European Haemophilia Network

EUHANET

D5: Lay version of final project report

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Introduction

This report describes the European Haemophilia Network (EUHANET) project, its aims and achievements and the benefits it is expected to bring.

EUHANET involved health professionals and patient organisations in Europe working together in a number of related areas to improve the care of European citizens with inherited bleeding disorders. There were four main areas of work:

1. assessing and classifiying the quality of care provided by haemophilia centres;
2. creating and maintaining a Haemophilia Central website, which provides useful information on the bleeding disorders and their treatment;
3. monitoring the safety of treatments for inherited bleeding disorders through the European Haemophilia Safety Surveillance (EUHASS) adverse event reporting system;
4. collecting prospective information on the diagnosis, health and treatment of patients with extremely rare bleeding disorders due to fibrinogen defects or deficiency of clotting factor XIII, for the prospective Rare Bleeding Disorders Database (PRO-RBDD).

EUHANET began in June 2012 and was co-funded by the European Commission and 12 major pharmaceutical companies until May 2015. Work was carried out jointly by the European Haemophilia Consortium (EHC) which represents patient organisations in 48 European countries, the European Haemophilia and Allied Disorders (EAHAD) health professionals' organisation and research teams based at the universities of Sheffield, Milan and Utrecht.

Background and Aims

The most widely known inherited bleeding disorders are Haemophilia A (due to a deficiency of clotting factor VIII) and Haemophilia B (deficiency of factor IX) and these affect 1 in 10,000 and 1 in 50,000 people respectively. Other bleeding disorders due to deficiencies in other clotting factors (such as factors I, II, V, VII, X, XI and XIII) are even rarer, affecting around 1 person in a million. People with severe forms of bleeding disorder may suffer from spontaneous bleeding into their joints and muscles, which causes damage. People with milder deficiencies suffer from excessive bleeding if they are injured. These problems are treated by injections of clotting factor concentrates into the vein and patients are usually cared for in haemophilia centres. These are very expensive disorders to treat, the cost of caring for one haemophilia patient can be over €100,000 each year.

There are huge differences in the availability and quality of care offered by the over 400 haemophilia centres across Europe. Centres use a variety of names – haemophilia centre, comprehensive care centre, national haemophilia centre – but there is no agreed definition of what these names mean. Some centres care for several hundred patients with severe haemophilia while others may have less than five patients. Some centres have good access to laboratories to help with accurate diagnosis and management of patients, while others do not. Faced with such variation, it is difficult for a patient to judge how comprehensive the care given by a particular centre is likely to be. This is an important issue if there are several centres in their local area, and also for patients travelling outside their own area or country and who need to know where they can get emergency care. The EUHANET project aimed to produce standards against which centres could be measured and create a certification scheme to classify centres according to the level they had reached.

The internet gives access to a wealth of information on inherited bleeding disorders but it is spread over many sites and its accuracy can be difficult to verify. EUHANET aimed to set up a public website providing a single location for access to key information on haemophilia and other rare bleeding disorders for patients, their carers and health professionals.

Use of clotting factor concentrates to treat rare inherited bleeding disorders can result in unwanted side effects (known as adverse events) but because of the rarity of these disorders large studies involving many treatment centres are needed to monitor the safety of haemophilia care. Some countries run national schemes for reporting of adverse events related to treatment but these include treatment for any illness, not just for bleeding disorders. Safety monitoring studies carried out by companies who make clotting concentrates are often on a small scale and cover a short time period. Over 60 concentrates are in use in Europe and an independent system covering the whole of Europe was needed to monitor the side effects of treatments. EUHANET monitors the safety of treatments for inherited bleeding disorders through the EUHASS adverse event reporting system.

For the very rare bleeding disorders other than haemophilia, their extreme rarity causes problems with their diagnosis and management, due to either a lack of experience or knowledge, or because no specific concentrates have been developed to treat them. EUHANET aims to improve this situation by collecting information on the diagnosis, health and treatment of patients with the extremely rare bleeding disorders due to fibrinogen defects or deficiency of clotting factor XIII.

Achievements

EUHANET has devised and implemented a European Haemophilia Centre certification scheme. It has produced a standards document which is used to categorise treatment centres into two types: European Haemophilia Treatment Centre (EHTC) or European Haemophilia Comprehensive Care Centre (EHCCC). Centres apply for assessment using a standard form downloaded from the project website which collects details on key areas such as number of patients registered with the centre, services offered, emergency care facilities and laboratory backup. 115 applications from 30 European countries were received and were assessed by a committee which included patients, nurses as well as doctors. 90 centres were certified as EHCCC, 23 as EHC and applications from 2 centres were rejected as they were judged to have had too few patients to provide specialist care. A dynamic map showing all the certified centres can be found on the project website www.euhanet.org/MappedCentres.aspx.

EUHANET has created and maintains the Haemophilia Central website www.haemophiliacentral.org which holds key information about inherited bleeding disorders all in one place. The site is freely available to patients as well as health care professionals. The regularly updated news section provides news relating to inherited bleeding disorders, while the information section provides information on all the bleeding disorders in terms of inheritance as well as clinical features and available treatments. Information on clinical guidelines is also available as well as a list of all the clinical trials currently ongoing in the bleeding disorder field. A summary table from the adverse event reporting scheme (EUHASS) is included and this is updated automatically as new events are reported. The website contains information on how haemophilia care is provided to visitors in Europe and gives details on all the clotting factor products used in Europe. All doctor, nurses and physiotherapy national and international organisations in Europe are listed with their access details and information on all available quality control schemes is also provided. In a collaborative project with EAHAD, there is access to the databases of all the mutations in the FVII, VIII, IX and VWF genes which are fully searchable and freely available.

Haemophilia Central also has information on haemophilia centre locations and facilities and links to the Haemophilia Centre Locator www.hclocator.org, a user friendly search engine to locate European Haemophilia Centres. The Locator can be accessed from computer, tablet or mobile phone and gives the 5 nearest haemophilia centres for any address; it can show them on a map and displays address and contact information on how to obtain care from the centre. As well as Europe, the site includes Australia, New Zealand and Canada and will shortly cover the whole world. Patients with bleeding disorders travelling either inside or outside Europe now have a way of knowing where to go to access specialist care.

EUHANET has continued and extended the EUHASS adverse event reporting system which monitors the safety of treatments for people with haemophilia and other inherited bleeding disorders in Europe www.euhass.org. EUHASS has been expanded to include reporting of adverse events in acquired haemophilia, acquired von Willebrand disease and severe inherited platelet disorders. Quarterly and annual reports are circulated widely to all treatment centres, to professional and regulatory bodies and to the pharmaceutical companies. As well as reporting adverse events, centres also supply data at the end of each year on the number of patients registered with them and the number treated with each concentrate. This allows the annual reports to include analysis of rates of adverse events by disorder. Reports can also be produced for individual countries and for individual clotting factor concentrates.

As part of the EUHANET project the PRO-RBDD has been developed to collect information on the rarer bleeding disorders. A completely new web accessible database has been set up for centres to enter patient data and an international network of treatment centres has entered data on patients affected with the two most severe rare bleeding disorder, fibrinogen and FXIII deficiencies http://eu.rbdd.org/. Information was collected on the laboratory and clinical expression of disease and the effectiveness and safety of treatment. Genetic analysis of the factor defects involved and assessments of the quality of laboratory testing for the defects have also been carried out. These data will be used to improve the identification of affected individuals worldwide.

Expected benefits

EUHANET's achievements will benefit patients with inherited bleeding disorders, the health care professionals who manage them and the health care funders and pharmaceutical companies. Patients now have the tools to identify the nearest haemophilia centre wherever they are in Europe and can have confidence in the level of care provision by a centre, if that centre has taken part in the EUHANET European Haemophilia Centre certification scheme. Health care professionals are more connected with each other and have access to information sources which help them to deliver a higher quality of care. National health services now have a way of identifying which hospitals in their countries deliver haemophilia care and at what level. Finally the pharmaceutical industry is able to benefit from the EUHASS data which provides ongoing safety reports on their products.

The future

Overall, the EUHANET project was successful in completing its objectives and has brought together the different members of the European haemophilia community to work towards a single aim. These collaborations will continue the different areas of work of the EUHANET project. The patients' and health professionals' organisations (EHC and EAHAD respectively) will be working together to back the future of the project, by applying to the European Commission for recognition as a European Reference Network for inherited bleeding disorders. While this application is proceeding, the various work strands of EUHANET will continue independently. The European Haemophilia Centre certification scheme will be continued by EAHAD. The EUHASS project is the largest safety surveillance project internationally and has secured funding from the pharmaceutical industry for at least up to 2018. This also covers the continuation of the haemophilia centre locator website and the directory of clotting factor concentrates on the Haemophilia Central website. The PRO-RBDD will continue to collect data on fibrinogen and FXIII disorders and it is hoped to expand to other defects such as factor V and factor X deficiencies, depending on the level of financial support obtained.

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Further information

EUHANET website www.euhanet.org

Haemophilia Centre certification scheme www.euhanet.org/MappedCentres.aspx.

Haemophilia Central Website www.haemophiliacentral.org

EUHASS adverse event reporting system www.euhass.org

PRO-RBDD network http://eu.rbdd.org/

CHAFEA http://ec.europa.eu/chafea/

European Reference Networks for rare diseases
http://ec.europa.eu/health/rare_diseases/policy/index_en.htm