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

ANNUAL REPORT

& Bleeding Disorder Statistics for the Financial Year 2024/25

2025

A report from the UKHCDO and NHD

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1.0 Chairperson's Report

Welcome to the 26th Annual General Meeting of the UKHCDO! The past year has been busy and eventful, marked by the completion of the 2024 peer review of Comprehensive Care Centres, the introduction of two novel therapies into clinical practice, and the successful implementation of gene therapy despite ongoing service pressures. Additionally, we are pleased to have finalised the first year of the action plan, transitioning from the UKHCDO Strategy finalisation to the implementation phase.

The 2024 peer review cycle has now concluded. The UKHCDO advisory committee has ratified Hull and Imperial as full Comprehensive Care Centres (CCC), while granting interim status to St George's Adult Service and Evelina paediatric service and congratulations to these centres. Furthermore, key criteria for CCC status have been agreed by the UKHCDO, independent of commissioning, based on peer-reviewed standards that reflect service capability and quality. We have actively engaged with the Clinical Reference Group about the revised national service specifications. The Peer Review Working Party is scheduled to develop updated peer review standards, guided by our thematic analysis and aligned with the new service specifications.

Despite the challenges, it has been an exciting time, marked by the introduction of two new therapies, efanesoctocog alfa and marsticimab, into routine clinical practice. Gene therapy pathways continue to be integrated into routine clinical care through commissioned hubs across the four nations, guided by national guidelines and the national hub panel to ensure consistency. A significant achievement this year was the ratification and implementation of the emicizumab policy for moderate haemophilia, years in development, and a testament to the collaborative efforts of the Clinical Reference Group, UKHCDO, and NHS England.

The current treatment landscape offers genuine choice, ushering in an era of personalised medicine for many. This also creates a responsibility on all of us to facilitate and promote equitable access. Where access is constrained by regulation or commissioning, UKHCDO will continue to work with all stakeholders to ensure timely and appropriate care for every PwBD.

Reflecting the Inquiry's findings, we are gradually integrating risk assessments that consider the potential consequences of both actions and inaction into our decision-making processes, while accounting for evidence gaps. Progress regarding the IBI psychological service has been slower than initially anticipated, and its availability remains inconsistent. We strongly encourage all stakeholders to assist in mobilising this service. We continue to actively engage with the government on Recommendation 9 of the Infected Blood Inquiry. Although discussions have been constructive, there has been no tangible progress on funding recombinant products for all PwBD or on developing the necessary infrastructure to meet the National Service Specifications. UKHCDO has provided a detailed analysis of the required infrastructure investment, which is currently under review. With regard to the Infected Blood Compensation Authority, we have submitted a comprehensive response, and several points have been incorporated into subsequent drafts; nonetheless, the level of engagement has been less than anticipated, and these efforts will be pursued further.

In June, we agreed on a Year 1 Action Plan comprising: a communication strategy—internal communications for members and a revised UKHCDO website; a pragmatic, NHS focused

leadership programme; scientific leadership for the National Haemophilia Database through creation of theme lead roles and a legal review with consolidated policies and clarified roles. NHD has now secured all necessary regulatory permissions to host the UK Bleeding Disorder Research Registry, and the consent process will be rolled out across centres over the coming year. Additionally, the deployment of the new NHD software alongside the appointment of theme leads will create time and analytical capacity, facilitating greater insights and expanding our observational research portfolio.

The Haemophilia and Bleeding Disorder Alliance, the inclusive PwBD and professional organisation, continues to meet to address current challenges. We acknowledge the publication of The Haemophilia Society's report on Women and Girls with Bleeding Disorders. We will make it a priority in the coming year, working closely with relevant working parties to ensure the meaningful implementation of its recommendations. Our sincere thanks to Dr Sarah Mangles, Chair of the Peer Review Working Party; Harry Evans, Project Manager; Debra Pollard, Advanced Nurse Practitioner; and Executive colleagues for drafting and reviewing the reports—and, above all, to the volunteers who made the site visits possible. We also thank the Clinical Reference Group and its lead, Dr Susie Shapiro, and our lead commissioners for raising the profile of haemophilia-related issues with NHS England. We are grateful to our working parties, the National Haemophilia Database, industry partners, PwBD societies, allied professional groups, and colleagues across the four nations for their continued support and collaboration.

Finally, on behalf of the Executive Committee and the wider organisation, I am pleased to formally express our deep gratitude to Dr Kate Talks for her steadfast support over the past six years as Vice-Chair. Her expertise, integrity, and balanced perspective have greatly enriched UKHCDO and all those privileged to collaborate with her. I am also delighted that Dr Gary Benson has stepped forward to serve as the new Vice-Chair, with the Treasurer to be appointed in due course.

Our mission remains clear: to promote equitable, comprehensive care for all people with bleeding disorders and to ensure that scientific advances are translated safely and promptly into benefits for every PwBD.

Prof Pratima Chowdary, Chair
Dr Kate Talks, Vice-Chair
Dr Mary Mathias, Secretary
Dr Gary Benson, Treasurer
November 2025

Bleeding Disorder Statistics for

April 2024 to March 2025

A report from the UK National Haemophilia Database

November 2025

Appendix 1 Glossary

AE	Adverse Event
AGM	Annual General Meeting
ASH	American Society of Hematology
BCSH	British Committee for Standards in Haematology
BMI	Body mass index
BMS	Biomedical Scientists
BSH	British Society for Haematology
CCC	Comprehensive Care Centre
CEO	Chief executive officer
CMWP	Co-morbidities Working Party
COVID-19	Corona Virus Disease
CPD	Continuing Professional Development
CQUIN	Commissioning for Quality and Innovation
CRG	Clinical Reference Group
DAG	Data Analysis Group
DMWP	Data Management Working Party
EAHAD	European Association for Haemophilia and Allied Disorders
EHL	Enhanced Half-life
EU	European Union
EUHASS	European Haemophilia Safety Surveillance
FEIBA	Factor eight inhibitor bypass agent
FIX	Factor nine
FVII	Factor seven
FVIII	Factor eight
GCP	Good clinical practice
GLH	Genomics Laboratory Hub
GLN	Genetic Laboratory Network
GOSH	Great Ormond Street Hospital
GWBD	Girls and women with bleeding disorders
GWP	Genetics Working Party
HA	Haemophilia A
HB	Haemophilia B

HC	Haemophilia Centre
HCC	Hepatocellular carcinoma
HCIS	Haemophilia Clinical Information System
HCPA	Haemophilia Chartered Physiotherapists' Association
HCV	Hepatitis C virus
HEE	Health Education England
HJHS	Haemophilia Joint Health Score
HNA	Haemophilia Nursing Association
HPA	Haemophilia Psychology Association
ICS	Integrated Clinical Academic
IPSG	International Prophylaxis Study Group
IQR	Interquartile range
ISTH	International Society on Thrombosis and Haemostasis
ITI	Immune tolerance induction
IU	International units
IU/dl	International units per decilitre
IU/kg	International units per kilogram
IWP	Inhibitor Working Party
JRCALC	Joint Royal Colleges Ambulance Liaison Committee
MAHA	Microangiopathic haemolytic anaemia
MDSAS	Medical Data Solutions and Services
MDT	Multidisciplinary team
MTP	Minimally treated people
NEQAS	National External Quality Assessment Service
NHD	National Haemophilia Database
NHF	National Hemophilia Foundation
NHS	National Health Service
NIBSC	National Institute for Biological Standards and Control
NIHR	National Institute for Health Research
PDF	Portable Document Format
pdFIX	Plasma-derived factor nine
pdFVIII	Plasma-derived factor eight
pdVWF	Plasma-derived von Willebrand factor
PPIE	Patient and Public Involvement and Engagement
PUP	Previously untreated person

PwAHA	Person/people with acquired haemophilia A
PwBD	Person/people with a bleeding disorder
PwHA	Person/people with congenital haemophilia A
PwHB	Person/people with congenital haemophilia B
PwMHA	Person/people with moderate haemophilia A
PwNSHA	Person/people with non-severe haemophilia A
PwNSHB	Person/people with non-severe haemophilia B
PWP	Paediatric Working Party
PwSH	People with severe haemophilia
PwSHA	Person/people with severe haemophilia A
PwSHAi	Person/people with severe haemophilia A and an inhibitor
PwSHB	Person/people with severe haemophilia B
PwVWD	Person/people with von Willebrand disease
RCEM	Royal College of Emergency Medicine
RCPCH	Royal College of Paediatrics and Child Health
RfPB	NIHR Research for Patient Benefit
rFIX	Recombinant factor IX
rFVIII	Recombinant factor VIII
rVWF	Recombinant von Willebrand factor
SAE	Serious Adverse Event
SD	Standard deviation
SHA	Severe Haemophilia A
SHB	Severe Haemophilia B
SHL	Standard Half-life
SOP	Standard operating procedure
TF	Task Force
THS	The Haemophilia Society
UK	United Kingdom
UKHCDO	United Kingdom Haemophilia Centre Doctors' Organisation
UKNEQAS	United Kingdom National External Quality Assessment Service
VWD	Von Willebrand disease
VWF	Von Willebrand factor
WAPPS-Hemo	Web-Accessible Population Pharmacokinetic Service — Hemophilia
WFH	World Federation of Hemophilia
WP	Working party

Appendix 2 Participating Centres

Centre Name	
Aberdeen	Lincoln
Bangor	Liverpool (Children's)
Barnstaple	Liverpool (R.I.)
Belfast - Adult's	Manchester (Children's)
Belfast - Children's	Manchester (R.I.)
Birmingham (Children's)	Newcastle
Birmingham (Queen Elizabeth's)	Newport
Bournemouth/Poole	North Hampshire
Bradford	Norwich
Brighton	Nottingham
Bristol (Infirmary & Children's)	Oxford
Cambridge	Peterborough
Canterbury	Plymouth
Cardiff	Portsmouth
Chichester	Royal Free, London
Coventry	Salisbury
Derby	Sheffield (Children's)
Dundee	Sheffield (Royal Hallamshire)
Edinburgh	Shrewsbury
Exeter	Southampton
Glasgow (R.H.S.C.)	St. George's Hospital, London
Glasgow (R.I.)	St. Thomas' and Guy's Hospitals, London
Great Ormond Street, London	Staffordshire (Stoke-on-Trent)
Hammersmith Hospital, London	Swansea
Inverness	Taunton/Yeovil
Ipswich	The Royal London Hospital
Kingston-upon-Hull	Torquay
Leeds	Truro
Leicester	Wolverhampton
Lewisham	York

2.0 Comments on the Bleeding Disorder Statistics for 2024/25

The statistics for the financial year 20224/5 follow. Most of the detailed explanation and commentary will be found with the tables and charts themselves.

Registrations:

Registrations of living people with bleeding disorders has increased markedly in the 23 years since the database was re-established in Manchester from about 16,000 individuals to over 42,000 (Table 1). Some of this increase is accounted for by a gradual increase in the number of people with haemophilia and von Willebrand's disease, although the largest contribution comes from increased registration of people with other bleeding disorders and mild bleeding disorders This increase is largely attributable to a decrease in management of mild disorders in peripheral hospitals and therefore increased registration and diagnosis of mild disorders by haemophilia centres.

Immigration from territories where consanguineous unions are common has significantly increased the number of registrants with severe recessive bleeding disorders such as type 3 von Willebrand's disease, which has increased in prevalence by almost 24% in the last 10 years. During the same period, there has been a 32% increase in Glanzmann's thrombasthenia registrations and a 43% increase in registrations of Bernard Soulier Syndrome and a 27% increase in people treated for severe factor XIII deficiency associated with a 30% increase in factor XIII units used. These trends have significant resource implications.

Treatment Trends:

The transfer of people with severe haemophilia A to emicizumab appears to have plateaued with only a few percentage points year on year increase. Of the 2276 PwSHA issued FVIII or emicizumab, 63.6% received emicizumab in the last year. About 80 people with non-severe haemophilia were using emicizumab. This report, finishing in March 2025, does not include data on the recent commissioning of emicizumab for non-severe haemophilia or Aluvoct (efenasoctocog alfa) for severe haemophilia. As a separate exercise, we investigated the dose-frequency and dosing of emicizumab. Fortnightly dosing was overwhelmingly the commonest dosing schedule used, in both adults and children, amongst those for whom we had a current bodyweight and compliant Haemtrack record. Dosing was very close to the 1.5mg/kg/week with little evidence of product wastage. There continues to be variation between centres in the proportion of PwSHA treated with emicizumab. Clinical opinion is clearly diverse, since some centres still treat most of their PwSHA with factor VIII prophylaxis, most commonly using an extended half-life product; 12.2% of PwSHA isused SHL rFVIII and 22.0% issued EHL rFVIII. Standard half-life factor VIII products are now mainly used for surgery and intercurrent bleeding.

The trend for PwSHB to switch to extended half-life factor IX product continues, such that only about 12% of factor IX prophylaxis is conducted using standard half-life factor IX. SHL FIX is still used extensively for intercurrent bleeding, surgery and for non-severe Haemophilia B (Figures 12-16).

The last ten years has witnessed a near-fourfold increase in the number of VW units issued to treat von Willebrand's disease. This is mirrored to some extent by an increase in the number of PwVWD issued VWF (Figure 22) but does not reflect an increase in the number of people with von Willebrand's disease registered, which has increased relatively modestly during this period. This increase is presumably attributable to increasing numbers of people with von Willebrand's disease undergoing surgery and increased use of prophylaxis amongst people with type 3 and type 2 von Willebrand's disease. During the last year there has been no significant change in the types of

concentrate used, the use of recombinant von Willebrand factor being restricted by its limited product license.

Clinical Outcomes:

Within-person analysis of Haemophilia joint Health Score (HJHS), shows that younger PwSH have and maintain very low joint scores (Tables 12 and 18). Older PwSHA/B have a higher joint score reflecting their past history of regular haemarthroses, but there has been modest improvement over the past ten years in these age groups, perhaps reflecting improved bleed outcomes (Figures 11 and 19). These results should be interpreted with caution, however, since they are based on relatively small numbers. Few centres reported HJHS at the start of the observation period, limiting the number of paired observations available for within-person analysis.

HJHS data tends to come from those centres who record the data electronically into HCIS using a laptop and is then centrally available for analysis. Where this data is recorded on paper, transcription is a labour-intensive task that in practice usually means that the data is not transmitted to the NHD. Increasingly, joint scoring data is entered directly into an electronic PwBD record rather than HCIS, which has led to a decline in reporting of joint scoring data to NHD. This is very important outcome data for us and so we are currently investigating various ways to make it easier for centres to send us this data.

Survival curves show that survival of people with severe and moderate haemophilia A has improved significantly. Causes of death reported by centres and NHS Digital indicate an increasing proportion dying from dementia and the diseases of old age, largely reflecting causes of death in the general population. More than half of our mortality and cause of death data comes from death certification data from NHS Digital rather than from centre reports.

Adverse events reports, assessed monthly by the Adverse Event Panel show the usual range of adverse events. All-drug-related adverse events are reported back to the manufacturer so that they can satisfy their statutory duty to report adverse events to the regulators. Pharmacovigilance is an increasingly important part of the work of the database. The report shows the mean number of adverse event reports per 100 PwBD per year for six anonymised centres selected to show the range of report frequency. Some centres report far more adverse events per PwBD than others. After the AGM, we will report to each centre their reporting statistics with the group median statistics by way of comparison.

The tables have not been “minimised” for small numbers. The recommendations of the Royal Statistical Society on data minimisation follow the common sense principle that if an individual can be identified from the data presented then the data should be minimised. Following these principles on a case by case basis, we concluded that individuals could not be identified from tables as currently presented and, accordingly, the data does not require minimisation.

I would like to take this opportunity to thank the staff of the haemophilia centres for collating and sending in all the data and for helping us with data-cleaning,

We hope that you find the statistics useful and of interest and if you have any suggestions for future issues, please let us know. We hold a report review meeting usually in January, attended by a group of stakeholders including commissioners, PwBD representatives and working party chairs.

Professor Charles RM Hay,

James Chetter, Dennis Glover, Ben Palmer & Dr Hua Xiang

On behalf of the UK National Haemophilia Database

Manchester, November 2025

Table 1 People with bleeding disorders by registered diagnosis, 2024/25

Diagnosis	Number of people registered, by sex as registered at birth		
	Male	Female	Total
Haemophilia A	6,898	0	6,898
Female with <40 IU/dl FVIII	0	943	943
Female with >=40 IU/dl FVIII	0	2,066	2,066
Haemophilia B	1,372	0	1,372
Female with <40 IU/dl FIX	0	376	376
Female with >=40 IU/dl FIX	0	423	423
Factor IX Leyden deficiency	31	0	31
Factor IX Leyden deficiency carrier	0	19	19
Haemophilia A/B with liver transplant	12	0	12
von Willebrand disease	4,487	7,902	12,389
Probable von Willebrand disease	80	193	273
Factor V deficiency	111	221	332
Factor VII deficiency	1,193	1,416	2,609
Factor X deficiency	166	230	396
Factor XI deficiency	1,850	2,769	4,619
Factor XIII deficiency	60	51	111
Prothrombin (factor II) deficiency	10	15	25
Dysfibrinogenemia	360	578	938
Hypofibrinogenemia	146	208	354
Hypodysfibrinogenemia	28	30	58
Afibrinogenemia	11	7	18
Bleeding disorder of unknown cause	226	1,655	1,881
Glanzmann's thrombasthenia	74	98	172
Bernard-Soulier syndrome	64	75	139
Platelet-type pseudo von Willebrand disease	16	30	46
Other platelet disorder	1,330	2,909	4,239
Combined factors V and VIII deficiencies	16	20	36
Other disorders**	4	7	11
Acquired haemophilia A	375	356	731
Acquired von Willebrand disease	126	101	227
Other acquired factor deficiency	28	26	54
Miscellaneous/multiple disorders	285	608	893
Total	**19,359**	**23,332**	**42,691**

**Other reported disorders include:

Thrombomodulin-associated coagulopathy

Combined factors II, VII, IX and X deficiencies

Alpha 2-antiplasmin deficiency

Reduced one stage FVIII and normal chromogenic FVIII

Table 1 (previous page): This shows the number of people registered with the NHD by diagnosis. Females registered as HA or HB carriers, or with FVIII or FIX deficiency, are reported by their factor level. Otherwise, people are listed by their registered diagnosis. People registered with multiple disorders are grouped together with people whose diagnosis is recorded as 'miscellaneous'. It is suspected that liver transplantation is under-reported. Haemophilia B Leyden is also under-reported, as it is sometimes reported as haemophilia B.

Important Note: Throughout the remainder of this report, haemophilia A results include carriers of HA and females with FVIII deficiency. Results for haemophilia B include carriers of HB, females with FIX deficiency, people with FIX Leyden and FIX Leyden carriers. People with multiple disorders are included in results for each of their individual blood disorder diagnoses. Where female or male sex is specified, this refers to sex as registered at birth. Age is calculated at the midpoint of the financial year, i.e. 30th September 2024.

Table 2 People with bleeding disorders by country of residence, 2024/25

Diagnosis	Number of people registered					
	England	Northern Ireland	Scotland	Wales	Not known	Total
Haemophilia A or carrier	8,299	376	861	437	26	9,999
Haemophilia B or carrier	1,844	44	230	108	11	2,237
von Willebrand disease, including probable VWD	10,702	166	1,348	553	56	12,825
Other bleeding disorders	14,956	135	1,857	777	95	17,820
Total	**35,627**	**721**	**4,287**	**1,869**	**187**	**42,691**

2.1 Congenital haemophilia A

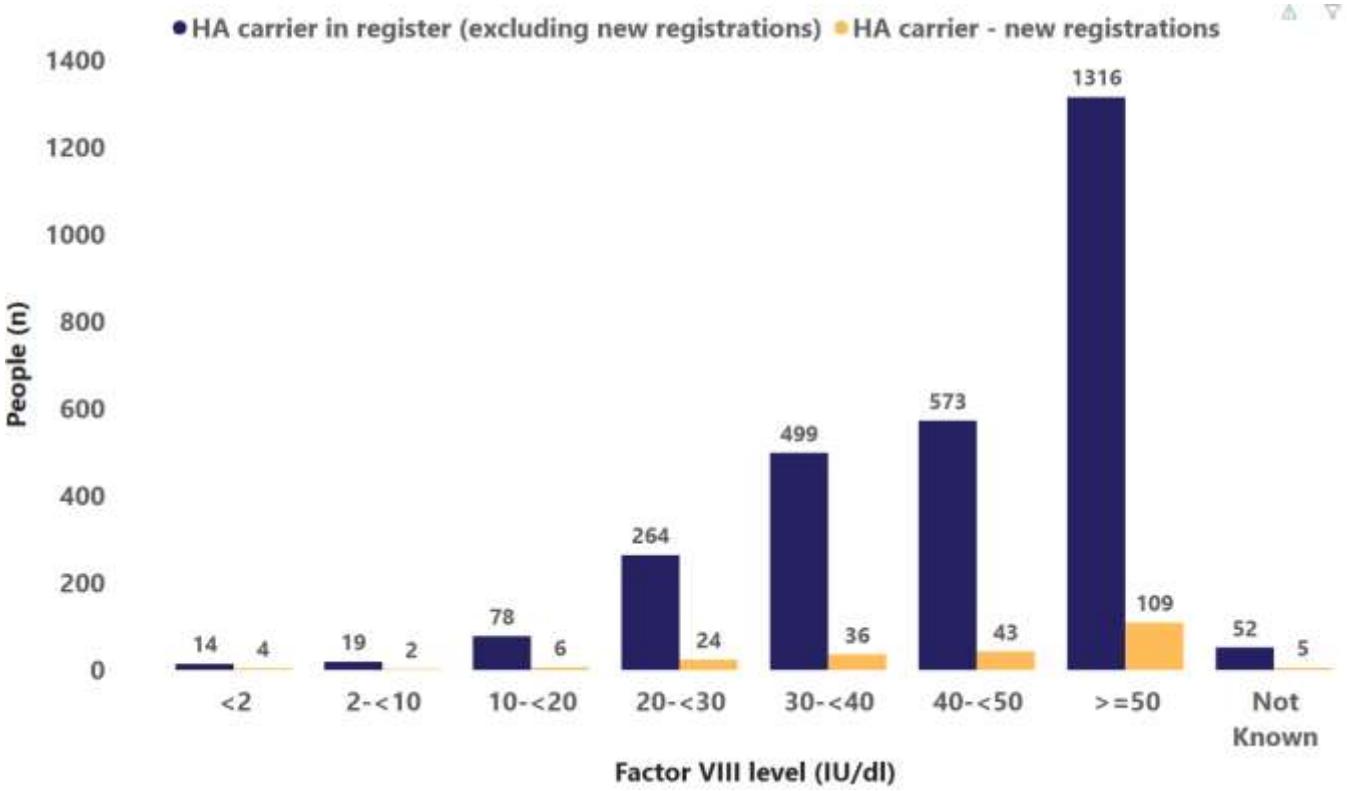
Table 3 People with congenital haemophilia A and carriers, registered and treated, 2024/25

Congenital haemophilia A	Age (years)	Number of people by factor VIII level (IU/dl)																	
		< 1			1 - 5			>5 & <40			≥ 40			People with unknown severity or liver transplant			Total		
		Male	Female	Total	Male	Female	Total	Male	Female	Total	Male	Female	Total	Male	Female	Total	Male	Female	Total
Total Registered	<18	744	7	751	187	3	190	676	155	831	33	218	251	6	3	9	1,646	386	2,032
	>=18	1,642	11	1,653	602	8	610	2,802	762	3,564	223	1,823	2,046	40	54	94	5,309	2,658	7,967
	Total	**2,386**	**18**	**2,404**	**789**	**11**	**800**	**3,478**	**917**	**4,395**	**256**	**2,041**	**2,297**	**46**	**57**	**103**	**6,955**	**3,044**	**9,999**
New Registrations**	<18	43	4	47	17	-	17	57	29	86	6	47	53	-	1	1	123	81	204
	>=18	11	-	11	8	1	9	56	38	94	9	105	114	3	4	7	87	148	235
	Total	**54**	**4**	**58**	**25**	**1**	**26**	**113**	**67**	**180**	**15**	**152**	**167**	**3**	**5**	**8**	**210**	**229**	**439**
Issued concentrate FVIII and/or Emicizumab in year***	<18	727	4	731	140	2	142	172	12	184	7	1	8	3	-	**3**	1,049	19	1,068
	>=18	1,541	4	1,545	364	4	368	581	58	639	18	16	34	11	-	**11**	2,515	82	2,597
	Total	**2,268**	**8**	**2,276**	**504**	**6**	**510**	**753**	**70**	**823**	**25**	**17**	**42**	**14**	**-**	**14**	**3,564**	**101**	**3,665**

Sex as registered at birth
***New registrations are a subset of the 'In Register' numbers*
****Excluding people only treated with DDAVP and tranexamic acid*

Table 3: This shows the total number of people with haemophilia A (including factor VIII carriers and factor VIII deficient females) registered and/or issued with FVIII concentrate or emicizumab in the UK during 2024/25, by age, sex at birth and disease severity.

Figure 1 Females with congenital FVIII deficiency and carriers of haemophilia A currently registered and newly registered, by baseline FVIII level, 2024/25



N.B: Includes carriers of haemophilia A and females with FVIII deficiency

Diagnosis	Number of people by factor VIII level (IU/dl)								
	<2	2-<10	10-<20	20-<30	30-<40	40-<50	≥50	Not Known	Total
Ongoing registrations (excluding new registrations)	14	19	78	264	499	573	1,316	52	2,815
New registrations	4	2	6	24	36	43	109	5	229
Total*	**18**	**21**	**84**	**288**	**535**	**616**	**1,425**	**57**	**3,044**

Sex as registered at birth

Figure 1: This shows the number of carriers of haemophilia A currently registered with the NHD by baseline FVIII level. This includes females registered by their centre as having FVIII deficiency or haemophilia A. New registrations of carriers with normal FVIII levels continues but is incomplete.

Table 4 New registrations of people with congenital haemophilia A and carriers, 2024/25

Congenital haemophilia A	Number of people by factor VIII level (IU/dl)																	
Age (years)	< 1			1 - 5			> 5 & < 40			≥ 40			People with unknown severity			Total		
	Male	Female	Total	Male	Female	Total	Male	Female	Total	Male	Female	Total	Male	Female	Total	Male	Female	Total
0 - 1	35	2	37	12	-	12	30	6	36	2	8	10	-	-	-	79	16	95
2 - 4	1	1	2	1	-	1	8	5	13	-	8	8	-	-	-	10	14	24
5 - 9	3	1	4	2	-	2	11	6	17	-	9	9	-	-	-	16	16	32
10 - 19	5	-	5	2	1	3	10	16	26	4	35	39	-	1	1	21	53	74
20 - 29	7	-	7	4	-	4	8	3	11	4	22	26	2	-	2	25	25	50
30 - 39	1	-	1	2	-	2	14	17	31	-	36	36	1	2	3	18	55	73
40 - 49	2	-	2	1	-	1	9	6	15	-	13	13	-	2	2	12	21	33
50 - 59	-	-	-	-	-	-	6	1	7	2	8	10	-	-	-	8	9	17
60 - 69	-	-	-	-	-	-	10	4	14	1	6	7	-	-	-	11	10	21
70 and above	-	-	-	1	-	1	7	3	10	2	7	9	-	-	-	10	10	20
Total*	**54**	**4**	**58**	**25**	**1**	**26**	**113**	**67**	**180**	**15**	**152**	**167**	**3**	**5**	**8**	**210**	**229**	**439**

Sex as registered at birth

Table 4: This shows the number of new registrations of haemophilia A broken down by reported severity and age at mid-year (30/09/2024). The underlying birth rate of people with severe haemophilia A born in the UK usually runs at 40-45 per year.

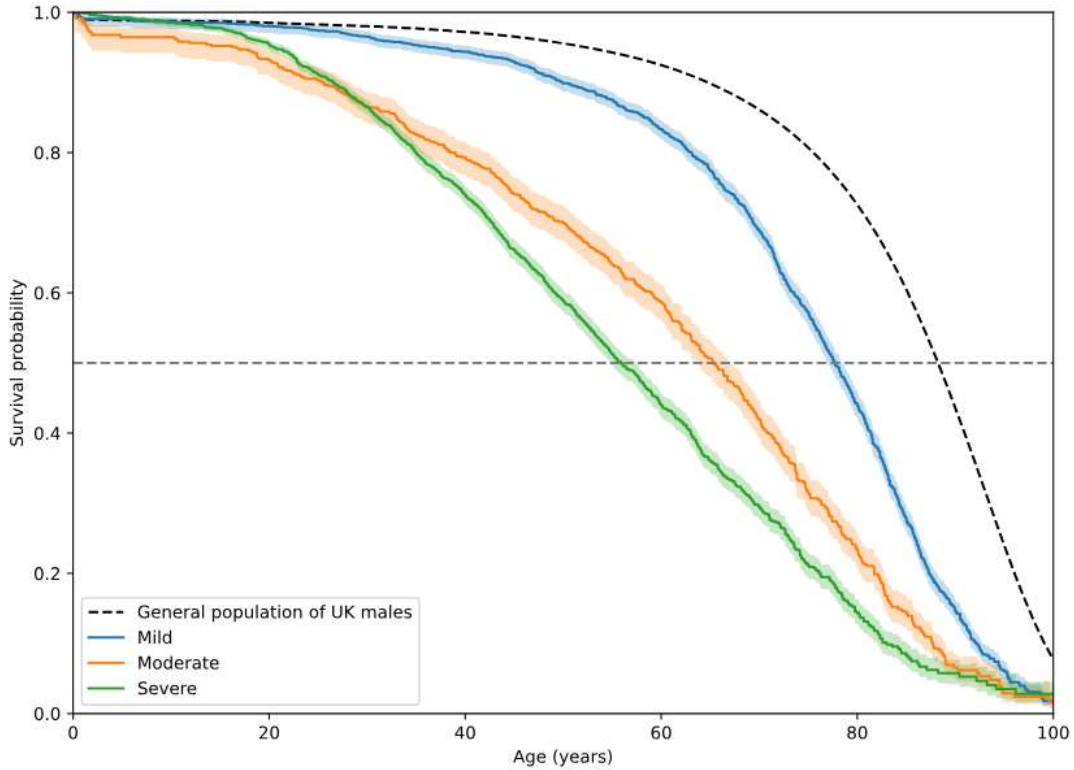
Table 5 New registrations of people with severe congenital haemophilia A aged 2 years and over, and subsequent treatment by year

	Registration year										Total
	2015/16	2016/17	2017/18	2018/19	2019/20	2020/21	2021/22	2022/23	2023/24	2024/25	2015/2025
New registrations this year	30	23	22	20	19	13	23	33	23	20	226
Issued product by year											
2015/16	23										23
2016/17	21	21									42
2017/18	18	21	18								57
2018/19	14	19	17	15							65
2019/20	13	18	16	17	18						82
2020/21	13	16	15	14	13	12					83
2021/22	14	17	13	10	13	11	20				98
2022/23	15	15	13	13	11	11	21	30			129
2023/24	13	15	14	13	11	9	21	30	23		149
2024/25	12	15	14	14	11	8	20	24	23	17	158
No issue records	4	1	2	1	0	1	1	2	0	3	15

Excludes people resident overseas.

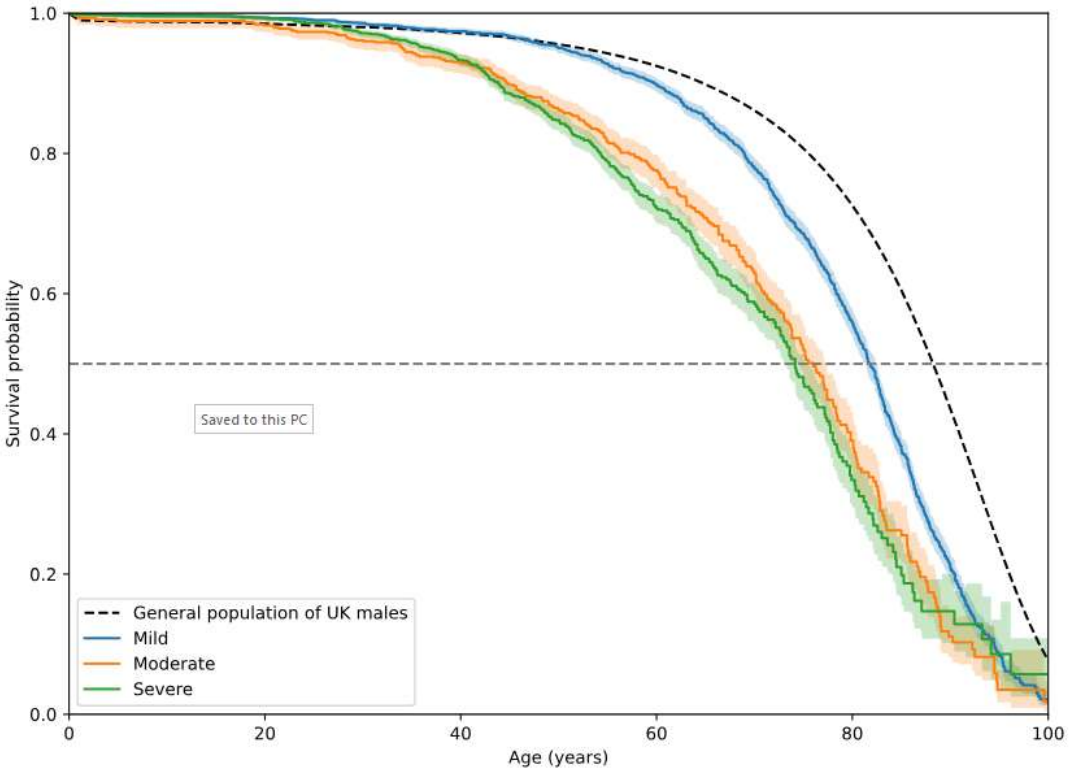
Table 5: This shows the number of people with severe haemophilia A over two years of age when newly registered (and therefore thought potentially to be migrants) each year from 2015/16, excluding those reported to be resident overseas. It also shows the number issued product in each year subsequent to their initial registration. This shows that up to 226 people with severe haemophilia A may have migrated to the UK since 2015/16. This may be an underestimate, since children under two years old are not included in this table. Product was issued to 158/226 of these PwSHA in 2024/25, suggesting that most remain and continue to require regular treatment.

Figure 2a Survival of people with congenital haemophilia A by severity, actively registered 1969-1995



Median survival time (years) for people with haemophilia A: severe: 55, moderate: 65, mild: 77

Figure 2b Survival of people with congenital haemophilia A by severity, actively registered 1996-2025



Median survival time (years) for people with haemophilia A: severe: 74, moderate: 75, mild: 81

Figures 2a-2b: These show survival curves for haemophilia A broken down by severity for the years 1969 to 1995 and 1996-present with 95% confidence intervals. The median life expectancy for people with severe haemophilia A of 55 years in 1969-95 is comparable with published data from that era from the United States. There has been significant improvement in median life expectancy of people with severe and moderate haemophilia A (74 and 75 years respectively), although it has still not completely corrected to levels seen in people with mild haemophilia A (median 81 years) and in the general population.

Figure 3 People with severe congenital haemophilia A by primary treatment, 2015/16 - 2024/25

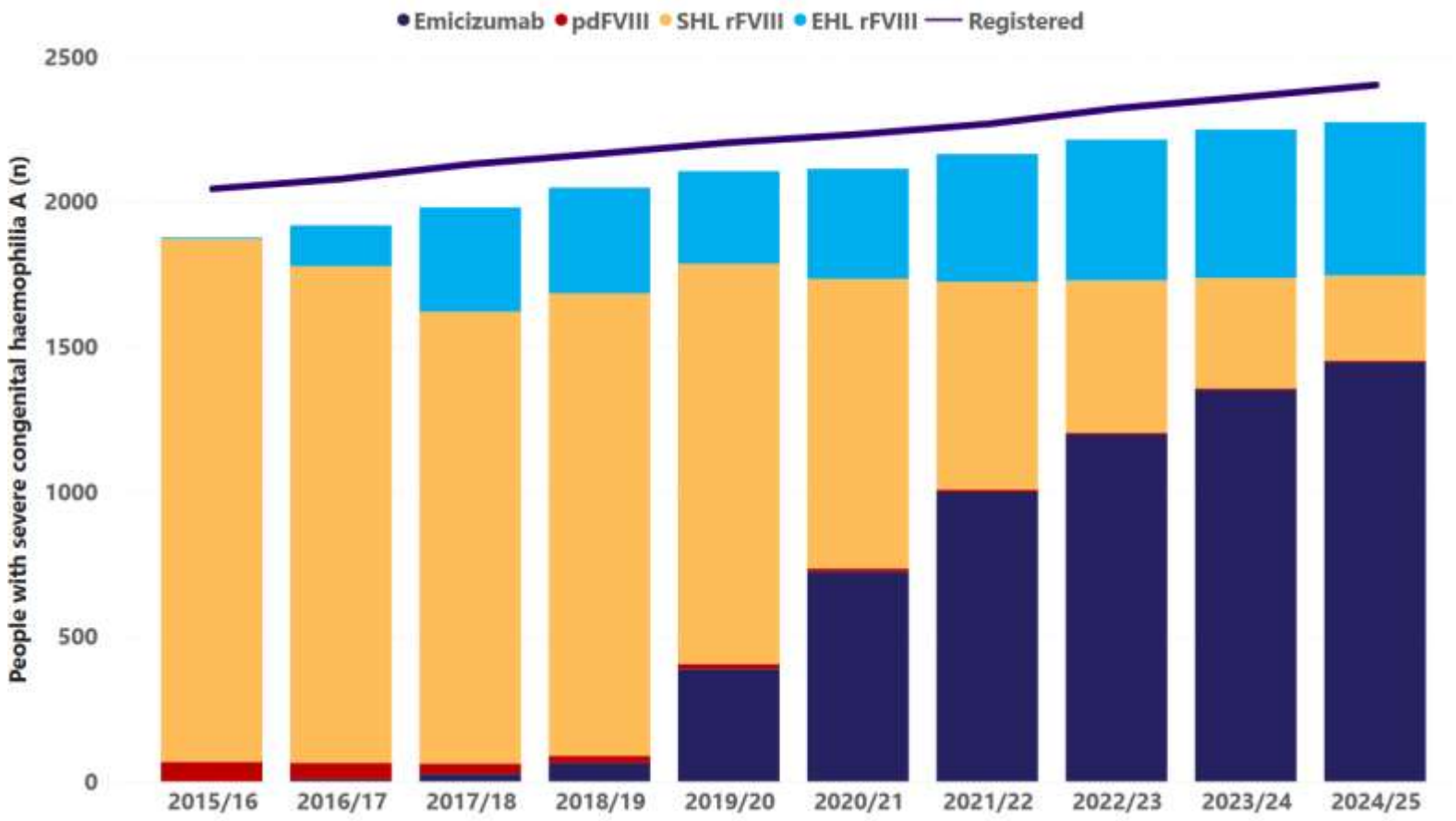


Figure 3: This shows the number of registered PwSHA (baseline FVIII level <1 IU/dl) issued FVIII or emicizumab as primary treatment from 2015/16 to 2024/25, including people with FVIII inhibitors. This chart shows a significant fall off in PwSHA issued FVIII as primary treatment from 2020/21, which is attributable to the introduction of emicizumab prophylaxis from September of 2019. There has also been a dramatic fall-off in the use of plasma-derived products over the past few years, which have almost completely fallen out of use. However, the proportion of EHL rFVIII has increased first with the introduction of Elocta, and more recently, Esperoct. No data on Altuvoct is shown, that having been commissioned on 1/7/25.

Figure 4 People with moderate severity congenital haemophilia A by primary treatment, 2015/16 - 2024/25

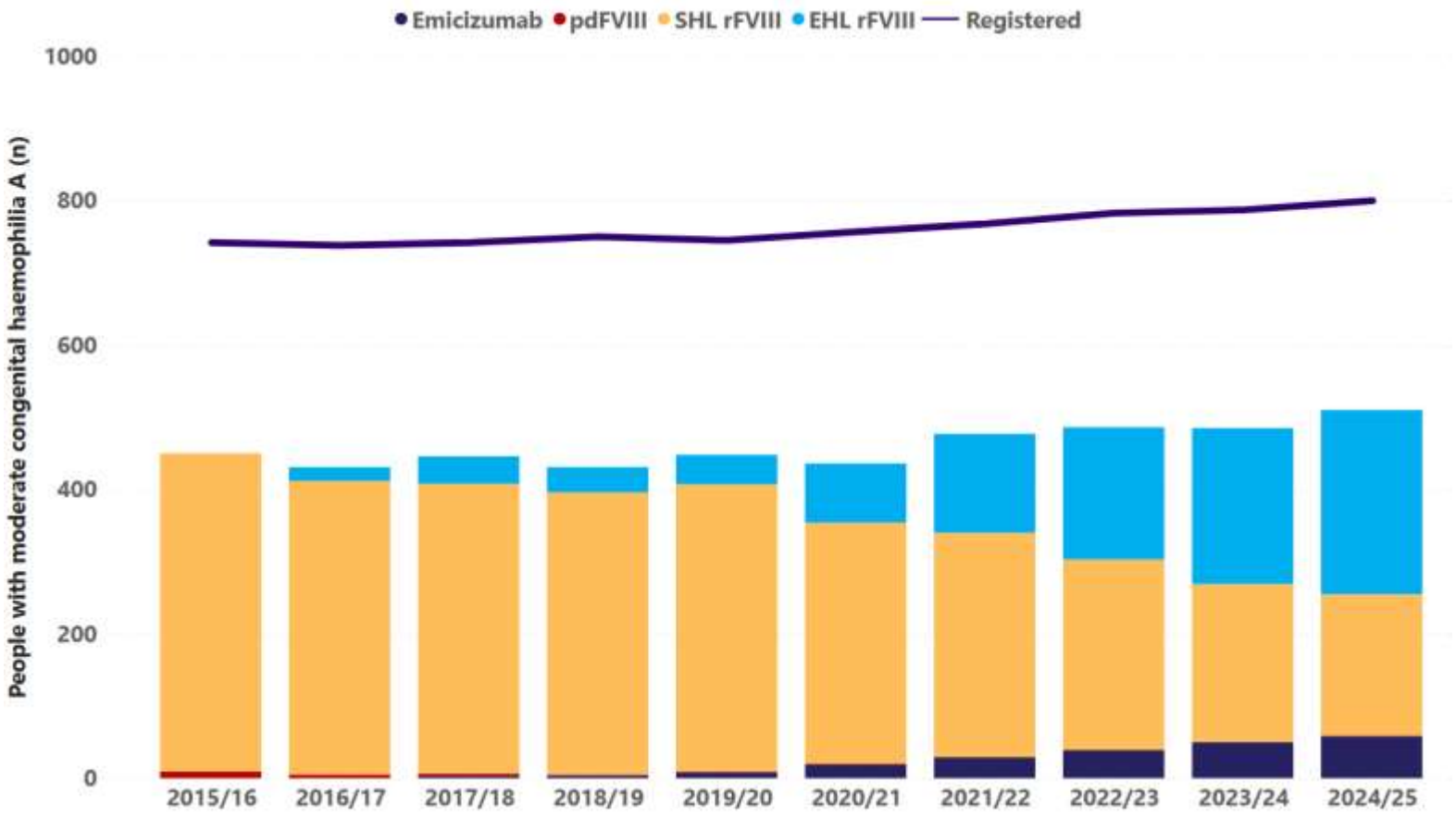


Figure 4: This shows the number of registered PwMHA (baseline FVIII level 1-5 IU/dl) issued FVIII or emicizumab as primary treatment from 2015/16 to 2024/25, including people with FVIII inhibitors. Emicizumab was not commissioned for use in people with moderate haemophilia without inhibitors at this time. The declining proportion of people issued SHL rFVIII, and increasing proportion of people issued EHL rFVIII can be observed. At the time of writing, Emicizumab has recently been commissioned for moderate haemophilia A and Atuvoct has only been commissioned for severe haemohilia A.

Data table for Figure 3: *People with severe congenital haemophilia A by primary treatment, 2015/16 – 2024/25*

Year	PwSHA issued pdFVIII		PwSHA issued SHL rFVIII		PwSHA issued EHL rFVIII		PwSHA issued emicizumab		PwSHA issued any FVIII or emicizumab		PwSHA registered
	n	% difference since 2015/16	n	% difference since 2015/16	n	% difference since 2016/17	n	% difference since 2016/17	n	% difference since 2015/16	n
2015/16	67	-	1806	-	5	-	-	-	1878	-	2046
2016/17	57	-14.9	1715	-5	141	-	7	-	1920	2.2	2080
2017/18	37	-44.8	1560	-13.6	360	155.3	25	257.1	1982	5.5	2131
2018/19	24	-64.2	1597	-11.6	365	158.9	64	814.3	2050	9.2	2167
2019/20	16	-76.1	1382	-23.5	320	127	389	5457.1	2107	12.2	2206
2020/21	11	-83.6	1000	-44.6	380	169.5	724	10242.9	2115	12.6	2234
2021/22	9	-86.6	717	-60.3	441	212.8	999	14171.4	2166	15.3	2270
2022/23	6	-91	526	-70.9	486	244.7	1198	17014.3	2216	18	2324
2023/24	6	-91	381	-78.9	513	263.8	1351	19200	2251	19.9	2363
2024/25	6	-91	294	-83.7	529	275.2	1447	20571.4	2276	21.2	2404

Data table for Figure 4: *People with moderate congenital haemophilia A by primary treatment, 2015/16 – 2024/25*

Year	PwMHA issued pdFVIII		PwMHA issued SHL rFVIII		PwMHA issued EHL rFVIII		PwMHA issued emicizumab		PwMHA issued any FVIII or emicizumab		PwMHA registered
	n	% difference since 2015/16	n	% difference since 2015/16	n	% difference since 2016/17	n	% difference since 2017/18	n	% difference since 2015/16	n
2015/16	9	-	441	-	-	-	-	-	450	-	742
2016/17	5	-44.4	407	-7.7	19	-	-	-	431	-4.2	738
2017/18	3	-66.7	402	-8.8	38	100	3	-	446	-0.9	742
2018/19	1	-	391	-11.3	35	84.2	4	33.3	431	-4.2	750
2019/20	1	-	398	-9.8	41	115.8	8	166.7	448	-0.4	745
2020/21	1	-	334	-24.3	82	331.6	19	533.3	436	-3.1	757
2021/22	0	-	311	-29.5	137	621.1	29	866.7	477	6	768
2022/23	0	-	264	-40.1	183	863.2	39	1200	486	8	783
2023/24	0	-	219	-50.3	216	1036.8	50	1566.7	485	7.8	787
2024/25	1	-	196	-55.6	255	1242.1	58	1833.3	510	13.3	800

Figure 5 Number of people with congenital haemophilia A issued non-trial products, 2015/16 – 2024/25

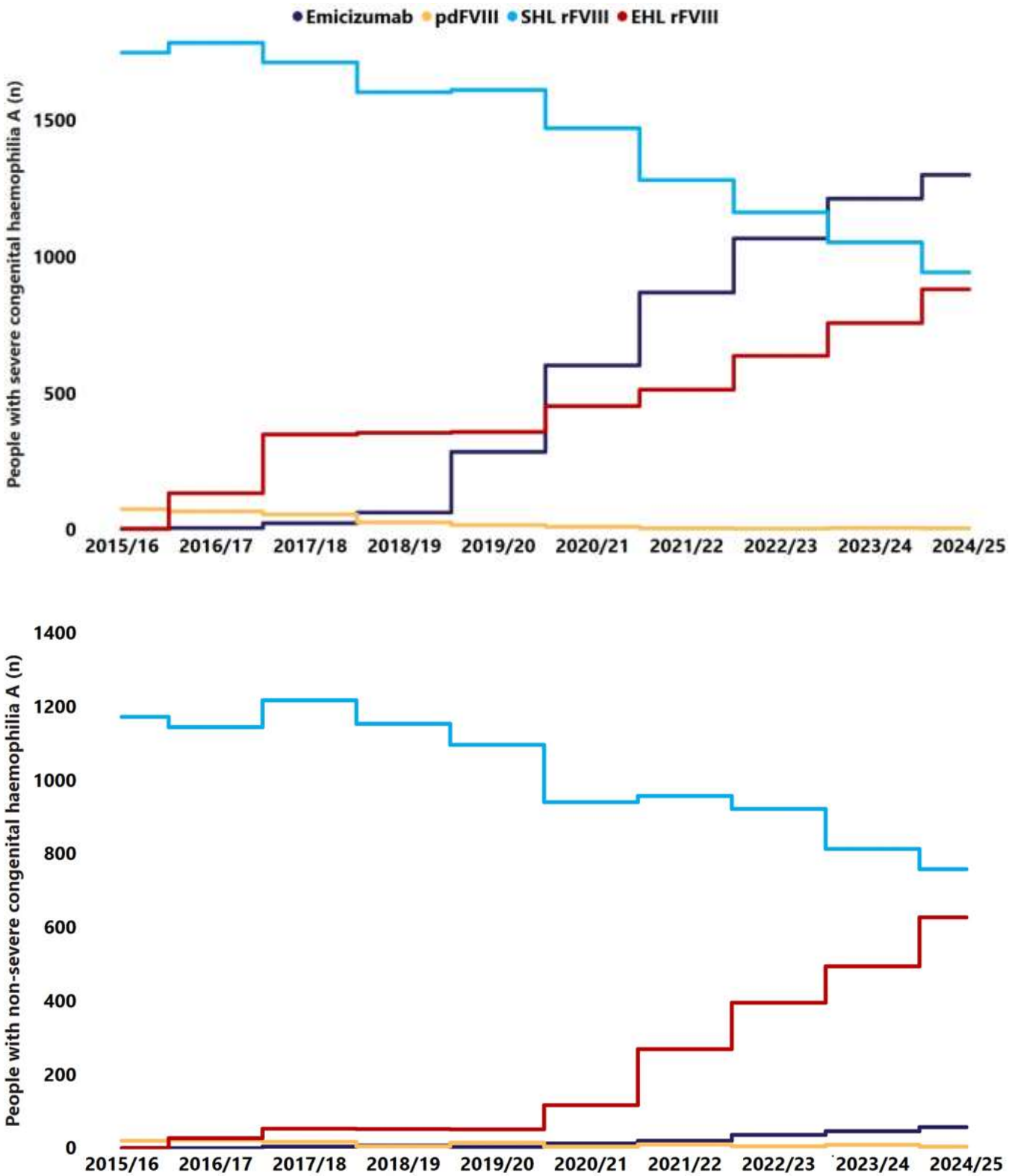


Figure 5 (previous page): This shows the number of people with haemophilia A (with and without FVIII inhibitors) using different product types, by severity. This shows a gradual introduction of enhanced half-life (EHL) FVIII from 2016. It also shows an overall reduction in the number of people with severe haemophilia A treated with standard half-life (SHL) FVIII as emicizumab was introduced for people with inhibitors in 2018 and for people with severe haemophilia A without inhibitors in 2019. As people may be issued with more than one product type in any given year, there is some double counting of people between product groups within years. Underlying data tables are available on request.

Figure 6 The proportion of people with severe congenital haemophilia A and no active inhibitor issued treatment by primary treatment 2018 Q2 - 2025 Q1

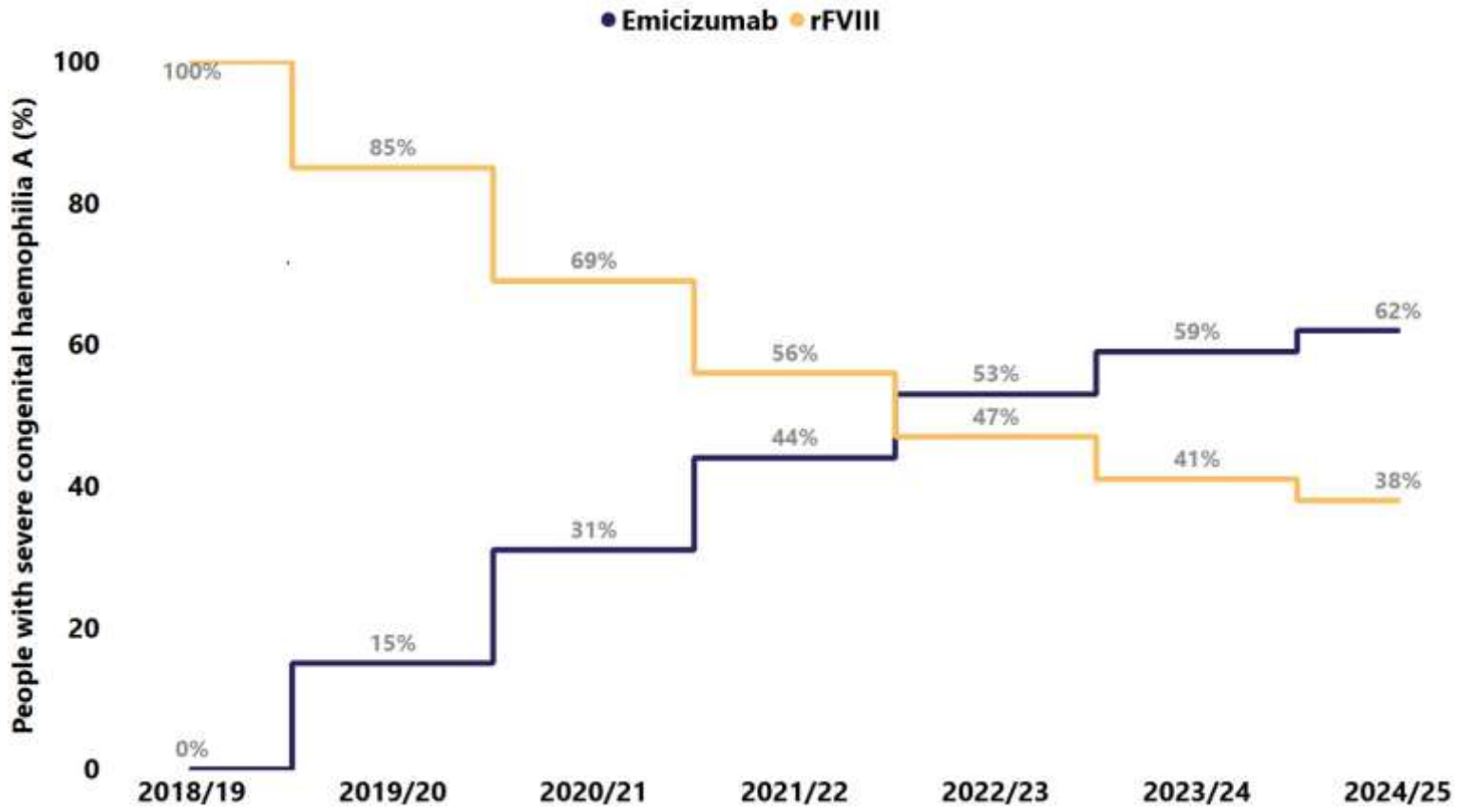


Figure 6: This shows a reduction in the proportion of people with severe haemophilia A, without inhibitors, receiving FVIII as their primary treatment since emicizumab was introduced to this group in 2019/20. People who received neither FVIII nor emicizumab are excluded.

Table 6 People with congenital haemophilia A issued with FVIII and not issued emicizumab by severity, 2015/16 – 2024/25

Year	Number of people with haemophilia A by factor VIII level (IU/dl)											
	<1			1 - 5			>5 & <40			≥ 40, unknown severity or people with a liver transplant		
	Registered n	Issued FVIII and not issued emicizumab n (%)	% change since 2015/16	Registered n	Issued FVIII and not issued emicizumab n (%)	% change since 2015/16	Registered n	Issued FVIII and not issued emicizumab n (%)	% change since 2015/16	Registered n	Issued FVIII and not issued emicizumab n (%)	% change since 2015/16
2015/16	2046	1948 (95.2)	-	742	464 (62.5)	-	3759	666 (17.7)	-	1364	38 (2.8)	-
2016/17	2080	1980 (95.2)	1.6	738	443 (60)	-4.5	3811	655 (17.2)	-1.7	1496	37 (2.5)	-2.6
2017/18	2131	2009 (94.3)	3.1	742	456 (61.5)	-1.7	3888	722 (18.6)	8.4	1614	49 (3)	28.9
2018/19	2167	2008 (92.7)	3.1	750	439 (58.5)	-5.4	3984	700 (17.6)	5.1	1721	43 (2.5)	13.2
2019/20	2206	1726 (78.2)	-11.4	745	452 (60.7)	-2.6	4042	699 (17.3)	5.0	1828	39 (2.1)	2.6
2020/21	2234	1398 (62.6)	-28.2	757	427 (56.4)	-8.0	4077	574 (14.1)	-13.8	1877	32 (1.7)	-15.8
2021/22	2270	1174 (51.7)	-39.7	768	462 (60.2)	-0.4	4136	678 (16.4)	1.8	2024	40 (2)	5.3
2022/23	2324	1027 (44.2)	-47.3	783	457 (58.4)	-1.5	4238	761 (18)	14.3	2101	57 (2.7)	50.0
2023/24	2363	906 (38.3)	-53.5	787	442 (56.2)	-4.7	4299	766 (17.8)	15.0	2250	53 (2.4)	39.5
2024/25	2404	831 (34.6)	-57.3	800	463 (57.9)	-0.2	4395	827 (18.8)	24.2	2400	46 (1.9)	21.1

Table 6: This table shows the number of PwHA issued FVIII in any amount, and not issued emicizumab, during the year, broken down by severity. The proportion of PwSHA in this group has declined rapidly in recent years. This is attributable to switching to emicizumab. People issued emicizumab who are bleed free may be issued a contingency stock of rFVIII only when their current stock goes out of date.

Table 7a Treatment intensity of people with severe congenital haemophilia A without inhibitors issued with standard half-life FVIII, 2015/16 - 2024/25

Year	People (n)		Treatment Intensity (Units/person/year)		Change in treatment intensity year on year (%)	
	<18 Years	≥18 years	<18 Years	≥18 years	<18 Years	≥18 years
2015/16	532	1,051	194,577	273,749	-	-
2016/17	472	1,039	197,383	274,553	1.4	0.3
2017/18	412	972	201,376	269,020	2.0	-2.0
2018/19	429	999	204,672	294,702	1.6	9.5
2019/20	361	886	213,890	292,385	4.5	-0.8
2020/21	257	660	220,732	272,691	3.2	-6.7
2021/22	190	456	237,473	271,104	7.6	-0.6
2022/23	125	352	270,450	266,753	13.9	-1.6
2023/24	78	265	293,647	270,341	8.6	1.3
2024/25	51	217	339,564	278,372	15.6	3.0

Table 7a: This shows that the number of people issued SHL rFVIII alone has reduced dramatically over the last ten years. For those who continue to use SHL rFVIII, treatment intensity has increased considerably in children, although it has remained stable in adults, corresponding with a gradual increase in the intensity of prophylaxis and improved bleed outcomes during that period. The proportion of people aged under 18 years with a past history of an inhibitor has increased from 19 to 39% over this period, whilst in those aged 18 years and above it has increased from 14 to 18%.

NOTE - exclusions from this analysis: People issued with emicizumab, people issued with both EHL- and SHL-FVIII, people using gene therapy, people resident overseas, people not registered for full treatment period, people for whom inhibitors are reported within the treatment period

Table 7b Treatment intensity of people with severe congenital haemophilia A without inhibitors treated with enhanced half-life FVIII, 2017/18- 2024/25

Year	People (n)		Treatment Intensity (Units/person/year)		Change in treatment intensity year on year (%)	
	<18 Years	≥18 years	<18 Years	≥18 years	<18 Years	≥18 years
2017/18	72	73	190,583	278,703	-	-
2018/19	116	175	180,696	276,603	-5.2	-0.8
2019/20	114	139	182,796	268,457	1.2	-2.9
2020/21	89	138	176,936	259,288	-3.2	-3.4
2021/22	89	217	202,686	320,951	14.6	23.8
2022/23	97	254	198,745	302,696	-1.9	-5.7
2023/24	105	288	223,705	307,564	12.6	1.6
2024/25	122	313	240,247	311,170	7.4	1.2

Table 7b: This shows a fluctuating upward trend in the treatment intensity (units/person/year) of EHL FVIII for severe haemophilia A. The increase in the number of adult PwSHA issued EHL, and the increased treatment intensity in 2021/22 corresponds with the widespread uptake of Esperoct. The proportion of people aged under 18 years with inhibitor histories has increased from 15 to 36% over this period, whilst in those aged 18 years and above it has increased from 19 to 25%.

NOTE - exclusions from this analysis:

People issued with emicizumab, people issued with both EHL and SHL FVIII, people using gene therapy, people resident overseas, people not registered for full treatment period, people for whom inhibitors are reported within the treatment period

Table 8a Treatment intensity of people with moderate severity congenital haemophilia A (0.01 - 0.03 IU/ml) treated with standard half-life FVIII, with no inhibitor, 2015/16 - 2024/25

Year	People (n)		Treatment Intensity (Units/person/year)		Change in treatment intensity year on year (%)	
	<18 Years	≥18 years	<18 Years	≥18 years	<18 Years	≥18 years
2015/16	73	189	135,521	132,332	-	-
2016/17	63	179	136,901	140,188	1.0	5.9
2017/18	69	167	105,743	121,516	-22.8	-13.3
2018/19	53	165	126,462	139,464	19.6	14.8
2019/20	54	168	141,912	144,418	12.2	3.6
2020/21	41	149	120,738	137,549	-14.9	-4.8
2021/22	37	123	89,980	133,827	-25.5	-2.7
2022/23	33	115	93,280	150,691	3.7	12.6
2023/24	30	87	77,883	152,299	-16.5	1.1
2024/25	27	76	89,056	137,816	14.3	-9.5

Table 8a: This table shows the trend in treatment intensity in people with haemophilia A and factor level between 0.01 - 0.03 IU/ml over the last ten years. Only people treated during this time are included. However, the range of baseline FVIII levels and bleeding phenotypes included in this data ranges from those on regular prophylaxis to those requiring only occasional treatment. This renders the data more difficult to interpret and impossible to compare directly with the relatively more homogeneous group of people with severe haemophilia A. Emicizumab was not commissioned for moderate severity haemophilia A at that time. This shows that treatment intensity fluctuates over time in both age groups. However, these changes may reflect PwHA selection more than anything else. "Heavy users" with a relatively high annualised bleed rate are more likely to be chosen to switch to an alternative EHL rFVIII treatment. The number of both adults and children issued product has reduced over recent years, as people have been

switched to EHL rFVIII. The proportion of people aged under 18 years with inhibitor histories has decreased from 11 to 0% over this period, whilst in those aged 18 years and above it has increased from 3 to 8%.

NOTE - exclusions from this analysis: People issued with emicizumab, people issued with both EHL and SHL FVIII, people using gene therapy, people resident overseas, people not registered for full treatment period, people for whom inhibitors are reported within the treatment period

Table 8b Treatment intensity of people with moderate congenital haemophilia A (0.01 - 0.03 IU/ml) treated with enhanced half-life FVIII, with no inhibitor, 2017/18 - 2024/25

Year	People (n)		Treatment Intensity (Units/person/year)		Change in treatment intensity year on year (%)	
	<18 Years	≥18 years	<18 Years	≥18 years	<18 Years	≥18 years
2017/18	3	8	101,488	251,000	-	-
2018/19	8	20	180,719	263,650	78.1	5.0
2019/20	8	23	226,969	216,924	25.6	-17.7
2020/21	6	22	94,125	283,534	-58.5	30.7
2021/22	17	42	77,785	271,583	-17.4	-4.2
2022/23	23	61	84,663	273,021	8.8	0.5
2023/24	29	79	139,129	241,503	64.3	-11.5
2024/25	34	95	126,934	230,144	-8.8	-4.7

Table 8b: This illustrates the introduction of EHL FVIII to a relatively small group of people with haemophilia A and a factor level between 0.01 - 0.03 IU/ml between April 2017 and March 2025. The proportion of people with inhibitor histories has increased from 0 to 6% in both age groups.

NOTE - exclusions from this analysis:

People issued with emicizumab, people issued with both EHL and SHL FVIII, people using gene therapy, people resident overseas, people not registered for full treatment period, people for whom inhibitors are reported within the treatment period,

Table 9 Non-trial products issued to treat people with congenital haemophilia A (all severities and including inhibitors), 2023/24 and 2024/25

Product Type	Manufacturer	Product	2023/24		2024/25	
			Units (IU)	People (n)	Units (IU)	People (n)
Non-factor	Roche	Emicizumab (mg)	6,005,003	1,420	6,535,150	1,528
Recombinant	LFB	Cevenfacta	101	2	369	3
	Novo Nordisk	NovoSeven (mg)	12,752	124	10,130	117
		Esperoct	102,856,028	643	108,175,060	796
		NovoEight	24,145,000	209	11,956,300	138
	Octapharma	Nuwiq	8,364,000	110	6,787,000	101
	Pfizer	ReFacto AF	50,676,650	560	43,200,500	501
	SOBI/Biogen	Elocta	84,041,500	653	98,165,600	779
	Takeda	Advate	86,376,000	1,061	76,096,890	1,061
		Adynovi	8,680,560	20	6,172,000	22
		Obizur	6,000	1	98,000	1
Plasma-derived	BPL	BPL FVIII 8Y	229,120	2	373,890	1
		Optivate	39,000	2	-	-
	Baxter	Com FVII	83	1	-	-
	CSL Behring	Riastap	-	-	2	1
		Voncento	310,000	8	205,500	5
	Grifols	Haemoctin	351,000	1	308,000	1
	Octapharma	Octanate	1,914,000	5	2,012,000	5
		Wilate	4,500	2	-	-
	Takeda	FEIBA	2,501,000	17	2,431,000	19

Units in IU unless otherwise stated
Products which include VWF and FVIII are reported in FVIII units
Excludes issues of investigational products

Table 9 (previous page): This shows a breakdown of product volumes, listed by supplier, issued to treat people with congenital haemophilia A during 2023/24 and 2024/25, including those with inhibitors. Please note these data include people with haemophilia A combined with other blood disorders, e.g. haemophilia B and von Willebrand disease. People may be issued multiple products. The data reflects the reduction in FVIII issues in recent years, as emicizumab was introduced to treat people with severe haemophilia A with and without inhibitors. Furthermore, there appears to be fluctuation in volumes issued between FVIII brands over the last two years.

These figures have been cross-checked against manufacturers' sales figures, where available. Whilst one would not expect a perfect match, there is a high level of correlation. These sales figures are not reported, by agreement with suppliers, for reasons of commercial sensitivity.

By and large, the plasma-derived products listed were used for immune tolerance induction. ITI is also being used less frequently when inhibitors arise or relapse. In general, there is a very steep decline in the use of plasma-derived factor VIII for prophylaxis, which has largely fallen out of use altogether.

Investigational products are not reported due to confidentiality agreements and commercial sensitivities. Their use has declined steeply in recent years as clinical trials have come to an end.

Figure 7 Number of people with congenital haemophilia A (all severities) issued treatment in the UK 2024/25 by non-trial product

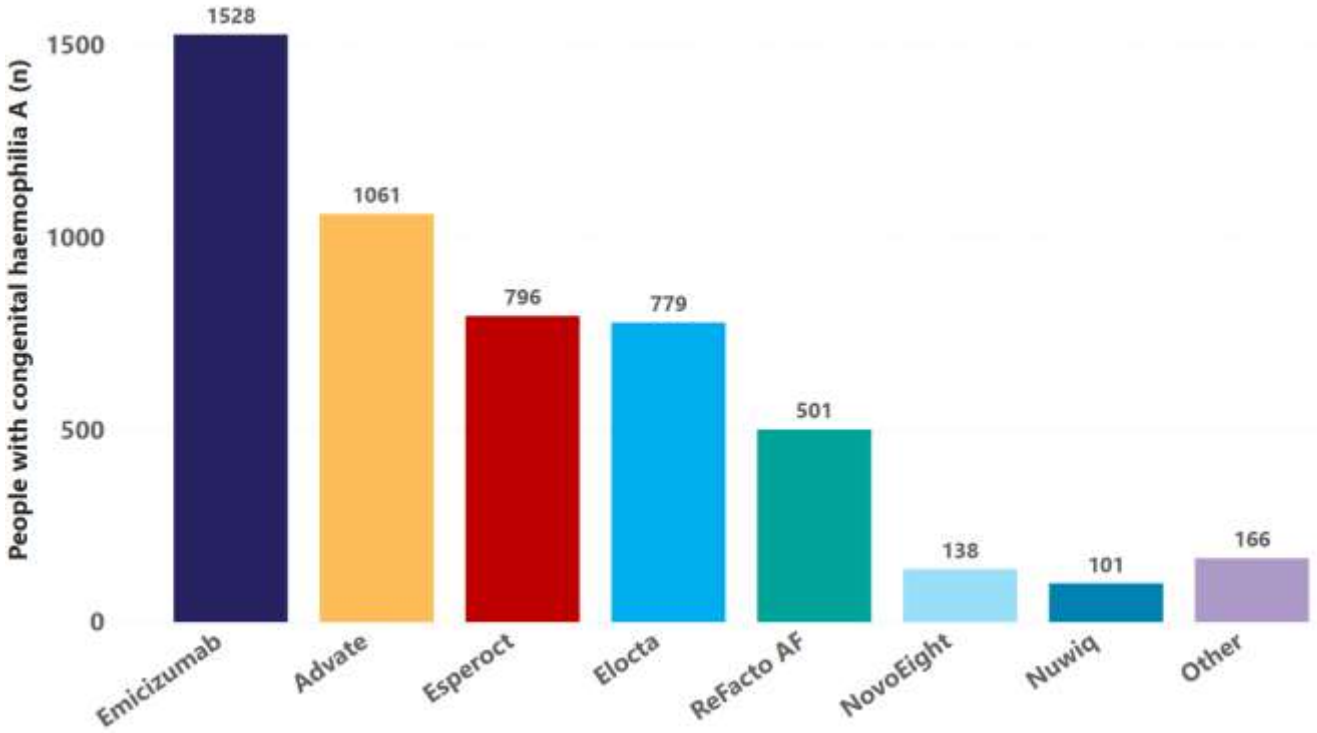


Figure 7: This shows the number of people with haemophilia A issued each product during the year, including those issued to people with an inhibitor. The data that informs this graph can be found in Table 10. People issued multiple products in the year are included in the numbers for each product, so there is some double counting. There were 3,678 individual people with haemophilia A issued these products during the year. Adynovi and Octanate have been withdrawn from the UK market.

Table 10 Non-trial Factor VIII and emicizumab treatment issued by UK haemophilia centres, by diagnosis 2024/25

Bleeding disorder	Factor VIII (IU)					Emicizumab		Total
	People (n)	Plasma-derived	Standard half-life recombinant	Enhanced half-life recombinant	Total	People (n)	mg	People (n)
Haemophilia A	3,106	2,899,390	138,138,690	212,512,660	353,550,740	1,528	6,535,150	3,649
von Willebrand disease	619	26,129,504	391,250	1,540,000	28,060,754	-	-	619
Acquired haemophilia A	53	5,000	4,019,500	79,000	4,103,500	7	18,480	57
Acquired von Willebrand disease	36	623,000	27,000	2,000	652,000	-	-	36
Combined factors V and VIII deficiencies	8	-	14,000	53,000	67,000	-	-	8
Multiple diagnoses	3	-	177,000	143,000	320,000	2	7,560	4
Miscellaneous	3	127,160	-	-	127,160	-	-	3
Bleeding disorder of unknown cause	2	-	2,500	-	2,500	-	-	2
Probable von Willebrand disease	1	8,000	-	-	8,000	-	-	1
Total	**3,831**	**29,792,054**	**142,769,940**	**214,329,660**	**386,891,654**	**1,537**	**6,561,190**	**4,379**

Excludes issues of investigational products

Table 10: This table shows FVIII and emicizumab issues by diagnosis, including any apparently anomalous reported use. Products used to treat VWD which include VWF and FVIII are reported in FVIII units. Issues of these products to treat VWD is provided in Table 21. Please note people issued with both FVIII and emicizumab are counted only once in the final column. Issues of emicizumab for acquired haemophilia A are presumed to be related to individual funding requests, as these indications are unlicensed and not yet subject to NHSE policy documents.

Figure 8 The number and proportion of people with severe congenital haemophilia A and no active inhibitor issued emicizumab by comprehensive care centre

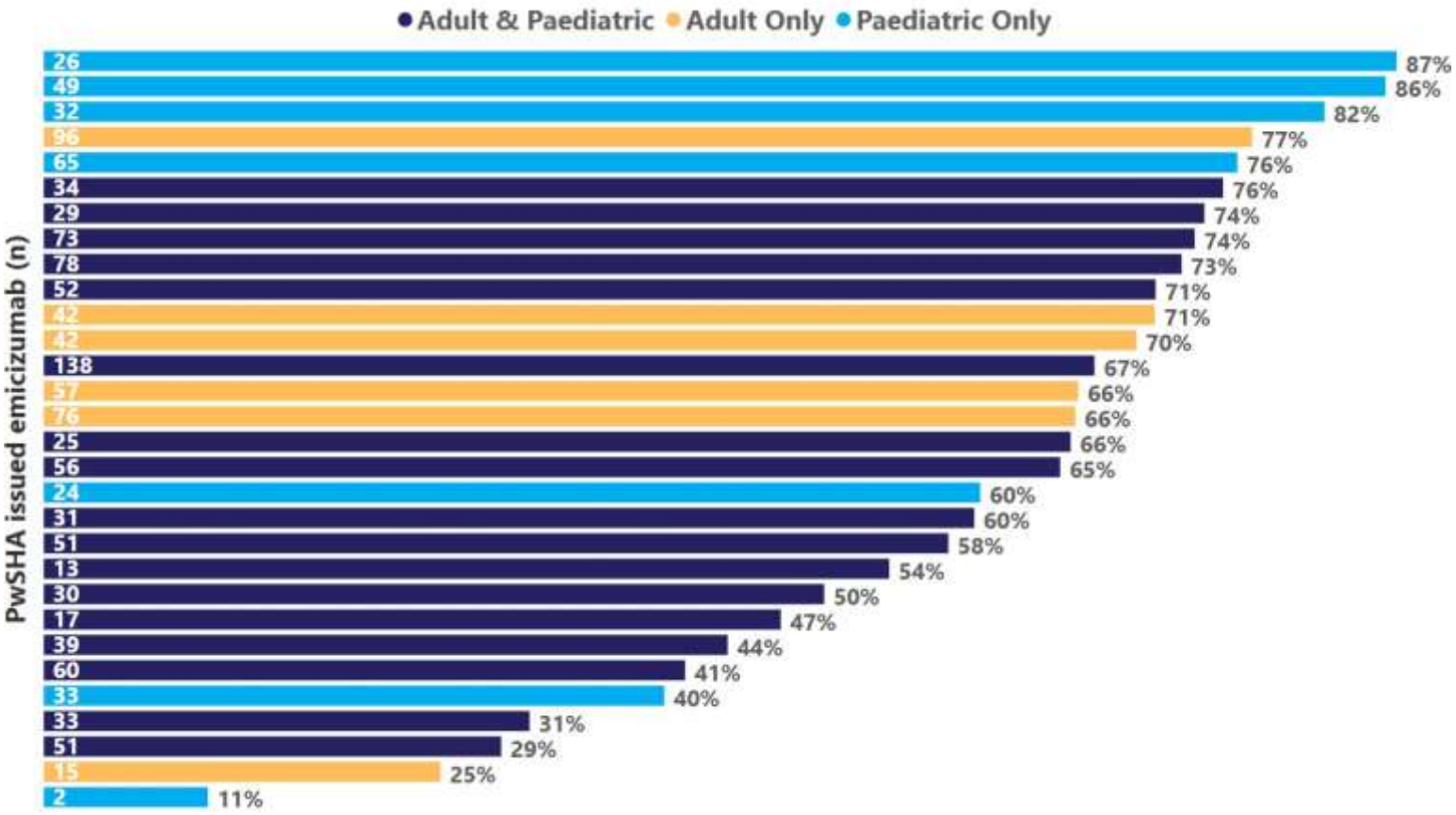


Figure 8: This shows the ranked proportion of people with severe congenital haemophilia A without an inhibitor who were issued emicizumab, by comprehensive care centre, in 2024/25. This shows considerable centre-to-centre variation (median 66%, IQR 46-74%), which reflects diversity of clinical opinion. As emicizumab is issued via CCCs, HCs are not included.

Figure 9 Mean mg/Kg/Week dose of non-factor concentrate by weight (weekly (n=58) and fortnightly (n=321) frequencies, FVIII severity <1%)

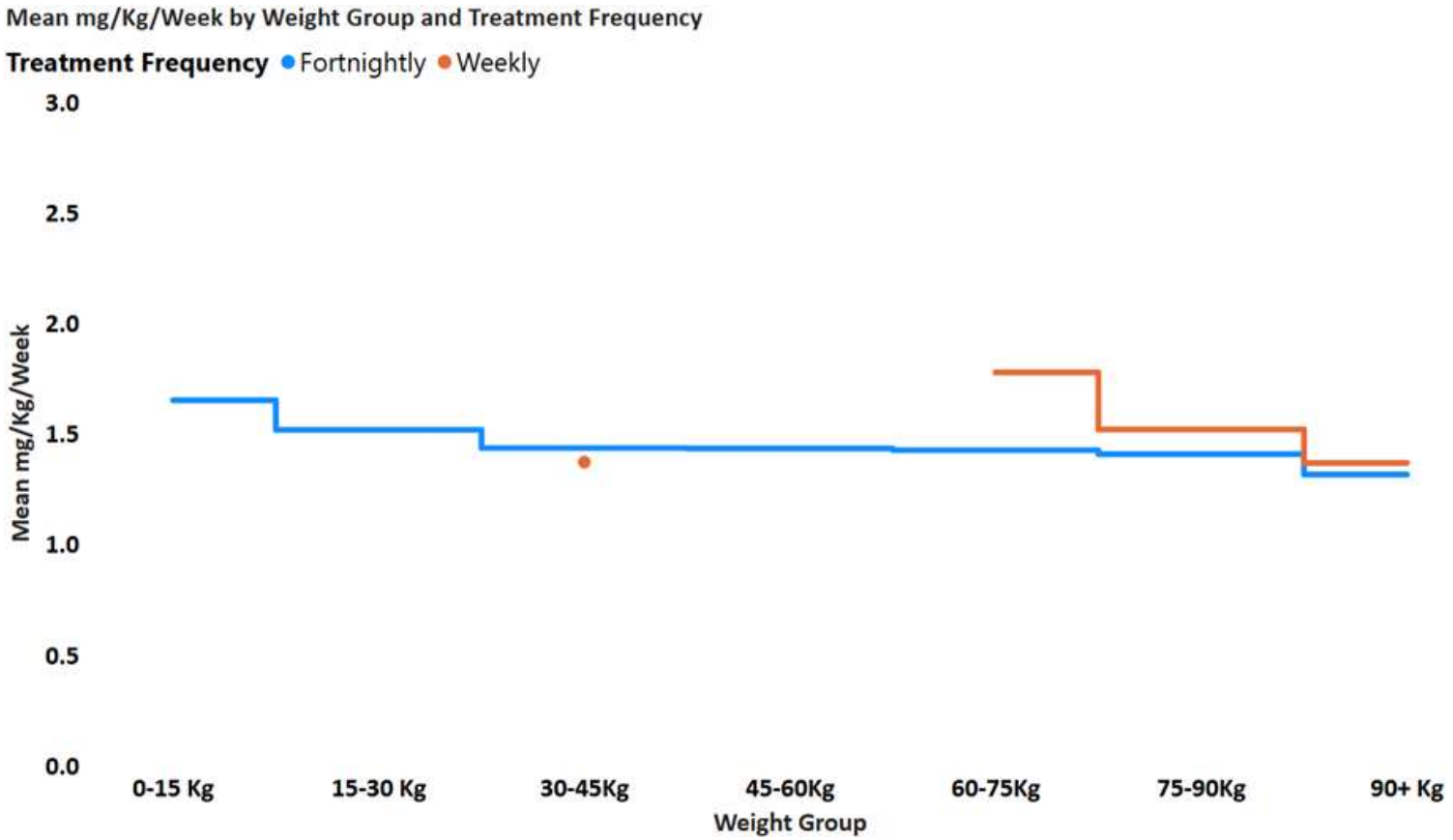
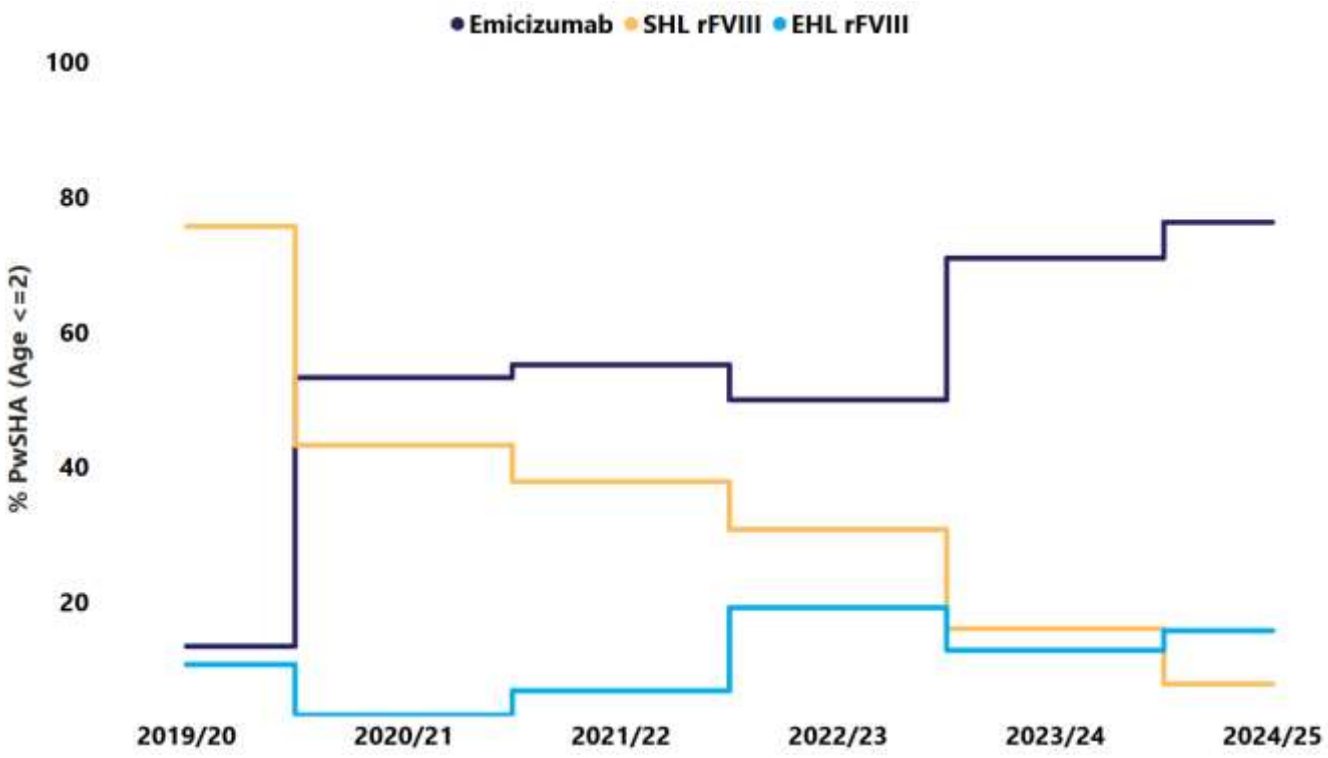


Figure 9: This shows dosage in mg/kg across the range of bodyweight by frequency of administration. This shows a slight reduction in mg/kg across the weight range. It also shows that the 60-75 Kg group treated weekly use significantly higher doses/kg than those dosing fortnightly, presumably because of the available vial sizes and "rounding up" of doses. Many centres have a marked preference for fortnightly dosing because it makes it easier to minimize wastage. Ideally, all PwSHA should use 1.5 mg/kg/week.

Figure 10 The proportion of previously untreated people with severe congenital haemophilia A by primary treatment 2019/20 - 2024/25



Treatment group	Previously untreated people with congenital severe haemophilia A aged <= 2 years					
	2019/20	2020/21	2021/22	2022/23	2023/24	2024/25
SHL rFVIII	28 (75.7%)	13 (43.3%)	11 (37.9%)	8 (30.8%)	5 (16.1%)	3 (7.9%)
EHL rFVIII	4 (10.8%)	1 (3.3%)	2 (6.9%)	5 (19.2%)	4 (12.9%)	6 (15.8%)
Emicizumab	5 (13.5%)	16 (53.3%)	16 (55.2%)	13 (50%)	22 (71%)	29 (76.3%)

Figure 10: This shows a marked treatment trend since 2019/20, with fewer previously untreated PwSHA aged under two years starting on SHL rFVIII, and the majority now receiving emicizumab as their first treatment. Some centres start all PUPwSHA on emicizumab, although the trend appears to have levelled off in the last two years. It is suspected that although the reduction in issues of rFVIII will continue, a proportion of PUPwSHA will start on Altuvoct. This may become apparent in the next year, now this product has been commissioned for SHA.

Table 11 Haemophilia joint health scores for people with congenital haemophilia A (FVIII <=5 IU/dl) without inhibitors 2014/15 - 2024/25

FVIII level	Age (years)	Year	Registered, n	HJHS submitted, n (% of registered)	HJHS evaluable, n (% of submitted)	HJHS = 0, n (% of submitted)	Median (iqr) evaluable HJHS
<1 IU/dl	4-9	2014/15	229	80 (34.9)	78 (97.5)	48 (60.0)	0.0 (0.0 -2.3)
		2019/20	228	82 (36.0)	77 (93.9)	49 (59.8)	0.0 (0.0 -1.5)
		2024/25	236	55 (23.3)	54 (98.2)	34 (61.8)	0.0 (0.0 -1.6)
	10-17	2014/15	264	91 (34.5)	88 (96.7)	34 (37.4)	1.0 (0.0 -5.9)
		2019/20	289	128 (44.3)	128 (100.0)	64 (50.0)	0.5 (0.0 -3.0)
		2024/25	324	86 (26.5)	85 (98.8)	48 (55.8)	0.0 (0.0 -2.8)
	18-49	2014/15	971	222 (22.9)	199 (89.6)	17 (7.7)	14.0 (6.0 -30.0)
		2019/20	1,080	349 (32.3)	304 (87.1)	35 (10.0)	13.0 (3.3 -28.0)
		2024/25	1,221	248 (20.3)	235 (94.8)	47 (19.0)	10.0 (1.0 -22.0)
	50 and above	2014/15	224	52 (23.2)	47 (90.4)	0 (0.0)	55.0 (37.0 -72.0)
		2019/20	301	95 (31.6)	79 (83.2)	0 (0.0)	44.0 (32.0 -59.0)
		2024/25	354	85 (24.0)	64 (75.3)	0 (0.0)	37.0 (26.5 -44.8)
1-5 IU/dl	4-9	2014/15	64	18 (28.1)	18 (100.0)	13 (72.2)	0.0 (0.0 -1.3)
		2019/20	56	11 (19.6)	11 (100.0)	8 (72.7)	0.0 (0.0 -3.0)
		2024/25	57	13 (22.8)	13 (100.0)	5 (38.5)	1.0 (0.0 -3.5)
	10-17	2014/15	85	21 (24.7)	21 (100.0)	8 (38.1)	1.0 (0.0 -3.3)
		2019/20	88	25 (28.4)	25 (100.0)	16 (64.0)	0.0 (0.0 -1.0)
		2024/25	89	14 (15.7)	14 (100.0)	9 (64.3)	0.0 (0.0 -2.3)
	18-49	2014/15	335	27 (8.1)	22 (81.5)	3 (11.1)	11.0 (3.5 -19.5)
		2019/20	338	46 (13.6)	40 (87.0)	13 (28.3)	5.5 (0.0 -20.8)
		2024/25	363	28 (7.7)	28 (100.0)	10 (35.7)	3.0 (0.0 -16.8)
	50 and above	2014/15	198	23 (11.6)	22 (95.7)	0 (0.0)	14.0 (7.9 -26.3)
		2019/20	201	25 (12.4)	18 (72.0)	1 (4.0)	26.5 (12.8 -39.5)
		2024/25	224	15 (6.7)	11 (73.3)	0 (0.0)	17.0 (6.0 -24.0)

Table 11 and Figure 11 (overleaf): These show HJHS for PwHA without inhibitors over the last ten years. It is hoped that the low submission of HJHS data for PwSHA and PwMHA can be improved. However, submission rates are lower in 2024/25 than 2019/2020. This is thought to reflect computerisation of medical records such that HJHS is recorded directly into the hospital record, and must be transcribed into HCIS. This shows that reported HJHS scores for children have been stable and close to zero for the 10-year period shown. It would appear that joint scores for older PwSHA have improved over the last decade. This may be a response to increasing factor VIII treatment intensity, as it antedates the introduction of emicizumab. It is notable that older PwMHA still have significant arthropathy, albeit with lower joint scores than those observed in PwSHA.

Figure 11 Median evaluable HJHS of people with congenital haemophilia A (FVIII <=5 IU/dl) without inhibitors 2014/15 - 2024/25

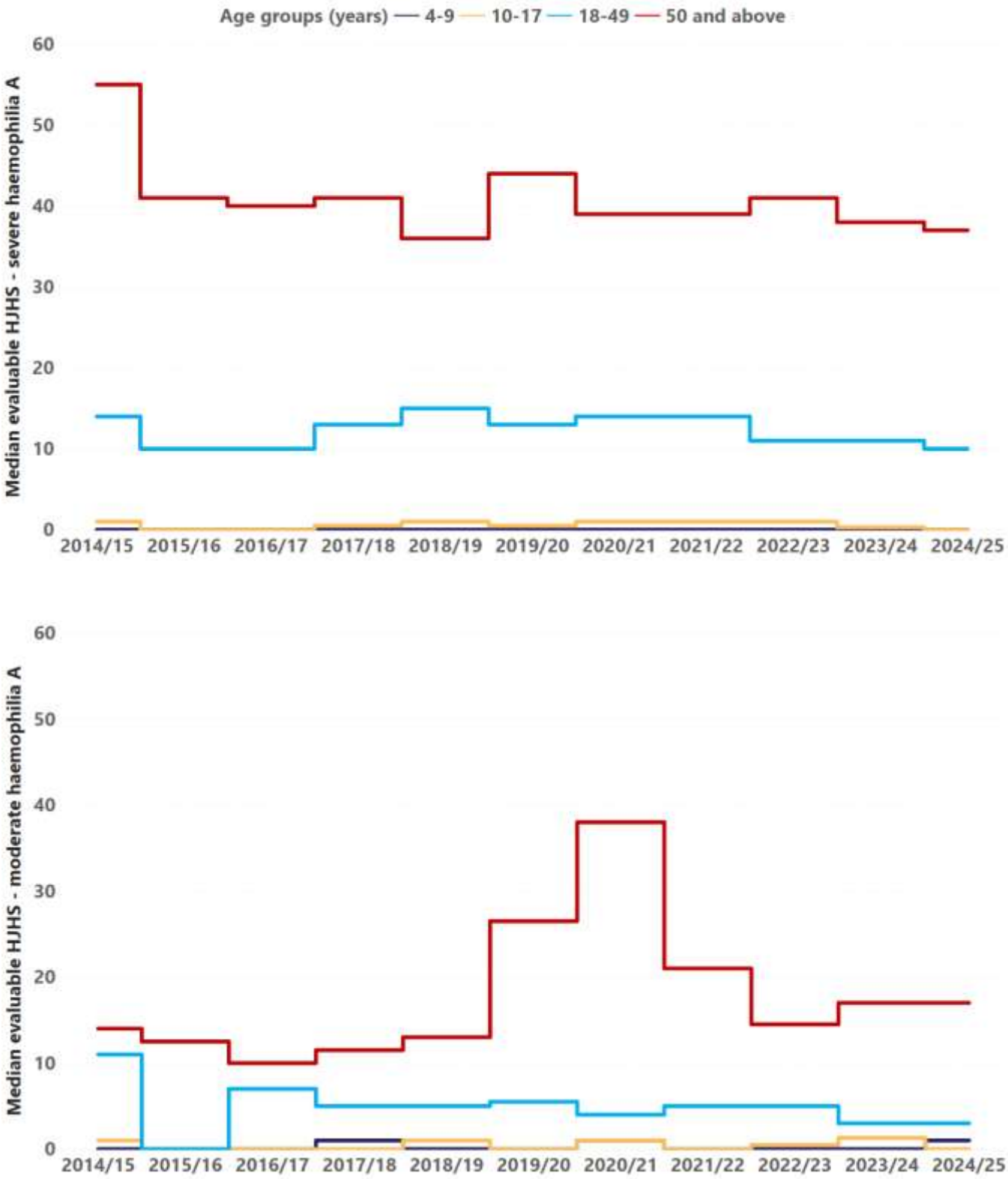


Table 12 Longitudinal haemophilia joint health scores for people with congenital haemophilia A (FVIII <=5 IU/dl) without inhibitors 2014/15 - 2024/25

FVIII level (IU/dl)	Age (years) 2014/15	Registered 2014/15 (n)	HJHS evaluable 2014/15 (n)	HJHS evaluable 2014/15 and 2024/25	Median (iqr) evaluable HJHS		
					2014/15	2019/20	2024/25
<1	4-9	229	78	26	0 (0-1.3)	0 (0-1.9)	0 (0-1)
	10-17	264	88	22	1.8 (0-4.5)	0 (0-2)	0 (0-3.3)
	18-49	971	199	62	9.8 (3-25.5)	14.5 (4.1-23)	16 (8.8-26.8)
	50 and above	224	47	9	37 (20-49.3)	32 (11.9-33.8)	32 (20.5-41.5)
1-5	4-9	64	18	3	0 (0-4)	2.3 (2.3-2.3)	0 (0-6)
	10-17	85	21	4	1 (0-2.4)	0.5 (0-2.5)	0 (0-1.5)
	18-49	335	22	6	14.5 (3-22.5)	7 (5-28)	15.5 (3-23.8)
	50 and above	198	22	2	16 (8-24)	-	16.5 (9-24)

Table 12: This shows longitudinal within-person HJHS for PwHA with HJHS assessed in 2014/15 and 2024/25. It can be observed that joint health in pre-adolescent children with SHA as of 2014/15 is maintained into their teenage years, and the joint health of adolescents with SHA in 2014/15 has improved as they have reached their twenties. The joint health of adults with SHA in 2014/15 appears to have stabilised by 2024/25. However, longitudinal HJHS data is available only for a minority of PwHA.

2.2 Congenital haemophilia B

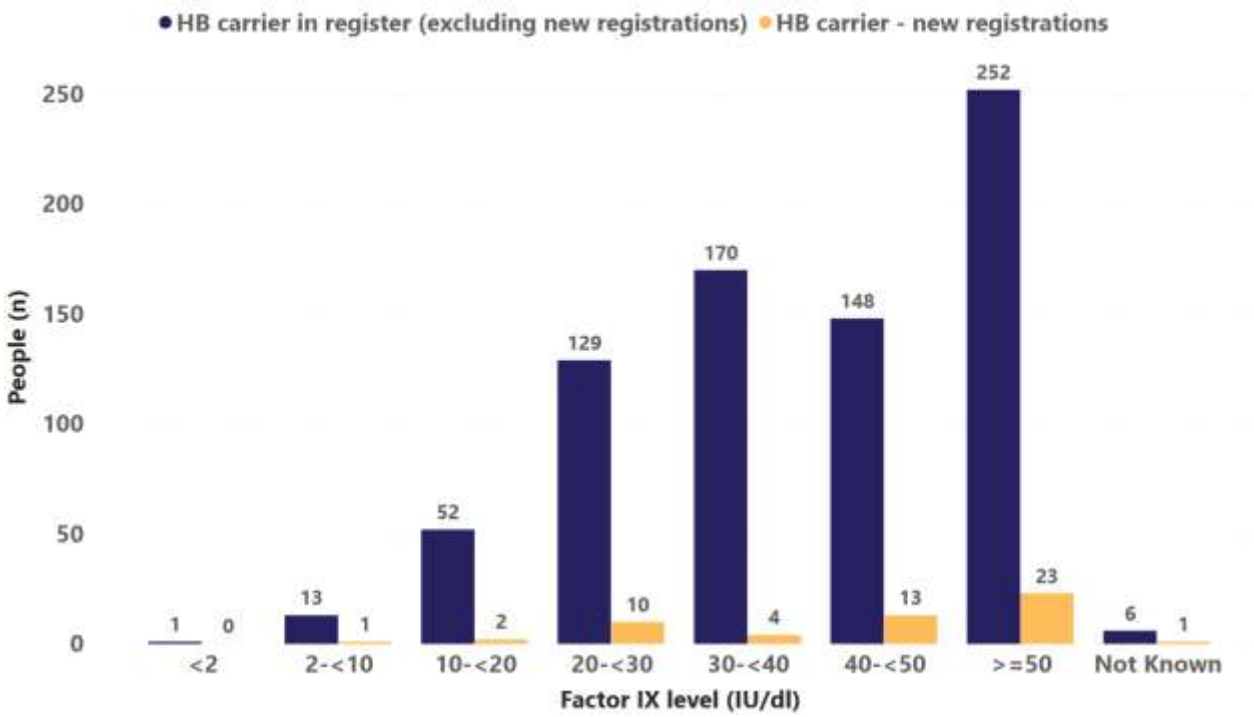
Table 13 People with congenital haemophilia B and carriers, registered and treated, 2024/25

Congenital haemophilia B	Age (years)	Number of people by factor IX level (IU/dl)																	
		< 1			1 - 5			>5 & <40			≥ 40			People with unknown severity or liver transplant			Total*		
		Male	Female	Total	Male	Female	Total	Male	Female	Total	Male	Female	Total	Male	Female	Total	Male	Female	Total
Total Registered	<18	137	1	138	80	0	80	109	91	200	7	54	61	3	1	4	336	147	483
	>=18	264	0	264	275	6	281	479	284	763	50	382	432	8	6	14	1,076	678	1,754
	Total	**401**	**1**	**402**	**355**	**6**	**361**	**588**	**375**	**963**	**57**	**436**	**493**	**11**	**7**	**18**	**1,412**	**825**	**2,237**
New Registrations **	<18	11	0	11	8	0	8	7	6	13	1	8	9	2	0	2	29	14	43
	>=18	1	0	1	4	0	4	8	11	19	3	28	31	0	1	1	16	40	56
	Total	**12**	**0**	**12**	**12**	**0**	**12**	**15**	**17**	**32**	**4**	**36**	**40**	**2**	**1**	**3**	**45**	**54**	**99**
Issued concentrate FIX in year***	<18	127	0	127	52	0	52	38	5	43	3	0	3	0	0	**0**	220	5	225
	>=18	238	0	238	146	0	146	123	42	165	5	13	18	2	0	**2**	514	55	569
	Total	**365**	**0**	**365**	**198**	**0**	**198**	**161**	**47**	**208**	**8**	**13**	**21**	**2**	**0**	**2**	**734**	**60**	**794**

Sex as registered at birth
***New registrations are a subset of the 'In Register' numbers*
****Excluding people only treated with DDAVP and tranexamic acid*

Table 13: This shows the number of people with haemophilia B (including FIX Leyden, carriers and females with factor IX deficiency), broken down by severity, sex at birth and age. The number of new registrations is also shown, as are the numbers issued FIX concentrate.

Figure 12 Females with congenital FIX deficiency and carriers of haemophilia B currently registered and newly registered, by baseline FIX level, 2024/25



N.B: Includes carriers of haemophilia B and females with FIX deficiency

Diagnosis	Number of people by factor IX level (IU/dl)								
	<2	2-<10	10-<20	20-<30	30-<40	40-<50	≥50	Not Known	Total
Haemophilia B carrier in register (excluding new registrations)	1	13	52	129	170	148	252	6	771
Haemophilia B carrier New registrations	0	1	2	10	4	13	23	1	54
Total	**1**	**14**	**54**	**139**	**174**	**161**	**275**	**7**	**825**

Figure 12: This shows the distribution of reported FIX levels amongst registered carriers of haemophilia B in the UK. All carriers should be registered.

It is interesting that there is a relatively large number of very low-level carriers. These mostly have an extreme degree of lyonisation, but some are homozygous daughters from consanguineous unions.

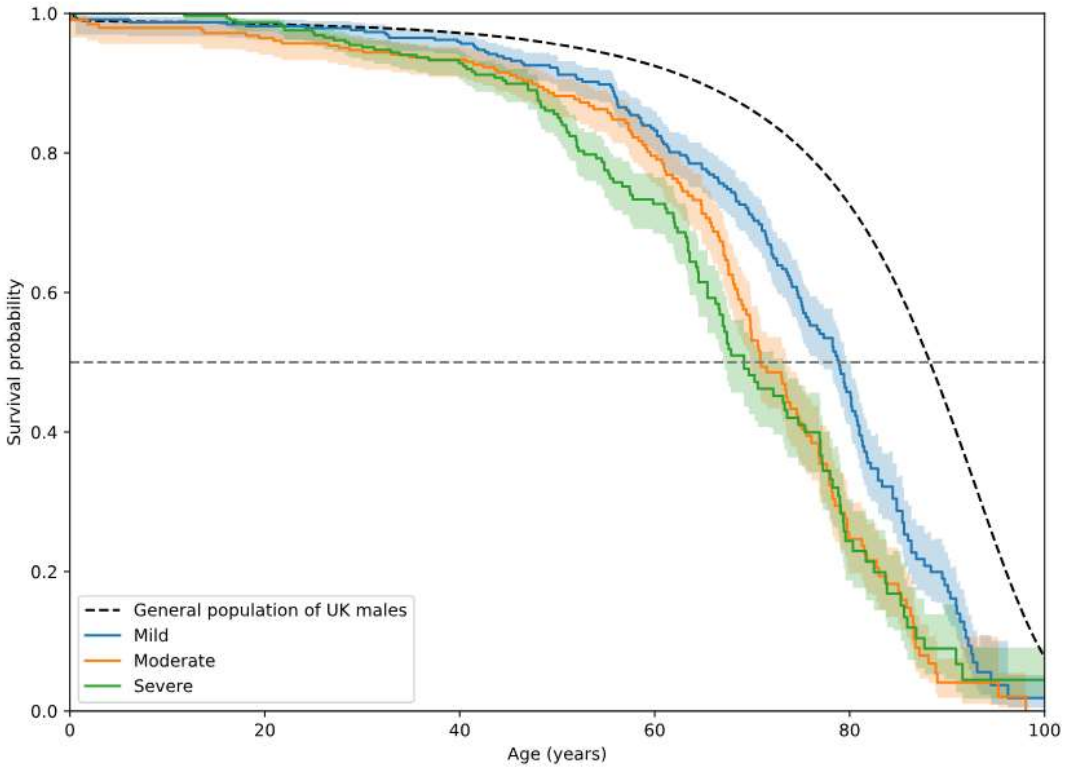
Table 14 New registrations of people with congenital haemophilia B and carriers, 2024/25

Congenital haemophilia B	Number of people by factor IX level (IU/dl)																	
Age (years)	< 1			1 - 5			> 5 & < 40			≥ 40			People with unknown severity			Total		
	Male	Female	Total	Male	Female	Total	Male	Female	Total	Male	Female	Total	Male	Female	Total	Male	Female	Total
0 - 1	10	0	10	6	0	6	1	5	6	0	0	0	2	0	2	19	5	24
2 - 19	1	0	1	2	0	2	6	2	8	1	9	10	0	0	0	10	11	21
20 and above	1	0	1	4	0	4	8	10	18	3	27	30	0	1	1	16	38	54
Total	**12**	**0**	**12**	**12**	**0**	**12**	**15**	**17**	**32**	**4**	**36**	**40**	**2**	**1**	**3**	**45**	**54**	**99**

**Sex as registered at birth*

Table 14: This shows new registrations of haemophilia B broken down by reported severity and age at mid-year. Less severe disease will often present at a later age and the proportion of that group not native to the UK has not been investigated. It is presumed that most of those with severe haemophilia registered at aged two years or above have migrated or are visitors to the UK.

Figure 13 Survival of people with congenital haemophilia B by severity, actively registered 1969-2025



Median survival time (years) for people with haemophilia B: severe: 69, moderate: 70, mild: 78

Figure 13: This shows survival of people with congenital haemophilia B broken down by severity from 1969 to 2025 with 95% confidence intervals. It is interesting that median survival of PwSHB appears greater than that for PwSHA. This probably reflects the milder bleeding phenotype and lower incidence of HIV in haemophilia B compared to haemophilia A.

Figure 14 People with severe congenital haemophilia B by primary treatment, 2015/16 - 2024/25

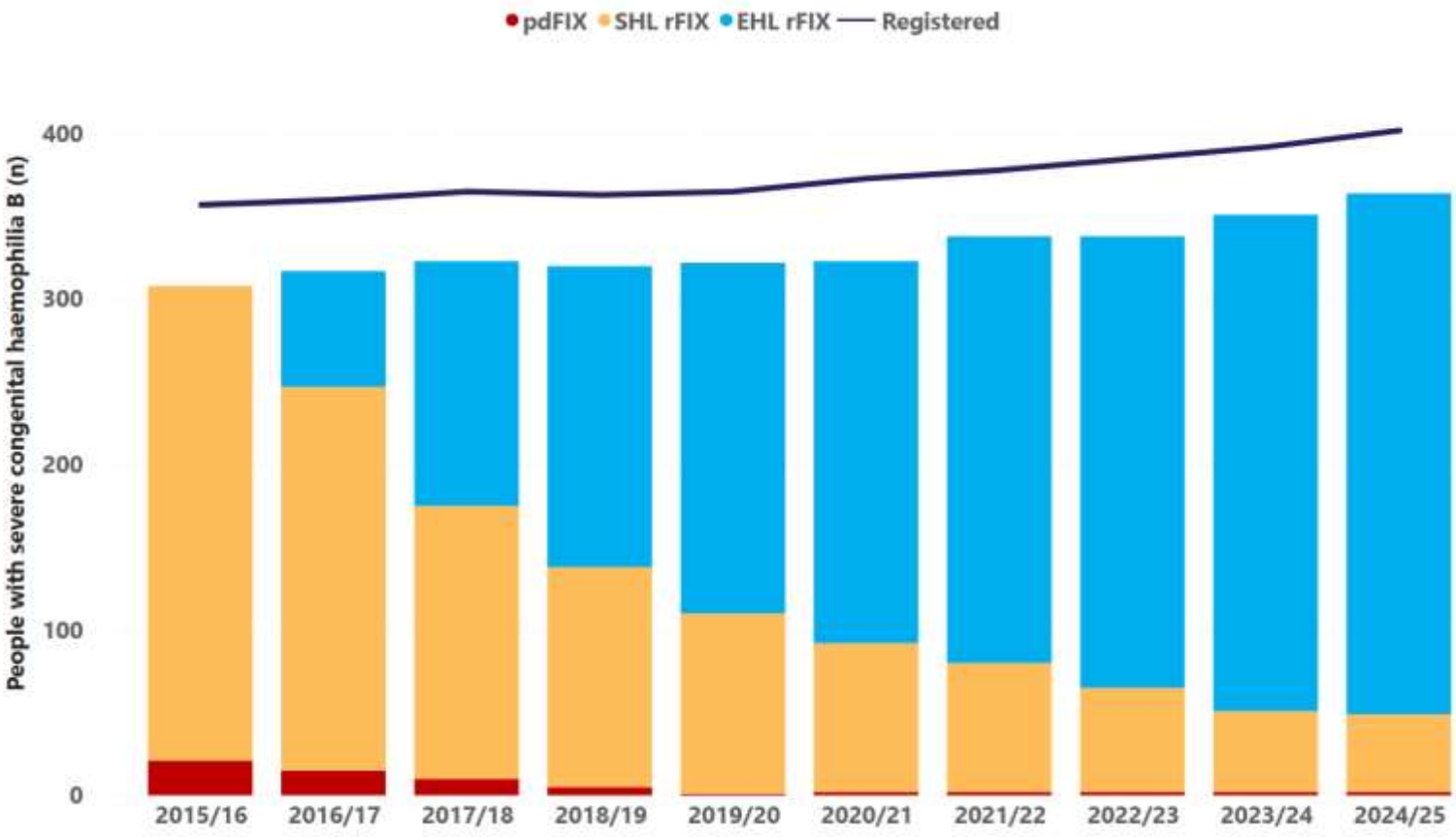


Figure 14: This shows the number of registered PwSHB issued FIX by primary treatment type from 2015/16 to 2024/25. The number of people reported to have been issued FIX shows a gradual, fluctuating increase. This shows that people continue to switch from standard half-life to extended half-life products, and plasma-derived factor IX concentrate is now used by relatively few people with severe haemophilia B. Gene therapy is also reducing the numbers requiring FIX to some degree but is not a marked trend.

Figure 15 People with moderate severity congenital haemophilia B by primary treatment, 2015/16 - 2024/25

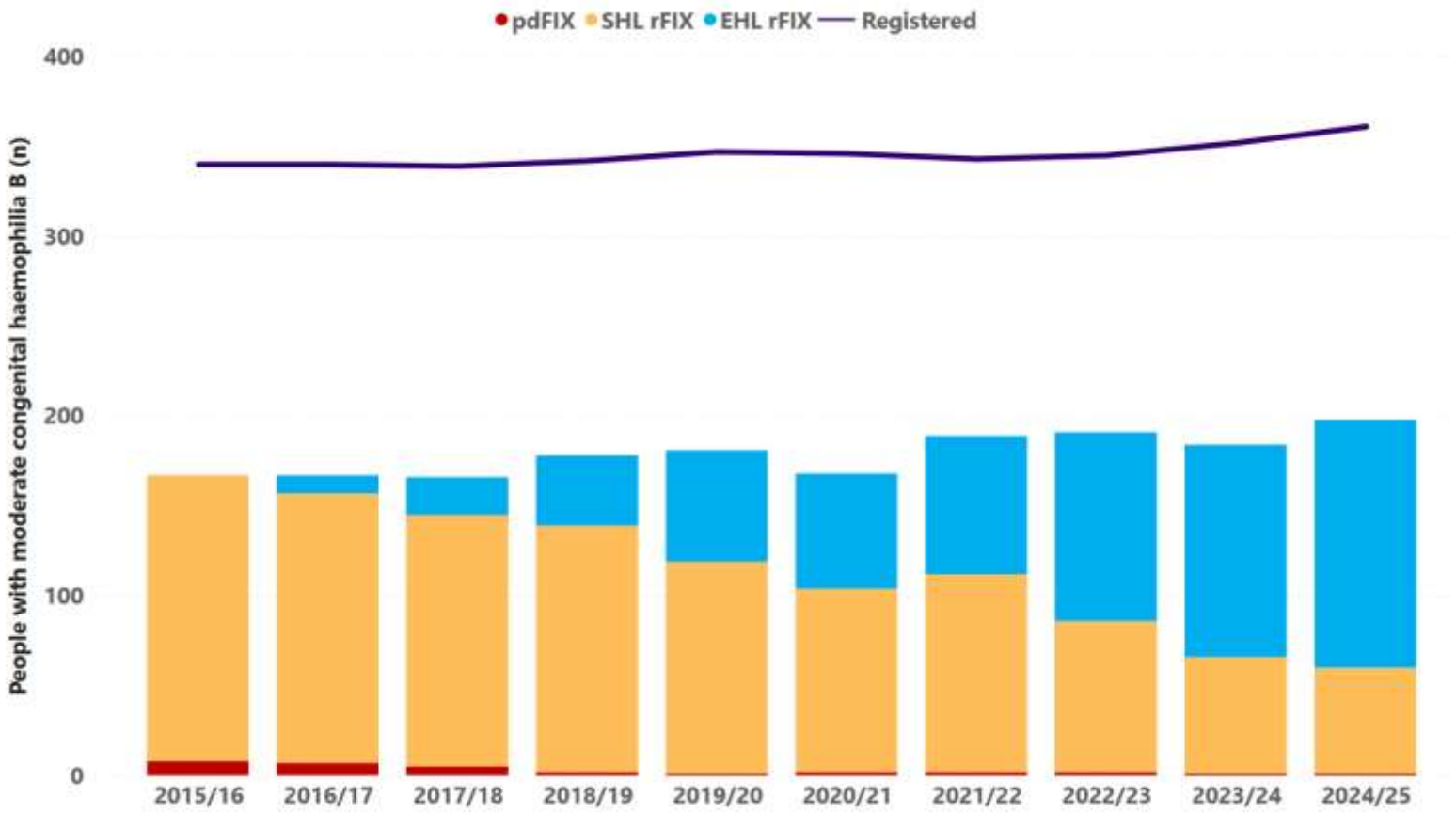


Figure 15: This shows the number of registered people with moderate haemophilia B issued FIX by primary treatment type from 2015/16 to 2024/25. The declining proportion of people issued SHL rFIX, and increasing proportion of people issued EHL rFIX can be observed.

Data table for Figure 14: *People with severe congenital haemophilia B by primary treatment, 2015/16 – 2024/25*

Year	PwSHB issued pdFIX		PwSHB issued SHL rFIX		PwSHB issued EHL rFIX		PwSHB issued any FIX		PwSHB registered
	n	% difference since 2015/16	n	% difference since 2015/16	n	% difference since 2016/17	n	% difference since 2015/16	n
2015/16	21	-	287	-	-	-	309	-	357
2016/17	15	-28.6	232	-19.2	70	-	317	2.6	360
2017/18	10	-52.4	165	-42.5	148	111.4	323	4.5	365
2018/19	5	-76.2	133	-53.7	182	160	320	3.6	363
2019/20	1	-	109	-62	212	202.9	322	4.2	365
2020/21	2	-	90	-68.6	231	230	323	4.5	373
2021/22	2	-	78	-72.8	258	268.6	338	9.4	378
2022/23	2	-	63	-78	273	290	338	9.4	385
2023/24	2	-	49	-82.9	300	328.6	351	13.6	392
2024/25	2	-	47	-83.6	315	350	364	17.8	402

Data table for Figure 15: *People with moderate congenital haemophilia B by primary treatment, 2015/16 – 2024/25*

Year	PwMHB issued pdFIX		PwMHB issued SHL rFIX		PwMHB issued EHL rFIX		PwMHB issued any FIX		PwMHB registered
	n	% difference since 2015/16	n	% difference since 2015/16	n	% difference since 2016/17	n	% difference since 2015/16	n
2015/16	8	-	159	-	-	-	167	-	340
2016/17	7	-12.5	150	-5.7	10	-	167	0	340
2017/18	5	-37.5	140	-11.9	21	110	166	-0.6	339
2018/19	2	-75	137	-13.8	39	290	178	6.6	342
2019/20	1	-87.5	118	-25.8	62	520	181	8.4	347
2020/21	2	-75	102	-35.8	64	540	168	0.6	346
2021/22	2	-75	110	-30.8	77	670	189	13.2	343
2022/23	2	-75	84	-47.2	105	950	191	14.4	345
2023/24	1	-87.5	65	-59.1	118	1080	184	10.2	352
2024/25	1	-87.5	59	-62.9	138	1280	198	18.6	361

Figure 16 Number of people with congenital haemophilia B issued product by severity, 2017/18 - 2024/25

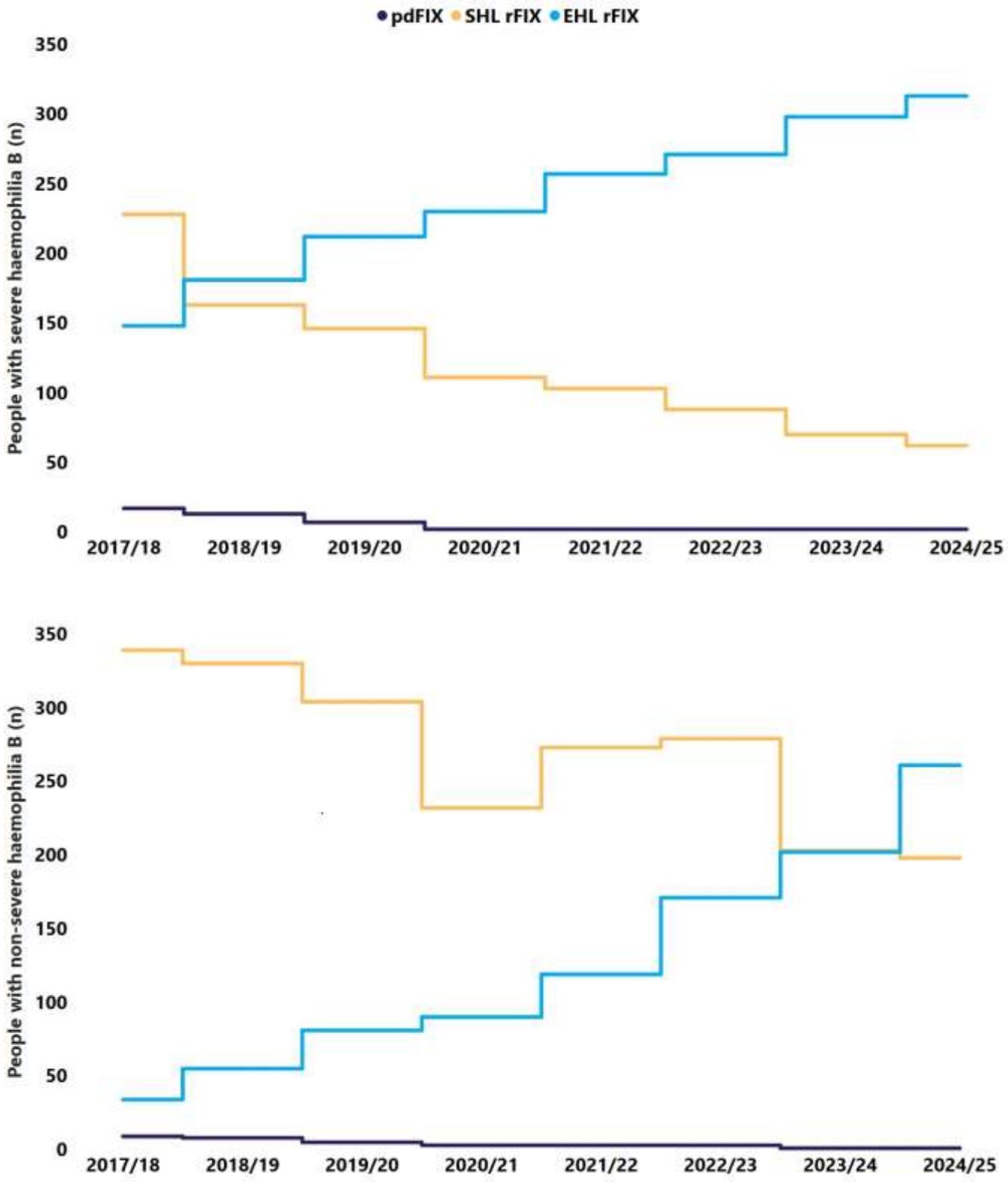


Figure 16 (previous page): This shows a decline in the number of people with severe haemophilia B issued SHL rFIX, probably caused by the increasing use of EHL rFIX. Plasma-derived IX issues have also declined to a very low level and have almost fallen out of use.

Table 15 Non-trial products issued to treat people with congenital haemophilia B (all severities and including inhibitors), 2023/24 - 2024/25

Product Type	Manufacturer	Product	2023/24		2024/25	
			Units (IU)	People (n)	Units (IU)	People (n)
Recombinant	CSL Behring	Idelvion	9,700,508	110	10,753,720	117
	LFB	Cevenfacta	-	-	712	2
	Novo Nordisk	Refixia	9,757,000	94	9,417,500	102
		NovoSeven (mg)	16,563	10	8,564	8
	Pfizer	Benefix	24,158,250	270	23,444,000	258
	SOBI/Biogen	Alprolix	40,667,761	308	45,917,450	368
	Takeda	Rixubis	868,000	3	507,000	2
Plasma-derived	BPL	BPL Replenine	982,000	3	820,000	3
	Takeda	FEIBA	10,000	1	45,000	1

Units in IU unless otherwise stated
Excludes issues of investigational products

Table 15: This gives a breakdown of the products issued to treat people with haemophilia B in the UK in 2023/24 and 2024/25, presented by supplier. Please note these may include people with haemophilia B combined with other blood disorders, e.g. haemophilia A and von Willebrand disease. These figures have been cross-checked against manufacturers' sales figures, where available. Whilst one would not expect a perfect match, there is a high level of correlation. These sales figures are not reported, by agreement with suppliers, for reasons of commercial sensitivity. The use of EHL FIX continues to increase.

The use of investigational FIX has largely ceased due to clinical trials coming to an end. *It is advised that data on trial products be reported to the NHD, anonymising the product, and at a local level with commissioners so that they have a realistic estimate of future product consumption and avoid any inadvertent reduction in future budget.*

Table 16 Non-trial Factor IX treatment issued for congenital and acquired haemophilia B by UK haemophilia centres, 2024/25

Bleeding disorder	Factor IX (IU)				
	People (n)	Plasma-derived	Standard half-life recombinant	Enhanced half-life recombinant	Total
Congenital haemophilia B	793	820,000	23,951,000	66,088,670	90,859,670
Acquired haemophilia B	1	-	-	1,250	1,250
Total	794	820,000	23,951,000	66,089,920	90,860,920

Excludes issues of investigational products

Table 16: This shows FIX issues reported to the NHD in 2024/25, broken down by product type and diagnosis.

Figure 17 Number of people with congenital haemophilia B (all severities) treated in the UK 2024/25 by non-trial products used

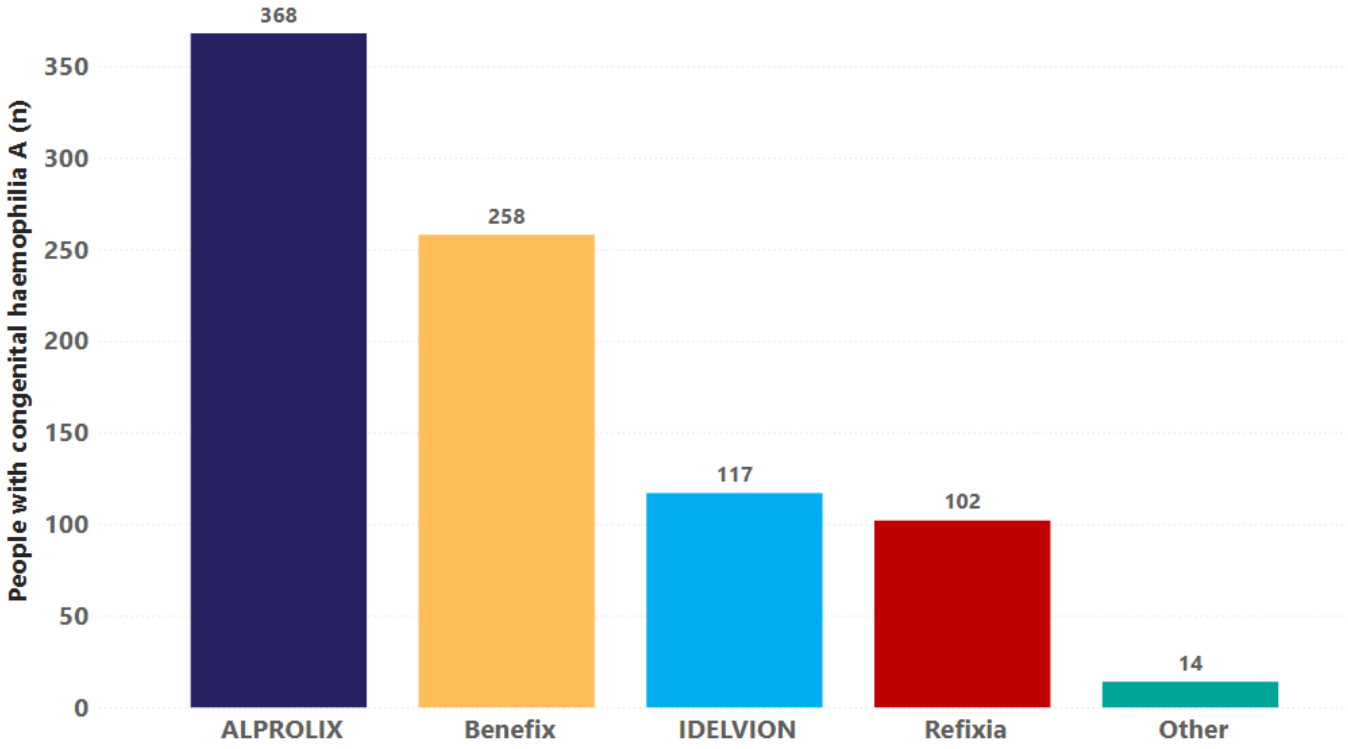


Figure 17: This shows the number of people with haemophilia B treated by product, including those issued to people with an inhibitor. The data that informs this graph can be found in Table 15. People issued multiple products in the year are included in the numbers for each product, resulting in some double counting. There were 801 individual people with haemophilia B issued these products during the year. The large number of people issued Benefix reflects episodic use for non-severe haemophilia B, episodic use for surgery or intercurrent bleeding, and continued use as prophylaxis for a declining proportion of people with severe haemophilia B.

Figure 18 The number and proportion of people with severe congenital haemophilia B and no inhibitor issued EHL products by centre

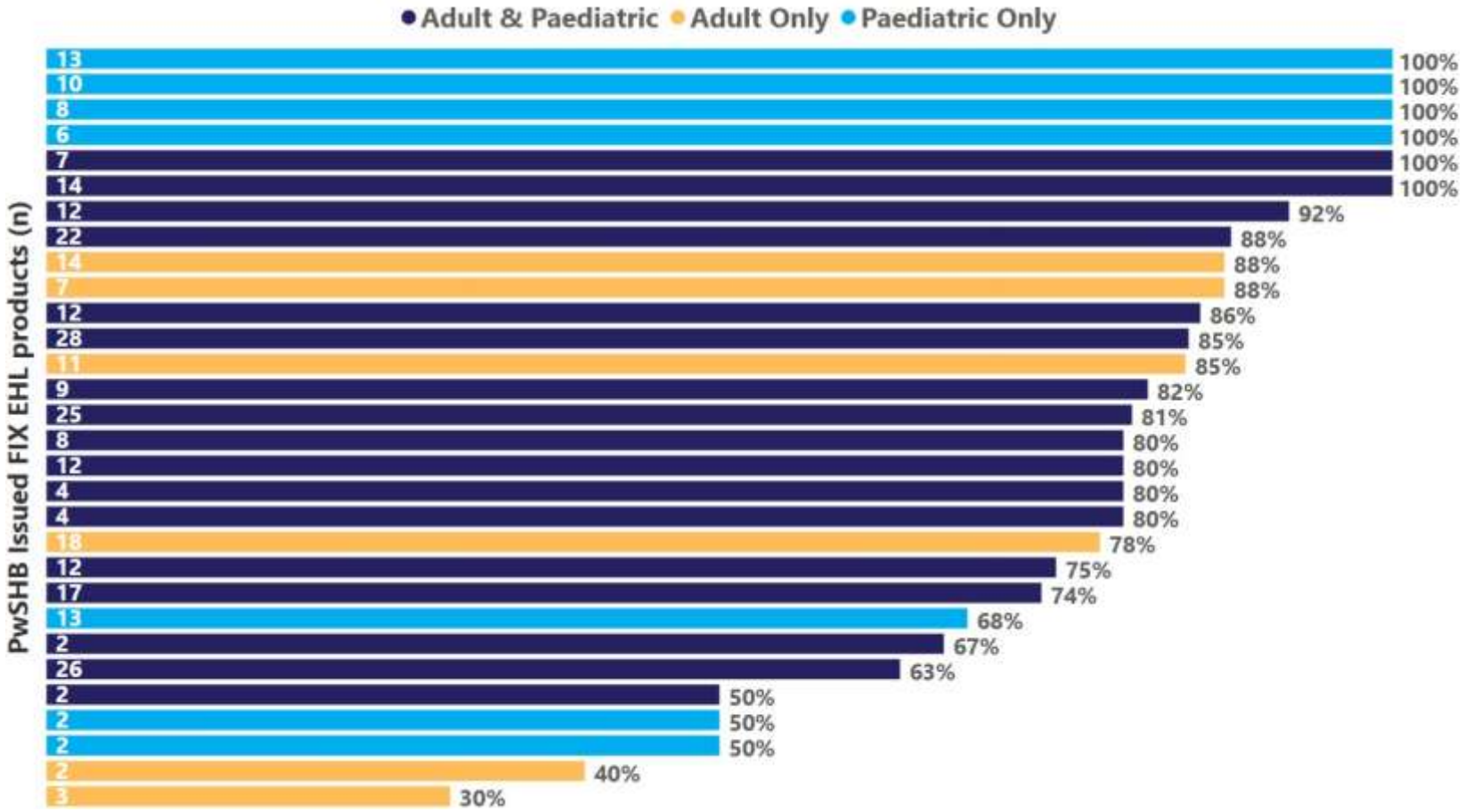


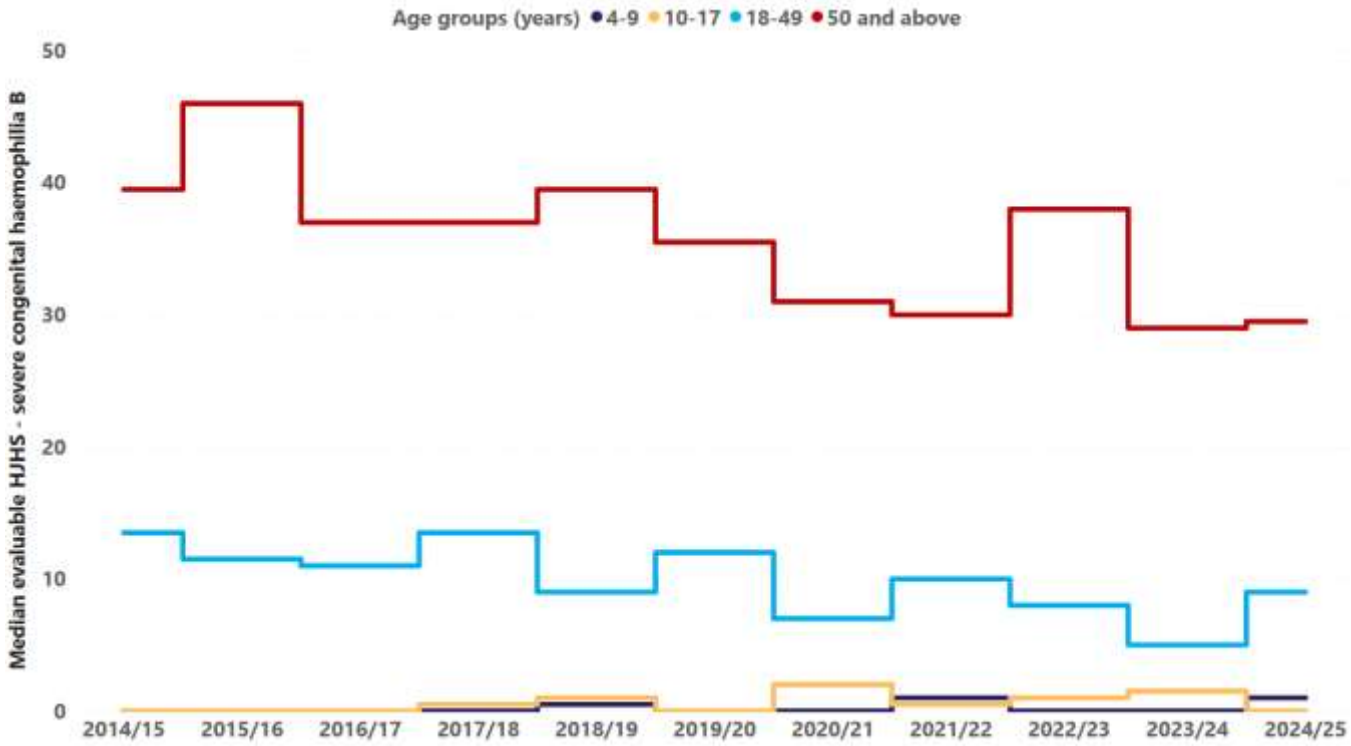
Figure 18: This shows the ranked proportion of people with severe haemophilia B without an inhibitor who were issued EHL products, by centre, in 2024/25. The centres are not identified. The number of people per centre (presented on each bar) is, in many cases, small. Proportional uptake of EHL rFIX concentrates appears to vary amongst centres, although it is, in general, increasing.

Table 17 Haemophilia joint health scores for people with congenital haemophilia B (FIX <=5 IU/dl) without inhibitors 2014/15 - 2024/25

FIX level	Age (years)	Year	Registered, n	HJHS submitted, n (% of registered)	HJHS evaluable, n (% of submitted)	HJHS = 0, n (% of submitted)	Median (iqr) evaluable HJHS
<1 IU/dl	4-9	2014/15	44	24 (54.5)	24 (100.0)	13 (54.2)	0.0 (0.0 -2.0)
		2019/20	45	19 (42.2)	19 (100.0)	13 (68.4)	0.0 (0.0 -1.0)
		2024/25	33	11 (33.3)	11 (100.0)	5 (45.5)	1.0 (0.0 -2.0)
	10-17	2014/15	45	8 (17.8)	8 (100.0)	5 (62.5)	0.0 (0.0 -2.8)
		2019/20	56	25 (44.6)	24 (96.0)	13 (52.0)	0.0 (0.0 -3.4)
		2024/25	67	21 (31.3)	21 (100.0)	11 (52.4)	0.0 (0.0 -3.0)
	18-49	2014/15	157	25 (15.9)	24 (96.0)	4 (16.0)	13.5 (4.3 -23.8)
		2019/20	153	50 (32.7)	49 (98.0)	5 (10.0)	12.0 (3.5 -25.5)
		2024/25	173	38 (22.0)	36 (94.7)	4 (10.5)	9.0 (3.0 -16.8)
	50 and above	2014/15	69	11 (15.9)	10 (90.9)	0 (0.0)	39.5 (26.3 -51.5)
		2019/20	80	17 (21.2)	14 (82.4)	0 (0.0)	35.5 (28.8 -44.8)
		2024/25	89	15 (16.9)	12 (80.0)	0 (0.0)	29.5 (23.5 -41.5)
1-5 IU/dl	4-9	2014/15	28	3 (10.7)	3 (100.0)	3 (100.0)	0.0 (0.0 -0.0)
		2019/20	29	4 (13.8)	4 (100.0)	2 (50.0)	0.5 (0.0 -1.4)
		2024/25	23	6 (26.1)	6 (100.0)	4 (66.7)	0.0 (0.0 -2.0)
	10-17	2014/15	24	5 (20.8)	5 (100.0)	3 (60.0)	0.0 (0.0 -2.0)
		2019/20	29	4 (13.8)	4 (100.0)	4 (100.0)	0.0 (0.0 -0.0)
		2024/25	43	10 (23.3)	10 (100.0)	7 (70.0)	0.0 (0.0 -1.3)
	18-49	2014/15	185	15 (8.1)	15 (100.0)	5 (33.3)	1.0 (0.0 -11.0)
		2019/20	176	23 (13.1)	23 (100.0)	8 (34.8)	2.0 (0.0 -19.0)
		2024/25	163	15 (9.2)	14 (93.3)	4 (26.7)	1.5 (0.0 -13.5)
	50 and above	2014/15	89	6 (6.7)	6 (100.0)	0 (0.0)	9.5 (2.8 -53.3)
		2019/20	98	8 (8.2)	8 (100.0)	0 (0.0)	7.5 (5.0 -23.0)
		2024/25	117	9 (7.7)	9 (100.0)	2 (22.2)	5.0 (1.0 -8.5)

Table 17 and Figure 19 (overleaf): This shows HJHS for PwHB without inhibitors over the last ten years. It is hoped that the low submission of HJHS data for people with severe or moderate haemophilia can be improved. It can be observed above that joint health in PwHB aged 18-49 years has improved over the last ten years.

Figure 19 Median evaluable HJHS of people with congenital haemophilia B (FIX <=5 IU/dl) without inhibitors 2014/15 - 2024/25



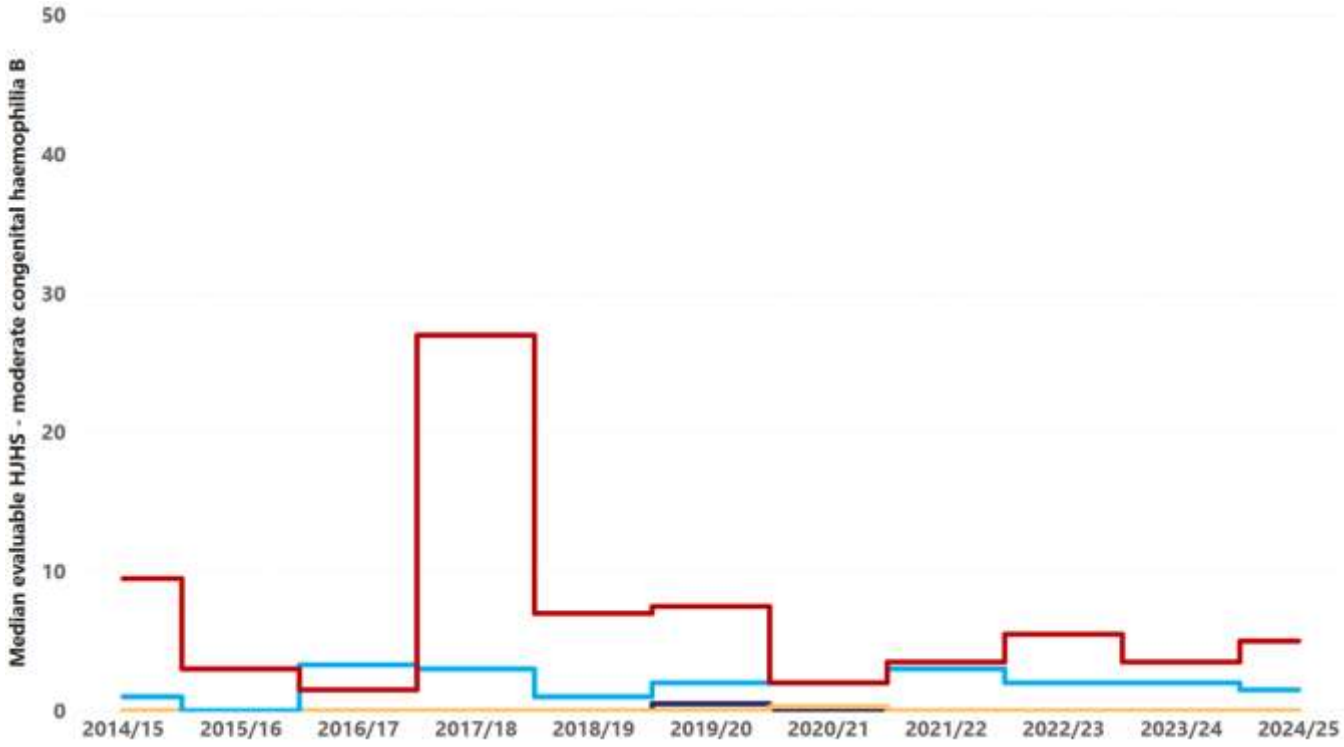


Table 18 Longitudinal haemophilia joint health scores for people with congenital haemophilia B (FIX <=5 IU/dl) without inhibitors 2014/15 - 2024/25

FIX level (IU/dl)	Age (years) 2014/15	Registered 2014/15 (n)	HJHS evaluable 2014/15 (n)	HJHS evaluable 2014/15 and 2024/25	Median (iqr) evaluable HJHS		
					2014/15	2019/20	2024/25
<1	4-17	89	32	12	0 (0-2)	0 (0-1)	0.5 (0-4)
	18 and above	226	34	8	12.5 (0-26.5)	25 (19-30)	23.5 (7.5-26.5)

Table 18: This shows longitudinal within-person HJHS for PwHB with HJHS assessed in 2014/15 and 2024/25. Age groups for SHB have been collapsed, and results for moderate severity haemophilia B not presented, due to the scarcity of follow-up HJHS data for this group, which impedes conclusions regarding the progression of their joint health.

2.3 Von Willebrand Disease

Table 19 People with von Willebrand disease registered and treated 2024/25

Type of von Willebrand disease	VWD Activity IU/dl										Total	Issued concentrate**
	<10	10 - <30	≥30	N/K	Subtotal	<10	10 - <30	≥30	N/K	Subtotal		
	<18 years					≥18 years						
Males												
1	23	244	275	2	**544**	120	508	792	7	**1,427**	**1,971**	108
2A	55	53	13	0	**121**	125	116	51	0	**292**	**413**	94
2B	12	10	5	0	**27**	21	44	26	0	**91**	**118**	26
2M	25	15	5	0	**45**	56	60	15	0	**131**	**176**	27
2N	1	2	3	0	**6**	3	5	37	0	**45**	**51**	9
2 unspecified	14	20	4	0	**38**	37	32	12	0	**81**	**119**	15
3	28	2	1	0	**31**	67	13	3	0	**83**	**114**	89
Low VWF	1	9	101	0	**111**	1	18	196	0	**215**	**326**	6
Other	1	0	1	0	**2**	0	2	2	0	**4**	**6**	1
Unreported	38	90	146	1	**275**	136	299	623	11	**1,069**	**1,344**	113
										Total males	**4,638**	**488**
Females												
1	24	187	195	0	**406**	173	918	2,026	11	**3,128**	**3,534**	169
2A	53	52	16	1	**122**	173	174	79	1	**427**	**549**	127
2B	9	21	5	0	**35**	25	57	46	1	**129**	**164**	47
2M	22	21	4	0	**47**	89	107	39	0	**235**	**282**	44
2N	1	2	11	0	**14**	9	11	93	0	**113**	**127**	14
2 unspecified	15	15	4	0	**34**	40	56	24	0	**120**	**154**	20
3	29	0	1	0	**30**	56	11	1	0	**68**	**98**	69
Low VWF	2	4	105	0	**111**	1	32	554	0	**587**	**698**	10
Other	0	0	1	0	**1**	0	1	8	0	**9**	**10**	0
Unreported	35	80	100	0	**215**	175	583	1,582	16	**2,356**	**2,571**	129
										Total females	**8,187**	**629**
										Total males and females	**12,825**	**1,117**

Sex as registered at birth

***Includes plasma-derived and recombinant products of any factor type*

Table 19 (previous page): This shows the number of people with von Willebrand disease registered and/or issued product, broken down by age, activity level, subtype, and sex at birth. Whilst there is no generally agreed severity classification for VWD, the data are reported by the subdivisions <10, 10-<30 and ≥30% VW activity to give some indication of the distribution of severity amongst the UK cohort.

A VW subtype is reported for approximately 69% of registrations. Efforts are always ongoing to tidy up this part of the database, but there are challenges, including changing classification over time, old data, and changing opinion in relation to the diagnosis of mild type 1 VWD, which may have been over-diagnosed in the past. The HCIS system has been redesigned to make it easier to update the second with the VW subtype. The diagnostic process for VWD is frequently in two stages (basic diagnosis and then subtyping) and the registration may be submitted to the NHD part-way through this process. The registration should be updated when the subtype becomes known. Type 1 defects should be reported at the time of original registration. The new database will send automatic reminders to complete registration of subtypes after initial incomplete registration. Changes in diagnosis should also be registered with the database. It is also going to be possible to capture temporal change in levels.

There remains a relative excess of adult females. For type 1 VWD, this reflects referral bias of women with menorrhagia and possible over-diagnosis of mild type 1 VWD in the past. Some people have been de-registered, or their diagnosis changed when they are re-investigated or when their VW activity level normalises with increasing age.

Table 20 (overleaf)**:** This shows that 580 people with von Willebrand disease were newly registered in the past year, of whom 99 (17%) were registered without a reported subtype.

This table supports previous reports of an apparent relative excess of new female compared to male registrants after menarche, which reflects referral bias.

Table 20 New registrations of von Willebrand disease between 2024/25, by age at mid-year, severity and gender

Type of von Willebrand disease	VWD Activity IU/dl								Total
	<30	≥30	N/K	Subtotal	<30	≥30	N/K	Subtotal	
	<18 years				≥18 years				
Males									
1	34	26	1	**61**	18	11	-	**29**	**90**
2A	14	3	-	**17**	7	1	-	**8**	**25**
2B	2	-	-	**2**	1	3	-	**4**	**6**
2M	7	1	-	**8**	4	1	-	**5**	**13**
2N	-	-	-	**-**	1	4	-	**5**	**5**
2 unspecified	4	2	-	**6**	3	2	-	**5**	**11**
3	4	-	-	**4**	3	-	-	**3**	**7**
Low VWF	-	15	-	**15**	3	23	-	**26**	**41**
Other	1	-	-	**1**	-	-	-	**-**	**1**
Unreported	5	5	-	**10**	12	15	-	**27**	**37**
								Total males	**236**
Females									
1	27	25	-	**52**	37	54	-	**91**	**143**
2A	7	2	1	**10**	11	2	-	**13**	**23**
2B	3	-	-	**3**	1	3	-	**4**	**7**
2M	4	-	-	**4**	3	3	-	**6**	**10**
2N	1	1	-	**2**	-	4	-	**4**	**6**
2 unspecified	4	-	-	**4**	6	1	-	**7**	**11**
3	2	-	-	**2**	-	-	-	**-**	**2**
Low VWF	1	22	-	**23**	-	55	-	**55**	**78**
Other	-	1	-	**1**	-	1	-	**1**	**2**
Unreported	7	8	-	**15**	12	34	1	**47**	**62**
								Total females	**344**
								Total males and females	**580**

Sex as registered at birth

Table 21 Non-trial products issued to treat people with von Willebrand disease (including inhibitors), 2023/24 and 2024/25

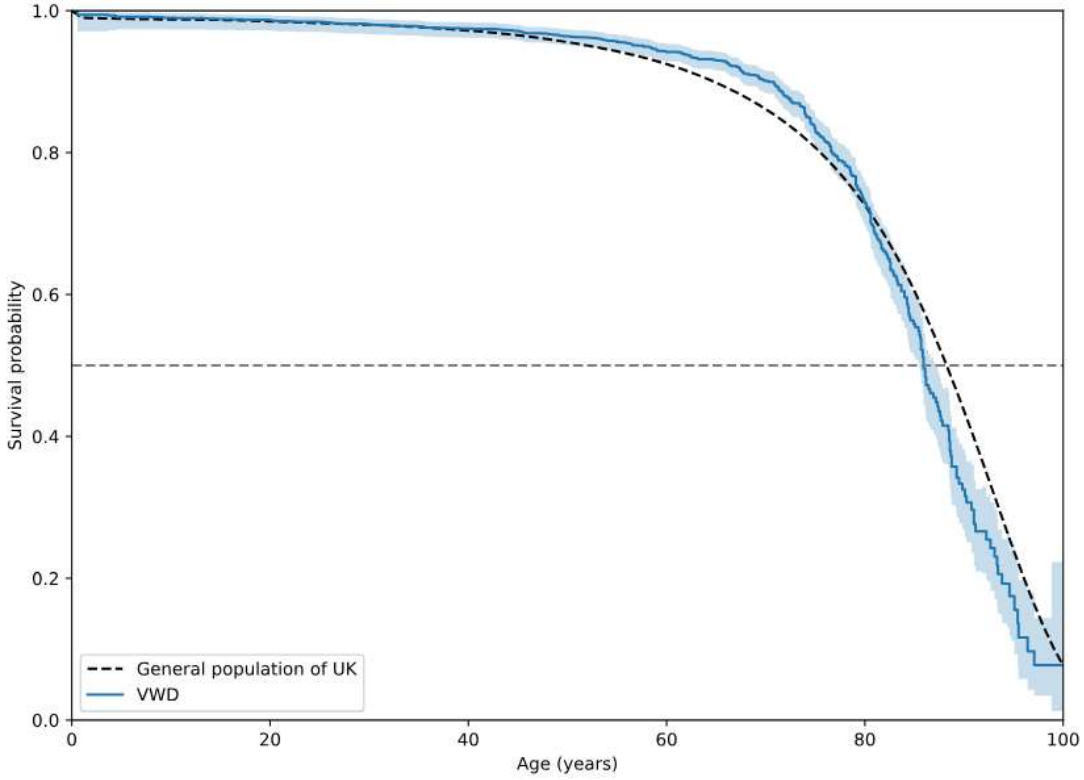
Product Type	Manufacturer	Product	2023/24		2024/25	
			Units (IU)	People (n)	Units (IU)	People (n)
Non-factor	Roche	Hemlibra (mg)	1,680	1	-	-
Recombinant	Novo Nordisk	NovoSeven (mg)	3,394	5	2,005	9
		Esperoct	442,200	5	665,500	7
		NovoEight	10,000	4	10,000	4
	Octapharma	Nuwiq	-	-	500	1
	Pfizer	ReFacto AF	95,000	8	59,000	9
	SOBI/Biogen	Elocta	790,000	6	1,768,250	7
	Takeda	Advate	1,384,250	69	1,717,250	103
		Veyvondi	9,534,003	433	13,576,929	635
Plasma-derived	BPL	BPL FVIII 8Y	8,000	1	-	-
	CSL Behring	Haemate P	2,000	1	-	-
		Voncento - FVIII	21,803,861	500	21,793,004	405
		Voncento - VWF	52,329,266	500	52,303,210	405
	LFB Biomedicaments	Willfact /Wilfactin	2,188,000	16	1,252,000	13
	Octapharma	Wilate - FVIII	7,821,148	191	4,347,500	123
		Wilate - VWF	7,821,148	191	4,347,500	123
		Octaplex	1,500	1	-	-
	Takeda	FEIBA	653,000	4	209,000	2

Units in IU unless otherwise stated
Products containing VWF and FVIII are reported separately in VWF and FVIII units

Table 21: This shows a breakdown of products issued to treat people with von Willebrand disease in the UK by supplier. Please note these exclude products issued to treat any additional blood disorders that people with von Willebrand disease may have. These products are generally listed by and priced by their labelled FVIII content, with the exception of Willfact (LFB) and Veyvondi (Takeda), which are labelled, priced and reported here only by VWF content. Veyvondi is currently licensed for surgery and on-demand use only. Where factor VIII and VW units are shown for Voncento, Alphanate and Willate, the VW units have been derived by converting the factor VIII units issued, using the published FVIII:VWF ratio.

Advate was issued to 156 PwVWD over the last two years, of whom eight also had haemophilia A. Elocta was issued to ten PwVWD over the last two years. Emicuzimab issued to people who also had haemophilia A is not reported here. The emicizumab issued to PwVWD reported in the table is presumed to have followed an individual funding request.

Figure 20 Survival of people with VWD, actively registered 1985-2025



Median survival time (years) for people with Von Willebrand disease is 85

Figure 20: This shows the survival curve (and 95% confidence intervals) for people registered with any type of von Willebrand disease, with a median survival of 85 years. Too few people are registered with subtypes of VWD for survival curves to be constructed for these groups. Consequently, the survival curve will be heavily weighted by people with mild type 1 VWD and milder type 2 phenotypes. People with von Willebrand disease, as a group, appear to have a life expectancy comparable to the general population.

Figure 21 Units of von Willebrand factor issued by UK haemophilia centres to treat von Willebrand disease 2015/16 - 2024/25

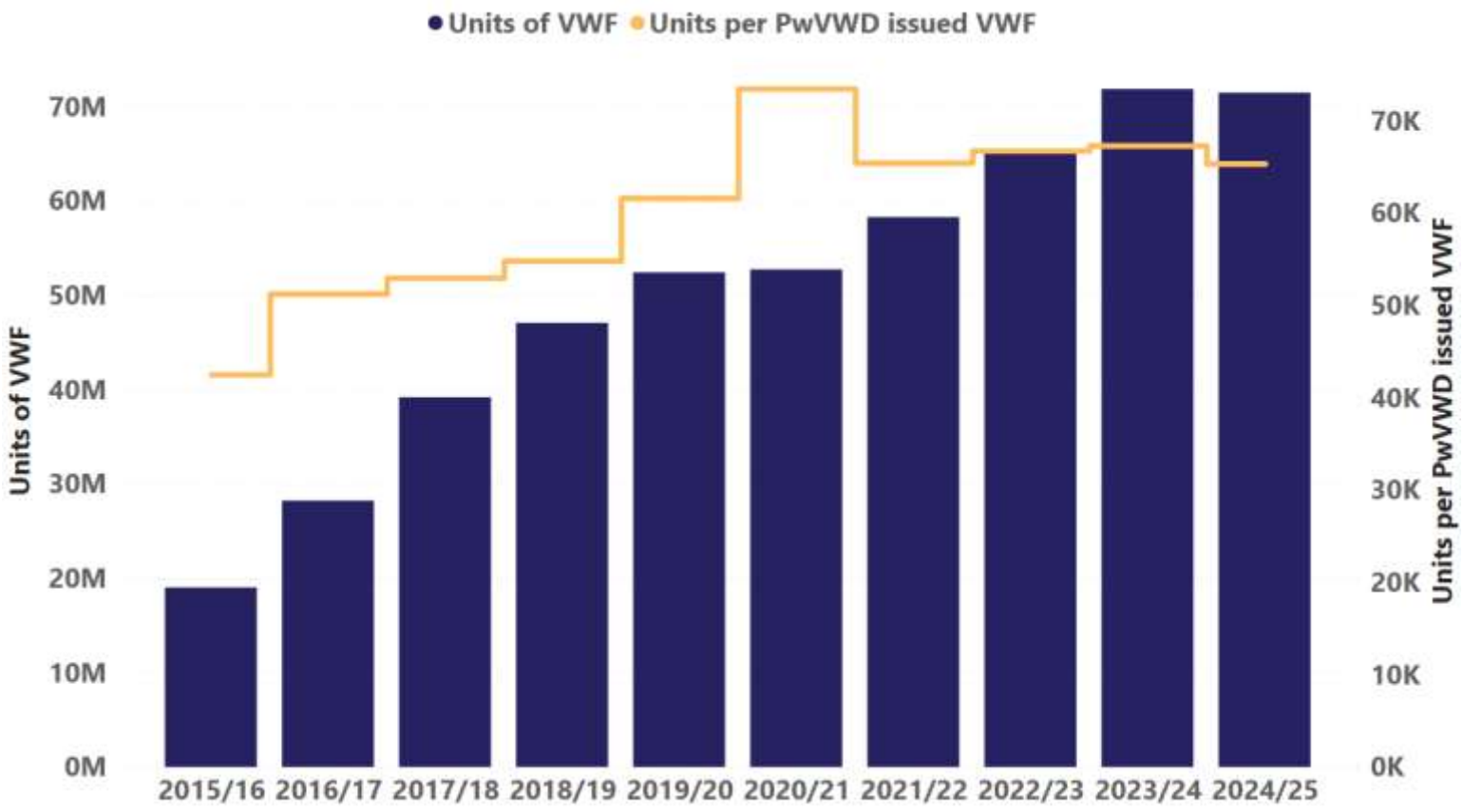


Figure 21: This shows von Willebrand factor units issued to treat von Willebrand disease from 2015/16 to 2024/25, in total and per PwVWD, regardless of type. This shows a steady and marked increase in issues of von Willebrand factor concentrate until 2023/24, probably attributable to increased numbers of PwVWD, increased surgical need and the greater use of prophylaxis in people with type 2 and type 3 VWD. The increased units per PwVWD in 2020/21 is probably attributable to deferral of surgery and other procedures during the COVID-19 pandemic, with issues restricted to intensively treated PwVWD. The number of units used per PwVWD appears relatively stable.

Figure 22 People with von Willebrand disease issued von Willebrand factor by UK haemophilia centres 2015/16 – 2024/25

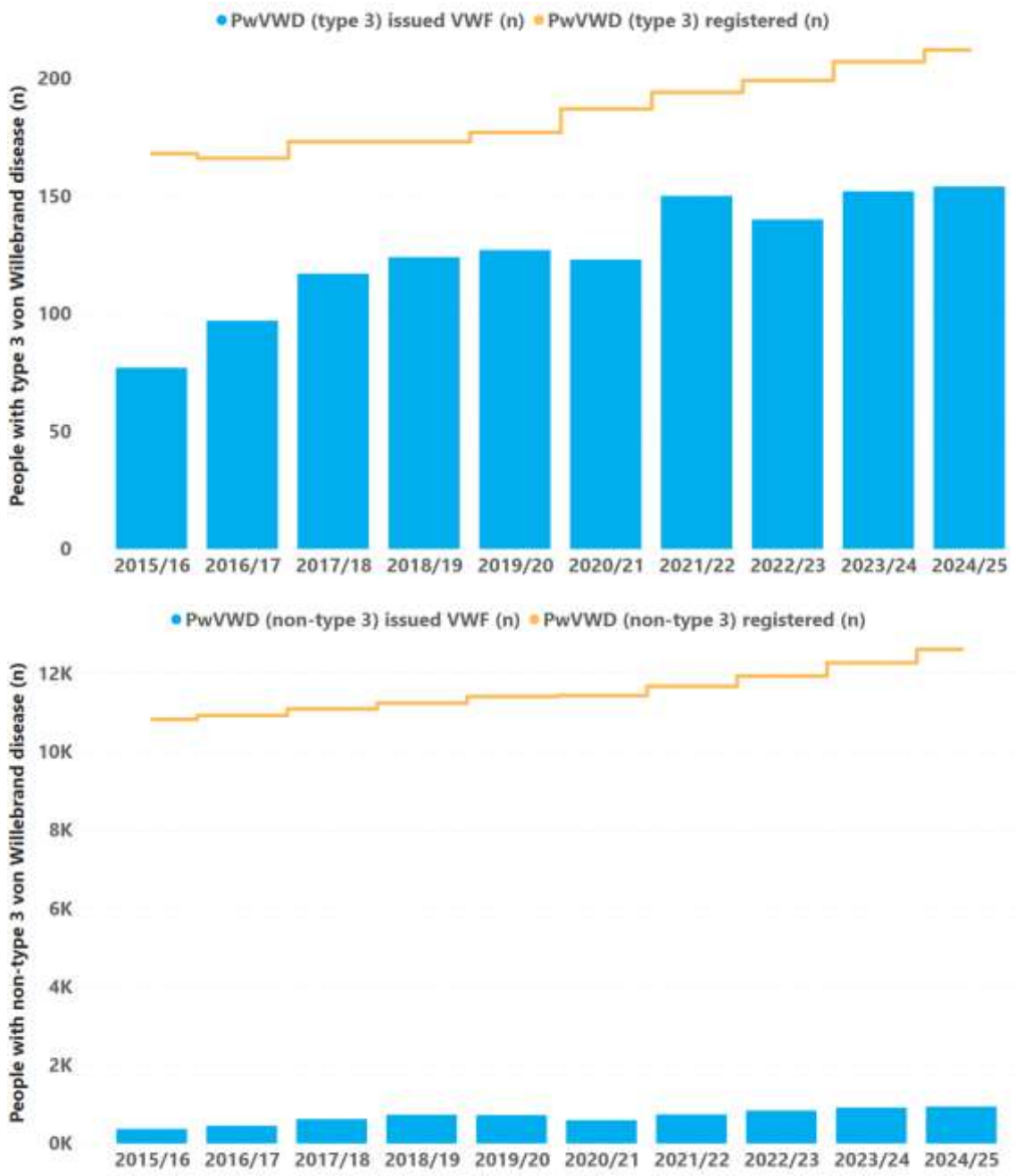


Figure 22: The apparent fall in the number of people issued VWF in 2020/21 is probably attributable to deferral of surgery and other procedures during the COVID-19 pandemic. Approximately 30% of people with type 3 VWD have a mild bleeding phenotype and, as the bar diagram shows, seldom need treatment. There has been a fourfold increase in VW concentrate issues for type 3 von Willebrand's disease over the last ten years. This is only partly attributable to the 100% increase in the number of people with type 3 VWD issued VWF over the same period, and is probably also attributable to increased use of prophylaxis in this group.

2.4 Congenital and Acquired factor VIII, IX and VW Inhibitors

Table 22 Inhibitors by disease severity – congenital haemophilia A, haemophilia B & von Willebrand disease

Diagnosis	Severity (IU/dl) / subtype	Inhibitor status, n (%)					
		Newly Reported	Ongoing	Past	Any inhibitor history	No inhibitor history	In register
Haemophilia A	<1	11 (0.5)	141 (5.9)	402 (16.7)	554 (23)	1,850 (77)	2,404 (100)
	1-5	1 (0.1)	30 (3.8)	45 (5.6)	76 (9.5)	724 (90.5)	800 (100)
	>5, not known or liver transplant	7 (0.1)	21 (0.3)	45 (0.7)	73 (1.1)	6,722 (98.9)	6,795 (100)
	Total	**19 (0.2)**	**192 (1.9)**	**492 (4.9)**	**703 (7.0)**	**9,296 (93)**	**9,999 (100)**
Haemophilia B	<1	1 (0.2)	8 (2.0)	13 (3.2)	22 (5.5)	380 (94.5)	402 (100)
	1-5	1 (0.3)	0 (0.0)	1 (0.3)	2 (0.6)	359 (99.4)	361 (100)
	>5, not known or liver transplant	0 (0.0)	0 (0.0)	1 (0.1)	1 (0.1)	1,473 (99.9)	1,474 (100)
	Total	**2 (0.1)**	**8 (0.4)**	**15 (0.7)**	**25 (1.1)**	**2,212 (98.9)**	**2,237 (100)**
Von Willebrand disease	Type 1	1 (0.0)	0 (0.0)	1 (0.0)	2 (0.0)	5,502 (100)	5,504 (100)
	Type 3	1 (0.5)	8 (3.8)	5 (2.4)	14 (6.6)	198 (93.4)	212 (100)
	Other/ unreported	0 (0.0)	2 (0.0)	1 (0.0)	3 (0.0)	7,106 (100)	7,109 (100)
	Total	**2 (0.0)**	**10 (0.1)**	**7 (0.1)**	**19 (0.1)**	**12,806 (99.9)**	**12,825 (100)**

New: Reported this year and not in previous years
Ongoing: Reported this year and in previous years
Past: Reported in previous years and not in this year
Any inhibitor history: Sum of new, ongoing and past

Table 22 (previous page): This table shows the incidence of new inhibitors in the past year, the history of inhibitors ever registered and the prevalence of those still considered active for haemophilia A, B and von Willebrand disease, broken down by disease severity. There has been a downward trend in the number of reports of new inhibitors in PwSHA over the last few years, from 25 in 2016/17 to eleven in the past year (**Figure 21)**. This is probably a consequence of introduction of emicizumab prophylaxis very early in life, deferring exposure to factor VIII.

Those labelled "new" were reported for the first time in the year 2024/25. Those labelled "ongoing" are reported in previous years and have not been eradicated and remain clinically significant in 2024/25. Those reported as "past" were reported in previous years and not reported to be ongoing in 2024/25 and are therefore assumed to have been tolerised.

An inhibitor is designated as "past" if it is no longer reported in the quarterly returns within 2024/25. There is necessarily some lack of precision surrounding this categorisation, as inhibitors thought to have been eradicated may persist at a variable low level, below the level of detection of the Bethesda assay although high enough to shorten the FVIII half-life. These inhibitors may re-emerge as have up to 25% of inhibitors after tolerisation (International ITI registry data) or after FVIII prophylaxis is replaced with emicizumab. We have received a number of reports of recurring inhibitors after switching to emicizumab. Some of these were clearly ongoing at a low level before emicizumab started. To add to the "softness" of the "inhibitor" designation, it is clear anecdotally that there may be inconsistency in reporting that an inhibitor has been eliminated when, in some cases, the factor VIII half-life remains very short.

The table shows that a history of inhibitor is over twice as prevalent in severe as in moderate haemophilia A and over twenty times more prevalent than in mild haemophilia A. The proportion of people with non-severe haemophilia A thought to have eliminated their inhibitor cannot be known with certainty, however, since some may have an undetectable inhibitor which may reappear as soon as they have FVIII replacement.

Inhibitors in haemophilia B are far less common, with a prevalence of 2.2% of people registered with severe haemophilia B, and <1% for people registered with non-severe haemophilia B. These arise early in the person's treatment and usually only in people with severe haemophilia B caused by FIX gene deletions. Inhibitors in von Willebrand disease appear in our cohort almost exclusively in type 3 VWD. The relatively low numbers of inhibitors in haemophilia B and von Willebrand disease marked as "past" is consistent with the known difficulty in eradicating factor IX inhibitors through immune tolerance.

Figure 23 The incidence of new FVIII inhibitors in people with congenital haemophilia A by year

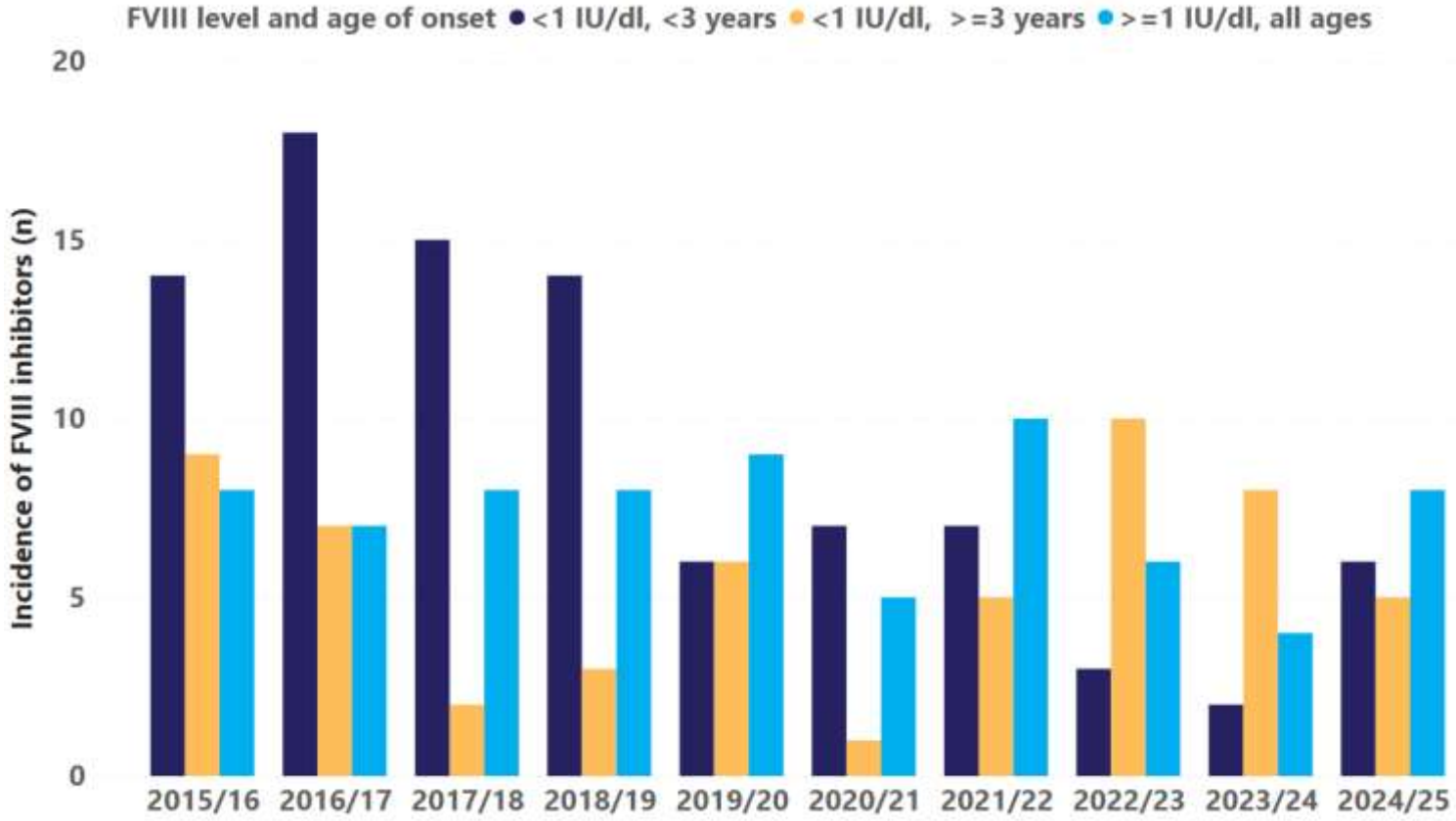


Figure 23: This shows the incidence of FVIII inhibitors from 2015/16 to 2024/25, by baseline FVIII level and age.

Table 23 (overleaf): This shows products reported to have been issued to people with a current inhibitor during 2023/24 and 2024/25, broken down by diagnosis, supplier and product.

Table 23 Non-trial products issued to treat people with congenital bleeding disorders reported to have a current inhibitor, 2023/24 and 2024/25

Product type	Manufacturer	Product	2023/24		2024/25	
			People (n)	Units (IU)	People (n)	Units (IU)
Haemophilia A						
Non-factor	Roche	Emicizumab (mg)	159	681,840	170	687,857
Recombinant	LFB	Cevenfacta	2	101	3	369
	Novo Nordisk	NovoSeven (mg)	105	10,757	93	8,335
		Esperoct	12	2,035,000	12	1,525,000
		NovoEight	4	1,720,000	2	47,000
	Octapharma	Nuwiq	8	1,628,000	7	1,502,500
	Pfizer	ReFacto AF	7	736,500	7	198,500
	SOBI/Biogen	Elocta	34	5,753,750	21	1,701,500
	Takeda	Adynovi	1	692,000	-	-
		Advate	24	3,051,750	19	2,366,750
		Obizur	-	-	1	98,000
Plasma-derived	BPL	BPL FVIII 8Y	1	149,120	-	-
	Octapharma	Octanate	2	1,020,000	1	10,000
	Takeda	FEIBA	9	1,222,000	14	1,762,000
Haemophilia B						
Recombinant	LFB	Cevenfacta	-	-	2	712
	Novo Nordisk	Refixia	-	-	1	50,000
		NovoSeven (mg)	9	14,763	7	8,554
	SOBI/Biogen	Alprolix	2	1,033,500	2	1,122,250
Plasma-derived	Takeda	FEIBA	1	10,000	1	45,000
von Willebrand disease						
Non-factor	Roche	Emicizumab (mg)	1	1,680	-	-
Recombinant	Novo Nordisk	NovoSeven (mg)	4	841	6	1,629
	SOBI/Biogen	Elocta	1	372,000	2	1,042,000
	Takeda	Advate	-	-	1	4,000
		Veyvondi	2	722,150	3	1,173,250
Plasma-derived	CSL Behring	Voncento	4	876,500	4	543,000
	Takeda	FEIBA	3	580,000	-	-
Bleeding disorder of unknown cause						
Plasma-derived	Takeda	FEIBA	1	8,000	-	-
Factor VII deficiency						
Recombinant	Novo Nordisk	NovoSeven (mg)	3	2,772	3	3,114
Plasma-derived	Takeda	FEIBA	1	130,000	1	172,000
Factor XI deficiency						
Recombinant	Novo Nordisk	NovoSeven (mg)	-	-	1	187
Plasma-derived	Takeda	FEIBA	-	-	1	270,000
Miscellaneous						
Plasma-derived	Takeda	FEIBA	-	-	1	154,000
Combined haemophilia A and von Willebrand disease						
Non-factor	Roche	Emicizumab (mg)	1	4,320	1	4,320
Recombinant	Novo Nordisk	NovoSeven (mg)	-	-	1	11
Plasma-derived	Takeda	FEIBA	1	73,000	1	207,000

Units in IU unless otherwise stated
Excludes issues of investigational products

Table 24 Non-trial products issued to treat people with acquired disorders, 2023/24 and 2024/25

Product type	Manufacturer	Product	2023/24		2024/25	
			People (n)	Units (IU)	People (n)	Units (IU)
Acquired haemophilia A						
Non-factor	Roche	Emicizumab (mg)	5	7,726	7	18,480
Recombinant	Novo Nordisk	NovoSeven (mg)	14	5,052	21	2,809
	Pfizer	ReFacto AF	1	8,000	1	6,000
	SOBI/Biogen	Elocta	1	2,000	2	79,000
	Takeda	Advate	3	591,000	1	5,000
		Obizur	36	1,881,505	49	4,008,500
Plasma-derived	Baxter	Com FVII	3	218	1	42
	CSL Behring	Voncento	-	-	1	5,000
	Octapharma	Fibryga	-	-	2	6
	Takeda	FEIBA	98	8,404,500	93	8,382,400
Acquired von Willebrand disease						
Recombinant	Novo Nordisk	NovoSeven (mg)	1	144	-	-
	SOBI/Biogen	Elocta	1	27,900	1	2,000
	Takeda	Advate	6	28,250	5	27,000
		Veyvondi	14	361,400	21	1,106,300
Plasma-derived	CSL Behring	Voncento	31	654,900	24	563,500
	LFB Biomedicaments	Willfact /Wilfactin	1	471,000	1	445,000
	Octapharma	Wilate	9	552,500	8	59,500
Other acquired factor deficiency						
Recombinant	Novo Nordisk	NovoSeven (mg)	1	120	-	-
	Pfizer	Benefix	1	251,000	-	-
	Takeda	Advate	1	2,000	-	-
Plasma-derived	-	Plasma	1	7,500	-	-
	BPL	BPL FXI	-	-	1	1,225
		Coagadex	2	26,000	-	-
	CSL Behring	Beriplex	1	1,500	1	2,500
		Fibrogammin P	1	21,750	-	-
	Octapharma	Octaplas	1	20,400	-	-
		Octaplex	2	24,000	1	2,500
	Takeda	FEIBA	1	12,000	3	277,000

Units in IU unless otherwise stated
Excludes issues of investigational products

Table 24: This shows products issued for people with an acquired inhibitors in 2024/25, broken down by diagnosis and supplier.

Table 25 FEIBA® issues: breakdown by diagnosis, 2022/23 - 2024/25

Diagnosis	2022/23		2023/24		2024/25	
	People (n)	Units (IU)	People (n)	Units (IU)	People (n)	Units (IU)
Haemophilia A	20	1,736,000	16	2,428,000	18	2,224,000
Haemophilia B	3	471,000	1	10,000	1	45,000
von Willebrand disease	3	290,000	3	580,000	1	2,000
Factor VII deficiency	1	500	1	130,000	1	172,000
Factor X deficiency	-	-	1	56,000	-	-
Factor XI deficiency	-	-	-	-	1	270,000
Bleeding disorder of unknown cause	1	10,000	1	8,000	-	-
Acquired haemophilia A	83	6,783,900	98	8,384,500	93	8,382,400
Other acquired factor deficiency	-	-	1	12,000	3	277,000
Combined haemophilia A and von Willebrand disease	1	81,000	1	73,000	1	207,000
Miscellaneous	1	9,000	-	-	1	154,000
Total	**113**	**9,381,400**	**123**	**11,681,500**	**120**	**11,733,400**

**This is the total excluding numbers which have been suppressed.* |

Table 26 NovoSeven® issues: breakdown by diagnosis, 2022/23 - 2024/25

Diagnosis	2022/23		2023/24		2024/25	
	People (n)	Units (mg)	People (n)	Units (mg)	People (n)	Units (mg)
Haemophilia A	116	7,762	124	12,752	116	10,119
Haemophilia B	12	13,792	10	16,563	8	8,564
von Willebrand disease	7	1,622	5	3,394	8	1,994
Factor V deficiency	3	81	3	102	2	529
Factor VII deficiency	80	9,042	77	7,894	84	6,712
Factor XI deficiency	2	26	-	-	2	195
Factor XIII deficiency	1	2	-	-	-	-
Bleeding disorder of unknown cause	1	8	2	16	-	-
Glanzmann's thrombasthenia	50	28,570	52	6,233	54	7,096
Bernard-Soulier syndrome	8	296	17	987	11	1,178
Other severe platelet disorder	-	-	1	21	-	-
Heritable platelet disorder	4	39	5	2,015	13	409
Other platelet disorder	2	6	-	-	1	5
Acquired haemophilia A	14	3,076	14	5,052	21	2,809
Acquired von Willebrand disease	-	-	1	144	-	-
Other acquired factor deficiency	-	-	1	120	-	-
Combined haemophilia A and von Willebrand disease	-	-	-	-	1	11
Combined factors VII and X deficiencies	1	1	-	-	-	-
Multiple diagnoses	1	2	1	1	2	200
Miscellaneous	1	9	5	54	3	3
Total	**303**	**64,334**	**318**	**55,348**	**326**	**39,824**

**This is the total excluding numbers which have been suppressed.*

Table 27 Emicizumab issues: breakdown by diagnosis, 2022/23 - 2024/25

Diagnosis	2022/23		2023/24		2024/25	
	People (n)	Units (mg)	People (n)	Units (mg)	People (n)	Units (mg)
Haemophilia A	1,250	5,114,478	1,417	5,997,713	1,526	6,527,950
von Willebrand disease	1	5,145	1	1,680	-	-
Combined factors V and VIII deficiencies	1	30	-	-	-	-
Acquired haemophilia A	1	5,760	5	7,726	7	18,480
Combined haemophilia A and von Willebrand disease	3	8,790	3	7,290	2	7,200
Multiple diagnoses	-	-	-	-	2	7,560
Total	**1,256**	**5,134,203**	**1,426**	**6,014,409**	**1,537**	**6,561,190**

**This is the total excluding numbers which have been suppressed.*

Tables 25, 26 (previous page) **& 27** show in greater detail how much FEIBA, NovoSeven and emicizumab were issued for each diagnosis over the last three years. People with any hereditary or acquired bleeding disorder, either with or without inhibitors, are included. Some off-license issues of emicizumab to isolated people with von Willebrand disease and acquired haemophilia A are reported, although there is no estimate of off-label issues, or issues for reversal of over-anticoagulation, as this occurs outside haemophilia centres and is consequently not systematically collected. The rapid decline in the number of PwHA with inhibitors issued FEIBA following the introduction of emicizumab appears to have eased, and the number of units issued has apparently stabilised. NovoSeven issues for congenital haemophilia A have fluctuated over the last three years. It is now used in preference to FEIBA for surgery in people with FVIII inhibitors who are co-prescribed emicizumab. Issues of emicuzimab for von Willebrand disease, acquired haemophilia A and combined factors V and VIII deficiencies are presumed to be related to individual funding requests.

Figure 24 The proportion of people with congenital haemophilia A and an inhibitor issued treatment products by year

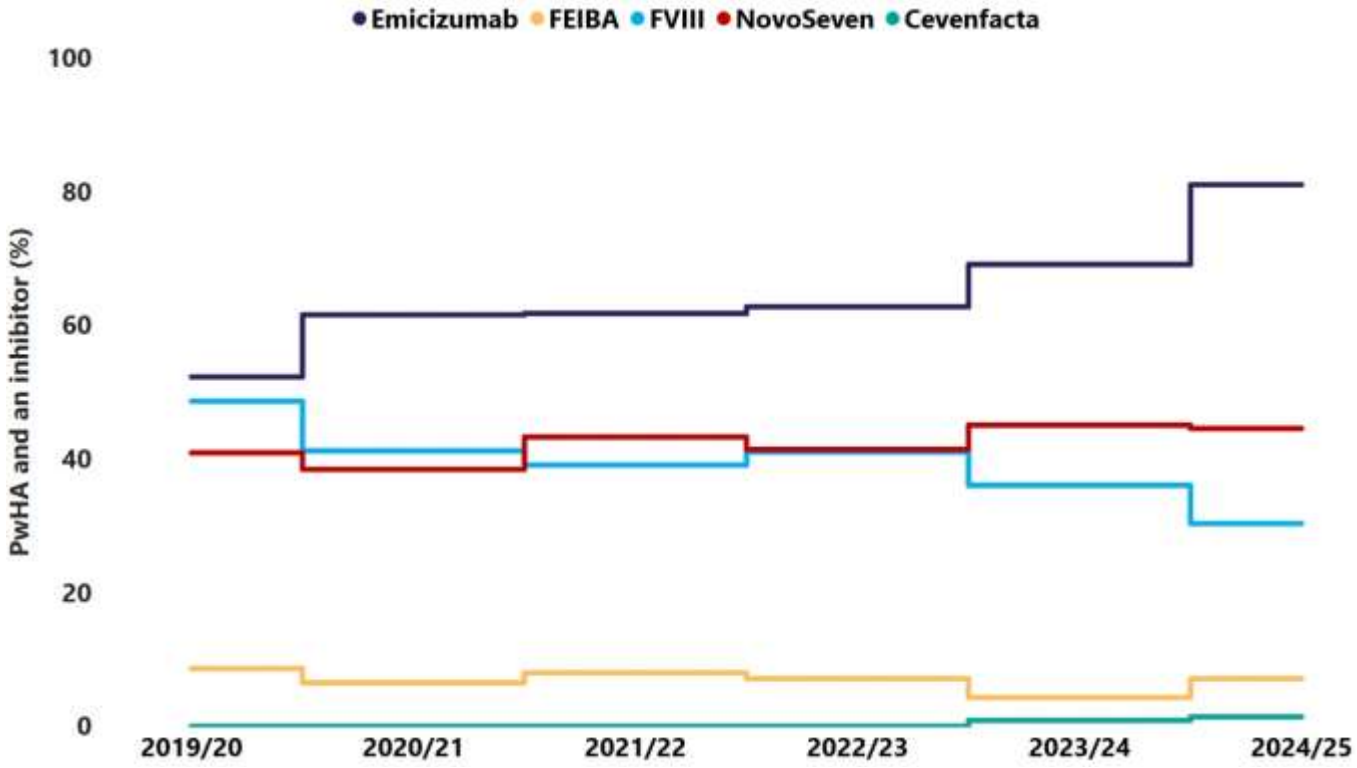


Figure 24: This shows the change in the proportion of PwHA and an inhibitor issued with FEIBA, NovoSeven, FVIII, emicizumab and cevenfacta since emicizumab was licensed for use in this group. People may be issued with more than one product during a given quarter. Novoseven, in particular, is often issued to people treated with other products. The introduction of emicizumab has led to a marked reduction in the use of FVIII and FEIBA in this group, although not in the use of NovoSeven, which may be used for surgery and intercurrent bleeding in preference to FEIBA for people prescribed emicizumab prophylaxis. The increase in the proportion of PwHA with inhibitors issued emicizumab appears to have slowed down.

Figure 25 The proportion of people with acquired haemophilia A issued treatment products by year

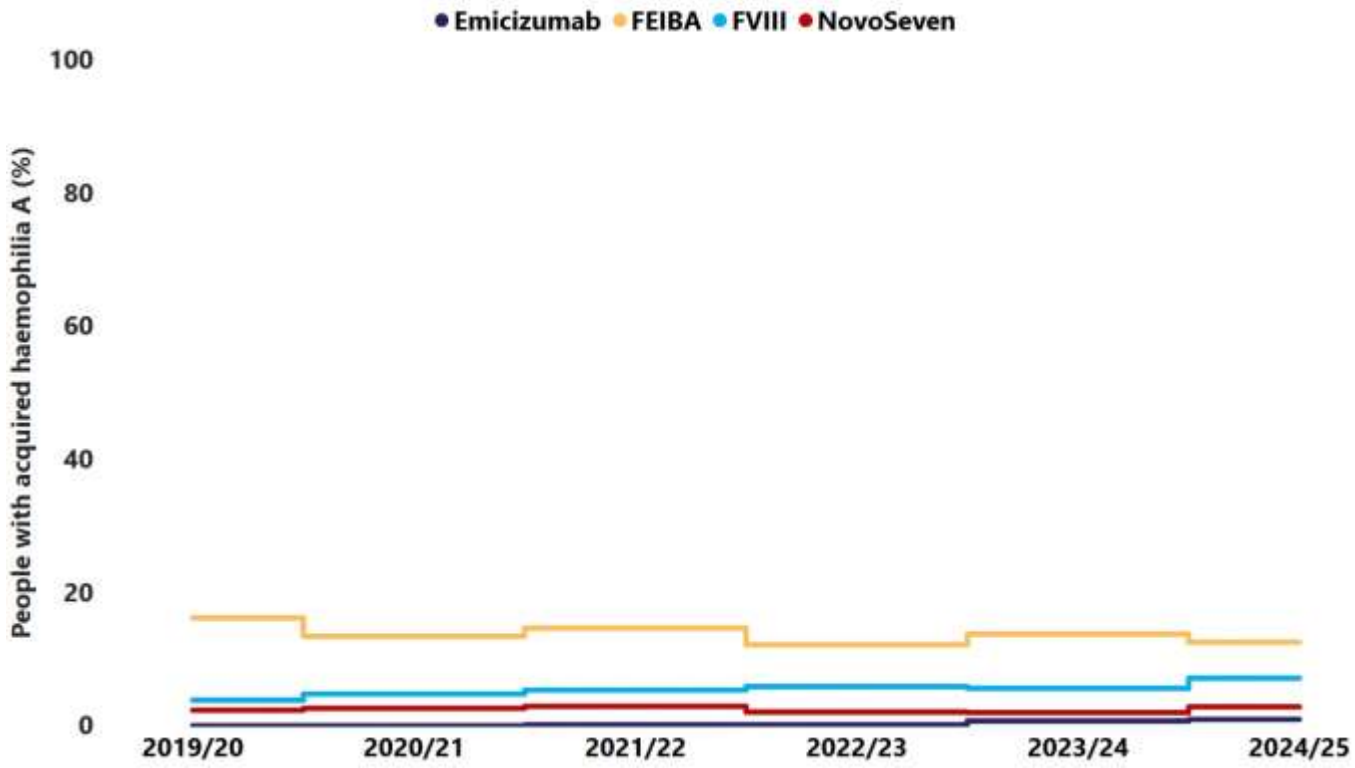


Figure 25: This shows the change in the proportion of people with acquired haemophilia A issued with FEIBA, NovoSeven, FVIII, emicizumab and cevenfacta since 2019/20.

Figure 26 The number and proportion of people with severe haemophilia A and an inhibitor issued with emicizumab by centre 2024/25

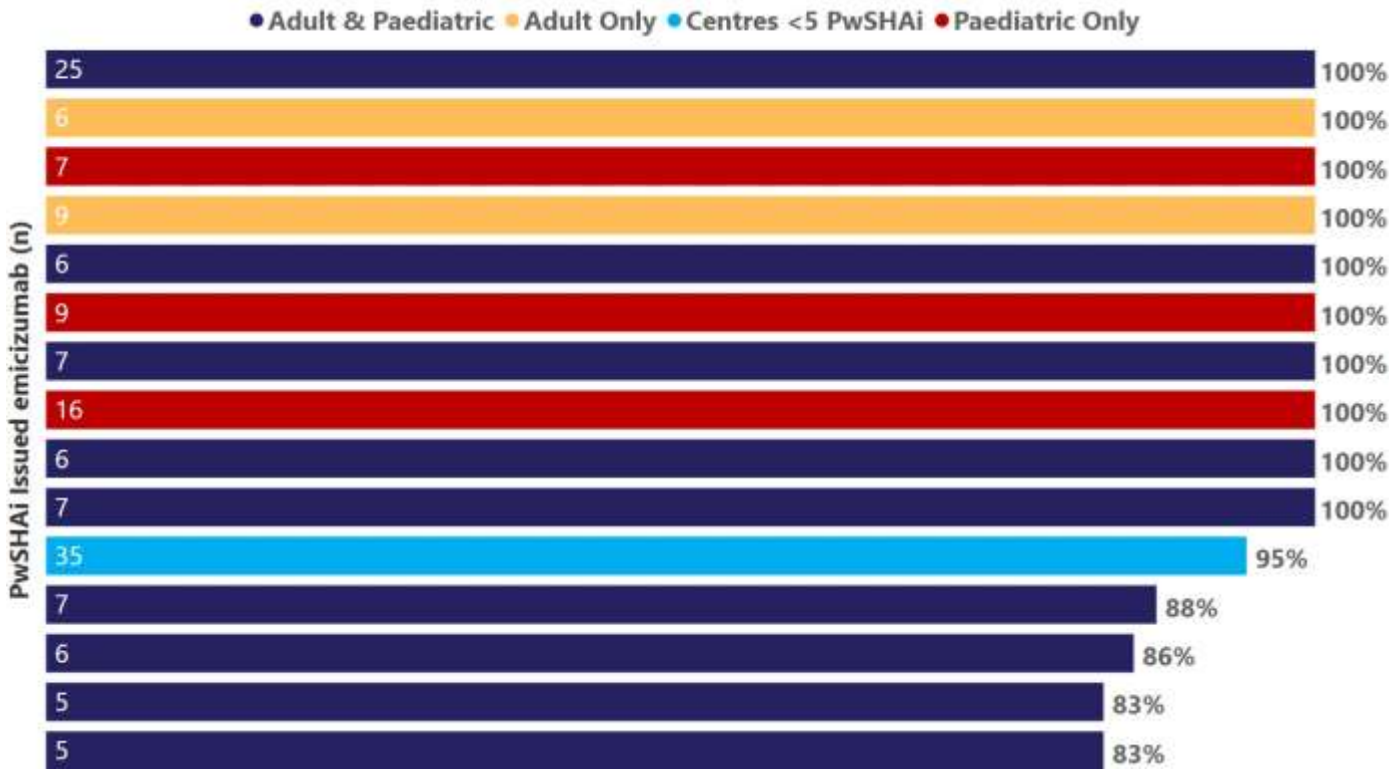


Figure 26: This bar diagram shows the proportion of people with severe haemophilia A and inhibitors issued with emicizumab, broken down by centre. Centres with five or fewer eligible people are aggregated and shown as the red bar. This shows that most centres issue emicizumab to all their PwSHAi (median 100%, IQR 88-100%).

2.5 Other Bleeding Disorders

Table 28 People with other bleeding disorders registered and issued treatment products 2024/25

Diagnosis	People in Register		Treatment product issues	
	Males	Females	Any product	Concentrate
Factor V deficiency	121	230	5	2
Factor VII deficiency	1,227	1,452	88	88
Factor X deficiency	182	243	44	44
Factor XI deficiency	1,915	2,855	48	47
Factor XIII deficiency	60	52	73	73
Prothrombin (factor II) deficiency	11	17	5	5
Dysfibrinogenemia	360	578	33	33
Hypofibrinogenemia	146	208	15	15
Hypodysfibrinogenemia	28	30	3	3
Afibrinogenemia	11	7	15	15
Bleeding disorder of unknown cause	226	1,655	2	2
Glanzmann's thrombasthenia	74	98	65	64
Bernard-Soulier syndrome	64	75	14	13
Platelet-type pseudo von Willebrand disease	16	30	0	0
Other platelet disorder	1,338	2,926	6	4
Combined factors V and VIII deficiencies	16	20	8	8
Other disorders	3	5	0	0
Acquired haemophilia A	375	356	128	68
Acquired von Willebrand disease	126	101	49	49
Other acquired factor deficiency	28	26	6	3
Miscellaneous/multiple disorders	153	457	15	13
Total	**6,443**	**11,377**	**622**	**549**

Table 28: This shows the number of people with blood disorders other than haemophilia and VWD registered and issued products during the year.

Table 29 People with selected other bleeding disorders registered and treated 2024/25, by factor level

Diagnosis	People in register by factor level (IU/dl)							
	<5		≥5		Not known		Total	
	Registered	Issued treatment products	Registered	Issued treatment products	Registered	Issued treatment products	Registered	Issued treatment products
Factor V deficiency	64	5	277	0	10	0	351	5
Factor VII deficiency	183	44	2,460	44	36	0	2,679	88
Factor X deficiency	49	36	356	8	20	0	425	44
Factor XI deficiency	336	19	4,311	23	123	6	4,770	48
Total	**632**	**104**	**7,404**	**75**	**186**	**6**	**8,171**	**185**

Please note desmopressin issues are excluded

Table 29: It is acknowledged that these other disorders have no recognised classification of disease severity. However, the table above gives an idea of the range of registered levels.

Table 30 New registrations of other bleeding disorders 2024/25, by coagulation defect and gender

Diagnosis	Male	Female	Total
Factor V deficiency	6	23	29
Factor VII deficiency	113	126	239
Factor X deficiency	14	12	26
Factor XI deficiency	117	185	302
Factor XIII deficiency	4	6	10
Prothrombin (factor II) deficiency	1	1	2
Fibrinogen disorder	51	67	118
Bleeding disorder of unknown cause	38	348	386
Glanzmann's thrombasthenia	5	4	9
Bernard-Soulier syndrome	5	9	14
Platelet-type pseudo von Willebrand disease	0	3	3
Other platelet disorder	124	241	365
Combined factors V and VIII deficiencies	0	2	2
Acquired haemophilia A	83	44	127
Acquired von Willebrand disease	17	16	33
Other acquired factor deficiency	5	5	10
Miscellaneous/multiple disorders	12	35	47
Total	594	1,123	1,717

Table 30: This table shows new registrations of bleeding disorders other than haemophilia and VWD during the year. This shows a disproportionately large number of newly registered females for all autosomal disorders, presumably reflecting referral bias of women with menorrhagia.

Table 31 Non-trial products issued to treat people with selected other bleeding disorders between 2024/25

Product type	Manufacturer	Product	Factor V deficiency		Factor VII deficiency		Factor X deficiency		Factor XI deficiency		Factor XIII deficiency	
			Units (IU)	People (n)	Units (IU)	People (n)	Units (IU)	People (n)	Units (IU)	People (n)	Units (IU)	People (n)
Recombinant	Novo Nordisk	NovoSeven (mg)	529	2	6,713	84	-	-	195	2	-	-
		NovoThirteen	-	-	-	-	-	-	-	-	1,087,500	38
Plasma-derived	BPL	BPL FXI	-	-	-	-	-	-	77,715	40	-	-
		Coagadex	-	-	-	-	3,027,750	35	-	-	-	-
	Baxter	Com FVII	-	-	871,800	2	-	-	-	-	-	-
	CSL Behring	Beriplex	-	-	-	-	217,500	5	-	-	-	-
		FXIII	-	-	-	-	-	-	-	-	25,750	2
		Fibrogammin P	-	-	-	-	-	-	-	-	896,250	45
	LFB Biomedicaments	Hemoleven (LFB XI)	-	-	-	-	-	-	2,000	1	-	-
	Octapharma	Octaplas (bags	26	4	-	-	-	-	600	1	-	-
		Octaplex	-	-	-	-	490,500	10	-	-	-	-
	Takeda	FEIBA	-	-	172,000	1	-	-	270,000	1	-	-
Blood component	National blood transfusion service	Platelets	4	1	-	-	-	-	-	-	-	-

Units in IU unless otherwise stated.
Excludes issues of investigational products.

Table 31: This gives a breakdown of products issued during 2024/25 for people with other bleeding disorders, broken down by diagnosis and supplier. People may be issued multiple products. Although cross-diagnosis NHD-reported Octaplas issues sum to 1% of manufacturer-reported sales, it is believed that there may be further, unreported Octaplas issues for Factor V deficiency from local hospitals.

2.6 Adverse Events

2.6.1 Introduction and Adverse Event Assessment Panel

Pharmacovigilance is an increasingly important function of the database. Treatments are monitored for safety signals and drug-related adverse events, including poor efficacy, are reported to the manufacturer and, through them, to the regulators. Consequently, the conduct of post-license safety and efficacy studies has become a routine for the database. This function has assumed even greater prominence as we enter a new therapeutic era, using non-replacement drugs and antibodies with novel modes of action and with side effect profiles very different from the traditional replacement therapies that we are used to and which they may replace.

In response to this increasing need and the need for an objective, independent and robust evaluation of adverse events an SOP was developed for managing and evaluating adverse events in 2019 and 2021. Adverse Events (AEs) and Serious Adverse Events (SAEs) were defined as for GCP clinical trials. Where required, adverse event reports are further investigated by the Adverse Event Panel.

The Adverse Event Panel meet once a month by video conference to adjudicate on all SAEs. scoring their severity (1-5) and the potential relationship to drug therapy (unrelated, possibly related, probably related, or definitely related), again using the same framework that would apply for AE and SAE reporting in GCP-standard clinical trials. Serious Adverse events may be evaluated more urgently by e-mail and ad-hoc meetings, if necessary. All reported adverse events are reviewed by the panel.

The NHD Adverse Event Assessment Panel membership is currently as follows:

Prof Charles RM Hay Chair, Representing NHD
Prof Pratima Chowdary,
Julia Spires HNA
Dr Sarah Mangles
Dr Mary Mathias, Representing the Paediatric Working Party
Dr Charles Percy, Representing the Inhibitor Working Party
Dr Kate Talks, representing the Data Management Working Party

The group meets once a month by video conference, and the meetings are recorded to facilitate production of accurate minutes. The group may meet or communicate between meetings, if required.

All adverse events reported to the database are considered by the group. Where further enquiries or follow up are required to resolve issues in relation to reported events, these are delegated to panel members to follow up and events are not “closed” until data queries have been resolved or exhausted.

Where adverse events are considered possibly or definitely drug-related, the event is reported on to the manufacturer and through them to the regulator.

Most adverse events are reported to the database spontaneously soon after they occur, using the electronic reporting system. Many adverse events are unresolved at the time of reporting and require follow up from the database and CMWP before the report can be concluded.

Causes of death are under-reported by centres. Where a PwBD has died at home or in another hospital, the centre may not have been informed of the death, and it can be difficult to find out the cause of death if the PwBD does not die in the base-hospital, since the medical records are sent back to the family practitioner committee after death. The NHD has an arrangement with NHS Digital and for NHS Scotland (but not for Northern Ireland) for them to share death certification data for all PwBD registered with the database. This data comes through quarterly, and more than half of our data on death and the causes of death is derived from those sources.

The extent of adverse event reporting varies considerably between centres and, it is clear, that some centres are under-reporting, or not reporting or recording adverse events. **Table 32** shows adverse

event statistics over three years from eight anonymised centres chosen purely to demonstrate the wide range of adverse event reporting by different centres. Group summaries across all centres are also shown. We will shortly send all centres their adverse event reporting statistics and the group mean for comparison so that they are better able to review their adverse event reporting practice.

Table 32 Adverse Events Reporting by Centre

PwBD registered at centre (n)	Mean events reported per 100 PwBD per year, April 2022 - March 2025
1994	2.0
757	1.7
1946	0.9
2995	0.7
2595	0.5
1951	0.2
809	0.1
852	0.0
Median (iqr) across all 65 centres:	
479 (181 - 193)	**0.35 (0.0 - 0.9)**

Reports on the following events are actively solicited with monthly reminder “orange email*”. (*successor to the orange reminder postcard!):

Acute or Allergic reactions
Deaths
Infections
Malignancy
Inhibitors and anti-drug antibodies
Intracranial Haemorrhage (paediatric and adult)
Neurological Events
Thrombotic events (including MAHA)
Unexpected poor efficacy
“Other” events

Adverse events are summarised overleaf, and serious adverse events described in more detail. Personal details have been supressed to minimise the risk of a breach of confidentiality.

All adverse events reported by centres who participate in the European Haemophilia Safety Surveillance (EUHASS) program are anonymised and automatically forwarded via the NHD website to avoid the need for double-reporting.

Table 33 Summary of Adverse Events reported between April 2024 & March 2025

Event	People (n)	Events (n)
Allergy Event	8	8
Death Event	140	140
ICH Event	11	11
Inhibitor Event	10	10
Malignancy Event (Liver Related)	8	8
Malignancy Event (Not Liver Related)	30	30
Neurological Event	1	1
Other Event	8	9
Poor Efficacy Event	5	5
Thrombotic Event	15	16
Infection Event	0	0
Total	**236**	**238**

Table 33: This summarises the number and category of adverse events *fully* reported to the database by haemophilia centres during 2024/25. These events are described in greater detail in the tables that follow.

We are aware of a total of 23 new inhibitors reported in 2024/5 (11 in PwSHA, 8 in PwNSHA, 2 in PwHB and 2 in PwVWD) from the quarterly returns, and event details on all these PwBD are being requested.

Although 140 deaths have been reported to the database by centres, we are aware of 442 deaths in all bleeding disorders through NHS Digital, who report death certification data to the database on a quarterly basis. Many of these deaths will have occurred in the community or in other hospitals and the haemophilia centre may be unaware of them or of the cause of death. The database will share this data with the managing haemophilia centre in future so that they can complete their record. These deaths and their primary cause of death is summarised in the section on mortality.

Of the allergic events, 7/8 were considered definitely (2), probably (3), or possibly (2) drug related and were reported to the manufacturer. All inhibitors were considered to have arisen secondary to factor VIII replacement. None of the thrombotic events were considered to be treatment-related. Of the “other” events, two were considered probably related. Adverse events considered definitely, probably or possibly related were reported to the manufacturer.

In future, events of special interest should probably not merely be broken down by bleeding diagnosis, treatment, and treatment-relationship, if any, but also expressed in events per period of risk so that it is possible to compare the incidence of such events in different treatment risk-groups.

Table 34 Allergic/ other reactions reported between April 2024 & March 2025

Event date	Diagnosis	Event	Material	Relationship to material	Outcome
2023/24	Haemophilia A	Reaction believed due to administration technique	Emicizumab	Unlikely	Hospitalisation with treatment
	von Willebrand disease	Red face, chest pain, loin pain	Wilate	Definite	Resolved
2024/25	Haemophilia A	Lower lip swelling	ADYNOVI	Probable	Switched to Advate. Resolved without treatment.
	Haemophilia A	Pulsating chronic lower back pain	Esperoct	Possible	Resolved with outpatient treatment
	von Willebrand disease	Burning sensation, back pain, shoulder pain	Voncento	Definite	Resolved with outpatient treatment
	von Willebrand disease	Muscle pain in back of legs and back during infusion	Veyvondi	Probable	Switched to Novoseven. Resolved with outpatient treatment
	Factor X deficiency	Seizure like symptom	Tranexamic acid	Possible	Resolved without treatment.
	Factor XIII deficiency	Dizziness and flushing	NovoThirteen	Probable	Resolved with outpatient treatment

Table 34: In each case the events were evaluated by the Adverse Events Assessment Panel and reported to the manufacturer.

Table 35 Intracranial haemorrhage (all ages) reported between April 2024 & March 2025

Event date	Diagnosis	Age group (years)	Trauma Present	Maintenance therapy	Outcome
2017/18	Haemophilia A, mild	>=70	None reported	Prophylaxis	Deceased
2023/24	Haemophilia A, severe	40-69	Trauma reported	Prophylaxis	Alive
	Haemophilia A, moderate	40-69	None reported	On demand	Deceased
	Haemophilia A, moderate	>=70	None reported	On demand	Deceased
2024/25	Haemophilia A, severe	<1	Trauma reported	ITI	Alive
	Haemophilia A, severe	1-9	None reported	Prophylaxis	Alive
	Haemophilia A, severe	40-69	Trauma reported	Prophylaxis	Alive
	Haemophilia A, severe	40-69	None reported	Prophylaxis	Alive
	Haemophilia A, mild	>=70	None reported	On demand	Deceased
	Haemophilia B, moderate	40-69	None reported	Prophylaxis	Alive
	von Willebrand disease	>=70	Trauma reported	On demand	Alive

Table 36 Infection Events reported between April 2024 & March 2025

There were no infection events reported this year.

Table 37 Inhibitors reported between April 2024 & March 2025

Event date	Diagnosis	Age group (years)	Material	Exposure days
2023/24	Haemophilia A, severe	1-9	Elocta	<=50
	Haemophilia A, non-severe	10-19	Advate	Unknown
2024/25	Haemophilia A, severe	1-9	Nuwiq	<=50
	Haemophilia A, severe	1-9	Emicizumab	<=50
	Haemophilia A, severe	1-9	Elocta	<=50
	Haemophilia A, severe	1-9	Elocta	<=50
	Haemophilia A, severe	10-19	Nuwiq	<=50
	Haemophilia A, severe	40-69	Emicizumab	>50
	Haemophilia A, non-severe	1-9	Advate	<=50
	Haemophilia A, non-severe	>=70	Esperoct	<=50

Table 37: Full reports were submitted for ten inhibitors between April 2024 and March 2025. These were all for PwHA, including three with non-severe haemophilia A.
See Table 22 for further details of new, ongoing and historic inhibitors.

Table 38 Malignancy Events reported between April 2024 & March 2025

Malignancy	People (n)
Gastrointestinal	5
Haematological malignancy	6
Liver-related	8
Lung	8
Other	10
Unknown	1

Table 38: This shows the malignancy events reported between April 2024 and March 2025, although the event date precedes April 2024 for 16 of the 38 cases. The bleeding diagnosis has not

been shown since the malignancies reflect both the types and frequencies seen in the general population, as a broad generalisation, and are found in all groups of people with bleeding disorders with no specific associations. The exception to this is hepatocellular carcinoma. Of the eight liver-related malignancies, seven were cases of HCC, of which five related to people with a history of HCV.

Table 39 Poor Efficacy Events reported between April 2024 & March 2025

Event date	Diagnosis	Event	Material	Trauma Present
2023/24	Haemophilia A	Breakthrough bleeding due to poor compliance. Very low drug levels, antidrug antibody negative	Emicizumab	None reported
	Haemophilia A	Mandibular pseudotumour. Plan to start FVIII prophylaxis.	Emicizumab	No
2024/25	Haemophilia A	Frank haematuria	Emicizumab	No
	Haemophilia A	Unsatisfactory FVIII levels pre- and post-surgery. Switched to ReFacto AF.	Esperoct	None reported
	Haemophilia A	Lower leg and knee pain. Switched to Esperoct.	Emicizumab	None reported

Table 40 Thrombotic Events reported between April 2024 & March 2025

Event date	Diagnosis	Event	Prophylaxis	Related to treatment material?
2021/22	Afibrinogenemia*	Pulmonary Embolism (PE)	Yes	No
2022/23	von Willebrand disease	Pulmonary Embolism (PE)	Yes	No
2023/24	Afibrinogenemia*	Left Internal Jugular Vein Thrombosis (line related)	Yes	No
	Severe haemophilia A	Hepatic artery thrombosis	Yes	No
	Mild haemophilia A	Myocardial infarction	No	No
2024/25	Acquired Haemophilia A	Myocardial infarction	No	No
	Acquired von Willebrands	Pulmonary Embolism (PE)	No	No
	Dysfibrinogenaemia	Thrombotic Stroke	Unknown	No
	FVII deficiency	Thrombotic Stroke	Unknown	No
	FXI Deficiency	Myocardial infarction	No	No
	Severe haemophilia A	Myocardial infarction	Yes	No
	Mild haemophilia A	Post op left radial artery occlusion, related to arterial line	No	No
	Mild haemophilia B	Thrombotic Stroke	No	No
	Mild haemophilia B	Myocardial infarction	No	No
	von Willebrand disease	Myocardial infarction	No	No
	von Willebrand disease	Thrombotic Stroke	Unknown	No

*Please note that these two events were related to one person with afibrinogenemia

Table 41 Neurological Events reported between April 2024 & March 2025

Event date	Diagnosis	Event	Cause of Event
2022/23	von Willebrand disease	Spontaneous epidural bleed	Unknown

Table 42 Other Events reported between April 2024 & March 2025

Event date	Diagnosis	Event	Material	Relationship to material	Outcome
2019/20	von Willebrand disease*	Transient hypertension and visual field floaters after administration	Voncento	Not reported	Resolved within sixty minutes
2023/24	FV deficiency	Inhibitor to factor V	Octaplas	Not reported	Not reported
	Severe haemophilia A	Increased global joint pain for 24 hours after every Emicizumab dose	Emicizumab	Probably related	Advised to try taking at night, with paracetamol and found to improve
	Severe haemophilia A	Local reactions at the injection site	Emicizumab	Not reported	Not reported
	Moderate haemophilia A	Suspected anti-drug antibody	Emicizumab	Probably related	Continues on emicizumab
2024/25	FVII deficiency	Died during surgery to resect chondrosarcoma. FVII deficiency not thought to have impact.	Unknown	Not reported	Death
	Severe haemophilia A	Extreme fatigue the day after every Emicizumab injection. Also has fibromyalgia.	Emicizumab	Not reported	Not reported
	Severe haemophilia A	Borderline anti-drug antibody	Emicizumab	Not reported	Elected to stop emicizumab
	von Willebrand disease*	Floaters in visual fields and hypertension lasting 30-60 minutes post-infusion	Veyvondi	Not reported	No haemostatic periprocedural concerns.

*Please note that these two events were related to one person with von Willebrand disease

These events were adjudicated by the membership of the Co-Morbidities Working Party.

2.7 Mortality

Table 43 Causes of death in people with congenital haemophilia A and B between April 2024 & March 2025

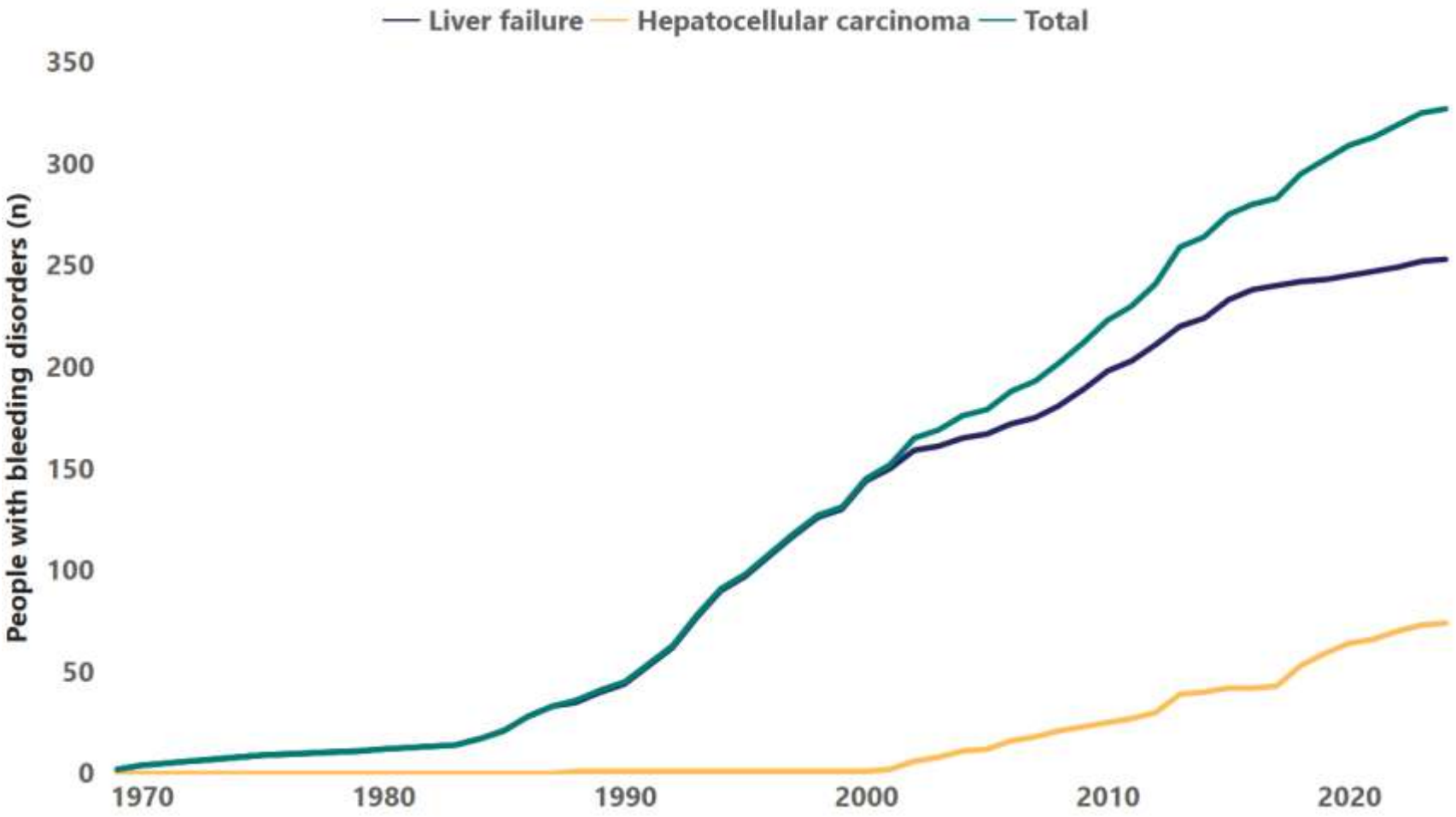
Cause of Death	Number of people with congenital haemophilia A by factor VIII level (IU/dl)			Number of people with congenital haemophilia B by factor IX level (IU/dl)		
	<1	>=1	Total	<1	>=1	Total
AIDS	0	0	0	0	1	1
Bleeding	1	1	2	0	0	0
Cancer (non-liver)	0	9	9	0	1	1
Cerebral palsy	0	1	1	0	0	0
Dehydration	0	1	1	0	0	0
Frailty/ dementia	1	1	2	0	1	1
Hypoglycaemia	0	1	1	0	0	0
Infection (bacterial)	3	7	10	0	0	0
Ischaemic heart disease	1	1	2	1	1	2
Stroke	0	0	0	0	1	1
Trauma	0	1	1	0	0	0
Other	0	0	0	0	2	2
Not known	7	49	56	4	10	14
Total	**13**	**72**	**85**	**5**	**17**	**22**

Tables 43 & 44 (overleaf): These show the causes of death amongst people with haemophilia A and B (including carriers), broken down by haemophilia severity (Table 42) and for other bleeding disorders (Table 43) during 2024/25. These include centre reports for the whole of the UK and death certification data provided by NHS Digital for England and Wales and NHS Scotland. We do not have death certification data for Northern Ireland, though we do have centre reports. The proportion of PwBD whose cause of death is unknown has increased since last year. Even NHS digital are unable to provide the cause of death in some registrants whose registration details may have been incorrect.

Table 44 Causes of death in people with other bleeding disorders between April 2024 & March 2025

Cause of Death	Number of people by bleeding disorder				
	Von Willebrand disease	Acquired haemophilia A	Other acquired disorders	Other bleeding disorders	Total
Abdominal aneurysm	0	0	1	0	1
Bleeding	1	2	0	3	6
Cancer (liver)	0	0	0	1	1
Cancer (non-liver)	7	3	0	3	13
Frailty/ dementia	1	5	1	2	9
Hepatocellular carcinoma	0	0	0	1	1
Infection (bacterial)	6	14	1	17	38
Ischaemic heart disease	2	3	0	6	11
Stroke	0	0	0	1	1
Thrombosis - venous	0	0	1	0	1
Other	1	3	0	3	7
Not known	57	70	11	108	246
Total	**75**	**100**	**15**	**145**	**335**

Figure 27 Cumulative incidence of deaths where primary cause of death is reported to be hepatocellular carcinoma or liver failure in people with bleeding disorders in the UK 1969 - 2024



Data table for Figure 27: *Cumulative incidence of deaths where primary cause of death is reported to be hepatocellular carcinoma or liver failure in people with bleeding disorders in the UK 1969 – 2024*

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
1996	0	10	10
1997	0	10	10
1998	0	9	9
1999	0	4	4
2000	0	14	14

Year	Hepatocellular Carcinoma	Liver Failure	Total
2001	1	6	7
2002	4	9	13
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	1	4	5
2015	2	9	11
2016	0	5	5
2017	1	2	3
2018	10	2	12
2019	6	1	7
2020	5	2	7
2021	2	2	4
2022	4	2	6
2023	3	3	6
2024	1	1	2
Total	**74**	**253**	**327**

Figure 27 (previous page): This shows deaths where liver failure or hepatocelular carcinoma are reported to be the primary cause of death. Liver disease may have been a contributory cause of death in other cases but that is far more difficult to determine and quantify and we do not have complete data on that. The table documents the number whose death certificate listed liver disease as the principal cause of death or who were reported to the NHD by their centre as having died from complications of HCV (i.e. Cause 1A on the death certificate). Liver disease may have been a subsidiary contributory factor in other deaths although not listed as the primary cause.

Deaths from hepatocellular failure or hepatocellular carcinoma have not levelled off despite successful HCV eradication from almost all people with bleeding disorders infected with that agent. As HCV has now been eradicated from almost all surviving people, one might expect a reduction in the incidence of hepatocellular carcinoma (HCC), which declines after viral eradication, even in the presence of ongoing cirrhosis. It is sobering, however, that new cases of hepatocellular carcinoma continue to be reported, occurring as a consequence of the legacy of serious liver disease in some PwBD. A reduction in this complication of hepatitis C may take some time to become evident. There may also be a delay before a further reduction in deaths from hepatocellular failure is seen, as some people have advanced cirrhosis with hepatocellular failure and not all are suitable for transplantation or have a donor. However, successful viral eradication will result in complete recovery of early cirrhosis or less advanced liver disease, and those individuals should not go on to develop advanced cirrhosis and will have a greatly reduced risk of HCC, which usually complicates cirrhosis.

3. Haemtrack Annual Report for the calendar year 2024

Introduction

This report is reported by calendar year rather than by financial year since 10% of the data is reported on paper, incurring a significant delay.

Haemtrack is used by people to report home therapy to their haemophilia centre and so its use is not limited to haemophilia A or B but includes other, rarer, bleeding disorders for which people also use home therapy. Both haemophilia centres and people vary in their reporting compliance with Haemtrack and so the quality of individual person self-reported data varies and the proportion of people using the system also varies from centre to centre.

In general, data quality is improving but for the first time since the system was introduced, we are observing a slight reduction in the proportion of people using the system. I would attribute this largely to PwBD becoming bleed-free on emicizumab. With little to report other than their weekly or fortnightly injection, some people stop reporting altogether. It remains important that they continue to report, however, because we need to document their drug compliance and their bleed rate and use of Haemtrack remains a condition of access to the drug.

Broadly speaking, reporting compliance is best for severe haemophilia A and B and less good for von Willebrand's disease and rarer conditions but is improving in those conditions.This data is extremely valuable however and we would urge centres to press their PWBD to report in all their home therapy so that we are in a better position to assess relative efficacy of different products and regimens.

Haemtrack should become a routine part of home-therapy management and a valuable tool for the review and optimisation of home-therapy and for person education. For that to happen, compliance will have to be reinforced by Health Care Professionals (HCP's) reviewing Haemtrack records with people in clinic and checking the data for accuracy before downloading into HCIS, where it should be checked by HCPs prior to uploading into the National Haemophilia Database.

Haemtrack should also be used in multidisciplinary team meetings (MDTs). Many haemophilia centres already do this and in those centres recruitment and data quality are steadily improving. This requires a consistent investment in time by the centre staff which results in improving compliance with both home therapy and record keeping.

Some haemophilia centres appear not to have realised the full clinical utility of this reporting system and consequently return sub-optimal data, which may reflect some degree of non-engagement by both HCPs and their PWBD. It is important to demonstrate to the person, by referring to Haemtrack data on screen in clinics, that collecting and reporting treatment data is useful for their clinical management, and that its collection is not just an empty bureaucratic exercise.

NHS funding is always under extreme pressure and NHS England, desiring responsible use of drugs and accountability, have made it clear that compliant use of Haemtrack will be a prerequisite for access to new treatments such as emicizumab and EHL-FIX and Veyvondi®. NHS England require evidence that new drugs are cost effective and lead to better clinical outcomes in normal use. Haemtrack provides such evidence.

Haemtrack Update

Haemtrack continues to be a fundamentally important component of the UKHCDO Haemophilia IT Strategy. It integrates with the Haemophilia Clinical Information System (HCIS) and the National Haemophilia Database (NHD) providing PwBD-entered data on treatments and bleeds that is unavailable from any other source. The data from Haemtrack is invaluable to support centres in the management of therapy for PwBD and optimising treatment regimens, whilst also providing its data nationally to support research and introduction of new treatments and regimens.

Haemtrack now has over 2.7 million PwBD-recorded entries, with over 100 thousand bleeds recorded. On average between 15 and 20 thousand entries a month are being added by people. The majority of people using the system are severe as would be expected, though some people with mild or moderate severity disease also use the system. The main diagnosis of people using the system are Haemophilia A, Haemophilia B, von Willebrand disease, Factor VII deficiency and Factor X deficiency. A video for PwBD explaining its use is available on-line (https://youtu.be/MVQbhJe7Rmk).

Haemtrack 3

Haemtrack 3, the new version of the hugely successful PwBD home therapy system has been operational for almost two years. The very latest technologies have been adopted in the new system and with its implementation we have hugely improved the scope and capability of the system. The technology approach taken is continuing to deliver significant benefits with the ability to release updates to the system within days, whereas previously it would take several months. Security has also been improved with the introduction of two factor authentication. The new system can also be extended and tailored for multiple use cases, including new developments such as gene therapy, PwBD reported outcome measures and clinical studies. Haemtrack 3 is a core component on the NHD re-development strategy, and its successful development and implementation is a huge achievement.

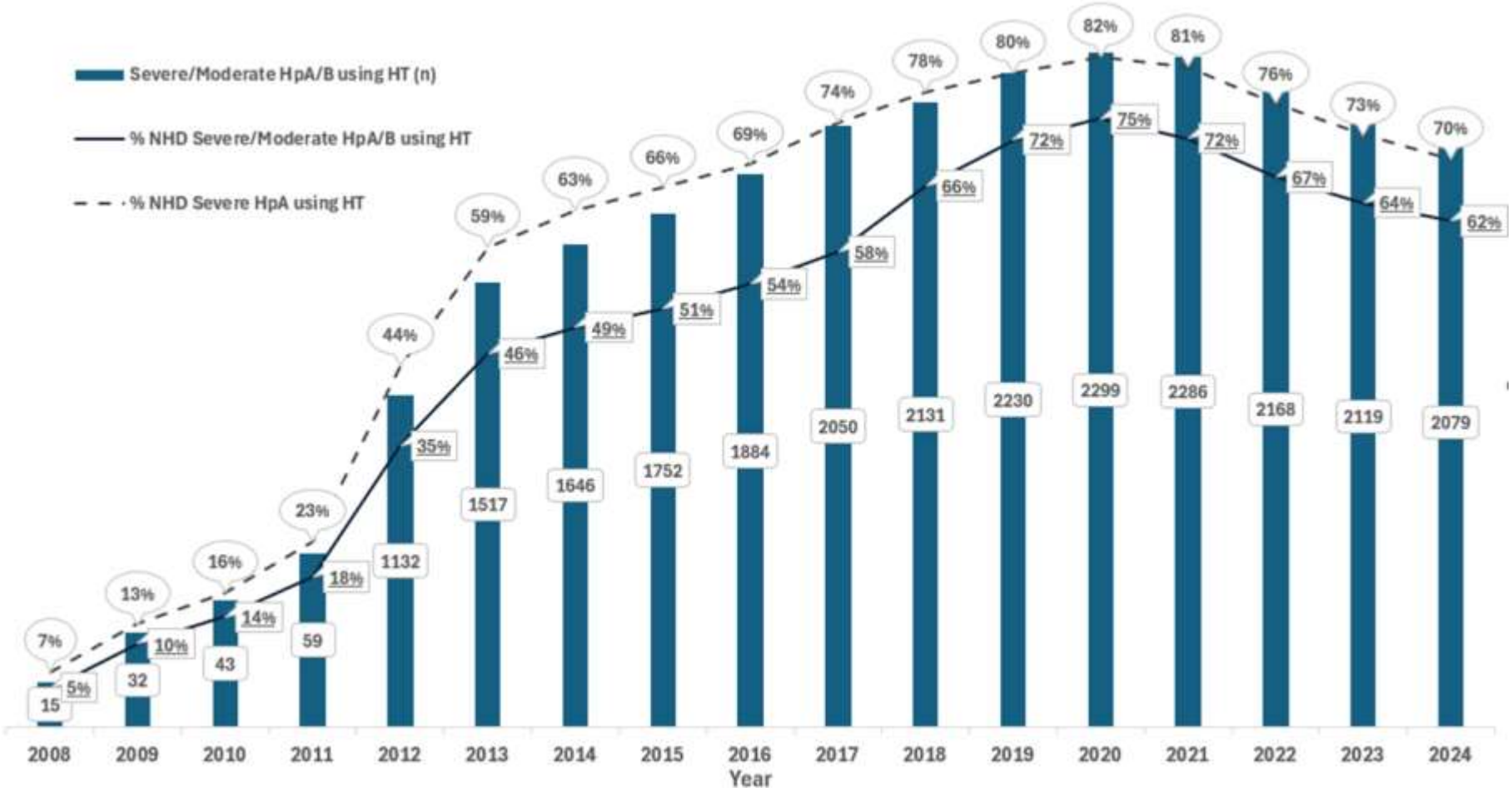
We have extended the use of Haemtrack to inpatient use (data entered by centre staff) so that it will provide a complete treatment record. Some centres are using this routinely and others not. We would encourage the universal adoption of inpatient use of Haemtrack so that the treatment record becomes complete.

3.1 People's Haemtrack Usage Analysis

3.1.1 Overall

Figure 1 (overleaf) presents an overview of people with severe and moderate haemophilia A or B using Haemtrack from 2008 to 2024. The blue columns give the numbers of people that use Haemtrack, and the lines show the proportion of NHD people who use Haemtrack. This illustrates rapid growth in 2012, when recruitment was the subject of a CQUIN and increased growth from 2017 when Haemtrack became a condition for access to new treatments. Figure 1 also reveals that there has been a gradual rise in the rate of recruitment, especially in severe haemophilia A cohort but that this has tailed off over the past two or three years during which time the proportion of people using the system has declined for the first time (see comments above).

Figure 1 Number of people using Haemtrack and recruitment rate, 2008-2024



3.1.2 Haemtrack usage analysis at centre level - people with severe haemophilia

Figures 2 and 3 display the proportion of people with severe haemophilia A/B using Haemtrack in each CCC (Figure 2) and HC (Figure 3).

Figure 2 Comparison of recruitment to Haemtrack by centre for people with severe haemophilia A/B: Comprehensive Care Centres (CCCs) in 2024

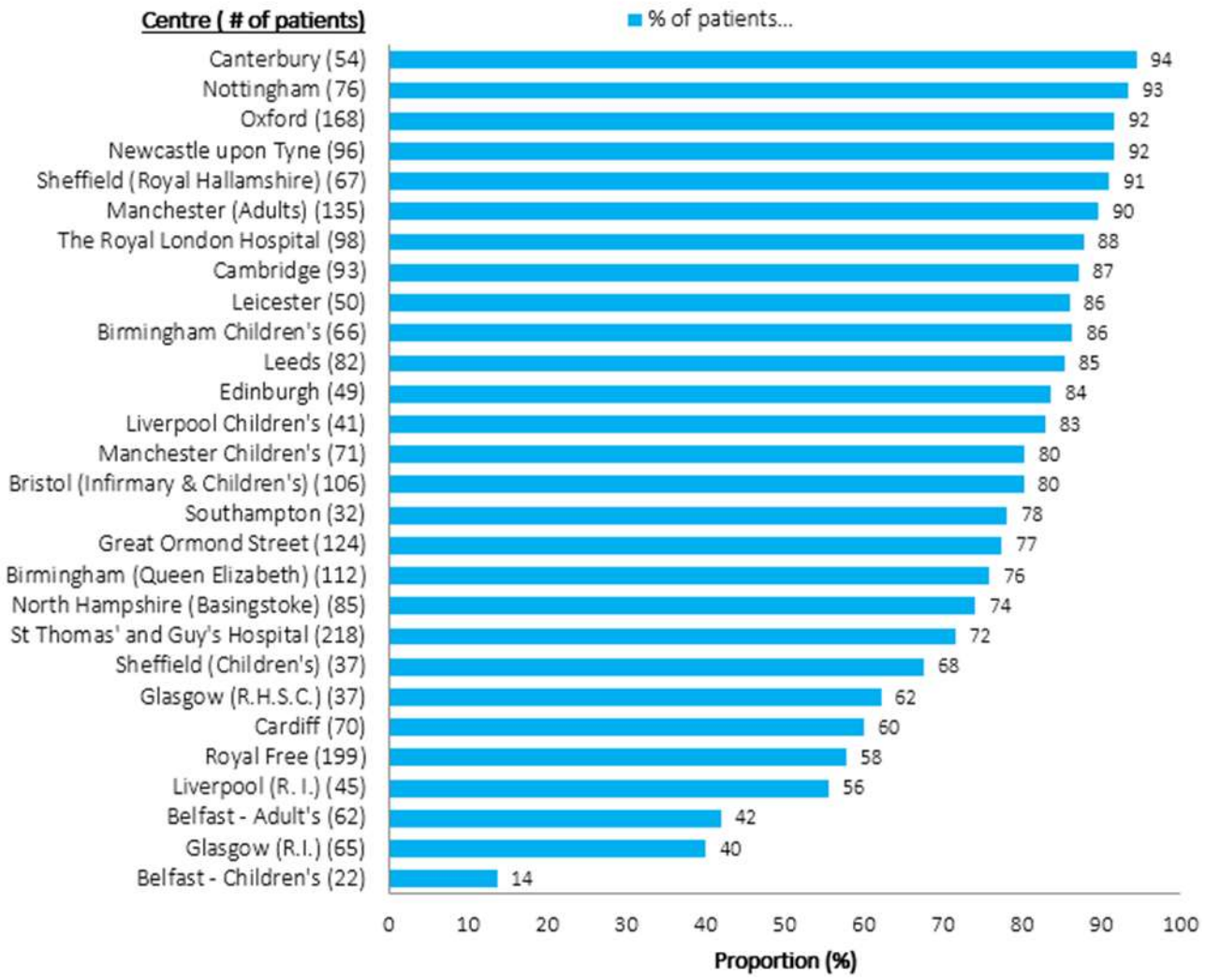
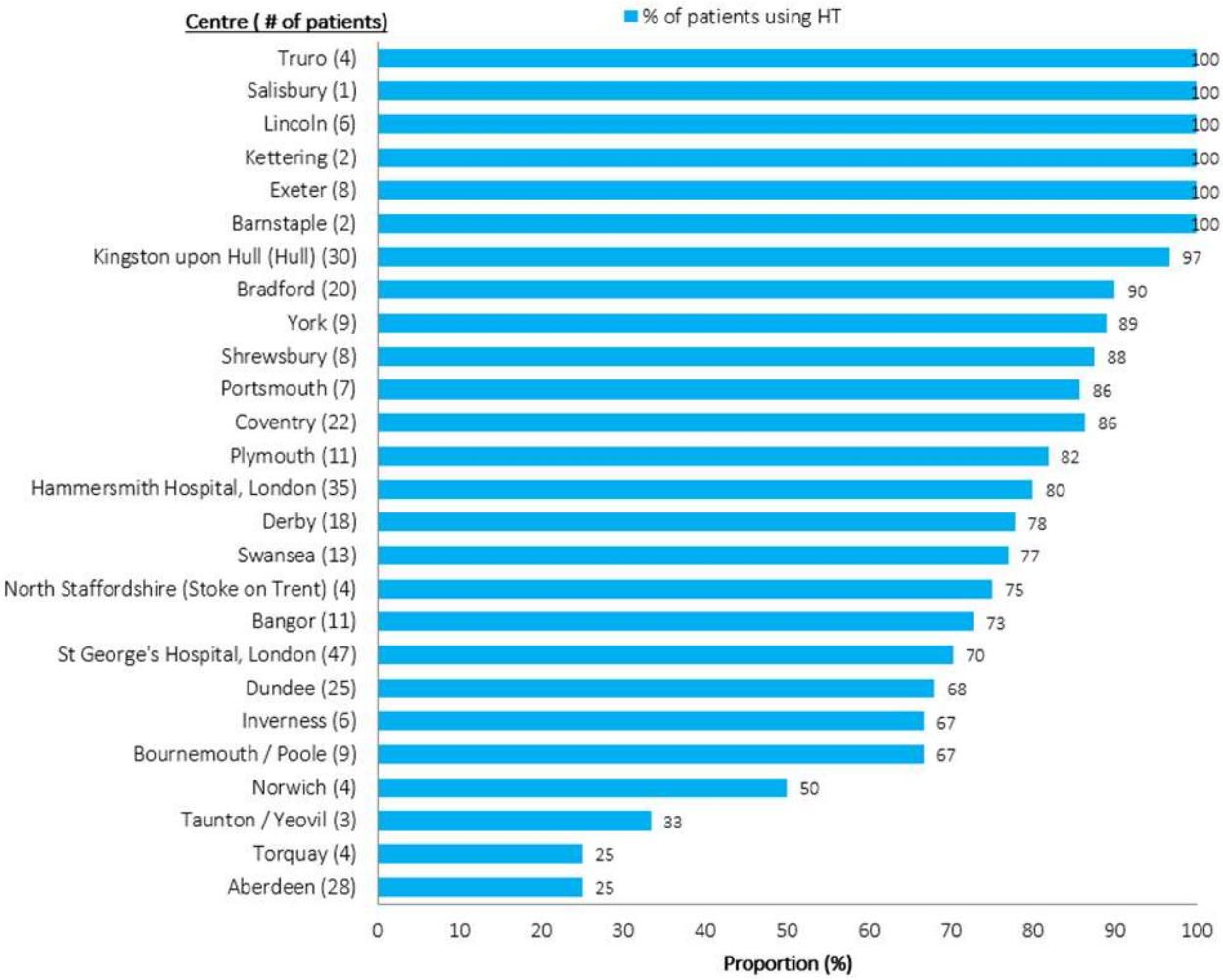


Figure 3 Comparison of recruitment to Haemtrack by centre for people with severe haemophilia A/B: Haemophilia Centres (HCs) in 2024



4. Data Management Working Party

Membership

Chair:	Dr Kate Talks
Commissioner representative:	Will Horsley
Co-Director of the National Haemophilia Database:	Professor Charles Hay
Co-Director of the National Haemophilia Database:	Prof Pratima Chowdary
Haemophilia Nurses Association representative:	Emma Franklin
Haemophilia Physiotherapists Group representative:	Dr David Stephensen
Haemophilia Society representative:	Kate Burt
MDSAS representative:	Dr Rob Hollingsworth
PwBD representatives:	William McKeown Paul Sartain

Representatives of Northern Ireland, Scotland and Wales:

Northern Ireland	Dr Gary Benson
Scotland	Dr Ryan Rodgers
Wales	Dr Samya Obaji

UKHCDO Working Party Chairs/ co-chairs:

Co-morbidities Working Party	Dr Susie Shapiro
Genetics Working Party	Dr Suthesh Sivapalarantnam
Inhibitor Working Party	Dr Charles Percy
Musculoskeletal Working Party	Dr G Benson
Paediatric Working Party	Dr Mary Mathias
Von Willebrand Working Party	Dr Carolyn Millar
Laboratory Working Party	Dr Will Lester / Annete Bowers
Women and Bleeding disorder Working Party	Dr Nicola Curry

UKHCDO Executive Committee:

Chair	Prof Pratima Chowdary
Secretary	Dr Mary Mathias
Treasurer	Dr Gary Benson

Members of the NHD as nominated by the Director(s) of the National Haemophilia Database:

NHD Manager	Andrew McNally
Business Support Partner - Administration & Executive Support	Greg Shone
Data and General Administrator	Blanca Martinez Grace Leach
Head of Data Analytics	Dr Hua Xiang
Data Scientist	Dennis Glover

Data Analyst	Ben Palmer
	James Chetter
Project Manager Peer Review	Harry Evans
Senior Research Nurse	Susan Caldwell

Meetings

Since the last report the UKHCDO Data Management Working Party (DMWP) has met twice via TEAMS on 20th May 2025 and 15th October 2025. In the course of the year there have been a number of changes in roles and new members welcomed; the list provided reflects the current membership.

The DMWP oversees the data collection and analysis of people with inherited bleeding disorders undertaken by the National Haemophilia Database (NHD). The DMWP and NHD are jointly responsible for the accuracy and completeness of the data collected. The DMWP has delegated most of the responsibility for assessing and overseeing requests for analysis of NHD data to the Data Analysis Group (DAG), which is a subcommittee of the DMWP.

The DMWP reviews the information that is collected on PwBD and revises this as necessary. Any member of UKHCDO can suggest changes to the data that are collected which will be considered by the DMWP. Any new data collection initiative that requires data collection across the UK also needs ratification from the advisory committee. The Database governance arrangements and information governance compliance are reviewed at each meeting and the database remains compliant with all the relevant information governance regulations: Data Protection Act 2018 (DPA); General Data Protection Regulation (2016), Caldicott Principles and NHS Information Governance guidance, legislation, and good practice. It has also been assessed and certified to ISO 9001.

A renewal submission was made in September 2024 to the UK Health Research Authority (HRA) for approval of the database as a research registry. To reflect the core data set more accurately the research registry name has been changed to the UK Bleeding Disorder Research Registry (UKBDRR). Following ethics committee review approval was received in November 2024 for a 5-year period. The process to record consent within the NHD has been updated and support for the rollout of this developed to ensure people are fully informed of their options and how their data may be used. The NHD also successfully reapplied to the Health Research Authority (HRA) Confidentiality Advisory Group (CAG) in England and Wales for section 251 support, which is required by NHS Digital to supply mortality data in Nov 2024. Approval is given for a 12-month period and will continue to be required to obtain mortality data. The HRA CAG have confirmed that a mixed model of consent use within the database is acceptable and does not put the section 251 application at risk. The Scottish Public Benefit and Privacy Panel for Health and Social Care have provided approval for Haemophilia Centres in Scotland to share data with the NHD. Consent of people in Northern Ireland is required for mortality data to be shared.

A new gene therapy form is now available to enable prospective clinical data entry for people receiving NHSE funded gene therapy. A new data collection form has been developed by the co-morbidity working party to collect data on the risk factors and the management of people with Haemophilia A and B who have developed atrial fibrillation with age matched and severity matched controls. The same process used to draft and pilot a form will be used to support the set up by other working parties of registries with new data sets.

The Adverse Events Panel supports the consistent and timely assessment of all Adverse Events (AE) reported electronically to the NHD. The panel meets monthly and is co-chaired by an NHD director and the DMWP chair with additional UKHCDO members, including representation from the paediatric and inhibitor working parties. Summary reports are provided to the DMWP and included in the annual NHD report.

Haemtrack

Commissioners for England and the devolved countries of the UK continue to encourage the use of Haemtrack as a means of capturing individual bleed events and treatment in PwBD. This has allowed important information about the impact of enhanced half-life factor VIII and IX and Emicizumab to be collated.

The current Haemtrack 3 version, a web app, was launched in September 2023 and has improved security and functionality for both PwBD and centre staff.

I would like to thank the Co-directors of NHD, Professor Charles Hay and Professor Pratima Chowdary, and all the other staff within the NHD for their commitment to collecting and analysing the data on behalf of the UKHCDO and Rob Hollingsworth and MDSAS for their continued support and maintenance of our national information systems.

I also wish to acknowledge and thank staff in the Haemophilia Centres and PwBD for their ongoing support providing data.

Dr Kate Talks,
Chair UKHCDO Data Management Working Party

November 2025

5. Data Analysis Group

Membership

Co-Chair (Chair of Data Management Working Party)	Dr Kate Talks
Co-Chair (Director of NHD)	**Professor Charles Hay**
User representatives:	Paul Sartain
	Dr Richard Gorman
UKHCDO members:	Dr Charles Percy
	Professor Pratima Chowdary
	Dr Susie Shapiro
	Dr Mary Mathias
	Dr Gillian Lowe
	Dr Ryan Rodgers
	Prof Peter Collins
Haemophilia Nurses and physiotherapy representatives:	Dr David Stephensen
Nurse representative	vacant
National Haemophilia Database:	Andrew McNally
	Ben Palmer
	Dr Hua Xiang
	Greg Shone
	James Chetter
	Dennis Glover
	Grace Leach
	Blanca Martinez
	Susan Caldwell

The Data Analysis Group (DAG) is a subgroup of the Data Management Working Party. Its role is to assess and prioritise applications for analyses data held by NHD, including Haemtrack and joint score data. Applications are assessed based on data governance considerations, scientific merit and available resources. The group meets once a month by videoconference which last on average 60-90 mins. The terms of reference of the DAG are available on the UKHCDO website. Requests for analyses are submitted on a standardised form, which is available on the NHD website. Requests for data analyses have been submitted by individual members of UKHCDO, UKHCDO working parties, Haemophilia Centres, NHS England, NHS Wales, PwBD societies and pharmaceutical companies. A number of requests have been made by NICE to support technical appraisals. The DAG reviews and discusses all applications. It provides feedback to the applicant and works with them to refine the proposal, if necessary.

Some straightforward requests, for example, those from NHS commissioners are often progressed on chair action and discussed at the next meeting. The data analyses that are generated from the requests are reviewed and commented on by the group and may be further revised, if necessary, before release. Reports often contain caveats about data limitations that impact on the interpretation of reports. The redevelopment of the NHD database as part of the NHD2 project provides an opportunity to provide automation of reports and develop online dynamic dashboards for different stakeholder groups. Work to define the requirements for different stakeholders and develop a service model is ongoing.

The contributions from the user representatives have been particularly useful in assessing the applications and interpreting results.

Publication: Emicuzimab utilization, safety and outcome in people with severe haemophilia and no inhibitors: 3-year follow-up. A report from the UK Haemophilia Doctors' Organisation. Res Pract Thromb Haemost. 2025 Sep 1;9 (6):103164

https://pubmed.ncbi.nlm.nih.gov/41050637/

All members of UKHCDO and UKHCDO working parties are encouraged to suggest analyses and are invited to collaborate with the DAG on these projects. Members of UKHCDO are encouraged to join the DAG and anyone interested should contact one of the Co-chairs. The DAG is also looking for a nurse representative to join the group.

Dr Kate Talks, Chair UKHCDO Data Management Working Party

Prof Charles Hay, Co-Director NHD

October 2025

6. Specialised blood disorders NHSE Clinical Reference Group

Membership

- Dr Susie Shapiro, National Speciality Adviser, and CRG Chair
- Will Horsley, Lead Commissioner and Pharmacist
- Dr Charles Percy, Clinical Member & Procurement Lead
- Dr Kate Talks, Clinical Member
- Dr John Grainger, Clinical Member representing paediatrics
- Dr Sarah Mangles, Clinical Member
- Prof Charles Hay, Affiliate Member, National Haemophilia Database (Director)
- Prof Pratima Chowdary, Affiliate Member, UK Haemophilia Centres Doctor's Organisation (Chair)
- Marie Eales, Affiliate Member, Haemophilia Nurses Assoc. (Chair).
  Role previously covered by Julia Spires.
- Robert Barnard, Affiliate Member, Haemophilia Chartered Physiotherapists Assoc. (Chair).
  Role previously covered by David Hopper.
- Debra Morgan, Haemophilia Society UK, PwBD and Public Voice representative
- Laurence Woollard, PwBD and Public Voice representative
- Giles Ratcliffe, Public Health Consultant

This group is designated as a 'Lead and Inform' NHSE Clinical Reference Group (CRG) which is the middle tier of three in terms of expected outputs and activity. The CRG covers haemophilia and other bleeding disorder services including von Willebrand disease, platelet disorders, acquired bleeding disorders, and some other coagulopathies such as thrombotic thrombocytopenic purpura.

The CRG meets routinely for two hours every three months using an online meeting interface; however there is additional activity between each CRG meeting.

During 2024/2025 the CRG and members were regularly engaged with the Infected Blood Inquiry (IBI), supporting and advising commissioners on the new national psychology service, and responding to each of the relevant recommendations in the IBI report.

The CRG is active across multiple other projects. During 2024 it oversaw the production of a new NHSE service specification (involving all four devolved nations) which is expected to be approved in 2025. Alongside the new specification will be a new suite of national service metrics. The CRG has played a key role in supporting development of new commissioning policies; notably, an updated commissioning position and funding agreement was obtained for vonicog alfa (on-demand) for all ages, and for the use of emicizumab for prophylaxis in people with moderate haemophilia A without inhibitors.

Will Horsley and Dr Susie Shapiro, August 2025

7. Co-Morbidities Working Party

Membership

Chair	Dr Susie Shapiro, Oxford
UKHCDO representatives:	Dr Gary Benson, Belfast Dr Gillian Evans, Canterbury Dr Sarah Mangles, Basingstoke Dr Emily Millen, Nottingham Dr Kate Musgrave, Newcastle Dr Kate Talks (DAG/DMWP)
Haemophilia Nurses Association Representative:	Cathy Harrison, Sheffield

Remit

To consider comorbidity issues in PwBD within the UK and review any unmet need with respect to data collection, guidelines and PwBD-related information / material.

To develop a Work Plan for the CMWP.

To advise the DMWP as to what data the NHD should collect regarding comorbidities in people with haemophilia and bleeding disorders.

Any publications arising from the CMWP should be approved by the UKHCDO Executive in line with the UKHCDO publication policy.

Meetings

The group has had 3 formal Teams meetings in the last year with additional 'sub-group' meetings with MDSAS and email discussions to support progression of Work Plan.

Activities

The main work has been to establish a prospective registry for haemophilia and atrial fibrillation (AF), with the support of the NHD. The registry will help to determine the prevalence, antithrombotic therapy and thrombotic complications of AF in people with haemophilia in the UK. The specific aims of the planned prospective registry for people with haemophilia and AF are: (a) To determine the prevalence of AF in PWH over the age of 50 years in a subset of haemophilia centres in the UK; (b) To document the use of antithrombotic therapy in PWH and AF; (c) To prospectively determine the bleeding and thrombotic risks in these PwBD. The plan is for data to be collected initially by the members of the working party from their CCCs, under research registry ethics of the NHD.

The group piloted draft forms for initial data collection in Excel and then worked with MDSAS to develop in to a more sophisticated data entry form within the NHD. Forms for initial data entry for

people with AF and for matched controls have been 'live' since May 2025. The CMWP members have therefore started initial data entry for their local PwBD with AF and matched controls.

Next steps include early data analysis and presentation of update to UKHCDO; consider whether other Centres would like to be involved; development of 'annual review' forms for prospective data collection of bleeding/thrombotic events.

Dr Susie Shapiro,
Chair, Co-Morbidities Working Party
August 2025

8. Genomics Working Party

The UKHCDO Genomics Working Party convenes virtually every two months.

In the last year, we have established the UKHCDO genomics MDT. At the first meeting, we discussed platelet genes that predispose individuals to cancer, and at the second, we discussed Hermansky-Pudlak syndrome. Both were virtual and well attended.

Current Membership:

- Dr Kate Talks - Newcastle
- Dr Matt Sims - Sheffield
- Dr Ruth Wheeler - London
- Dr Brenda Sempasa - Manchester
- Dr Jayashree Motwani - Birmingham
- Ms Victoria Cloke - Edinburgh
- Mrs Claire Forrester - Stoke on Trent
- Dr Suthesh Sivapalaratnam - Chair – London
- Megan Sutherland - Manchester
- Catriona Keenan - Dublin
- Prof. Keith Gomez – London
- Dr Claire Lentaigne - Plymouth
- Dr Andrew Page – Edinburgh

Contact:

greg.shone@ukhcdo.org

Objectives for 2025/2026:

1. To produce a comprehensive report on the current state of genomics in Haemostasis and Thrombosis, incorporating a survey on current practices in the UK
2. To create a FAQ document addressing common queries from PwBD, clinical staff, and scientists on genomics and genomic testing. This resource will be made available on various platforms in the future, including the UKHCDO website.
3. To explore the integration of gene variants into the National Haemophilia Database (NHD) and investigate the feasibility of linking genetic laboratory outputs directly to the NHD, aiming to enhance diagnostics and support research initiatives.

Dr Suthesh Sivapalaratnam,
Chair, Genomics Working Party

October 2025

9. Genetic Laboratory Network (UKHCDO-GLN)

Background

The UKHCDO GLN was formed in 2002, arising out of the UKHCDO Genetic Working Party (UKHCDO GWP), with the aim of improving collaboration between laboratories and of ensuring quality and equity of service across the U.K. The network currently comprises of 8 centres involved in the molecular genetic analysis of inherited bleeding, thrombotic and platelet disorders (many of the laboratories are also involved in other areas of haematology as well). These laboratories include Belfast City Hospital, Central and South Genomic Laboratory Hub (GLH), North East and Yorkshire GLH, North West GLH, Royal Free Hospital London, Royal Infirmary Edinburgh, South East GLH, and St. James' Hospital Dublin.

Representatives of the Network attend meetings of the UKHCDO Genetics Working Party.

Meetings

The UKHCDO GLN holds bi-annual meetings. The GLN held our first in-person meeting for a few years in November 2024. The network also met virtually in July 2025.

Chair & Secretary

Megan Sutherland and Catriona Keenan continue in their roles as Chair and Secretary, respectively.

Current activities

1. **NHS England Genomic Laboratory Hubs:** The new structure of service provision in England went live in February 2021. The NHS England genomic test directory specifies which disorders and gene targets will be investigated under this service model and by which methodologies they should be performed (https://www.england.nhs.uk/publication/national-genomic-test-directories/). The 'Non-malignant Haematology' service, including the investigation of bleeding, thrombotic and platelet disorders, is provided by four Genomic Laboratory Hubs in England.

2. **Laboratory Audit - ISO 15189**: Laboratories in the Network are accredited by the United Kingdom Accreditation Service (UKAS). Laboratories are required to adhere to ISO 15189 quality standards. The GLN continues to share examples of good practice, practical advice and knowledge as the inspection process is applied to member laboratories. All laboratories continue to participate in the UKAS inspection process cycle.

3. **Bleeding Disorder Genetic Analysis Best Practice Guidelines:** The UK Best Practice Guidelines (BPG) for Haemophilia A and Haemophilia B are in the process of being reviewed and updated in accordance with the NHS England genomic test directory. In addition, a working group has been established to produce a BPG for the genetic investigation of rare bleeding disorders, this will be produced in conjunction with the UKHCDO GWP. The UK Best Practice Guidelines (BPG) for phenotypic and genetic analysis of VWD were previously published (Platton et al (2024) PMID: 38532595).

4. **UK NEQAS Genetics of Heritable Bleeding and Thrombotic Disorders scheme:** The UK NEQAS Genetics of Heritable Bleeding and Thrombotic Disorders EQA scheme provides four EQA exercises per year, two of these are the traditional 'wet' exercises which always include analysis of one of *F8*, *F9* or *VWF*. The two additional 'paper' exercises provide a clinical scenario and genetic variant for a PwBD which are to be interpreted by the participants and a report produced. The paper exercises aim to expand the scope of gene targets for interpretation to include rare bleeding and thrombotic disorders. The results for each round of the scheme are reviewed and discussed at the following GLN meeting and any relevant comments fed back to the NEQAS steering group.

5. **Participation in other groups:** Representatives of the Network input to the UKHCDO GWP. A representative of the Network is a member of the World Federation of Hemophilia Laboratory Science Committee.

6. **General:** At each of the GLN meetings there is an open forum to discuss scientific and technical issues. We also aim to include an educational session at least once a year to focus on difficult areas for interpretation. Our main focus continues to be on the classification of rare or novel variants with reference to the current ACGS variant classification guidelines, and the sharing of this information throughout the network. It is recognised within the group that the sharing of knowledge and expertise is an essential mechanism for the interpretation of previously uncharacterised genetic variants.

7. **Discussion of the ClinGen Coagulation/VWD Expert Panel Specifications to the ACMG/AMP Variant Interpretation Guidelines for *F8, F9* and *VWF* variants:** At the in-person meeting in November, the educational session focussed on the discussion of the disease specific specifications for classification of *F8*, *F9* and *VWF* sequence variants. The group discussed each of the specification criteria in turn for each gene, to determine the use for variant classification, along with the ACGS classification criteria commonly used in the UK genetics laboratories.

Megan Sutherland,
Chair, UKHCDO Genetic Laboratory Network
July 2025

10. Gynaecology Working Party

Date of inception: 23rd October 2023

List of members: Professor R Abdul-Kadir (Co-Chair), Dr. N Curry (Co-Chair), Dr. G Benson, Dr. T Biss, Dr. A Carvalhosa, Professor J Clarke, Dr. S Garside, Dr. C Lentaigne, Dr. Gill Lowe, Dr. E Millen, Dr. P Raheja, Ms. C Warren, Dr J Mistry (secretary), Dr C Brown, Hayley Wiles.

Co-opted members:

- Members from BSH guideline writing committee, as standard, for the best practice guideline
- Doctor-in-training co-opted member planned to assist with data collection work stream and PPIE

Meetings: October 2024, January 2025, April 2025 - whole committee, Teams meetings

The aims of the GWBD working party were set out in our agreed terms of reference, and are as follows:

Aims: To direct and lead national changes to both clinical and PwBD-focused practices which will improve the care of girls and women with IBD across the UK, with an initial focus:

1.1 To develop a core data set of a) clinically important and b) PwBD-reported outcomes with the aim of piloting data collection at a small number of sites

1.2 To set standards for the care of GWBD

1.3 To support and facilitate research in the field of girls and women with bleeding disorders

1.4 To share expertise and best practices from existing joint clinics (gynae-haem and obs-haem) to then support UK haemophilia centres develop an MDT service for girls and women

1.5 To support educational programmes to increase awareness of GWBD among all HCPs involved in care of girls and women

The agreed aims for 2025-2026 for this group continue as last year, and are two-fold:
A: Developing a national standard for an MDT haem-gynae service, with a focus on all age groups - particularly adolescent age group. Looking at best practices for GP referrals into secondary care/management of acute HMB in ED and how to support NHS trusts to recognise the importance of these types of clinics. The group has formed a smaller Good Practice paper writing group, which is taking this forward with the BSH H&T writing committee. This is progressing well and is near completion.

B: Data collection for GWBD across all ages. A smaller data group has been formed with the plan to develop a core data set for collection of robust data relating to the care of GWBD. There are ongoing discussions with the NHD about how we can develop the data collection further, including whether

it may be possible to add gynaecological bleeding into the HaemTrack application. In addition, we are working with PwBD, and the Haemophilia Society, through several PPIE groups to look at how best to collect real-time PwBD-relevant data for GWBD.

An audit of practice has been completed across 13 hospitals - on an individual PwBD-level to look at the care of GWBD, with a particular focus on heavy menstrual bleeding. In addition to this, a national survey of centre-specific practice has been conducted, and the data includes 100% of CCC and approximately 30% of the haemophilia centres. The audit and centre-specific data are being put together into a manuscript for publication later this year.

Guidelines: The UKHCDO Gynaecology Working Party has responsibility for the Gynaecology Guideline that was published in late 2022. This will continue to be reviewed annually, and no update is currently required.

Publications:

Curry N, Lowe G, Clark TJ. Inherited bleeding disorders in heavy menstrual bleeding: The case for joint haematological and gynaecological care. BJOG. 2022 Dec 2. doi: 10.1111/1471-0528.17353.

Curry N, Bowles L, Clark TJ, Lowe G, Mainwaring J, Mangles S, Myers B, Kadir RA. Gynaecological management of women with inherited bleeding disorders. A UKHCDO Guideline. Haemophilia. 2022 Nov;28(6):917-937. doi: 10.1111/hae.14643. Epub ahead of print. PMID: 35976756.

Professor R Abdul-Kadir, Dr. N Curry
Co-chairs, Gynaecology Working Party
August 2025

11. Inhibitor Working Party

Membership

Charles Percy (Chair)

John Grainger

Mary Mathias

Ben Palmer

Anne Riddell

Kate Talks

Meetings

There have been no formal meetings of the inhibitor working party since the last AGM. Communication has been virtual. The revision of the guideline on acquired coagulation factor inhibitors has been submitted for publication. Other activities have included the following:

1. Submission of a commissioning policy proposal for emicizumab for acquired haemophilia A. Feeback is awaited from NHS England.
2. Data from the acquired haemophilia A project has been analysed and used for the development of the policy proposal and support modelling of the cost of emicizumab for acquired haemophilia. I
3. Participation in other meetings: Data Management Working Party, NHD Adverse Events Review Panel.

Following the last AGM, calls for new members have resulted in two volunteers. Meetings are therefore restarting; the work plan for the next 12 months will include review of the guideline on the diagnosis and treatment of factor VIII and IX inhibitors in congenital haemophilia.

Dr Charles Percy
Chair, Inhibitor Working Party
September 2025

12. Laboratory Working Party

Membership

Laboratory

Anne Riddell	Royal Free Hospital, London
Annette Bowyer (Co-chair)	Royal Hallamshire Hospital, Sheffield
Caroline Lawrence	Glasgow Royal Infirmary
Sean Platton	Royal London Hospital
Jill Martin	Aberdeen Royal Infirmary
Jannene Sanders	Hull Royal Infirmary
(steps down in July 2025-replacement needed)	
Stephen Macdonald	Addenbrooke's Cambridge

Clinical

Dr Mohammed Khan	Aberdeen Royal Infirmary, Aberdeen
Dr Andrew Page (Co-chair)	Edinburgh Royal Infirmary

Invited representation

UKNEQAS Blood Coagulation	Anna Williams
NIBSC	Stella Williams

Activities

The group held online committee meetings over the past 12 months on 10/4/24, 9/10/24, 15/1/25 and 14/4/25, and several ad hoc meetings to create the Altuvoct guideline. There is a requirement for a replacement scientist (for Janenne Sanders).

The most recent activity has been construction of guidelines for the laboratory monitoring of Altuvoct (efanesoctocog alfa). The manuscript is undergoing peer review at Haemophilia Journal. Response to reviewers' comments is in progress. Publication expected late Summer.

Joint collaboration between UKHCDO, the Haemostasis and Thrombosis Task Force of BSH and UK NEQAS lead to the following publication:

Jennings, I., Baker, P., Bowyer, A., Crossette-Thambiah, C., Gomez, K. and Lester, W. (2025), Reporting haemostasis assays in IU/dL–A recommendation for harmonisation from the British Society of Haematology Haemostasis and Thrombosis Task Force, UK NEQAS for Blood Coagulation and the UK Haemophilia Centres Doctors Organisation. Br J Haematol. https://doi.org/10.1111/bjh.70003

Items in progress

On-going data evaluation for the laboratory FVIII, FIX and treatment monitoring questionnaires that have been redistributed to haemostasis centres.

To develop an educational networking program for laboratory scientists with an interest in haemostasis.

Development of test repertoire for haemophilia centre peer review. A current practice questionnaire will be sent to CTC and HTC but distribution has been paused until the conclusion of the aforementioned questionnaires.

Exploration of a committee-wide project for the laboratory monitoring of Veyvondi.

Dr Annette Bowyer Co-Chair, Laboratory Working Party
August 2025

13. Musculoskeletal Working Party

Co-chairs:

- Dr Gary Benson (Haematologist, Belfast)
- Dr Paul McLaughlin (Physiotherapist, Royal Free London)

Members:

- Trupti Bhandari (Paediatric Physiotherapist, Evelina)
- Dr Nicola Curry (Haematologist, Oxford)
- Dr Emma Fosbury (Paediatric Haematologist, Manchester)
- Jessica Hedden (Physiotherapist, Swansea)
- Dr Emily Symington (Haematologist, Cambridge)
- Dr Thynn Thynn Yee (Haematologist, Royal Free London)

As part of the ongoing work, the group has set about establishing the landscape of data submitted/ held by the NHD pertaining to MSK health. Much of this revolves around the reporting of the HJHS. Whilst many centres complete the score, as part of the routine clinical follow up of PwBD, confirmed by the Quality Assurance audit report, there remains a lack of submission to HCIS which is deemed to be required to track changes as therapy options change.

The members of the group have taken part in the external quality assurance review in relation to physiotherapy and MSK provisions throughout the whole of the UK and look forward to the final report being submitted.

It is hoped to undertake a snapshot review of the current state of MSK health in PwBD within the UK and determine specific areas which may be an issue with regards to targeting support e.g. ankle health and interventions.

Dr Gary Benson
Co-chair, Musculoskeletal Working Party
September 2025

14. Paediatric Working Party

Membership

Mary Mathias (Chair)	London (GOSH)
Jeanette Payne (Secretary)	Sheffield
Jayanthi Alamelu	London (Evelina)
Neha Bhatnagar	Oxford
Tina Biss	Newcastle
Jayashree Motwani	Birmingham
John Grainger	Manchester
Simone Greene	Hull
Simone Stockley	Nottingham
Anne Kelly	London (GOSH)

Meetings

Since the last AGM the PWP has held 3 teleconference meetings in January, May and September.

Summary of activities

1. We have written up the **emicizumab PUPs and MTPs** data at a national level. Data was returned on 88 boys from 19/23 centres with eligible PwBD: 41 PUPs and 47 MTPs.
2. **PUP registry on the NHD.** The data collection process via the NHD/MDSAS continues. Reminders for PwBD updates are sent quarterly.
3. **Access to Veyvondi for children**

   Veyvondi is commissioned for adolescent children on the same basis as adults (surgery and bleed treatment) after completion of a Blueteq form and is now available for younger children (on the same basis), pending the paediatric license. Documentation of an paediatric MDT discussion is required. Individual paediatric CCC's have rolled this out to their families in varying ways.
4. **The national advisory group/MDT** continues 3 monthly teleconferences, with terms of reference, for discussion and recording of outcomes. Calls for cases to discuss are sent out to paediatric treating centres prior to the dates with a data pro forma. Ad hoc emailing of complex cases in between meetings is possible if there is a clinical necessity, with follow up and minuting at the next meeting. Minutes are circulated and saved at the NHD.
5. **PWP membership at other UKHCDO meetings:** Inhibitor WP, DMWP, DAG and Adverse events panel.
6. **Current membership** has been in place for 5 years and is due for renewal - an email with calls for interest has been circulated

7. **Guidelines relevant to WP:** Prophylaxis and Inhibitor, both of which should be reviewed in the coming year.

Dr Mary Mathias
Chair, Paediatric Working Party
September 2025

15. Peer Review Working Party

Membership

Chair

Dr Sarah Mangles, Southern Haemophilia Network (Basingstoke)

UKHCDO representatives

Pratima Chowdary (Royal Free)

Kate Talks (Newcastle)

Gary Benson (Belfast)

John Grainger (Manchester Childrens)

Joannes Hermans (Nottingham)

Gillian Lowe (Birmingham)

Gillian Evans (Kent and Canterbury)

Keith Sibson (GOSH)

Haemophilia Nurse Association representatives

Elsa Aradom	(Royal Free)
Amy Conquergood	(Oxford)
Kate Forsyth	(Barts and Royal London)
Emma Franklin	(Bristol)
Lara Oyesiku	(Basingstoke and SHN)
Julia Spires	(GOSH)

HCPA representatives

David Hopper	(Newcastle)
Stephanie Taylor	(Oxford)

Psychology representatives

Grainne O'Brien	(Edinburgh)

PwBD representatives

Nigel Miller	(Haemophilia Wales)

Debra Morgan (Haemophilia Society - England)

Alan Martin (Haemophilia Society - Scotland)

Project manager

Harry Evans

Remit

To review previous peer review standards and update

To work with project manager to ensure all CCC, interim CCC and HC are audited in each peer review cycle

To ensure training of all peer reviewers

To write summary report once all peer reviews completed

Meetings

Virtual meetings to discuss peer review standards, report templates, plan for peer review, as well as email review of documentation.

Activities

1. All peer review audits completed in 2024 - 29 CCC, 2 interim CCC and 1 HTC
2. Process to review and standardise reports before sending out to all centres set up with UKHCDO committee members
3. All reports once finalised will be uploaded to UKHCDO website
4. Questionnaire to be sent to all HCs in 2025 - less than 50% response rate, centres then called by member of working party to obtain more information
5. Summary report to be completed autumn 2025
6. Plans to revisit standards autumn 2025, arranged in themes and ensure no overlap and distribute for comment 2026

Dr Sarah Mangles

Chair, Peer Review Working Party

September 2025

16. Von Willebrand Working Party

Date of inception: 2020

Meetings since last AGM: 05.12.2024, 17.03.2025, 14.05.2025, 17.09.2025

Current membership

Cathy Farrelly

Vince Jenkins

Will Lester

Carolyn Millar (Chair)

Susie Shapiro

Purpose

The purpose of the Working Party is to:

1. Monitor, review and update BSH/UKHCDO Guidelines on the Clinical Diagnosis and Management of VWD
2. Registration, update and reclassification of PwVWD within the National Haemophilia Database.

The principal focus of the VWD working party continues to be the revision and update of the BCSH/UKHCDO 2014 guidelines. A writing group was formed in 2023 to develop a BSH guideline for the clinical diagnosis and management of VWD, approval was obtained from the BSH taskforce and guideline committee in 2024. This complements the recently published guideline for the laboratory diagnosis and monitoring of VWD. The 1st draft guideline was submitted to BSH taskforce in September 2025, with the aim to publish in early 2026.

The WP has worked with the NHD to develop the new PwVWD registration process and work continues on the update, reclassification and removal of people from the NHD.

Dr Carolyn Millar,
Chair, Von Willebrand Working Party
September 2025

17. Dental Task Force

Membership

Dr Julia Anderson (Chair, Consultant Haematologist)

Dr Sara Boyce (Secretary, Consultant Haematologist)

Mr Andrew Brewer (Associate Specialist, Oral Surgery)

Dr Janet Davies (Consultant Paediatric Dental Surgeon, British Paediatric Dental Association representative)

C/N Jayne Keaney (Haemophilia Nurses Association)

Dr Bella Madan (Consultant Haematologist)

Dr Lochana Nanayakkara (Consultant Dental Surgeon)

Dr Emily Symington (Consultant Haematologist)

Dr Alice Taylor (Consultant Paediatric Haematologist)

Dr Albert Yeung (Public Health Dentist)

The Dental Task Force met on 12 October 2024, 3 December 2025 18 March 2025, 3 June 2025 and 19 July 2025.

The group membership consists of adult and paediatric haemophilia consultants, adult and paediatric dental and oral surgeons, and nurse representatives. The group are finalising a new set of guidance and aiming to publish a 2-part serial in the British Dental Journal. The guidance is primarily intended for dental practitioners, and also haemophilia treaters.

There are sections on gene therapy, extended half-life products, emicizumab, local anaesthesia and an updated section on dental procedures.

The manuscript has had comments from Haemophilia Scotland, Northern Ireland and Wales, in addition to the British Society Special Care Dentists, and is awaiting comments from the Haemophilia Society, British Society of Oral Surgeons and the British Paediatric Dental Association. After an updated literature review the draft will be circulated around the UKHCDO Advisory Board for comment.

Julia A M Anderson

Chair, UKHCDO Dental Task Force

August 2025

18. Emergency Care Guideline Re-write Task Force

Over the course of the last year, the group has published an educational "Practice Review" in the Emergency Medicine Journal (Management of haemophilia and heritable bleeding disorders in the ED *Emergency Medicine Journal* Published Online First: 27 June 2025. doi: 10.1136/emermed-2024-214669) This review is aimed at emergency medicine physicians and health care professionals and conveys simple messages aimed at supporting safer practice when people with bleeding disorders attend the Emergency Department.

The group has also completed the writing of the updated and revised UKHCDO emergency care guidance and, at the time of producing this report, submission of the manuscript to Haemophilia is underway. The guidance is in line with the NHSE service specification and UKHCDO peer review standards. There are 14 standards in total, emphasising the need for rapid triage and assessment, prioritising treatment with haemostatic products over investigation, and promoting early haemophilia specialist advice. The new guidance also addresses more specific recommendations in relation to ambulance transport and emphasises the use of electronic alerts to aid rapid PwBD recognition. The use of emergency care plans whenever possible is recommended. We have addressed the need for more robust arrangements for making commonly used coagulation products available in district hospitals to which PwBD may present. The guidance contains a specific section on novel therapies to encourage rapid escalation of PwBD using these treatments to a haemophilia specialist who is familiar with these treatments. In line with the IBI recommendations to strengthen networks, we have promoted the use of networking arrangements to provide more secure cover when necessary for smaller haemophilia treatment centres. We have also acknowledged the potential valuable role in emergency care of advanced clinical practitioners, as an emerging professional group in the care of people with bleeding disorders. The role of provision of an appropriate level of training to PwBD, and to all health care professionals who may be involved in emergency care of our PwBD group is also incorporated in the standards. Finally, for implementation of better emergency care, we have highlighted that the standards should be promoted and embedded in clinical governance structures within hospitals in all relevant departments to which people with bleeding disorders may present. Some KPIs are suggested.

In addition to the writing output of the group, we have engaged with the Royal College of Emergency Medicine (RCEM). The RCEM Quality Committee is producing a "Best Practice Guideline" with some assistance from our group.

The work of the group will also be represented at the Haemophilia Society's annual "Big Get Together" in November.

Lishel Horn

4 November 2025

19. Haemophilia Gene Therapy Hub Panel

Membership

1. Catherine Bagot and Emily Symington - Co-chairs
2. Representation from each of the gene therapy hub centres
3. Will Horsley - Lead commissioner for specialised blood disorders for NHSE
4. Susan Caldwell - Senior Research Nurse, UKHCDO
5. Rob Hollingsworth - National haemophilia database
6. Greg Shone - Administrator, UKHCDO

Introduction

The Haemophilia Gene Therapy Hub Panel was established in its current form in September 2024. Over the last year the panel has met monthly to support the setup of the gene therapy hub centres, according to UKHCDO and NHSE guidelines. This annual report outlines the key activities, progress, and future goals of the hub panel, reflecting on the collaborative efforts made to ensure the safe and effective implementation of gene therapy for the treatment of haemophilia in England and the devolved nations.

Meeting Frequency and Format

- Monthly meetings of the panel with CSL Behring joining for the last 30 minutes
- Each meeting is 60-90 minutes
- All meetings have been virtual to date

Activities and Achievements

The primary purpose of the Haemophilia Gene Therapy Hub panel in this first year has been to support the setup of the commissioned gene therapy hubs. This has included:

- Hub readiness including CSL Behring onboarding,
- Trust concerns regarding information governance, indemnity, and AAV5 antibody testing
- Support and advice for the hubs and spokes who have dosed the first people with gene therapy

Guidelines and Review Status

The Haemophilia Gene Therapy Hub panel is responsible for several guidelines that help govern the safe and ethical use of gene therapy in clinical settings. The following documents were written in 2024/2025:

- UKHCDO PwBD information leaflet for Gene Therapy with Hemgenix

- Healthcare Professional checklist for pre-consent PwBD discussion and consent form
- Panel's Terms of reference

Future Plans

Looking ahead, the Haemophilia Gene Therapy Hub panel to focus on the following key areas in 2025:

- Transitioning to the title of National Haemophilia Gene Therapy Panel once all the hubs are established in NHS England
- Supporting the haemophilia gene therapy hubs as more people are treated
- Supporting timely and accurate data entry, pertaining to people who are receiving gene therapy, into the NHD
- Supporting the devolved nations in setting up gene therapy hubs by sharing experience accumulated to date

Dr Catherine Bagot and Dr Emily Symington

Co-chairs, Haemophilia Gene Therapy Hub Panel

October 2025

20. Haemophilia Nurses' Association

HNA-UK 2024-2025 Report Committee:

Chair: Marie Eales (Oxford University Hospitals)
Vice Chair: Helen Hupston (Queen Elizabeth Hospital Birmingham)
Secretary: Hannah Larkin (Royal Free Hospital, London)
Treasurer: Natalie Gingell (Birmingham Children's Hospital)
Committee member and Education Leader: Julia Spires (Great Ormand St Hospital)
Committee member: Dawn Jenner (Southampton General Hospital)
Committee member: Clare James (Evelina Children's Hospital, London)
Committee member: Katie Moore (Cardiff University Hospital)
Committee member: Geraldine Green (University Hospitals Coventry and Worcester)
Education Team:
Director of Education: Debra Pollard (Royal Free Hospital, London)
Education leader: Cathy Harrison (Sheffield Teaching Hospital, Sheffield)
Education leader: April Jones (Newcastle Upon Tyne HNS Foundation Trust)
Education leader: Sarah Pool (Oxford University Hospital NHS Trust)
Education leaders (Physiotherapist Experts): *Paul McLaughlin and Melanie Bladen*

HNA-UK members: Current membership as of 20.11.2025: Approx 211 members

In partnership with The Haemophilia Society (registered charity no 288260 in England and Wales, SCO39732 in Scotland)

Introduction

The Haemophilia Nurses Association UK (HNA-UK) continues to advocate for specialist nurses and advanced practitioners committed to caring for individuals with bleeding disorders throughout the UK. Following another highly successful meeting in January 2025, the HNA-UK is planning for the next exciting gathering moving to the spring of 2026. The date of the meeting is now March 6th-7th 2026. It has been a busy year for many and over the past year, the HNA-UK committee has met both virtually and in person to strategically advance our objectives and support the continued professional development of our members.

The HNA-UK Education team, who are nurses, physiotherapists and medical professional have been working considerably hard to maintain the education and training for our nurses and other allied health professionals in haemophilia centres across the country.

Committee Changes

In the summer, the HNA-UK committee went through some changes. Debra Pollard, our Director of Education, formally stepped down from the HNA-UK committee this year. Debra leaves behind an unparalleled legacy, having been one of the association's most passionate advocates. We are deeply grateful for her dedication, and we will continue to champion her work and uphold the standards she helped to establish.

The committee welcomed new members to support the delivery of upcoming initiatives and contribute to shaping future goals for haemophilia nursing in the UK. Our new committee members Dawn, Clare and Geraldine will bring fresh new ideas to the committee meetings and help in the planning and delivery of our next meeting in March. As per our Terms of Reference 2024, committee members will hold office for a period of 5 years, with the option of being reelected for a further three-year period. We remain committed to fostering collaboration and peer support, and we aim to ensure these values remain central to our committee's work.

Partnership with The Haemophilia Society

Our ongoing partnership with **The Haemophilia Society** has continued to provide financial and administrative stability to HNA-UK. This collaboration has been instrumental in supporting the organisation of our annual meetings and AGMs, as well as in delivering high-quality educational programmes for nurses working in the field of bleeding disorders.

Education

Although Debra has stepped down from the committee, she continues to play an active role in our education team. She works closely with nursing colleagues **Cathy, April,** and **Sarah** to deliver:

- **Introduction to Bleeding Disorders**
- **Contemporary Care Course (CCC)**

These courses are now an essential part of the educational portfolio within haemophilia centres and are a **peer-review requirement** for allied health professionals within their first two years of joining a bleeding disorders multidisciplinary team (MDT). In the last year there have been 4 "Introduction to Bleeding Disorders" Courses attended by >80 health care professionals (HCP's), this course remains free for all. 17 HCP's completed CCC 2024 and 23 HCP's are currently working through CCC 2025.

The CCC continues to demonstrate strong engagement and maintains its accreditation through Middlesex University, reflecting its high standards and robust quality assurance. Designed and delivered by experienced nurses and physiotherapists from haemophilia centres, with contributions from external experts, the CCC covers all aspects of multidisciplinary care for individuals with bleeding disorders.

Nursing Support During the Infected Blood Compensation Scheme

Nurses across the UK have played a vital role in supporting individuals and families affected by the Infected Blood Inquiry and subsequent compensation schemes. For many PwBD, nurses have been the first point of contact—providing not only clinical guidance but also emotional support during what has been an incredibly difficult and traumatic time. In many cases, nurses have gone above and beyond their traditional roles, acting as trusted intermediaries between PwBD, legal representatives, and health services. HNA-UK strives to support our nursing members in seeking peer support and sharing their experiences across the country, fostering a culture of collaboration and shared learning

As the compensation schemes continue to evolve, the contribution of HNA-UK members remains critical. Their involvement not only facilitates access to justice and financial redress but also helps PwBD feel heard, supported, and respected throughout the process.

Past and Future

We continue to work hard to adapt to changes within our teams and continue to deliver exceptional care to our PwBD. We are proud to see many of our nursing members practising at an advanced level, bringing enhanced autonomy and expertise to their roles. We are keen to support nurses who wish to attain future professional development opportunities and signpost to suitable courses or offer support when submitting work.

We have bid farewell to several colleagues across the country who have retired or transitioned to new roles, and we wish them all the best in their future endeavours.

Looking ahead to 2026, we are excited about upcoming opportunities for education and international networking through key events such as EAHAD and ISTH. We encourage centre directors to support their nursing teams in attending these events.

Our members continue to be actively involved in a wide range of initiatives, including: Steering committees for EAHAD and WFH, the Clinical Reference Group (CRG) for Specialised Blood Disorders and several UKHCDO working parties.

We are incredibly grateful to everyone who has contributed to these groups and provided valuable feedback, ensuring the national voice of haemophilia nursing remains strong and wellrepresented.

Marie Eales (Chair on the HNA-UK)

Haemophilia Nurses Association UK

Marie, Hannah, Katie, Helen, Julia, Natalie, Dawn, Clare & Geraldin

HNA- UK Committee

21. Haemophilia Chartered Physiotherapists' Association

The HCPA consists of Specialist Physiotherapists working in Haemophilia and Allied Bleeding Disorders services across the UK and Ireland. We aim to define, promote and encourage best practice for physiotherapy within haemophilia care, providing professional leadership and directing national physiotherapy policy.

Executive Committee:

Chair: Rob Barnard
Vice Chair: Jim Cartwright; Caroline Clegg
Secretary: Katie Lindsay
Treasurer: Steph Taylor (Kate Millar incumbent October 2025)
Research Lead: Prof. David Stephensen

General Committee: David Hopper (former chair); Zamira Jaffer; Mark McGowan; John Snaith

The committee was elected at the 2025 AGM in February. Executive committee members will be able to stand for a second term at the 2027 AGM.

National Service Specification

David Hopper led HCPA representation in the formulation of new service specifications. HCPA members contributed to the public consultation. Rob Barnard will now represent on this group as HCPA chair.

HCPA Constitution

The HCPA Constitution has been revised in 2025. Notable additions include the creation of a General Committee to support the Executive Committee. From 2025, retiring Executive Committee members will join the General Committee for 1-2 years, to support the transition of the new Executive Committee.

HCPA-Constitution-2025.pdf

Physiotherapy Standards of Care

The Service Provision of Physiotherapy, for both adults and paediatrics have been updated in 2025. They are available on the UKHCDO Website.

Adult Service Provision of Physiotherapy in Haemophilia and Other Inherited and Acquired Bleeding Disorders

Children's Service Provision of Physiotherapy in Haemophilia and Other Inherited and Acquired Bleeding Disorders

Working Parties

Working Party	HCPA Representative
Musculoskeletal	Dr Paul McLaughlin
Peer Review	David Hopper; Steph Taylor
Emergency Guidelines	David Hopper
National Service Specification	Rob Barnard
Comorbidities	Steph Taylor; Louise Crossley
Data Management	Prof David Stephensen
Gene Therapy	David Hopper; Steve Classey

Ongoing Research

HCPA members are actively involved in numerous research projects. Currently key focus is on the development of a competency framework for the use of point of care ultrasound in bleeding disorders, understanding the needs of women and girls with bleeding disorders and the Physiotherapists' that care for them. Identifying physical performance screening tools for routine PwBD care and expanding best practice to support our aging population.

AGM

The AGM was held virtually, on 3rd April 2025. 19 members attended. The new committee were formally elected, with transition of roles occurring following the 2025 Educational Conference.

Educational Conference

Held at the Clayton Hotel on 12/13th June 2025. With 43 members attending (including 7 virtual). This meeting was kindly sponsored by CSL Behring, Novo Nordisk, Roche, Sobi.
The first day of conference focused on the management of hypermobility, with theoretical and practical aspects. The second day hosted a range of expert presenters from within our field,

as well as Orthopaedics and elite sport. This day included a free papers session for members to showcase their work in the format of a five-minute oral presentation. This conference hosted the inaugural HCPA Debate

International Conferences

EAHAD 2025

HCPA members presented posters at EAHAD in Milan, with Mark McGowan and colleagues recognised with a top poster award for Physiotherapy. Dr Paul McLaughlin won the SLAM award, with Prof. David Stephensen also presenting. Dr Megan Kennedy received an EAHAD Research Grant for her EMPOWER study.

WFH 2025

HCPA members attended WFH in Dubai, where they participated in oral presentations and poster submissions.

International Representation

International Prophylaxis Study Group (IPSG)

Prof. David Stephensen is co-chair, with Mel Bladen a member of this group. IPSG are a collaborative group of health care professionals involved with the assessment and care of individuals with inherited bleeding disorders, which is currently exploring the utility and modification of the Haemophilia Joint Health Score (HJHS)

EAHAD

Dr Paul McLaughlin and Paula Loughnane currently sit on the EAHAD Physiotherapists' Committee.

This committee will be calling for nominations in 2026 and the HCPA will encourage members to apply.

Publications 2024/25

1. Chowdary P, Duran B, Batty P, Lowe G, Jones A, Pollard D, Boyce S, Motwani J, Amirloo B, Musgrave K, **Hopper D**, **Classey S**, Whitaker S, Dunn N, Bowyer A, Shapiro S. UKHCDO gene therapy taskforce: Guidance for implementation of haemophilia gene therapy into routine clinical practice for adults. Haemophilia. 2025;31(1):26-38. doi:10.1111/hae.15125.
2. Horn EH, Forsyth K, Gooding R, Gorman R, Gosrani D, **Hopper D**, Mainwaring J, Percy CL, Thomas W, Smith S; UK Haemophilia Doctors Organisation Task Force on Emergency Care. Management of haemophilia and heritable bleeding disorders in the ED. Emerg Med J. 2025:emermed-2024-214669. doi:10.1136/emermed-2024-214669. Epub ahead of print.

3. **Wells A**, Gray D, Husted M, **Stephensen D**. The effects of traumatic pain memories on current pain experience in men with hemophilia. Can J Pain. 2025;9(1):2530966. doi: 10.1080/24740527.2025.2530966.
4. **McLaughlin P**, Chowdary P, Harrison C, **Stephensen D**, Roussel N. Gap Analysis Investigating Healthcare Professionals' Skills and Knowledge for Pain Management in Haemophilia-A Qualitative Study. Haemophilia. 2025;31(5):988-996. doi:10.1111/hae.70112.
5. McKeown W, **Taylor S**, Shapiro S. Frailty and frailty syndromes in persons with hemophilia: a review. J Thromb Haemost. 2025:S1538-7836(25)00470-2. doi: 10.1016/j.jtha.2025.07.020.
6. **McLaughlin P**, De la Corte-Rodriguez H, Burke T, Nissen F, Aizenas M, Moreno K, O'Hara J. An assessment of burden associated with problem joints in children and adults with moderate or severe haemophilia A: analysis of the CHESS-Paediatrics and CHESS II cross-sectional studies. Orphanet J Rare Dis. 2025;20(1):18. doi:10.1186/s13023-024-03514-1.
7. **Holdsworth C**, **Bladen M**, **Harbidge H**, Drechsler W, Mahaffey R, Perez-Alenda S, **Sayers F**, Strike K, Timmer M, **Stephensen D**. Measuring Physical Function Capacity in Persons With Haemophilia: A Systematic Review of Performance-Based Methods. Haemophilia. 2025;31(5):840-864. doi: 10.1111/hae.70081.
8. Kelly C, **McGowan M**, Larkin N, Mc Donnell JM, Hilshof AM, Byrne M, Bergin C, O'Gara A, Ryan K, O'Donovan M, O'Connell N, Synnott K, Butler JS, Darwish S, O'Mahony B, **Kennedy M**, Turecek PL, O'Donnell JS, Gormley J, Lavin M. Spinal Stenosis: An Emerging Complication of Ageing in People With Haemophilia. Haemophilia. 2025:31:5:1043-1053. doi: 10.1111/hae.70089.
9. **McLaughlin P**, Chowdary P, Khair K, Smith C, **Stephensen D**, Hurley M. Exercise-based telerehabilitation for the management of chronic pain in people with severe haemophilia: a mixed-methods feasibility study. Pilot Feasibility Stud. 2024;10(1):128. doi:10.1186/s40814-024-01550-z.
10. Mancuso ME, **McLaughlin P**, Forsyth AL, Valentino LA. Joint health and pain in the changing hemophilia treatment landscape. Expert Rev Hematol. 2024;17(8):431-444. doi: 10.1080/17474086.2024.2378936.
11. Blokzijl J, Pisters MF, Aspdahl M, de Boer W, Dybvik Matlary RE, Douma-van Riet D, de Kleijn P, Lobet S, **Loughnane P**, **McLaughlin P**, **Bladen M**, Roche S, **Stephensen D**, van Vlimmeren L, van Vulpen LFD, Timmer MA; EAHAD physiotherapy committee. A clinical practice guideline for primary care physiotherapy in patients with haemophilia. Haemophilia. 2024; 30(5):1115-1129 doi: 10.1111/hae.15065.
12. **Taylor S**, Barker K, **Stephensen D**, Williamson E. Using evidence-based co-design to develop a hybrid delivered exercise intervention that aims to increase confidence to exercise in people with haemophilia. Haemophilia. 2024; 30(3):817-826. doi: 10.1111/hae.14972.
13. Laffan M, **McLaughlin P**, Motwani J, Alamelu J, Austin S, **Classey S**, Dolan G, Eales M, Gooding R, Grainger J, Harrison C, Jones A, Kelly AM, Oyesiku L, Rodgers R,

Stephensen D, Talks K, Sonecha S, Danquah A. Expert United Kingdom consensus on the preservation of joint health in people with moderate and severe haemophilia A: A modified Delphi panel. Haemophilia. 2024;30(2):306-319.

2025/26

Clinical and Academic

The HCPA will continue to encourage members to take active steps in research activities.
The HCPA will support ongoing service specification review, providing recommendations on staffing provisions.

Meetings

AGM to be held in Spring 2026
Educational Conference to be held in June 2026

Andrew Clements Award

The HCPA will present the inaugural Andrew Clements Award at the 2026 Educational Conference, for outstanding contribution to the bleeding disorder community. This in in commemoration of our friend and colleague Andrew Clements, who sadly passed away this year.

Rob Barnard
Chair, Haemophilia Chartered Physiotherapist Association
September 2025

22. Haemophilia Psychology Association

The Haemophilia Psychology Association (HPA) is made up of Practitioner Psychologists working within the NHS. There has been a significant increase in our membership over the past 12 months due to the creation of new psychology posts in NHS England, to support individuals and families, impacted by Infected Blood. These posts are located within Haemophilia centres. A primary role of the HPA is to support and educate those new to working in the area.

We meet virtually once a month for case discussions and sharing of best practice and peer support. This has been particularly helpful for Inquiry information sharing, considerations around the establishment of the English Infected Blood Psychology Service and discussions of Gene therapy psychology support and the practicalities of how best to provide this.

The HPA had a recent CPD event on Gene therapy, with a useful post-meeting discussion about the creation of an agreed upon psychology-specific guidance document/proforma to support parity of access to psychology.

Members from the HPA have represented the group at meetings and working groups for the development and implementation of the English Infected Blood psychology service, the updating of the commissioning standards for Haemophilia Centres and the Peer Review Working Group. Members have worked alongside the Haemophilia support groups across the nations whenever possible to support their PwBD events and work.

The HPA has representation on the Psychosocial committees of both the World Federation of Haemophilia and the European Association for Haemophilia and Allied Disorders, with presentations from members planned at both Congresses in 2026.

Dr Gráinne O' Brien
Clinical Psychologist & Chair, Haemophilia Psychology Association
September 2025

23. British Society for Haematology Haemostasis & Thrombosis Task Force

Membership

Dr Will Lester (Chair)
Dr Ian Jennings
Dr Stella Williams (MHRA Representative)
Dr Karen Breen
Dr Lara Roberts
Dr Jayashree Motwani
Dr Martin Scott (UKHCDO Representative)
Dr Nicola Curry
Dr Renu Riat
Dr Khalid Saja
Dr Anette Bowyer
Dr Suzanne Maynard (Taskforce Trainee)
Ms Roslind Bird
Ms Emma Gee
Mr Sean Platton (Vice Chair)
Mr Peter Baker
Mr Stephen MacDonald

Meetings

Dr Lester chairs the BSH H&T Task Force, and Sean Platton is Vice-Chair. The Task Force met on 10 January 2025, 24 March 2024, 10 June 2024 and 17 September 2025

UKHCDO Guidelines and other publications published 2025

Reporting haemostasis assays in IU/dL–A recommendation for harmonisation (BSH H&T Task Force / UK NEQAS / UK HCDO)

Management of haemophilia and heritable bleeding disorders in the ED. Elizabeth Helen Horn, Kate Forsyth, Richard Gooding, Richard Gorman , Deepan Gosrani, David Hopper, Jason Mainwaring, Charles Llewellyn Percy , Will Thomas, Simon Smith. Emergency Medicine Journal Published Online First: 27 June 2025. doi: 10.1136/emermed-2024-214669

BSH Guidelines Published 2025

Guidelines on the investigation and management of antiphospholipid syndrome

Measurement of heparin, direct oral anti-coagulants and other non-coumarin anti-coagulants and their effects on haemostasis assays

Guidelines in Preparation:

Guideline for the management of hepatitis B and C infection and subsequent liver disease surveillance (where indicated) in people with congenital bleeding disorders: a joint guideline from the British Society of Haematology and the British Viral Hepatitis Group. Writing group Chair: Dr S Shapiro

Investigation and Management of Acquired Coagulation Factor Inhibitors: A joint BSH/UKHCDO guideline. C Percy, A Riddell, K Talks, J Anderson, S Cotton, R Riat, K Saja, M Makris. Writing group chair: Dr C Percy.

Clinical diagnosis and management of von Willebrand Disease (VWD). Writing group chair: Dr C Millar

BSH guideline of D-Dimer testing. Writing group chair: Dr W Lester

Guidance on the Dental Management of Patients with Haemophilia and Inherited Bleeding Disorders (UKHCDO guideline: working party members: Chair: Dr J Anderson, Sara Boyce, Andrew Brewer, Janet Davies, Jayne Keaney, Bella Madan, Lochana Nanayakkara, Emily Symington, Alice Taylor, Albert Yeung.

Dr Martin Scott

UKHCDO Representative for BSH Haemostasis and Thrombosis Taskforce

October 2025

24. Haemophilia Society

This year we experienced unprecedented demand for our services and have had to manage our limited resources carefully to meet the diverse needs of the bleeding disorders community.

Maintaining in-person events, providing information and advocating for change amid a backdrop of diminishing funding sources continues to be a challenge for our small charity.

We have 5,500 members from across the UK living with or caring for someone with a bleeding disorder who look to us to help them get the best treatment and care. As well as members with haemophilia A and B and von Willebrand disorder we also support people with rarer conditions, including Glanzmann thrombasthenia and factor V deficiency.

A major part of our work this year has been in support of the contaminated blood community. The government's Infected Blood Compensation Scheme, which was published in August 2024, was welcome but its complexity created uncertainty and stress. With very little information available from either the government or the Infected Blood Compensation Authority (IBCA), many people turned to the Haemophilia Society for help. We became experts in the scheme and related issues in order to support our members.

We liaised regularly with IBCA and the Cabinet Office to try to improve the system and to highlight areas where the compensation scheme was not working. An intervention by the Infected Blood Inquiry earlier this year strengthened our arguments and important improvements to the compensation scheme have now been accepted by the government.

We were also members of working groups to look at the Infected Blood Inquiry's recommendations on improvements in haemophilia care, we sat on an expert advisory panel on the new Infected Blood Psychological Service and we are a founder member of the Haemophilia and Bleeding Disorder Alliance that brings together PwBD and clinician groups from across the UK to push for higher standards and better access to treatment and care.

In addition to our contaminated blood campaigning and advocacy, we have continued to support our community through events, research and publications.

Our Big-Get-Together conference in November 2024 and our Newly Diagnosed Weekend for parents with children recently diagnosed with a bleeding disorder in April 2025 brought our members together to share experiences at the free events and hear the latest treatment news from specialist clinicians.

We were honoured to host a seminar with the Royal Society of Medicine in London in February 2025 which brought the challenges of bleeding disorder care to a new audience, including resident doctors and trainee physiotherapists. The event looked to the future and, thanks to our expert panels, offered insight and thought-provoking discussion about what lies ahead.

Our 12 booklets and 19 downloadable factsheets on all aspects of bleeding disorder care continue to be in demand from haemophilia centres as well as individuals.

The launch of our ground-breaking report, 'Underserved, Overlooked: How our health systems are failing women and girls with a bleeding disorder', into the treatment of women girls with a bleeding disorder in July 2025 was the culmination of a three-year research project. The report highlights the urgent need to tailor more services to women and girls' needs and for more research to be

commissioned into treatment. With the support of clinicians and trusts, we hope our report will drive up standards and promote a fresh approach to supporting women and girls with a bleeding disorder. We look forward to working with haemophilia centres on this issue in the year ahead.

This year marked a change in our leadership, with Conan McIlwrath taking over from Clive Smith as our Chair of Trustees. Clive has accepted the role of THS President, so remains an important part of our charity.

As always, we are thankful for the support of many specialist haematology professionals in volunteering at our events and for providing advice and sharing expertise. Together we will try to ensure that everyone with a bleeding disorder receives the care and advice they need.

Kate Burt,
CEO, The Haemophilia Society
September 2025

25. Haemophilia Scotland

Haemophilia Scotland's vision is for Scotland to be a place where people with haemophilia, von Willebrand and other rare bleeding disorders, along with their families and carers, can live healthy, happy lives.

Membership is free and open to anyone affected, including family, friends, carers, and healthcare professionals.

Events and Projects

We continued with our ongoing objective this year to organise and host more accessible in-person events for members, including four *Learn and Connect* meetings, our Over 50s Activity Weekend, and our AGM/Members Day in Stirling.

The year began with a small delegation attending the World Federation of Haemophilia Congress in Madrid, offering the chance to hear from leading experts around the world on future treatments and advances in care. The *Learn and Connect* events in Inverness, Aberdeen, Edinburgh and Dundee encouraged networking, provided opportunities for attendees to share their experiences, and introduced our new Financial Wellbeing Service, offering members specialist advice and support on benefits and welfare matters.

Other highlights included Comic Book workshops in Edinburgh and Elgin to help shape our next *Living with a Bleeding Disorder* comic which is due for publication in late summer 2025, and the Over 50s weekend featuring sessions on wellbeing, pain management, and creative activities such as photography and art.

Engagement with Other Organisations

Haemophilia Scotland works closely with PwBD organisations that share our focus on health, wellbeing, and education, enabling us to share expertise, pool resources, and enhance support for our community.

Collaboration with other PwBD organisations and campaign groups increased significantly following the publication of the Infected Blood Inquiry's Final Report in May 2024, as we worked together to engage with government to highlight questions and concerns from the wider community regarding the progress in implementing the report's recommendations.

In September 2024, UK PwBD organisations and clinical representatives formed the Haemophilia and Bleeding Disorders Alliance (HBDA) to tackle key challenges in care and treatment. The Alliance has since met five times to review the Inquiry's recommendations and findings from the 2023-2024 UK Peer Reviews.

Regular contact has further strengthened our relationships with Scotland's haemophilia centres, leading to increased membership to the charity through greater awareness, more referrals to our Financial Wellbeing Service, and generous support from clinical staff at our events. An ongoing joint project with paediatric nurses is developing improved, standardised resources for school and nursery visits.

Advocacy

Advocacy remains central to Haemophilia Scotland's mission, ensuring the voices of people with bleeding disorders in Scotland are heard and their needs addressed.

This year, our work was shaped by the Infected Blood Inquiry's Final Report, which exposed decades of systemic failings and made 12 recommendations, including a compensation scheme for infected and affected individuals. We engaged with both the UK and Scottish Governments as the outcomes from the Inquiry recommendations evolved, and the scheme was established.

We joined the Scottish Government's Oversight and Assurance Group to explore implementing the recommendations in Scotland, and continued to support the Infected Blood Tribute Fund, led by community members, to create a permanent memorial honouring those lost and recognising the suffering caused by the infected blood disaster.

We also worked closely with haemophilia centres, NHS Scotland, and clinicians to improve treatment access and resolve individual care issues. In June, we submitted written and oral evidence to the Scottish Medicines Consortium on a new gene therapy for haemophilia B, which received provisional approval in August 2024. We remain committed to ensuring the PwBD voice is included in the decision-making processes for future treatment assessments.

Alan Martin

Director, Haemophilia Scotland

August 2025

26. National Haemophilia Database - IT Update

We're pleased to share the latest updates on the National Haemophilia Database (NHD) IT systems, highlighting recent developments, ongoing enhancements, and upcoming features designed to support the NHD, clinical teams and PwBD. As the digital landscape continues to evolve, we remain committed to ensuring the NHD infrastructure is secure, efficient, and aligned with the needs of all bleeding disorders stakeholders. This update provides insight into developments during the year, including improvements in data quality, systems and security. Thank you to all Haemophilia stakeholders for your continued collaboration and support of NHD IT systems.

Haemtrack (Home Therapy Platform)

The Haemtrack (Home Therapy) Platform continues to evolve as a secure, user-friendly digital solution designed to support PwBD undergoing treatment at home, particularly those managing long-term conditions such as Haemophilia. Almost 3 million treatments have been entered by PwBD, enabling monitoring by clinical teams, which in-turn enhances the quality and safety of home-based care, improving outcomes, reducing hospital visits, and ensuring a more personalised healthcare experience.

This year, in addition to a number of functional updates, technological developments have been made which allow the rapid generation of new data collection templates, to support the introduction of quality-of-life evaluation tools. In addition, the new Haemtrack (Home Therapy) platform supports use for multiple different specialities. Utilising this ability, work is beginning on an expansion of its use for new projects such as women with bleeding disorders.

Haemophilia Clinical Information System (HCIS 2)

The HCIS 2 system is now implemented and operational in a number of treatment centres and under testing in many more.

Rollout to Haemophilia Centres has been continuing throughout the year, though progress has not been as fast as we would have hoped. Largely this has been due to the need for reliance on internal hospital IT departments for implementation processes. Required governance processes (different at every site) and obtaining the necessary implementation requirements have been challenging. A number of adaptations in rollout processes have been carried out to help fast-track progress with local IT departments.

The new HCIS system offers many benefits over the old platform, with multiple improvements to security, functionality and technology stack. New version releases have been introduced in relation to feedback from sites as rollout progresses. A key strength of the new HCIS system is the ability to rapidly deploy new version releases when functionality updates are required.

We would very much appreciate support from local centres in the rollout process, particularly in relation to communications with IT departments to obtain the necessary IT requirements for HCIS 2 implementation.

Gene Therapy

With the advent of PwBD now starting to receive Gene Therapy, the new NHD Gene Therapy Database is successfully being used to record details of this therapy. Positive feedback has been received in relation to the functionality and usability of the new database. Its functionality continues to evolve in response to feedback, to provide centres with functionality to support them in the administration and management of the gene therapy process.

Adverse Events

The new NHD adverse event system went live as planned during the year and has been successful in improving the management of adverse event reporting nationally. The new functionality allows NHD to have a full overview of all adverse events reported nationally. It also provides treatment centres with improved ability to manage the reporting and review of adverse events they have submitted.

The automated interface between the European Haemophilia Safety Surveillance System (EUHASS) has been successfully maintained, with the new adverse event forms being designed to ensure this. For UK centres participating in EUHASS, all adverse events reported to the NHD are automatically transferred to EUHASS with no additional effort required. Changes have recently been made to EUHASS to further simplify the reporting requirements for centres. Treatment centres that are not already participating in EUHASS, are encouraged to make contact should they wish to participate.

Consent

The NHD is about to re-start the PwBD consent process. To support this a new consent tool has been built into the NHD IT platform. This provides NHD with the ability to centrally audit and report on the consent process. It also provides centres with a full PwBD consent management function, highlighting PwBD that require consenting and providing the appropriate consent forms automatically.

Once PwBD consent has been obtained, centres can upload the completed consent form to the NHD using the new tool. Multiple mechanisms are provided to simplify the upload of consent forms.

A training programme for centres will be announced shortly, ahead of the planned go-live of the consent process.

Automated Data Quality Management

The new systems implemented to carry out automated data quality management have been operating successfully over the past year, validating data submitted by centres, improving data quality, and reducing manual burden for NHD.

The rules developed for data quality validation, across data items such as factor levels, diagnoses, inhibitors, product usage etc., have been refined and expanded, to increase the use of automated

data quality checking in the NHD. This has included updates to the NHD Validation Power BI dashboard, to improve overview of data quality at a national level.

UKHCDO Tenant

The UKHCDO Microsoft Teams tenant went live as planned during the year. The benefits of this are now fully realised with UKHCDO now having its own independent secure infrastructure, supporting Microsoft Teams, Email accounts, SharePoint document management, Microsoft Office licencing, and Calendars.

Security

We are pleased to announce this year our successful certification with the International Security Management System standard (ISO 27001), a significant milestone that reflects our commitment to constant improvements in information security. Achieving this internationally recognized standard demonstrates that we have implemented robust systems and processes to protect sensitive data, manage risk effectively, and continually improve our security practices. This accomplishment also strengthens our dedication to maintaining the highest standards of data integrity and confidentiality. It's a testament to the hard work and collaboration of the entire team.

Monitoring and review of security is a major ongoing focus, with adaption of security configurations and policies in relation to any threats identified. Regular cyber security meetings and audits are carried out, identifying new opportunities for improvements to our security infrastructure. Through this approach, several new software tools have been introduced during the year, enhancing the security infrastructure of the database.

Artificial intelligence (AI)

With the increase in availability of AI tools, it is important for us to assess how best AI could be used to support NHD operation. Used correctly it could significantly enhance efficiency, accuracy, and innovation across all areas of operation. With automation of repetitive tasks, allowing employees to focus on more strategic and creative work, and improving productivity.

However, while AI offers many advantages, it also comes with significant risks that must be carefully assessed. One major danger is data privacy and security, as AI systems rely on entry of information to return results, and contain large amounts of sensitive information, this makes them a target for cyberattacks and potential data misuse.

It is important that we embrace the benefits of AI, but in a structured and controlled way that minimises risk.

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