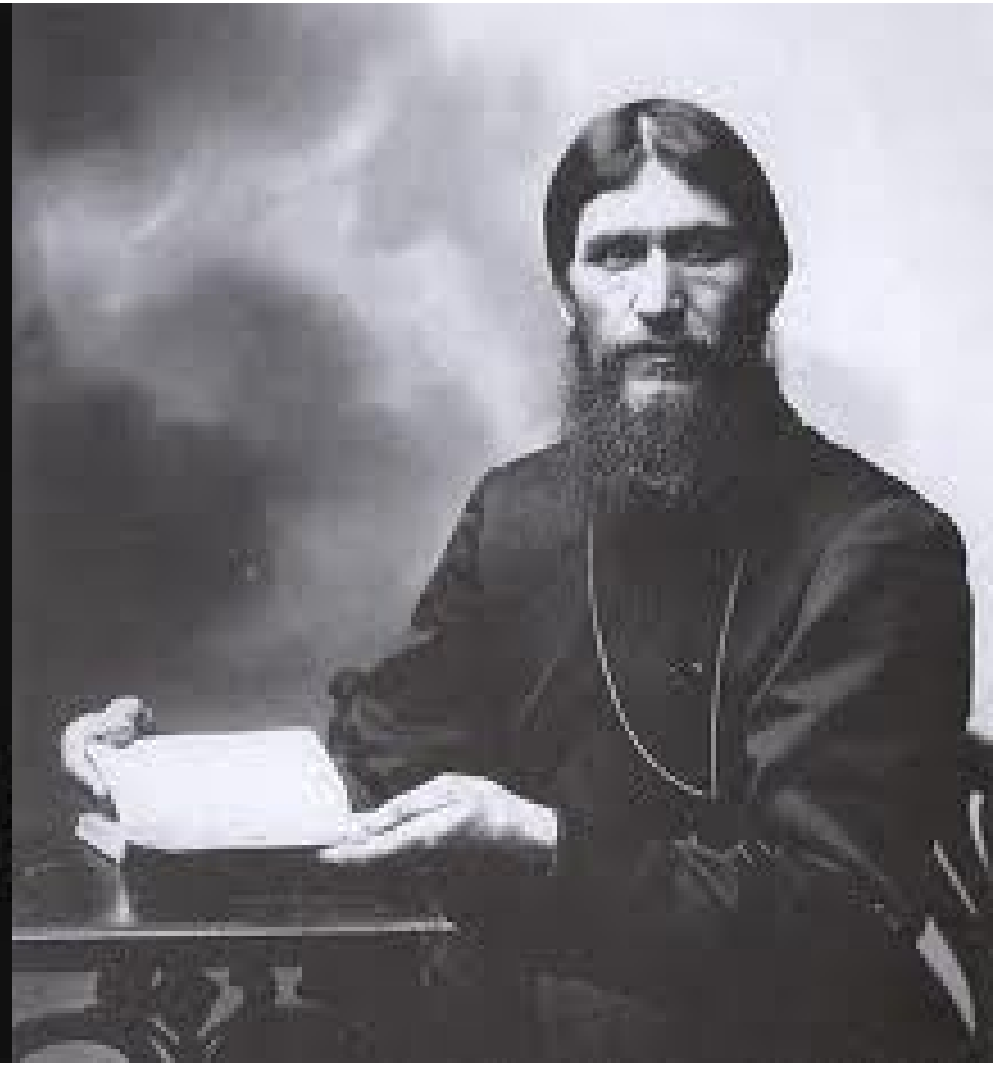
History of Comprehensive Care

Alison Street

HFA/HFNZ meeting October 2015

A hundred years ago

A supportive family



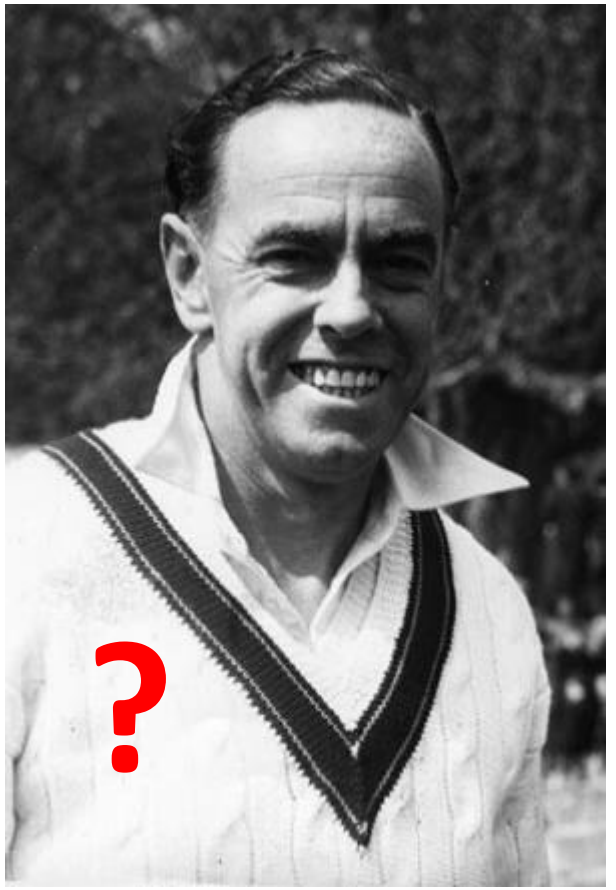
Working with a carer

Oxford U.K.

Giangrande P British Journal of Haematology, 2003, 121, 703–712

In Melbourne

Ron Sawers was a pioneer in diagnosis and care of patients with bleeding disorders



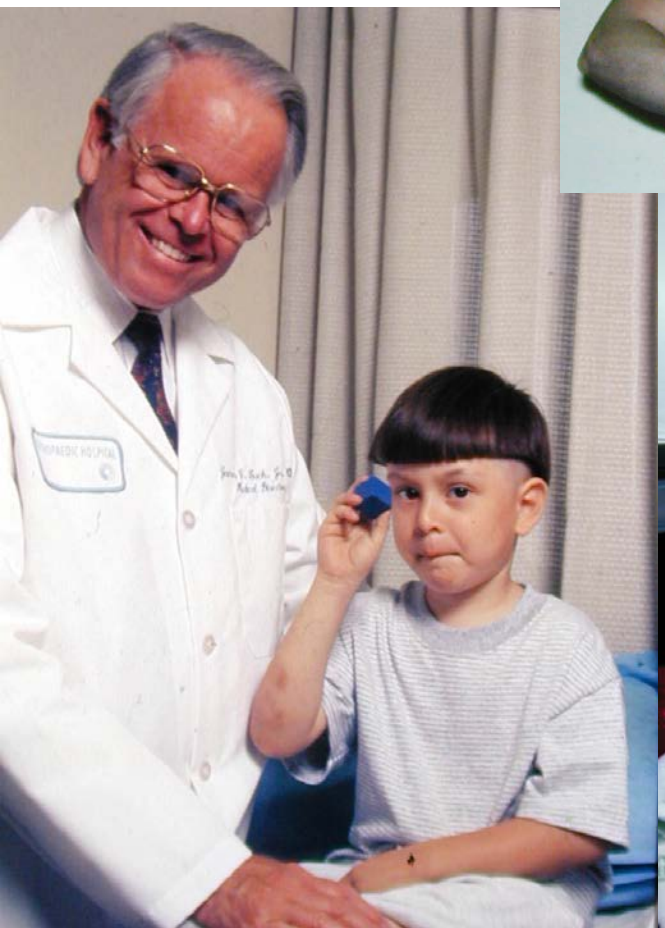
The first description of comprehensive care

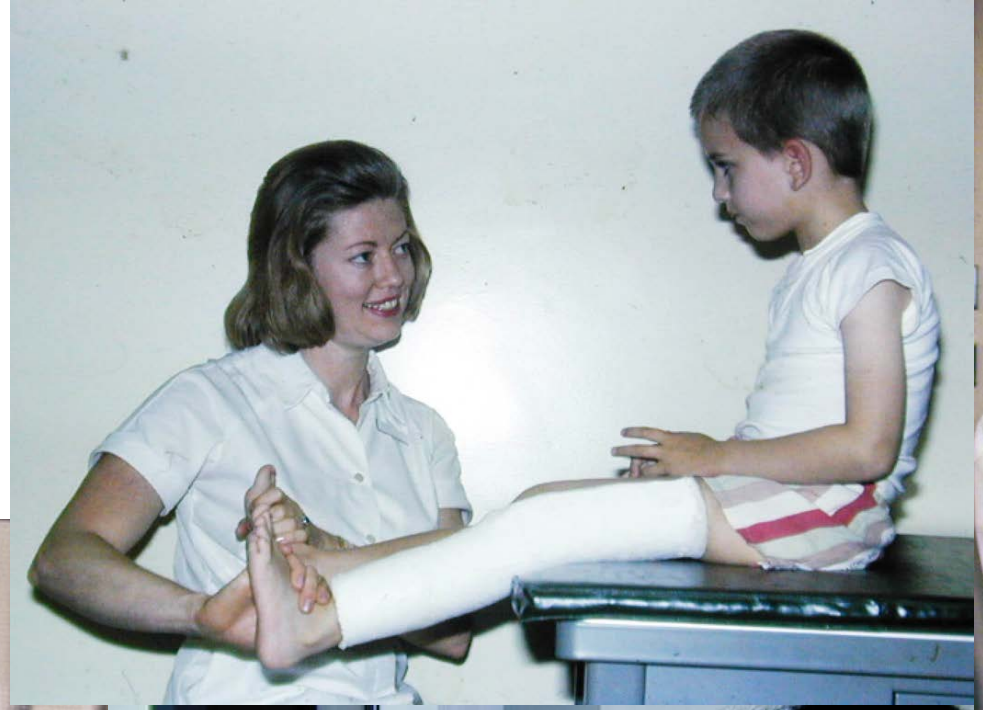
Treatment of Haemophilia and other Coagulation Disorders.

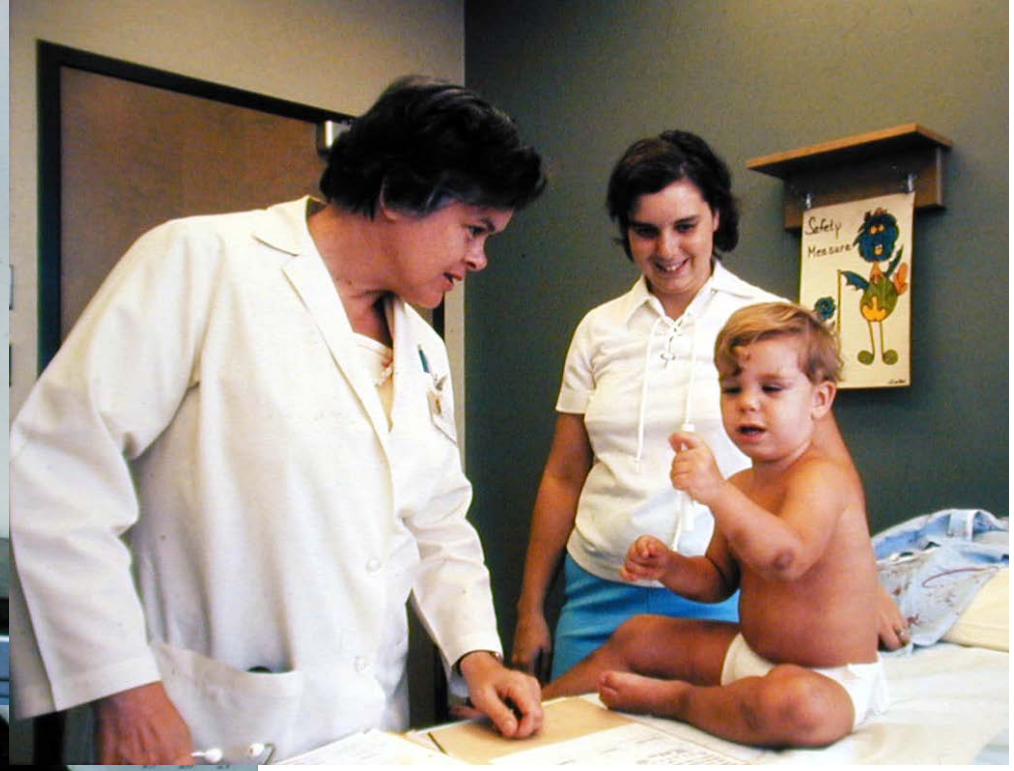
Edited by Rosemary Biggs, B.Sc.(Lond.), Ph.D.(Toronto), M.D.(Lond.), Medical Research Council Blood Coagulation Research Unit, the Churchill Hospital, Oxford; and R. G. Macfarlane, *C.B.E.,* M.A.(Oxon.), M.D.(Lond.),F.R.S., Professor of Clinical Pathology, University of Oxford; Director, Medical Research Council Blood Coagulation Research Unit, the Churchill Hospital, Oxford.

9 x 6½ in. Pp. xvii + 391, with 71 figures, 27 plates, some in colour, and 60 tables. Index. 1966. Oxford: Blackwell Scientific Publications. Price 75s

And in Los Angeles

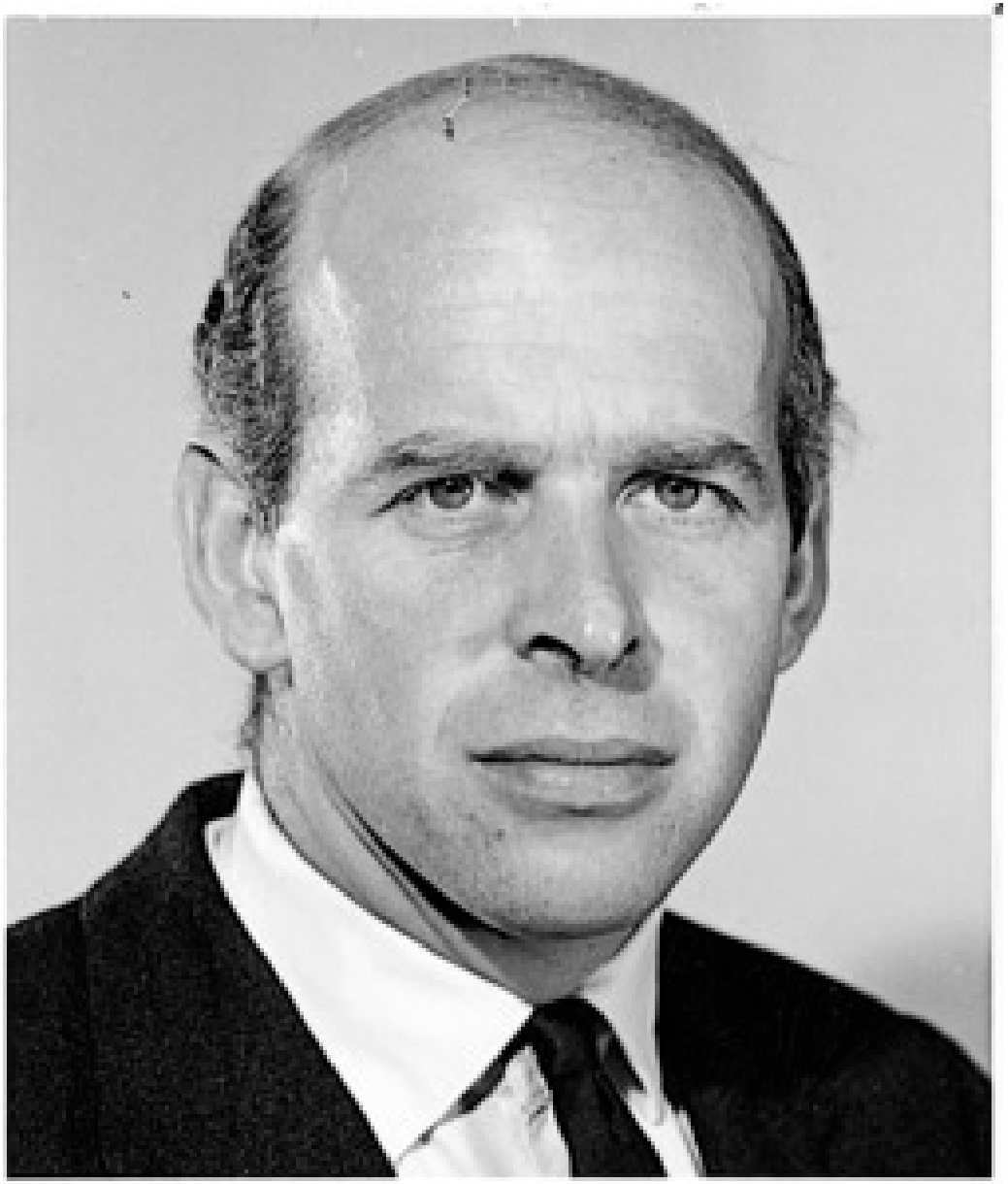






After development of cryoprecipitate in San Francisco in 1961

Meanwhile in Sydney



"The need to develop a broadly based service at **Royal Prince Alfred Hospital** became apparent about ten years ago when it was realised that haemophiliacs were using the hospital staff as their sole source of advice and guidance on every conceivable personal and social matter… having a constant source of support and guidance in the hospital clinic appeared to be the most helpful factor to the patients and relatives.

' 'accordingly fulltime services of social worker and nurse were made available to the clinic' '

Kerr CB. Comprehensive care for Haemophilia
J. Roy. Coll. Physicians. London 1971 5,3,263-267

With product availability the studies began

‘ ‘Increasing number of haemophiliacs have become involved in self-therapy programs’ ’

‘ ‘increases patient responsibility and decreases physician supervision’ ’

To detect and minimise problems as well as to maximize benefits and long term gains.. we developed a **systematic multidisciplinary approach to each individual**-and a comprehensive health care program’ ’

Levine PH et al. Comprehensive Health Care Clinic for Hemophiliacs. Arch Intern Med. 1976,138,792-4

And treatment outcomes described

Showed reduced hospital days and costs of care

Comprehensive Health Care Clinic for Hemophiliacs

Peter H. Levine, MD; Bernard A. McVarry, MD; Allyn E. Segelman, DMD; Catherine M. Cranford; Seymour Zimbler, MD

AJPH 1984;74;6:616-617

PUBLIC HEALTH BRIEFS

The Benefits of Comprehensive Care of Hemophilia: A Five-Year Study of Outcomes

PETER S. SMITH, MD, PETER H. LEVINE, MD, AND THE DIRECTORS OF ELEVEN PARTICIPATING HEMOPHILIA CENTERS*

Showed decreases in school absence, hospital admissions and unemployment

Showing cost effectiveness with “episodic” treatment

Hemophilia comprehensive care as developed from the 1960s was a pioneer model of chronic disease management

Studies from 1990’s showed that mortality and hospitalization rates decreased in persons with hemophilia treated in a hemophilia treatment centre (HTC).[1]

Costs of care were reduced with aggregation of services

1. Soucie JM, et al. Blood 2000; 96:437-442

Prophylaxis the recent clinical evidence

- Long term observational data from the 1960s in Sweden had shown reduced number of joint bleeds with better preservation of joint status as assessed by Xray changes [1].
- The randomised Joint Outcomes Study from USA reported in 2007 in children starting routine prophylactic therapy (RPT) before age three years confirmed these findings using MRI assessment [2]
- A similar study of secondary prophylaxis reported in 2013 showed reduced bleeding rates and improved joint functional scores in adolescents and adults [3,4]

1 Nilsson I.M. et al J Intern Med 1992. Jul;232(1):25-32

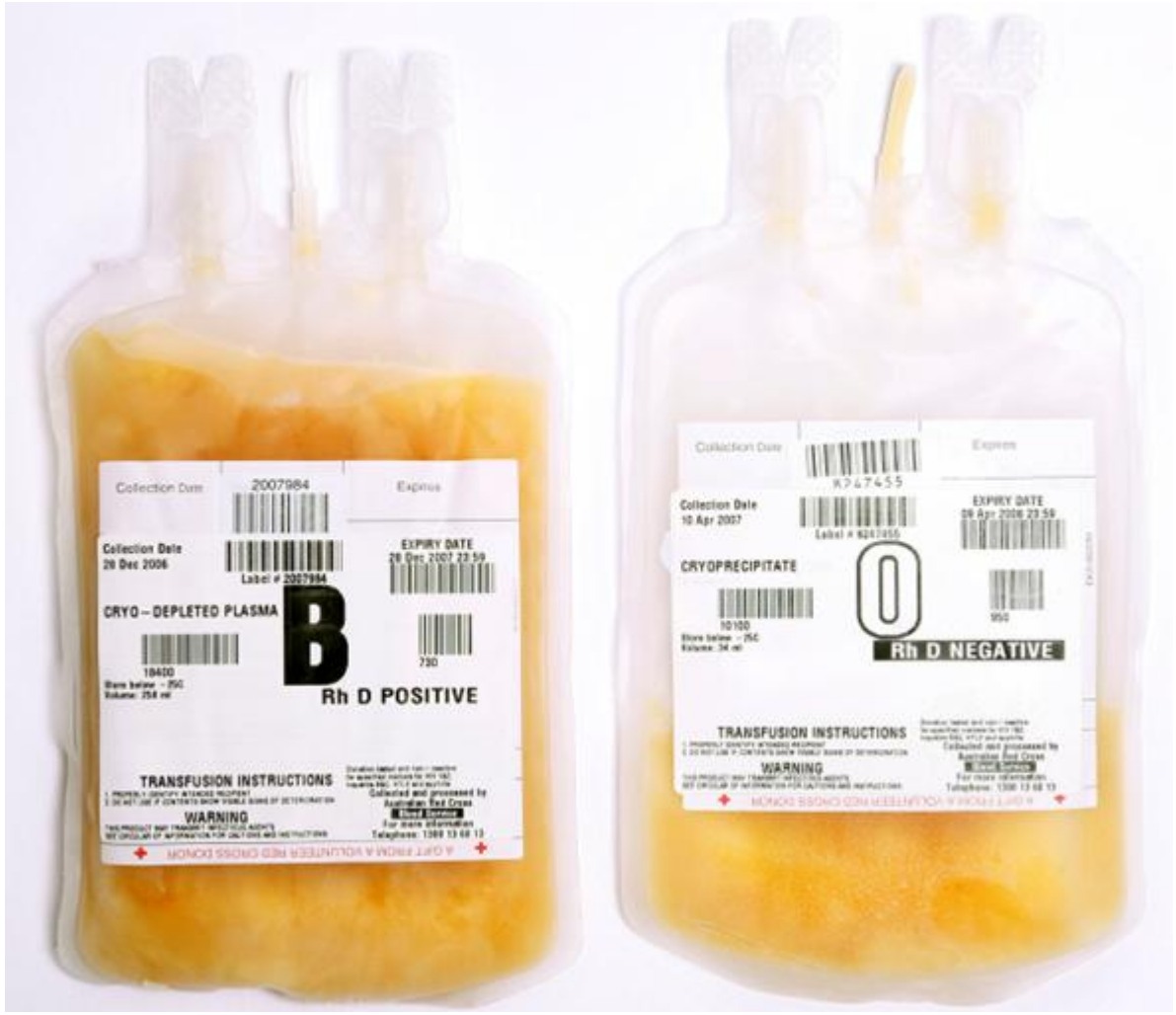
2 Manco-Johnson M.C. et al N Engl J Med.2007;357:535-44

3 Manco-Johnson M.C. et al J Throm Haemost.2013;11:460-6

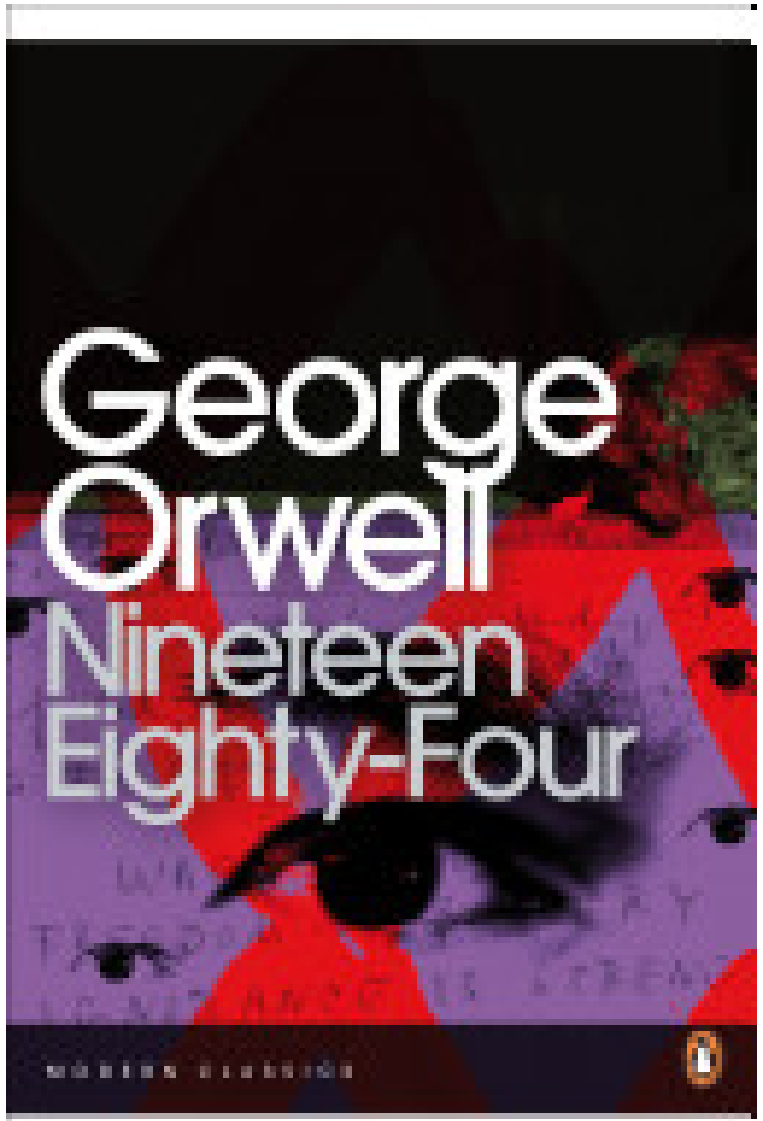
4 Tagliaferri A et al Thromb Haemost.2015;114

Australasian product development

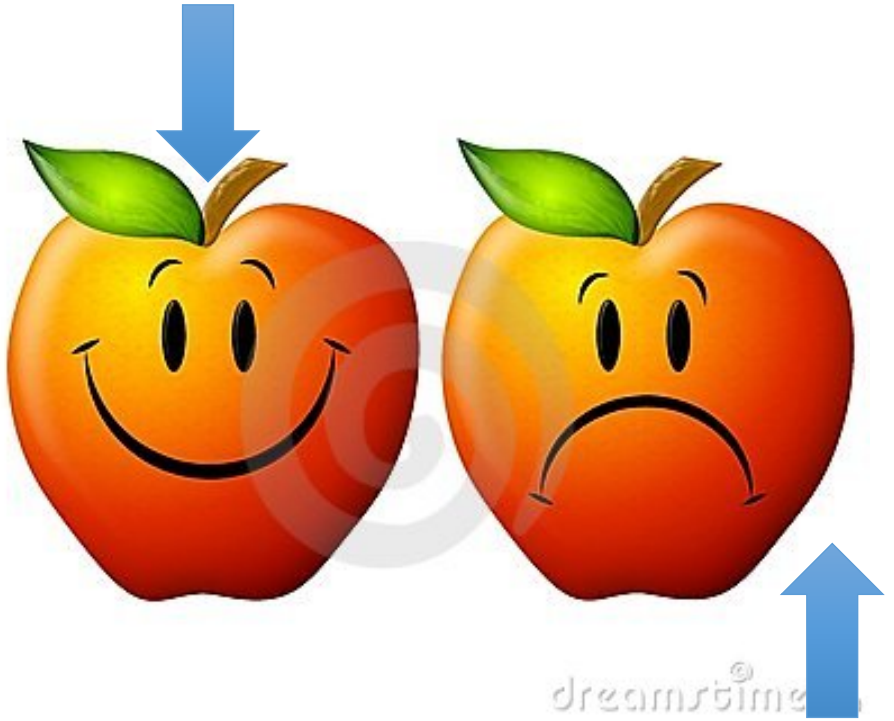
- 1950s Fresh blood transfusion including direct blood transfusion
- 1960s Local Red Cross Blood Services provided cryoprecipitate (FVIII) and fresh frozen plasma derivatives (FIX)
- 1970s Limited introduction of non virally inactivated FVIII and FIX concentrates



And then the huge catastrophe

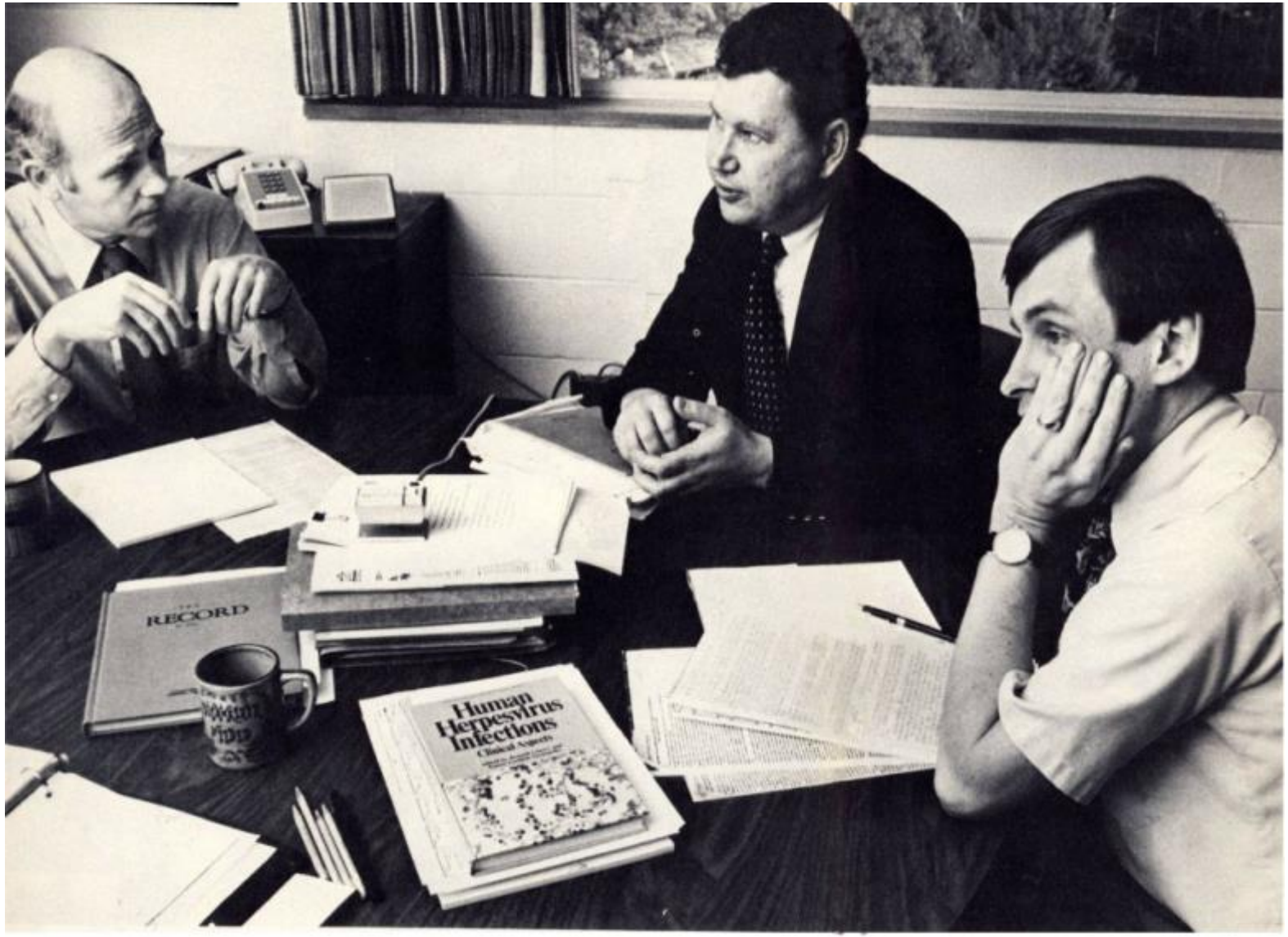


F VIII CLONED



HIV INFECTION

Bruce Evatt



Journal of Thrombosis and Haemostasis, 4: 2295–2301

HISTORICAL SKETCH

The tragic history of AIDS in the hemophilia population, 1982–1984

B. L. EVATT
Vice President Programs, World Federation of Hemophilia, and Director (Retired), Division of Hematology, Centers for Disease Control, Atlanta, GA, USA

To cite this article: Evatt BL. The tragic history of AIDS in the hemophilia population, 1982–1984. J Thromb Haemost 2006; 4: 2295–301.

Introduction: building the paradigm of hemophilia care

During the first four decades of the 20th century, life for patients with hemophilia was at best miserable. Usually disabled before the age of 20, life expectancy for these patients averaged 27 years because of early deaths, often from bleeding into vital organs [1]. Because of the improvements in transfusion technology made during World War II [2–3], hemophilic patients could receive infusions of fresh whole blood or fresh frozen plasma containing the missing clotting factor. As a result, the life expectancy for a severe hemophilic patient reached 39.7 years by 1960, but the crippling effects of repeated bleeds left a substantial proportion of the population disabled and unemployed. Development of cryoprecipitate and subsequent fractionation procedures in the 1960s allowed storage of a therapeutic form of clotting factor VIII (FVIII), the missing clotting factor in hemophilia A [4–6]. Commercial adaptation yielded lyophilized clotting factor concentrates that immediately raised the missing clotting factor to normal levels, could be carried with patients on trips and could be self-administered.

Both patients and physicians regarded clotting factor concentrates as the ultimate solution to hemophilia. Home care programs grew and comprehensive hemophilia treatment centres (HTC) developed [7–9]. Patients attending HTCs experienced substantial improvement of medical care and better quality of life as dependency on the medical community decreased. Mortality rates fell dramatically, employment levels increased, and school and work absences diminished greatly as hospitalizations and complications of hemophilia decreased [10–11]. Life expectancy reached 60 by 1980, nearly that of normal males.

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Consequently, plasma demand rose significantly, and the need for volume rather than quality drove the plasma industry. Plasma was often obtained from paid donors who had high risks of blood-borne diseases (those who were extremely poor, prisoners, alcoholics, etc.) [2]. As a result, clotting factor concentrates, derived from pools of up to 20 000 donors with inadequate donor screening and infective agent testing, almost uniformly infected patients with hepatitis [12–13]. Considering the enhanced quality of life and increased longevity, these high infectivity rates were deemed an acceptable risk by patients, physicians, industry and government; viral inactivation technology was not vigorously pursued.

The epidemic begins

It was in this setting that a new blood-borne disease, acquired immune deficiency syndrome (AIDS), was spawned in Africa and transmitted by social and sexual intercourse of populations at high risk for blood-borne disease into the Caribbean, the USA and other countries of the developed world.

First apparent in the homosexual population in the USA in the last quarter of 1980, the disease possessed unusual properties that initially obscured it as a distinct infectious disease. Previously healthy victims had no specific symptoms but presented with either secondary infections or tumors associated with immune deficiency [i.e. *Pneumocystis carinii* pneumonia (PCP) or Kaposi's sarcoma] [14,15]. A long incubation time made it difficult to identify person-to-person spread. Laboratory methods needed to culture and identify the etiologic agent were lacking. Leading scientists focused on non-infectious causes, such as antibodies to sperm or reaction of the immune system to chemicals such as inhaled amyl nitrites that homosexuals used to maintain prolonged erections [16,17].

The course of the investigation began to change in 1982. The Centers for Disease Control (CDC), the federal agency responsible for investigating new infectious diseases, had just experienced a major reorganization and severe budgetary and staff reductions. The author directed the Division of Host Factors, which was responsible for investigation of new drugs, one of which was pentamidine, the drug used to treat

Bruce waited 20 years to publish this

The culture of fear

Variable HIV Infection rates

AUSTRALIAN HEMOPHILIAC RECIPIENTS OF VOLUNTARY DONOR BLOOD PRODUCTS LONGITUDINALLY EVALUATED FOR AIDS. A CLINICAL AND LABORATORY STUDY, 1983-1986

R. J. GARSIA
Research Fellow, Clinical Immunology Research Centre, University of Sydney, Honorary Registrar, Royal Prince Alfred Hospital, NSW

P. A. GATENBY
Staff Specialist, Department of Immunology, Royal Prince Alfred Hospital, NSW

A. BASTEN
Professor of Immunology and Director, Clinical Immunology Research Centre, University of Sydney, NSW

D. F. KENNY
Senior Scientific Officer, Clinical Immunology Laboratory, Royal Prince Alfred Hospital, NSW

K. J. GALLAGHER
Scientific Officer, Clinical Immunology Laboratory, Royal Prince Alfred Hospital, NSW

K. A. RICKARD
Director, Haemophilia Treatment Centre, Department of Haematology, Royal Prince Alfred Hospital, NSW

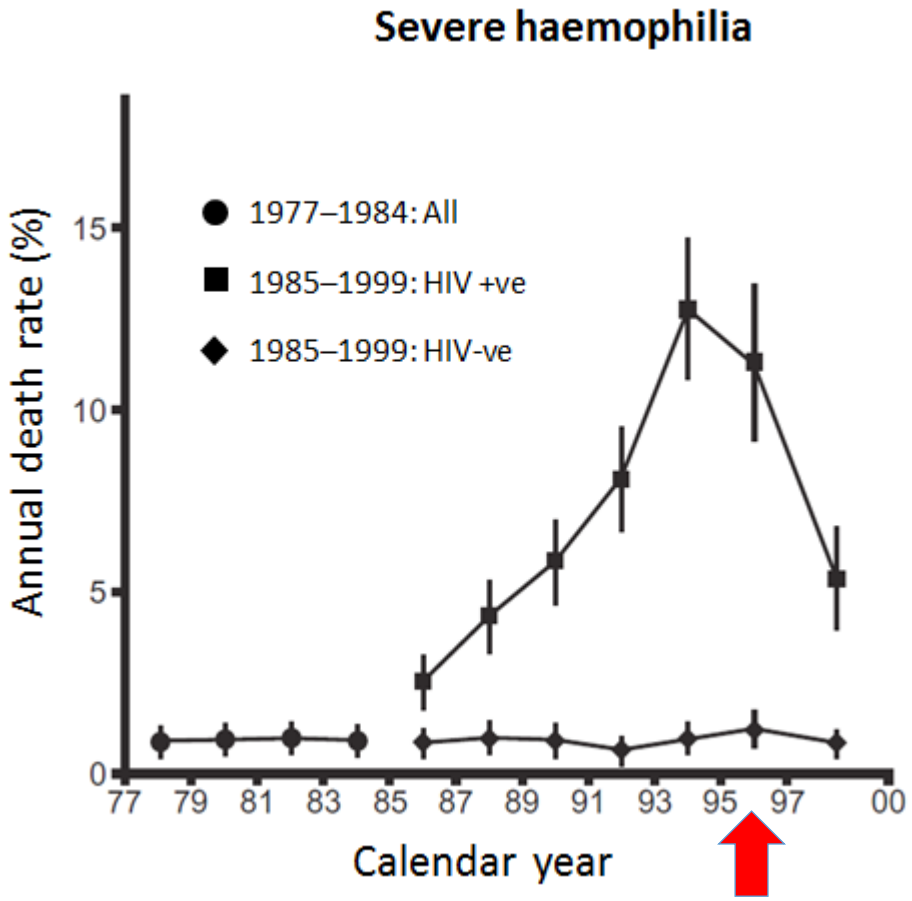
I. D. GUST
Professor of Virology, Fairfield Hospital for Infectious Diseases, Fairfield, Vic.

W. MASKILL
Scientist, National HTLV-III Reference Laboratory, Fairfield, Vic.

Overall rate in infused patients 35% up to 85% in severe FVIII deficiency

Garsia et al Aust NZ J Med 1987;17 371-374

Impact of HIV on mortality rates in the UK haemophilia population



1246 patients infected 1979 - 86

Highly Active Antiretroviral treatment introduced 1996

Reductions in mortality occurred with effective anti-HIV treatment

Darby SC et al. *AIDS* 2004;18:525–33

Not dead yet

Colleagues in adversity

And the scientists responded

- 1980s Introduction of virally inactivated concentrates (low yield)

Risks of HIV/HBV/HCV eliminated

- 1990s Limited introduction of recombinant products

- 2000s Recombinants rule

The era of supply constraint was over

Michael factors in blood breakthrough

By GABRIELLA COSLOVICH

FOR a long time, the simple joys of youth — shooting goals, taking screamers or hitting the ball for a six — were out of bounds for Michael Stewart.

Each time the 14-year-old haemophiliac joined his school mates in a game of basketball, cricket or footy he risked injuring his muscles and joints and suffering from internal bleeding.

Only a dose of an anti-clotting agent, the plasma-derived Factor VIII, could stem the haemorrhaging.

More than 2000 blood donations are needed for just one batch of Factor VIII, the clotting agent usually missing in haemophiliacs, and severe shortages of the product are common.

But since January, the availability of a new synthetic alternative has given Michael a new lease of life.

Rather than having to wait for an injury to occur before receiving Factor VIII, the constant supply of the synthetic Recombinant Factor VIII has allowed him to be placed on a preventive program.

Three times a week, Michael injects himself with the genetically engineered, non-human product to ensure the amount of Factor VIII in his blood stays at a level high enough to protect him from bleeding into joints and muscles.

Michael is now looking forward to joining the school basketball team.

"It's a miraculous leap forward," Dr Alison Street, the head of the Alfred Hospital's haemotology unit, said yesterday.

"When Michael was not on this program, he had to treat himself after a bleed once or twice a week, but since he started on the program three months ago, he has only had one or two bleeds," Dr Street said.

The preventive treatment of haemophiliac children helps them avoid the crippling joint and muscle damage that occurs as a result of bleeding.

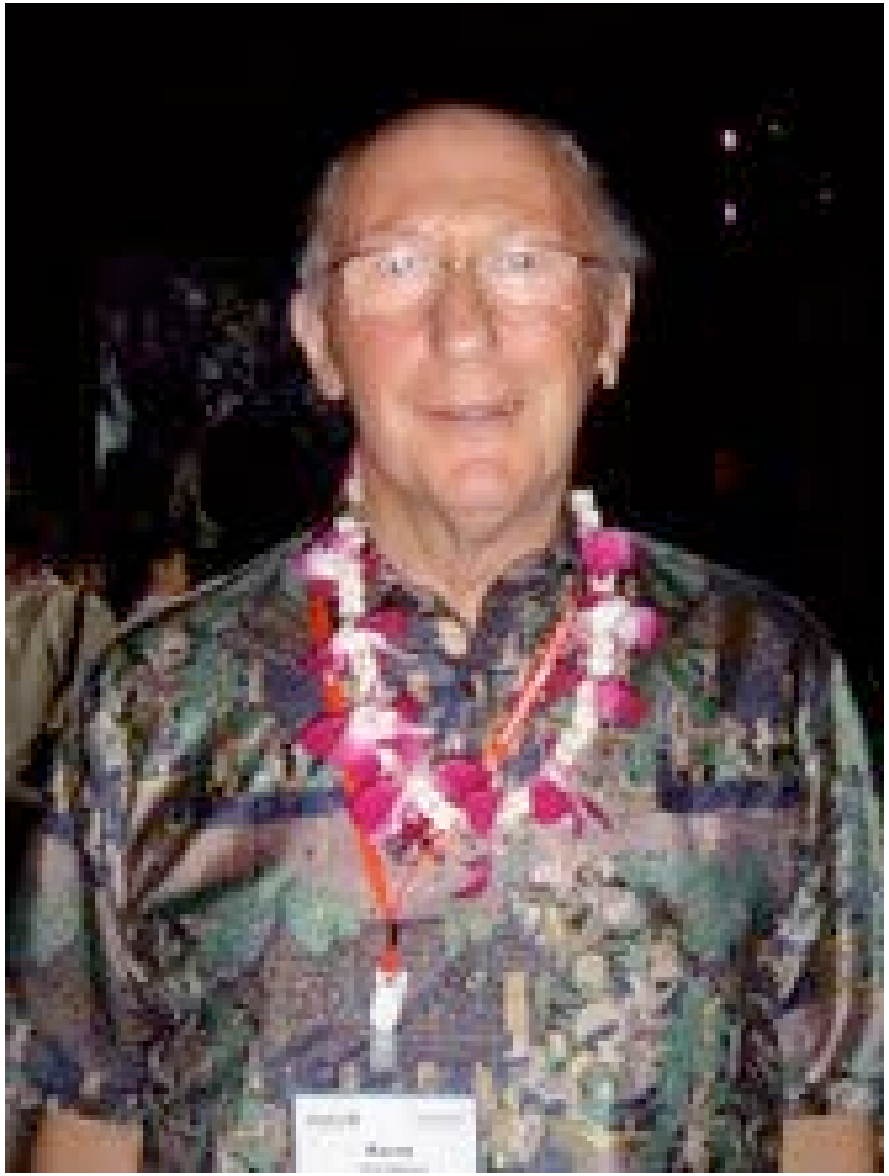
And because Recombinant Factor VIII is a non-human product, the chances of acquiring viruses such as HIV or hepatitis C from it are minimal.

Michael is among the first patients in Australia to have access to the blood-clotting agent, which has been bought from the United States with State and Commonwealth Government funds.

New life: *Michael Stewart, 14, with Dr Alison Street.*

THE AUSTRALIAN
FEBRUARY 1996

Kevin Rickard



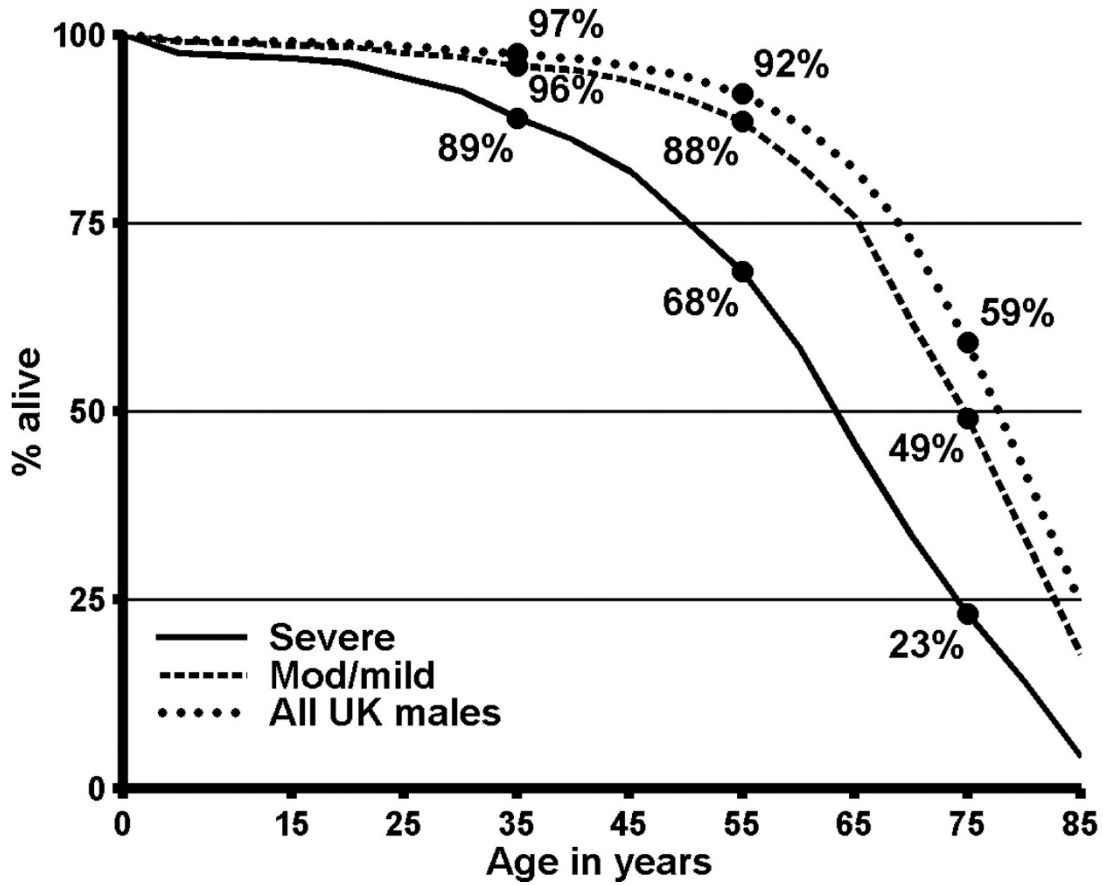
Henry Ekert

What happened next ?

The future brightened for people with bleeding disorders with **continuing challenges:**

- That current therapies still induce **inhibitors**…..
- To manage **increasing consumption** of FVIII/IX with need for accountability and affordability
- To improve clinical/cost effectiveness using **individualised dosing.**
- **To recognise increasing longevity** of people with haemophilia and the morbidity of the **HCV epidemic** (up to 1992.)
- Recognition of **other bleeding disorders** and the identification of **women who bleed**

Beyond the era of viral infection, men with haemophilia are approaching a normal life expectancy



Darby S C et al. Blood 2007;110:815-825

Back to comprehensive care

Memoranda/Mémorandums

Prevention and control of haemophilia: Memorandum from a joint WHO/WFH meeting*

Haemophilia, the commonest hereditary bleeding disorder, arises because of the absence of, decrease in, or deficient functioning of plasma coagulation factor VIII or factor IX. With rare exceptions, exclusively males are affected. This Memorandum summarizes the discussions and recommendations for the prevention and control of haemophilia made by participants at a joint WHO/World Federation of Haemophilia Meeting, held in Geneva on 26–28 March 1990.

Professor Pier Mannucci and Professor Carol Kasper

A model for chronic disease management

Delivery of haemophilia treatment

Optimum delivery of haemophilia care is based on a comprehensive interdisciplinary approach (distributed from specialized centres) using supervised self-treatment. Comprehensive centralized care enables the patients to receive care from knowledgeable experts in an efficient manner, and allows new therapies to be instituted quickly.

Report from WHO-WFH 1992

Functions of a comprehensive care centre

To provide and co-ordinate inpatient and outpatient care

To support home treatment where possible

To educate patients, families and their communities about bleeding disorders

To collect data and conduct audit and research to improve care

WHO-WFH 1992

A current definition of comprehensive care

Comprehensive haemophilia care has been defined as the continuing supervision of all medical and psychosocial factors affecting the person with haemophilia. Services offered by haemophilia treatment centres (HTCs) adopting the comprehensive care model include establishing prophylaxis and other treatment protocols, development of psychosocial, education and research programme, maintenance of a patient registry, genetic and reference diagnostic services and orchestration and management of a wide variety of multidisciplinary interventions. Most centres practising this model of care are based in developed countries and can meet costs for plentiful treatment products through government or insurance-company funding

Bruce Evatt 2001

Into the twenty first century

Delivery of Treatment for Haemophilia

Report of a Joint WHO/WFH/ISTH Meeting
London, United Kingdom, 11 - 13 February 2002

Professor Christine Lee

Emphasis on registry, audit and research

It is recommended that home treatment is the treatment of choice for patients with severe haemophilia

It is recommended that **all treatment be dispensed from a haemophilia centre that is integrated into the existing healthcare system.**

The diagnosis should be made and the patient should be **listed on a registry**

There should be a **protocol for dosing and follow up and this information should be entered on the registry together with clinical details of progress**

It is recommended that **regular audit and research and development should be conducted in order to establish optimal treatment guidelines, which are quality assessed and drive practice improvement**

Haemophilia (2008), **14**, 361–374 DOI: 10.1111/j.1365-2516.2007.01625.x

SPECIAL ARTICLE
European Association for Haemophilia and associated disorders (EHAD)

European principles of haemophilia care

B. T. COLVIN,* J. ASTERMARK,† K. FISCHER,‡ A. GRINGERI,§ R. LASSILA,¶ W. SCHRAMM,** A. THOMAS†† and J. INGERSLEV‡‡ FOR THE INTER DISCIPLINARY WORKING GROUP

**Barts and The London – Queen Mary's School of Medicine & Dentistry, London, UK; †Department for Haematology and Coagulation Disorders, Malmö University Hospital, Malmö, Sweden; ‡Van Creveld KLINIEK, Department of Haematology, UMC Utrecht, The Netherlands; §Centro Emofilia A. Bianchi Bonomi, Milan, Italy; ¶Department of Haematology and Coagulation Disorders, Helsinki University Hospital, Helsinki, Finland; **Abt. Hämostaseologie u. Transfusionswesen, Klinikum der Universität München, München, Germany; ††Paediatric Department, Royal Hospital for Sick Children, Edinburgh, UK; and ‡‡Centre for Haemophilia and Thrombosis, Skejby University Hospital, Aarhus, Denmark*

Summary. As the management of haemophilia is complex, it is essential that those with the disorder should have ready access to a range of services provided by a multidisciplinary team of specialists. This document sets out the principles of comprehensive haemophilia care in Europe. Within each country there should be a national organization which oversees the provision of specialist Comprehensive Care Centres that provide the entire spectrum of clinical and laboratory services. Depending upon the size and geographical distribution of the population, a network of smaller haemophilia centres may also be necessary. There should be arrangements for the supply of safe clotting factor concentrates which can also be used in home treatment and prophylaxis programmes. A national register of patients is recommended along with collection of treatment statistics. As comprehensive haemophilia care is multidisciplinary by nature, the need for education and research programmes for all staff members is emphasized: Members of the Interdisciplinary Working Group not represented in the list of authors are mentioned in Section 4 of this document.

Keywords: haemophilia, haemophilia care, management, principles, treatment

Everyone bleeds differently

Age at 1st joint bleed

Differences in physical activities

M.Carcao

2-3 fold Differences in Pharmacokinetics

Biological differences (FVL; FII mutation)

Individualised Treatment

- Driven by patients experience of bleeding, informed by patient/product pharmacokinetic studies and delivered by a team
- Dependent on physical activity, age, adherence to product infusion schedule
- Analysable by patient and treaters from treatment diary only with the commitment of both
- To achieve optimised physical and social outcomes with routine prophylactic treatment

Multidisciplinary team model

Hematologist

Orthopedists

Physical Therapists

Patient and product

Laboratory Technicians

Nurses

Social Workers

Dentists

Other clinical, data and financial expertise also required

WFH 2012 WORLD CONGRESS
PARIS, FRANCE
Sunday, July 8 - Thursday, July 12

NATIONAL BLOOD AUTHORITY
AUSTRALIA

Annual report 2013-4

www.blood.gov.au


Success Story
ABDR

Persons registered by condition 2013-4

• Haemophilia A	2238
• Haemophilia B	539
• Von Willebrand Disorder	1947
• Other	786
• **TOTAL**	**5510**

5713 as of Sept 2015

NBA ABDR report 2013-4
http://www.blood.gov.au/data-analysis-reporting

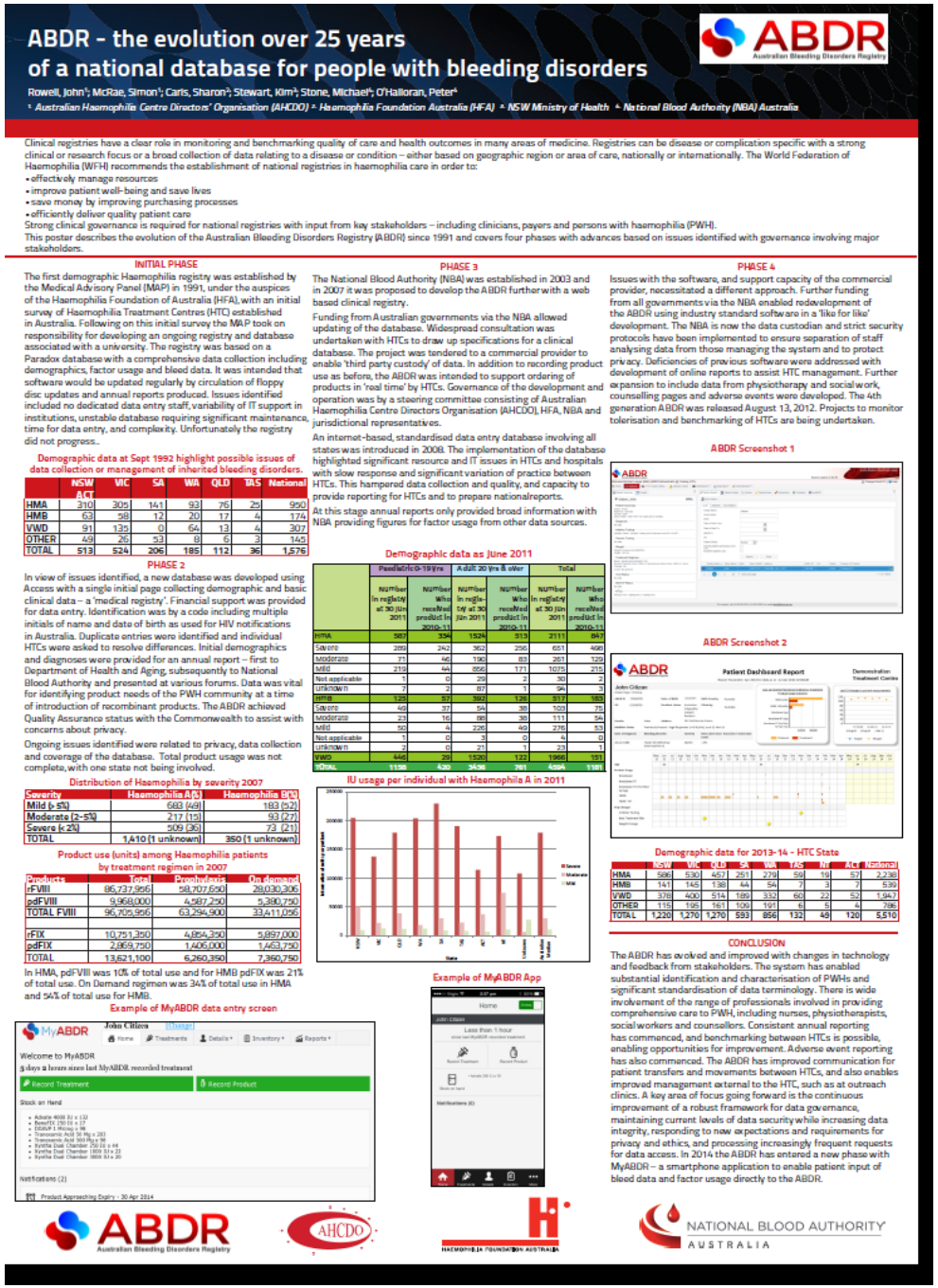
Diagnosis	Number in ABDR Registry*					Number who Received Product 2013-4				
	2009-10	2010-11	2011-2	2012-3	2013-4	2009-10	2010-11	2011-12	2012-3	2013-4
HMA†	2,116	2,217	2,316	2,391	**2,181**	833	880	895	983	**964**
HMB†	501	527	544	564	**530**	183	191	189	205	**204**
Other‡	156	165	214	144	151	<5	<5	7	<5	10
Other Factor Deficiency	277	306	326	306	**318**	<5	26	35	35	**43**
Platelet Disorder	179	204	224	222	233	<5	9	<5	14	15
Vascular	8	9	9	7	9	-	-	-	-	
Fibrinogen				36	40	-	-	-	8	6
VWD	1,815	1,940	2,068	2,127	**1,912**	190	161	169	215	**242**
Unclassified				10	11	-	-	-	5	<5

Products Issued 2013-4

	Mild	Moderate	Severe	Unknown*	Total**
HMA (IU FVIII Products)†	5,300,750	16,102,250	125,521,950	10,000	**146,934,950**
On Demand	3,741,750	6,091,750	21,586,500	1,500	31,421,500
Prophylaxis	707,250	9,752,000	94,752,950		105,212,200
ITT - Tolerisation	76,000		7,915,250		7,991,250
Unknown*	775,750	258,500	1,267,250	8,500	2,310,000
HMB (IU FIX Products)‡	2,972,000	7,573,750	19,434,500	0	**29,980,250**
On Demand	2,351,000	3,096,750	4,031,000		9,478,750
Prophylaxis	471,000	4,178,000	12,403,500		17,052,500
Tolerisation			2,978,000		2,978,000
Unknown	150,000	299,000	22,000		471,000

Products Issued 2013-4

VWD (IU FVIII Product) ++	644,250	541,000	4,377,750	451,503	6,014,503
On Demand	367,750	483,000	1,653,000	379,003	**2,882,753**
Prophylaxis	128,000	10,000	2,623,750	35,500	**2,797,250**
Unknown*	148,500	48,000	101,000	37,000	**334,500**



ABDR - the evolution over 25 years of a national database for people with bleeding disorders

Rowell, John[1]; McRae, Simon[1]; Carls, Sharon[2]; Stewart, Kim[3]; Stone, Michael[4]; O'Halloran, Peter[4]
[1] Australian Haemophilia Centre Directors' Organisation (AHCDO) [2] Haemophilia Foundation Australia (HFA) [3] NSW Ministry of Health [4] National Blood Authority (NBA) Australia

Clinical registries have a clear role in monitoring and benchmarking quality of care and health outcomes in many areas of medicine. Registries can be disease or complication specific with a strong clinical or research focus or a broad collection of data relating to a disease or condition – either based on geographic region or area of care, nationally or internationally. The World Federation of Haemophilia (WFH) recommends the establishment of national registries in haemophilia care in order to:

- effectively manage resources
- improve patient well-being and save lives
- save money by improving purchasing processes
- efficiently deliver quality patient care

Strong clinical governance is required for national registries with input from key stakeholders – including clinicians, payers and persons with haemophilia (PWH).
This poster describes the evolution of the Australian Bleeding Disorders Registry (ABDR) since 1991 and covers four phases with advances based on issues identified with governance involving major stakeholders.

INITIAL PHASE

The first demographic Haemophilia registry was established by the Medical Advisory Panel (MAP) in 1991, under the auspices of the Haemophilia Foundation of Australia (HFA), with an initial survey of Haemophilia Treatment Centres (HTC) established in Australia. Following on this initial survey the MAP took on responsibility for developing an ongoing registry and database associated with a university. The registry was based on a Paradox database with a comprehensive data collection including demographics, factor usage and bleed data. It was intended that software would be updated regularly by circulation of floppy disc updates and annual reports produced. Issues identified included no dedicated data entry staff, variability of IT support in institutions, unstable database requiring significant maintenance, time for data entry, and complexity. Unfortunately the registry did not progress..

Demographic data at Sept 1992 highlight possible issues of data collection or management of inherited bleeding disorders.

	NSW ACT	VIC	SA	WA	QLD	TAS	National
HMA	310	305	141	93	75	26	950
HMB	63	58	12	20	17	4	174
VWD	91	135	0	64	13	4	307
OTHER	49	26	53	8	6	3	145
TOTAL	513	524	206	185	112	36	1,576

PHASE 2

In view of issues identified, a new database was developed using Access with a single initial page collecting demographic and basic clinical data – a 'medical registry'. Financial support was provided for data entry. Identification was by a code including multiple initials of name and data of birth as used for HIV notifications in Australia. Duplicate entries were identified and individual HTCs were asked to resolve differences. Initial demographics and diagnoses were provided for an annual report – first to Department of Health and Aging, subsequently to National Blood Authority and presented at various forums. Data was vital for identifying product needs of the PWH community at a time of introduction of recombinant products. The ABDR achieved Quality Assurance status with the Commonwealth to assist with concerns about privacy.

Ongoing issues identified were related to privacy, data collection and coverage of the database. Total product usage was not complete, with one state not being involved.

Distribution of Haemophilia by severity 2007

Severity	Haemophilia A(1)	Haemophilia B(2)
Mild (> 5%)	683 (49)	183 (52)
Moderate (2–5%)	217 (15)	93 (27)
Severe (< 2%)	509 (36)	73 (21)
TOTAL	1,410 (1 unknown)	350 (1 unknown)

Product use (units) among Haemophilia patients by treatment regimen in 2007

Products	Total	Prophylaxis	On demand
rFVIII	86,737,956	58,707,650	28,030,306
pdFVIII	9,968,000	4,587,250	5,380,750
TOTAL FVIII	96,705,956	63,294,900	33,411,056
rFIX	10,751,250	4,854,350	5,897,000
pdFIX	2,869,750	1,406,000	1,463,750
TOTAL	13,621,100	6,260,350	7,360,750

In HMA, pdFVIII was 10% of total use and for HMB pdFIX was 21% of total use. On Demand regimen was 34% of total use in HMA and 54% of total use for HMB.

Example of MyABDR data entry screen

PHASE 3

The National Blood Authority (NBA) was established in 2003 and in 2007 it was proposed to develop the ABDR further with a web based clinical registry.

Funding from Australian governments via the NBA allowed updating of the database. Widespread consultation was undertaken with HTCs to draw up specifications for a clinical database. The project was tendered to a commercial provider to enable 'third party custody' of data. In addition to recording product use as before, the ABDR was intended to support ordering of products in 'real time' by HTCs. Governance of the development and operation was by a steering committee consisting of Australian Haemophilia Centre Directors Organisation (AHCDO), HFA, NBA and jurisdictional representatives.

An internet-based, standardised data entry database involving all states was introduced in 2008. The implementation of the database highlighted significant resource and IT issues in HTCs and hospitals with slow response and significant variation of practice between HTCs. This hampered data collection and quality, and capacity to provide reporting for HTCs and to prepare nationalreports.

At this stage annual reports only provided broad information with NBA providing figures for factor usage from other data sources.

Demographic data as June 2011

	Paediatric 0-19 Yrs		Adult 20 Yrs & older		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	354	1524	513	2111	867
Severe	289	242	362	256	651	498
Moderate	71	46	190	83	261	129
Mild	219	66	956	171	1075	215
Not applicable	1	0	29	2	30	2
unknown	7	2	87	1	94	3
HMB	125	57	382	126	317	183
Severe	46	37	54	38	100	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

IU usage per individual with Haemophilia A in 2011

Example of MyABDR App

PHASE 4

Issues with the software, and support capacity of the commercial provider, necessitated a different approach. Further funding from all governments via the NBA enabled redevelopment of the ABDR using industry standard software in a 'like for like' development. The NBA is now the data custodian and strict security protocols have been implemented to ensure separation of staff analysing data from those managing the system and to protect privacy. Deficiencies of previous software were addressed with development of online reports to assist HTC management. Further expansion to include data from physiotherapy and social work, counselling pages and adverse events were developed. The 4th generation ABDR was released August 13, 2012. Projects to monitor tolerisation and benchmarking of HTCs are being undertaken.

ABDR Screenshot 1

ABDR Screenshot 2

Demographic data for 2013-14 - HTC State

	NSW	VIC	QLD	SA	WA	TAS	NT	ACT	National
HMA	586	530	457	251	279	59	19	57	2,238
HMB	141	145	138	44	54	7	3	7	539
VWD	378	400	514	189	332	60	22	52	1,947
OTHER	115	195	161	109	191	6	5	4	786
TOTAL	1,220	1,270	1,270	593	856	132	48	120	5,510

CONCLUSION

The ABDR has evolved and improved with changes in technology and feedback from stakeholders. The system has enabled substantial identification and characterisation of PWHs and significant standardisation of data terminology. There is wide involvement of the range of professionals involved in providing comprehensive care to PWH, including nurses, physiotherapists, social workers and counsellors. Consistent annual reporting has commenced, and benchmarking between HTCs is possible, enabling opportunities for improvement. Adverse event reporting has also commenced. The ABDR has improved communication for patient transfers and movements between HTCs, and also enables improved management external to the HTC, such as at outreach clinics. A key area of focus going forward is the continuous improvement of a robust framework for data governance, maintaining current levels of data security while increasing data integrity, responding to new expectations and requirements for privacy and ethics, and processing increasingly frequent requests for data access. In 2014 the ABDR has entered a new phase with MyABDR – a smartphone application to enable patient input of bleed data and factor usage directly to the ABDR.

This poster describes the history of the national database, illustrating how the information is aggregated. The next slide shows how such information may be further analysed as a benchmarking activity

Further such studies are planned for when more clinical data is reported and issues of research governance are resolved

Comparative Use of Factor VIII & FIX Concentrates in Children with Severe Haemaophilia A & B in Two Paediatric Haemophilia Centres. A Model for Benchmarking Haemophilia Centres?

SA Brown[1], C Barnes[2], L Mason[1], J Ekert[2], J McCosker[1].

[1]Haemophilia Centre, Royal Children's Hospital, Brisbane, Australia; [2]Haemophilia Centre, Royal Children's Hospital, Melbourne, Australia .

INTRODUCTION

In Australia there are 2860 individuals with haemophilia A or B of which 826 have severe haemophilia (ABDR Annual report 2011-12). The care of these individuals is predominantly undertaken by staff at Haemophilia Treatment Centres (HTCs). Within Australia there are 20 HTCs providing comprehensive care for individuals with inherited bleeding disorders. The management of haemophilia can be complex and expensive due to the cost of clotting factor concentrates; during 2011-12 administration of prophylaxis to individuals with severe haemophilia A & B consumed >87 million units of FVIII & FIX concentrate (ABDR Annual report 2011-12). In view of the rarity of haemophilia, the complexity of it's management and cost of clotting factor concentrates consumed there is growing interest in assessment of the quality and effectiveness of the care provided for individuals with haemophilia. A review of the recently published World Federation of Hemophilia Treatment Guidelines (2nd Edition, 2013) show that guidelines for the management of haemophilia are frequently based on low levels of evidence, with few randomised controlled studies informing clinical practice in this area.

An alternative approach to improving practice is through *Benchmarking*. Benchmarking aims to improve practice/performance through comparing "yourself"to and learning from other centres of excellence. As such benchmarking in healthcare needs to be multidimensional to assess not only the financial outcomes measured by business, but also patient outcomes as well as the safety and quality of care.

The Australian Bleeding Disorders Registry (ABDR) is a clinical registry used by all HTCs to record information on the management of patients with inherited bleeding disorders. Therefore, the ABDR has the potential to act as a source of data that could be utilised to benchmark the HTCs across Australia.

The aim of this pilot study was to investigate the feasibility of comparing clotting factor concentrate use between two Paediatric HTCs as one domain in a possible process of benchmarking practice across HTCs.

METHOD

Data for factor VIII & IX concentrate usage (in units) for all children (n=114; 14% of all individuals with severe haemophilia in Australia) with severe haemophilia A (91, 80%) and severe haemophilia B (23, 20%) was collected for an 8 month period from data entered into the ABDR or Centre records. The body weight (in kg) recorded for each child in the ABDR was obtained from the corresponding time period. Individual treatment regimens as recorded in the ABDR were used to calculate the predicted use of factor VIII or IX usage for the same 8 month period. The data was then used to calculate the actual and predicted usage of factor VIII & IX concentrate in units/kg/month. Descriptive statistics were used to compare the actual factor VIII & IX concentrate usage by a Box and Whisker plot (Figure 1). The difference in the actual factor concentrate usage as compared to the prescribed treatment regimen was expressed as a percentage of the predicted factor concentrate use.

RESULTS

The 119 individuals whose data was analysed were included as they had 8 months of data on clotting factor concentrate that could be analysed. Individuals excluded from the analysis included those: receiving on demand therapy, who commenced prophylaxis within the 8 month period, who moved to one of the two HTCs during the 8 month period (and so had incomplete data on factor concentrate usage), and those individuals receiving concentrate as part of a phase 3 trial. At one HTC 9 individuals were excluded for these reasons. In addition, two individuals on immune tolerisation (ITI) were excluded from the analysis from the same HTC; the factor VIII concentrate consumption for the two individuals on ITI was 696.5 and 1686.1 U/kg/month.

Figure 1 shows the median and inter-quartile usage of factor concentrate for individuals with severe haemophilia A and B at the two centres; For centre 1 and centre 2 the median usage of factor VIII & IX concentrates were 388.6 and 342.7 U/kg/month & 187.8 and 203.6 U/kg/month, respectively. Review of the deviation of factor concentrate usage from the prescribed treatment regimen (Fig. 2) shows several individuals either use over 20% more or less than the prescribed regimen. Interestingly, the 8 of the 10 individuals with a usage of 600 U/kg/month or above were taking a dose as prescribed or lower than prescribed by their HTC. A 4 of these 10 individuals had previously undergone ITI, and increased factor concentrate consumption compared to the median usage post-ITI is in keeping with data from the UK (C Hay personal communication).

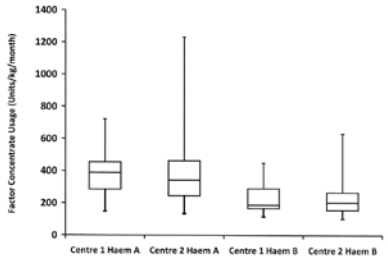


Figure 1 – Box and Whisker plot of factor VIII & FIX concentrate usage (U/kg/month) at two Paediatric HTCs for individulas with severe haemophilia A (Haem A) and severe haemophilia B (Haem B).

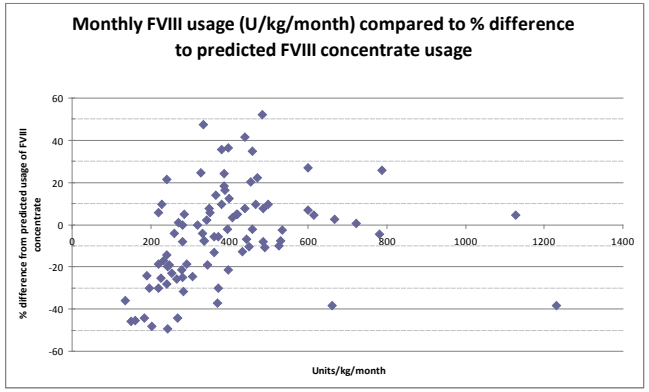


Figure 2 – Monthly factor VIII concentrate usage (U/kg/month) compared to percentage deviation of actual factor concentrate usage from prescribed treatment regimen. Combined data for 91 individuals with severe haemophilia A in 2 Paediatric HTCs.

DISCUSSION

Data collected by HTCs in the ABDR has been used to analyse and review factor VIII & factor IX concentrate usage in two Paediatric Haemophilia Centres. Review of this data at each individual HTC has allowed evaluation of the individual usage of factor concentrate against their predicted usage as determined by their treatment regimen. The extraction and analysis of this data was comparatively straightforward and the Data Managers at the two HTCs were able to obtain the data in a timely manner. As such this type of analysis appears to be amenable to be used by all HTCs to analyse factor concentrate usage and help inform clinical review of individual patients. Analysis of the actual usage against the predicted usage was important to identify those individuals who are utilising significantly more or less factor concentrate than expected. Review of these patients in clinic can focus on understanding the reasons for this deviation in factor usage and addressing issues that may relate to under or over utilisation and rationalisation of their treatment regimen. The data on % deviation from predicted use is important, as factor concentrate usage based purely on units/kg/month does not provide information of adequate granularity to inform the clinical review and discussions with the family.

In addition to the local review of concentrate usage, the data has allowed a comparison of factor usage between two centres as a proof of principle of a benchmarking exercise. The data from the two centres has shown comparable consumption based on the analysis of the usage as median value and inter-quartile values. It would be interesting to extend this benchmarking process to all paediatric Haemophilia Centres in Australia. The aim would be to identify if there is significant variation in factor concentrate usage between any HTCs and to use this as a starting point to investigate the reasons for the variation. However, it is clear that more work is required to make benchmarking meaningful with respect to the totality of care and in particular with respect to the outcomes of prophylaxis. Any meaningful benchmarking process will require collection of standardised data and outcomes, along with data on comparative staffing levels at each HTC. Ideally these standardised outcomes need to be clearly defined and routinely collected (ideally through the ABDR). It will be interesting to see if the funders of health care in Australia adopt the implementation of guidelines based mainly on poor levels of evidence or a robust benchmarking program.

Benchmarking activities in haemophilia

- Undertaken between centres, where collected data elements are sufficiently alike to be compared
- Are helpful to identify and address differences in practices and outcomes
- Assist accountability of resources
- Strengthen clinical-lay-government partnerships

Six years later and background for future exercices

These studies were performed in 2008-9

- before adoption of outcome assessment tools in most centres and
- with a less mature ABDR database than is now available

The paediatric study template was modified for the subsequent adult study

No stakeholders other than staff were interviewed.

The objective was to compare range of service delivery and human resource not physical, emotional or financial value, or quality of service

Adult benchmarking study

- HTCs for adult patients provided good core services.
- Similar to paediatric centres there were marked differences in staffing levels between centres.
- Many differences in policies occured between HTCs, related to local factors and need for different ways to achieve similar objectives.
- The different service provision practices describe alternate ways of achieving optimised patient care.

John Lloyd draft report 2009

Australian Paediatric Haemophilia Centres Benchmarking Study

- All centres provided core services considered essential for paediatric haemophilia care in access to product and home treatment
- Services fell short of international benchmarks related to infrastructure and personnel.
- Linkages to clinical outcome assessments and comparative product usage data would give opportunities to discuss resources with funders

Chris Barnes

nba.gov.au/abdr 2009

Haemophilia Foundation Australia recommends

HAEMOPHILIA FOUNDATION AUSTRALIA

- Haemophilia Treatment Centres (HTCs) which offer **comprehensive care for children and adults with bleeding disorders** in every Australian state and territory
- The **development and maintenance of national standards** for comprehensive care in HTCs
- Designation or Registration of HTCs in each jurisdiction; the designation process to include **ongoing compliance with HTC national standards**
- **Adequate funding** by jurisdictions to enable HTCs **to provide full comprehensive care services**

POLICY BRIEF MAY 2010

Haemophilia. 2013 Jan;19(1):35-43. doi: 10.1111/j.1365-2516.2012.02928.x. Epub 2012 Aug 29.

The European Principles of Haemophilia Care: a pilot investigation of adherence to the principles in Europe.

Fischer K[1], Hermans C; European Haemophilia Therapy Standardisation Board.

Author information

Abstract

In 2008 the 10 Principles of Haemophilia Care were outlined to provide a benchmark for haemophilia treatment. The EHTSB performed a survey to establish to what extent the Principles of Haemophilia Care were being applied throughout Europe. In total, 21 centres from 14 countries (France, UK, Germany, Switzerland, Sweden, Norway, the Netherlands, Belgium, Poland, Portugal, Slovakia, Spain, Greece and Italy), were surveyed. A central organization of haemophilia care (principle 1) was present in 79%, and a central patient registry in 57% (principle 2). National haemophilia care decision-making was performed by clinicians, ministries and patient organizations (principle 4). All had designated comprehensive care centres (CCC--principle 3), responsible the majority of severe patients, but in 36% some patients were treated outside CCC/haemophilia treatment centres (HTC)s. Clotting factor concentrates were available everywhere, without dosing restraints (principle 5), including recombinant products in 86% of countries. Prophylactic treatment was available for all children but not for all adults (principle 7). Immune tolerance was available in all countries (principle 9). Home treatment was supported and taught by all centres (principle 6). At centre level, 86% had 24-h laboratory facilities and all participated in education and research (principle 10). An experienced haematologist was available at all centres, a paediatrician in 47%, and prompt out of hours review was available in all (principle 8). The Principles of Haemophilia Care were generally applied throughout Europe. Some aspects of centralization, national organization of care, use of registries, formal paediatric care and prophylaxis for adults may be improved.

PMID: 22931302 [PubMed - indexed for MEDLINE]

Publication Types, MeSH Terms

LinkOut - more resources

It's all well and good to have Principles BUT what about Standards and Accountability?

Service Specifications for Haemophilia and Related Bleeding Disorders in New Zealand

Created: May 2012

Current version: July 2014

Appendix 4. National audit framework

Clinical

Treatment data, including number of patients:

- With severe haemophilia
- On home therapy
- On prophylaxis
- With inhibitors

On-call arrangements

Availability of support services, including paediatrics, orthopaedics, dental services
Arrangement for access to specialised HIV and Hepatitis care
Access to genetic services
Inspection of facilities
Review of staffing levels
Review of previous internal clinical audits
Arrangements for education and training
Clinical governance
Patient satisfaction

Laboratory

CPA accreditation
Review of NEQAS results
Review of internal audit
Availability of specialised coagulation assays during routine and emergency hours

Data handling

Coagulation factor concentrate stock control and handling
Annual returns to centralised database
Home therapy returns
Communication with other haemophilia centres and general practitioners

Protocols

Treatment delivery
Prophylaxis
Home therapy
On-call arrangements

Closing the audit loop

Issues arising from previous external audits
Publication and communication of results to interested parties

No national database or outcome measures available Oct 2015

HFNZ

“we don’t want treatment we want care”

What should we be able to measure and benchmark?

- Product accountability/Individualised care quality measures
  e.g. Breakthrough bleeding and Pharmacokinetic assessments
- Locally validated Musculo-skeletal and Psychosocial assessments
- Research Trial enrolments and publications
- Internal /External Audit compliance and performance

Further benchmarking and standardised internal and external audits, with wider stakeholder involvement, are needed to compare outcomes, for accountability and practice improvement

The resources required to perform, document, analyse and assure these activities are

- Agreed principles and validated assessment and audit tools
- Trained and sufficient clinicians
- Informed and engaged patients and community
- Supportive hospital administrators
- State and Federal Health managers

All committed to a research, quality and accountable culture

COMPENDIUM OF ASSESSMENT TOOLS

Hemophilia is a rare disease and its management is multi-faceted. With advances in medical care including prophylactic factor replacement, the need for outcome assessment tools that are valid, reliable, sensitive to change, and predictive has become increasingly apparent. Physical status (joint health), functional ability, bleeding symptoms, and quality of life can now be measured using standardized tools. Ultrasound and MRI scores are also under development. Many of these tools have been developed by working groups comprised of international experts, and have been tested in a variety of settings.

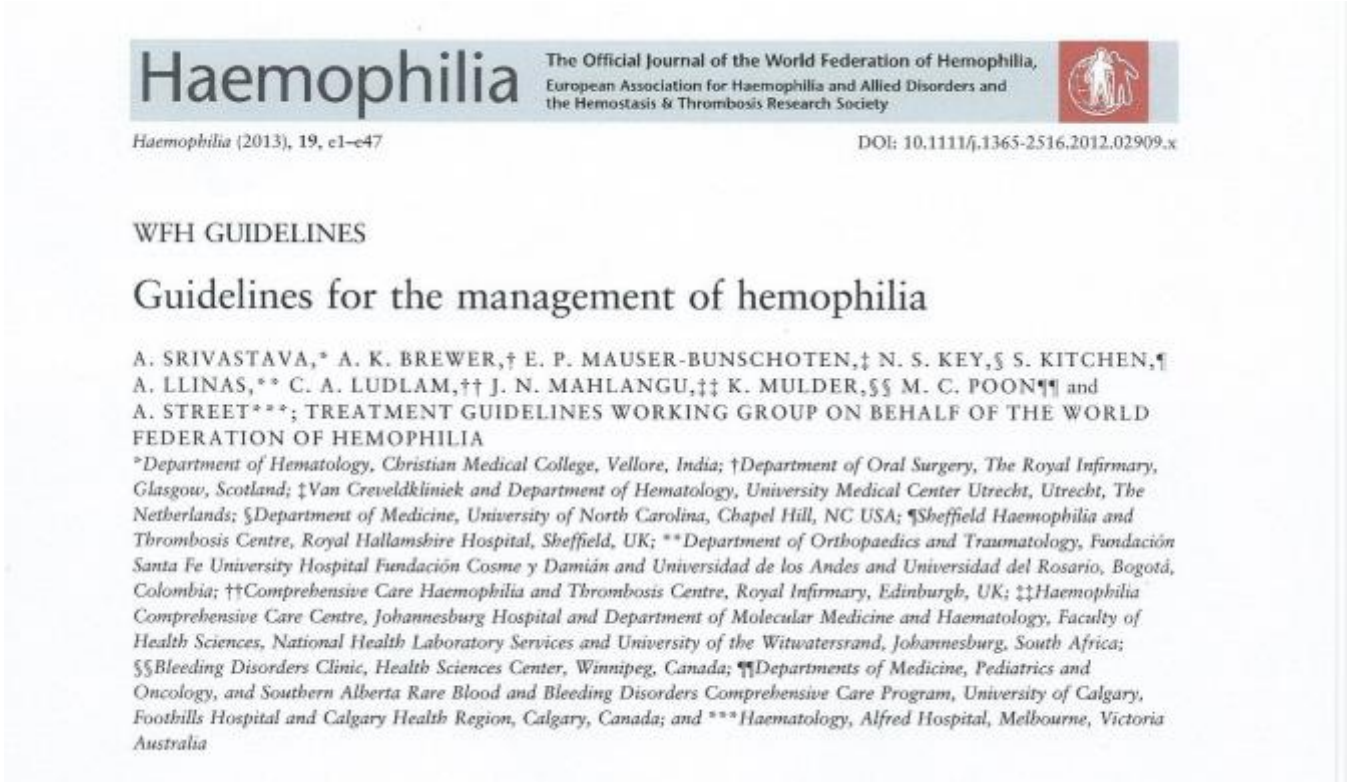
The purpose of this compendium is to provide hemophilia caregivers and researchers with an evaluation of new and existing assessment tools suitable for use in hemophilia. Adequate use of these tools will ultimately facilitate research and inform best practice.

Bleeding assessment tools

Functional and physical examination tools

www.wfh.org

Soon for adapting or adopting by AHCDO



Haemophilia
The Official Journal of the World Federation of Hemophilia,
European Association for Haemophilia and Allied Disorders and
the Hemostasis & Thrombosis Research Society

Haemophilia (2013), **19**, e1–e47 DOI: 10.1111/j.1365-2516.2012.02909.x

WFH GUIDELINES

Guidelines for the management of hemophilia

A. SRIVASTAVA,* A. K. BREWER,† E. P. MAUSER-BUNSCHOTEN,‡ N. S. KEY,§ S. KITCHEN,¶ A. LLINAS,** C. A. LUDLAM,†† J. N. MAHLANGU,‡‡ K. MULDER,§§ M. C. POON¶¶ and A. STREET***; TREATMENT GUIDELINES WORKING GROUP ON BEHALF OF THE WORLD FEDERATION OF HEMOPHILIA

**Department of Hematology, Christian Medical College, Vellore, India; †Department of Oral Surgery, The Royal Infirmary, Glasgow, Scotland; ‡Van Creveldkliniek and Department of Hematology, University Medical Center Utrecht, Utrecht, The Netherlands; §Department of Medicine, University of North Carolina, Chapel Hill, NC USA; ¶Sheffield Haemophilia and Thrombosis Centre, Royal Hallamshire Hospital, Sheffield, UK; **Department of Orthopaedics and Traumatology, Fundación Santa Fe University Hospital Fundación Cosme y Damián and Universidad de los Andes and Universidad del Rosario, Bogotá, Colombia; ††Comprehensive Care Haemophilia and Thrombosis Centre, Royal Infirmary, Edinburgh, UK; ‡‡Haemophilia Comprehensive Care Centre, Johannesburg Hospital and Department of Molecular Medicine and Haematology, Faculty of Health Sciences, National Health Laboratory Services and University of the Witwatersrand, Johannesburg, South Africa; §§Bleeding Disorders Clinic, Health Sciences Center, Winnipeg, Canada; ¶¶Departments of Medicine, Pediatrics and Oncology, and Southern Alberta Rare Blood and Bleeding Disorders Comprehensive Care Program, University of Calgary, Foothills Hospital and Calgary Health Region, Calgary, Canada; and ***Haematology, Alfred Hospital, Melbourne, Victoria Australia*

Srivastava et al, Haemophilia (2013),19,e1-e47

Buckets of product

Current status

But the people and audit and assessment data are missing

AUSTRALIAN AND NZ SCORECARDS 2015

- **PRODUCT**

  Safety

  Supply

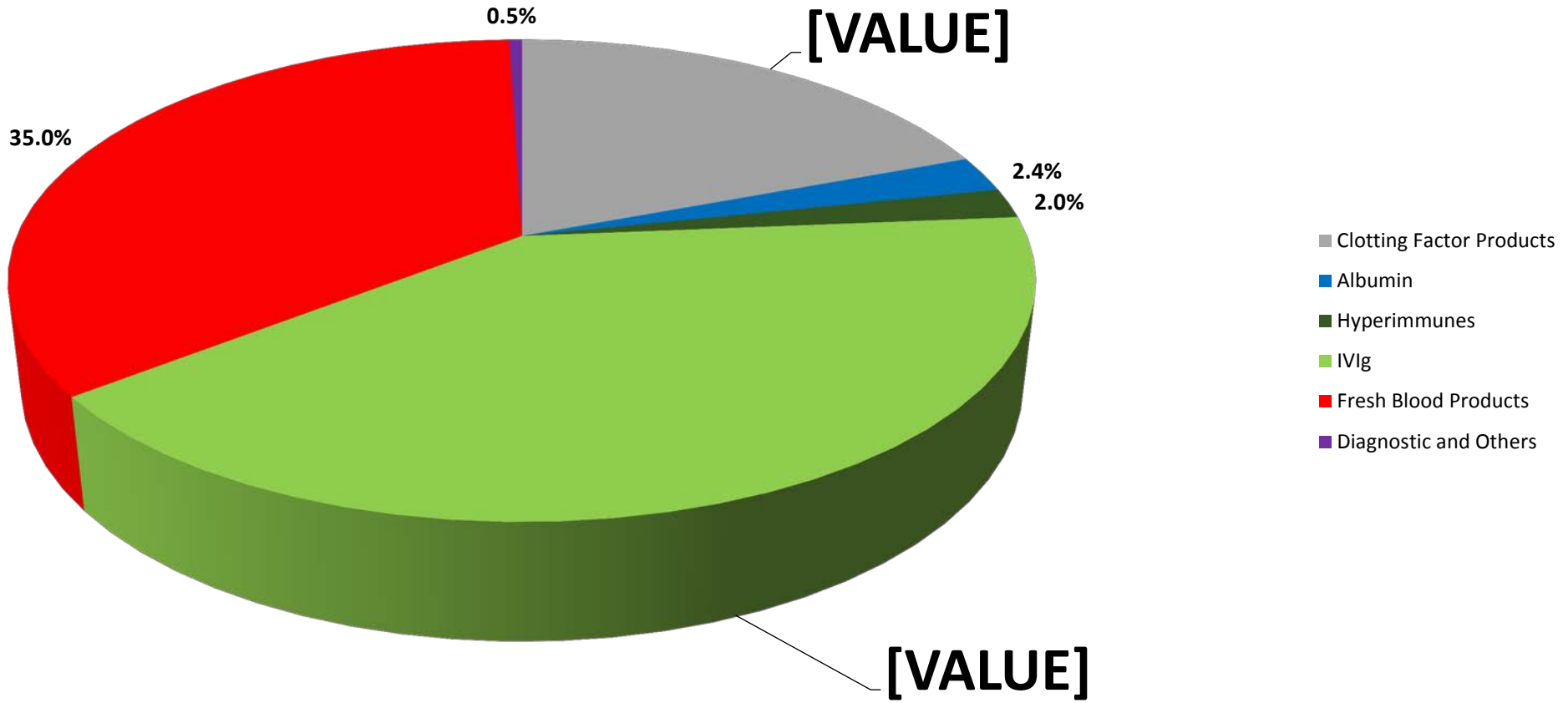
  Availability

- **HUMAN RESOURCES**

- **POLICY & GUIDELINES**

- **BENCHMARKING AND AUDIT**

With $201.8 million invested in product in 2013-4 a ”Whole of disease” funding model is required



Median value for the paediatric age group (0-17 years) was 1,059 IU/kg/year and the adult population, (18 years and over) was 1,212 IU/kg/year.

A$ 176.5 million for clotting factor products projected for 2015-6

Good value which needs to be proven

Or 50 All Blacks

With cost argument and advocacy

Haemophilia
The Official Journal of the World Federation of Hemophilia,
European Association for Haemophilia and Allied Disorders and
the Hemostasis & Thrombosis Research Society

Haemophilia (2013), 1–11 DOI: 10.1111/hae.12121

ORIGINAL ARTICLE

Treatment for life for severe haemophilia A– A cost-utility model for prophylaxis vs. on-demand treatment

A. FARRUGIA,*†‡ J. CASSAR,§ M. C. KIMBER,* M. BANSAL,* K. FISCHER,¶ G. AUSERSWALD,**
B. O'MAHONY,†† K. TOLLEY,‡‡ D. NOONE†† and S. BALBONI*
*Plasma Protein Therapeutics Association, Annapolis, MD, USA; †School of Surgery, University of Western Australia, Crawley, Western Australia; ‡School of Medicine, Australian National University, Canberra, Australia; §Faculty of Health, University of Canberra, Canberra, Australia; ¶Van Creveldkliniek, University Medical Center Utrecht, Utrecht, The Netherlands; **Ambulanz fuer Thrombose und Haemostasestoerungen Prof.- Hess Kinderklink, Klinikum Bremen-Mitte, Germany; ††Irish Haemophilia Society, Dublin, Ireland; and ‡‡Tolley Health Economics Ltd, Derbyshire, UK

Farrugia et al, Haemophilia (2013), 1-11

The New Generation

HAEMOPHILIA FOUNDATION VICTORIA

Our future

HAEMOPHILIA FOUNDATION AUSTRALIA

Love that poem and family

I am a Bleeder

I watch them as they laugh and play;
and know I'm not the same.
I see them fall and laugh it off;
to them it's just a game.
I know that I am not like them,
I'm different deep inside,
To me a bruise is painful,
to them it's a mark of pride.
But me I hold no shame in this,
for sadness wastes my youth,
I cannot do the things you do,
but why should I in truth?

I watch the clotters at school camp,
these games I cannot play,
So I sit in the shade and watch
as they run round all day.
They spend all hours in the sun
and start to sweat and stink,
They say that my life must be dull,
but that's not what I think;

So yes I am a Bleeder,
it's true I cannot lie,
It doesn't mean that when I'm cut
I'll slowly bleed and die.
And yes I am a Bleeder,
short on factor eight or nine,
I know this life ain't perfect
but it's good because it's mine.

And yes I am a Bleeder,
I'm telling you, no joke,
What don't kill me
makes me stronger
and we're strong
we Bleeder folk.

An excerpt taken from "I am a Bleeder" by Ben Inglis

HAEMOPHILIA FOUNDATION AUSTRALIA

With plentiful treatment (product) we must now focus on plentiful care

Acknowledgements and thanks

- AHCDO and NZTG colleagues present and past
- HFA and HFNZ
- Staff of Ronald Sawers Haemophilia Centre

And to all patients and their families with congenital bleeding disorders

PHTC core team	Proportion of FTE* spent managing children with bleeding disorders							
	1	2	3	4	5	6	7	8
Med	1.0	0.1	0.4	0.5	0.4	0.1	0.2	0.6
Nurse	1.3	0.4	0.3	1.0	1.0	0.2	0.3	0.3
PT	0	0	0.1	0.4	0.2	0	0	0.1
MSW	0	0.1	0.6	1.0	0.2	0	0.2	0.2

- Do our funders know also?
- What opportunities are there to contain costs?
- Do we understand and know the costs and how care is funded

We need to develop with all stakeholders agreed “whole of disease” funding models to include the costs of not funding care

- Definition
- Where it started
- Early Australian/NZ experience
- HIV/HCV
- Products including safety and supply
- ABDR and registry (WHO-WFH)
- Principles of care including outcome assessments,auditing and benchmarking
- Documents on CCCs and HTCs
- Scorecard buckets of product very few people

NBA ABDR report 2013-4
http://www.blood.gov.au/data-analysis-reporting

Diagnosis	Patients Numbers in ABDR Registry*			Number who Received Product during the year
	Bleeding Disorder 1	Bleeding Disorder 2	Bleeding Disorder 3	
HMA†	2,181	38	<5	10
HMB†	530	4		<5
Other‡	151	<5		-
Other Factor Deficiency	318	18		<5
Platelet Disorder	231	8		
Vascular	9	-		
Fibrinogen	40	<5		
VWD	1,912	31	<5	<5
Unclassified	11			
Total	5,385	102	<5	14

Demographic data for 2013-14 – HTC by State

	NSW	VIC	QLD	SA	WA	TAS	NT	ACT	National
HMA	586	530	457	251	279	59	19	57	2,238
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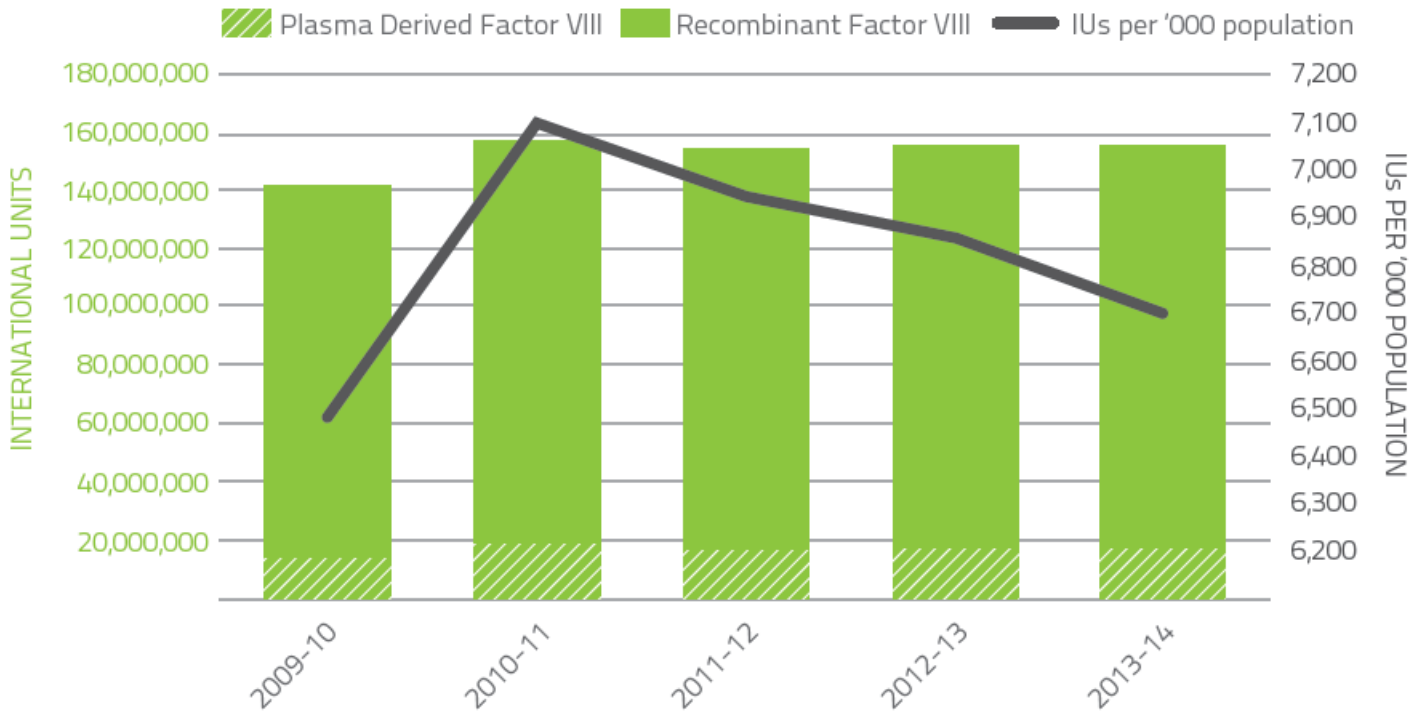
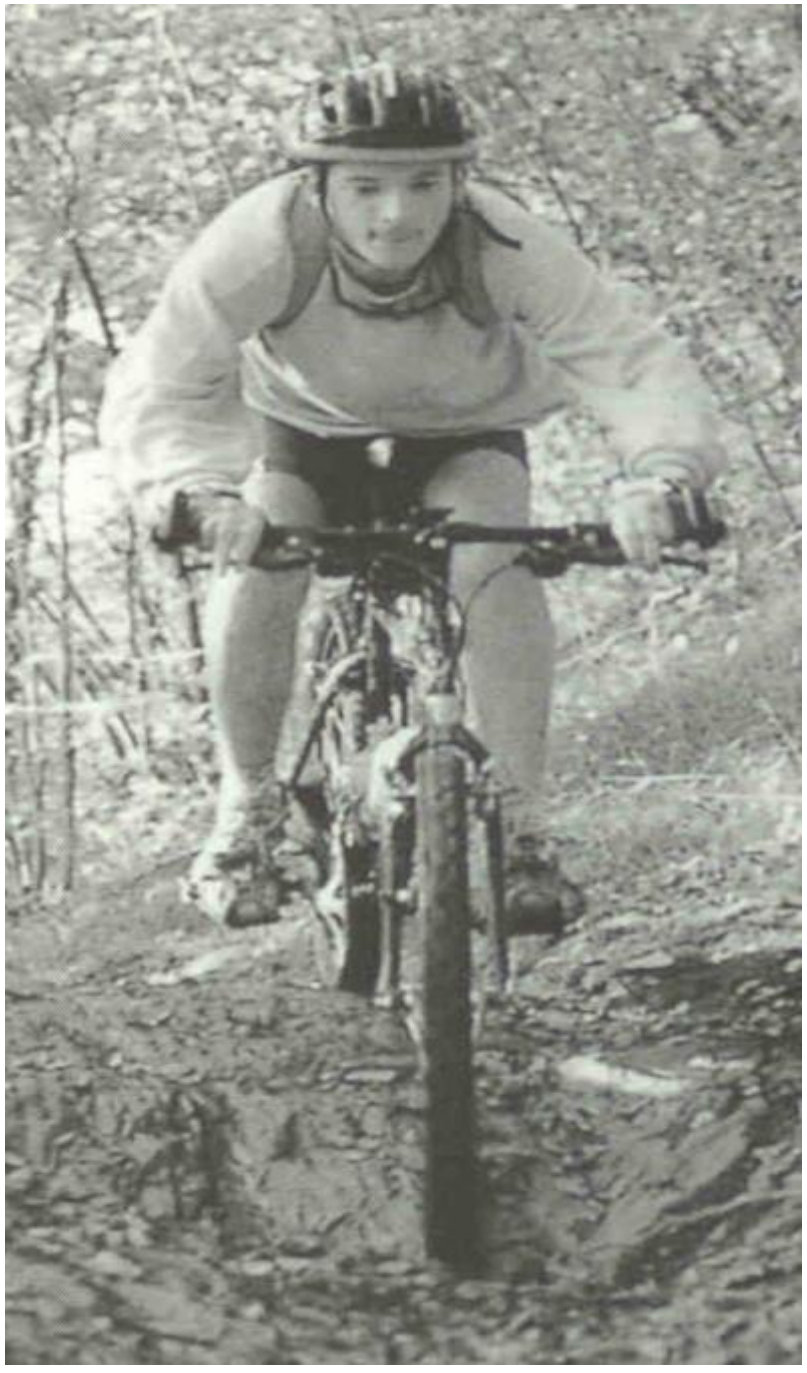

Plasma Derived Factor VIII
Recombinant Factor VIII
IUs per '000 population
INTERNATIONAL UNITS
180,000,000
160,000,000
140,000,000
120,000,000
100,000,000
80,000,000
60,000,000
40,000,000
20,000,000
7,200
7,100
7,000
6,900
6,800
6,700
6,600
6,500
6,400
6,300
6,200
IUs PER '000 POPULATION
2009-10
2010-11
2011-12
2012-13
2013-14

A NATIONAL SERVICE SPECIFICATION FOR HAEMOPHILIA AND RELATED CONDITIONS

WWW.HAEMOPHILIAALLIANCE.ORG.UK



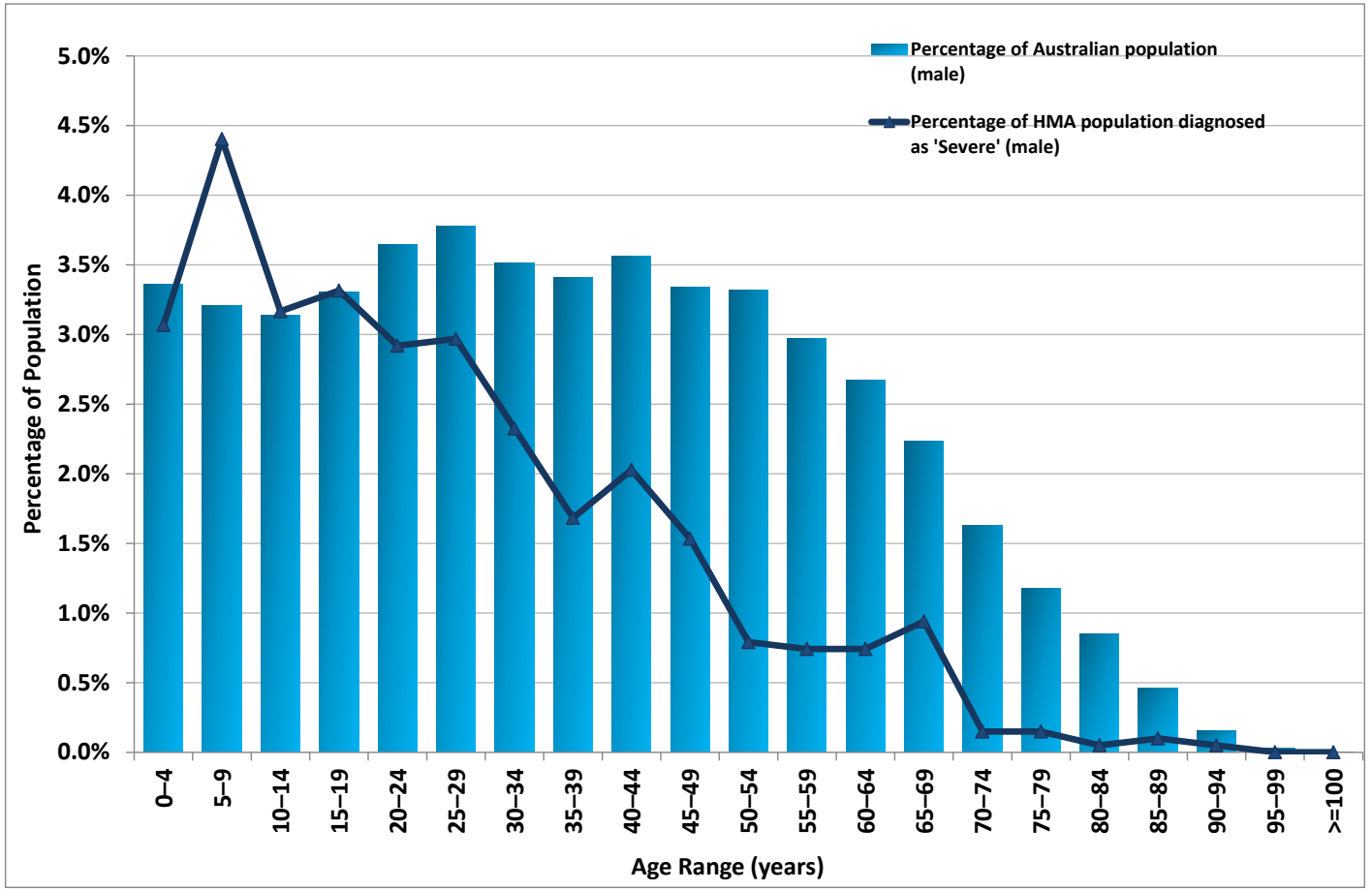
NBA ABDR report 2013-4
http://www.blood.gov.au/data-analysis-reporting

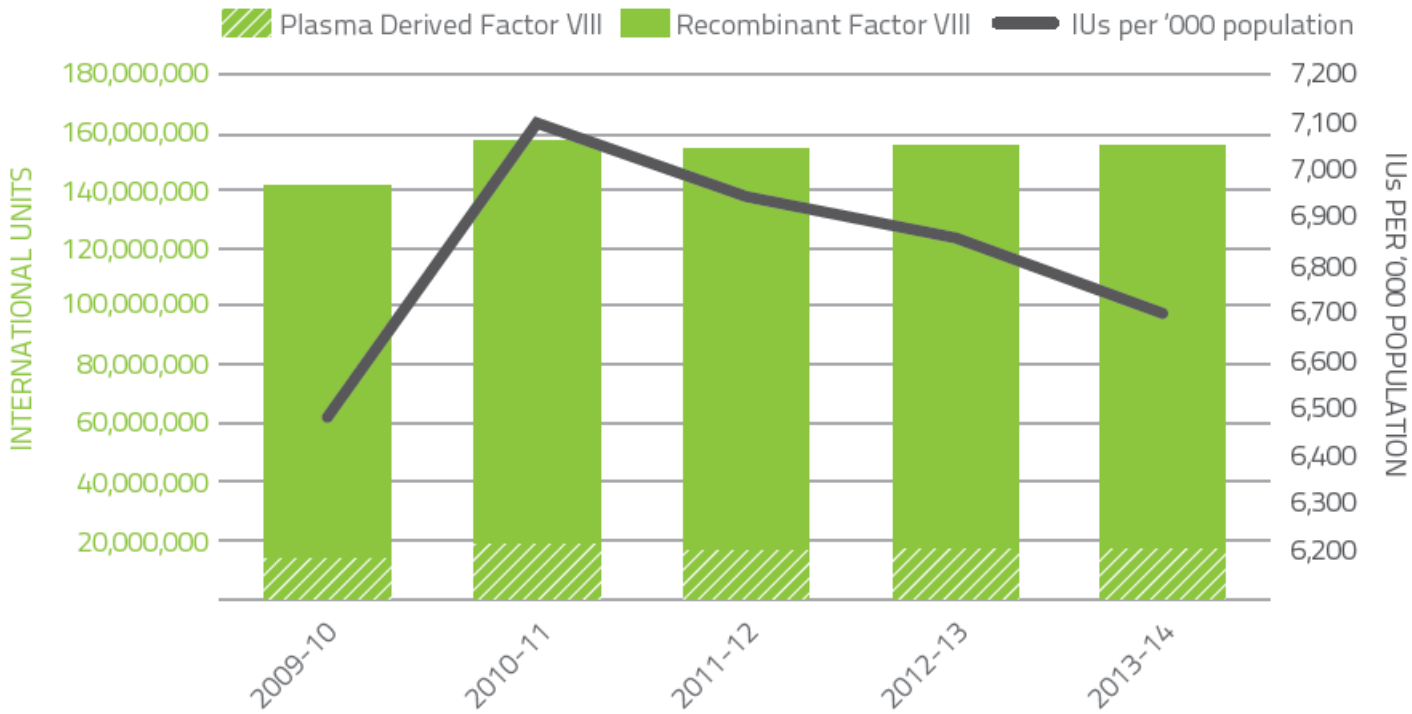
Diagnosis	Patients Numbers in ABDR Registry*			Number who Received Product during the year
	Bleeding Disorder 1	Bleeding Disorder 2	Bleeding Disorder 3	
HMA†	2,181	38	<5	10
HMB†	530	4		<5
Other‡	151	<5		-
Other Factor Deficiency	318	18		<5
Platelet Disorder	231	8		
Vascular	9	-		
Fibrinogen	40	<5		
VWD	1,912	31	<5	<5
Unclassified	11			
Total	5,385	102	<5	14

NBA ABDR report 2013-4
http://www.blood.gov.au/data-analysis-reporting

	Number in ABDR Registry*					Number who Received Product during the year				
Adult (aged 18 years and over)	2009-10	2010-11	2011-12	2012-13	2013-14	2009-10	2010-11	2011-12	2012-13	2013-14
HMA										
Mild	903	963	1,010	1,064	989	113	160	188	219	203
Moderate	186	191	199	190	153	70	86	82	89	81
Severe	428	444	466	504	391	253	272	280	328	336
HMB										
Mild	232	250	258	271	248	38	52	50	54	44
Moderate	82	88	91	94	88	29	31	40	45	48
Severe	56	58	61	69	56	37	40	39	47	48
VWD										
Mild	945	1,014	1,087	1,143	979	26	50	41	77	85
Moderate	187	205	227	229	234	22	34	32	43	51
Severe	106	113	120	128	121	25	38	32	46	48

Country	Population	HMA/HMB	VWD	OBD	HMA/HMB per100,000	VWD per 100,000	OBD per 100,000
Australia	22,015,576	2,860	2,068	773	12.99	9.39	3.51
New Zealand	4,327,944	421	195	31	9.73	4.51	0.72
UK	63,047,162	6,742	9,697	8,355	10.69	15.38	13.25
USA	313,847,465	18,628	8,035	1,796	5.94	2.56	0.57
Canada	34,300,083	3,657	3,963	1,693	10.66	11.55	4.94
France	65,630,692	6,035	1,496	413	9.20	2.28	0.63
Sweden	9,103,788	1,014	1,474	332	11.14	16.19	3.65
Germany	81,305,856	4,660	4,450	-	5.73	5.47	-
Spain	47,042,984	1,953	710	211	4.15	1.51	0.45
Netherlands	16,730,632	1,210	2,500	46	7.23	14.94	0.27


Percentage of Australian population (male)
Percentage of HMA population diagnosed as 'Severe' (male)
5.0%
4.5%
4.0%
3.5%
3.0%
2.5%
2.0%
1.5%
1.0%
0.5%
0.0%
Percentage of Population
0–4
5–9
10–14
15–19
20–24
25–29
30–34
35–39
40–44
45–49
50–54
55–59
60–64
65–69
70–74
75–79
80–84
85–89
90–94
95–99
>=100
Age Range (years)


Plasma Derived Factor VIII
Recombinant Factor VIII
IUs per '000 population
INTERNATIONAL UNITS
180,000,000
160,000,000
140,000,000
120,000,000
100,000,000
80,000,000
60,000,000
40,000,000
20,000,000
7,200
7,100
7,000
6,900
6,800
6,700
6,600
6,500
6,400
6,300
6,200
IUs PER '000 POPULATION
2009-10
2010-11
2011-12
2012-13
2013-14

Developing models of financial care

- The biggest component of cost of hemophilia care is the cost of treatment products
- Cost Effectiveness Reviews and Health Technology Assessments are undertaken to understand the cost and clinical benefits of care and define areas of continuing research needs
- Much more data and analysis is required to inform clinician (and patient) decisions for “optimal/optimised care”

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Audit in healthcare

- a review and evaluation of ***health care*** procedures and documentation for the purpose of comparing the quality of care provided with accepted standards

Pediatric benchmarking study

PHTC core team	Proportion of FTE* spent (actual) managing children with bleeding disorders							
	1	2	3	4	5	6	7	8
Med	1.0 (1.0)	0.1 (0.1)	0.4 (0.2)	0.5 (0.3)	0.4 (0.2)	0.1 (0.1)	0.2 (0.2)	0.6 (0.2)
Nurse	1.3	0.4	0.3	1.0	1.0	0.2	0.3	0.3
PT	0	0**	0.1	0.4	0.2	0 **	0**	0.1
MSW	0	0.1	0.6†	1.0‡	0.2	0**	0.2	0.2

*

Benchmarking in Healthcare

- Performance benchmarking is an activity of comparing performance levels to identify gaps
- Process benchmarking is the identification of root causes, which lead to achievement of superior performance.
- Patient experience benchmarking focuses upon meeting patients' expectations

With proper assessment tools we get information

- To record treatment effectiveness for clinicians and patients
- to tailor individual treatment programs
- for funders and the wider community about clinical and cost-effectiveness and social capital of treatment of bleeding disorders

Haemophilia (2008), 14, 361–374 DOI: 10.1111/j.1365-2516.2007.01625.x

SPECIAL ARTICLE
European Association for Haemophilia and associated disorders (EHAD)

European principles of haemophilia care

B. T. COLVIN,* J. ASTERMARK,† K. FISCHER,‡ A. GRINGERI,§ R. LASSILA,¶ W. SCHRAMM,** A. THOMAS†† and J. INGERSLEV‡‡ FOR THE INTER DISCIPLINARY WORKING GROUP

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Summary. As the management of haemophilia is complex, it is essential that those with the disorder should have ready access to a range of services provided by a multidisciplinary team of specialists. This document sets out the principles of comprehensive haemophilia care in Europe. Within each country there should be a national organization which oversees the provision of specialist Comprehensive Care Centres that provide the entire spectrum of clinical and laboratory services. Depending upon the size and geographical distribution of the population, a network of smaller haemophilia centres may also be necessary. There should be arrangements for the supply of safe clotting factor concentrates which can also be used in home treatment and prophylaxis programmes. A national register of patients is recommended along with collection of treatment statistics. As comprehensive haemophilia care is multidisciplinary by nature, the need for education and research programmes for all staff members is emphasized: Members of the Interdisciplinary Working Group not represented in the list of authors are mentioned in Section 4 of this document.

Keywords: haemophilia, haemophilia care, management, principles, treatment

Data management is a critical clinical tool

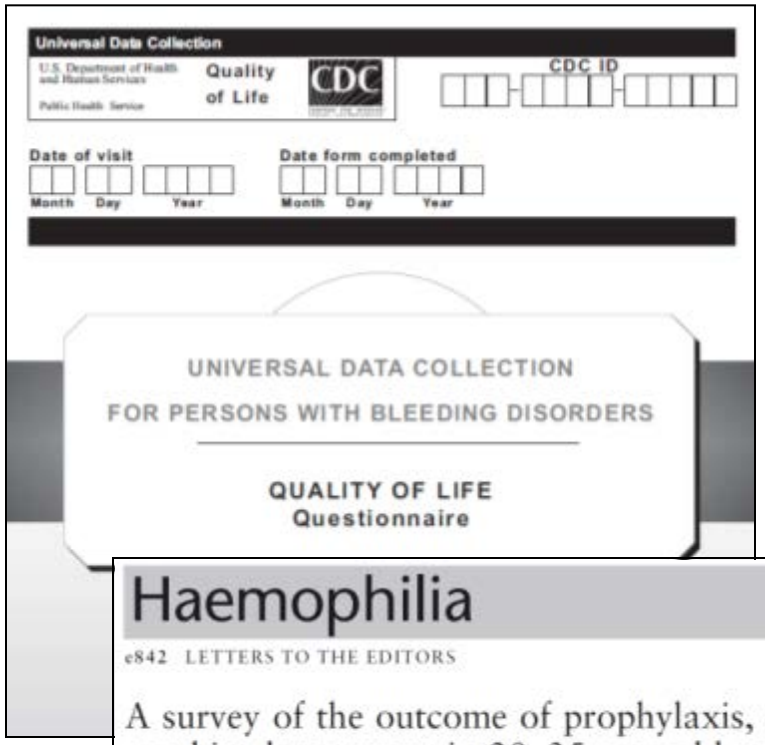
- Basic demographics
- Accurate diagnosis
- Product type and usage
- Clinical details (Inhibitors,HCV, HBV etc)
- Enrolment in clinical or product studies
- Serial standardised clinical assessment (clinical outcome) using validated tools

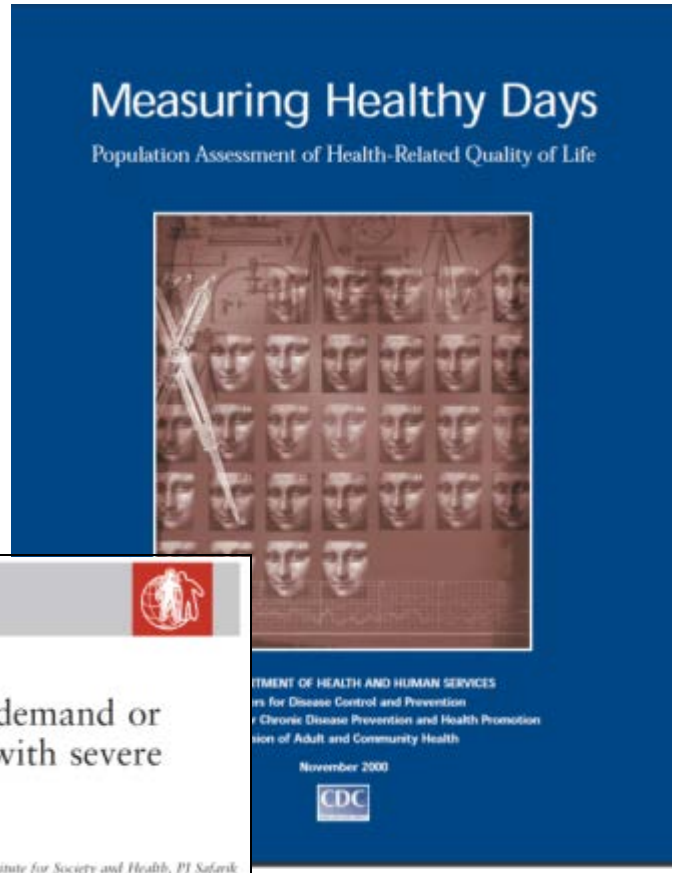
What processes improve their care?

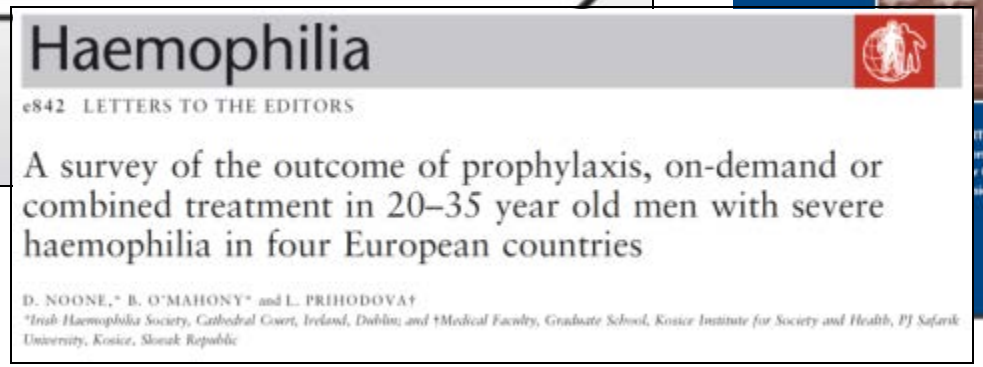
- Serial standardised physical and psychosocial assessments
- Regular review of product usage, bleeding pattern and product pharmacokinetics
- Tailored treatment plans
- Prompts for further reviews and intervention
- Performance of Auditing and Benchmarking activities

Which are all dependent on excellent data collection and analysis

and all have different experiences





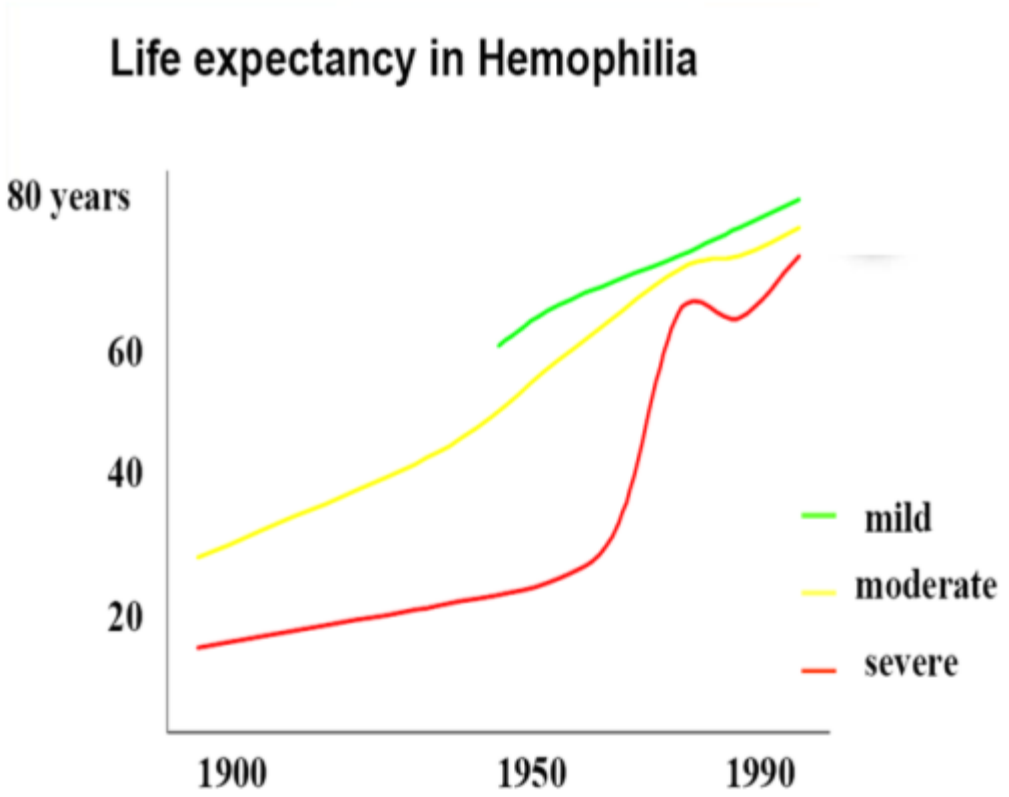


And its affordability

- Do our funders know also?
- What opportunities are there to contain costs?
- Do we understand and know the costs and how care is funded

We need to develop with all stakeholders agreed “whole of disease” funding models to include the costs of not funding care

Hemophilia is a (rare) medical success story

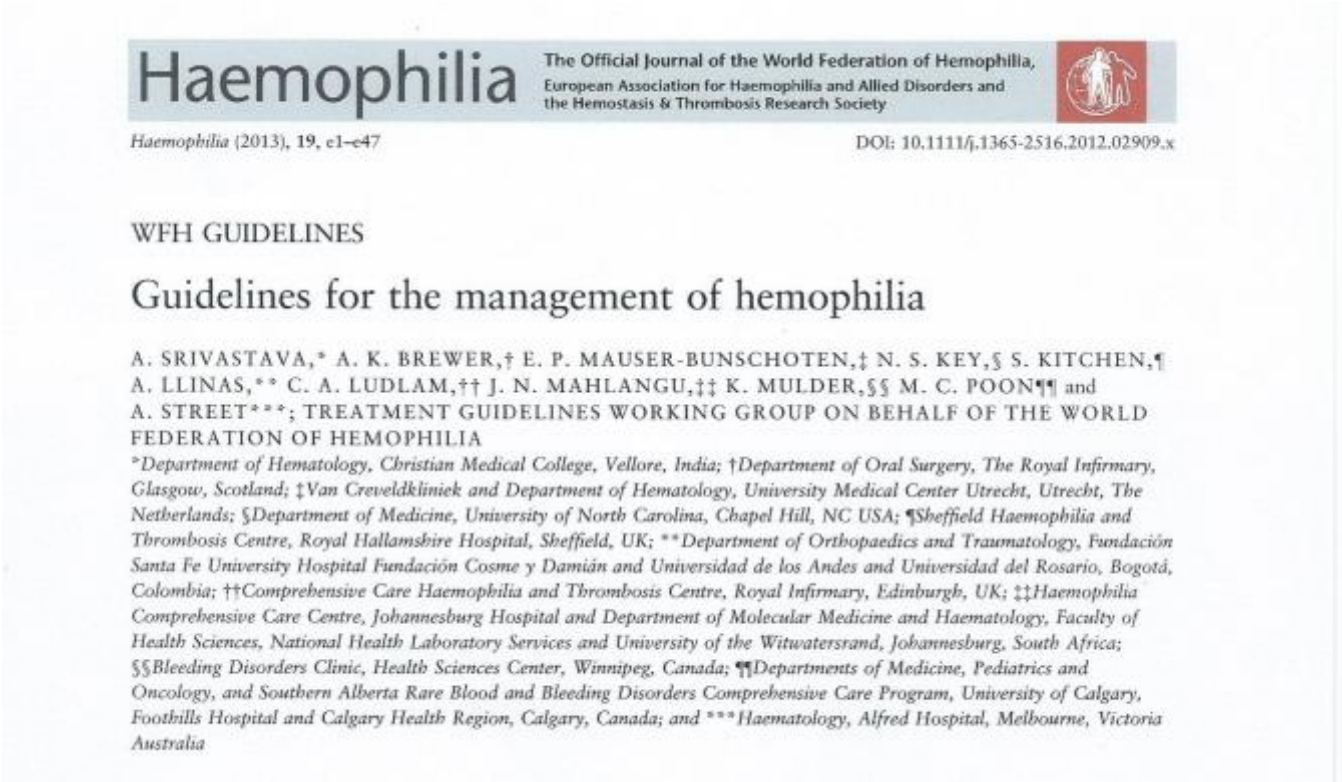


- Hemophilia
- Cystic fibrosis
- Thalassemia major
- Muscular dystrophy

☐ 75 years
☐ 37 years
☐ 30 years
☐ 10-20 years

rowell,drjohn.pdf

For adapting or adopting



Haemophilia
The Official Journal of the World Federation of Hemophilia,
European Association for Haemophilia and Allied Disorders and
the Hemostasis & Thrombosis Research Society

Haemophilia (2013), **19**, e1–e47 DOI: 10.1111/j.1365-2516.2012.02909.x

WFH GUIDELINES

Guidelines for the management of hemophilia

A. SRIVASTAVA,* A. K. BREWER,† E. P. MAUSER-BUNSCHOTEN,‡ N. S. KEY,§ S. KITCHEN,¶ A. LLINAS,** C. A. LUDLAM,†† J. N. MAHLANGU,‡‡ K. MULDER,§§ M. C. POON¶¶ and A. STREET***; TREATMENT GUIDELINES WORKING GROUP ON BEHALF OF THE WORLD FEDERATION OF HEMOPHILIA

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Srivastava et al, *Haemophilia* (2013),19,e1-e47

How does this DM model benefit patients?

- Comprehensive care is known by this term to health planners as being effective in improving patient outcomes,
- reducing hospital and emergency department admissions
- and being “patient” rather than disease focussed
- We should introduce this term when we talk about comprehensive care for patients with bleeding disorders with government and other health bodies

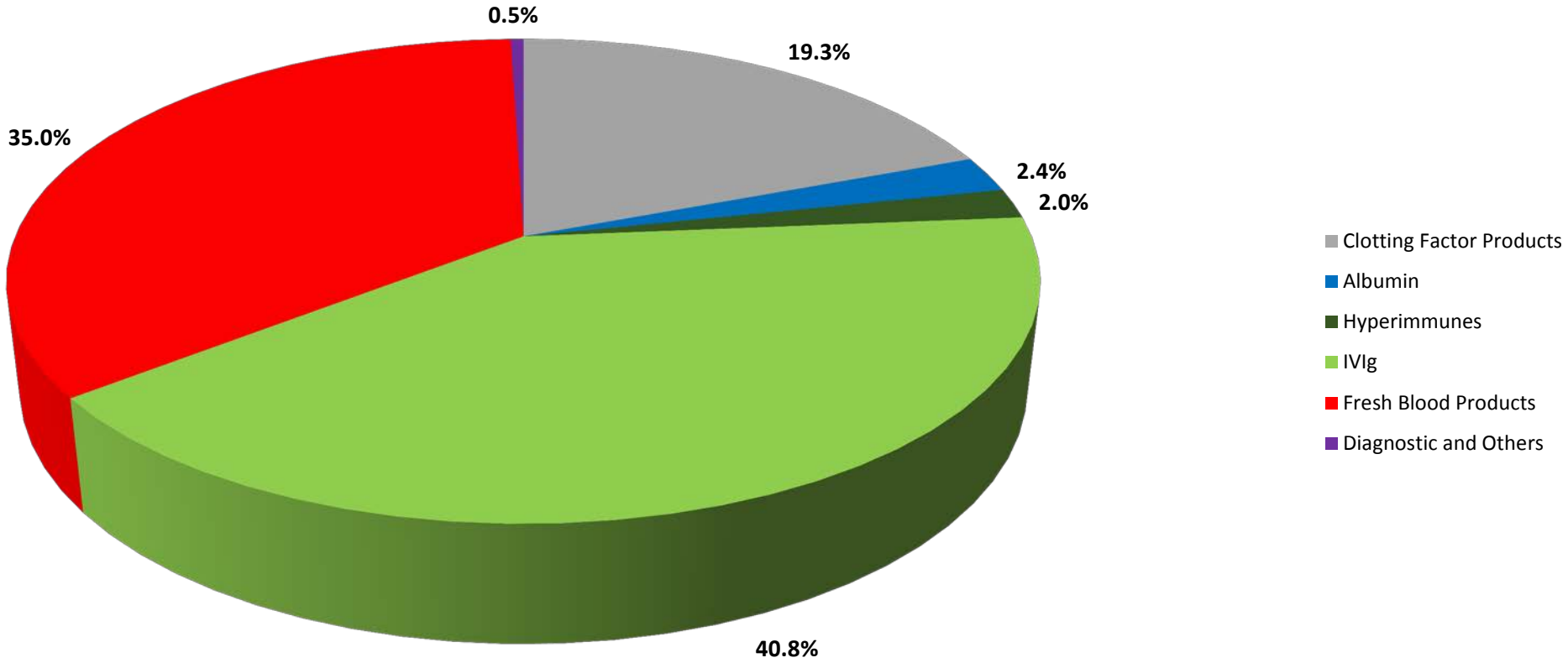
Chronic disease management

- A recently described system of co-ordinated healthcare interventions and communications for populations with conditions in which patient self-care efforts are significant
- May be disease specific (bleeding disorders) and supports the clinician and patient relationship in developing plans of care
- Requires multidisciplinary expertise, data, research and audit

Benchmarking in Healthcare

- Performance benchmarking is an activity of comparing performance levels to identify gaps
- Process benchmarking is the identification of root causes, which lead to achievement of superior performance.
- Patient experience benchmarking focuses upon meeting patients' expectations

$201.8 million bought



Median values for the paediatric (includes adolescents) age group was 1,059 IU/kg/year (0 to 17 years) and the adult population, (18 years and over) had a median value of 1,212 IU/kg/year.

	Paediatric 0-19 yrs		Adult 20 yrs & over		Total	
	Number in registry at 30 Jun 2011	Number who received product in 2010-11	Number in regis- try 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		

Demographic data for 2013-14 - HTC State

	NSW	VIC	QLD	SA	WA	TAS	NT	ACT	National
HMA	586	530	457	251	279	59	19	57	2,238
HMB	141	145	138	44	54	7	3	7	539
VWD	378	400	514	189	332	60	22	52	1,947
OTHER	115	195	161	109	191	6	5	4	786
TOTAL	1,220	1,270	1,270	593	856	132	49	120	5,510

	Mild	Moderate	Severe	Unknown*	Total**
HMA (IU FVIII Products)†	5,300,750	16,102,250	125,521,950	10,000	146,934,950
On Demand	3,741,750	6,091,750	21,586,500	1,500	31,421,500
Prophylaxis	707,250	9,752,000	94,752,950		105,212,200
ITT - Tolerisation	76,000		7,915,250		7,991,250
Unknown*	775,750	258,500	1,267,250	8,500	2,310,000
HMB (IU FIX Products)‡	2,972,000	7,573,750	19,434,500	0	29,980,250
On Demand	2,351,000	3,096,750	4,031,000		9,478,750
Prophylaxis	471,000	4,178,000	12,403,500		17,052,500
Tolerisation			2,978,000		2,978,000
Unknown*	150,000	299,000	22,000		471,000
VWD (IU FVIII Product) ++	644,250	541,000	4,377,750	451,503	6,014,503
On Demand	367,750	483,000	1,653,000	379,003	2,882,753
Prophylaxis	128,000	10,000	2,623,750	35,500	2,797,250
Unknown*	148,500	48,000	101,000	37,000	334,500

Haemophilia (2012), 18, 649–654 DOI: 10.1111/hae.12000

COMMEMORATIVE ARTICLE

The AIDS epidemic in haemophilia patients II: pursuing absolute viral safety of clotting factor concentrates 1985–1988

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"A man should look for what is, and not for what he thinks should be."

Albert Einstein

Introduction

The primary phase of the AIDS epidemic in the haemophilia population ended abruptly in 1985 [1,2]. Unfortunately, the manner of its ending left unanswered questions destined to affect the haemophilia community until the next decade.

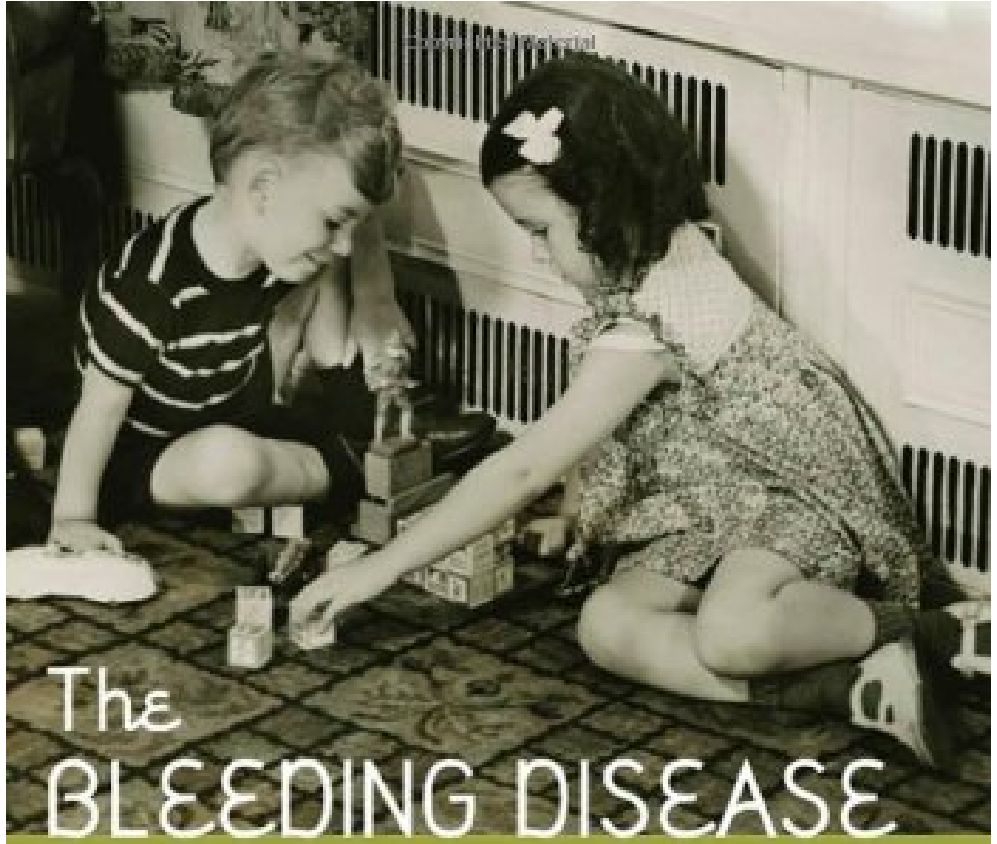
In July 1984, the author [then Director of the Division of Host Factors (DHF; DHF is now known as Division of Blood Disorders, Centers for Disease Control and Prevention), Centers for Disease Control (CDC)] presented data on the effectiveness of heat treatment on inactivation of the AIDS virus at the World Federation of Hemophilia (WFH) Congress in Rio de Janeiro. Upon hearing further confirmatory data by DHF in October 1984, the National Hemophilia Foundation's (NHF) Medical and Scientific Advisory Council (MASAC) issued recommendations that 'treaters using coagulation factor concentrates should strongly consider changing to heat-treated products' [3,4]. The haemophilia community widely adopted these recommendations in 1985. The true impact of these recommendations on the epidemic would not be known until DHF's studies of birth cohorts in the United States and Universal Data Collection (UDC) surveillance data retrospectively confirmed, more than a decade later, that US patients were not infected with HIV from heat-treated factor subsequent to their adoption as standard of care [2,5]. However, the period from 1985 to 1990 was a period of uncertainty about clinical safety and the haemophilia community, the treating physicians, the manufacturers of coagulation products and regulatory agencies had to make difficult decisions about the reliability of products, manufacturing practices and therapeutic choices with little guidance. Some of these decisions contributed to adverse outcomes.

Lack of clinical data creates uncertainty

In 1985, the use of heat-treated products for the prevention of AIDS was in fact an 'off label' application; that is, the heat-treated products were not used for the purpose for which they had been licensed by the Federal Drug Administration (FDA). Four US manufacturers – Cutter Biological, Armour Pharmaceutical, Alpha Therapeutics and Hyland Therapeutics of Baxter Healthcare – received licenses for dry heat-treated products in 1983 and early 1984 (prior to identification of the HIV virus as the causal AIDS agent) to reduce risk of hepatitis infections in recipients rather than to reduce the risk of AIDS infection [1]. Only subsequently did the *in vitro* (laboratory-based) heating experiments suggest that heat-treated products might reduce (if not eliminate) the risk for transmitting HIV, but no actual clinical (*in vivo*) data existed on the efficacy of heat-treated factor in reducing HIV infection. Normally, clinical efficacy, determined by prospective clinical trials, would be required before licensing. However, a significant and growing portion of the haemophilia population was being infected in 1984 and the haemophilia community was desperate for any possible preventive measure. Most readily accepted the use of heat-treated concentrates based only on the *in vitro* data with evaluation of the level of viral safety by subsequent surveillance [1].

Although DHF established surveillance mechanisms to identify possible HIV seroconversions in patients taking heat-treated clotting factors, several problems made the task difficult. Logistically, the surveillance was voluntary and passive, rendering it less sensitive. Second, the majority of infected haemophilia patients were still unidentified, either by clinical symptoms or testing. These patients had to be distinguished from persons seroconverting from the new heat-treated products. Patients often used more than one brand of clotting factor concentrate; when these persons were included, identifying an unsafe product depended on statistical analysis of a number of suspected seroconversions. Finally, although most patients in the United States were using heat-treated clotting factors in early 1985, some physicians and organizations still objected to its use.

An important and often neglected history



The BLEEDING DISEASE

Hemophilia and the Unintended Consequences of Medical Progress

STEPHEN PEMBERTON