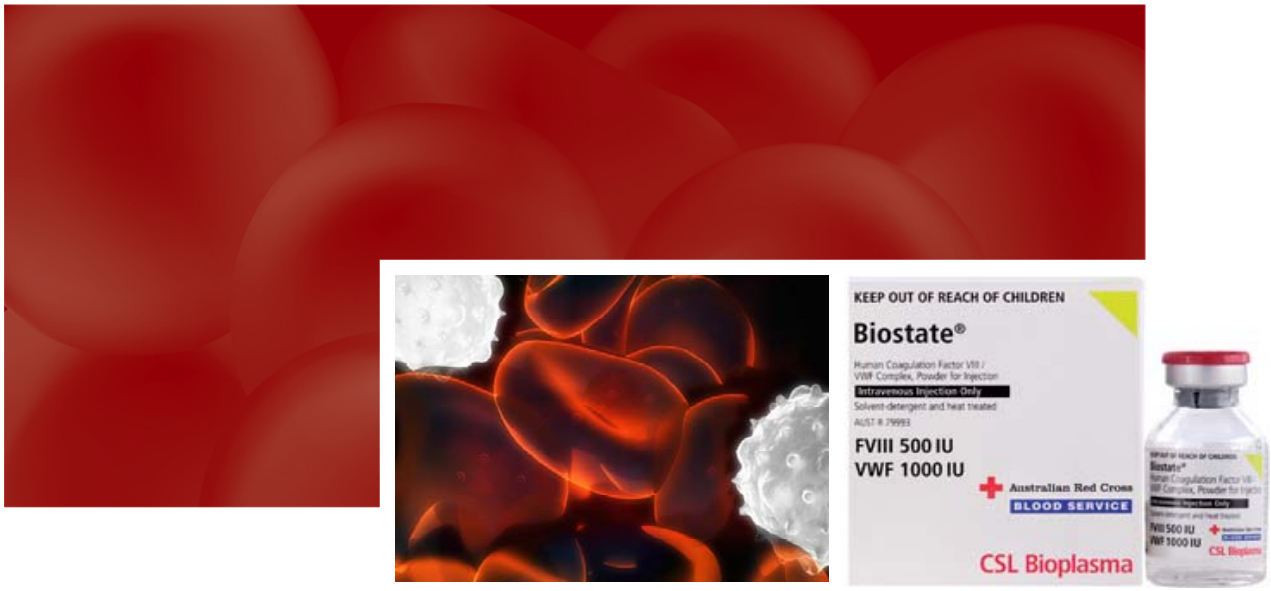

ABDR

Australian Bleeding Disorders Registry

Annual Report 2010-2011

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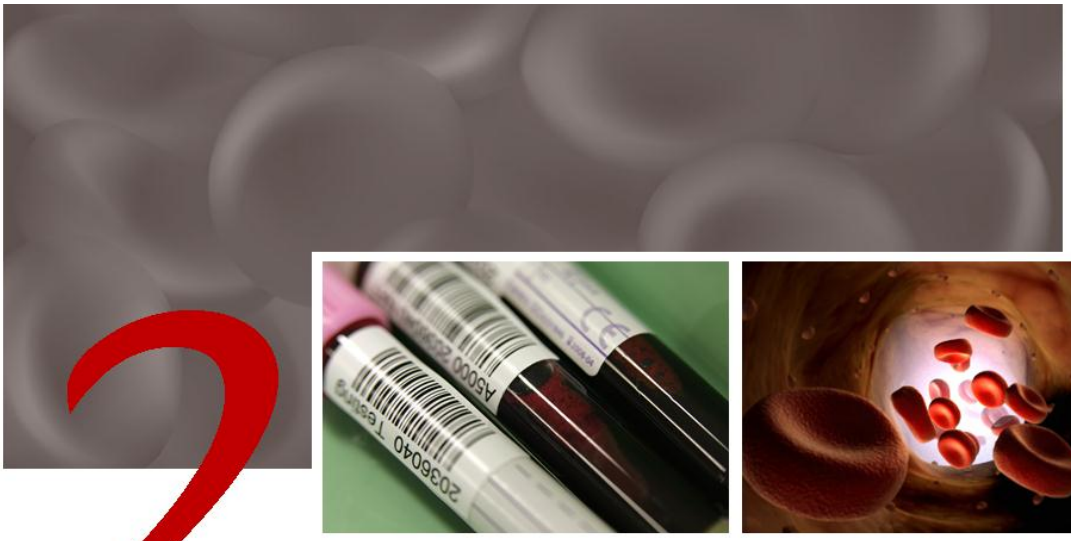
1.1. Glossary (main terms)

Abbreviation	Meaning
NBA	National Blood Authority
HTC	Haemophilia Treatment Centre
AHCDO	Australian Haemophilia Centres Directors' Organisation
HFA	Haemophilia Foundation Australia
ABDR	Australian Bleeding Disorders' Registry
IDMS	Integrated data management system – The NBA's integrated data management system.
PWBD	People with a bleeding disorder
HmA	Haemophilia A (Factor VIII deficiency)
HmB	Haemophilia B (Factor IX deficiency)
vWD	von Willebrand disease
DDAVP	Desmopressin (1-desamino-8-D-arginine vasopressin, abbreviated DDAVP) a derivative of the antidiuretic hormone, used to treat patients with von Willebrand disease. It does not come under the national blood agreement funding arrangements and its use is often not recorded in the NBA's issues database.



2.

Executive Summary

2.1. Purpose

The purpose of this report is to provide an integrated view of current clinical and demographic information on people with bleeding disorders in Australia and the resultant demand for clotting factor products. It draws on data from the Australian Bleeding Disorders Registry (ABDR) and other National Blood Authority (NBA) supply and contract sources. Some international data comparisons have also, where meaningful, been included.

The 2010-11 report represents the second analysis of the ABDR data since a redevelopment in 2008. This analysis builds on feedback from clinicians and other stakeholders following publication of the first report. It also provided impetus to continue data quality improvements.

This report has been developed through the close collaboration of all stakeholders involved in the management and governance of the ABDR, namely:

- Australian Haemophilia Centre Directors' Organisation (AHCDO)
- Haemophilia Foundation of Australia (HFA)
- National Blood Authority (NBA).

2.2. Key observations

The data contained in the report shows:

- between 2009-10 and 2010-11, there was an increase of 283 patients registered in the ABDR, bringing the total number to 5260
- the overall growth in numbers of people with bleeding disorders captured in the Registry was 5.7 per cent; the major contributions to this were growth in numbers captured of people with von Willebrand Disease (vWD) 2.2 per cent, Haemophilia A (HmA) + Haemophilia (HmB) 2.5 per cent and other disorders 1.0 per cent
- the number of patients with a severity ranking of 'severe' increased by 25 (haemophilia patients only). This brings haemophilia patients with a 'severe' ranking to 14 per cent of the total number of all people in the registry;
- there was no significant change in the proportion of people in the registry treated between reports
- there was a large percentage increase in the number of people in the registry with Factor XI deficiency (13.5 per cent) and for platelet disorders (12.4 per cent), the total number of these patients is, however, very small
- there was an 8 per cent increase in the number of females recorded in the registry.

A proportion of these increases seen reflects retrospective data entry of existing patients that were not previously recorded in the national system. Further, these results are tempered by some data quality issues. These data were cleansed to allocate to the correct product use where other data were available. In some areas inconsistent definitions appear to be used for some fields.

3.

Background

3.1. What are bleeding disorders?

3.1.1. Haemophilia

Haemophilia occurs in 1 in 6,000-10,000 males internationally.

In Australia, there are approximately 2,600 people with varied degrees of severity of this condition. There are 2 types of Haemophilia:

- Haemophilia A (classical Haemophilia) is the most common type and caused by a deficiency of blood clotting factor VIII
- Haemophilia B (Christmas Disease) is due to a deficiency of blood clotting factor IX.

Haemophilia is an inherited condition and occurs in families. In one-third of cases, however, it appears in families with no previous history of the disorder. Haemophilia is due to a mutation in the Factor VIII or Factor IX gene which is on the X chromosome. The haemophilia gene is passed down from mother to child through generations. Some women and girls who carry the haemophilia gene may also experience bleeding problems.

The deficiency in clotting factor is associated with recurrent bleeding episodes, usually into the joints, muscles or internally, possibly affecting vital organs. These bleeding episodes, or "bleeds", may occur spontaneously, or as a result of trauma or injury. The bleeding is arrested by infusion of the appropriate clotting factor. Over a period of time recurring bleeding into joints and muscles can cause serious sequelae, including arthropathy, and chronic pain syndromes.

With appropriate treatment haemophilia can be managed effectively.

3.1.2. von Willebrand disease

vWD is a related bleeding disorder which affects both men and women. This disorder is more common and is caused by a deficiency and/or dysfunction of von Willebrand factor.

Table 1 Major bleeding disorders and their cause

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

3.1.3. 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 of vWD and other coagulation factor deficiencies is variable.

Table 2 Severity and the concentration of clotting factors[1]

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.

Notes † 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.

3.1.4. Treatment of bleeding disorders

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 other medical facilities, or at home. The treatments may be regular and preventative (prophylaxis), or on demand (when a bleed occurs).

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, abbreviated as DDAVP). In some patients, therapy is complicated when their body develops inhibitors that destroy the replacement clotting factors and other treatment is necessary.

3.2. Treatment arrangements in Australia

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 coagulation deficiencies. The comprehensive care model ensures that preventative and general treatment on the complex aspects of haemophilia are given in a co-ordinated way by a multi-disciplinary team within the one centre.

HTCs were established under a decision by Australian Health Ministers Advisory Council (AHMAC) in February 2001, to provide a leadership role within their hospital, city and outlying areas to ensure optimal care and an equitable distribution of professional and therapeutic resources, together with responsible record-keeping. Specific roles of these Centres are defined in Appendix A. The locations of the HTCs in Australia are shown in Figure 1.

[1] Modified from Bolton-Maggs, PH & Pasi, KJ 2003, 'Haemophilias A and B', Lancet, 361 (9371), pp. 1801–1809. See also: White GC et al. Definitions in Hemophilia: Recommendation of the Scientific Subcommittee on Factor VIII and Factor IX of the Scientific and Standardization Committee of the International Society on Thrombosis and Haemostasis. *Thromb Haemost* 2001;85:560.

The manner in which these functions are delivered varies between jurisdictions including the degree to which they:

- operate within centralised compared to distributed treatment models;
- focus on treating paediatric or adult patients compared to treating all age groups; and
- have relatively small numbers of patients compared to relatively large numbers – commonly based on geography.

Nevertheless, there are some patients that receive product from clinicians not associated with a HTC. The proportion of product that is used in these circumstances varies across jurisdictions and there is some variability in the data capture rate within jurisdictions. Accordingly, data on total volume of products recorded from the ABDR may not be consistent with data provided by the NBA from other sources.

Figure 1 Location of HTCs

A description of the aims and governance of HTCs is provided at Appendix A.

3.3. What is the ABDR?

The Australian Bleeding Disorders Registry (ABDR) is a database that is designed to collect all clinical information related to the treatment of people with bleeding disorders. This includes information about patient diagnosis, including 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 current version of the ABDR has been in existence since December 2008 and background on the development of the system is at Appendix A.

3.4. ABDR Management and Governance

The ABDR is managed on a day to day basis by the NBA in accordance with the guidance and policy oversight provided by the ABDR Steering Committee. The Committee consists of representatives of the key stakeholders involved in the clinical management, advocacy and funding of treatment for people with bleeding disorders. In 2010/11 the Steering Committee representatives were:

- Dr John Rowell – Chair – representative of Australian Haemophilia Centre Directors' Organisation (AHCDO)
- Dr Chris Barnes – Chair of Australian Haemophilia Centre Directors' Organisation (AHCDO)
- Ms Sharon Caris – Executive Director, The Haemophilia Foundation Australia (HFA)
- Mr Geoff Simon, Queensland Health – Jurisdictional Blood Committee nominee
- Ms Stephanie Gunn – Acting General Manager, National Blood Authority

3.4.1. Accessing the data

Patient confidentiality is paramount, with personal data available only to the individual treating HTC and levels of authorisation/access determined by the interaction of staff with individual patients. National reporting is with aggregate, de-identified, data. All use of data, and discussion about the system, other than within the individual HTC, is considered by the ABDR Steering Committee.

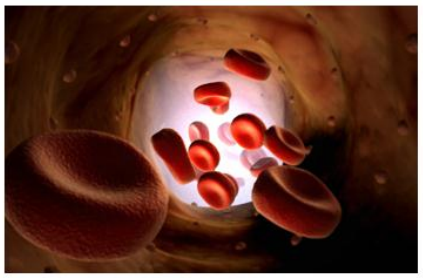
The database provides a capability for all HTC staff to enter data on the interactions with patients to provide treating clinicians with a comprehensive picture of the health and wellbeing of patients. The database provides for both real time ordering of product and retrospective collection of data to provide national clotting factor usage data to inform and assist planning and funding. Future development of the system will provide for inclusion of information on physiotherapy and social work interactions with patients.

To ensure appropriate management of the information, the NBA has instigated a detailed governance framework for a data analyst to use a Business Intelligence tool to store and access the de-identified data. This tool is called Big Red. It includes data marts for the Integrated Data Management System (IDMS – which records products issued by suppliers) and the ABDR. Big Red can provide fixed reports and *ad hoc* queries.

3.5. Data quality issues

There are a number of data quality issues in the ABDR. These include incomplete records with missing fields or "not stated" entries. As the system is still relatively young, the data in some fields is also characterised by a lack of consistency in the interpretation of specific fields. These caveats are highlighted on specific tables. The Steering Committee has initiated strategies to improve the data quality and over time the reporting from the ABDR will become more robust.

For this report the NBA has expended more effort on cleaning the data and filling in gaps, particularly in relation to cases where clotting factor from hospital stocks was used. The clotting factor use corrections applied to 2009-10 and 2001-11.



4.

Patients in the Registry

This section of the report presents the key patient data collected by the ABDR. The determination of when a patient is in the register is based on their record creation date. Where another data field implied an earlier creation date the earlier date was used.

Box 1: Data Comparisons Note

The data extract used in this year's report reflects the current state of the data. If a patient was diagnosed in 2009-10 but was only added to the registry during 2010-11 they will now be included in the count of people in the registry at 30 June 2010. This explains difference to the previously reported numbers for 30 June 2010.

The largest contributions to the revisions to the "in registry on 30 June 2009" are in vWD (38 per cent of total revisions). Others are: 37 per cent; HmA 29 per cent; and unknown 27 per cent. These seem to indicate that there is now better coverage of vWD patients in the Registry. The unknown diagnoses have been better allocated to a meaningful diagnosis.

These changes, while complicating the ability to undertake trend analysis at this stage, do reflect an improved data capture rate for the Registry and this scale of adjustment to previous years data is not expected to occur in future years.

In summary – where the data for 2010-11 is compared with the numbers published in the 2009-10 report, growth is likely to reflect improvements to the data rather than simply changing patterns in numbers of patients with particular diagnoses.

4.1. By diagnosis

Table 3 Number of people in the register and treated by latest broad diagnosis

	Number in register at 30 Jun 2009	Number who Received product in 2008-09	Number in register at 30 Jun 2010	Number who Received product in 2009-10	Number in register at 30 Jun 2011	Number who Received product in 2010-11
HmA†	1918	693	2015	833	2111	858
HmB†	466	153	490	186	517	185
vWD	1714	101	1856	183	1966	153
Other Factor Deficiency	235	21	261	18	284	22
Platelet Disorder	161	1	170	4	191	8
Vascular	5	0	5	0	6	0
Other	138	2	143	0	146	2
Unknown	35	0	37	0	39	0
Total	**4672**	**971**	**4977**	**1224**	**5260**	**1228**

Note: † Includes some female carriers who are symptomatic.
As noted in Box 1 the historical data has been revised from the 2009-10 report reflecting changes in data capture rates between years.

Table 4 Number of people in the register and treated by detailed diagnosis HmA, HmB & vWD

Detailed diagnosis	Number in register at 30 Jun 2009	Number who Received product in 2008-09	Number in register at 30 Jun 2010	Number who Received product in 2009-10	Number in register at 30 Jun 2011	Number who Received product in 2010-11
Factor VIII Deficiency (Haemophilia A)	1670	680	1745	812	1814	843
Asymptomatic Carrier Factor VIII Deficiency (Haemophilia A) †	224	7	238	12	261	8
Acquired Factor VIII Inhibitor (Acquired Haemophilia A)	24	6	32	9	36	7
Factor IX Deficiency (Haemophilia B)	412	152	430	178	446	181
Asymptomatic Carrier Factor IX Deficiency (Haemophilia B) †	54	1	60	8	71	4
von Willebrand Disease - Uncharacterised	476	7	500	17	519	13
von Willebrand Disease Type 1	914	33	1005	79	1076	65
von Willebrand Disease Type 2 - Uncharacterised	101	9	111	20	118	16
von Willebrand Disease Type 2A	59	10	61	15	65	13
von Willebrand Disease Type 2B	41	6	44	11	43	8
von Willebrand Disease Type 2M	56	12	66	11	73	10
von Willebrand Disease Type 2N	18	2	19	2	21	3
von Willebrand Disease Type 3	42	18	43	24	43	25
Acquired von Willebrand Factor Disease	7	4	7	4	8	0
Total	**4098**	**947**	**4361**	**1202**	**4594**	**1196**

Notes: † The practice of applying definitions does at this stage vary between HTCs and future work will focus on ensuring consistent approaches are used.

It is rare for asymptomatic carriers to require product. They do not get spontaneous haemorrhages, but some may need product support in times of major trauma, surgery or at parturition.

‡ Not all with von Willebrand disease are treated through HTCs and these figures therefore do not represent the total number of vWD patients in Australia. Note also that the specific classification of vWD is incomplete in the ABDR at this stage.

As noted in Box 1 the historical data has been revised from the 2009-10 report to give a more accurate indication of trends.

Table 5 Number of people in the registry and treated by diagnosis of "other disorders"

Detailed diagnosis	Number in registry at 30 Jun 2009	Number who received product in 2008-09	Number in registry at 30 Jun 2010	Number who received product in 2009-10	Number in registry at 30 Jun 2011	Number who received product in 2010-11
Factor V Deficiency	11	2	13	1	13	1
Factor VII Deficiency	51	6	52	6	53	6
Factor X Deficiency	11	2	12	1	12	1
Factor XI Deficiency	115	3	133	3	151	5
Factor XII Deficiency	16	0	17	0	17	0
Factor XIII Deficiency	16	6	16	7	17	7
Fibrinogen – Afibrinogenaemia	6	2	7	0	9	1
Fibrinogen – Dysfibrinogenaemia	7	0	8	0	9	1
Fibrinogen - Hypofibrinogenaemia	2	0	2	0	2	0
Fibrinogen Dysfunction – Uncharacterised	0	0	1	0	1	0
Vascular disorders - Ehlers Danlos Syndrome	5	0	5	0	6	0
Other (Please specify) †	138	2	143	0	146	2
Unknown	35	0	37	0	39	0
Total	**413**	**23**	**446**	**18**	**475**	**24**

Note: † This represents incomplete data which will be addressed for the next year's report.
Those with fibrinogen disorders may have been treated with the only funded source of fibrinogen – cryoprecipitate which is not routinely collected within the ABDR.

As noted in Box 1 the historical data has been revised from the 2009-10 report to give a more accurate indication of trends.

Table 4 and Table 5 indicate that the growth in numbers of people registered in the ABDR is consistent with the historical proportion of this group of patients to the overall Australian population. This proportion and the growth is largely consistent with that illustrated in the United Kingdom Haemophilia Centre Doctors' Organisation (UKHCDO[2]) annual report. Both show a growth in the year of about 5 per cent. It is expected that with further improvements in data quality, these comparisons will be made with greater confidence.

[2] http://www.ukhcdo.org/

Table 6 Number in registry and receiving product by diagnosis of "Platelet disorders"

Detailed diagnosis	Number in registry at 30 Jun 2009	Number who received product in 2008-09	Number in registry at 30 Jun 2010	Number who received product in 2009-10	Number in registry at 30 Jun 2011	Number who received product in 2010-11
Platelet - Bernard-Soulier	1	0	1	0	1	0
Platelet - Glanzmann's Thrombasthenia	6	0	7	2	10	1
Platelet - Macrothrombocytopenias	0	0	0	0	1	0
Platelet - May Hegglin	4	0	4	0	4	0
Platelet - Primary Secretion Defect	1	0	1	0	1	1
Platelet - Storage Pool (Dense Granule) Deficiency	10	0	14	0	19	0
Platelet - Uncharacterised	139	1	143	2	155	6
Total	**161**	**1**	**170**	**4**	**191**	**8**

Notes: † This represents incomplete data which will be addressed for the next year's report.
Platelet disorders may be treated with DDAVP, platelet infusion (not routinely collected in the ABDR) or rVIIa (collected within the ABDR).

As noted in Box 1 the historical data has been revised from the 2009-10 report to give a more accurate indication of trends.

Table 6 shows that data on platelet disorders is not heavily recorded in the ABDR. These disorders tend to be under reported and are typically treated at facilities outside the HTC framework and will, therefore, not necessarily be recorded in the ABDR. Nevertheless, this field will be more valuable when clear definitions are applied to those patients currently recorded in the ABDR as uncharacterised and this will be a priority for future work.

Table 7 People in the register at 30 Jun 2011 by broad age group, diagnosis and severity

	Paediatric 0 – 19 years		Adult 20 years and over		Total	
	Number in registry at 30 Jun 2011	Number who Received product in 2010-11	Number in registry at 30 Jun 2011	Number who Received product in 2010-11	Number in registry at 30 Jun 2011	Number who Received product in 2010-11
HmA	587	334	1524	513	2111	847
Severe	289	242	362	256	651	498
Moderate	71	46	190	83	261	129
Mild	219	44	856	171	1075	215
Not Applicable	1	0	29	2	30	2
Unknown	7	2	87	1	94	3
HmB	125	57	392	126	517	183
Severe	49	37	54	38	103	75
Moderate	23	16	88	38	111	54
Mild	50	4	226	49	276	53
Not Applicable	1	0	3	0	4	0
Unknown	2	0	21	1	23	1
vWD	446	29	1520	122	1966	151
Total	**1158**	**420**	**3436**	**761**	**4594**	**1181**

Note: Mild cases of HmA, HmB and vWD are often treated with DDAVP and the use of this product may not be recorded in the ADBR.
† Includes some female carriers.
‡ Not applicable – may represent data input inconsistencies or incomplete data, and will be addressed for next year's report.
Unknown represent patients not yet classified or symptomatic carriers not classified.
As noted in Box 1 the historical data has been revised from the 2009-10 report to give a more accurate indication of trends.

Table 7 illustrates that a higher proportion of paediatric patients with HmA receive product than do adults and that this difference is less marked for patients with HmB. Moreover, the number of paediatric HmA and HmB in the Registry increased by 7.7 per cent whereas the adult HmA and HmB in the Registry increased by 4.3 per cent.

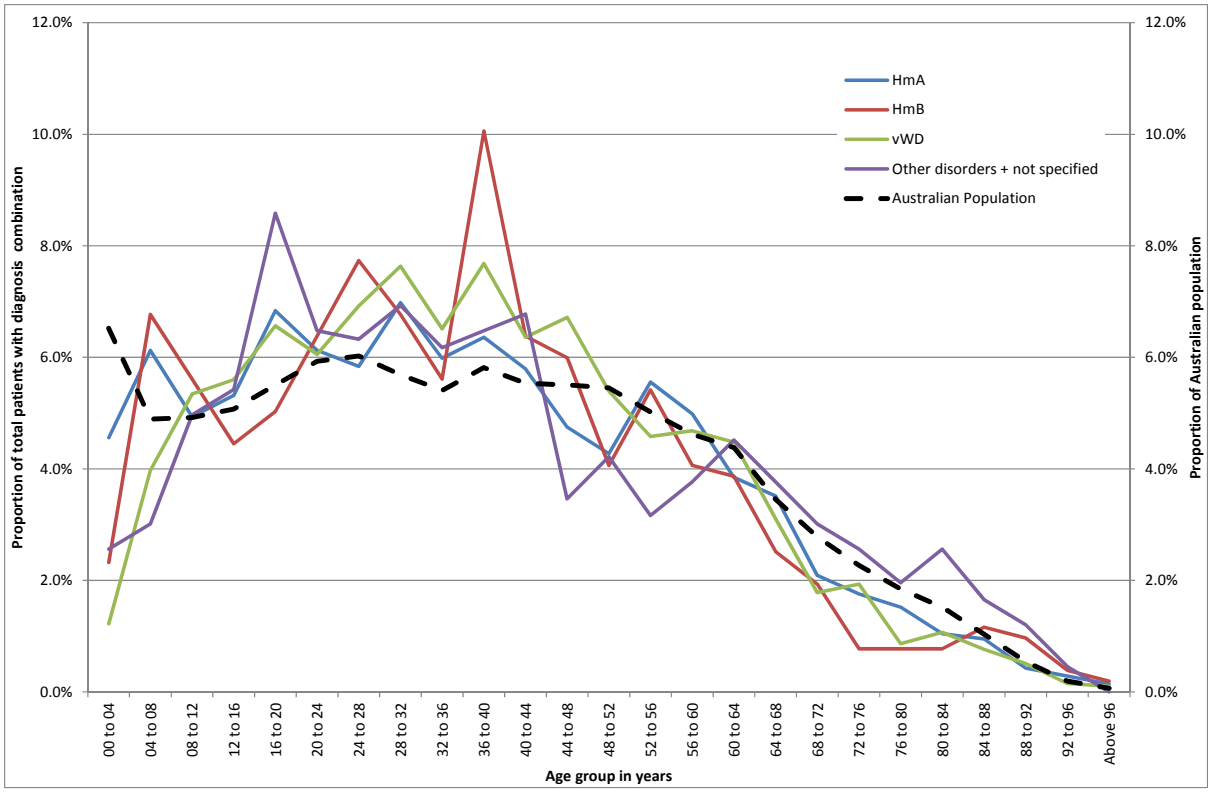


Figure 2 Age, in years, distribution of people in the ABDR by diagnosis at 30 June 2011

Figure 2 illustrates that the proportion of patients with all disorders currently aged between 35-40 years is higher than the proportion of the Australian population at that age group. Notably, the proportion of patients aged 5-10 with HmB and those aged between 15 and 20 with HmA are also substantially higher than the proportion of the Australian population at that age. However, the proportion of patients aged between 60 and 80 with the most common forms of bleeding disorders is substantially lower than that for the rest of the population. The proportion with 'other bleeding' disorders, which are more typically associated with ageing and diagnosis when undergoing associated medical treatments, is the reverse of this picture.

Table 8 von Willebrand Disease in the registry at 30 Jun 2011 by broad age group and vWD classification

	Paediatric 0 – 19 years		Adult 20 years and over		Total	
	Number in registry at 30 Jun 2011	Number who received product in 2010-11	Number in registry at 30 Jun 2011	Number who received product in 2010-11	Number in registry at 30 Jun 2011	Number who Received product in 2010-11
von Willebrand Disease - Uncharacterised	101	2	418	11	519	13
von Willebrand Disease Type 1	242	10	834	54	1076	64
von Willebrand Disease Type 2 - Uncharacterised	41	5	77	11	118	16
von Willebrand Disease Type 2A	13	2	52	10	65	12
von Willebrand Disease Type 2B	9	1	34	7	43	8
von Willebrand Disease Type 2M	25	3	48	7	73	10
von Willebrand Disease Type 2N	1	0	20	3	21	3
von Willebrand Disease Type 3	14	6	29	19	43	25
Total	**446**	**29**	**1512**	**122**	**1958**	**151**

Notes: As noted in Box 1 the historical data has been revised from the 2009-10 report to give a more accurate indication of trends.

vWD is typically a mild disorder and data collected within the ABDR represents only a proportion of the total population affected with vWD. The primary purpose of ABDR is to record the use of products (in this case plasma derived FVIII) and a higher proportion of these patients will be Type 2/3 compared to Type 1. Type 3 is overrepresented as they are the most likely patients to have treatment and prophylaxis.

These data will be interesting to track over time as there is now an increased awareness of this disease and it is often diagnosed during surgical interventions. As the population ages and seeks more surgical intervention, more von Willebrand patients are likely to be diagnosed, treated and included in the ABDR.

Table 9 Comparison of the proportion of patients in the registry and treated, UK and Australia, major diagnoses 2011

	Female			Male		
	Number in registry at 30 Jun 2011	Number who received product in 2010-11	Proportion treated	Number in registry at 30 Jun 2011	Number who received product in 2010-11	Proportion treated
Australia						
HmA	337	25	7.4%	1772	833	47.0%
HmB	97	13	13.4%	420	172	41.0%
vWD	1135	90	7.9%	831	63	7.6%
Other conditions	337	15	4.5%	329	17	5.2%
UK[†]						
HmA	1119	63	5.6%	5372	2919	54.3%
HmB	347	47	13.5%	1134	611	53.9%
vWD	5770	666	11.6%	3342	386	11.6%

Notes: [†] The UK's reporting year is 1 April to 30 March whereas Australia's 1 July to 30 June.

The UK population is approximately three times that of Australia. The treatment rates shown in Table 9 are comparable between Australia and the UK although on current data, the proportion of Australian HmB patients receiving product does appear lower. The incidence of most conditions seems higher in the UK than Australia. This may reflect some patients' participation in clinical trials which would mean that their treatment may not be recorded in the ABDR.

4.2. By geography

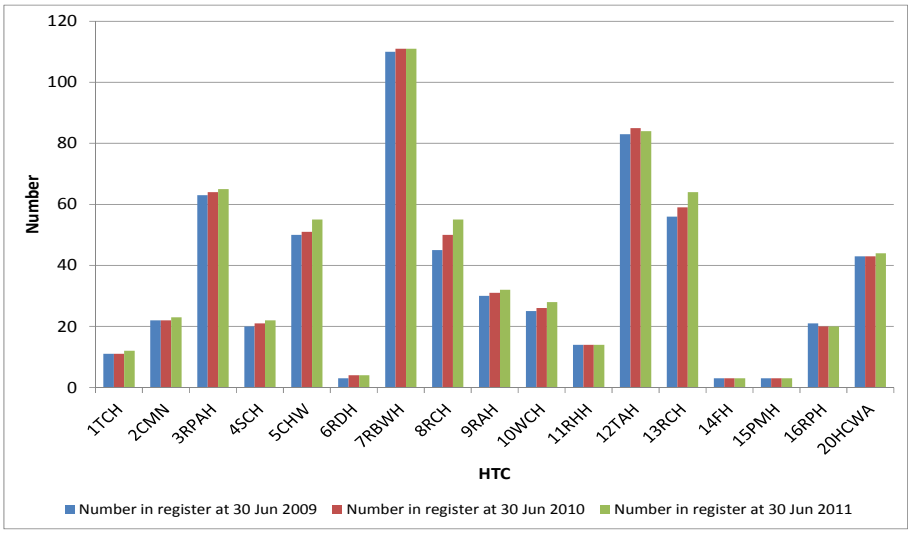


Figure 3 Distribution of severe Haemophilia A patients by Primary HTC by year

Note: Some records in the database, for Western Australia, are still recoded to against the old HTC codes rather than the new 20HCWA.
Some data in this table may contain a small number of duplicate records. These will form part of the data quality focus in 2012.

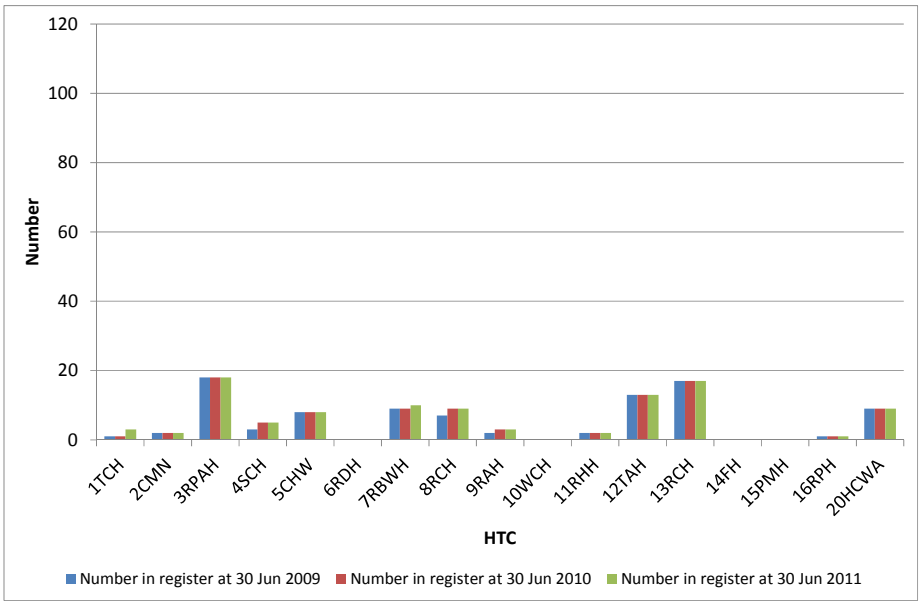


Figure 4 Distribution of severe Haemophilia B patients by Primary HTC by year

Note: Some records in the database, for Western Australia, are still recoded to against the old HTC codes rather than the new 20HCWA.

The figures above illustrate variability in the numbers of severe patients registered in each HTC mainly reflecting their catchment size and modest growth in numbers of severe patient numbers over time.

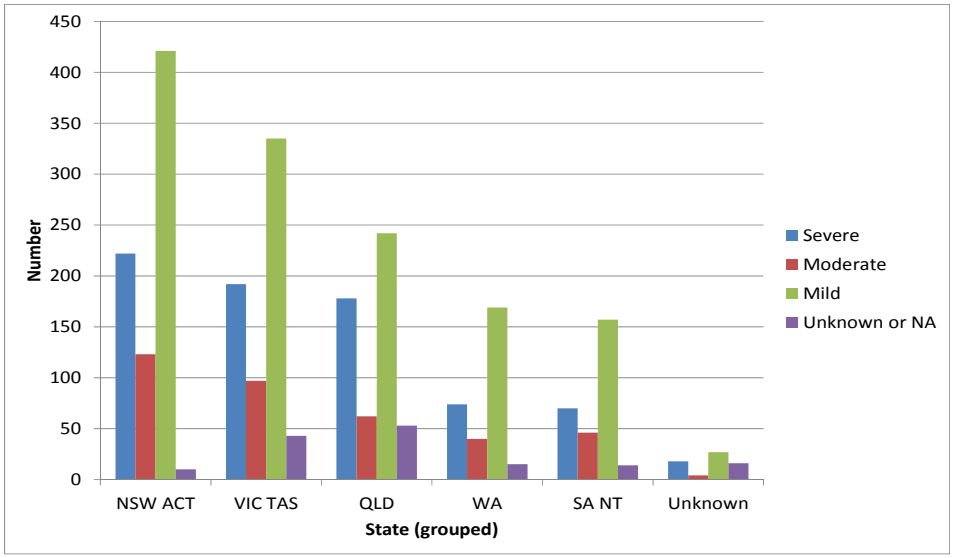


Figure 5 People in the registry at 30 June 2011 with Haemophilia A or Haemophilia B by severity and home jurisdiction (grouping small jurisdictions)

Figure 5 illustrates that numbers of patients with haemophilia are largely consistent with population size. The category 'unknown' includes patients who may be temporally overseas and patients who have less severe conditions and have not had recent contact with their HTC.

4.3. Incidence of major disorders

Table 10 shows that Haemophilia A in males has the highest incidence with nearly 15 per 100,000 males. The next highest incidence is for von Willebrand disease in females at nearly 9 per 100,000.

Table 10 Incidence of major disorders in Australia people with bleeding disorders per 100,000 of relevant population

	Female			Male			Persons		
	In registry at 30 Jun 2009	In registry at 30 Jun 2010	In registry at 30 Jun 2011	In registry at 30 Jun 2009	In registry at 30 Jun 2010	In registry at 30 Jun 2011	In registry at 30 Jun 2009	In registry at 30 Jun 2010	In registry at 30 Jun 2011
HmA all	2.5	2.7	3.0	15.0	15.4	15.7	8.7	9.0	9.3
HmA severe	0.1	0.1	0.1	5.5	5.5	5.6	2.8	2.8	2.9
HmB all	0.7	0.7	0.9	3.6	3.7	3.7	2.1	2.2	2.3
vWD all	8.9	9.5	10.0	6.7	7.1	7.4	7.8	8.3	8.7

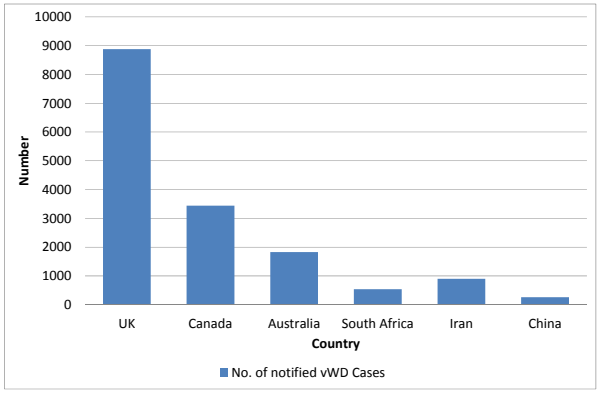
The numbers in

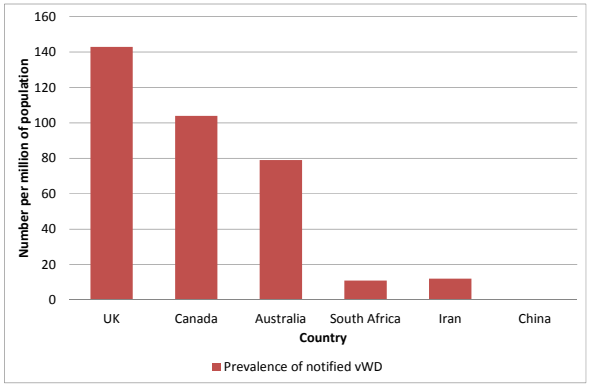
Table 10 are calculated by dividing the number of people in the registry at the date with the broad diagnoses by the total corresponding Australian estimates population at the same date and multiplying by 100,000

Table 11 Incidence of bleeding disorders selected countries 2009 (per 100,000)

Country	Population	Number of PWH[3] (HmA or HmB)	Number of people with vWD	Number of people with OBD[4]	Number of PWH per 100,000	Number of people with vWD per 100,000	Number of people with OBD per 100,000
Australia	**21,515,754**	**2,283**	**1,904**	**1,652**	**10.6**	**8.8**	**7.7**
France	64,057,792	5,153	1,017	320	8.0	1.6	0.5
Germany	82,282,988	4,230	4,674	–	5.1	5.7	–
Netherlands	16,783,092	1,397	257	65	8.3	1.5	0.4
Spain	40,548,753	1,985	710	210	4.9	1.8	0.5
Sweden	9,074,055	1,020	1,538	–	11.2	16.9	–
United Kingdom	61,284,806	6,460	8,773	6,308	10.5	14.3	10.3
United States	310,232,863	16,667	12,524	1,607	5.4	4.0	0.5

Source: World Federation Hemophilia Report on the annual global survey 2009





Source: Favaloro EJ, von Willebrand Disease: Local Diagnosis and management of a Globally Distributed Bleeding Disorder; Semin Thromb Hemost (2011) V 37:440-455

[3] PWH – people with haemophilia.

[4] OBD – other bleeding disorders.

Figure 6 Number and prevalence of notified vWD Cases (Source Population) Registry Data

Table 12 Incidence of Haemophilia A in males in OECD countries (per 100,000)

Country	1998	1999	2000	2001	2002	2003	2004	2005	2006	Mean
Iceland	37.7	NA	NA	39.4	NA	39.3	38.1	38.5	NA	38.6
United Kingdom	19.0	19.4	17.4	17.6	NA	17.2	NA	22.6	20.7	19.1
Netherlands	15.3	16.0	15.9	17.7	NA	17.5	18.6	18.0	18.5	17.2
New Zealand	18.5	17.0	16.7	21.6	NA	17.8	11.7	12.3	21.9	17.2
Ireland	12.5	16.5	17.5	16.7	NA	16.6	17.8	18.9	18.3	16.8
Sweden	15.5	16.2	14.9	NA	NA	14.9	15.0	NA	NA	15.3
Switzerland	14.5	13.3	14.6	11.9	NA	12.1	13.2	12.7	14.2	13.3
Canada	11.9	13.1	12.6	12.5	12.4	NA	14.0	14.3	14.6	13.2
Denmark	11.8	11.8	12.5	12.4	NA	12.6	12.1	13.1	NA	12.3
Czech Republic	12.2	12.2	12.2	NA	NA	NA	NA	NA	NA	12.2
France	12.7	12.7	14.8	NA	NA	NA	NA	9.8	11.0	12.2
Greece	10.7	10.8	12.2	12.4	NA	12.4	12.8	13.0	13.0	12.2
Belgium	10.5	11.8	12.1	12.4	12.4	12.3	NA	NA	NA	11.9
Norway	11.6	11.5	11.6	NA	NA	11.9	NA	12.3	12.2	11.9
Italy	9.0	12.3	NA	NA	NA	NA	13.8	13.8	9.4	11.7
Australia	**10.8**	**10.6**	**10.5**	**10.4**	**NA**	**NA**	**8.8**	**12.8**	**13.5**	**11.1**
Luxembourg	10.0	9.0	13.5	NA	NA	NA	NA	NA	NA	10.8
Germany	NA	13.2	9.6	9.8	NA	10.0	10.0	10.0	10.0	10.4
Portugal	8.7	8.7	8.7	8.6	NA	10.3	9.6	9.6	9.7	9.2
Finland	NA	NA	NA	NA	NA	8.6	NA	9.0	9.1	8.9
Spain	10.9	8.5	8.5	10.0	NA	9.7	7.6	7.6	7.6	8.8
Austria	NA	8.4	8.4	NA	NA	8.7	8.7	NA	NA	8.5
United States	7.6	7.5	7.7	7.8	NA	7.6	7.8	7.9	8.0	7.8
Japan	5.3	5.5	NA	5.9	NA	6.2	NA	6.3	6.5	5.9
Korea	4.7	5.0	5.2	5.3	5.3	NA	5.5	5.8	5.9	5.3

Source: World Federation of Hemophilia – Facts and figures (December 2010)

Table 12 shows that of the OECD countries Australia is placed in the middle for documented incidence of Haemophilia A.

Table 13 Incidence of Haemophilia A in males in selected countries by severity (per 100,000)

Country	Year	Overall	Severe	Severe as a proportion of overall
Canada	2008	14.3	4.3	30%
Greece	1992	12.0	3.6	30%
Italy	2006	9.5	4.8	51%
Netherlands	2001	11.7	NA	NA
United Kingdom	2008	21.5	7.1	33%
United States	1998	10.4	4.4	42%
Australia – In registry (current ABDR at 30 June)	2011	15.7	5.6	36%

Source: World Federation of Hemophilia – Facts and figures (December 2010); ABDR

Table 13 shows that proportion of severe cases in Australia is broadly comparable to other countries.

4.4. By age distribution

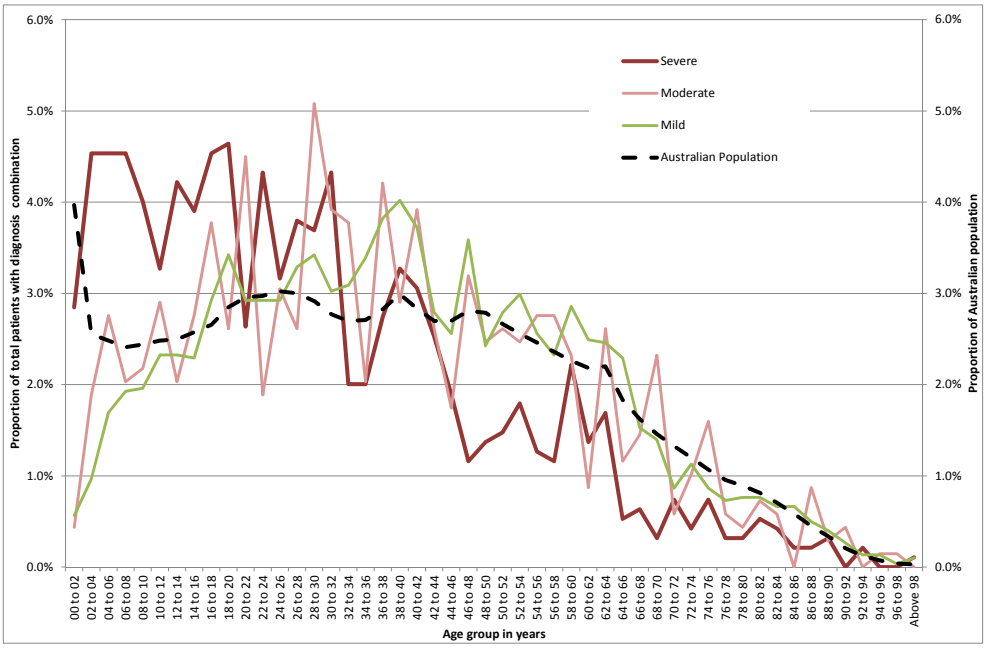


Figure 7 Age distribution of people in the registry at 30 June 2011 by severity

In Figure 7 we see that the age distribution of those living with severe diagnosis is much younger than the other groups and the Australian population as a whole. There is also a dip in the distribution of the severe group around the ages between 40 and 50. The higher proportion at younger ages reflects early diagnosis of the more severely affected. Note severity is not applied consistently for von Willebrand patients. ***Figure 8*** show the age distribution for HmA and HmB only where there is a more consistent definition of severity applied.

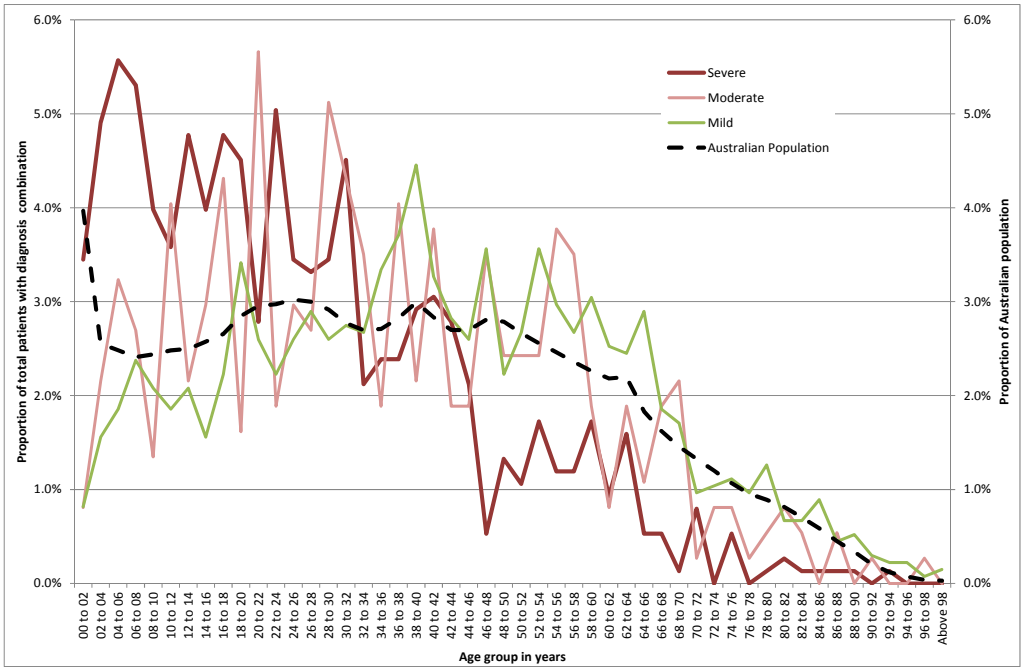


Figure 8 Age distribution of people in the registry at 30 June 2011 with Haemophilia A and Haemophilia B by severity

4.5. By Inhibitors

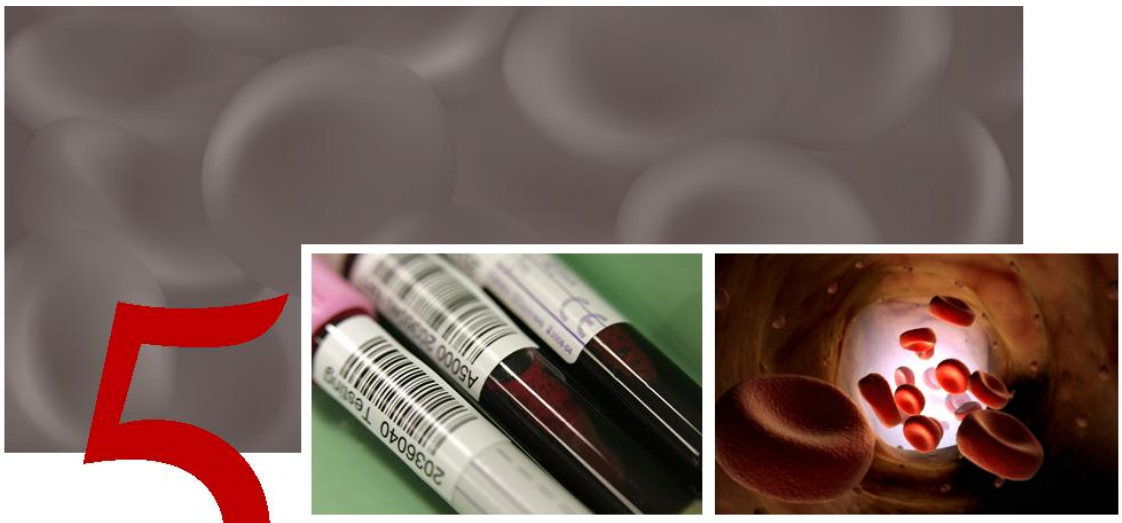
Table 14 Number of patients with inhibitors and comparison with UK in 2010-11

	Severe			Moderate			Mild			Total	
	Inhibitor present	Proportion of total with inhibitors	Total Severe	Inhibitor present	Proportion of total with inhibitors	Total Moderate	Inhibitor present	Proportion of total with inhibitors	Total Mild[†]	Proportion of total with inhibitors	Total in Registry at 2011
Australia											
HmA	133	20.4 %	651	15	5.7%	261	37	3.4%	1075	9.3%	1987
HmB	7	6.8%	103	0	0.0%	111	0	0.0%	276	1.4%	490
vWD[‡]	2	1.4%	138	0	0.0%	251	0	0.0%	1577	0.1%	1966
UK											
HmA	374	20.1 %	1860	36	6.7%	534	59	2.0%	2976	8.7%	5370
HmB	15	3.7%	401	0	0.0%	244	1	0.2%	486	1.4%	1131
vWD	6	4.8%	125	2	1.2%	161	4	0.1%	8214	0.1%	8500

† For vWD severity is not always defined. Included in the mild vWD are those with severity 'mild', 'not applicable' and 'unknown'.

‡ Inhibitors are usually only seen in type 3 (severe) VWD - so if the number of type 3(31) were to be used then becomes similar to UK proportion

There is some concern at the data entry consistency in this field, however, the Australian data appears broadly comparable with the UK data.



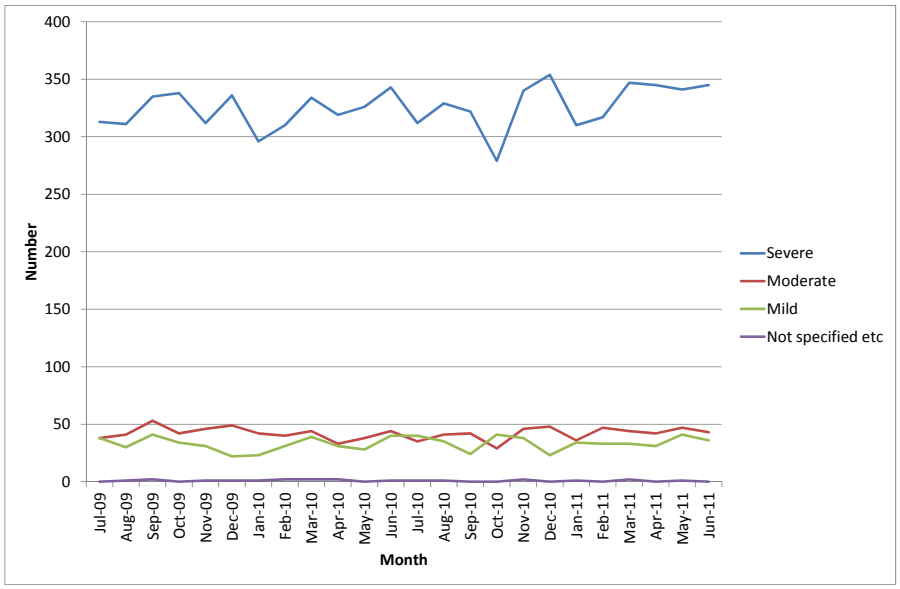
5.

Patients who received treatment

The following graphs are based on product use reported in the ABDR

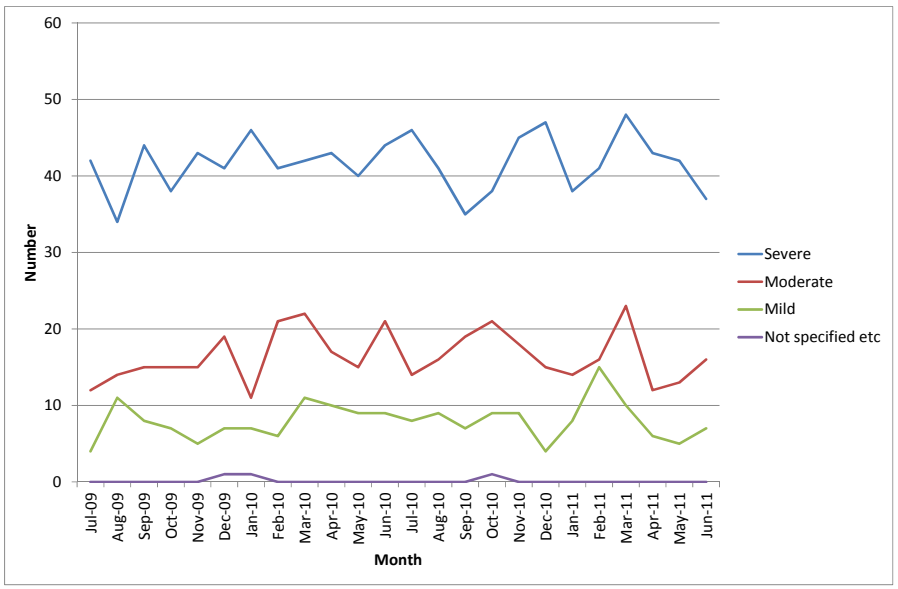
5.1. By severity

As would be expected, more severe patients are treated more often and receive more product. It is important for supply planning purposes to understand trends in the proportion of patients diagnosed as severe to ensure adequacy of supply and likely demand in the future.



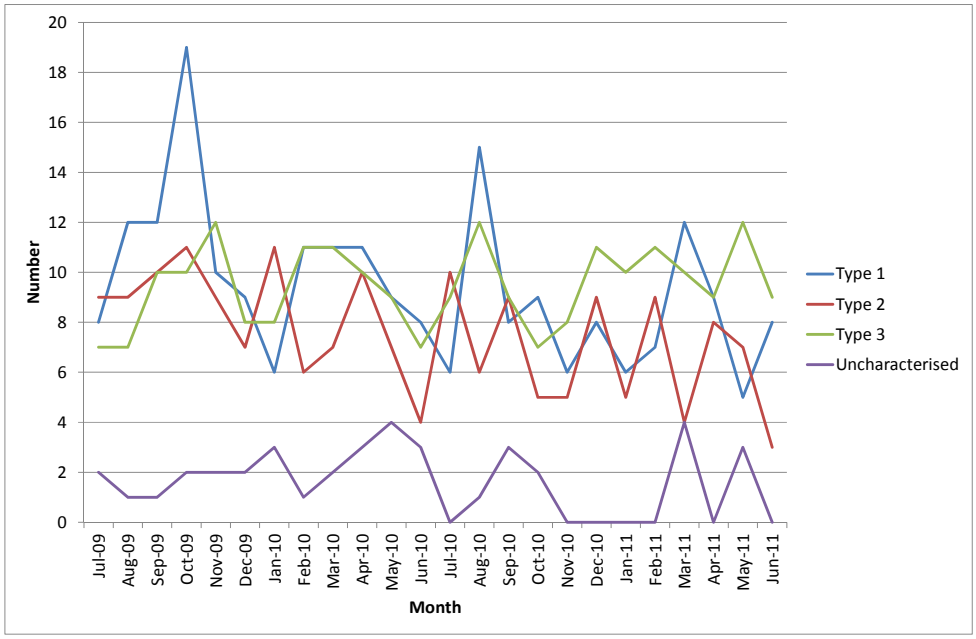
Source: ABDR

Figure 9 HmA (Symp + Asymp) – Number of patients receiving treatment by severity



Source: ABDR

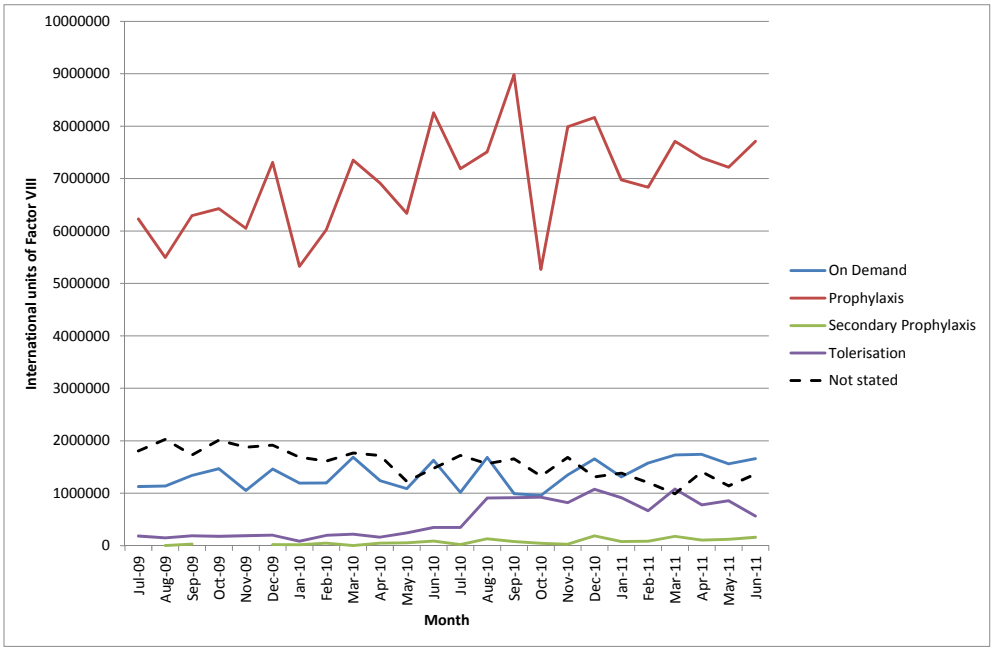
Figure 10 HmB (Symp + Asymp) – Number of patients receiving treatment by severity



Source: ABDR

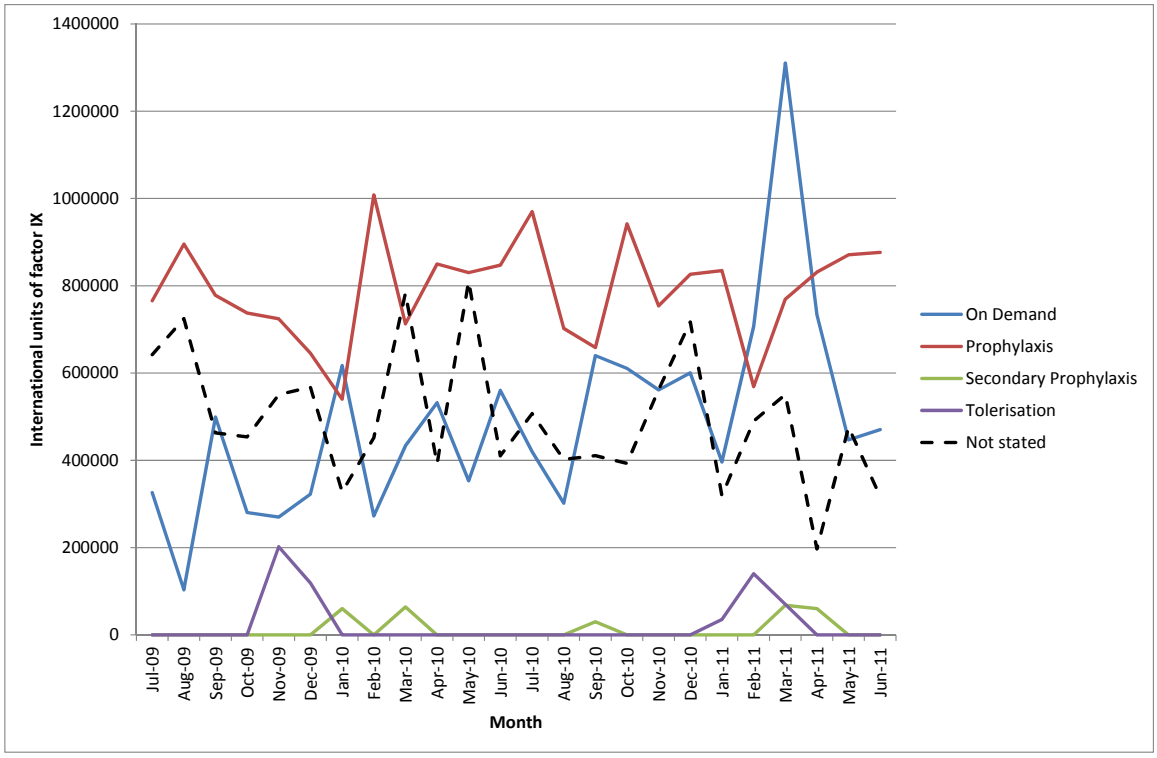
Figure 11 vWD – Number of patients receiving treatment by type of vWD

As would be expected, a higher proportion of those patients with severe diagnosis, receive treatment during the year although the pattern for patients with vWD is less predictable than other diagnoses as numbers are small. Note that many patients with vWD particularly Type 1 will receive DDAVP.



Source: ABDR

Figure 12 International Units of FVIII received by HmA patients by treatment regimen



Source: ABDR

Figure 13 International Units of FIX received by HmB patients by treatment regimen

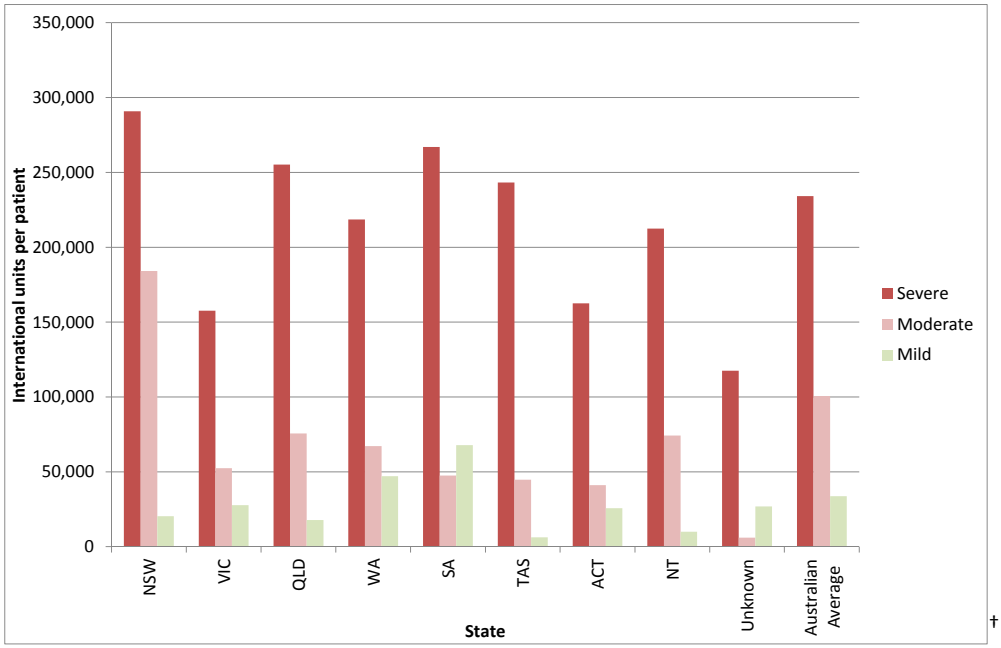
Figure 12 and Figure 13 show the impact of prophylaxis on the volume of clotting factors used in the treatment of people with haemophilia. For Haemophilia A, most of the product is used for prophylaxis.

Last year's report included a section on use by treatment purpose. This year's report does not include a section based on purpose due to the ongoing concerns of the data quality and difficulties in consistently coding the purpose field. The purpose field in the ABDR still needs further clarification. The classifications by purpose currently provided are not mutually exclusive and some patients may have been counted more than once. Further, different HTCs or clinicians may interpret them differently. The definitions and data entry protocols will be an area of focus for future work. A likely revamp of this field will be to record this information by:

- Location – home or hospital
- Regimen – prophylaxis or on demand
- Purpose – bleed, surgery, procedure or prevention (prophylaxis).

5.2. National average issues of Factor VIII by severity

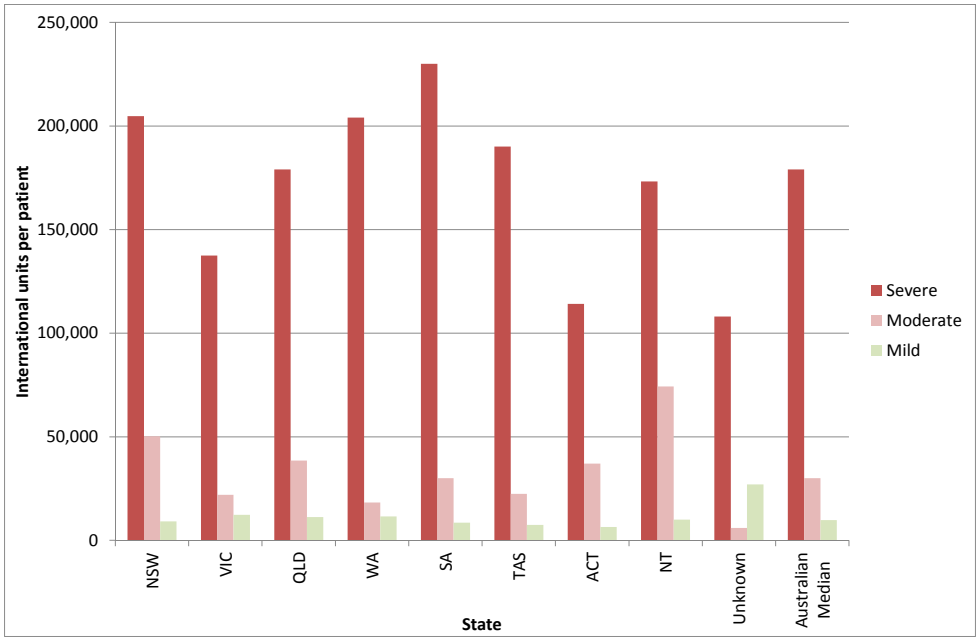
There are many other factors affecting the use of product including body weight and severity. Figure 14 shows the average issue of factor VIII by severity by jurisdiction. The more severe the condition is, the greater the amount of product given. Moreover, there is considerable difference between the jurisdictions.



†

Only patients who received Factor VIII in 2010-11 are included.

Figure 14 Mean units per Haemophilia A patient† of Factor VIII recorded in the ABDR by home jurisdiction of patient and severity in 2010-11



Note: *Unknown: Where location, as at the time of reporting was indeterminate*

† Only patients who received Factor VIII in 2010-11 are included.

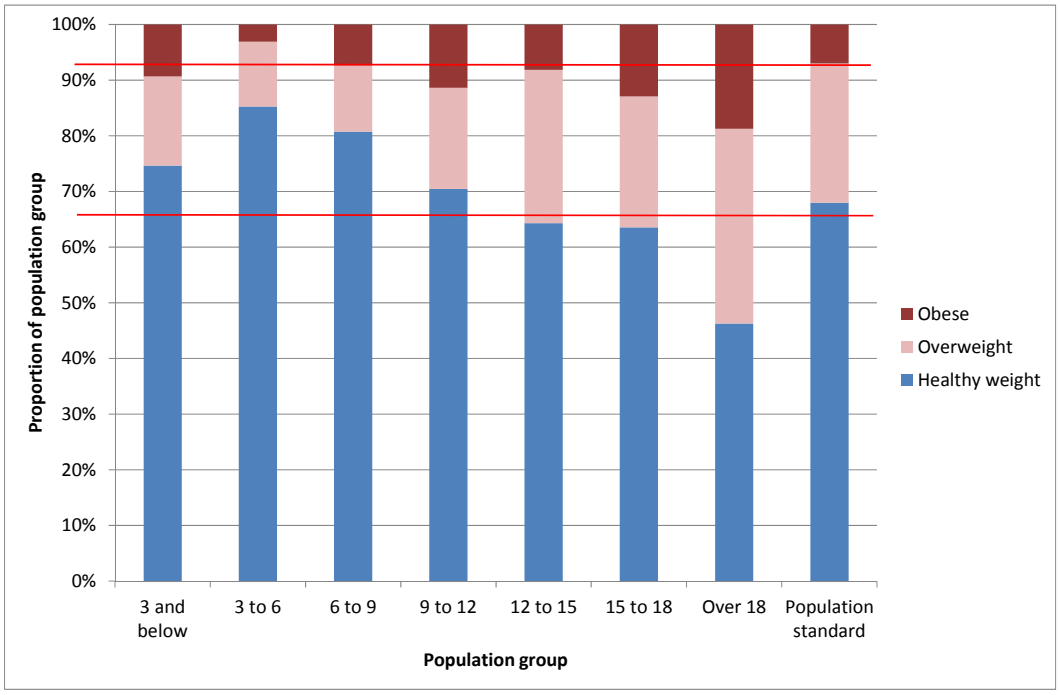
Figure 15 Median Factor VIII per Haemophilia A patient† by jurisdiction and severity in 2010-11

The mean is larger than the median because it is distorted by small numbers of people receiving very large amounts of clotting factor such as those undergoing tolerisation. The median is considered a more robust measure of the 'average' dosage.

Table 15 Issues of Factor VIII to Haemophilia A patients UK and Australia in 2010-11

	United Kingdom			Australia		
	Number treated	Total units of FVIII	Units per patient	Number treated	Total units of FVIII	Units per patient
Severe HmA	1,657	390,678,763	235,775	485	113,593,000	234,212
Non severe HmA	1,068	58,570,945	54,842	347	20,454,000	58,945
Tot HmA	2725	449,249,708	164,862	832	134,047,000	161,114

5.3. By weight and height



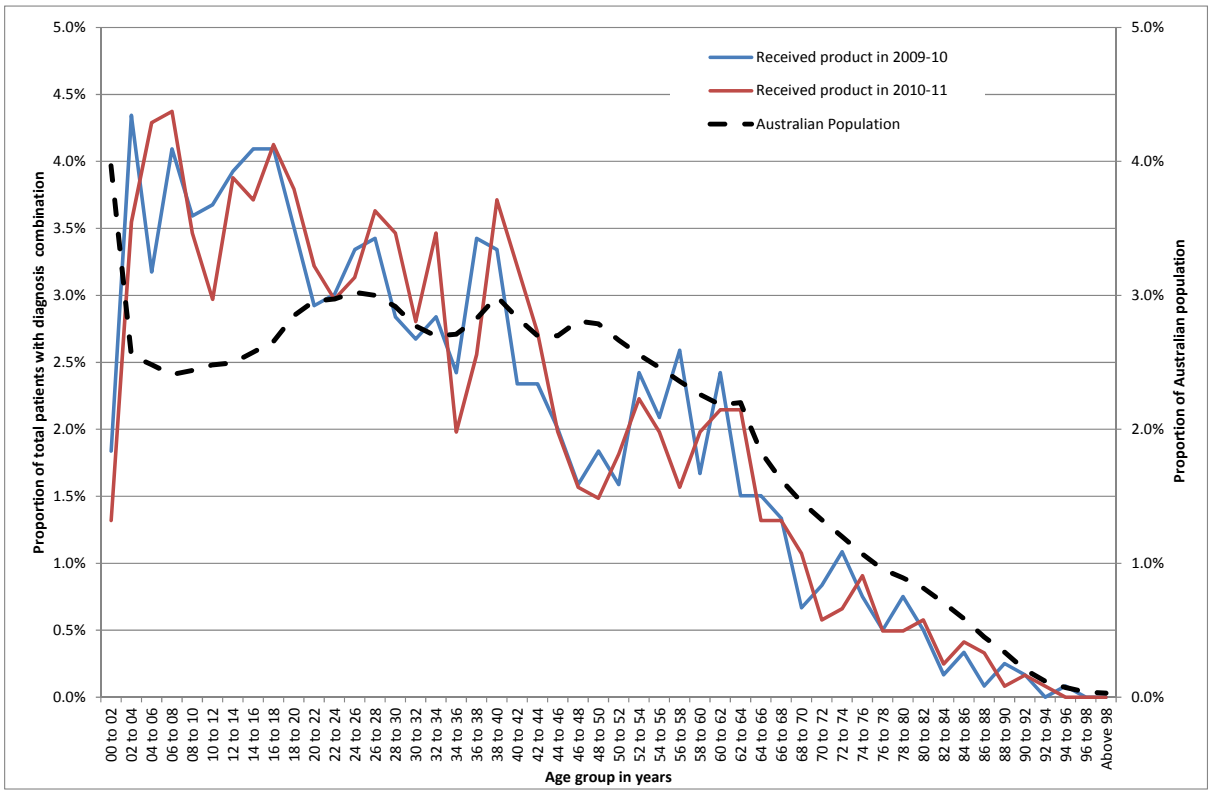
Source: ABDR and Victorian Department of Health

Figure 16 Weight and height data for ABDR patients receiving treatment in 2010-11

Figure 16 was constructed using age, weight and height data collected in ABDR. These data were combined to calculate the body mass index (BMI). The calculated BMIs were compared with an age appropriate BMI scale produced by Victorian Department of Health to determine whether the BMI corresponded to that patient being overweight or obese. Generally 25 per cent of the population are considered overweight and 7 per cent obese. These data may not be fully representative for adult patients as the height and weight data is less complete for them. As dosage is often related to weight, high proportions of overweight and obese patients may lead to an increase in the rate of growth of demand for products.

5.4. By age distribution

The following section shows the age distribution of patients who received product in at least one of the last two financial years.



Source: ABDR and ABS Population statistics

Figure 17 Age distribution of patients who received treatment in 2009-10 or 2010-11 compared with the Australian population

Figure 17 shows that those patients in the ABDR who are receiving treatment are generally younger than the Australian population as a whole. Also of note is the dip in the distribution at patients aged around 40 to 50. These distributions are similar to that for patients with severe conditions in Figure 7 and ***Figure 8***.

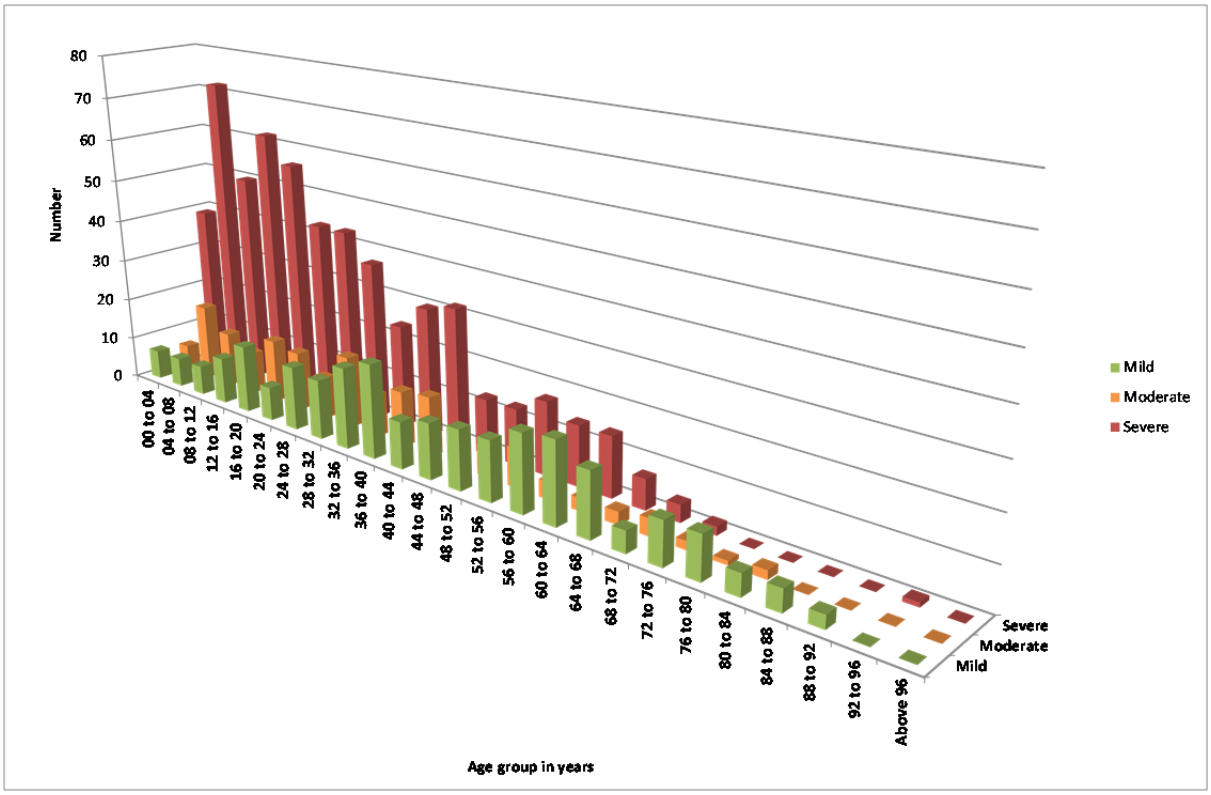
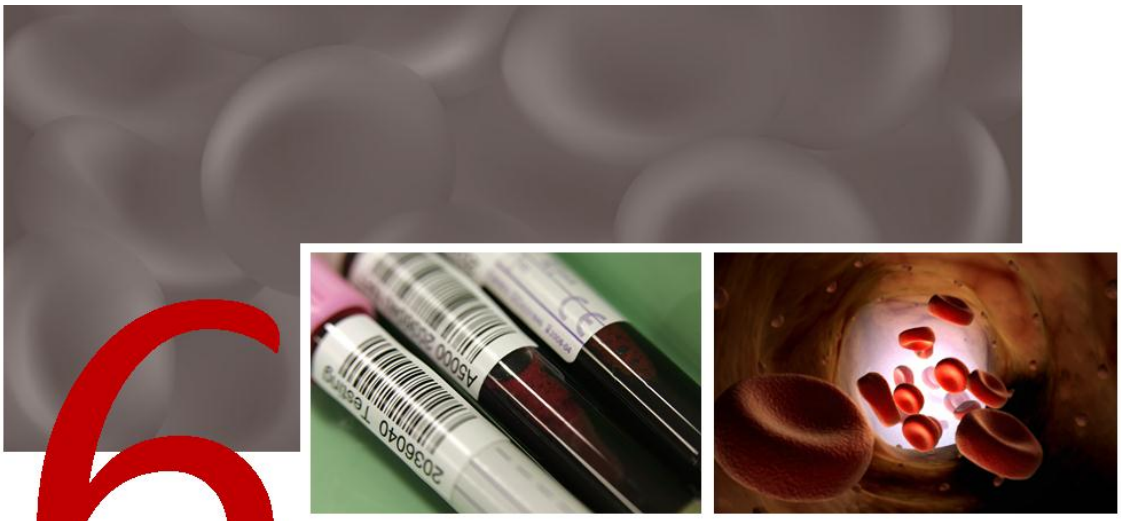


Figure 18 Age distribution at 30 June 2011 of Haemophilia A and Haemophilia B patients who received product in 2010-11

Figure 18 shows a large proportion of the severe Haemophilia A and Haemophilia B population are in the younger age groups. If the higher numbers in the younger age groups flow into older age groups, overall growth in demand for clotting factors may be significantly impacted.

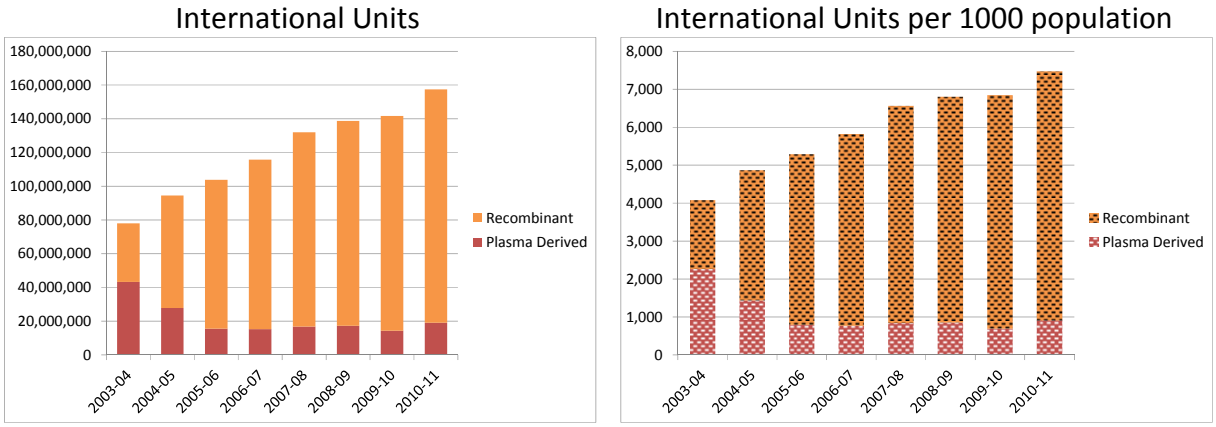


6.

Volume & cost of products used in the treatment of bleeding disorders

Figure 19 shows both the strong growth of clotting factors used in the treatment of people with haemophilia and the even stronger growth in the use of recombinant clotting factors.

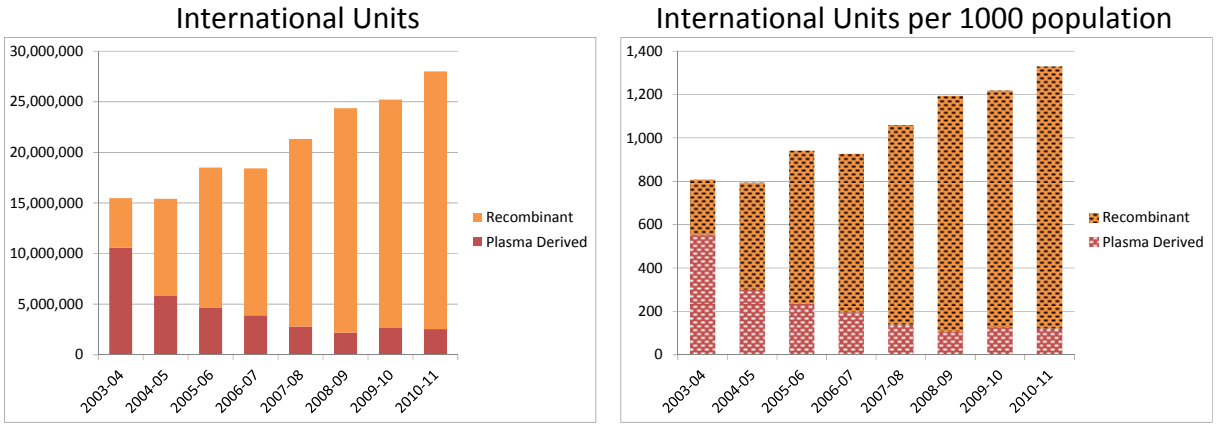
6.1. National issues by product



Source: IDMS database of issues

Figure 19 Annual use of Factor VIII issued nationally 2003-04 to 2010-11

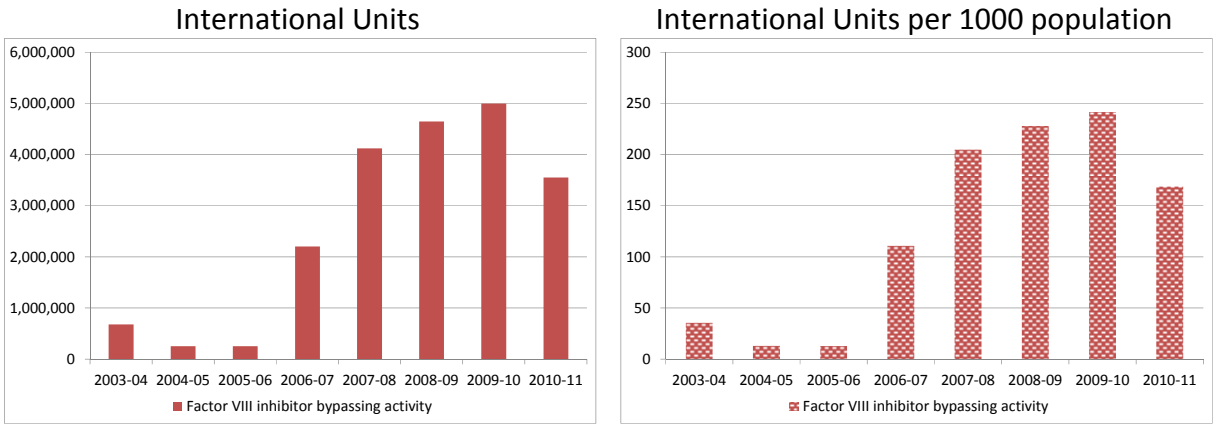
This shows that demand for Factor VIII has increased by 102 per cent over the years 2003-04 to 2010-11 (an average rate of 10.5 per cent per annum) and that the growth rate from 2008-09 to 2009-10 was below trend at 2.1 per cent. This figure also illustrates the rapid uptake of recombinant product. Growth in 2010-11 for total Factor VIII issues was 11.2 per cent. It will be important to monitor the growth rate against the change in age distribution of severe patients depicted at ***Figure 8***. As the use per 1000 population is growing, the growth in Factor VIII use is greater than population growth.



Source: IDMS database of issues.

Figure 20 Annual use units of Factor IX issued nationally 2003-04 to 2010-11

Similar to Figure 19, Figure 20 shows the strong growth of factor IX use since 2003 (81 per cent or 8.9 per cent per annum) and the substitution of recombinant for plasma derived product.



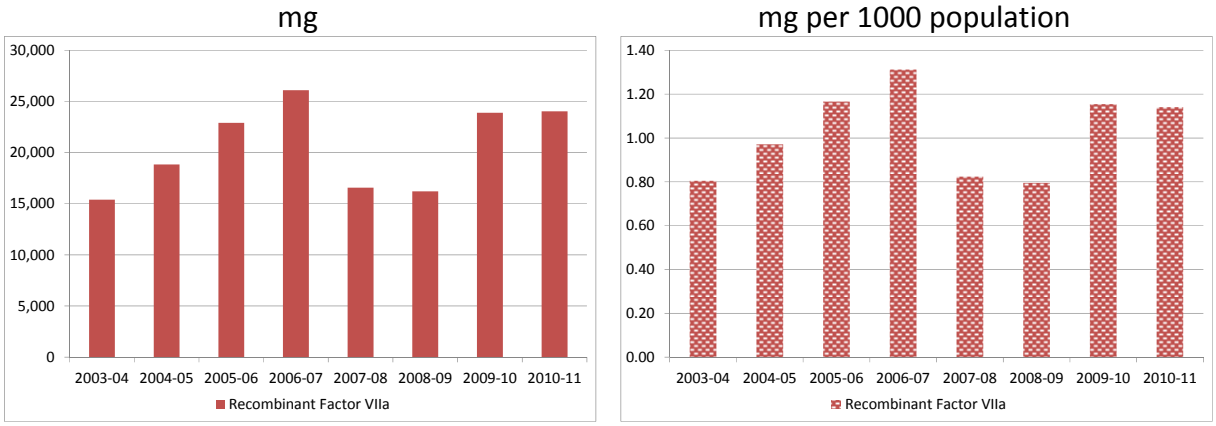
Source: IDMS database of issues.

Figure 21 Annual use of Factor VIII inhibitor Bypassing Activity (FEIBA) issued nationally 2003-04 to 2010-11

From 2005-06 to 2009-10 demand for FEIBA increased significantly; in 2005-06 approximately 251,500 IUs were issued compared with almost 4.8 million IUs in 2009-10 (Figure 21). Part of this growth relates to FEIBA becoming more widely available and government funded in 2006-07. There was a rapid fall in the use in 2010-11 with demand dropping nearly 30 per cent. Early indications for 2011-12 are that the demand for FEIBA will be lower.

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.

FEIBA and recombinant factor VIIa (rVIIa) (brand name NovoSeven) are both used to treat patients that have developed inhibitors. In the setting of managing inhibitors for haemophilia, the drivers for clinical demand for FEIBA are similar to those for NovoSeven. The work of AHCDO in standardising protocols in haemophilia management will likely assist with driving a consistency of approach to the use of FEIBA and similar/related agents.



Source: IDMS database of issues.

Figure 22 Annual use of Recombinant Factor VIIa (NovoSeven) 2003-04 to 2010-11

Demand for NovoSeven to treat patients with haemophilia under the National Blood Arrangements increased sharply from 2003-04 (15,400mg) to a high point in 2006-07 (25,900mg) but then declined to a more stable level in 2007-08 and 2008-09. However, high growth (47%) has been seen again from 2008-09 to 2009-10 (Figure 22). There was little change in demand between 2009-10 and 2010-11. Demand for NovoSeven is very difficult to predict due to small patient numbers and patient specific requirements. At times very large doses can be needed by a single patient.

6.2. Reported use of Factor IX by HmB patients selected countries

The World Federation of Hemophilia collects use data from various countries. This is used to compare Australia's use.

Table 16 The reported factor IX (FIX) use (IUs per capita) was determined from the reported number of FIX international units (IUs) used in the treatment of haemophilia B for a country from 1996 to 2006 divided by its total population in the relevant year selected countries

Country	2003	2004	2005	2006	**Mean**	SD	CV
Ireland	2.7986	2.6055	2.2529	2.0303	**2.1593**	0.6412	30%
New Zealand	0.5249	0.9387	1.7860	1.9566	**1.2478**	0.5584	45%
Sweden	1.1888	NA	1.3940	NA	**1.1722**	0.1175	10%
Canada	NA	0.9701	1.1289	1.1665	**1.0885**	0.1042	10%
United States	0.8985	1.0645	1.0872	1.2446	**0.8551**	0.2061	24%
Australia	1.3354	NA	0.7589	0.8354	**0.7731**	0.2790	36%
Denmark	0.8909	0.6478	1.0153	NA	**0.7364**	0.1981	27%
Germany	0.6056	NA	0.6050	0.9680	**0.6813**	0.1316	19%
Netherlands	NA	NA	0.6124	NA	**0.6480**	0.2653	41%
United Kingdom	0.5362	NA	1.0119	0.7606	**0.6391**	0.1996	31%

Source: J S Stonebraker, P H B Bolton-Maggs, M Brooker, A Farrugia and A Srivastava, *A study of reported factor IX use around the world*; WFH Fact and Figures August 2011 No 11.
The mean, standard deviation and coefficient of variation are calculated over the available years in the range 1996 to 2006. This table only shows the years 2003 to 2006.

Only the ten countries with the largest reported mean use are included in Table 16. These countries are all developed countries. Australia is ranked six in this list. As shown in Table 16, the use in Australia for 2010-10 was 1.3291 IU per patient. The three countries with the lowest reported use (Pakistan, Indonesia and Bangladesh), have mean use of no greater than 0.0005 IU per patient.

6.3. National Costs for products issued

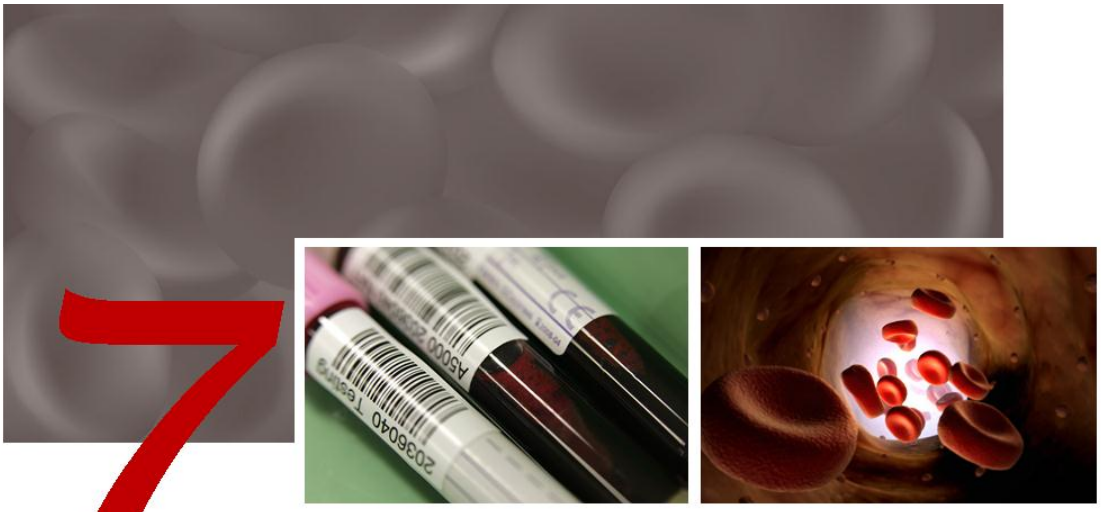
Total expenditure on clotting factors for 2010/11 was $210.8 million. This was an increase of $22.3 million on 2009/10.

This represents approximately 23 per cent of the total blood and blood product budget for Australia in 2010/11. Expenditure by product is detailed in the NBA Annual Report, available at www.nba.gov.au and is summarised below.

Table 17 National clotting factor costs 2008-09, 2009-10 and 2010-11 in $ millions

	2008-09	2009-10	2010-11
Factor VIII	104.3	109.3	128.1
Factor IX	26.1	26.7	31.6
Recombinant Factor VIIa	17.4	26.4	27.4
FEIBA	13.5	14.5	10.3
Other	12.3	11.5	13.5
Total	173.6	188.5	210.8

Source: NBA systems



7.

Appendices

Appendix A Haemophilia Treatment Centres

1. The objectives of HTCs

Haemophilia Centres provide comprehensive care for people with haemophilia. Their roles include:

- Compilation and distribution of guidelines for best practice directed toward optimal care of patients with disorders of haemostasis
- Providing protocols for the accurate diagnosis of patients with bleeding disorders
- Providing protocols for the regular review of infectious disease markers in patients and their families
- The allocation and distribution of therapeutic blood and recombinant products together with advice regarding the usage of blood and recombinant products, at a State and National level.
- The establishment of review programs to assess outcomes of therapies.
- Provision of regularly updated data to the national Haemophilia Registry.
- Participation in basic and clinical research

2. Operating concept

Haemophilia 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. One collaborative centre for each State and Territory may suffice but this must include adult and paediatric type centres.

The role of the Haemophilia Centres is to provide:

- a single point accountability for the care of patients with bleeding disorders with responsibility for the coordination, allocation and distribution of therapeutic resources for the treatment of patients, ie coagulation products derived either from blood donors or recombinant technologies
- 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.
- a system of record for all investigations, treatments, allocation of therapeutic products and adverse reactions.
- a capability to participate in research including clinical trials
- educational programs 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
    - Designated supervising medical practitioner

All isolated patients where care is managed in an outreach program should be registered at and be reviewed regularly by a Haemophilia Treatment Centre which would arrange delivery of and monitor the supply of therapeutic coagulation products.

The Centre must maintain on-going dialogue with the 'client' group in each State and Territory. The role of State and Territory Governments is to designate 'Haemophilia Centres' and negotiate the funding of the Centres including the purchase of therapeutic blood and recombinant products for distribution within States (or regions) and Territories. In some States committees have been established to consider and schedule elective surgery.

Advantages of the Proposed Model of Haemophilia Data Co-ordination

- Accurate and complete data entry
- Dedicated and focused data management
- Regular reporting and analysis of collated information
- New product initiation of unresolved haemophilia care related questions
- Clinical audit of current policies and monitoring of agreed national standards

Table 18 List of operational HTCs and their ID codes

#	Hospital	Haemophilia Treatment Centre	ID	State
1	The Canberra Hospital	Haemophilia Clinic	1TCH	ACT
2	Calvary Mater Newcastle	Haemophilia Treatment Centre	2CMN	NSW
3	Royal Prince Alfred Hospital	Haemophilia Treatment Centre	3RPAH	NSW
4	Sydney Children's Hospital	Centre for Children's Cancer and Blood Disorders	4SCH	NSW
5	The Children's Hospital at Westmead	Haemophilia Treatment Centre	5CHW	NSW
6	Royal Darwin Hospital	Haemophilia Treatment Centre	6RDH	NT

#	Hospital	Haemophilia Treatment Centre	ID	State
7	Royal Brisbane and Women's Hospital	Queensland Haemophilia Centre	7RBWH	QLD
8	Royal Children's Hospital	Queensland Haemophilia Centre Child and Adolescent Service	8RCH	QLD
9	Royal Adelaide Hospital	South Australia Haemophilia Treatment Centre	9RAH	SA
10	Women's and Children's Hospital	South Australia Haemophilia Treatment Centre	10WCH	SA
11	Royal Hobart Hospital	Tasmanian Haemophilia Treatment Centre	11RHH	TAS
12	The Alfred Hospital	Ronald Sawyers Haemophilia Centre	12TAH	VIC
13	Royal Children's Hospital	Henry Ekert Haemophilia Treatment Centre	13RCH	VIC
20	The Haemophilia Centre of WA	Incorporating: • Fremantle Hospital • Princess Margaret Hospital • Royal Perth Hospital	20HCWA	WA
99	*NBA*	*Offshore Patient - Long Term*	*99OPL*	*ACT*
98	*Inactive Patients*	*Inactive Patients*	*98DPG*	*ACT*

3. Data Quality of HTC data collections

The following organisations are represented at various HTCs nationally:

- Australian Haemophilia Nurses Group (AHNG)
- Australia/New Zealand Haemophilia Social Workers' and Counsellors' Group (ANZHSWCG)
- Australia/New Zealand Physiotherapist Group (ANZHPG)
- Haemophilia Foundation of Australia (HFA)

These member representatives have provided input into the initial design of the ABDR and continue to provide input from their respective areas of specialty.

The Data Managers at each HTC have elected Data Manager Co-Chairs. These Co-Chairs coordinate teleconferences, between all Data Managers, on a regular basis. The Data Manager Co-Chairs also have the functionality of raising issues, to the NBA, on behalf of their group. ACHDO has a major role in providing support to ABDR Data Managers through the funded model for Data Managers.

Data Quality initiatives implemented in 2010-11 include:

- Regular teleconferences for ABDR Data Manager Group (DMG)
- 'Advanced Search' functionality of the ABDR whereby Data Managers are able to extract information from the ABDR on an *ad hoc* basis. 'Work around' strategies have been developed to deal with data validity issues.
- Review of data definitions undertaken by DMG Co Chairs
- NBA financial support, through AHCDO funding, for HTC Data Managers
- *The ABDR Update* is a functional tool in the form of a Newsletter. This provides an update on issues such as changes to the ABDR and functionality enhancements. This update is a means of keeping all ABDR stakeholders informed.

Data Quality initiatives in 2010-11 have addressed:

- Currency of data
- Unpopulated data fields, particularly citizenship, nationality and ethnicity data fields
- National consistency of data quality
- On-going issues of information exchange when persons with haemophilia (PWH) move between states and haemophilia treatment centres
- Historical data (immediately prior to the deployment of the redeveloped ABDR in December 2008
- Timely entry of product order timeframes

Appendix B History and development of the ABDR

The ABDR was first established in 1988 using a 'Paradox' database at each Haemophilia Treatment Centre in Australia. The aims of the ABDR were to provide a clinical tool for improved management and national demographics of patients with haemophilia and other inherited bleeding disorders.

In 2000, a revised ABDR was established using 'Access' database platform at each Haemophilia Treatment Centre with a national collection of de-identified data every six months. Dedicated data base managers in individual centres improved data collection. On-going concerns regarding privacy prevented collection of national demographics such as age and gender.

To provide better sharing and access to the database it was decided in 2006 to move to an internet interface to central database. Genix Ventures was the successful tender with all Australian governments providing funding and the National Blood Authority providing the project management.

The redeveloped ABDR was deployed in December 2008 at all HTCs.

4. Benefits of the redeveloped ABDR

The NBA redeveloped the ABDR and deployed the redeveloped ABDR in December 2008. It provides the following benefits:

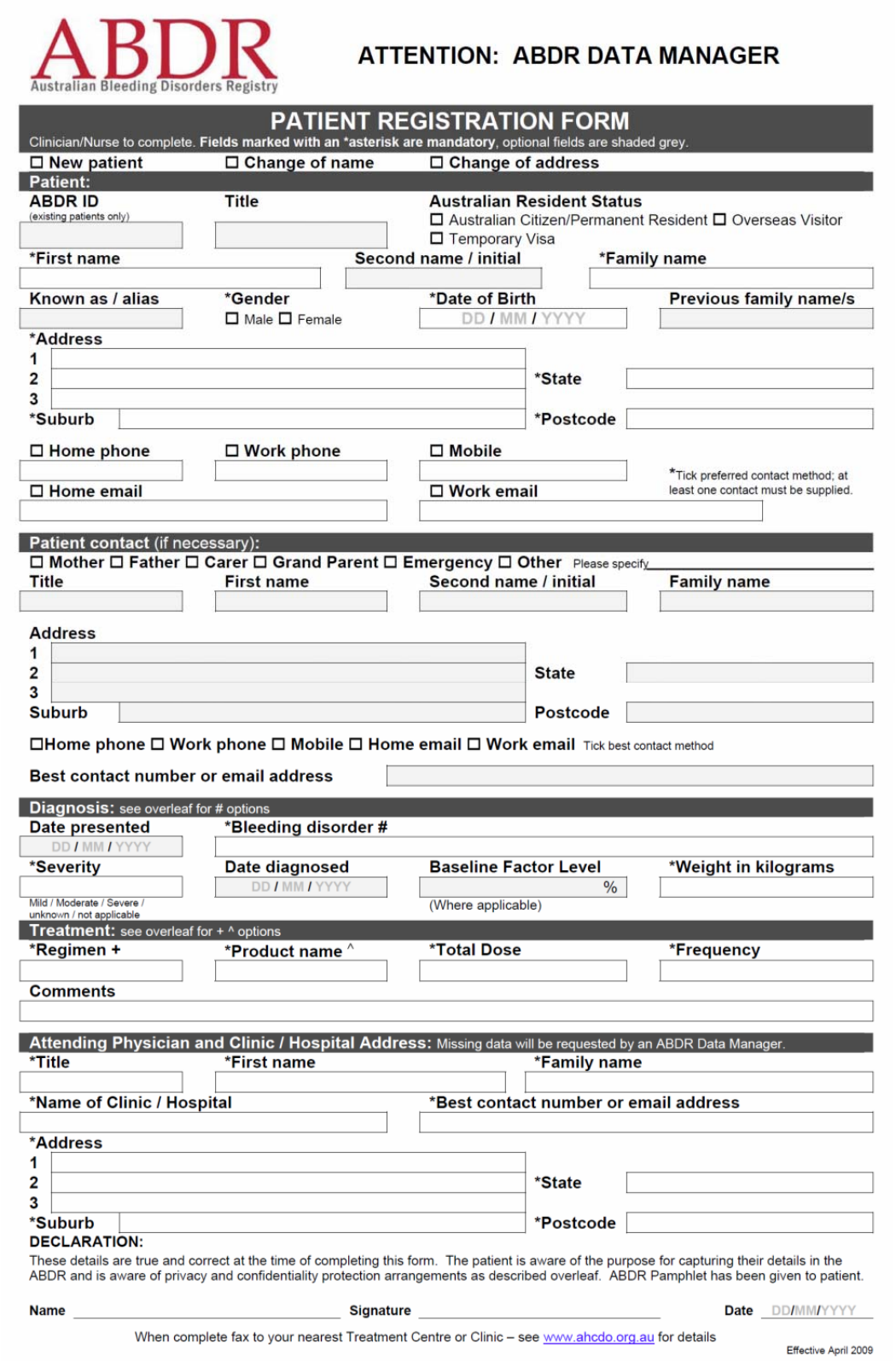
- Single point of access for clinicians for treatment of patients
- Patient information relating to all clinical information associated with the treatment of haemophilia
- Information exchange between states and Haemophilia Treatment Centres
- National demographic information (age, gender etc) of persons with bleeding disorders
- National data on inhibitor incidence and outcomes of treatment
- Allied health (physiotherapy and social work) monitoring and outcomes
- Recording of personal usage of factor replacement for clinical monitoring
- Data for forward planning and funding of factor concentrates on a national basis
- High usage patterns

5. Current position of the development of the ABDR

Today the Australian Bleeding Disorders Registry is fully operational. The ABDR Steering committee continues to oversee the project.

The National Blood Authority's role continues around provision of resources to maintain ABDR operations and to ensure timely and accurate reporting from the ABDR through provision of support to Data Managers. Data Managers, funded and supported by AHCDO, are located at HTCs across Australia.

Appendix C ABDR data collection (registration form)



ABDR
Australian Bleeding Disorders Registry

ATTENTION: ABDR DATA MANAGER

PATIENT REGISTRATION FORM

Clinician/Nurse to complete. **Fields marked with an *asterisk are mandatory**, optional fields are shaded grey.

☐ New patient ☐ Change of name ☐ Change of address

Patient:

ABDR ID (existing patients only)

Title

Australian Resident Status
☐ Australian Citizen/Permanent Resident ☐ Overseas Visitor
☐ Temporary Visa

***First name** **Second name / initial** ***Family name**

Known as / alias ***Gender** ☐ Male ☐ Female ***Date of Birth** DD / MM / YYYY **Previous family name/s**

***Address**
1
2
3
***Suburb** ***State** ***Postcode**

☐ Home phone ☐ Work phone ☐ Mobile

*Tick preferred contact method; at least one contact must be supplied.

☐ Home email ☐ Work email

Patient contact (if necessary):

☐ Mother ☐ Father ☐ Carer ☐ Grand Parent ☐ Emergency ☐ Other Please specify

Title **First name** **Second name / initial** **Family name**

Address
1
2
3
Suburb **State** **Postcode**

☐Home phone ☐ Work phone ☐ Mobile ☐ Home email ☐ Work email Tick best contact method

Best contact number or email address

Diagnosis: see overleaf for # options

Date presented DD / MM / YYYY ***Bleeding disorder #**

***Severity** Mild / Moderate / Severe / unknown / not applicable **Date diagnosed** DD / MM / YYYY **Baseline Factor Level** % (Where applicable) ***Weight in kilograms**

Treatment: see overleaf for + ^ options

***Regimen +** ***Product name ^** ***Total Dose** ***Frequency**

Comments

Attending Physician and Clinic / Hospital Address: Missing data will be requested by an ABDR Data Manager.

***Title** ***First name** ***Family name**

***Name of Clinic / Hospital** ***Best contact number or email address**

***Address**
1
2
3
***Suburb** ***State** ***Postcode**

DECLARATION:

These details are true and correct at the time of completing this form. The patient is aware of the purpose for capturing their details in the ABDR and is aware of privacy and confidentiality protection arrangements as described overleaf. ABDR Pamphlet has been given to patient.

Name _____ **Signature** _____ **Date** DD/MM/YYYY

When complete fax to your nearest Treatment Centre or Clinic – see www.ahcdo.org.au for details

Effective April 2009

ABDR
Australian Bleeding Disorders Registry

ATTENTION: ABDR DATA MANAGER

#bleeding disorder

Factor II deficiency (Prothrombin)
Factor V deficiency
Factor VII deficiency
Factor VIII deficiency (Haemophilia A)
Factor IX deficiency (Haemophilia B)
Factor X deficiency
Factor XI deficiency
Factor XII deficiency
Factor XIII deficiency
Symptomatic Carrier Factor VIII deficiency (Haemophilia A)
Symptomatic Carrier Factor IX deficiency (Haemophilia B)
Asymptomatic Carrier Factor VIII deficiency (Haemophilia A)
Asymptomatic Carrier Factor IX deficiency (Haemophilia B)
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
Fibrinogen – Afibrinogenemia
Fibrinogen – Hypofibrinogenemia
Fibrinogen – Dysfibrinogenemia
Fibrinogen dysfunction – Uncharacterised
Platelet – Glanzmann's thrombasthenia
Platelet – Bernard-Soulier
Platelet – May Hegglin
Platelet – Macrothrombocytopenias
Platelet – Storage pool (dense granule) deficiency
Platelet – Primary secretion defect
Platelet – Uncharacterised
Acquired factor VIII inhibitor (Acquired Haemophilia A)
Acquired von Willebrand's Disease
Vascular disorders – Ehlers Danlos Syndrome
Vascular disorders – Uncharacterised
Other, please specify

+treatment regimen

On demand
Prophylaxis
Tolerisation
Secondary Prophylaxis

^Product Name (type)

Advate® (rFVIII)
Fresh Frozen Plasma (FFP)
BeneFIX® (rFIX)
Biostate® (pdFVIII)
Ceprotin® (Protein C)
Cryoprecipitate
DDAVP (Synthetic hormone)
Factor Eight Inhibitor Bypass Agent (FEIBA®) (Bypassing Agent)
Factor VII Concentrate® (pdFVII)
Factor XI bpl® (pdFXI)
Factor XI LFB Hemoleven® (pdFXI)
Fibrogammin P® (pdFXIII)
Fresh Frozen Plasma (FFP)
Haemocomplettan P 1g (pdFXIII)
Intravenous Immunoglobulin (IVIg)
MonoFIX® - VF (pdFIX)
NovoSeven® (rFVIIa)
NovoSeven RT® (rFVIIa)
Platelets
Prothrombinex™ - VF (pdPCC)
Recombinate® (rFVIII)
ReFacto® (rFVIII)

ABDR Patient Pamphlet

What is the ABDR? The Australian Bleeding Disorders Registry (**ABDR**) is a database that collects all clinical information related to the treatment of people with bleeding disorders, like an electronic medical file. This includes information about patient diagnosis, 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 by staff at haemophilia treatment centres. The ABDR is managed by a service provider engaged by the National Blood Authority. The ABDR was first established in 1988 and has been upgraded many times with the latest significant upgrade in 2008.

Why do you need it? The ABDR provides your health care team and support staff with a record enabling them to monitor and manage your treatment over time to improve your quality of life. Depersonalised information available from the ABDR may be used 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 make sure there is enough available to meet the needs of all Australians with bleeding disorders.

What about privacy? Only the health care team and support staff involved in providing medical services to you have access to your personal information. Other authorised users only have access to limited, depersonalised and/or summary information where all identifying information is removed to protect your privacy.

Does information about me have to be included? A minimum amount of information about you is required to ensure the continuous supply of clotting factor product is available to meet your treatment needs.

Where can I get more information? Further information about the ABDR can be obtained from the Australian Haemophilia Centre Directors' Organisation (AHCDO) on (03) 9885 1777, email info@ahcdo.org.au or visit www.ahcdo.org.au

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 an overview 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 practise 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

Copies of this pamphlet can be obtained by contacting the ABDR Secretariat at abdr@nba.gov.au or 02 6211 8311.

When complete fax to your nearest Treatment Centre or Clinic – see www.ahcdo.org.au for details

Effective April 2009

7.1. List of Figures

7.2. List of tables