Haemophilia

This Guide offers an overview of haemophilia and outlines how GKA's unparalleled expertise could make a real difference to your research project

GKA has been conducting research in the haemophilia arena since 1992. As well as steadily building its expertise in the field, the fieldwork agency has built a panel of respondents which, given the small target population, is outstanding and guarantees GKA's clients access to many of the leading decision makers working in haemophilia.

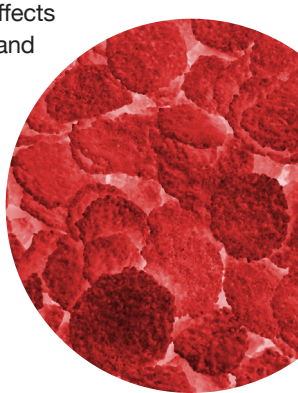
As part of its drive to fully understand the condition and its ramifications, GKA has built very strong relationships with the wider haemophilia community; from key opinion leaders to patients to parents. This Guide presents a short overview of the disease and shows why GKA could make a difference to your project.

The Lowdown

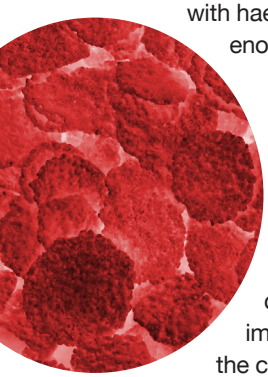
Haemophilia is a genetic condition which affects the body's ability to control blood loss or the coagulation process. Due to its hereditary nature, haemophilia is generally seen in men and it can affect people of any race or nationality. A boy can be born with haemophilia even though there is no family history of the condition – in such cases it is likely that the mutation developed spontaneously at some point in the boy's maternal line. If both parents carry the haemophilia gene, there is a possibility that a girl may be born with haemophilia.

Contrary to popular belief, haemophiliacs do not experience uncontrolled bleeding but instead tend to suffer from prolonged bleeding due to the body's inability to stop the bleeding process by clotting. In general, platelet cells, which are responsible for the clotting process, rely on specific proteins called clotting factors to stop bleeding. People with the mutated haemophilia gene do not have enough clotting factors.

There are two main types of the condition: haemophilia A, which affects 80% of the population and haemophilia B, which affects 20%[1]. However, depending on the level of clotting factor in the blood, it is also identified as mild,



moderate or severe. In haemophilia A, sufferers are lacking factor VIII while those with haemophilia B don't have enough factor IX[2].



In very rare instances, a person can develop rather than inherit the condition, which is known as acquired haemophilia. This is caused by the body's immune system identifying the clotting factor as a foreign substance and making antibodies, or inhibitors, to neutralise it.

Haemophilia has long been recognised as a distinct condition; cases were documented as early as the second century. However, it was not until 1940s that two distinct types were identified by Dr Pavlosky, a physician working in Buenos Aires[3]. Interestingly, at one point, haemophilia was termed the 'Royal Disease' after 10 of Queen Victoria's descendants, including her son Prince Leopold along with three of her grandchildren, were stricken with, and died, of the condition[4].

Incidence

In the UK, some 6,000 people, mostly males, have haemophilia. Worldwide, it is estimated that one in every 5,000 boys will be born with haemophilia A and one in every 30,000 with haemophilia B[5]. By contrast, acquired haemophilia is very rare and is only seen in 1-2 people in a million.

Haemophilia is usually diagnosed in early childhood when the infant is learning to walk and thus more susceptible to injury. However, some people with a mild form of the condition are not diagnosed until adulthood after they suffer a serious injury resulting in significant bleeding[6].

There are three types of bleeding in haemophilia:

- Mild: This includes injuries such as minor cuts and nosebleeds. They are not normally a problem and most individuals are well-practised at assessing and managing such bleeds.

KEY FACTS AT A GLANCE

- Haemophilia is an inherited or genetic condition that nearly always affects males
- There are two types of haemophilia: A (lacks factor VIII) and B (lacks factor IX)
- At least 10 of Queen Victoria's direct descendants died from the condition
- In the UK, some 6,000 people, mostly males, have haemophilia
- Treatment is either on demand or prophylactic
- The lower the levels of clotting factor, the more likely that so-called spontaneous bleeding will occur
- Women who carry the haemophilia gene may also experience some bleeding problems themselves, such as heavy periods

- Moderate: This covers joint and muscle bleeds. These are more serious and will need more care, including treatment with factor VIII or IX. Joints that are injured or affected repeatedly may become more susceptible to future bleeds.
- Severe: The most serious kind of bleed, this tends to occur following any kind of injury to the head, neck, throat or stomach. It is the most likely to require medical assistance.

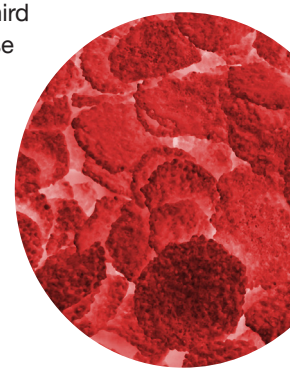
Bleeding may also occur spontaneously where there is no clear reason or injury. Although, the lower the levels of clotting factor, the more likely that so-called spontaneous bleeding will occur. Women who carry the haemophilia gene may also experience some bleeding problems themselves, such as heavy periods.

Treatments

The treatment of haemophilia depends on the severity of the condition. Those with mild haemophilia tend to use the treatment when needed, or 'on demand' such as before elective surgery or after a significant injury. In these cases, the treatment is either clotting factor or, in haemophilia A, a drug called desmopressin. This temporarily increases the amount of factor VIII in the blood.

People with moderate or severe haemophilia tend to be given regular preventive or prophylactic injections of clotting factor. Most sufferers will administer injections of the clotting factor themselves and may even have a permanent access point device through which to inject it.

Historically, clotting factors were extracted from donor blood plasma. However, before screening this led to viruses such as hepatitis and HIV/Aids being unwittingly transmitted into the haemophilia population. Although this stopped once screening became routine, scientists developed recombinant proteins or clotting factors. At first, these relied on human or animal-derived protein but the latest, third generation products, use no human or animal-derived proteins at all so the risk of transmitting infections has now been all but eliminated[7].



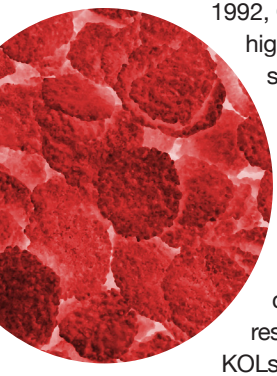
References:

1, 7: Haemophiliacare.co.uk
2, 6: BUPA
3: Hemophilia.org
4: Englishmonarchs.co.uk
5: NHS Choices

The GKA Difference

Fieldwork agency GKA has long since developed an expertise in the area of haemophilia. It has worked on some 80 haemophilia projects and, thanks to the depth of its knowledge, the team is now regularly asked to develop initiatives targeting this small, hard-to-reach target population.

Having worked in the area for over 20 years, it is no surprise that the team has an unsurpassed understanding of the condition and its ramifications. Since 1992, GKA has worked with high level haemophilia specialists and many of the leading of key opinion leaders (KOLs) active in the area.



Given that haemophilia is such a specialist condition, many of the respondents are typically KOLs, which is why the agency is able to continuously recruit new and fresh members to its panel. GKA has more than 700 respondents on its Haemophilia Panel, including:

- Over 500 Haematologists
- Nearly 200 Specialist Nurses.

It is important to note that while not all of these Haematologists and Specialist Nurses treat haemophilia, a high proportion of them work closely with haemophilia patients.

Furthermore, thanks to its heritage in haemophilia, GKA works alongside most of the patient organisations supporting the condition. Given the strong partnerships that have developed over the years, most of these groups are more than willing to help with patient recruitment.

This support, coupled with its partnership with a host of dedicated finders, means that despite it being a very complex area, GKA's patient recruitment ability in the haemophilia field is incredibly strong. It has also established a process for recruiting to, and conducting, ethnographic interviews with patients – a key selling point for many clients.

Given that most haemophilia projects rely on patient studies, this ability stands GKA apart from many of its competitors. By leveraging its strong relationships with both its finders and the patient organisations, GKA can recruit large numbers of validated patients across many different methodologies.

In short, GKA's experience of working on haemophilia market research projects is second to none. It regularly meets and overcomes the many challenges faced by fieldwork agencies and market researchers working in this area. The agency only works with healthcare professionals and patient organisations who really understand the challenges wrought by haemophilia, not just for the patients but also for parents, carers and even carriers of this genetic condition.

Call today to learn more

The GKA team is constantly building and maintaining strong relationships within the haemophilia community while developing its strengths in the therapy area. If you have been asked to run a research project in the haemophilia arena and would like to talk to someone who really understands all aspects of this condition, why not give us a call today?

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