NATIONAL BLOOD AUTHORITY
AUSTRALIA

AUSTRALIAN BLEEDING DISORDERS REGISTRY

Annual Report 2020-21

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Purpose of this document

The Australian Bleeding Disorders Registry (ABDR) is a clinical registry for patients in Australia with bleeding disorders. It is used daily by clinicians in all Australian Haemophilia Treatment Centres (HTCs) to assist in managing the treatment of people with bleeding disorders and to gain a better understanding of the incidence and prevalence of bleeding disorders. Patients also contribute data to ABDR through the MyABDR app, which allows patients to record home treatments and bleeds.

This Annual Report summarises patient and product data from the ABDR and other National Blood Authority (NBA) sources to provide a high-level overview of who has bleeding disorders, how they are treated and what products are used. This report may be of interest to clinicians providing care to patients, patient advocacy organisations and government organisations.

For more information see www.blood.gov.au.

Key findings 2020-21 – patients and products

There were 7,040 patients active in ABDR as at 30 June 2021. Almost 36% of patients have hereditary haemophilia A (HMA), followed by hereditary von Willebrand Disease (VWD).

Patients	HMA (Hereditary)	HMB (Hereditary)	vWD (Hereditary)	Acquired and Other
Number of patients	2,529	601	2,460	1,450
Number of severe patients	725	111	148	
Patients who received product	1,117	253	312	142
Percentage of all patients	35.9%	8.5%	34.9%	20.6%

Bleeding disorder type and severity are the main determinants of whether a patient will require treatment with clotting factor products. In 2020-21, 80% of product was used by patients with HMA.

Products	HMA (Hereditary)	HMB (Hereditary)	vWD (Hereditary)	Acquired and Other
Factor FVIII or Factor IX (IU)	148,281,900 (FVIII)	26,673,500 (FIX)	9,724,500 (FVIII)	168,500 (FVIII)
FEIBA (IU)	939,000			357,000
NovoSeven (mg)	6,465	2,396		7,705
% of total FVIII & FIX IUs	80.2%	14.4%	5.3%	0.1%

Key findings 2020-21 – demand

Overall demand for clotting factors in 2020-21

10.7% of total cost of blood and blood products

Decreased from 13.7% in 2019-20

Demand for factor VIII

Decreased by 13.5% from 2019-20

→ Mostly due to the introduction of Hemlibra

- Recombinant VIII decreased by 13.2%
- Plasma derived FVIII decreased by 15.7%

Demand for factor IX

Increased by 0.5%

- Recombinant FIX increased by 0.5%
- Plasma derived FIX decreased by 2.5%

Source: NBA Annual Report 2020-21

Treatment of bleeding disorders in Australia

In Australia, and for the purposes of this report, bleeding disorders are grouped as set out in Table 1. There are also some patients with Fibrinogen and Vascular disorders. Patient numbers by disorder are provided later in this report. More detail on disorders and grouping is included at Appendix A: Bleeding Disorders.

TABLE 1 - MAJOR BLEEDING DISORDERS AND THEIR CAUSE

Disorder group	Cause
Haemophilia A	Deficiency of Factor VIII
Haemophilia B	Deficiency of Factor IX
von Willebrand Disease	Deficiency, or dysfunction, of von Willebrand Factor
Other Factor deficiencies	Deficiency of other coagulation factors
Platelet Disorder	Inherited deficiency of effective platelet function

Types of haemophilia

- The most common type of haemophilia is Haemophilia A. This means the person does not have enough clotting Factor VIII (factor eight).
- Haemophilia B is less common. A person with Haemophilia B does not have enough Factor IX (factor nine). The symptoms are the same for people with Haemophilia A and B; that is, they bleed for a longer time than normal.

Severity

Haemophilia A and B are classified according to their severity, as this informs the treatment regimens required. The definitions of severity that are applied within the ABDR are listed in Table 2. Definition of Severity definitions for VWD and other coagulation factor deficiencies are not standardised.

TABLE 2 - SEVERITY AND CONCENTRATION OF CLOTTING FACTORS [1, 2]

Severity	Concentration of Clotting Factor	Typical Bleeding Picture
Severe	<0.01 IU/ml (<1% of normal)	Frequent bleeding episodes common, predominantly into joints & muscles. Bleeding can occur spontaneously or after minor injury.
Moderate	0.01 – 0.05 IU/ml (1–5% of normal)	Can bleed after minor injury. May have joint bleeding. Severe bleeding with trauma, surgery, invasive procedures.
Mild	>0.05 – 0.40 IU/ml (>5-40% of normal)	Spontaneous bleeding does not occur. Bleeding with major trauma, surgery, invasive procedures.

Mild conditions may require no treatment or treatment only under special circumstances, such as surgery. More severe conditions may require regular interventions. Treatment may occur in hospital or

[1] Modified from Srivastava A, Brewer AK, Mauser-Bunschoten EP, Key NS, Kitchen S, Llinas A, Ludlam CA, Mahlangu JN, Mulder K, Poon MC, Street A; Treatment Guidelines Working Group on Behalf of The World Federation Of Hemophilia (2013). Guidelines for the management of hemophilia, Haemophilia 19(1):e1-47.

[2] Normal concentration of Factor VIII or IX is defined as 100% or one unit of Factor VIII activity per ml of plasma - 100 U/dL (Kasper, CK 2004, Hereditary plasma clotting factor disorders and their management. Treatment of Hemophilia Monograph Series, No. 4, World Federation of Hemophilia, Montreal, Canada). Levels of FVIII above 40% are usually considered sufficient for normal haemostasis.

other medical facilities, or at home. The treatments may be regular and preventative (prophylaxis), or on demand (when a bleed occurs). In some patients, therapy is complicated when their body develops inhibitors that destroy the replacement clotting factors and other treatment is necessary.

Often the treatments involve providing replacement for the missing or defective clotting factors. Products used include plasma derived and recombinant clotting factors, cryoprecipitate and Desmopressin (1-desamino-8-D-arginine vasopressin; DDAVP) which can stimulate the release of Factor VIII and von Willebrand Factor from stores in the body (this is not used in Haemophilia B or Factor IX deficiency).

Treatment of patients with bleeding disorders is managed through Haemophilia Treatment Centres (HTC). See Appendix B: Haemophilia Treatment Centres for details about the roles and services provided by HTCs.

The Australian Bleeding Disorders Registry (ABDR)

Patient details are captured in the Australian Bleeding Disorders Registry (ABDR) to enable health care and support staff to monitor and manage treatment over time from a single point of reference.

ABDR is subject to robust governance and privacy arrangements and has been endorsed by both the Haemophilia Foundation Australia (HFA) and the Australian Haemophilia Centre Directors' Organisation (AHCDO).

Endorsement from Haemophilia Foundation Australia

Haemophilia Foundation Australia supports the ABDR. It helps doctors and other treating health professionals to understand more about the care and treatment needs of people affected by bleeding disorders. The ABDR will assist and guide planning to ensure treatment product is available when it is needed. We are confident the steps in place will mean accurate, reliable and confidential data is available and that no patient details can be identified outside haemophilia centres.

www.haemophilia.org.au

Endorsement from Australian Haemophilia Centre Directors' Organisation

The ABDR is a valuable tool that provides a summary of those affected with haemophilia and other bleeding disorders in Australia. Data from the ABDR is the best information available for clinicians to advise governments making policy decisions regarding treatment needs and product availability.

National statistics available through the ABDR will give AHCDO an overview of practice and allow opportunities for improvement. This data can be pooled to compare Australian treatment standards with international benchmarks. The ABDR will continue to provide the ability to assess quality of life and other important clinical questions arising across Australia.

AHCDO's partnership on this initiative with the National Blood Authority, Haemophilia Foundation Australia and other specialist health professional groups is vital to the pursuit of excellence in clinical treatment practices.

www.ahcdo.org.au

For more details about the history of ABDR and the privacy and governance arrangements which apply to data in ABDR, please see Appendix C: About ABDR.

Patients

Table 3 shows the numbers of patients in the ABDR and the numbers of patients who received products during the years 2016-17 to 2020-21.

TABLE 3 - NUMBER OF PEOPLE IN THE REGISTRY AND TREATED BY BROAD DIAGNOSIS

Diagnosis	Number in ABDR Registry					Number who received product				
	2016-17	2017-18	2018-19	2019-20	2020-21	2016-17	2017-18	2018-19	2019-20	2020-21
Hereditary										
HMA	2,365	2,302	2,372	2,449	2,529	1,009	1,040	1,104	1,083	1,117
HMB	564	541	558	585	601	218	227	247	235	253
vWD	2,141	2,146	2,221	2,324	2,460	248	239	307	273	312
Acquired										
HMA	68	74	78	92	90	11	12	15	12	15
HMB	<5	<5	<5	<5						
vWD	25	27	32	34	33	10	5	10	9	7
Other Diagnoses										
Other	193	162	181	195	233	14	12	18	12	16
Other Factor Deficiency	427	449	469	510	557	50	51	58	49	67
Platelet Disorder	288	302	323	355	380	10	8	22	19	14
Vascular	9	7	7	8	8					
Fibrinogen Disorder	74	91	113	133	149	20	13	23	14	23
Total	**6,155**	**6,102**	**6,355**	**6,686**	**7,040**	**1,590**	**1,607**	**1,804**	**1,706**	**1,824**

Notes: Included in the table are patients active as at 30 June 2021. The ABDR allows for a diagnosis of 'Other' to be recorded for patients with rare and less prevalent disorders or difficult to classify disorders eg mild VWD.

Table 4 shows the incidence statistics for Australia compared with other countries from the World Federation of Hemophilia (WFH) Annual Global Survey 2020 released in 2021. The full survey can be found at https://wfh.org/research-and-data-collection/.

TABLE 4 - INCIDENCE STATISTICS FROM WORLD FEDERATION OF HEMOPHILIA GLOBAL SURVEY 2020

Country	Population	HMA/ HMB	vWD	OBD	HMA/HMB per 100,000	VWD per 100,000	OBD per 100,000	Factor VIII per capita
Australia	25,687,041	2,827	2,324	994	11.01	9.05	3.87	7.48
New Zealand	5,084,300	318	90	41	6.25	1.77	0.81	
UK	67,215,293	8,509	11,183	12,171	12.66	16.64	18.11	7.27
USA	329,484,123	14,816	8,919	3,990	4.50	2.71	1.21	6.53
Canada	38,005,238	3,924	4,709	2,459	10.32	12.39	6.47	7.72
France	67,391,582	8,661	2,992	1,127	12.85	4.44	1.67	6.73
Sweden	10,353,442	1,011	908		9.76	8.77		9.70
Germany	83,240,525	4,658	3,498		5.60	4.20		7.82
South Africa	59,308,690	2,365	659	219	3.99	1.11	0.37	1.23
Japan	125,836,021	6,738	1,438	437	5.35	1.14	0.35	5.29

Note this data will match last year's ABDR Annual Report (2019-20), not this current report.

Prevalence of haemophilia A varies considerably among countries, including among the wealthiest of countries[3]. Prevalence data is extremely valuable information for planning by national healthcare agencies in setting priorities and allocating resources for the treatment of bleeding disorders.

[3] Stonebraker JS, Bolton-Maggs PHB, Soucie JM, Walker I, Brooker M. (2010). A study of variations in the reported hemophilia A prevalence around the world. Haemophilia 16(1): 20–32.

Products

The NBA is charged with providing an adequate, safe, secure and affordable supply of blood products, blood-related products and blood-related services in Australia; and promoting safe, high-quality management and use of blood products, blood-related products and blood-related services in Australia.

A key element in ensuring security of supply of products for the treatment of bleeding disorders is the NBA's role in developing, coordinating and monitoring the annual national supply plan and budget, including obtaining annual approval from health ministers. Details on national supply and demand trends over time can be found in Appendix D: National Supply of Products.

Figure 1**Error! Reference source not found.** shows the total issues and market shares for recombinant FVIII products from 2016-17 to 2020-21 and illustrates the changes that have occurred during that period, brought about by new national supply arrangements, with extended half-life (EHL) products added to the mainstream product offering (these were previously trial products). New supply contracts commenced on 1 July 2020, providing further efficiencies in supply and cost. A brief history of the availability of products is at Appendix D.

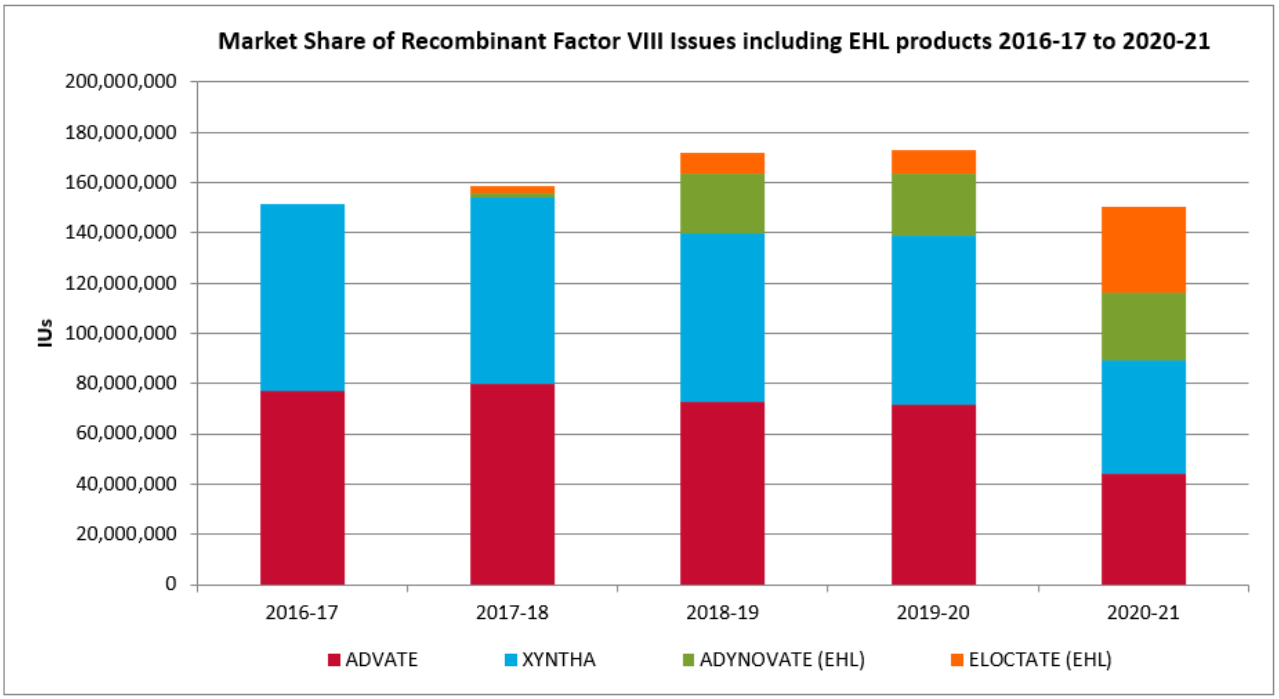


FIGURE 1 - MARKET SHARE OF RECOMBINANT FVIII ISSUES 2016-17 TO 2020-21

During 2020-21, Hemlibra was added to the National Product Price List, and this has had an impact on the use for FVIII products. Hemlibra offers a better and less invasive therapy for patients and will result in lower costs over time. See Figure 2 for more information about Hemlibra, and Figure 7 in Appendix D for the impact on expenditure.

ENABLING ACCESS TO HEMLIBRA

The introduction of Hemlibra (emicizumab) into Australia's national blood arrangements since 2 November 2020 has significantly improved the quality of life for some haemophilia patients. Hemlibra is supplied to the NBA by Roche as a prophylactic therapy for adult and paediatric patients with severe and moderate haemophilia A.

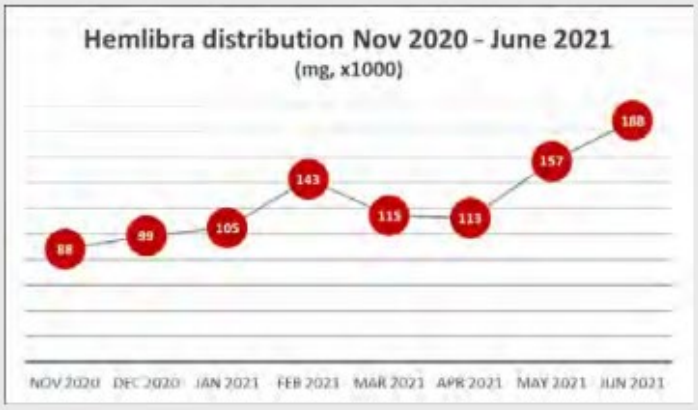
Haemophilia A is a genetic bleeding disorder where blood does not clot properly. Untreated, patients suffer life-threatening, severely disabling internal bleeding and/or recurrent joint damage. Patients can self-administer Hemlibra and have less frequent treatments than alternative products.

People with severe or moderate haemophilia A are usually treated with a clotting factor (FVIII) multiple times a week to reduce rates of bleeding or when a bleeding episode occurs. Conventional FVIII therapy can require frequent intravenous infusions and can also lead to the development of antibody responses (known as inhibitors) which impede the effectiveness of this treatment and subsequently require treatment with alternative, expensive bypassing agent products.

Hemlibra is a novel monoclonal antibody that mimics the action of FVIII to allow the normal clotting cascade to continue. Doses of Hemlibra are delivered subcutaneously, allowing patients to self-administer the treatment on a weekly, 2-weekly or 4-weekly basis depending on clinical need. This results in improved patient quality of life and a decrease in the risks associated with intravenous infusion.

The introduction of Hemlibra as an alternative to clotting factor therapy for haemophilia A has had an impact on clotting factor supply arrangements. Over 1 million milligrams of Hemlibra were distributed between November 2020 and June 2021. The supply contract negotiated by the NBA is expected to save governments $78 million over five years.

The supply of Hemlibra through the national blood arrangements administered by the NBA will ensure patients and clinicians have holistic and consistent national access to therapies for haemophilia A. It is a good outcome for patients, being a better and less invasive therapy, and a good outcome for governments that involves lower costs.



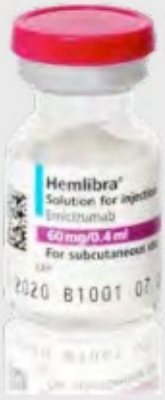


Photo credit: © 2021 F. Hoffmann-La Roche Ltd
https://www.roche.com/products/product-details.htm?productId=4889d5d4-c688-4db5-8805-12a57b1c95a1

FIGURE 2 - ENABLING ACCESS TO HEMLIBRA

Source: NBA Annual Report 2020-21 https://www.blood.gov.au/about-nba#annual-report

Inhibitor status

Inhibitors are immunoglobulins made by the body's immune system to react against replacement clotting factor proteins. This occurs when the immune system perceives the proteins as foreign or harmful to the body. When this happens, the inhibitors prevent the usual replacement factors (Factor VIII or IX) from working properly to stop bleeding. The most challenging aspect of HMA management is the development of FVIII inhibitors. Patients with severe Haemophilia A with high-titre inhibitors are most at risk for recurrent bleeds and chronic haemarthroses.

FEIBA and Recombinant Factor VIIa (brand name NovoSeven) are both used to treat patients that have developed inhibitors. Predicting or interpreting changing demand trends is not possible with any accuracy, as the product is only used in a small number of patients each year. Use patterns will vary from year to year and will not only depend on the number of patients treated, but their severity of disease, the potency of inhibitors, whether secondary prophylaxis is practiced, the number and severity of spontaneous bleeds, and the amount of elective surgery undertaken in this patient group.

In 2020-21, there were 105 HMA patients with inhibitors and 13 patients with other bleeding disorders who had inhibitors. The amount of FEIBA and NovoSeven used by patients with HMA, HMB and VWD during the year is shown in the key findings section, and in Table 6, Table 7, and Table 8 below.

Treatment

The data in this section relates to patients who received treatment (products) during the 2020-21 financial year. Figure 3 shows the proportion of hereditary HMA patients receiving treatment (1,117 patients in 2020-21) by severity. Figure 4 shows the proportion of hereditary HMB patients receiving treatment (253 patients in 2020-21) by severity.

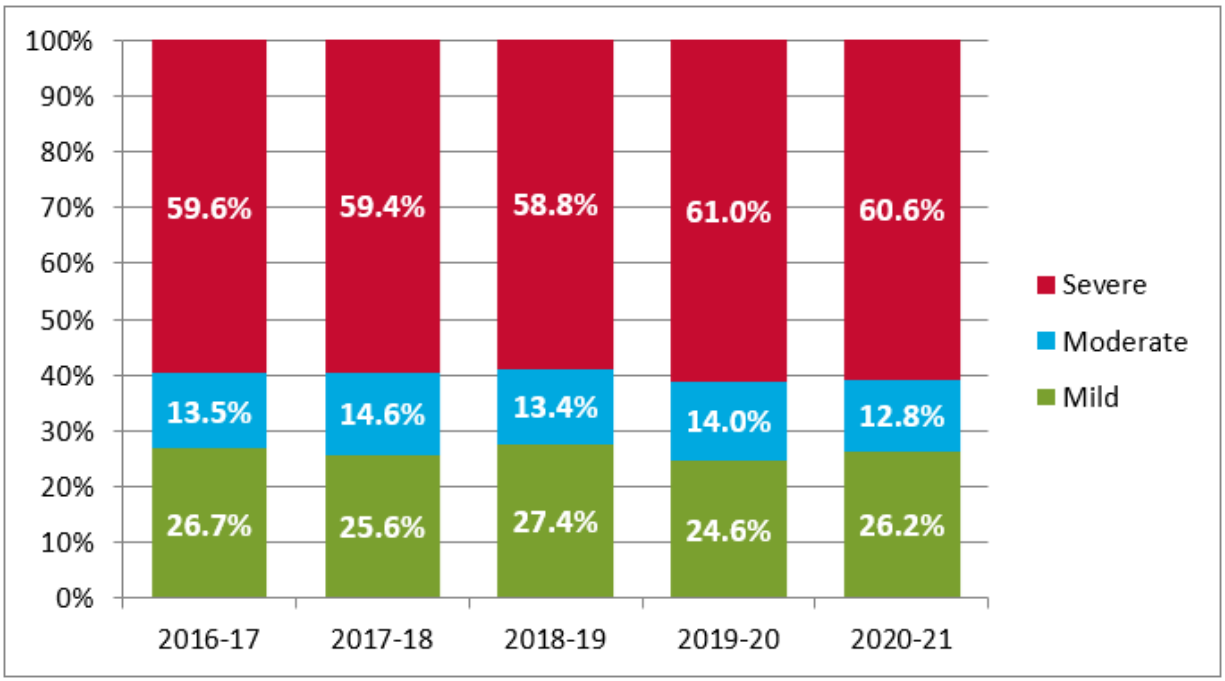


FIGURE 3 - PERCENTAGE OF HEREDITARY PATIENTS RECEIVING PRODUCT BY SEVERITY FOR HMA

Note: A very small number of patients have a severity recorded as Not Applicable or Unknown. These are not shown in the above chart.

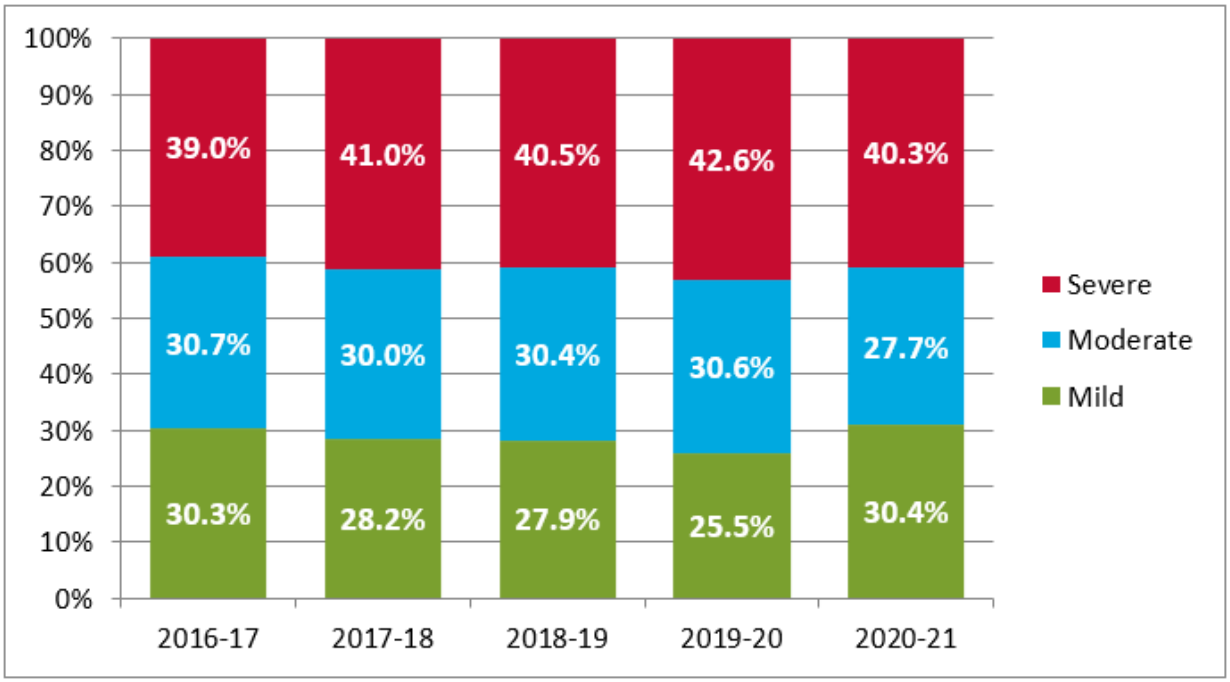


FIGURE 4 - PERCENTAGE OF HEREDITARY PATIENTS RECEIVING PRODUCT BY SEVERITY FOR HMB

Note: Proportion of patients receiving product by severity for HMB – Acquired bleeding disorders are too small to present in graphical format. A very small number of patients have a severity recorded as Not Applicable or Unknown. These are not shown in the above chart.

In 2020-21, 86% (by volume) of FVIII products issued for patients with HMA were for patients with a severe disorder and around 64% (by volume) of FIX products issued for patients with HMB were for those with a severe disorder (Table 5). 87% of Hemlibra was used by severe patients during 2020-21.

Around 36% of patients are diagnosed with HMA (see Table 3), however these patients use around 80% of total factor products. In relative terms, HMA is the most important consideration for national supply planning, and the key factor is the issue of product to severe HMA patients. The volume issued for prophylactic treatment of severe HMA is the single greatest determining factor for supply planning.

TABLE 5 - VOLUME (IU) OF PRODUCT ISSUED BY SEVERITY AND TREATMENT REGIMEN IN 2020-21

	Prophylaxis	OnDemand	Tolerisation	Unknown	Total
HMA (IU FVIII Products)	**129,489,500**	**16,161,300**	**3,409,500**	**160,600**	**149,220,900**
Mild	1,373,000	4,439,750	135,000	147,000	6,094,750
Moderate	11,437,750	3,529,250		10,100	14,977,100
Severe	116,678,750	8,185,300	3,274,500	3,500	128,142,050
Unknown		7,000			7,000
HMB (IU FIX Products)	**19,676,500**	**6,147,000**	**259,000**	**591,000**	**26,673,500**
Mild	272,500	2,085,250		189,000	2,546,750
Moderate	3,930,250	2,793,000		395,000	7,118,250
Severe	15,473,750	1,267,250	259,000	1,000	17,001,000
Unknown		1,500		6,000	7,500
vWD (IU FVIII Product)	**6,048,000**	**2,743,250**	**695,000**	**238,250**	**9,724,500**
Mild	182,750	289,750		108,500	581,000
Moderate	329,000	413,000		59,000	801,000
Severe	3,538,500	949,750	695,000		5,183,250
Unknown	1,997,750	1,090,750		70,750	3,159,250

Unknown treatment regimen: represents a blank/not completed/empty field for the treatment regimen in the ABDR.
Unknown in severity: The severity of a patient's condition is not always known at initial presentation. This table includes product issues to patients with unknown severities.

Table 6, Table 7 and Table 8 show more detailed breakdowns by state, severity, gender, age range, regimen, IU/kg/year and product for HMA, HMB and VWD, the three largest groups of patients and for which most product is used.

TABLE 6 - DETAILED BREAKDOWNS FOR HEREDITARY HMA PATIENTS

Haemophilia A (Hereditary)	NSW	VIC	QLD	SA	WA	TAS	NT	ACT	National
Number of hereditary patients	689	631	492	263	309	71	16	58	2,529
Severe	*199*	*173*	*171*	*57*	*85*	*18*	*<5*	*19*	*725*
Moderate	*78*	*52*	*33*	*39*	*19*	*6*	*<5*	*6*	*234*
Mild	*384*	*292*	*260*	*160*	*203*	*44*	*11*	*32*	*1,386*
Not applicable/Unknown	*28*	*114*	*28*	*7*	*<5*	*<5*	*<5*	*<5*	*184*
Patients who received product	308	232	240	138	140	29	5	25	1,117
Severe	*183*	*160*	*164*	*55*	*78*	*18*	*<5*	*17*	*677*
Male	583	496	404	234	257	52	9	45	2,080
Female	105	135	88	29	52	19	7	13	448
Unknown	<5								<5
Age range									
0 - 19	*218*	*142*	*142*	*72*	*86*	*17*	*<5*	*16*	*695*
20 - 39	*196*	*194*	*162*	*74*	*90*	*26*	*9*	*15*	*766*
40 - 59	*149*	*179*	*111*	*62*	*76*	*8*	*<5*	*16*	*605*
60 - 79	*98*	*101*	*71*	*48*	*49*	*18*	*<5*	*10*	*396*
80 and over	*28*	*15*	*6*	*7*	*8*	*<5*		*<5*	*67*
Average Weight (kg)	68	73	73	72	72	68	67	72	71
Total FVIII IUs for HMA patients	48,831,500	31,176,500	30,982,750	14,307,250	16,965,000	3,695,900	758,000	2,504,000	149,220,900
% Prophylaxis	*86%*	*92%*	*82%*	*89%*	*87%*	*91%*	*51%*	*88%*	*87%*
% On Demand	*13%*	*7%*	*11%*	*9%*	*12%*	*9%*	*49%*	*12%*	*11%*
% of total product used by severe patients	*83%*	*88%*	*92%*	*79%*	*86%*	*99%*	*37%*	*83%*	*86%*
Av IU/kg/yr all hereditary HMA patients	2,603	2,392	2,891	1,724	2,216	2,358	1,961	1,600	2,436
Av IU/kg/yr severe hereditary HMA patients	3,674	3,124	3,933	3,051	3,397	3,488	1,426	2,137	3,476
By product (IU unless otherwise noted)									
rFVIII	*46,258,250*	*31,049,500*	*27,554,750*	*13,951,750*	*16,601,500*	*3,695,100*	*758,000*	*2,504,000*	*142,372,850*
Biostate	*1,933,750*	*73,500*	*3,253,500*	*355,500*	*292,000*	*800*			*5,909,050*
FEIBA (units)	*639,500*	*53,500*	*174,500*		*71,500*				*939,000*
NovoSeven (mg)	*257*	*2,981*	*2,265*	*322*	*640*				*6,465*

TABLE 7 - DETAILED BREAKDOWNS FOR HEREDITARY HMB PATIENTS

Haemophilia B (Hereditary)	NSW	VIC	QLD	SA	WA	TAS	NT	ACT	National
Number of hereditary patients	176	166	146	42	55	6	<5	9	601
Severe	*40*	*34*	*20*	*6*	*8*	*<5*		*<5*	*111*
Moderate	*46*	*22*	*39*	*7*	*12*	*<5*		*<5*	*129*
Mild	*82*	*82*	*72*	*27*	*33*	*<5*	*<5*	*<5*	*303*
Not applicable/Unknown	*8*	*28*	*15*	*<5*	*<5*	*<5*		*<5*	*58*
Patients who received product	89	49	66	19	22	<5	<5	<5	253
Severe	*37*	*28*	*20*	*6*	*8*	*<5*		*<5*	*102*
Male	143	123	109	35	47	<5	<5	6	468
Female	33	43	37	7	8	<5		<5	133
Age range									
0 - 19	*49*	*43*	*42*	*6*	*5*	*<5*		*<5*	*147*
20 - 39	*51*	*47*	*41*	*10*	*20*	*<5*		*<5*	*174*
40 - 59	*46*	*41*	*43*	*14*	*11*	*<5*	*<5*	*<5*	*162*
60 - 79	*22*	*30*	*20*	*11*	*18*			*<5*	*102*
80 and over	*8*	*5*		*<5*	*<5*			*<5*	*16*
Average Weight (kg)	71	69	80	80	82	75	75	65	74
Total FIX IUs for HMB patients	9,979,000	5,089,750	5,910,500	2,357,500	2,282,750	306,000	17,000	731,000	26,673,500
% Prophylaxis	*70%*	*78%*	*75%*	*88%*	*83%*	*98%*	*0%*	*7%*	*74%*
% On Demand	*28%*	*20%*	*25%*	*12%*	*15%*	*2%*	*0%*	*27%*	*23%*
% of total product used by severe patients	*57%*	*77%*	*71%*	*51%*	*60%*	*45%*	*0%*	*64%*	*64%*
Av IU/kg/yr all hereditary HMB patients	2,014	1,694	1,364	1,337	1,265	1,356	227	2,574	1,655
Av IU/kg/yr severe hereditary HMB patients	3,143	2,392	3,261	2,083	2,110	2,156		3,252	2,803
By product (IU unless otherwise noted)									
rFIX	*9,929,000*	*5,089,750*	*5,910,500*	*2,357,500*	*2,282,750*	*306,000*	*17,000*	*389,000*	*26,281,500*
MonoFIX - VF	*50,000*							*342,000*	*392,000*
NovoSeven (mg)	*2,046*	*350*							*2,396*

TABLE 8 - DETAILED BREAKDOWNS FOR HEREDITARY VWD PATIENTS

von Willebrand Disease (Hereditary)	NSW	VIC	QLD	SA	WA	TAS	NT	ACT	National
Number of hereditary patients	528	452	672	239	398	85	29	57	2,460
Severe	*46*	*18*	*28*	*10*	*32*	*<5*	*8*	*<5*	*148*
Moderate	*68*	*44*	*51*	*27*	*69*	*9*		*8*	*276*
Mild	*215*	*168*	*366*	*139*	*297*	*59*	*18*	*43*	*1,305*
Not applicable/Unknown	*199*	*222*	*227*	*63*		*15*	*<5*	*<5*	*731*
Patients who received product	107	23	77	39	56	<5		7	312
Severe	*22*	*<5*	*6*	*7*	*13*			*<5*	*54*
Male	200	178	243	74	121	32	10	21	879
Female	328	274	429	165	277	53	19	36	1,581
Age range									
0 - 19	*118*	*84*	*109*	*24*	*65*	*12*	*6*	*8*	*426*
20 - 39	*176*	*138*	*272*	*65*	*136*	*35*	*11*	*20*	*853*
40 - 59	*124*	*138*	*168*	*88*	*134*	*22*	*8*	*15*	*697*
60 - 79	*83*	*79*	*108*	*49*	*52*	*15*	*<5*	*12*	*402*
80 and over	*27*	*13*	*15*	*13*	*11*	*<5*		*<5*	*82*
Average Weight (kg)	68	68	69	71	70	67	68	71	69
Total FVIII IUs for vWD patients	4,932,750	232,250	2,138,000	843,500	1,342,000	19,250		216,750	9,724,500
% Prophylaxis	*71%*	*51%*	*42%*	*80%*	*61%*	*90%*			*62%*
% On Demand	*14%*	*48%*	*49%*	*18%*	*38%*	*10%*		*97%*	*28%*
% of total product used by severe patients	*65%*	*50%*	*9%*	*62%*	*72%*			*74%*	*53%*
Av IU/kg/yr all hereditary vWD patients	1,041	290	397	721	476	153		467	646
Av IU/kg/yr severe hereditary vWD patients	2,088	531	402	994	1,229			1,283	1,436
By product (IU unless otherwise noted)									
rFVIII			*10,000*						*10,000*
Biostate	*4,932,750*	*232,250*	*2,128,000*	*843,500*	*1,342,000*	*19,250*		*216,750*	*9,714,500*

TABLE 9 - VOLUME (IU), PATIENT COUNTS AND AVERAGE IU/KG BY PRODUCT AND TREATMENT REGIMEN – HEREDITARY HMA, HMB AND VWD PATIENTS

Table 9 shows volume of product issued by IU/mg, number of hereditary patients and average IU/kg for the 2020-21 year, by treatment regimen.

	OnDemand			Prophylaxis			Tolerisation			Not specified		
	No of patients	Total Units	Avg. IU/KG	No of patients	Total Units	Avg. IU/KG	No of patients	Total Units	Avg. IU/KG	No of patients	Total Units	Avg. IU/KG
HMA (IUs)												
rFVIII	*397*	*15,227,000*	*100*	*618*	*126,031,750*	*257*	*8*	*967,500*	*244*	*14*	*146,600*	*47*
Biostate	*15*	*362,300*	*130*	*25*	*3,224,750*	*598*	*8*	*2,308,000*	*1046*	*<5*	*14,000*	*164*
FEIBA	*<5*	*572,000*	*265*	*<5*	*233,000*	*585*	*<5*	*134,000*	*1072*			
NovoSeven (mgs	*14*	*3,170*		*13*	*3,186*		*<5*	*109*	*2*			
rFIX	*<5*	*33,000*	*55*									
HMB (IUs)												
rFIX	*139*	*6,014,000*	*132*	*116*	*19,676,500*	*222*				*10*	*591,000*	*119*
MonoFIX - VF	*<5*	*133,000*	*326*				*<5*	*259,000*	*466*			
rFVIX	*<5*	*18,000*	*33*									
NovoSeven (mgs	*<5*	*356*	*1*	*<5*	*2,040*	*2*						
VWD (IUs)												
rFVIII	*<5*	*7,000*	*28*							*<5*	*3,000*	*41*
Biostate	*190*	*2,736,250*	*58*	*28*	*6,048,000*	*275*	*<5*	*695,000*	*921*	*26*	*235,250*	*23*

There are much smaller numbers of patients with acquired HMA, HMB and VWD. These are set out below, along with state breakdowns for patients with other bleeding disorders.

TABLE 10 - PATIENTS WITH ACQUIRED AND OTHER BLEEDING DISORDERS, BY STATE

Acquired and Other Bleeding Disorders	NSW	VIC	QLD	SA	WA	TAS	NT	ACT	National
Acquired HMA, HMB, vWD									
Acquired haemophilia A	*8*	*45*	*13*	*13*	*11*				*90*
Acquired haemophilia B									
Acquired von Willebrand Disease	*<5*	*9*	*14*	*6*	*<5*				*33*
Other Factor Deficiency	86	166	86	77	130	<5	6	5	557
Factor V Deficiency	*8*	*5*	*<5*	*5*					*22*
Factor VII Deficiency	*22*	*27*	*32*	*12*	*13*			*<5*	*109*
Factor X Deficiency	*<5*	*5*	*<5*	*<5*	*6*				*20*
Factor XI Deficiency	*42*	*109*	*39*	*55*	*103*	*<5*	*<5*	*<5*	*354*
Factor XII Deficiency	*<5*	*<5*	*5*	*<5*	*<5*		*<5*		*18*
Factor XIII Deficiency	*7*	*15*	*<5*	*<5*	*<5*				*30*
Acquired Other Factor Deficiency		*<5*		*<5*	*<5*				*<5*
Platelet Disorder	67	85	102	70	48	<5	<5	<5	380
Fibrinogen	17	50	31	18	31	<5		<5	149
Vascular	<5		7						8
Other	39	43	14	40	51	5	<5		194
No Bleeding Disorder recorded	<5	<5	5	<5	25	<5	<5	<5	39

TABLE 11 - PRODUCTS USED BY PATIENTS WITH ACQUIRED AND OTHER BLEEDING DISORDERS

Acquired and Other Bleeding Disorders	Product	Units	NSW	VIC	QLD	SA	WA	TAS	NT	ACT	National
Acquired HMA, HMB, vWD											
Acquired haemophilia A	*rFVIII*	*IU*			*2,000*						*2,000*
	FEIBA	*units*			*58,000*	*121,500*	*170,000*	*121,500*	*170,000*		*349,500*
	NovoSeven	*mg*	*7*	*2,528*	*125*	*757*	*432*	*757*	*432*		*3,849*
Acquired von Willebrand Disease	*Biostate*	*IU*	*3,000*	*2,000*	*106,500*	*9,500*	*3,000*	*9,500*	*3,000*		*124,000*
Other Factor Deficiency											
Factor VII Deficiency	*NovoSeven*	*mg*	*1,081*	*9*	*1,250*	*165*	*56*			*995*	*3,556*
Factor XI Deficiency	*Factor XI bpl*	*IU*	*2,362*	*17,801*	*27,200*	*15,000*	*31*				*62,394*
Factor XI Deficiency	*NovoSeven*	*mg*		*29*							*29*
Factor XII Deficiency	*NovoSeven*	*mg*					*4*				*4*
Factor XIII Deficiency	*Fibrogammin*	*IU*	*56,250*	*15,597*			*20,000*				*91,847*
Factor XIII Deficiency	*NovoThirteen*	*IU*		*87,500*			*15,000*				*102,500*
Platelet Disorder											
	Biostate	*IU*					*7,500*				*7,500*
	NovoSeven	*mg*		*15*	*174*	*30*					*219*
Fibrinogen											
	RiaSTAP	*g*	*28*	*38*	*365*		*144*				*575*
Other Factor Deficiency											
	rFVIII	*IU*	*2,000*								*2,000*
	Biostate	*IU*			*17,000*	*3,500*	*2,500*				*23,000*
	FEIBA	*units*					*7,500*				*7,500*
	NovoSeven	*mg*					*48*				*48*

Appendix A: Bleeding Disorders

The information in this section has been drawn from the materials and websites of two peak bodies for haemophilia; the World Federation of Hemophilia (www.wfh.org) and Haemophilia Foundation Australia (www.haemophilia.org.au).

In people with bleeding disorders, the clotting process doesn't work properly. As a result, people with bleeding disorders can bleed for longer than normal, and some may experience spontaneous bleeding into joints, muscles, or other parts of their bodies.

Bleeding disorders are inherited (hereditary) or acquired

Bleeding disorders are almost always inherited or passed through families; they have a genetic basis and the genes responsible for the disorders are passed from parents to children. A person can also spontaneously develop a bleeding disorder, although this is rare. Acquired bleeding disorders are not inherited or passed through families. Most acquired bleeding disorders have an identifiable root cause.

Haemophilia

Haemophilia causes excessive bleeding following trauma or surgery and can be related to spontaneous haemorrhages into muscles and joints. People with haemophilia do not bleed any faster than normal, but they can bleed for a longer time.

Haemophilia is an X-linked disorder that typically affects males, whereas females are normally classified as carriers. However, affected males will pass on the haemophilia gene to their daughters, and women carrying a F8 or F9 gene mutation may have reduced factor levels and should therefore be classified as having haemophilia. Most carriers are asymptomatic. Carriers with clotting factor levels in the haemophilia range may be symptomatic, with bleeding manifestations commensurate with their degree of clotting factor deficiency, particularly during trauma and surgery. Symptomatic carriers are classified as haemophilia in line with the World Federation of Hemophilia (www.wfh.org) guidelines.

Haemophilia fast facts

- The most common type of haemophilia is Haemophilia A. This means the person does not have enough clotting Factor VIII (factor eight).
- Haemophilia B is less common. A person with Haemophilia B does not have enough Factor IX (factor nine). The symptoms are the same for people with Haemophilia A and B; that is, they bleed for a longer time than normal.
- Haemophilia occurs in 1 in 6,000-10,000 males internationally.
- Bleeding is most commonly internal into the joints and/or muscles. Less commonly, bleeding into internal organs can also occur. It can happen without an obvious cause (sometimes called 'spontaneous'), or as a result of injury.
- Over time this internal bleeding into joints ('bleeds') can cause severe arthritis, chronic pain and disability.
- Specialised treatment is needed to help blood clot normally. With appropriate treatment haemophilia can be managed effectively.
- Haemophilia is an inherited condition and occurs in families, however in 1/3 of cases it appears in families with no previous history of the disorder. The haemophilia gene is passed down from parent to child through generations. Men with haemophilia will pass the gene on to their daughters but not their sons. Women who carry the haemophilia gene can pass the haemophilia gene on to their sons and daughters. Sons with the gene will have haemophilia. Some women and girls who carry the gene may also experience bleeding problems.

Von Willebrand disorder/disease (VWD)

Von Willebrand disease is the most common type of bleeding disorder. People with VWD have a problem with von Willebrand Factor (VWF), a protein in their blood that would normally help control bleeding. When a blood vessel is injured and bleeding occurs, VWF helps cells in the blood, called platelets, adhere to damaged blood vessels and mesh together and form a clot to stop the bleeding. People with VWD do not have enough VWF, or it does not work the way it should. It takes longer for blood to clot and for bleeding to stop.

VWD is generally less severe than other bleeding disorders. Many people with VWD may not know that they have the disorder because their bleeding symptoms are very mild. For most people with VWD, the disorder causes little or no disruption to their lives except when there is a serious injury or need for surgery. However, with all forms of VWD, there can be bleeding problems. VWD is difficult to accurately diagnose as laboratory values can fluctuate and values in those with mild bleeding symptoms can overlap with normal laboratory values.

From some studies, it is estimated that up to 1% of the world's population has VWD, but because many people have only very mild symptoms, only a small number of them are diagnosed. Research has shown that as many as 9 out of 10 people with VWD have not been diagnosed. It is estimated that VWD affects approximately 200,000 people in Australia, but symptomatic individuals possibly less.

Types of VWD

There are three main types of VWD. Bleeding symptoms can be quite variable within each type depending in part on the VWF activity. It is important to know which type of VWD a person has, because treatment is different for each type.

- Type 1 VWD is the most common form. People with Type 1 VWD have lower than normal levels of VWF. Symptoms are usually mild. Still, it is possible for someone with Type 1 VWD to have serious bleeding.
- Type 2 VWD involves a defect in the VWF structure. The VWF protein does not work properly, causing lower than normal VWF activity. There are different Type 2 VWD defects. Severity of symptoms can vary.
- Type 3 VWD is usually the most serious form. People with Type 3 VWD have very little or no VWF. Symptoms are more severe. People with Type 3 VWD can have bleeding into muscles and joints, sometimes without injury.

Rare clotting factor deficiencies

Rare clotting factor deficiencies are a group of inherited bleeding disorders caused by a problem with one of several clotting factors other than factor VIII or factor IX. Clotting factors are proteins in the blood that control bleeding. Many different clotting factors work together in a series of chemical reactions to stop bleeding. This is called the clotting process.

Rare clotting factor deficiencies are bleeding disorders in which one of the other clotting factors (i.e., factors I, II, V, V+VIII, VII, X, XI, or XIII) is missing or not working properly. The World Federation of Hemophilia produced a summary (Table 12) of the characteristics of rare clotting factor deficiencies, the severity of bleeds associated with them, and the treatment typically required.

TABLE 12 - CHARACTERISTICS OF RARE CLOTTING FACTOR DEFICIENCIES

Missing Factor	Incidence*	Inheritance	Severity of Bleeding	Treatment
Factor I Afibrinogenemia Hypofibrinogenemia Dysfibrinogenemia	5 in 10 million not available 1 in 1 million	Autosomal recessive Recessive or dominant Recessive or dominant	Usually mild, except in afibrinogenemia	•Fibrinogen (not funded in Australia) •Cryoprecipitate •Fresh frozen plasma
Factor II	1 in 2 million	Autosomal recessive	Moderate to severe when factor levels are low; usually mild	•Prothrombin complex •Fresh frozen plasma
Factor V	1 in 1 million	Autosomal recessive	Moderate to severe when factor levels are low; usually mild	•Fresh frozen plasma
Combined Factor V and Factor VIII	1 in 1 million†	Autosomal recessive‡	Usually mild	•Fresh frozen plasma •Factor VIII •Desmopressin
Factor VII	1 in 500,000	Autosomal recessive	Severe when factor levels are low	•Recombinant Factor VIIa •Factor VII •Fresh frozen plasma
Factor X	1 in 1 million	Autosomal recessive	Moderate to severe when factor levels are low	•Prothrombin complex •Fresh frozen plasma
Combined deficiency of vitamin K dependent clotting factors	not available	Autosomal recessive	Usually mild, but a few families have reported very low levels and more severe symptoms	•Vitamin K •Prothrombin complex •Fresh frozen plasma
Factor XI	1 in 100,000	Recessive or dominant	Mild to moderate when factor levels are low	•Factor XI •Antifibrinolytic drugs •Fibrin glue •Fresh frozen plasma
Factor XIII	1 in 3 million	Autosomal recessive	Moderate to severe when factor levels are low	•Factor XIII •Cryoprecipitate •Fresh frozen plasma

Note: Australian Prothrombin Complex Concentrate is not used for FVII deficiency

* Estimates only

† 1 in 100,000 in some populations, including Israel, Iran, and Italy

‡ Very rarely, Factor VIII deficiency can be inherited separately from only one parent

Platelet function disorders

Platelets are small parts of cells that circulate in the blood. They are involved in the formation of blood clots and the repair of damaged blood vessels.

When a blood vessel is injured, platelets stick to the damaged area and spread along the surface to stop the bleeding (this process is called adhesion). At the same time, chemical signals are released from small sacks inside the platelets called granules (this process is called secretion). These chemicals attract other platelets to the site of injury and make them clump together to form what is called a platelet plug (this process is called aggregation).

Sometimes the platelet plug is enough to stop the bleeding. However, if the wound is large, other proteins called clotting factors are recruited to the site of injury. These clotting factors work together on the surface of the platelets to form and strengthen the blood clot.

Platelet function disorders are conditions in which platelets don't work the way they should, resulting in a tendency to bleed or bruise. Since the platelet plug does not form properly, bleeding can continue for longer than normal. Since platelets have many roles in blood clotting, platelet function disorders can lead to bleeding disorders of various intensities.

Special issues for girls and women

Women with clotting factor deficiencies may have additional symptoms because of menstruation and childbirth. Girls may have especially heavy bleeding when they begin to menstruate. Women with clotting factor deficiencies may have heavier and/or longer menstrual flow, which can cause anaemia (with low levels of iron, which results in weakness and fatigue). Women with clotting factor deficiencies should receive genetic counselling about the risks of having an affected child well in advance of any planned pregnancies and should see an obstetrician as soon as they suspect they are pregnant. The obstetrician should work closely with the staff of the haemophilia/bleeding disorder treatment centre in order to provide the best care during pregnancy and childbirth and to minimize the potential complications for both the mother and the newborn child.

Women with certain rare factor deficiencies (such as Factor XIII deficiency and afibrinogenemia) may be at greater risk of miscarriage and placental abruption (a premature separation of the placenta from the uterus that disrupts the flow of blood and oxygen to the foetus). Therefore, these women require treatment throughout the pregnancy to prevent these complications.

The main risk related to pregnancy is postpartum haemorrhage. All bleeding disorders are associated with a greater risk of increased bleeding after delivery. The risk and the severity of the bleeding can be reduced with appropriate treatment. This treatment is different for each woman and depends on her personal and family history of bleeding symptoms, the severity of the factor deficiency, and the mode of delivery (vaginal birth vs. caesarean section). Factor replacement may be necessary in some cases.

The bleeding disorders captured in ABDR have been summarised to higher level groups to enable this report to be more concise than previous reports. The bleeding disorders included in each group are:

Haemophilia A
Factor VIII Deficiency (Haemophilia A)
Asymptomatic Carrier Factor VIII Deficiency (Haemophilia A)
Symptomatic Carrier Factor VIII Deficiency (Haemophilia A)
(Acquired) Factor VIII Deficiency (Haemophilia A)
Haemophilia B
Factor IX Deficiency (Haemophilia B)
Asymptomatic Carrier Factor IX Deficiency (Haemophilia B)
Symptomatic Carrier Factor IX Deficiency (Haemophilia B)
(Acquired) Factor IX Deficiency (Haemophilia B)
Von Willebrand Disease
Von Willebrand Disease Type 1
Von Willebrand Disease Type 2 - Uncharacterised
Von Willebrand Disease Type 2A
Von Willebrand Disease Type 2B
Von Willebrand Disease Type 2M
Von Willebrand Disease Type 2N
Von Willebrand Disease Type 3
Von Willebrand Disease - Uncharacterised
(Acquired) Von Willebrand Disease - Uncharacterised
(Acquired) Von Willebrand Disease Type 1
(Acquired) Von Willebrand Disease Type 2 - Uncharacterised
(Acquired) Von Willebrand Disease Type 2A
(Acquired) Von Willebrand Disease Type 3
Other Factor Deficiency
Factor V Deficiency
Factor VII Deficiency
Factor X Deficiency
Factor XI Deficiency
Factor XII Deficiency
Factor XIII Deficiency
(Acquired) Factor V Deficiency
(Acquired) Factor X Deficiency
(Acquired) Factor XI Deficiency
(Acquired) Factor XIII Deficiency
Platelet Disorder
Platelet Dysfunction - Bernard-Soulier
Platelet Dysfunction - Glanzmann's Thrombasthenia
Platelet Dysfunction - Macrothrombocytopenias
Platelet Dysfunction - May Hegglin
Platelet Dysfunction - Primary Secretion Defect
Platelet Dysfunction - Storage Pool (Dense Granule) Deficiency
Platelet Dysfunction - Uncharacterised
Fibrinogen
Fibrinogen - Afibrinogenemia
Fibrinogen - Dysfibrinogenemia
Fibrinogen - Hypofibrinogenemia
Fibrinogen Dysfunction - Uncharacterised
Vascular
Vascular Disorders - Ehlers Danlos Syndrome
Other
A diagnosis of 'Other' may be recorded for patients with rare and less prevalent disorders or difficult to classify disorders.

Note: Acquired disorders may be included in the group or shown separately depending on the table.

Appendix B: Haemophilia Treatment Centres

The majority of people with these conditions are treated at Haemophilia Treatment Centres (HTCs) which are specialist centres that provide comprehensive care to people with haemophilia and other bleeding disorders. The comprehensive care model ensures that preventative and general treatment on the complex aspects of haemophilia is given in a co-ordinated way by a multi-disciplinary team with specialised expertise within the one centre.

HTCs were established following a decision by Australian Health Ministers Advisory Council (AHMAC) in 1998. The locations of the HTCs in Australia are shown in Figure 5.

FIGURE 5 - LOCATION OF HAEMOPHILIA TREATMENT CENTRES

Haemophilia Treatment Centres coordinate and, where possible, integrate patient care, research and education to provide the optimal use of expertise and resources within hospitals and the community. The model for HTCs varies between jurisdictions in relation to centralisation of services, size and age of patient population. HTCs maintain on-going dialogue with the client group in each state and territory. The role of State and Territory Governments is to designate 'Haemophilia Treatment Centres' and negotiate the funding of the HTC including the purchase of therapeutic blood and recombinant products for distribution within states (or regions) and territories.

Haemophilia Centres provide:

- a single point of care for patients with bleeding disorders with responsibility for the coordination, allocation and distribution of therapeutic resources for the treatment of patients
- a clinical service by experienced staff for patients with bleeding disorders and their families at short notice at any time of the day or night
- organisation of home therapy programs by the centre or in collaboration with other haemophilia treatment facilities
- a counselling and advisory service for people with haemophilia and their families including genetic counselling and family planning
- specialist medical expertise, principally haematology, surgery (the surgeons would have to be accredited to the Haemophilia Centre) rheumatology, infectious diseases and dental services
- specialist allied health services to include physiotherapy, social work and podiatry
- a laboratory service able to carry out all investigations required for the accurate diagnosis of haemophilia and other inherited disorders of haemostasis and to have access, in association with other centres, to specialised testing facilities, for example gene typing
- records for all investigations, treatments, allocation of therapeutic products and adverse reactions, including data entry into ABDR
- a capability to participate in research including clinical trials
- educational programs and guidelines for medical staff, other personnel, patients and their families which promote care of patients with disorders of haemostasis
- an outreach service to isolated patients and treating medical services. The outreach service may include: a haemophilia treatment facility located in a hospital that does not provide all the specialist services and a designated supervising medical practitioner
- data management for ABDR, to aid in capturing data critical to HTC staff for the day-to-day management of people with bleeding disorders and for supply management and policy purposes.

Appendix C: About ABDR

The ABDR is a database that is designed to collect all clinical information related to the treatment of people with inherited bleeding disorders. This includes information about patient diagnosis, viral status, treatment details, hospital admissions and administrative information as well as details on ordering, supply and use of clotting factor products. Information is entered into the ABDR web enabled software by staff at HTCs.

The ABDR provides health care teams and support staff with a record enabling them to monitor and manage treatment over time to improve patients' quality of life. De-identified information from the ABDR may be used for research purposes by authorised organisations to understand and improve treatment for bleeding disorders. The ABDR also provides governments with information on total clotting factor product requirements to inform supply planning to meet the needs of all Australians with bleeding disorders.

The current version of the ABDR has been in existence since December 2008, building on the original registry which was first developed in 1988. In August 2012 the 4th generation ABDR was implemented. The ABDR has evolved with improvements in technology and feedback from stakeholders. In 2014 the ABDR entered a new phase with MyABDR, a secure app for smartphones (Android and iOS) and a web site for people with bleeding disorders or parents/caregivers to record home treatments and bleeds. MyABDR is an internet-based online system that gives patients a quick, easy and reliable way to:

- record treatments and bleeds
- manage treatment product stock
- share the information with a Haemophilia Treatment Centre through the ABDR
- update contact and personal details.

A more in-depth history of the development of the ABDR is available at Appendix D of the 2019-20 ABDR Annual Report, available from: https://www.blood.gov.au/data-analysis-reporting.

For more information about the ABDR, including patient privacy, governance arrangements and support materials, see https://www.blood.gov.au/abdr.

ABDR management and governance

The ABDR is managed under a robust governance framework by the NBA in accordance with the guidance and policy oversight provided by the ABDR Steering Committee. The Committee consists of representatives involved in the clinical management, advocacy and funding of treatment for people with bleeding disorders.

In 2020-21 the Steering Committee representatives were:

- Dr Simon McRae (Chair) – Australian Haemophilia Centre Directors' Organisation
- Dr Huyen Tran – Chair of Australian Haemophilia Centre Directors' Organisation
- Ms Sharon Caris – Executive Director, Haemophilia Foundation Australia
- Mr Michael Furey, VIC Health – Jurisdictional Blood Committee nominee
- Dr Edward Saravolac – National Blood Authority (to 31 May 2021)
- Ms Jo Cameron – National Blood Authority (from 1 June 2021).

Patient privacy and consent in ABDR and MyABDR

The ABDR and MyABDR are provided by the NBA. The NBA is required to ensure that patient information in ABDR and MyABDR is collected and managed in a way which complies with the Commonwealth *Privacy Act 1988*. There are also parallel requirements which may apply under state and territory laws. Privacy requirements under the *Privacy Act 1988* were tightened in 2014, and a new Privacy Policy for these systems was implemented from 26 January 2015.

A patient's personal information may be entered into the ABDR, either at a HTC or when a patient enters data directly via MyABDR This information becomes part of an electronic record about the patient's bleeding disorder condition. Security protocols are embedded into the technical architecture of the ABDR. These control access to personal data, so information is only accessible to treating health professionals and authorised support staff.

In accordance with the ABDR/MyABDR Privacy Policy, a patient's consent is required for the recording of their data in ABDR (consent may be given by a parent, guardian or authorised representative where relevant). Where a patient does not consent then details will not be aggregated in this report, and therefore patient numbers and product use may be understated.

More information about the management of patient privacy in ABDR and MyABDR can be found at http://www.blood.gov.au/privacy-info-abdr-myabdr, including a copy of the ABDR/MyABDR Privacy Policy together with further information, forms and other implementation resources.

Data quality issues

There are several historic data quality issues in the ABDR. These include incomplete records with empty fields or entries. The data captured in some fields has also inconsistent in some cases. Data quality has improved greatly over the years included in some tables in this report. Patient and product details have now been reported consistently for at least the last five years, however comparison with reports from before 2014-15 will be difficult. Improvements in data quality in other specific areas of the system continue to be made through data analysis and cleansing.

There are also some patients whose treatment is managed by clinicians who are not associated with an HTC. The proportion of product that is used in these circumstances varies across jurisdictions and there is some variability in the data capture for this activity between jurisdictions. Accordingly, data on total volume of products recorded from the ABDR may not be consistent with data from other sources.

Data Projects

As data quality improves, various data projects can be undertaken to provide insights into further opportunities for improvement in data entry, or additional information to assist with managing patients and treatments. The following projects were commenced and/or progressed during 2020-21:

- Genetic Landscape Project – review of the genetic profile of patients with bleeding disorders and the correlation between types of mutations and the risk of inhibitor development.
- Inhibitor Project – 24.9% of severe patients developed an inhibitor. Overall development of inhibitors was 17.5%. After more than 50 exposure days, the risk decreases drastically.
- Extended EHL Project – investigation of the effectiveness of prescribed EHL treatment regimen on bleed outcomes and correlation with pharmacokinetics. Pharmacokinetic data is being analysed.
- Joint Score Project – characterisation of clinician practices regarding the use of the Haemophilia Joint Health Score (HJHS) in routine assessment of HMA and HMB patients and identification of potential barriers to HJHS tool usage.
- Hemlibra prophylaxis, bleed, surgery Project – investigate the effectiveness of treatment and outcomes in HMA patients who have transitioned to Hemlibra. Currently analysing data. Results will be presented in the next annual report.

Projects completed during 2020-21 include:

- Hepatitis C Project – this project looked at the prevalence of Hepatitis C (HCV) among patients with a bleeding disorder and the impact of subsidised medication for HCV.
- SIPPET (Survey of Inhibitors in Plasma-Products Exposed Toddlers) Project – this project included Previously Untreated Patients (PUPs) born between 2011 and 2017. There was little change in prescribing practice in terms of product choice.
- Switch Project –Some Haemophilia A patients were switched between recombinant FVIII products. The results indicated that switching products did not increase the risk of inhibitor development, however switching between product types may impact inhibitor development.
- Transition from SHL to EHL Project – the aim of this project was to look at medical factors around EHL product use. Overall bleed rates decreased tremendously, and the proportion of patients with no bleeds increased significantly (44% to 64%).

Publications setting out progress and findings relating to the projects are listed on the AHCDO website: https://www.ahcdo.org.au/guidelines/ahcdo-research-fellow-publications and https://www.ahcdo.org.au/guidelines/ahcdo-member-publications.

Appendix D: National Supply of Products

The NBA is responsible for managing the national blood supply to ensure that healthcare providers have sustainable, reliable and efficient access to blood and blood products needed for patient care. The NBA ensures blood supply security by working with states and territories to determine and manage an annual supply plan and budget and negotiating and managing blood supply contracts and arrangements with local and overseas suppliers.

A key element of the NBA's role in ensuring security of supply is to develop, coordinate and monitor the annual national supply plan and budget, including obtaining annual approval from health ministers.

This is achieved by:

- developing a national estimate of product demand
- liaising with jurisdictions and stakeholders to refine the estimated demand for products
- collecting and distributing data on product issued and reporting variations to jurisdictions on the approved supply plan
- intensively managing products if they are in short supply.

Figure 6 illustrates the national supply by product category for 2020-21. This year, Hemlibra, which is a monoclonal antibody, was added to the National Product Price List. Issues of clotting factor products clotting factors and emicizumab (Hemlibra) totalled 10.7% of expenditure ($149.9 million).

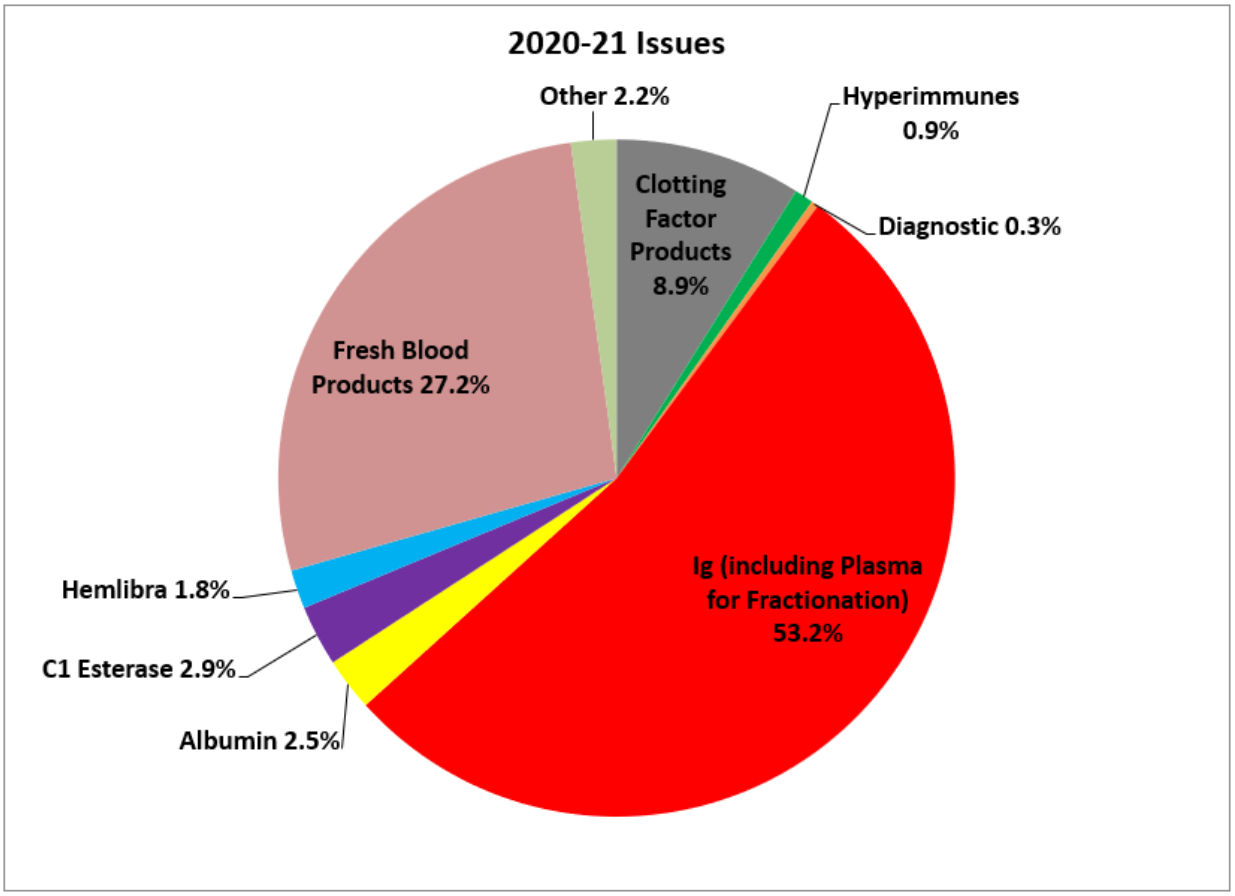


FIGURE 6 - NATIONAL ISSUES BY PRODUCT CATEGORY 2020-21

Note: Plasma for Fractionation costs paid to the Blood Service for collection has been attributed to IVIg and Hyperimmunes.

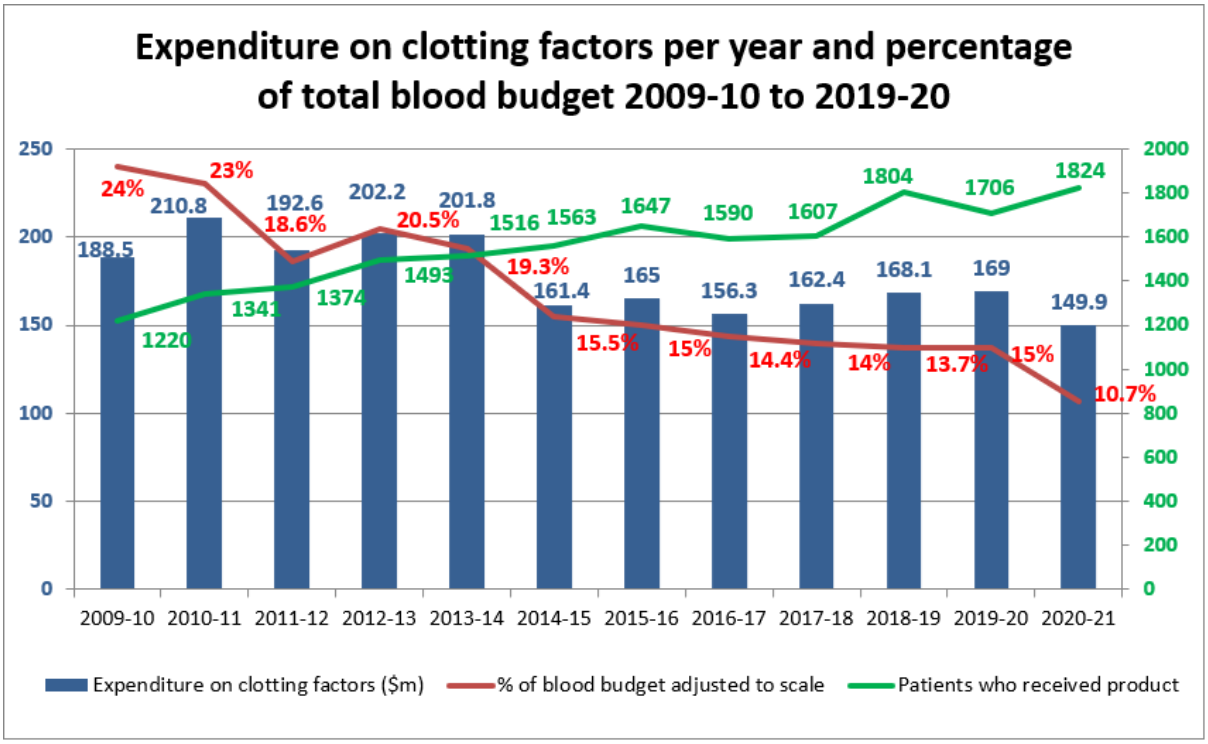


FIGURE 7 - EXPENDITURE ON CLOTTING FACTORS AND PERCENTAGE OF BLOOD BUDGET 2009-10 TO 2020-21

Note: 2020-21 includes Hemlibra, a monoclonal antibody product.

Figure 7 illustrates the variations in total expenditure on clotting factors and the percentage of the blood and blood products budget that comprised each year for 2009-10 to 2020-21. It also shows that the number of patients who received products has grown significantly over the 12 years to 2020-21. Overall expenditure is down over the 12-year period, remaining relatively steady in the last 5 years. Contract negotiation processes have led to falls in average costs per IU from 2012-13 to 2020-21. The introduction of Hemlibra has already had an impact on the need for FVIII products, although it has only been used for part of the year (since November 2020). Full year data will be provided in the next ABDR Annual Report.

Throughout 2020-21, products were supplied to meet clinical demand and supply risks were effectively managed. The approved budget for 2020–21 covering the supply and management of blood and blood products and services under contract was $1,357.10 million, comprising $700.06 million for fresh blood products and plasma collection and $635.62 million for plasma and recombinant products. There is also an additional $21.42 million included for items such as support for the publication of PBM Guidelines, maintenance of the Australian Haemophilia Centre Directors' Organisation (AHCDO) and administration of the Australian Bleeding Disorders Registry (ABDR), BloodSafe eLearning, Blood Sector ICT Systems and the operations of the NBA.

Issues of clotting factors

Issues of clotting factor products represent those deliveries from suppliers to all Australian Health Providers (including hospitals and HTCs) and home delivery of products to patients.

Figure 8 indicates that the demand for Factor VIII products in 2020-21 decreased by 13.5 per cent when compared to 2019-20. The demand for recombinant Factor VIII decreased by 13.2 per cent from 2019-20. Plasma derived Factor VIII demand decreased by 15.7 per cent. These decreases are due to the introduction of Hemlibra (emicizumab) in November 2020. In the period November 2020 to June 2021 over 1 million mgs of Hemlibra were issued.

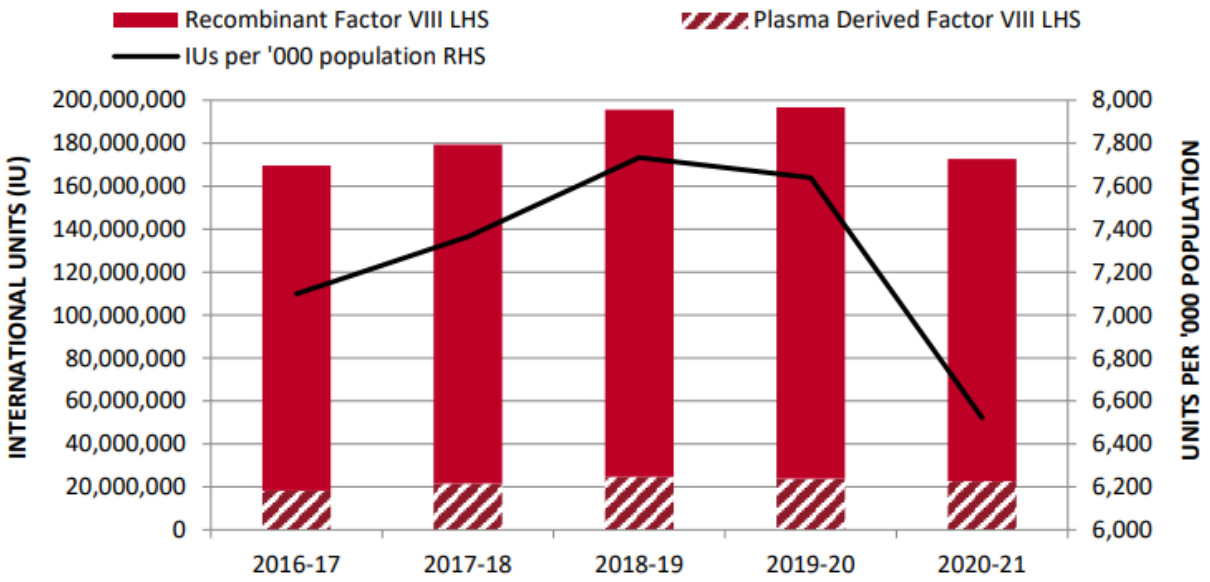


FIGURE 8 - ISSUES OF FACTOR VIII PRODUCTS, 2016-17 TO 2020-21 PER '000 POPULATION

Figure 9 indicates that demand for factor IX products in 2020-21 increased by 0.5 per cent compared to 2019-20. Plasma derived factor IX demand decreased by 2.5 per cent in 2020-21 due to a reduction in specific patient requirements. Demand for recombinant factor IX increased by 0.5 per cent in 2020-21. The introduction of the extended half-life recombinant factor IX clotting factor products during 2020-21 under the national supply arrangements contributed to the overall low year-to-year growth for these products.

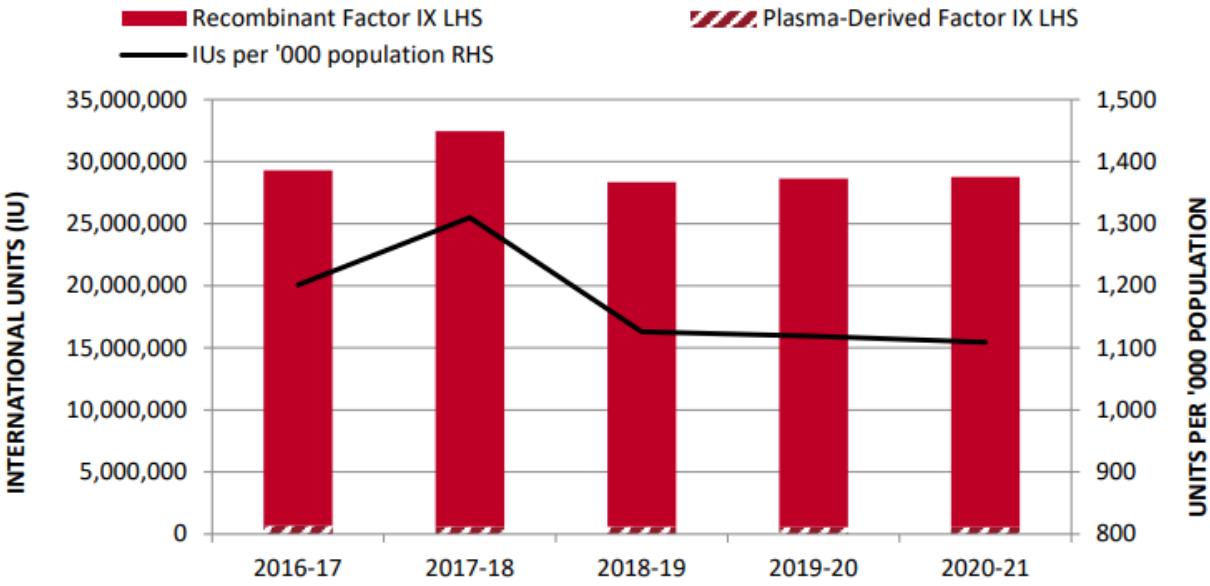


FIGURE 9 - ISSUES OF FACTOR IX PRODUCTS, 2016-17 TO 2020-21 PER '000 POPULATION

Figure 10 and Figure 11 show demand for recombinant factor VIIa decreased by 6.8 per cent and demand for FEIBA decreased by 42.3 per cent compared to 2019-20. Demand for recombinant factor VIIa and FEIBA can change significantly from year to year as a result of the variable needs of a small number of patients. This variability has also been influenced by ongoing clinical trials of new products for haemophilia therapies.

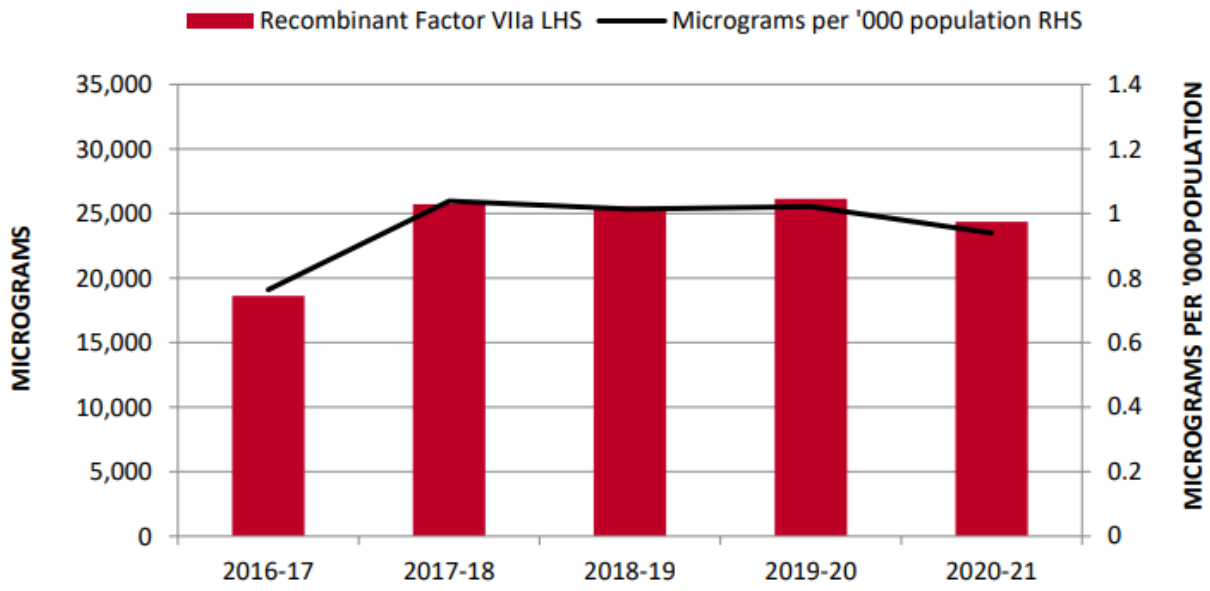


FIGURE 10 - ISSUES OF RECOMBINANT FACTOR VIIA PRODUCTS, 2016-17 TO 2020-21 PER '000 POPULATION

The introduction of Hemlibra (emicizumab) also contributed to the decline in growth as indicated in Figure 11 as patients required fewer or no bypass agents when being treated with this product.

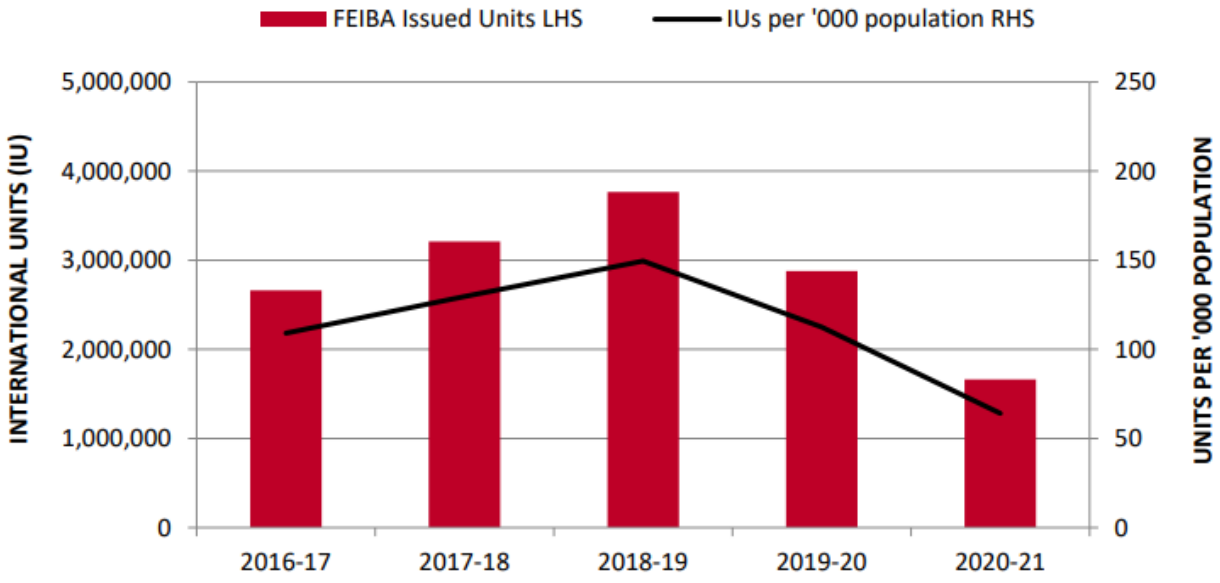


FIGURE 11 - ISSUES OF FEIBA, 2016-17 TO 2020-21 PER '000 POPULATION

Chronology of products supplied

Various products have been supplied through national arrangements. Since 2011-12 the following arrangements for the supply of products have occurred.

TABLE 13 - PRODUCTS BY YEAR

2011-12	• Commenced supply of Kogenate
2012-13	• Ceased supply of vFVIII Recombinate and vFVIII Advate
2014-15	• Commenced supply of Advate and Rixubis
2015-16	• Ceased supply of Factor VII Concentrate • Commenced supply of RiaSTAP
2016-17	• Ceased supply of Kogenate FS
2017-18	• Commenced supply of extended half-life products Eloctate, Alprolix, Adynovate (as a limited trial) • Commenced supply of Novo Thirteen
2020-21	• Eloctate, Alprolix and Adynovate made fully available under national supply arrangements • Commenced supply of Hemlibra (monoclonal antibody)

Appendix E: Glossary of terms

ABDR	Australian Bleeding Disorders Registry
AHCDO	Australian Haemophilia Centre Directors' Organisation
BU (BU/ml)	Bethesda unit (expressed as Bethesda units per millilitre)
DDAVP	Desmopressin (1-desamino-8-D-arginine vasopressin)
EHL	Extended half-life
FEIBA	Factor VIII Inhibitor Bypassing Activity
FVIIa / rFVIIa	Factor VIIa (seven 'a') / Recombinant Factor VIIa
FVIII / rFVIII	Factor VIII (eight) / Recombinant Factor VIII
HFA	Haemophilia Foundation Australia
HMA	Haemophilia A (Factor VIII deficiency)
HMB	Haemophilia B (Factor IX deficiency)
HTC	Haemophilia Treatment Centre (see Appendix B for more information)
IDMS	The NBA's Integrated Data Management System
IU	International Units
mg	milligrams
MyABDR	an app and web site for people with bleeding disorders to record home treatments and bleeds
NBA	National Blood Authority
OBD	Other bleeding disorders
SHL	Standard half-life
VWD	von Willebrand disease
VWF	von Willebrand factor
WFH	World Federation of Hemophilia