5
	Cerebral haemorrhage	1	0	2	0	0	3
	COAD	0	0	1	0	0	1
	Haemorrhage (misc)	1	0	0	0	0	1
	Ischaemic Heart Disease	0	1	2	0	0	3
	Lymphoproliferative Malignancy	0	1	0	0	0	1
	Unknown	1	2	28	2	0	33
Haemophilia B	ARDS	0	1	0	0	0	1
	Carcinoma	2	1	0	0	0	3
	Hepatocellular Carcinoma	1	0	0	0	0	1
	Infection (Bacterial)	0	0	1	0	0	1
	Liver Failure	0	1	0	0	0	1
	Lymphoproliferative Malignancy	0	1	0	0	0	1
	Mixed drug toxicity	0	0	1	0	0	1
	Stroke	1	0	0	0	0	1
	Unknown	2	2	4	2	1	11
	Total	11	11	39	5	2	68

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Table 28 Causes of death in other coagulation defects between April 2016 & March 2017

Coagulation Defect	Cause of Death	Total
Haemophilia B with Liver Transplant	Leukaemia	1
F.V deficiency	Unknown	2
F.VII deficiency	ARDS	1
	Carcinoma	1
	Ischaemic Heart Disease	1
	Unknown	5
F.X deficiency	Unknown	2
F.XI Deficiency	Carcinoma	2
	Infection (Bacterial)	2
	Ischaemic Heart Disease	1
	Unknown	25
F.XIII Deficiency	Unknown	2
Combined V+VIII Deficiency	Unknown	1
Co-inherited diagnoses	Unknown	1
Acquired Haemophilia A	ARDS	3
	Carcinoma	4
	COAD	1
	Epilepsy	1
	Haemorrhage (misc)	3
	Infection (Bacterial)	14
	Ischaemic Heart Disease	1
	Senility/Alzheimer's disease	1
	Unknown	56
Acquired F.XIII Deficiency	Unknown	1
Acquired von Willebrand Disease	Unknown	12
Acquired Deficiency (other)	Carcinoma	1
	Unknown	1
Fibrinogen Deficiency	Unknown	1
Dysfibrinogenemia	Ischaemic Heart Disease	1
	Unknown	4
Hypofibrinogenemia	Unknown	2
Glanzmann's Thrombasthenia	Haemorrhage (Misc)	1
	Unknown	1
Platelet defects	Lymphoproliferative Malignancy	1
	Unknown	21
Miscellaneous	Unknown	1
Unclassified bleeding disorder	Unknown	2
von Willebrand disease	Carcinoma	3
	Cerebral haemorrhage	1
	Infection (Bacterial)	3
	Ischaemic Heart Disease	1
	Unknown	40
	Total	229

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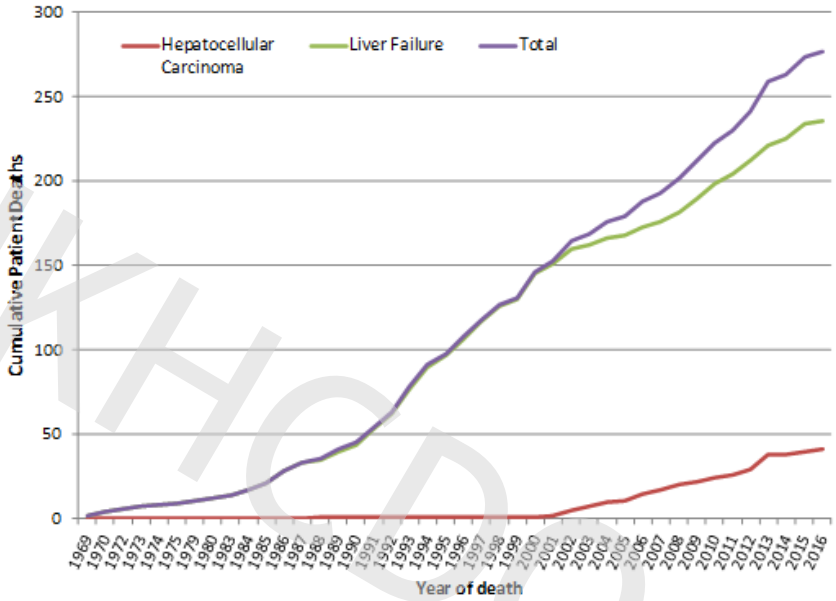
Figure 20 Cumulative incidence chart of deaths from hepatocellular carcinoma or liver failure in UK patients with bleeding disorders 1969 - 2016



Year	Hepatocellular Carcinoma	Liver Failure	Total
1969	0	2	2
1970	0	2	2
1972	0	2	2
1973	0	1	1
1974	0	1	1
1975	0	1	1
1979	0	2	2
1980	0	1	1
1983	0	2	2
1984	0	3	3
1985	0	4	4
1986	0	7	7
1987	0	5	5
1988	1	2	3
1989	0	5	5
1990	0	4	4
1991	0	9	9
1992	0	9	9
1993	0	15	15
1994	0	13	13
1995	0	7	7

Year	Hepatocellular Carcinoma	Liver Failure	Total
1996	0	10	10
1997	0	10	10
1998	0	9	9
1999	0	4	4
2000	0	15	15
2001	1	6	7
2002	3	9	12
2003	2	2	4
2004	3	4	7
2005	1	2	3
2006	4	5	9
2007	2	3	5
2008	3	6	9
2009	2	8	10
2010	2	9	11
2011	2	5	7
2012	3	8	11
2013	9	9	18
2014	0	4	4
2015	2	9	11
2016	1	2	3
Total	**41**	**236**	**277**

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Table 29 Summary of patients 'at risk' of vCJD for public health purposes who received UK sourced plasma products as reported by Centres

Summary table of 'at risk' bleeding disorder patients who received UK sourced plasma products				
		Implicated batches	Non-implicated batches	Combined
Current status of 'at risk' patients	Alive	684	2561	3245
	Dead	125	656	781
	Total	809	3217	4026
Genotype	MM	-	-	5
	MV	-	-	4
	VV	-	-	1
	Not known	809	3217	
Sex	M	769	2640	3409
	F	40	577	617
Current age band of living 'at risk' patients	0-19	0	13	13
	20-39	309	799	1108
	40-59	276	1052	1328
	60-79	94	584	678
	80+	5	111	116
	Not known	0	2	2

These data were last updated on 30/06/2017

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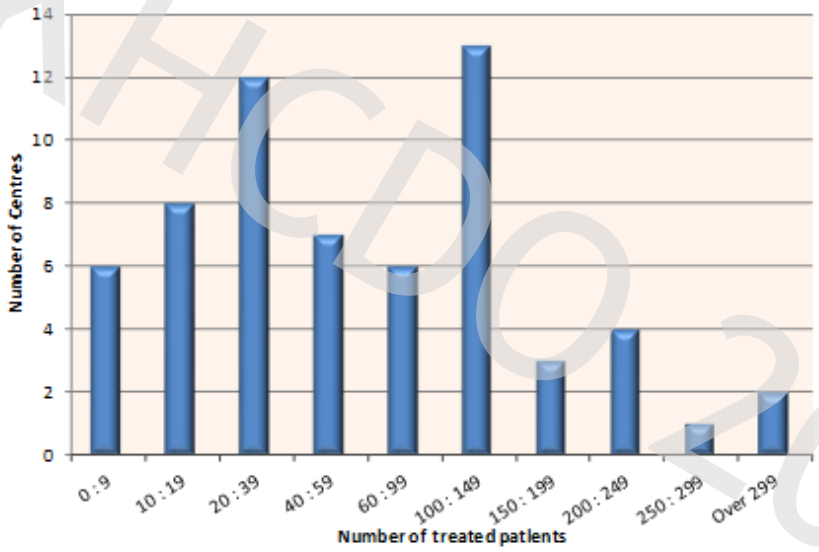
Figure 21 Total number of patients with Haemophilia A, Haemophilia B (including carriers) or von Willebrand Disease treated by UK Haemophilia Centres



N.B: Haemophilia A includes: Carrier of Haemophilia A and Females with FVIII deficiency
Haemophilia B includes: Carrier of Haemophilia B, Females with FIX deficiency, Patients with FIX Leyden and FIX Leyden carriers

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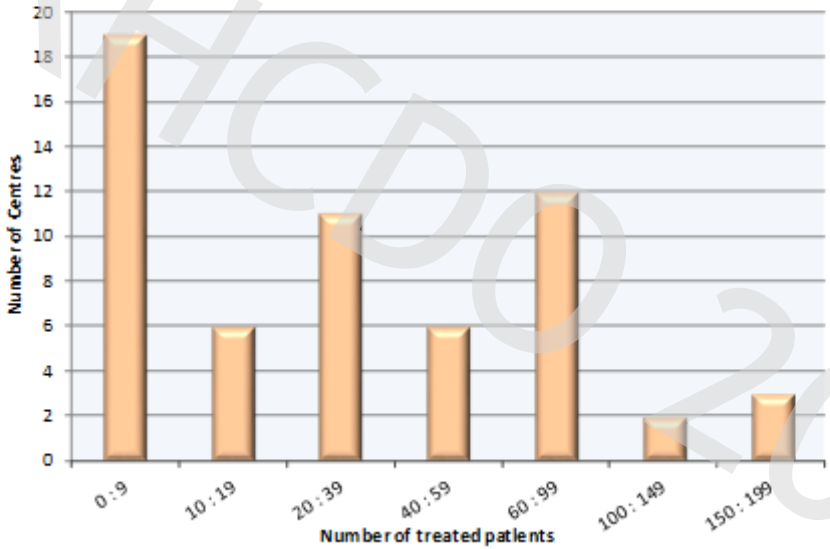
Figure 22 Total number of patients with severe Haemophilia A and Haemophilia B (including carriers) treated by UK Haemophilia Centres



N.B: Haemophilia A includes: Carrier of Haemophilia A and Females with FVIII deficiency
Haemophilia B includes: Carrier of Haemophilia B, Females with FIX deficiency, Patients with FIX Leyden and FIX Leyden carriers

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