NATIONAL BLOOD AUTHORITY
AUSTRALIA

AUSTRALIAN BLEEDING DISORDERS REGISTRY

Annual Report 2011-12

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NATIONAL BLOOD AUTHORITY
AUSTRALIA

Locked Bag 8430
Canberra ACT 2601
Phone: 13 000 BLOOD (13000 25663)
Email: data@blood.gov.au
www.blood.gov.au

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Purpose of this document

The intention of this document is to present the reader with an integrated view of current clinical and demographic information on people with inherited 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.

This document will be used by people involved in providing care for patients with bleeding disorders, and may also be useful for patient advocacy groups and those in administrative and government positions.

Key findings

The data contained in this reports shows:

- There were 5,588 patients in the Australian Bleeding Disorders Registry (ABDR) in 2011-12
    - 2,316 patients with Haemophilia A (724 patients with severe Haemophilia A)
    - 544 patients with Haemophilia B (102 patients with severe Haemophilia B)
    - 2,068 patients with von Willebrand Disease
    - There are also a number of other bleeding disorders with smaller numbers of patients
- A total of 133,315,740 IU of recombinant Factor VIII products were used by Haemophilia A patients in 2011-12
    - Prophylactic use by severe Haemophilia A patients accounted for 81,029,500 IU, which was 60.8% of the volume issued.
- A total of 21,370,750 IU of recombinant Factor IX products were used by Haemophilia B patients in 2011-12
    - Prophylactic use by severe Haemophilia B patients accounted for 6,797,500 IU, which was 31.8% of the volume issued.
- New national supply arrangements altered the availability of recombinant Factor VIII products
    - At the start of 2011-12 Xyntha, Recombinate and Advate were available
    - By the end of 2011-12 Xyntha and Kogenate FS were the available products
- There was a slight decrease in demand for recombinant clotting factor products in 2011-12.
    - The reduction in growth for Factor VIII may be attributed to a number of factors, including a reduction in the number of patients undergoing tolerisation, a number of high volume patients participating in clinical trials and the continuing number of patients stabilising onto prophylaxis home treatment.
- A total of $192.6 million was budgeted by governments on clotting factor products in 2011-12.

Background

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 the Haemophilia Foundation of Australia (www.haemophilia.org.au).

WHAT ARE BLEEDING DISORDERS?

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 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. However, 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. Men and women are equally likely to be affected by an acquired bleeding disorder, and the potential for problems is high.

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

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.

TYPES OF HAEMOPHILIA

- The most common type of haemophilia is called 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 FAST FACTS

- Haemophilia occurs in 1 in 6,000-10,000 males internationally.
- Currently in Australia there are 2,860 people with haemophilia A and B, with varied degrees of severity, in the Australian Bleeding Disorders Registry (ABDR).
- 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 (VWD) is the most common type of bleeding disorder. People with VWD have a problem with a protein in their blood called von Willebrand factor (VWF) that helps 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. Currently there are 2068 people with VWD in the ABDR which will not reflect the numbers with symptomatic VWD.

TYPES OF VWD

There are three main types of VWD. Within each type, the disorder can be mild, moderate, or severe. 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. 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.

Problems with factor VIII and factor IX are known as haemophilia A and B, respectively. 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. Less is known about these disorders because they are diagnosed so rarely.

The World Federation of Hemophilia produced a summary Table 15 (Appendix A, p36) of the characteristics of rare clotting factor deficiencies, the severity of bleeds associated with them, and the treatment typically required.

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 anemia (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 fetus). 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.

INHERITED PLATELET 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.

WHAT ARE PLATELET FUNCTION DISORDERS?

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.

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 SEVERITIES 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.

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; DDAVP) which can stimulate the release of Factor VIII and VWF from stores in the body (this is not used in haemophilia B or Factor IX deficiency). In some patients, therapy is complicated when their body develops inhibitors that destroy the replacement clotting factors and other treatment is necessary.

[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.

Treatment of bleeding disorders 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 bleeding disorders. 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 with specialised expertise within the one centre.

HTCs were established following a decision by Australian Health Ministers Advisory Council (AHMAC) in 1998, 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. The roles of these Centres are defined in Appendix A. The locations of the HTCs in Australia are shown in Figure 1.

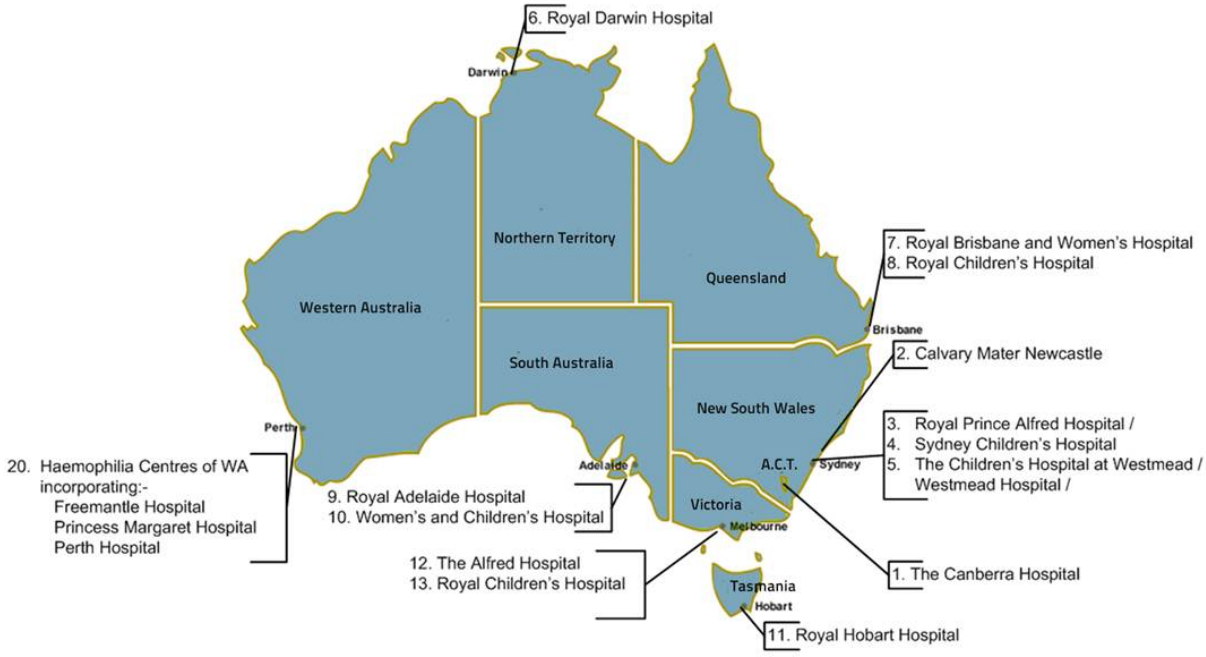

FIGURE 1 LOCATION OF HAEMOPHILIA TREATMENT CENTRES

The model for HTCs varies between jurisdictions in relation centralisation of services; size and age of patient population.

There are also some patients whose treatment is managed by clinicians who are 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 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. A description of the aims and governance of HTCs is provided at Appendix B (page 37).

The Australian Bleeding Disorders Registry (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 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 current version of the ABDR has been in existence since December 2008 and background on the development of the system is at Appendix D History of the ABDR. In August 2012 the 4th generation ABDR was implemented.

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. Considerations for the release of any information for research are made under specific governance arrangements. 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.

ABDR MANAGEMENT AND GOVERNANCE

The ABDR is managed on a day to day basis by the National Blood Authority (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 2011-12 the Steering Committee representatives were:

- Dr John Rowell (Chair) – Australian Haemophilia Centre Directors' Organisation
- Dr Chris Barnes – Chair of Australian Haemophilia Centre Directors' Organisation
- Ms Sharon Caris – Executive Director, The Haemophilia Foundation Australia
- Ms Kim Stewart, NSW Health – Jurisdictional Blood Committee nominee
- Ms Stephanie Gunn / Mr Michael Stone – National Blood Authority

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

DATA GOVERNANCE

There is an extremely robust Governance framework that oversees the management and operation of the ABDR. An AHCDO member chairs the Steering Committee tasked with these responsibilities. The Steering Committee also includes the Executive Director of Haemophilia Foundation Australia to ensure patient needs are met. Patient privacy and confidentiality are paramount to these arrangements.

In addition, there are stringent security protocols embedded into the technical architecture of the ABDR. These effectively control access to personal data ensuring this information is only accessible to treating health professionals and authorised support staff.

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.

DATA QUALITY ISSUES

There are a number of data quality issues in the ABDR. These include incomplete records with empty fields or entries. The data entered into some fields has also been characterised by a lack of consistency. This issue in the interpretation of specific fields will be addressed with the development of a data dictionary for users. The ABDR Steering Committee has initiated strategies to improve the data quality and over time the reporting from the ABDR has become more robust. However, there are still some data quality issues that impact the data presented in this report.

NEW ABDR SYSTEM

The 4th Generation ABDR was successfully implemented on 13 August 2012. Training for all Haemophilia Training Centres was provided in the week of the release. Feedback to date is that the next generation is already showing better performance and ease of use.

COMPARING DATA FROM PREVIOUS ABDR ANNUAL REPORTS

Comprehensive automated and manual data cleansing and validation processes (that occurred as part of the implementation of the new system) enhanced the ABDR data accuracy and consistency presented in this report. This will make it difficult to undertake comparisons with data published in previous reports particularly in regards to multiple diagnoses, treatment plans, ages and dates of death.

PATIENTS WITH MULTIPLE BLEEDING DISORDERS

Individual patients may have more than one bleeding disorder, and will be registered with more than one diagnosis. There are 101 patients with multiple diagnoses in the registry for 2011-12. In these cases, a patient may be counted more than once in the data in this report (e.g. if a patient has two bleeding disorders, that patient will be counted in the totals for each disorder).

CONSISTENT APPLICATION OF DIAGNOSES AND DEFINITIONS

The application of definitions for bleeding disorders (e.g. VWD subtypes) varies between HTCs, and work will continue to ensure consistent approaches are used, including alignment of the severity ratings and treatment regimens for some patient records.

VON WILLEBRAND DISEASE

Not all patients with VWD are treated through HTCs and the figures in this report do not represent the total number of VWD patients in Australia.

The diagnosis of VWD subtypes and the assignment of a severity rating to the disorder can vary between HTCs. Often the treatments for VWD involve providing replacement for the missing or defective clotting factors, and use of these products is included in this report.

TREATMENTS NOT INCLUDED IN THE ABDR

The treatments for bleeding disorders often involve providing replacement for the missing or defective clotting factors. The use of commercially produced clotting factors is the subject of this report.

However, there are other clinically appropriate treatments for bleeding disorders which are not counted in this report. Other products used include cryoprecipitate (a fresh blood product), platelets (a fresh blood product) and Desmopressin (1-desamino-8-D-arginine vasopressin, abbreviated as DDAVP).

Mild cases of HMA, HMB and VWD are often treated with DDAVP. Platelet disorders may be treated with DDAVP, platelet infusion or FVIIa.

Supply of products for treatment

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. Further details on national supply and demand trends can be found in Appendix C (page 40).

The range of products available to clinicians has changed over the years. Figure 2 shows the total issues and market shares for recombinant products from 2007-08 to 2011-12.

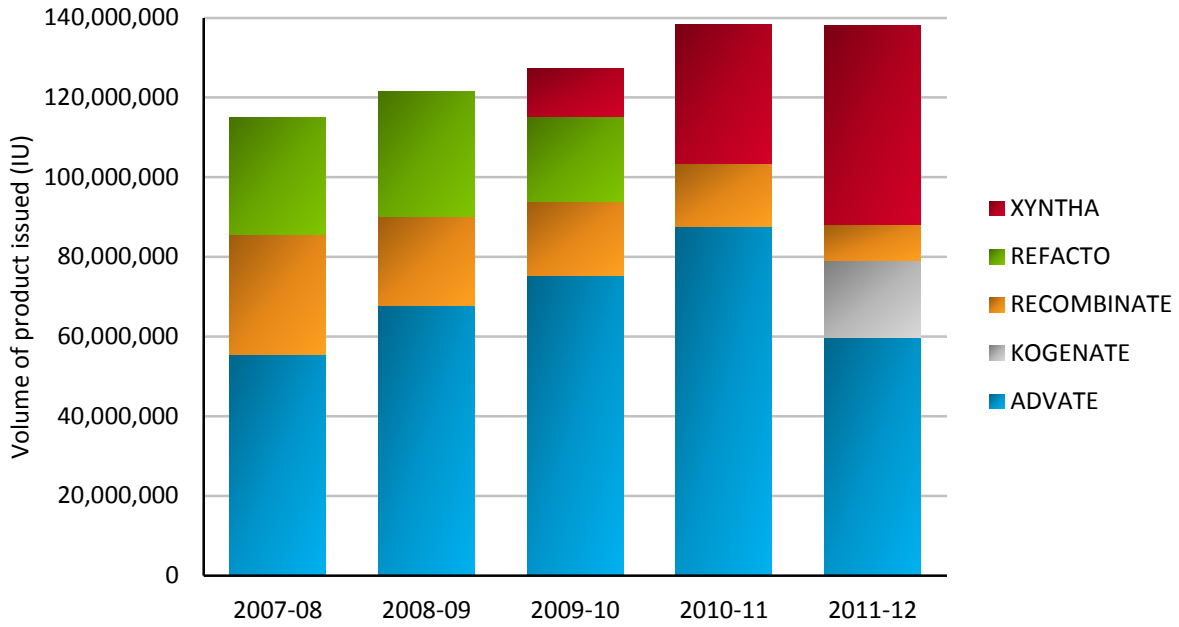

FIGURE 2 MARKET SHARE OF RECOMBINANT FVIII ISSUES, 2007-08 TO 2011-12

Figure 3 illustrates the changes that occurred during 2011-12, brought about by new national supply arrangements, with a transition away from Advate and Recombinate, an increase in the issue of Xyntha and the introduction of Kogenate. The new supply arrangements have provided high level national efficiencies without detriment to the patient population.

The most challenging aspect of HMA management is the development of FVIII inhibitors; previously untreated patients are at the highest risk for inhibitor formation. Currently, first-, second- and third-generation rFVIII products are commercially available. Whereas first-generation rFVIII concentrates (Kogenate and Recombinate) are stabilized with human albumin, second-generation rFVIII products (ReFacto) contain sucrose instead of albumin in the final formulation. Finally, third-generation rFVIII products (Advate and Xyntha) are manufactured without additional human or animal plasma proteins.

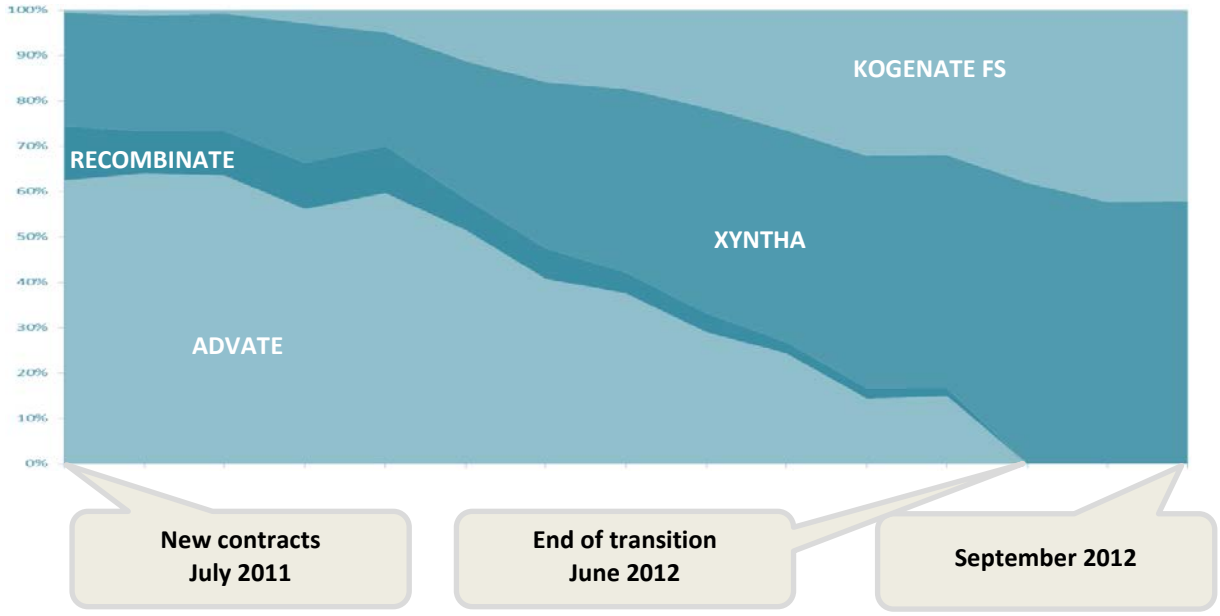

FIGURE 3 TRANSITION OF FVIII PRODUCTS ISSUED DURING 2011-12

A recent study on whether the type of factor VIII product administered and switching among products are associated with the development of clinically relevant inhibitory antibodies was conducted using 574 paediatric patients[2]. Recombinant and plasma-derived factor VIII products conferred similar risks of inhibitor development, and the content of von Willebrand factor in the products and switching among products were not associated with the risk of inhibitor development. Second-generation full-length recombinant products were associated with an increased risk, as compared with third-generation products. Further confirmation of these findings will be required.

A recent systematic review[3] performed using selective criteria concluded that the type of FVIII product (i.e. plasma-derived versus recombinant FVIII concentrates) does not influence the inhibitor rate in previously untreated patients with severe HMA.

These issues highlight the importance of clinical observation and registries to monitor uncommon events associated with treatment products to inform the haemophilia community, clinicians, and funding governments.

In the future, further research and reporting may be possible on the impact for patients in the ABDR of changing the products with which they are treated. Ongoing improvements to the quality and integrity of the data in the ABDR will enhance this research capacity. However, at present, detailed patient record audits and case studies provide greater insight into the possible impacts for Australian patients. The Australian Haemophilia Centres Directors' Organisation (AHCDO) and the NBA will continue to monitor the ABDR closely.

[2] Gouw, S.C, et.al. (2013) Factor VIII products and inhibitor development in severe hemophilia A. The New England journal of medicine 368 (3): 231-9.
[3] Franchini, M., et.al. (2012) Cumulative inhibitor incidence in previously untreated patients with severe hemophilia A treated with plasma-derived versus recombinant factor VIII concentrates: a critical systematic review. Critical reviews in oncology/hematology 81 (1): 82-93.

ABDR patient demographics

This section of the report presents the key patient demographic data collected by the ABDR.

DIAGNOSES

The following tables present the numbers of patients in the ABDR registry and the numbers of patients who received therapeutic products during the years 2008-09 to 20011-12. As noted in the section on *Data quality issues* (page 14) comprehensive automated and manual data cleansing and validation processes that occurred as part of the 4th Generation ABDR Redevelopment project released in August 2012 enhanced the ABDR data accuracy and consistency presented in this report. This may make it difficult to undertake comparisons with data published in previous reports.

Table 3 lists the number of people in the registry and the number treated by latest broad diagnosis for the years 2008-09 to 2011-12. An individual patient may have more than one diagnosis/disorder; in these cases they will be counted for each diagnosis. Table 3 shows slight growth in the number of patients in the four year period, and a pronounced increase in the number of patients receiving treatment with a clotting factor product for HMA, HMB and VWD. These increases are also reflected in the data on national Supply of products for treatment (page 16). These trends may also reflect absent data during the early years of the ABDR, with more stable data acquisition in later years. Table 4 and Table 5 expand the data in Table 3 to show the number of people in the registry and the number treated by detailed diagnosis for the years 2008-09 to 2011-12.

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

	Number in ABDR Registry*				Number who Received Product*			
	2008-09	2009-10	2010-11	2011-12	2008-09	2009-10	2010-11	2011-12
HMA†	2,019	2,116	2,217	2,316	667	833	880	895
HMB†	478	501	527	544	147	183	186	184
Other‡	145	156	165	214	-	-	<5	6
Other Factor Deficiency	249	277	306	326	22	20	22	33
Platelet Disorder	166	179	204	224	-	<5	9	<5
Vascular	6	8	9	9	-	-	-	-
VWD	1,675	1,815	1,940	2,068	93	170	151	153

* As noted in the section *Data quality issues* (p14) the data has been improved since previous ABDR Annual Reports. The figures presented here represent the most accurate data currently available. The census date for number of people in the registry is 30 June, the last day of the financial year.
† Includes some female carriers who are symptomatic.
‡The ABDR allows for a diagnosis of 'Other' to be recorded for patients with rare and less prevalent disorders.

TABLE 4 NUMBER OF PEOPLE IN THE REGISTRY AND TREATED BY DETAILED DIAGNOSIS FOR HMA, HMB & VWD

	Number in ABDR Registry*				Number who Received Product*†			
	2008-09	2009-10	2010-11	2011-12	2008-09	2009-10	2010-11	2011-12
HMA								
Factor VIII Deficiency (Haemophilia A)	1,722	1,793	1,852	1,918	653	818	856	873
Asymptomatic Carrier Factor VIII Deficiency (Haemophilia A)	198	210	233	253	5	5	6	6
Symptomatic Carrier Factor VIII Deficiency (Haemophilia A)	74	82	95	103	<5	8	13	11
Acquired Factor VIII Inhibitor (Acquired Haemophilia A)	25	33	40	47	6	<5	5	5
HMB								
Factor IX Deficiency (Haemophilia B)	409	422	435	449	141	171	175	176
Asymptomatic Carrier Factor IX Deficiency (Haemophilia B)	45	50	60	63	<5	6	<5	<5
Symptomatic Carrier Factor IX Deficiency (Haemophilia B)	24	29	32	32	<5	6	9	6
VWD†								
Acquired von Willebrand Factor Disease	10	11	12	15	<5	<5	-	-
von Willebrand Disease - Uncharacterised	403	424	442	462	8	13	10	12
von Willebrand Disease Type 1	949	1,038	1,122	1,200	38	78	59	64
von Willebrand Disease Type 2 - Uncharacterised	82	93	99	110	<5	9	16	10
von Willebrand Disease Type 2A	67	70	75	84	9	14	13	14
von Willebrand Disease Type 2B	45	48	48	53	7	12	9	7
von Willebrand Disease Type 2M	63	73	81	85	12	9	10	15
von Willebrand Disease Type 2N	16	17	20	21	<5	<5	<5	<5
von Willebrand Disease Type 3	44	45	45	47	16	29	31	27

* As noted in the section *Data quality issues* (p14) the data has been improved since previous ABDR Annual Reports. The figures presented here represent the most accurate data currently available. The census date for number of people in the registry is 30 June, the last day of the financial year.
†Those with VWD may have been treated with DDAVP and this is not recorded.

TABLE 5 NUMBER OF PEOPLE IN THE REGISTRY AND TREATED BY DETAILED DIAGNOSIS FOR 'OTHER DISORDERS'

	Number in ABDR Registry*				Number who Received Product*			
	2008-09	2009-10	2010-11	2011-12	2008-09	2009-10	2010-11	2011-12
Other Factor Deficiency								
Factor V Deficiency	12	14	15	15	<5	<5	<5	<5
Factor VII Deficiency	51	52	53	53	6	5	5	9
Factor X Deficiency	13	14	14	18	<5	<5	<5	<5
Factor XI Deficiency	124	142	162	170	<5	<5	5	7
Factor XII Deficiency†	18	22	24	25	-	-	-	-
Factor XIII Deficiency	17	17	18	18	6	7	8	9
Fibrinogen - Afibrinogenemia	<5	<5	6	6	<5	-	<5	<5
Fibrinogen - Dysfibrinogenemia	12	12	12	18	<5	<5	<5	<5
Fibrinogen - Hypofibrinogenemia	<5	<5	<5	5	-	-	-	-
Fibrinogen Dysfunction - Uncharacterised	-	<5	<5	<5	-	-	-	-
Platelet Disorder‡								
Platelet - Bernard-Soulier	<5	<5	<5	<5	-	-	-	-
Platelet - Glanzmann's Thrombasthenia	7	8	12	14	-	<5	<5	<5
Platelet - Macrothrombocytopenias	8	8	9	9	-	-	-	-
Platelet - May Hegglin	<5	<5	<5	<5	-	-	<5	-
Platelet - Primary Secretion Defect	<5	<5	<5	<5	-	-	-	-
Platelet - Storage Pool (Dense Granule) Deficiency	11	17	23	29	-	-	-	<5
Platelet - Uncharacterised	133	139	153	164	-	<5	5	-
Vascular								
Vascular Disorders - Ehlers Danlos Syndrome	6	8	9	9	-	-	-	-
Other	145	156	165	214	-	-	<5	6

* As noted in the section *Data quality issues* (p14) the data has been improved since previous ABDR Annual Reports. The figures presented here represent the most accurate data currently available. The census date for number of people in the registry is 30 June, the last day of the financial year.
†Factor XII Deficiency does not require treatment with products, but is included as a diagnostic category.
‡The number of patients with platelet disorders who received product refers only to those who received recombinant clotting factor products. It does not include other treatments such as platelet transfusions.

AGE, DIAGNOSIS AND SEVERITY

In the following tables patients are categorised as either Adult (aged 18 years and over) or Paediatric and Adolescent (aged under 18 years) patients[4]. Table 6 and Table 7 detail the numbers of patients in the registry who received product (therapeutic treatment) during the period 2008-09 to 2011-12; by broad diagnosis and by severity.

The growth in patient population on the ABDR over time is evident. The majority of patients receiving treatment for bleeding disorders have HMA, specifically those patients with severe HMA (Appendix C, page 40).

There are some discrepancies in the data regarding the coding of severity when a patient receives treatment, and data cleansing and patient record updates are continuing. This will improve the forecasting for the national supply plan and budget for future years. It should be noted that the national forecasting and supply management process are currently performing very well.

Whilst the data discrepancies affect the analysis for this annual report, there is minimal impact on patient care as Haemophilia Treatment Centre staff have full access to their patient records for the provision of care and treatment.

[4] In previous ABDR Annual Reports the threshold age between paediatric and adult patients was 20 years of age. This threshold has been adjusted in the present report to better reflect the manner in which patients are treated in HTCs.

TABLE 6 NUMBER OF ADULTS IN THE REGISTRY AND TREATED BY BROAD DIAGNOSIS AND SEVERITY FOR HMA, HMB & VWD

	Number in ABDR Registry*				Number who Received Product*			
Adult (aged 18 years and over)	**2008-09**	**2009-10**	**2010-11**	**2011-12**	**2008-09**	**2009-10**	**2010-11**	**2011-12**
HMA								
Mild	859	903	963	1,010	133	160	188	178
Moderate	179	186	191	199	70	86	82	87
Severe	409	428	444	466	253	272	280	289
HMB								
Mild	217	232	250	258	38	52	50	48
Moderate	81	82	88	91	29	31	40	38
Severe	52	56	58	61	37	40	39	35
VWD								
Mild	865	945	1,014	1,087	26	50	41	42
Moderate	174	187	205	227	22	34	32	33
Severe	98	106	113	120	25	38	32	35

* As noted in the section *Data quality issues* (p14) the data has been improved since previous ABDR Annual Reports. The figures presented here represent the most accurate data currently available. The census date for number of people in the registry is 30 June, the last day of the financial year. Patients can have their severity categorised as 'unknown' or 'not applicable' during the initial diagnosis procedures, and these figures are not shown in this table.

TABLE 7 NUMBER OF PAEDIATRIC AND ADOLESCENT PATIENTS IN THE REGISTRY AND TREATED BY BROAD DIAGNOSIS AND SEVERITY FOR HMA, HMB & VWD

	Number in ABDR Registry*				Number who Received Product*			
Paediatric and Adolescent (aged less than 18 years)	**2008-09**	**2009-10**	**2010-11**	**2011-12**	**2008-09**	**2009-10**	**2010-11**	**2011-12**
HMA								
Mild	171	179	175	178	38	50	45	46
Moderate	65	68	68	65	41	43	50	50
Severe	245	246	258	258	213	238	241	258
HMB								
Mild	45	43	39	44	7	8	<5	8
Moderate	24	25	24	22	13	14	18	16
Severe	42	42	43	41	35	40	37	39
VWD								
Mild	219	227	241	236	5	15	8	11
Moderate	36	41	45	46	-	<5	6	5
Severe	33	32	30	30	6	13	15	12

* As noted in the section *Data quality issues* (p14) the data has been improved since previous ABDR Annual Reports. The figures presented here represent the most accurate data currently available. The census date for number of people in the registry is 30 June, the last day of the financial year.

BY AGE GROUP AND DETAILED DIAGNOSIS

In the next two tables, data is presented for Adult (aged 18 years and over) and Paediatric and Adolescent (aged under 18 years) patients[5]. Table 8 and Table 9 detail the numbers of patients in the registry who received product (therapeutic treatment) during the period 2008-09 to 2011-12; the numbers are subdivided by detailed diagnosis.

The data shows slight growth in the number of patients in the four year period, and a pronounced increase in the number of patients receiving treatment with a clotting factor product for HMA, HMB and VWD. These increases are also reflected in the data on national supply of products for treatment (Figure 2, page 16).

[5] In previous ABDR Annual Reports the threshold age between paediatric and adult patients was 20 years of age. This threshold has been adjusted in the present report to better reflect the manner in which patients are treated in HTCs.

TABLE 8 NUMBER OF PEOPLE IN THE REGISTRY DIAGNOSED WITH HMA OR HMB BY AGE GROUP AND DISEASE CLASSIFICATION

	Number in ABDR Registry*				Number who Received Product*			
	2008-09	2009-10	2010-11	2011-12	2008-09	2009-10	2010-11	2011-12
HMA – Adult (aged 18 years and over)								
Factor VIII Deficiency (Haemophilia A)	1,258	1,313	1,365	1,430	444	504	532	538
Asymptomatic Carrier Factor VIII Deficiency (Haemophilia A)	192	206	229	249	5	5	6	6
Symptomatic Carrier Factor VIII Deficiency (Haemophilia A)	59	67	78	84	<5	8	9	8
Acquired Factor VIII Inhibitor (Acquired Haemophilia A)	25	33	40	47	6	<5	5	6
HMA – Paediatric (aged less than 18 years)								
Factor VIII Deficiency (Haemophilia A)	464	480	487	488	292	333	339	357
Asymptomatic Carrier Factor VIII Deficiency (Haemophilia A)	6	<5	<5	<5	-	-	-	-
Symptomatic Carrier Factor VIII Deficiency (Haemophilia A)	15	15	17	19	<5	-	<5	<5
Acquired Factor VIII Inhibitor (Acquired Haemophilia A)	-	-	-	-	-	-	-	-
HMB – Adult (aged 18 years and over)								
Factor IX Deficiency (Haemophilia B)	303	317	333	346	100	112	119	113
Asymptomatic Carrier Factor IX Deficiency (Haemophilia B)	43	47	56	59	<5	5	<5	<5
Symptomatic Carrier Factor IX Deficiency (Haemophilia B)	20	25	29	29	<5	6	9	6
HMB – Paediatric (aged less than 18 years)								
Factor IX Deficiency (Haemophilia B)	106	105	102	103	55	62	59	63
Asymptomatic Carrier Factor IX Deficiency (Haemophilia B)	<5	<5	<5	<5	-	<5	-	-
Symptomatic Carrier Factor IX Deficiency (Haemophilia B)	<5	<5	<5	<5	-	-	-	-

* As noted in the section *Data quality issues* (p14) the data has been improved since previous ABDR Annual Reports. The figures presented here represent the most accurate data currently available. The census date for number of people in the registry is 30 June, the last day of the financial year.

TABLE 9 NUMBER OF PEOPLE IN THE REGISTRY DIAGNOSED WITH VWD BY AGE GROUP AND DISEASE CLASSIFICATION

	Number in ABDR Registry*				Number who Received Product*			
	2008-09	2009-10	2010-11	2011-12	2008-09	2009-10	2010-11	2011-12
VWD – Adult (aged 18 years and over)								
Acquired von Willebrand Factor Disease	10	11	12	15	<5	<5	-	-
von Willebrand Disease - Uncharacterised	330	347	365	386	7	8	10	12
von Willebrand Disease Type 1	749	833	897	977	33	66	48	51
von Willebrand Disease Type 2 - Uncharacterised	57	62	67	75	<5	6	10	<5
von Willebrand Disease Type 2A	55	57	61	69	9	14	11	14
von Willebrand Disease Type 2B	37	40	43	45	<5	11	8	7
von Willebrand Disease Type 2M	46	52	59	64	13	6	9	13
von Willebrand Disease Type 2N	16	17	19	20	<5	<5	<5	<5
von Willebrand Disease Type 3	30	31	33	35	18	20	21	19
VWD – Paediatric (aged less than 18 years)								
von Willebrand Disease - Uncharacterised	73	77	77	76	<5	5	<5	<5
von Willebrand Disease Type 1	200	205	225	223	5	13	11	14
von Willebrand Disease Type 2 - Uncharacterised	25	31	32	35	<5	<5	6	6
von Willebrand Disease Type 2A	12	13	14	15	<5	-	<5	<5
von Willebrand Disease Type 2B	8	8	5	8	<5	<5	<5	-
von Willebrand Disease Type 2M	17	21	22	21	-	<5	<5	<5
von Willebrand Disease Type 2N	-	-	<5	<5	-	-	-	-
von Willebrand Disease Type 3	14	14	12	12	<5	9	11	8

* As noted in the section *Data quality issues* (p14) the data has been improved since previous ABDR Annual Reports. The figures presented here represent the most accurate data currently available. The census date for number of people in the registry is 30 June, the last day of the financial year.

BY LOCATION

Figure 4 depicts the geographic distribution of all patients in the ABDR. Patient distribution is largely in line with the distribution of the general population. However, a more detailed analysis of geographic distribution could be expected to reveal the clustering effects often associated with the distribution of genetic disorder.

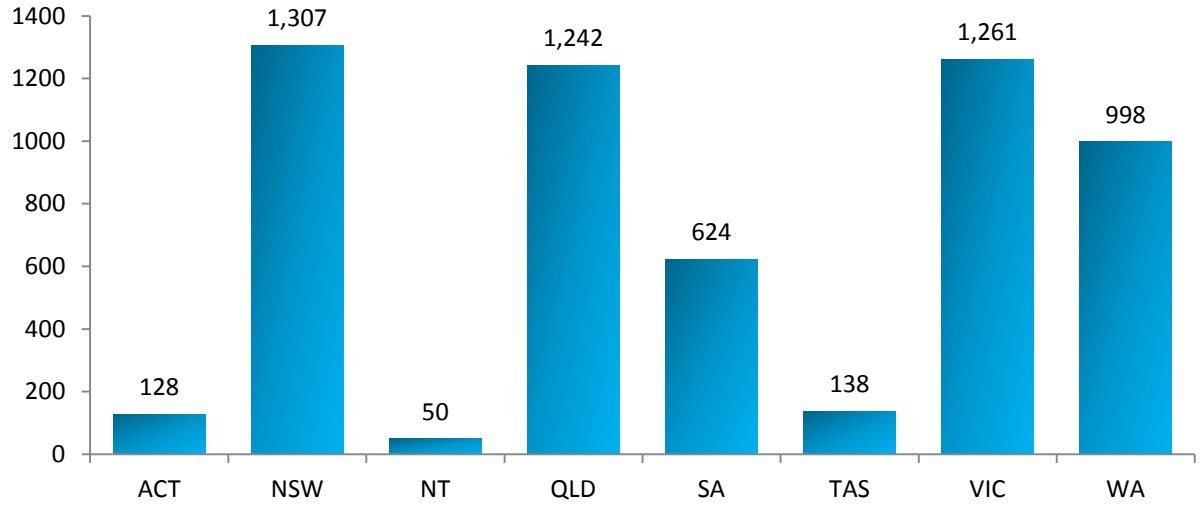

FIGURE 4 NUMBERS OF PEOPLE IN THE REGISTRY AS AT 30 JUNE 2012

Table 10 lists the numbers of patients with severe HMA and HMB by State/Territory.

TABLE 10 NUMBERS OF PATIENTS WITH SEVERE HMA AND HMB BY LOCATION

State/Territory	HMA	HMB
ACT	19	<5
NSW	209	31
NT	8	-
QLD	169	20
SA	68	5
TAS	15	<5
VIC	193	39
WA	83	12
Grand Total	**724**	**102**

BY SEX AND AGE DISTRIBUTION

The figures in this section present the sex and age distribution of patients in the ABDR at 2011-12, compared to the general Australian population[6]. The general population are represented by vertical bars and the ABDR patients are represented by line plots.

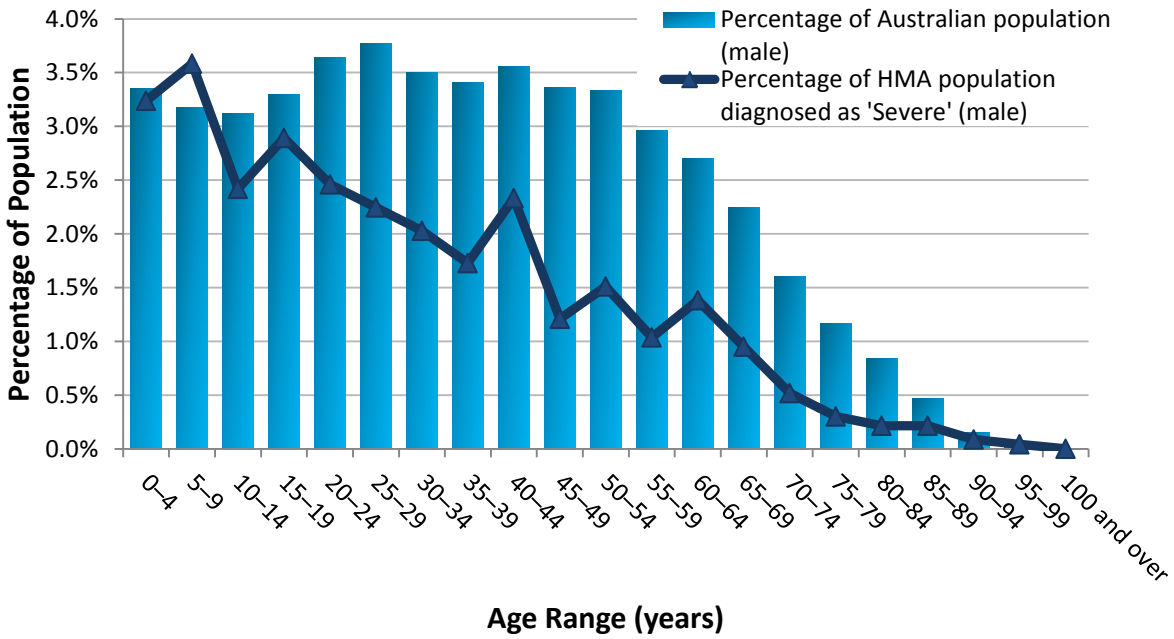

FIGURE 5 DISTRIBUTION OF MALE SEVERE HMA PATIENTS BY AGE IN 2011-12

Figure 5 charts the distribution of male severe HMA patients against the male population. The disorder is genetically linked to a patient's sex, and usually affects males. There is a relatively lower number of older patients (from the age grouping of 45-49 years onwards). The life expectancy of HMA patients has improved dramatically[7] in recent decades. The younger cohorts can expect to survive longer, which will increase the overall patient population and the demand for product in the future.

[6] Australian Demographic Statistics, June 2012. Australian Bureau of Statistics, Cat. No. 31010.
[7] Oldenburg J, Dolan G, Lemm G (2009).Haemophilia care then, now and in the future. Haemophilia 15, S1: 2-7.

Figure 6 charts the distribution of male severe HMB patients against the male population. As with HMA, HMB is also genetically linked to a patient's sex, and usually affects males. The observed male severe HMB population does not conform to the same pattern as the general male population, however there are a low patient numbers (n=100) in this group and no conclusions should be drawn.

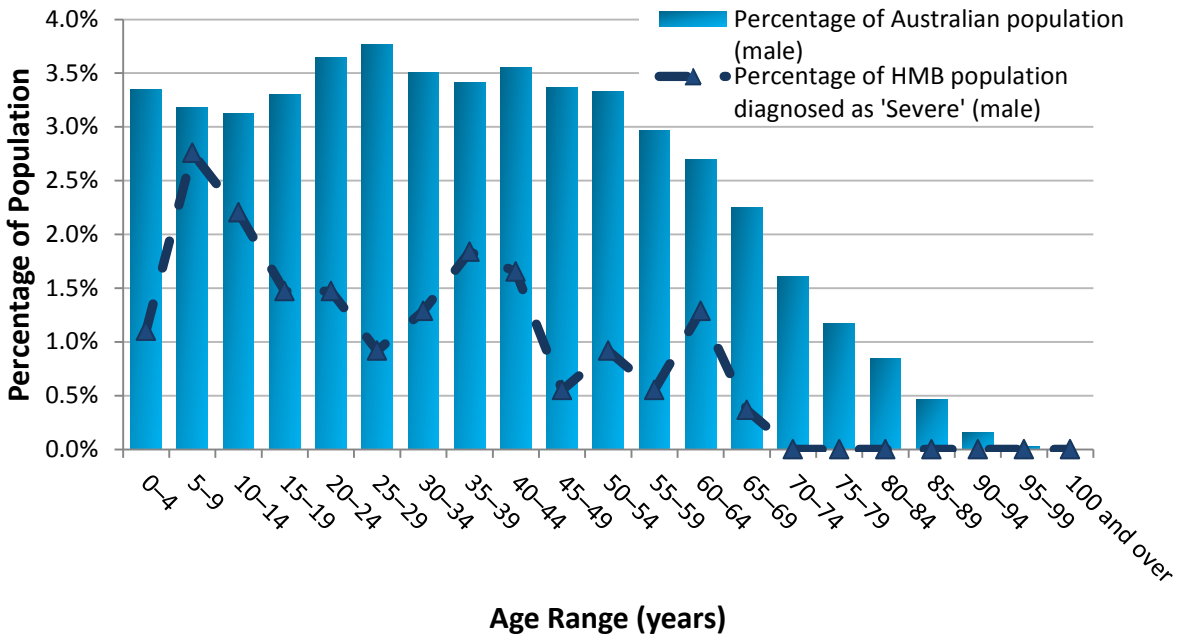

FIGURE 6 DISTRIBUTION OF MALE SEVERE HMB PATIENTS BY AGE IN 2011-12

INCIDENCE OF MAJOR DISORDERS

When we consider the incidence of bleeding disorders in global terms we see great variety in data and the reported prevalence. Table 11 details the incidence statistics from the World Federation of Hemophilia (WFH) global survey 2011.

TABLE 11 INCIDENCE STATISTICS FROM WORLD FEDERATION OF HAEMOPHILIA GLOBAL SURVEY 2011

Country	Population	HMA/HMB	VWD	OBD	HMA/HMB per100,000	VWD per 100,000	OBD per 100,000
Australia	**22,620,600**	**2,628**	**1,966**	**666**	**11.62**	**8.69**	**2.94**
New Zealand	4,405,200	416	186	23	9.44	4.22	0.52
UK	62,641,000	6,575	9,301	7,583	10.50	14.85	12.11
USA	311,591,917	17,485	13,239	1,772	5.61	4.25	0.57
Canada	34,482,779	3,380	3,563	1,460	9.80	10.33	4.23
France	65,436,552	5,735	1,330	375	8.76	2.03	0.57
Sweden	9,453,000	1,020	1,538	-	10.79	16.27	-
Germany	81,726,000	4,654	4,447	-	5.69	5.44	-
Spain	46,235,000	1,953	710	211	4.22	1.54	0.46
Netherlands	16,696,000	1,397	2,500	65	8.37	14.97	0.39

Abbreviations; OBD - other bleeding disorders; defined in the WFH Global Survey 2011 as "rare factor deficiencies, and inherited platelet disorders" (i.e. not HMA, HMB, VWD)

In 2010, Stonebreaker *et al*[8] reported on prevalence data for 106 countries from the WFH annual global surveys and the literature. They found that the reported HMA prevalence varied considerably among countries, even among the wealthiest of countries. Prevalence data reported from the WFH compared well with prevalence data from the literature, but patient registries (such as the ABDR) generally provided the highest quality prevalence data.

In 2011, the same group reported on the prevalence of haemophilia B[9]. Data was reported for 105 countries from the WFH annual global surveys. They reported that the prevalence varied considerably among countries, even among the wealthiest of countries.

Prevalence data is extremely valuable information for the planning efforts of national healthcare agencies in setting priorities and allocating resources for the treatment of bleeding disorders.

Table 12 details the incidence in 2011-12 of HMA, HMB and VWD per 100,000 people in Australia by broad diagnosis and severity.

[8] 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.

[9] Stonebraker JS, Bolton-Maggs PHB, Soucie JM, Walker I, Brooker M. (2011). A study of variations in the reported hemophilia B prevalence around the world. Haemophilia 18(3): 1-4.

TABLE 12 INCIDENCE OF HMA, HMB AND VWD PER 100,000 IN AUSTRALIA BY BROAD DIAGNOSIS AND SEVERITY

	Male				Female				Persons			
	2008-09	2009-10	2010-11	2011-12	2008-09	2009-10	2010-11	2011-12	2008-09	2009-10	2010-11	2011-12
HMA	**16.0**	**16.5**	**16.4**	**16.2**	**2.5**	**2.7**	**2.7**	**2.7**	**9.2**	**9.6**	**9.5**	**9.4**
Mild	7.5	7.7	7.6	7.5	1.6	1.7	1.7	1.7	4.5	4.7	4.6	4.6
Moderate	2.4	2.4	2.5	2.4	0.0	0.0	0.0	0.0	1.2	1.2	1.2	1.2
Severe	5.9	6.1	6.0	5.9	0.1	0.1	0.1	0.1	3.0	3.1	3.1	3.0
HMB	**3.7**	**3.8**	**3.7**	**3.7**	**0.7**	**0.8**	**0.8**	**0.7**	**2.2**	**2.2**	**2.2**	**2.2**
Mild	1.8	1.9	1.8	1.8	0.5	0.5	0.5	0.5	1.2	1.2	1.2	1.2
Moderate	0.9	0.9	0.9	0.9	0.0	0.0	0.0	0.0	0.5	0.5	0.5	0.5
Severe	0.9	0.9	0.9	0.9	0.0	0.0	0.0	0.0	0.4	0.4	0.4	0.4
VWD	**6.9**	**7.3**	**7.3**	**7.2**	**8.9**	**9.4**	**9.4**	**9.3**	**7.9**	**8.4**	**8.3**	**8.2**
Mild	4.3	4.5	4.5	4.4	6.1	6.5	6.5	6.3	5.2	5.5	5.5	5.4
Moderate	1.0	1.1	1.1	1.1	1.0	1.0	1.0	1.0	1.0	1.1	1.1	1.1
Severe	0.7	0.7	0.7	0.7	0.6	0.6	0.6	0.6	0.6	0.6	0.6	0.6

Patient Treatment in 2011-12

The data in this section relates to patients who received treatment (products) during the 2011-12 financial year. Figure 7 and Figure 8 show data for the period 2008-09 to 2011-12, and chart the relative volume of therapeutic products issued according to patient severity. Patients with greater severity of bleeding disorders received more products.

PRODUCTS ISSUED

Figure 7 shows the proportion of HMA patients receiving treatment (shown as IU of product received) by severity. For the four financial years, around 60% (by volume) of all FVIII products issued were for patients with severe HMA.

Figure 8 shows the proportion of HMB patients receiving treatment (shown as IU of product received) by severity. For the four financial years, around 40% (by volume) of all FIX products issued were for patients with severe HMB. There are far fewer HMB patients in the registry than there are HMA patients.

About half of the patients in the ABDR are diagnosed with HMA (see Table 3, p7). 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.

Table 13 (p33) details the volume (IU) of product issued for HMA and HMB patients in 2011-12. The volumes are subdivided by severity and treatment regimen. The largest and most important sectors are products for severe HMA patients for *on demand* and *prophylactic* treatment regimens. The volume issued for prophylactic treatment of severe HMA is the single greatest determining factor for supply planning.

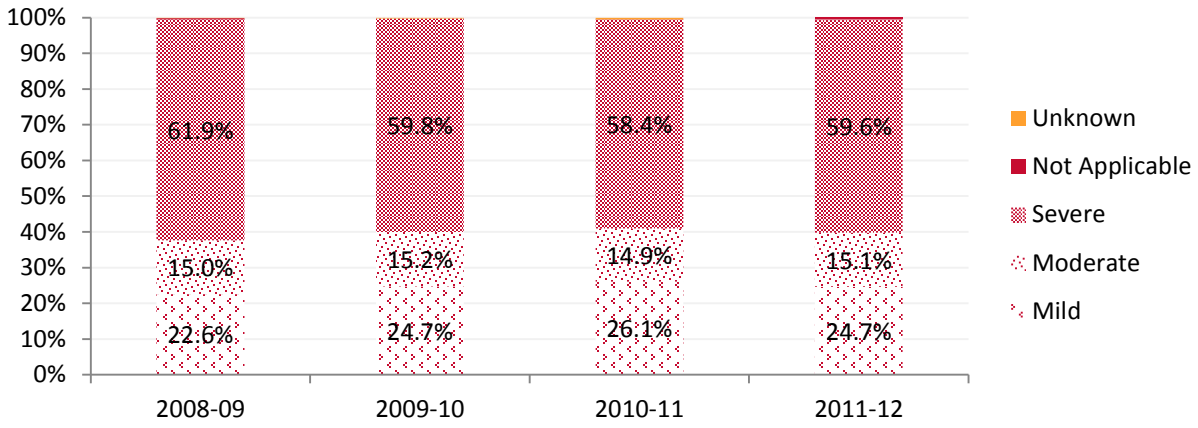

FIGURE 7 PROPORTION OF PATIENTS RECEIVING PRODUCT BY SEVERITY FOR HMA

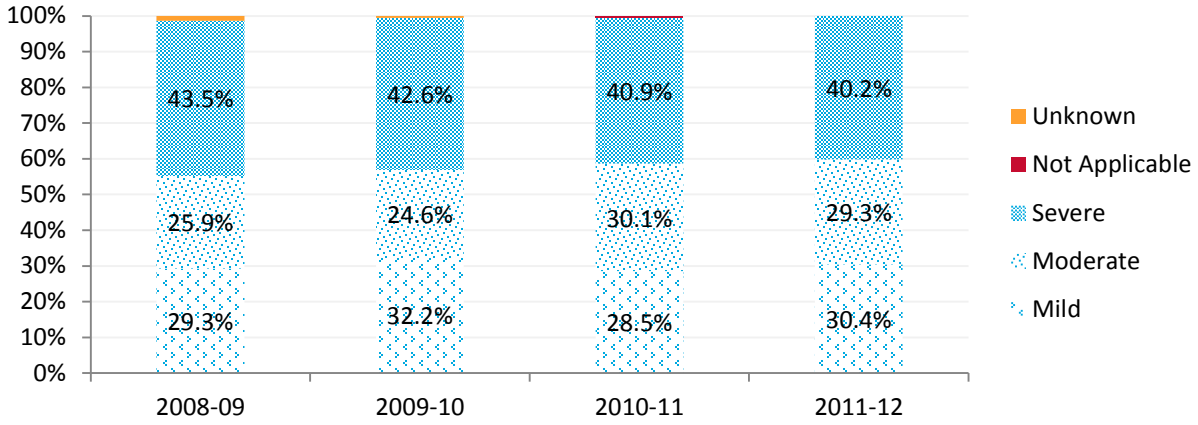

FIGURE 8 PROPORTION OF PATIENTS RECEIVING PRODUCT BY SEVERITY FOR HMB

TABLE 13 IU OF PRODUCT ISSUED FOR HMA AND HMB PATIENTS, BY SEVERITY AND TREATMENT REGIMEN IN 2011-12

	Mild	Moderate	Severe	Total**
HMA (IU FVIII Products)†	**5,672,500**	**13,010,000**	**114,633,240**	**133,315,740**
On Demand	3,173,250	3,254,250	16,954,490	23,381,990
Prophylaxis	957,000	7,595,250	81,029,500	89,581,750
Secondary Prophylaxis	-	6,500	1,700,500	1,707,000
Tolerisation	52,000	-	7,367,250	7,419,250
Unknown*	1,490,250	2,154,000	7,581,500	11,225,750
HMB (IU FIX Products)‡	**2,533,750**	**6,080,000**	**12,757,000**	**21,370,750**
On Demand	1,642,500	2,478,500	3,105,500	7,226,500
Prophylaxis	-	2,915,500	6,797,500	9,713,000
Secondary Prophylaxis	-	-	500,000	500,000
Tolerisation	-	-	-	-
Unknown*	891,250	686,000	2,354,000	3,931,250

† FVIII Products included are Advate, Biostate, Kogenate, Recombinate, ReFacto and Xyntha
‡ FIX Products included are BeneFIX and MonoFIX
* This represents a blank/not completed/empty field for the treatment regimen in the ABDR.
** The total in this table combines the values for patients with mild, moderate and severe conditions. The severity of a patient's condition is not always known at initial presentation. This table does not include product issues to patients with unknown severities.

VOLUME (IU) OF PRODUCTS ISSUED FOR HMA AND HMB

Table 14 lists the volumes (IU) issued by age group and treatment regimen. In both the adult and paediatric age groups the majority of product is issued for patients on prophylactic treatment regimens, followed by on demand regimens. The ABDR issues data contains a large amount of records where the treatment regimen is blank, unknown and not specified. There are ongoing efforts to rectify this.

Severe haemophilia requires lifelong treatment with expensive products. Clotting factor consumption is often expressed in IU/kg/year, and the ranges reported vary by population.[10,11] Figure 9 shows the clotting factor consumption during 2011-12 for severe HMA patients aged 0-18 years (IU/kg/year). There is a wide range of use across these age groups, which are not normally distributed. Median values for the age groups were 3764 IU/kg/year (0-4 years), 4096 IU/kg/year (5-9 years), 3961 IU/kg/year (10-14 years) and 3422 IU/kg/year (15-18 years).

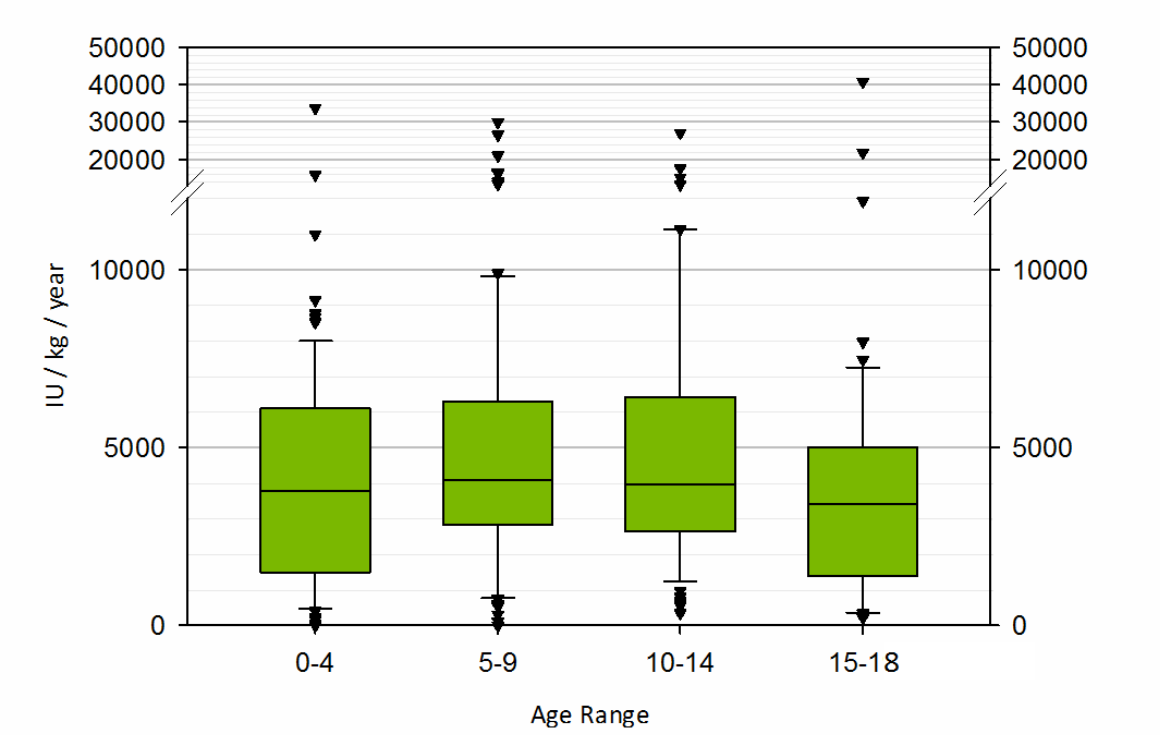

FIGURE 9 PRODUCT USAGE (IU/KG/YEAR) IN SEVERE HMA PATIENTS AGED 0-18 YEARS

These figures are higher than some of those reported in the literature for adult patients, but reflect the shift in treatment practice towards regular prophylactic infusions to prevent bleeds, especially in children. Recent theoretical work allowed for the comparison of different treatment strategies, ranging from long-term on demand therapy to different prophylactic strategies.[12] In time, the ABDR data should provide further insight into these issues.

[10] Schramm W, Royal S, Kroner B, Berntorp E, Giangrande P, Ludlam CA, et al. (2002). Clinical outcomes and resource utilization associated with haemophilia care in Europe. Haemophilia 8(1): 33-43.
[11] Aledort LM, Haschmeyer RH, Pettersson H (1994) A longitudinal study of orthopaedic outcomes for severe factor-VIII-deficient haemophiliacs. The Orthopaedic Outcome Study Group. J Intern Med. 236(4): 391-399.
[12] Fischer K, Pouw ME, Lewandowski D, Janssen MP, van den Berg HM, van Hout BA (2011). A modelling approach to evaluate long-term outcome of prophylactic and on demand treatment strategies for severe hemophilia A. Haematologica 96(5): 738-743.

TABLE 14 VOLUME (IU) OF PRODUCTS ISSUED IN 2011-12 TO ADULT AND PAEDIATRIC PATIENTS BY TREATMENT REGIMEN

	Adult					Paediatric				
	On Demand	Prophylaxis	Tolerisation	Not specified	Adult Total	On Demand	Prophylaxis	Tolerisation	Not specified	Paediatric Total
HMA	**19,607,500**	**48,198,250**	**1,568,000**	**10,362,000**	**79,735,750**	**3,834,240**	**43,114,500**	**5,851,250**	**956,250**	**53,756,240**
Advate	7,892,500	19,200,000	-	5,118,000	32,210,500	2,367,750	21,068,750	750,500	419,750	24,606,750
BeneFIX	-	-	-	-	-	-	7,500	-	-	7,500
Biostate	89,000	971,750	-	219,250	1,280,000	104,000	1,316,000	5,050,750	184,250	6,655,000
Kogenate FS	2,385,000	4,324,000	-	626,000	7,335,000	720,000	6,723,750	-	102,500	7,546,250
Recombinate	1,700,000	2,712,000	-	138,500	4,550,500	175,250	4,195,250	-	106,500	4,477,000
ReFacto	-	-	-	6,000	6,000	-	-	-	-	-
Xyntha‡	7,541,000	20,990,500	1,568,000	4,254,250	34,353,750	467,240	9,803,250	50,000	143,250	10,463,740
HMB	**6,614,000**	**5,383,000**	**-**	**3,489,500**	**15,486,500**	**624,500**	**4,830,000**	**-**	**441,750**	**5,896,250**
BeneFIX	6,032,000	4,253,000	-	3,297,500	13,582,500	619,500	4,830,000	-	441,750	5,891,250
Biostate	12,000	-	-	-	12,000	-	-	-	-	-
MonoFIX - VF	570,000	1,130,000	-	192,000	1,892,000	5,000	-	-	-	5,000

‡ Combines totals for Xyntha and Xyntha Dual Chamber

Appendix A Characteristics of Rare Clotting Factor Deficiencies

Table 15 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 conc. (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 conc. •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 conc. •Desmopressin
Factor VII	1 in 500,000	Autosomal recessive	Severe when factor levels are low	•Recombinant Factor VIIa conc. •Factor VII conc. •Prothrombin complex conc. •Fresh frozen plasma
Factor X	1 in 1 million	Autosomal recessive	Moderate to severe when factor levels are low	•Prothrombin complex conc. •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 conc. •Fresh frozen plasma
Factor XI	1 in 100,000	Recessive or dominant	Mild to moderate when factor levels are low	•Factor XI concentrate •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 conc. •Cryoprecipitate •Fresh frozen plasma

* 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

Appendix B Haemophilia Treatment Centres

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

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.

Haemophilia Centres 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, i.e. 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
- data management to facilitate the use of an information system database, such as the Australian Bleeding Disorder Registry, used in the clinical environment to aid in the capturing of data critical to HTC staff for the day to day management of people with bleeding disorders and for supply management and policy purposes

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.

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 are members of the Data Managers' Group (DMG). DMG Co-Chairs are elected and coordinate teleconferences, between all Data Managers, on a regular basis. The DMG Co-Chairs also have the functionality of raising issues, to the NBA, on behalf of their group. AHCDO has a major role in providing support to ABDR Data Managers through the funded model for Data Managers.

The advantages of this model of Haemophilia Data Co-ordination are:

- 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

A number of ongoing data quality initiatives were first implemented in 2010-11, including:

- Regular teleconferences for ABDR DMG
- 'Advanced Search' functionality of the ABDR whereby Data Managers are able to extract information from the ABDR on an ad hoc basis
- Reviews 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.

Comprehensive automated and manual data cleansing and validation processes that occurred as part of the 4th Generation ABDR Redevelopment project released in August 2012 enhanced the ABDR data accuracy and consistency presented in this report. The 4th Generation ABDR was successfully implemented on 13 August 2012.

However, there are still some data quality issues that impact the data presented in this report. Some post migration tasks for Data Managers to clean the data include

- Verify patients with more than one diagnosis
- Duplicate diagnoses to be deleted and Inhibitor Tests to be combined under the persisting diagnosis
- Verify severity ratings and treatment regiments for some patient records
- There are also a number of low level data verification activities

LIST OF HTCS

Hospital	Haemophilia Treatment Centre	State
The Canberra Hospital	Haemophilia Clinic	ACT
Calvary Mater Newcastle	Haemophilia Treatment Centre	NSW
Royal Prince Alfred Hospital	Haemophilia Treatment Centre	NSW
Sydney Children's Hospital	Centre for Children's Cancer and Blood Disorders	NSW
The Children's Hospital at Westmead	Haemophilia Treatment Centre	NSW
Royal Darwin Hospital	Haemophilia Treatment Centre	NT
Royal Brisbane and Women's Hospital	Queensland Haemophilia Centre	QLD
Royal Children's Hospital	Queensland Haemophilia Centre Child and Adolescent Service	QLD
Royal Adelaide Hospital	South Australia Haemophilia Treatment Centre	SA
Women's and Children's Hospital	South Australia Haemophilia Treatment Centre	SA
Royal Hobart Hospital	Tasmanian Haemophilia Treatment Centre	TAS
The Alfred Hospital	Ronald Sawyers Haemophilia Centre	VIC
Royal Children's Hospital	Henry Ekert Haemophilia Treatment Centre	VIC
The Haemophilia Centre of WA	Incorporating:	
	· Fremantle Hospital	WA
	· Princess Margaret Hospital	WA
	· Royal Perth Hospital	WA

Appendix C National Supply of Products

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.

The approved budget for 2011-12 covering the supply and management of blood and blood products and services under contract was $1,035.5 million, comprising $548.0 million for fresh blood products and plasma collection and $473.2 million for plasma and recombinant products. The remaining $14.3 million included items such as contributions for the National Managed Fund, interest monies, and support for the Australian Haemophilia Centre Directors' Organisation and administration of the ABDR.

Figure 10 illustrates the national supply budget by product category for 2011-12, and shows that 18.6% ($192.6 million) was budgeted for clotting factor products.

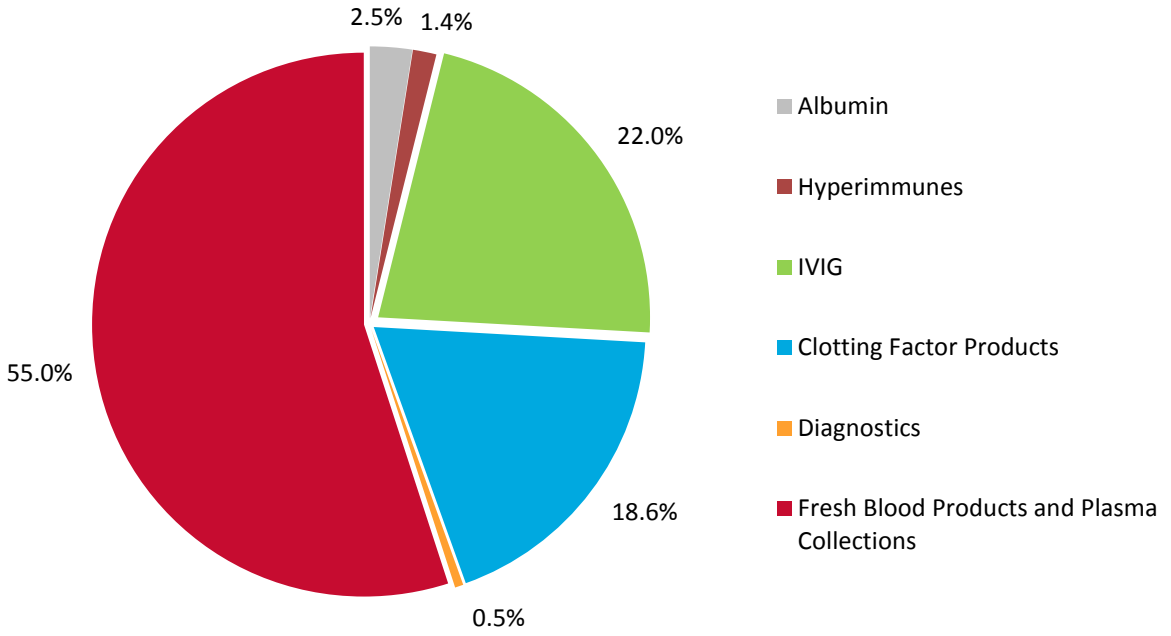

FIGURE 10 NATIONAL EXPENDITURE BY PRODUCT CATEGORY 2011-12

Two factors contributed to a positive impact on national expenditure on clotting factor products. There were significant savings against the 2011-12 approved budget as a result of the successful tender processes for the supply of recombinant FVIII (rFVIII) which delivered substantial savings for governments for the year. rFVIII products account for the majority of expenditure on clotting factor products. These new supply arrangements are in place until July 2014 and will return further savings.

Figure 11 and Figure 12 indicate that the demand for Factor VIII products in 2011-12 was one per cent less than in 2010-11. The annual growth rates for this product since 2009-10 have been 2.1 per cent, 11.3 per cent and -1 per cent respectively. The demand for plasma-derived product has decreased significantly, from 33.2 per cent growth in 2010-11 to a -6.3 per cent reduction in demand in this reporting year. Demand for rFVIII has also decreased in 2011-12 resulting in a reduction in growth from 8.6 per cent in 2010-11 to -0.3 per cent for 2011-12.

The reduction in growth for Factor VIII may be attributed to a number of factors, including a reduction in the number of patients undergoing tolerisation, a number of high volume patients participating in clinical trials and the continuing number of patients stabilising onto prophylaxis home treatment.

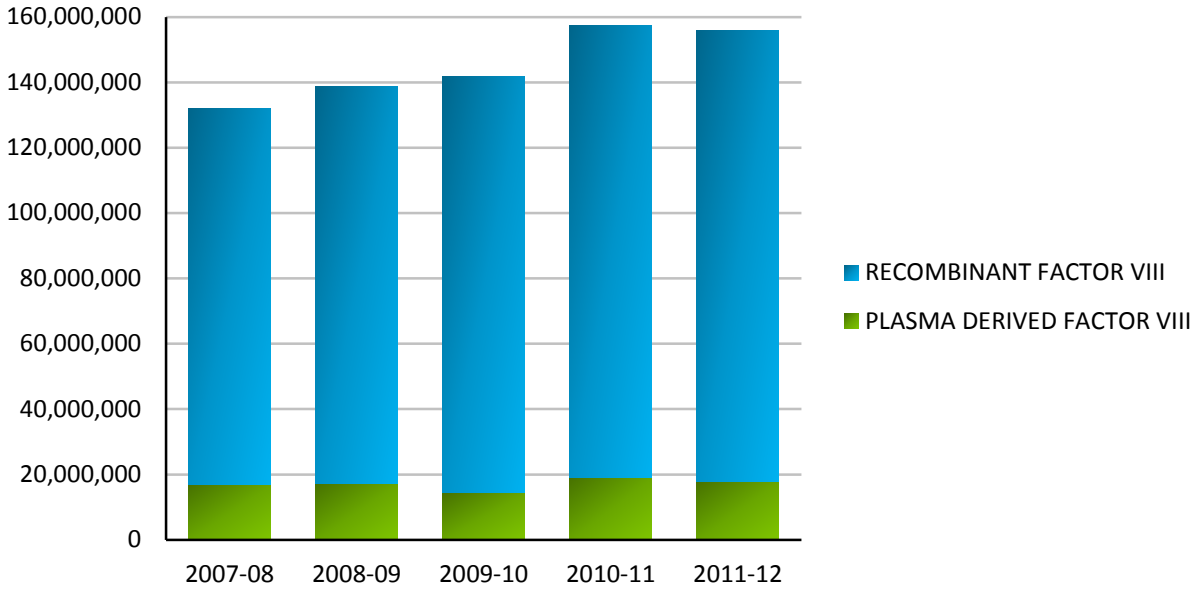

FIGURE 11 ISSUES OF FVIII PRODUCTS, 2007-08 TO 2011-12

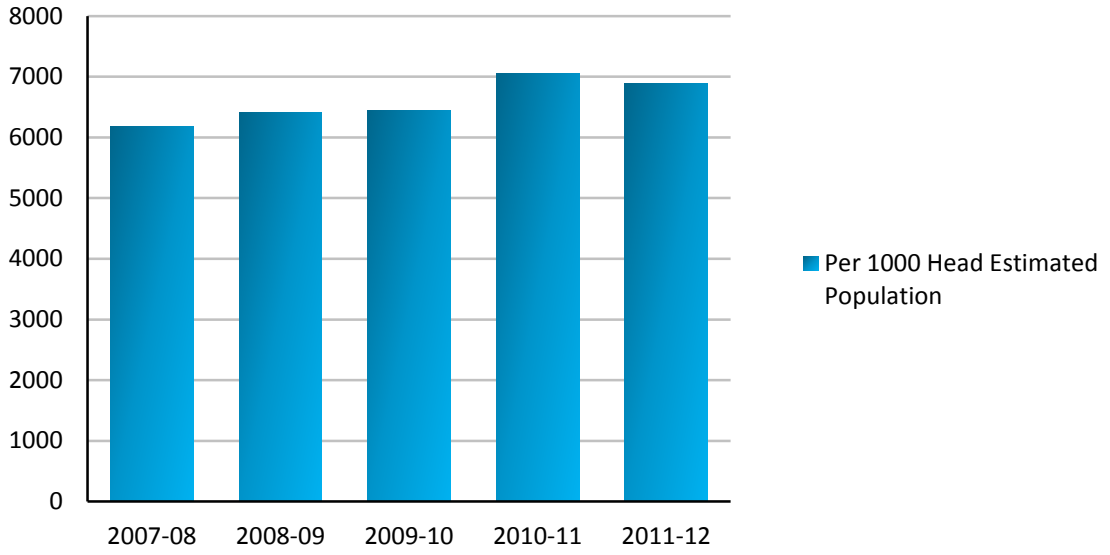

FIGURE 12 ISSUES OF TOTAL FVIII PER 1000 HEAD POPULATION, 2007 TO 2011-12

Demand for Factor IX (FIX) products in 2011-12 reduced by 4.4 per cent compared to 2010-11. This reduction was consistent with discussions during consultations with jurisdictions on the national supply plan and budget (see Figure 13 and Figure 14)

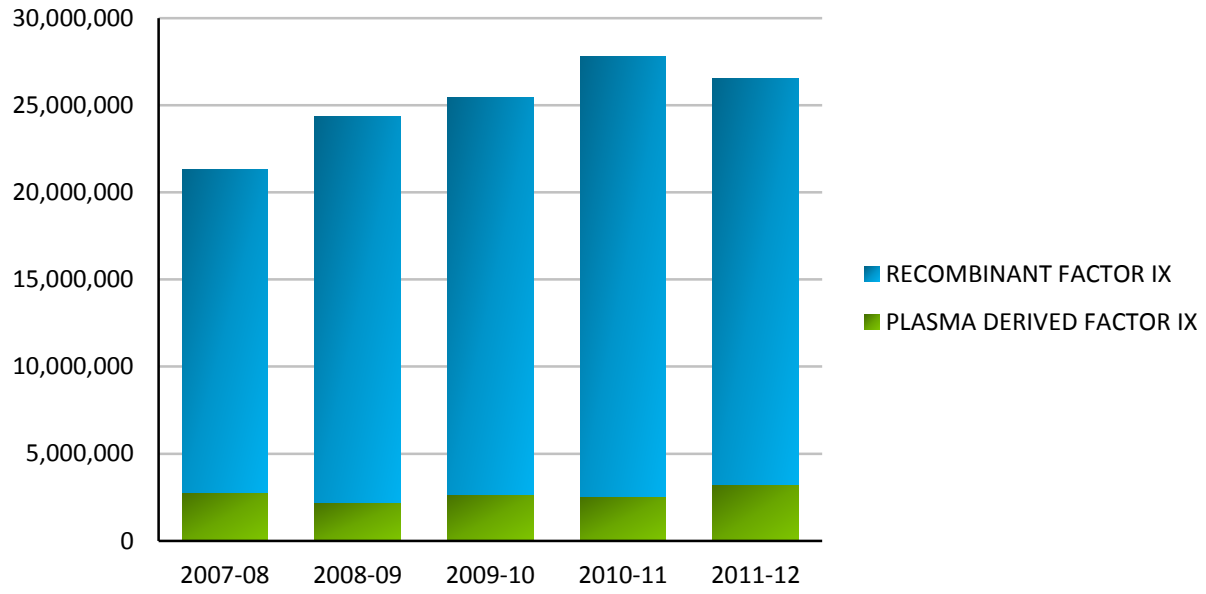

FIGURE 13 ISSUES OF FIX PRODUCTS, 2007-08 TO 2011-12

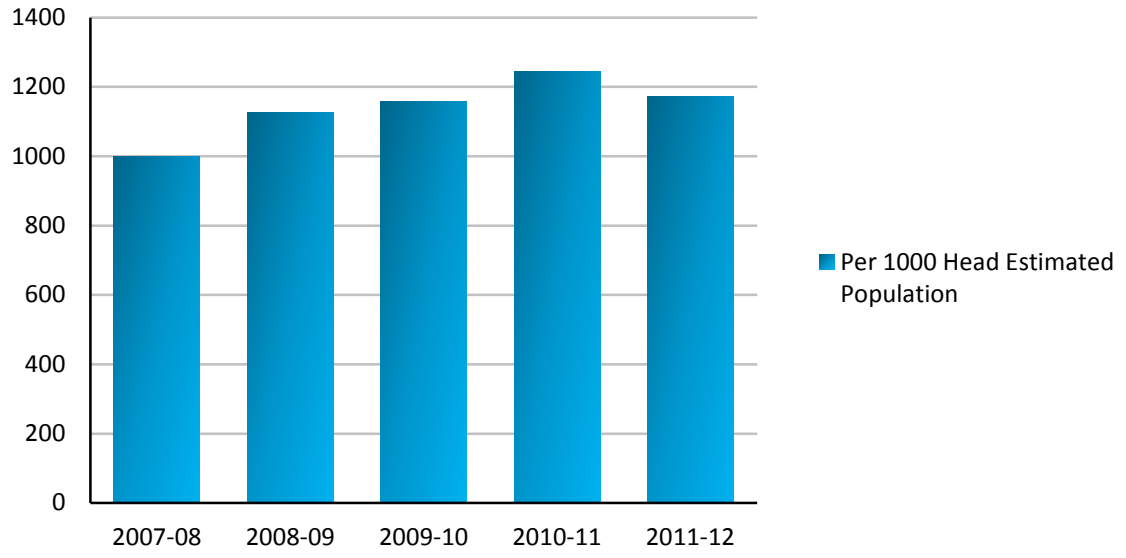

FIGURE 14 ISSUES OF FIX PRODUCTS PER 1000 HEAD OF POPULATION, 2007-08 TO 2011-12

It is acknowledged that a very small number of patients experiencing very high needs may considerably affect overall demand for recombinant Factor VIIa (rFVIIa) and Factor Eight Inhibitor Bypassing Activity (FEIBA) (data not shown). The 2011-12 level of demand for rFVIIa was 7 per cent below plan and 16 per cent below demand in 2010-11. This trend is expected to continue, although at slightly lower rates of decrease, for the foreseeable future. There has been a significant growth reduction in use of FEIBA which has resulted in 2011-12 demand being 28 per cent less than the supply plan, and 3.2 per cent less than 2010-11.

The driver for this reduction is patient-specific with some high volume patients having now completed tolerisation.

Appendix D History 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 sex.

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.

BENEFITS OF THE RE-DEVELOPED 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, sex 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

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 E Patient 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

☐ Home email ☐ Work email

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

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

Acronyms and glossary of terms

ACRONYMS

ABDR	Australian Bleeding Disorders Registry
AHCDO	Australian Haemophilia Centres Directors' Organisation
BU (BU/ml)	Bethesda unit (expressed as Bethesda units per millilitre)
DDAVP	Desmopressin (1-desamino-8-D-arginine vasopressin; 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.
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
IDMS	Integrated data management system – The NBA's integrated data management system.
IU	International Units
NBA	National Blood Authority
OBD	Other bleeding disorders
PWBD	People with a bleeding disorder
VWD	von Willebrand disease
WFH	World Federation of Hemophilia

GLOSSARY OF TERMS

bleeding disorders	Diseases that cause abnormal or exaggerated bleeding and poor blood clotting
blood products	Products manufactured from donated blood
fractionation	Blood plasma fractionation refers to the general processes of separating the various components of blood plasma.

National Blood Authority
Australia