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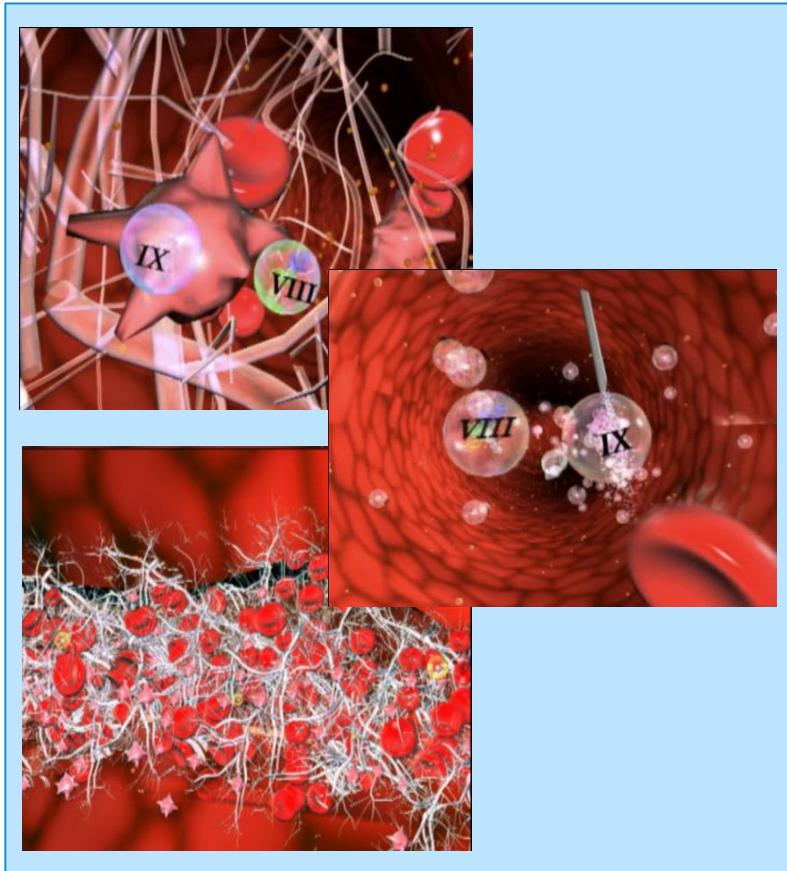


Experience with implementing the principles of care for haemophilia

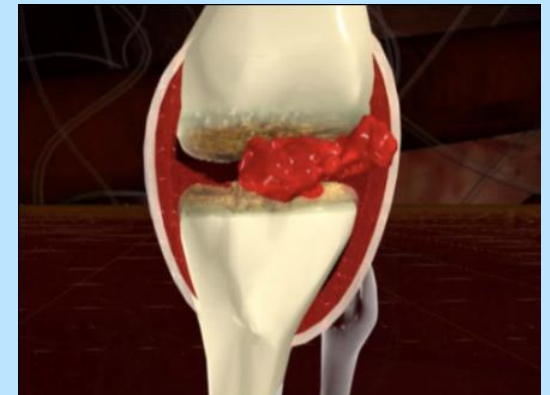
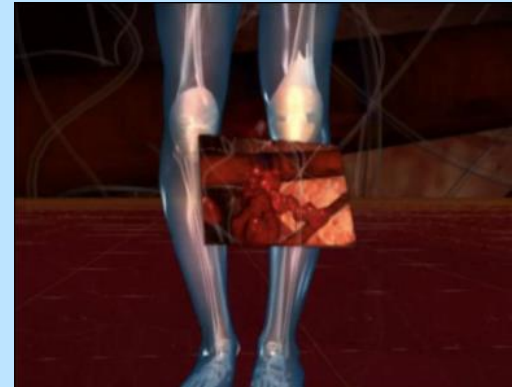
Disclosures

- Consultancy – Advisory board
 - Baxter, Bayer, Pfizer, CAF-DCF, SOBI, Ipsen, LFB, CSL-Behring, Novo Nordisk, Octapharma
- Research grants / Lecture Chairs
 - Baxter, Bayer, Pfizer, CAF-DCF, CSL-Behring, Novo Nordisk, Octapharma, Ipsen

Haemophilia



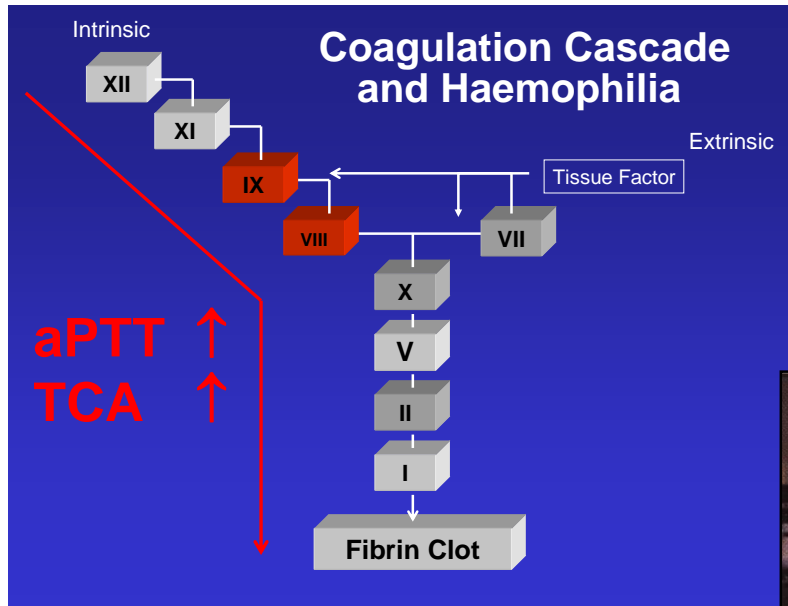
Blood Coagulation Defect



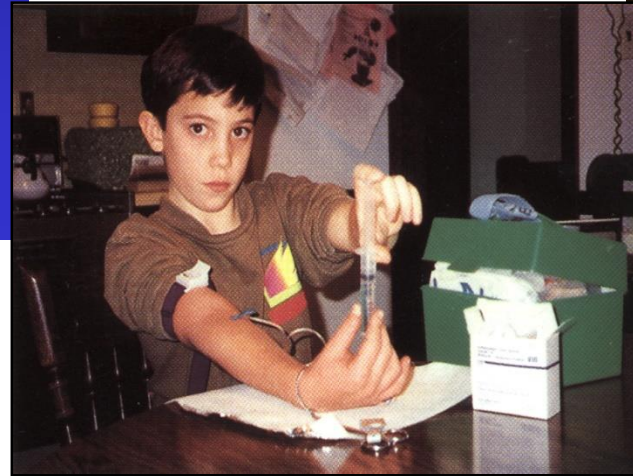
Deliberating Arthropathy

*From the Movie « Haemophilia in 3D » by Cedric Hermans et al
www.hémophilie-ucl.be also available on YOU TUBE*

Treatment of Haemophilia



Coagulation defect



Regular Self-infusions
of FVIII / FIX at home
Multidisciplinary follow-up in a
comprehensive haemophilia centre



Arthropathy

The 10 European Principles of Hemophilia Care

1. A central hemophilia organisation with supporting local groups
2. National hemophilia patient registries
3. Comprehensive care centres and hemophilia treatment centres
4. Partnership in the delivery of hemophilia care
5. Safe and effective concentrates at optimum treatment levels
6. Home treatment and delivery
7. Prophylaxis treatment
8. Specialist services and emergency care
9. Management of inhibitors
10. Education and research



European principles of haemophilia care

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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. 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.



Practice versus Principles

- The level of service provision within different countries in Europe compared to the recommendations set out in the Principles of Care has recently been audited by two studies;

– Patients' organisations



– Physicians



Haemophilia



Haemophilia (2010), 1–6

DOI: 10.1111/j.1365-2516.2010.02362.x

ORIGINAL ARTICLE

Haemophilia care in Europe: a survey of 19 countries

B. O'MAHONY,* D. NOONE,* P. L. F. GIANGRANDE† and L. PRIHODOVA‡

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Haemophilia

The Official Journal of the World Federation of Hemophilia,
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the Hemostasis & Thrombosis Research Society



Haemophilia (2013), 19, 35–43

DOI: 10.1111/j.1365-2516.2012.02928.x

ORIGINAL ARTICLE *Clinical haemophilia*

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

K. FISCHER,* and C. HERMANS† ON BEHALF OF THE EUROPEAN HAEMOPHILIA THERAPY STANDARDISATION BOARD (EHTSB)

*Van Creveldklinik, Department of Haematology, University Medical Center, Utrecht, The Netherlands; and †Haemostasis and Thrombosis Unit, Division of Haematology, Cliniques universitaires Saint-Luc, Bruxelles, Belgium

O'Mahony B, Noone D, Giangrande PL, Prihodova L. Haemophilia care in Europe: a survey of 19 countries. *Haemophilia* 2011 Jan;17(1):35-40.

Fischer K, Hermans C. The European Principles of Haemophilia Care: a pilot investigation of adherence to the principles in Europe. *Haemophilia* 2013 Jan;19(1):35-43.

Principle 1

A Central Haemophilia Organisation with Supporting Local Groups

- In each country there should be a central organisation for haemophilia care supported by centres operating at the local level
- These organizations will provide a focus for:
 - Provision of safe concentrates
 - Effective allocation of resources
 - Collection of data on concentrate usage
 - Recording of adverse reactions
 - Sharing of developments in care
 - Coordination of research

Adherence to Principle 1

- Central organizations of haemophilia care (mostly physicians' treatment boards), present in 11/14 (79%) of the European countries surveyed.
- Belgium, Spain and Portugal had not established such organizations.

Principle 2

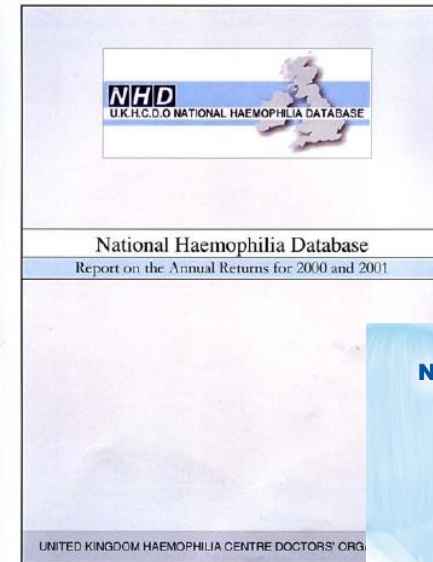
- **National Haemophilia Patient Registry**



- Each country should have a national haemophilia patient registry administered by the central haemophilia organisation
- Registries facilitate resource planning and allocation, as well as provide accurate data on patient numbers, prescribing patterns, geographical spread and adverse events

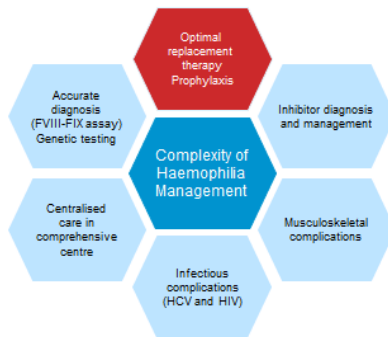
Adherence to Principle 2

- National registries in 8/14 (57%) of the countries surveyed
- Belgium, Sweden, the Netherlands, Norway, Poland and Portugal reported that registries had not been not established in their country.
- Overall, only 7/14 (50%) countries complied with both principles 1 and 2.



Principle 3

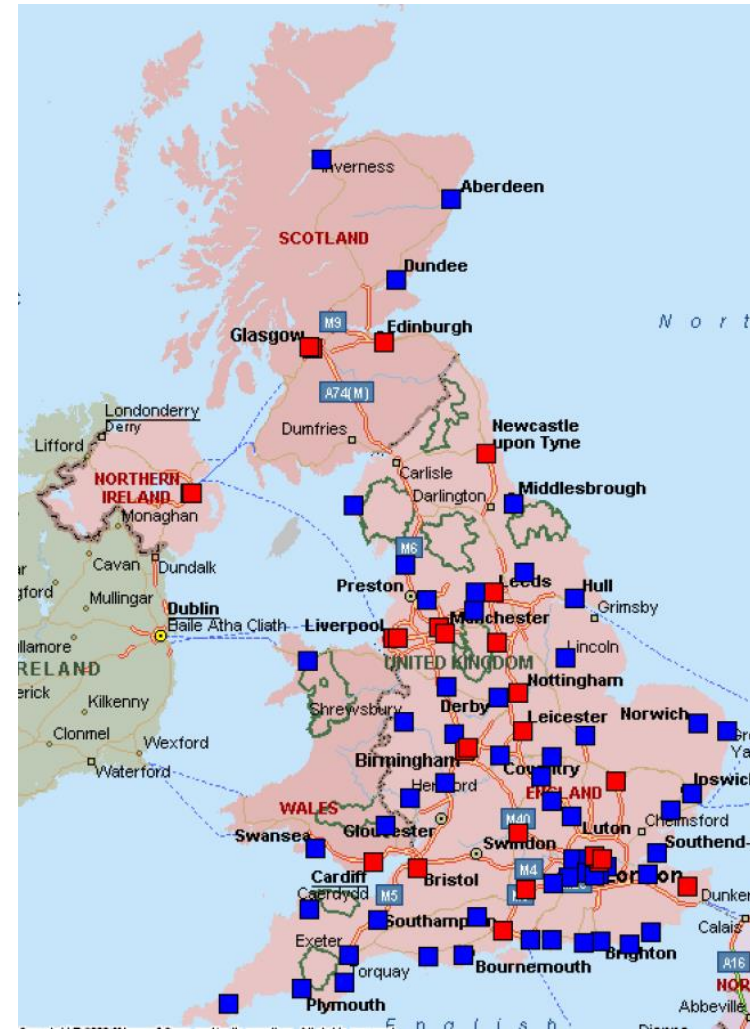
- **Comprehensive Care Centres and Haemophilia Treatment Centres**
 - Comprehensive Care Centres and Haemophilia Treatment Centres should be established to ensure that people with haemophilia have access to the full range of clinical specialties and appropriate laboratory services



**Wessex Haemophilia
Comprehensive Care Network**

Adherence to Principle 3

- All 14 countries surveyed had designated CCCs.
- Every country with the exception of Sweden had HTC; only Sweden had just CCCs.
- In 9/14 (64%) of countries, all patients with haemophilia were seen in either a CCC or HTC; the exceptions were Belgium, Germany, Poland, Portugal and Switzerland.
- Some moderate and mild patients were treated in other hospitals or private practices.



Comprehensive Care Centres

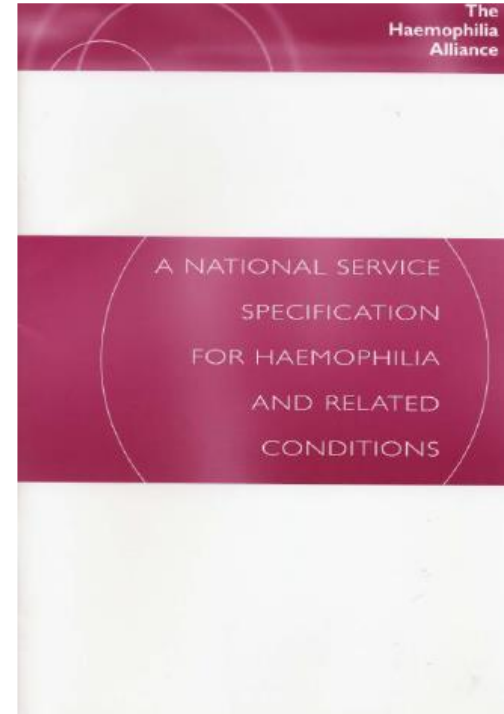
Haemophilia Centres

Summary of adherence to Principles 1,2 and 3 and 7 according to country

Country	No of Centres	Principle 1 Central Organisation	Principle 2 Patient Registry	Principle 3 All patients treated in CCC/HTC	No of CCC/HTC per Million inhabitants	Principle 7 % of Children on prophylaxis	Principle 7 % of Adults on prophylaxis
Belgium	1	No	No	No	0.83	75-100	50-75
France	1	Yes	Yes	Yes	0.71	75-100	1-25
Germany	2	Yes	Yes	No	0.89	75-100	50-75
Greece	1	Yes	Yes	Yes	0.37	75-100	1-25
Italy	3	Yes	Yes	Yes	0.81	75-100	1-25
Netherlands	2	Yes	No	Yes	0.78	75-100	50-75
Norway	1	Yes	No	Yes	0.40	75-100	50-75
Poland	1	Yes	No	No	0.84	75-100	1-25
Portugal	1	No	No	No	3.77	75-100	1-25
Slovakia	1	Yes	Yes	Yes	7.78	75-100	1-25
Spain	3	No	Yes	Yes	0.91	75-100	1-25
Sweden	1	Yes	No	Yes	0.32	75-100	75-100
Switzerland	1	Yes	Yes	No	1.27	75-100	1-25
United Kingdom	2	Yes	Yes	Yes	1.06	75-100	50-75
	Total 21	79% Yes	57% Yes	64% Yes	Median 0.84 IQR0.62-1.11		

Principle 4

- **Partnership in the Delivery of Haemophilia Care**
 - Clinicians and patient representatives should be part of national and/or regional haemophilia care decision making in partnership with ministries of health and social affairs, as well as those organisations that deliver haemophilia care via a formal mechanism such as a National Haemophilia Co-ordinating Group



Adherence to Principle 4

- About one third of the 14 countries had formal mechanisms in place to ensure collaboration.
- Government health bodies were involved to some degree in all countries.
- Clinicians were strongly involved in national or regional care decision-making in all countries with the exception of Belgium and Poland, where clinicians were only involved to some degree.

Principle 5

- **Access to Safe and Effective Concentrates at Optimum Treatment Levels**
 - People with haemophilia should have access to safe and effective replacement factor treatment concentrates at optimum treatment levels
 - This improves physical health and reduces the psycho-social and economic impact of this bleeding disorder on the patient. It also reduces the amount of long-term support required from family, community and government

Adherence to Principle 5

- No constraints in dosage of prescribed factor concentrate.
- All countries used plasma-derived factor VIII (pd-FVIII) and all except Poland used recombinant factor VIII (r-FVIII).
- All countries used pd-FIX.
- r-FIX was used in all countries except Slovakia and Poland.
- Only the UK had a national guideline concerning the prescribing of recombinant concentrates for all patients.
- Five other countries had a policy of prescribing recombinant concentrates for children.

Principle 6

- **Access to Home Treatment and Delivery**
 - Home treatment and home delivery should be available in each country to facilitate immediate and effective treatment
 - This results in a reduction in hospital visits, prevents short- and long-term disability and allows those with haemophilia to have the freedom to lead lives that are as normal as possible

Adherence to Principle 6

- Home treatment was supported and taught by all centres.
- 11 centres directly or indirectly provided treatment by trained personnel at the patient's own home; 10 centres did not.

Principle 7

- **Access to Prophylactic Therapy**
 - Prophylactic treatment should be available to people with haemophilia as it has been shown to prevent and improve chronic joint disease
 - Prophylaxis also promotes health and social well-being

Proportion of patients on prophylaxis according to country and centre

	Nr of countries N=14		Nr of centres N=21	
% of patients on prophylaxis	Children	Adults	Children (19 centres)*	Adults (21 centres)
0%				-
1-25%		8 (57%)		8 (38%)
26-50%				5 (24%)
51-75%		5 (36%)		6 (28%)
76-100%	14 (100%)	1 (7%)	21 (100%)	2 (10%)
* two centres did not treat children				

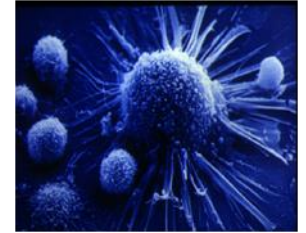
Principle 8

- **Specialist Services and Emergency Care**
 - Haemophilia care requires the co-ordination of a number of services to make sure that the particular needs of those with haemophilia are met
 - In critical situations, people with haemophilia need immediate access to treatment as well as skilled care through Accident & Emergency departments and to the range of specialists required to ensure their safety

Adherence to Principle 8

- All centres were able to provide day care for patients with haemophilia.
- In all centres prompt review could be provided by junior staff within 1 h, with senior medical staff available for treatment advice on a 24 h basis.
- **Paediatric care was less organized in some centres (Paediatricians in staff in 9/19 centres).**
- Overall, a designated physiotherapist was available in 14 (67%) centres.
- A designated orthopaedic surgeon was available in 14/21 (67%) centres.

Principle 9



- **Management of Inhibitors**

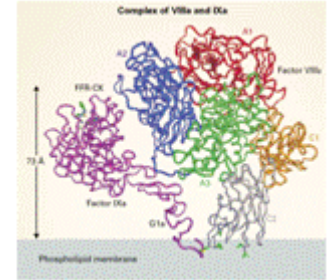
- Some people with haemophilia develop “inhibitors”, when their bodies inactivate the replacement clotting factor treatment. Those affected need to have immediate access to optimum treatments
- Where appropriate, immune therapy induction therapy (ITT) and the management of bleeding should be administered by clinicians with the necessary expertise, in hospitals with appropriate clinical and laboratory resources

Adherence to Principle 9

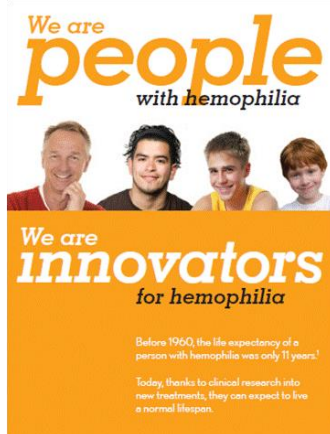
- The median number of patients with inhibitors per centre was eight (range 0–41).
- In all centres all patients with inhibitors had access to immune tolerance induction (ITI).

Principle 10

- **Education and Research**



- Recruitment and education of physicians in the area of thrombosis and haemostasis is an important task for the future to secure high quality care
- Further research into haemophilia is also required



Adherence to Principle 10

- Haemophilia centres were usually associated with a university.
- 18 of the 21 centres were part of a University Hospital.
- All undertook teaching about haemophilia and all centres were engaged in clinical trials or research studies.
- In addition, 10 centres reported that they had initiated studies.

Conclusions

- 😊 The Principles of Haemophilia Care were generally applied throughout Europe.
- 😊 A crude estimate of the number of centres per 1 million of population shows considerable variation.
- 😊 Clotting factor concentrates were available, but purchasing strategies varied.
- 😊 Home treatment was taught everywhere.
- 😊 Prophylaxis was available for all children but not for all adult patients.
- 😊 All patients had access to immune tolerance induction (ITI).

Conclusions

- ☹️ National registries were not used everywhere.
- ☹️ Not all moderate and mild haemophilia patients were treated in designated centres.
- ☹️ At centre level, dedicated physiotherapists, formal paediatric care and 24 h FVIII/FIX assays were lacking in some.

EUHANET

EUROPEAN HAEMOPHILIA NETWORK



www.euhanet.org



The EUHANET PROJECT.

EUHANET is a project aimed at establishing a network of haemophilia centres to work together on a number of related projects to improve the care of European citizens with inherited bleeding disorders.

PARTICIPANTS

The project will be directed from Sheffield in the UK by Dr Mike Makris. There are five associated partners: European Haemophilia Consortium (EHC), European Association for Haemophilia and Allied disorders (EAHAD), University Medical College Utrecht, Medical Data Solutions and Services Ltd (MDSAS) and Fondazione IRCCS Ca' Granda in Milan.

In addition 84 centres from 26 countries have already committed to participating and were part of the original funding application.

FUNDING THE PROJECT

Sixty percent of the funding is provided by the European Commission via its Executive Agency for Health and Consumers (EAHC) and the rest will be raised from industry.

PROJECT DURATION

The project started on 1st June 2012 and will continue until 31st May 2015.

AREAS OF WORK

There are four main areas that EUHANET will work on:



1. Assessment and standardisation of the quality of care of haemophilia centres.

There are 420 known haemophilia centres in Europe at locations shown on this map. The size and services offered vary enormously with some centres caring for more than 350 persons with severe haemophilia whilst others care for less than five. The times and extent of available care also vary significantly.

During the first year of the project the criteria defining two levels of haemophilia care will be developed. There will be extensive consultation during this development.

Once the criteria have been developed centres will be able to apply for European Certification of the level of care they provide based on satisfaction of the criteria.

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In addition, ready-made centres are the ones that will be involved.

FUNDING
Sixty per cent of the project is financed by the European Union. The remaining 40% is raised by the project partners.

PROJECT
The project will continue for three years.

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EAHAD
2016

9th ANNUAL CONGRESS
OF THE EUROPEAN ASSOCIATION
FOR HAEMOPHILIA AND ALLIED DISORDERS

MALMÖ
3-5 February 2016
SWEDEN



Photo by Fredrik Johansson



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