Haemophilia 180

Taking control

A better future for younger people with haemophilia

The Haemophilia 180 report was fully funded and developed by Roche and Chugai, with support from The Haemophilia Society, Little Bleeders, healthcare professionals (HCPs) and people living with haemophilia. No compensation was paid by Roche and/or Chugai to the patient organisations or individuals who contributed to the report. The report is a valuable resource for anyone living with or working in the area of haemophilia. The personal views set out in the report are those of the contributors and do not necessarily reflect those of Roche and Chugai. The report can be downloaded from the websites of The Haemophilia Society and Little Bleeders.

This document was prepared in March 2018. Due to ongoing research and development the information in this report may change. To ensure you have the latest version of this document, contact the Roche PR team.
Job number: RCUKCOMM00263
Date of prep: March 2018

Haemophilia 180

CHUGAI

Roche

> Together we can achieve a better future for younger people with haemophilia.

Foreword

Baroness Meacher,
President of The Haemophilia Society

This is an encouraging time for people with haemophilia. There have been vast advances in treatment in recent years meaning that young people with haemophilia on the best treatment regimens rarely, if ever, need to experience painful bleeds that can lead to disability.

However this has not always been the case. Haemophilia has been associated with serious disability and has been a substantially life-limiting condition in the past. Furthermore, revolutionary new treatments in the 70s and 80s, instead of helping patients who were treated, caused them to be infected with blood-borne viruses. Many died and the rest were left with long-term health impacts.

Safety and effectiveness of treatments improved in the subsequent decades and, more than ever, the families of newly diagnosed children can look forward to a future where haemophilia will not hold them back from taking part in most sports and hobbies or following the careers they desire.

However, still not all younger people are getting the best support and treatment, particularly services like physiotherapy and mental health support. This report points to some of the reasons why not all younger people are seeing the outcomes you would expect in light of these improvements in treatment, and suggests changes which we can make to help change this.

I hope that policy makers and clinicians take up the suggestions in this report but most of all I hope that people with haemophilia and their families are empowered to take control of their treatment.

Together we can achieve a better future for younger people with haemophilia.

THE HAEMOPHILIA SOCIETY

Charity reg. no: 288260 in England
& Wales SC039732 in Scotland

Contents

Thanks and acknowledgements

This report was made possible thanks to all those individuals across the UK who have let their expert voices be heard, including people with haemophilia, carers, parents, clinical specialists and patient group representatives.

THE HAEMOPHILIA SOCIETY

Charity reg. no: 288260 in England & Wales SC039732 in Scotland

Introduction

For all teenagers, the transition to young adulthood can be challenging as they aim to establish their independence. For teenagers with life-long bleeding disorders, such as haemophilia, taking responsibility for managing their own treatment is essential. For some teenagers and families this transition is smooth and painless but for others it can be a very difficult period. [1,2]

To better understand these current and future challenges, this report has been created based on an extensive series of first-hand interviews with people who deal with severe haemophilia A on a daily basis. These people include teenagers, young adults, parents, carers and clinical specialists.

Advances in research and medical management over the past 30 years have dramatically enhanced the treatment, and thus the lives, of people with haemophilia A. More patients can now use 'prophylactic' replacement therapies to prevent and reduce their bleeding episodes. [3,4]

In addition, these replacement therapies are available as recombinant concentrates that are not made from human plasma. Recombinant clotting factors are made using genetically engineered clotting proteins grown in a laboratory. They have practically eliminated the risk of spreading blood-borne infections in haemophilia treatment. These recombinant therapies are also easy to store, mix, and use at home. [5]

However, people with haemophilia A in the UK still face several other challenges in terms of social attitudes and public knowledge of the condition, as well as inconsistent approaches to haemophilia care across the country. Improved medical treatment of haemophilia alone will not optimise quality of life. The wide range and changing needs of these patients and families will also require better local support from multi-disciplinary care teams who can provide tailored services including psychological, physiological and holistic support. [2,6]

Haemophilia A is becoming a manageable condition for many people if they are given the right treatment, education and support. The Haemophilia Society and Little Bleeders believe these advances now present the opportunity for a 180° shift in focus in haemophilia from the negative outcomes of the past to a positive, hopeful future for young people.

> These advances now present the opportunity for a **180°** shift in focus in haemophilia from the negative outcomes of the past to a positive, hopeful future for young people.

About Haemophilia

Haemophilia is a bleeding disorder where a protein made by the body to help blood clot is either partly or completely missing. This deficiency can lead to excessive internal and external bleeding that is difficult to control. Haemophilia is a genetic condition which is usually passed on from parents. [7]

There are two main types of haemophilia: [8]

Haemophilia **A** which is more common, is caused by a deficiency of clotting factor VIII (eight) in the blood. [8] Around 7,800 people in the UK have haemophilia A. [9]

Haemophilia **B** is caused by a deficiency of clotting factor IX (nine) in the blood. [5]. Only around 1,700 people in the UK have haemophilia B. [9]

Symptoms

Haemophilia mainly affects boys and men. Symptoms can be mild, moderate or severe, depending on the level of clotting factors that an affected person has in the blood, and include: [8]

A tendency to bruise easily, especially in early childhood

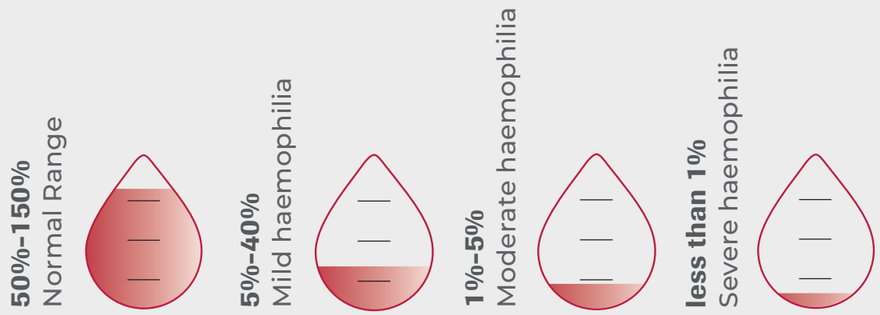
Excessive bleeding from cuts that take a long time to stop

A tendency to bleed internally into joints and muscles causing pain, swelling, damage and limitation of movement

In severe cases of haemophilia: continuous bleeding after minor trauma or even in the absence of injury (spontaneous bleeding)

Severity of haemophilia

Haemophilia is classified as mild, moderate or severe. The level of severity depends on the amount of clotting factor that is missing from a person's blood. [8]



Female carriers

Women who carry one copy of the faulty haemophilia gene are described as ‘carriers’. However, around a third are also affected by haemophilia and experience bleeding symptoms similar to men with mild haemophilia. They can also have symptoms that are specific to women, such as heavy or prolonged menstrual bleeding. In very rare cases women can have two faulty genes which will lead to more severe bleeding symptoms. [10]

Treatment approaches

People with haemophilia require life-long care delivered through access to a specialist haemophilia centre. [11] Although there is no cure for haemophilia, current treatments usually work well. Treatment mainly consists of replacing the missing clotting factor (replacement therapy) and preventing complications that are associated with the disorder. [12] This current treatment mainly involves two approaches: [12]

Preventative treatment (prophylactic or prophylaxis therapy) Where medicine is used routinely to prevent episodes of bleeding and subsequent joint and muscle damage. This involves regular injections of clotting factor medicine into a vein – normally every other day.

On-demand treatment In mild or moderate haemophilia cases, where treatment is used as an immediate response to episodes of prolonged bleeding. This treatment means clotting factor is used as required rather than to a schedule.

In some cases, especially young children, injections may be given into a device called a port-line or portacath, which can be surgically placed under the skin. This device is connected to a blood vessel near the heart, using a catheter, which is a long, thin, flexible tube to make injections easier. [12]

Most cases of severe haemophilia A require prophylactic therapy. Repeated internal bleeding without prompt treatment can damage the bones in a joint, leading to arthritis and disability. This damage can start in childhood, depending on the number and severity of joint bleeds. [7]

Besides being debilitating and life-threatening, haemophilia bleeds are also extremely painful. Therefore, it is important for people with haemophilia to have access to proper pain management. [13]

Rapid home based infusions

People can be trained to give medical infusions at home. This training is especially important for people dealing with severe haemophilia A because the infusion works best when given within one hour of a bleeding episode. The main advantage of this is that with regular infusions there isn't a need to come into hospital every time. In general, rapid treatment is important because it reduces pain and damage to the joints, muscles or other affected tissues or organs. [11]

A mother's insight into 30 years of management

Jan Dowsett is the mother of British racing cyclist Alex Dowsett

Alex, aged 29, was diagnosed with haemophilia A as a baby. Jan shares her own insight into the progress and challenges for today's families.

"The treatment and management of haemophilia for children, teenagers and families has improved enormously over the past 30 years. However, there are still many issues that need addressing. Becoming your own patient expert and educating non-specialist doctors are still very relevant today.

Years ago, we often had to wait many hours at the children's unit to get treatment from a specialist doctor because many junior doctors were very nervous about injecting babies/ toddlers intravenously. Knowing how to mix and inject clotting factor VIII is essential today for parents and patients.

The haemophilia nurses were brilliant in acting as our SOS resources, but newly diagnosed parents, especially mums with no family history of haemophilia, should be able to access counselling, something which we just didn't get. I certainly struggled to come to terms with the shock and realisation of what Alex had and how life might be for him. Rather than just telling children what activities they can't do, haemophilia centres should be telling them what sports they can do.

Everyone with haemophilia is different, regardless of their factor eight levels. Doctors should make it very clear that reaction time to a bleed is crucial, especially for the newly diagnosed patients."

Current and future haemophilia treatments

Factor replacement therapies

These are the mainstay of treatment for haemophilia. Concentrates of clotting factor VIII (eight for haemophilia A) or clotting factor IX (nine for haemophilia B) are injected into a vein. [5] Parents can learn to do this at home and ideally, a child will be able to do this themselves by the age of 9 or 10. These infusions replace the clotting factor in patients that is missing or low. These therapies can be developed from blood-donated plasma or recombinant (genetically engineered) concentrates. [12]

Extended half-life replacement factors

These therapies for haemophilia A and B may permit less frequent intravenous infusions and may also allow patients to maintain higher trough levels. A trough level is the lowest concentration reached by a drug before the next dose is administered, as determined by drug monitoring. Trough levels are used to make sure the amount of medication in the patient's bloodstream is still high enough to be therapeutic. [14]

Non-factor replacement therapies

Newer approaches to the management of haemophilia include treatments which are not made from blood plasma, but instead from proteins which replace the activity of the missing clotting factor or re-balance clotting capacity in people with haemophilia. [3]

Gene therapy

Researchers are also working on potential new ways to insert genes into the liver cells of people with haemophilia which allows their cells to produce the missing clotting factor. [15] At the moment, the way that works is to put the new gene inside a viral particle. These will generally migrate to the liver cells, where they start producing factor VIII and IX. This isn't the place that clotting factor is made normally: it is typically produced in the lining of the blood vessels (endothelial cells). Liver cells are very good at making things and secreting them into the blood. [16]

Inhibitors

Some patients develop antibodies against their factor replacement treatment, called 'inhibitors'. This means that the body creates an immune response to the factor replacement treatment and therefore prevents the clotting factor from functioning normally. [17]

Inhibitors are a serious complication of haemophilia treatment. They often result in more frequent hard-to-control bleeds, leading to an increased severity of the disease. At least 30% of people with severe haemophilia A will develop inhibitors, while this is the case for 1.5% to 3% of people with haemophilia B. [17]

Clinician's view:

Dr Pratima Chowdary, Consultant Haematologist,
Katharine Dormandy Haemophilia and Thrombosis Centre. Royal Free Hospital, London

5 ways we've improved care at our Haemophilia centre

"As clinicians, we are aware there are many gaps in current haemophilia care, and we don't have all the answers to these yet. Besides offering the most appropriate medical treatments, we're still learning about the best tools, services and resources to empower young people with chronic conditions. Ideally, every UK haemophilia centre would provide the same quality of care and services but, it often comes down to the limited resources some centres have. At the Royal Free Haemophilia Centre we have found five key ways to improve overall care.

1 2 3 4 5

Change behaviour with bleed history

It has been shown that patients accurately recall only about 14% of what they are told in a face-to-face consultation. What we find works well in educating patients is showing them a summary of their bleed history, such as their bleeds from 3 years ago, or their trough levels, compared with the total number they've recently had. These changes are often due to their own actions or inactions in controlling their haemophilia. Seeing this history seems to have the biggest single impact on getting patients to change their behaviour about recording their bleeds. This, in turn, has allowed us to have conversations about their treatment regimen.

Start a teen treatment routine

A lot of what you see in young people with haemophilia is an offshoot of their parents' approach to managing the condition. If parents set up a regular treatment routine for their children, then kids are more likely to follow this as they get older. You get a good chance to instil this when they're in their early teens. Instilling this in older teenagers is harder as they have different priorities. But once they've gone to university or got a job, many young people suddenly want more control over their haemophilia; see the benefits, and are more open to medical advice.

Offer physiotherapy advice

Over the past ten years, having a full-time haemophilia-dedicated physiotherapist at the Royal Free Haemophilia Centre has helped with patient wellbeing and confidence. Many young patients are keen to go to the gym, and they really appreciate the chance to ask a physiotherapist about their individual options for staying fit. Availability of physiotherapy support at other haemophilia centres varies across the country. Having the physiotherapist as part of our multi-disciplinary team enables us to provide informed, appropriate advice regarding various exercise routines, and this is greatly valued by patients.

Extend psychological support

We are fortunate to have a family therapist as part of our team which allows us to offer people psychological support. This gives them access to a service that is independent from the consultant or nurse. Not all people want to bare their entire lives to their consultant, so the psychologist can often provide some privacy to patients.

Pick up problems using checklists

We've also found checklists to be very useful in picking up incidental problems. We know patients are attending the haemophilia clinic because they have a complex, chronic condition. If you ask them directly if they have had any problems since their last visit, they are very quick to deny any, as in their judgement they may not consider these to be an issue. But when you ask them a checklist of around 15 questions, the discussion is more factual and often we identify some sort of related problem."

Educational Services

- Educate patients at first diagnosis & puberty
- Ensure patients visit their regional comprehensive care centre (CCC)
- Empower teenagers with awareness & confidence
- Increase psychological counselling services
- Inform young people of career choices

Treatment Services

- Guide patients to valid online resources
- Standardise multidisciplinary care & advice
- Increase access to physiotherapy advice/services
- Improve education for healthcare profesionals
- Listen to the expert patient
- Raise awareness of sexual health in haemophilia
- Expand public education to reduce stigma
- Assess patient reported outcome measures (PROMS) in haemophilia

New UK haemophilia goals

Haemophilia is becoming a more manageable condition for many people if they are given the right treatment, education and support resources. The Haemophilia Society recommends that new haemophilia goals should now be set for UK patients, parents, carers, healthcare professionals, NHS services, educational services, healthcare providers and policy makers who deal with haemophilia.

Taking steps towards achieving these goals will help all parties to establish and maintain a better future for young people with haemophilia. These goals are discussed throughout the rest of this report.

In the UK, people are treated at ‘haemophilia centres’, which is an umbrella term that includes around 70 care units across the country. [18] These are divided into two types: Haemophilia Treatment Centres (HTCs) and Comprehensive Care Centres (CCCs).

Ensure patients visit their regional CCCs

Generally, HTCs are smaller than CCCs. HTCs sometimes treat only a few people in the local area, which means that they often don’t have a complete range of specialist treatments and services on offer. CCCs are larger and have the resources to cope with more complex treatment issues, as well as routine care. Therefore, it is important to ensure that patients visit their regional CCC for regular reviews or that their HTC is part of a network of centres that works closely with their CCC. Currently there are 29 CCCs in the UK and about 41 HTCs. [18]

Standardise multidisciplinary care and advice

All people with haemophilia need a comprehensive care programme, with access to a multi-disciplinary team who can monitor each aspect of their care. [6] Comprehensive care is especially important when a child is first diagnosed with haemophilia, and when teenagers are transitioning to self-care of their condition.

Since haemophilia can affect various clinical, practical, physical and emotional aspects of a patient’s life, this professional care team should ideally include a medical doctor, a nurse specialist, a physiotherapist, a laboratory expert and psychosocial health experts. [6]

However, there are various inconsistencies in the quality and availability of multi-disciplinary teams at different HTCs and CCCs across the country.

Patients need standardised care at all haemophilia centres to limit the need to travel to other centres for better services. All centres should give people equal access to relevant therapy services, and information to help them connect to patient support groups like The Haemophilia Society and Little Bleeders.

Clinician’s view:

Dr Daniel Hart, Consultant Haematologist,
The Royal London Hospital Haemophilia Centre Trustee and Lead Clinician at Little Bleeders Patient Group

Empower teenagers for the future

“It only takes two to three bleeds for a child with haemophilia to develop premature arthritic joints, pain and disability later in life. So, in young people with haemophilia, our first clinical goal is to optimise prophylactic treatment to minimise bleed rates early on to reduce any musculoskeletal damage as children get older. This means optimising the current factor treatment agents we’ve got now and ideally using a future agent that takes them further away from their bleeding threshold. The patchy provision of multi-disciplinary services – for example, physiotherapy and psychology – in CCCs around the UK is wholly unacceptable. However, although patients want local care, it’s unrealistic to expect small HTCs to suddenly get up to speed with haemophilia services and knowledge.

1

Encourage awareness & active engagement

We can empower kids and adolescents in various key ways. First, initial education and open consulting styles by CCCs can help young people with haemophilia to have the confidence to ask questions about the condition in general. Secondly, awareness and active engagement with strong support groups like The Haemophilia Society or Haemophilia Scotland – which often run educational camps and events for teenagers – can enable them to go back and voice their views on what they need from their centre on a personal basis.

2

Build confidence & expertise

Although the UK has a lot more CCCs than countries such as Germany or Spain it’s educating people with haemophilia about how to optimally use those centres that matters. A young person with haemophilia needs to become an expert over and above most non-specialist medics they will meet outside their haemophilia centre.

It can be hard for people to advocate for themselves in the face of a clinician who might know very little about managing haemophilia. So, if they need medical attention, that’s when they need to know how to take action and get in touch with their CCC. Most CCCs will offer expert advice by phone 24 hours a day, 7 days a week. We can empower people to use this option by building their confidence. This is as important for those living with non-severe as those with severe haemophilia.

3

Assess PROMs in haemophilia

In the meantime, the NHS also needs to assess patient-reported outcome measures (PROMs) in haemophilia to assess the needs of and the quality of care delivered to people with haemophilia from their perspective. Many commissioners assume that because the NHS is paying for the clinical treatment, all other services should just fall into place, but they don’t. That’s where the inequality of access to physiotherapy, dental care, foot care and psychological care needs more attention.

4

Aim for a normal life

At Little Bleeders, our main aspiration is that kids living with haemophilia should be leading as normal a life as possible. They might not be playing rugby for England or boxing for Wales but ideally, they’re active and not bleeding, so they’re not missing school or physical education and are participating in education to the point where they can get what they want out of life and work. We want to continue to communicate this aspiration to kids as they’re growing up – through their school life, teenage and young adult years.”

Optimise positive support for teenage treatment adherence

Results from a 2017 psychological research study co-authored by Dr Daniel Hart showed that support from...

haemophilia treatment centres, families and educational services each play a positive role in encouraging teenagers with haemophilia to adhere to prophylactic haemophilia treatment. The study involved around 90 teenagers and young men at 16 haemophilia centres across the UK. [19]

"At least 80% of people with haemophilia stayed on prophylactic self-treatment with relatively little skipping or non-adherence. Many of them attributed their adherence to the support structure they received from their family and treatment centres," Dr Hart explained in a separate interview.

Greater treatment adherence by people with haemophilia was associated with stronger beliefs in the necessity of prophylaxis, stronger emotional responses to haemophilia (such as fear, anger or distress), more positive outcome expectations, more social support from their families, and being more satisfied with this support. [19]

> Many of them attributed their adherence to the support structure they received from their famliy.

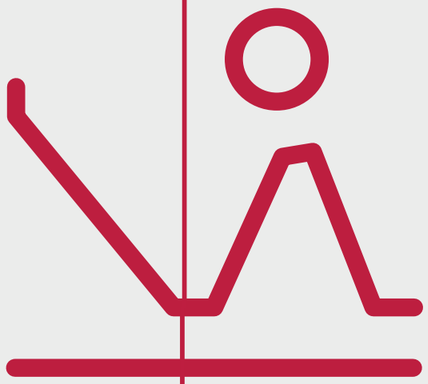
Build clinical trust with continuity of care

Continuity of care between haemophilia centres can help people to build trust in healthcare professionals. Some patients are calling for more "joined-up" care between HTCs and CCCs. Liam, who has severe haemophilia A, aged 23, and living in Fort William says, "I've had to travel to CCCs in Glasgow, Inverness and Edinburgh.

Every centre has a different way of doing things. [One centre] wants to be involved in everything I do, whereas [another centre] is more laid back. I rarely see the same doctor and have to keep explaining my haemophilia to each one at each centre."

Matt aged 28, lives in Dartford but travels to London for his regular care. He must take half a day off work to attend these appointments but feels this time is worthwhile."I'm more likely to trust the nurses and doctors at [my central London CCC] and listen to what they say. Even if the appointment is half an hour late, if you trust the people, it doesn't matter," he explains.

Strengthen local physiotherapy services



The musculoskeletal system is frequently affected by bleeding disorders. Physiotherapy is recognised as a way to improve quality of life, improve healing times, decrease frequency of haemophilia bleeds and to help prevent joint destruction due to multiple bleeds. [20]

Physiotherapists are specialists in the assessment and treatment of the musculoskeletal system. A physiotherapist is important not only as a treatment specialist but also as a consultant in the prevention and management of musculoskeletal impairments and disabilities. [20]

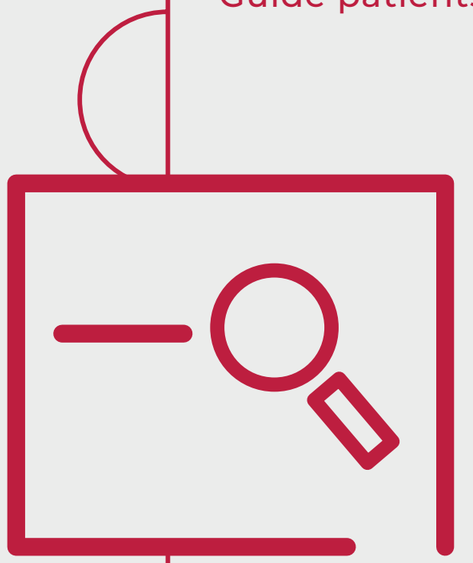
"Support for the musculoskeletal stuff seems to be non-existent in Scotland," points out Liam. "I've heard from people who've moved down south for physiotherapy care and found it's made a big difference. My doctor told me to stop playing the drums because it could damage my elbow joints. There's no physio expert I can ask for advice, so I've got no choice."

"We don't have a CCC in our area, so I had to ask for physiotherapy," says Hannah, whose 12-year old son has severe muscle weakness in his lower limbs. Following her request, her son now travels to London to attend a two-week residential centre for intensive physiotherapy rehabilitation and he is making good progress. "Other haemophilia centres need to start offering physio to teenagers," she emphasises.

Provide education at first diagnosis and puberty

People with haemophilia are also calling for better education for affected mothers, children and teenagers. This is especially important for young people as they adapt to self-care. “When you’re younger, you need better education about accepting haemophilia – it’s hard to accept you’re going to be excluded from things other children can do, and will experience physical pain,” says Matt, who was diagnosed at birth.

Guide patients to valid online resources



Jay, aged 20, attended an educational transition session as an older teenager, but received an aloof response. “I said I wished certain things had been explained to me when I was younger. But I just got told: ‘You should have asked for that information at that time’,” he recalls. “But why should I have to ask? Yeah, I can go to ten different web pages, all saying different things, but which ones do I believe?”

Research shows healthcare professionals are not the first source of support that patients turn to. Peer-to-peer support is very important for people with haemophilia. People find that sharing their personal experiences with other haemophilia patients and families online helps to improve their own mental and psychological health. People trust other people with haemophilia in website forums to give them valuable, practical information about living with and managing their condition.

For reliable online information The Haemophilia Society website is a good place to start: it provides a range of resources about haemophilia for families and young people. [22]

Better sexual education for LGBT teenagers

Jay, who is gay, stresses the importance of proactively...

providing better information about haemophilia and sexual health to people at puberty – including those in the lesbian, gay, bisexual and transgender (LGBT) community.

“As a teenager with haemophilia sexual health is a difficult topic to discuss, especially with doctors you don’t know or trust. It should be discussed openly with all young and LGBT patients.”

Mark, aged 49, also feels young people with haemophilia should be educated in personal safety and awareness which includes their sexual health at an earlier age. He says, “Some sexual activities can be high risk, as well as dangerous for causing bleeding or bruising throughout the body such as the throat, genitals, wrists and hips. As a teenager, I had no idea of the dangers, no role model, no information and certainly no one to ask for support or advice.

“If you start educating today’s young lads about these things now, then you empower them to take control of their own bodies, sexual and psychological health.” He notes that for many years, LGBT people with haemophilia have been ignored right across the haemophilia world. “Here we are in 2018, globally there is not one haemophilia LGBT Ambassador.” We all need to be treated equally, with better education and understanding which will tackle discrimination head on.”

Increase access to psychosocial counselling

In interviews, many young people and carers who deal with haemophilia are calling for access to more psychosocial counselling and holistic care services across the UK. While some haemophilia CCCs, PAGs and hospitals currently offer this type of counselling, it is not routinely available at all centres in the UK.

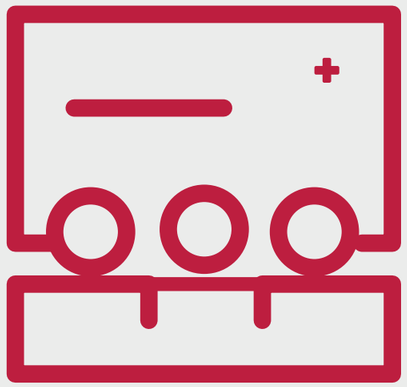
Mark says, "Social workers have helped me deal with my haemophilia emotional problems. I can talk to someone who understands – you feel more at ease. We need to start offering more psychological counselling to mothers and young people with haemophilia." Hannah adds: "There's a massive gap for emotional support for mothers from local centres. I was supported from a medical point of view, but not emotionally."

"You need a safe place to talk to someone about your emotional, sexual and lifestyle problems – not a medical judge and jury," says Jay, who lives in Edinburgh. "I finally got a chance to openly discuss my sexual health and haemophilia after being referred to a psychologist in Scotland, which was really helpful."

Educate healthcare professionals

In interviews with young people, mothers and carers, many people expressed frustrations around a lack of understanding about haemophilia from non-specialist healthcare professionals.

"I still find many of them seem quite unaware of haemophilia, which makes it a challenge to trust the judgement of certain medical professionals at times," says Bernadette. "Our local GP was reluctant to give my son his jabs as a baby so we had to travel to my local haemophilia hospital for these. At one point, I was sent home from a hospital whilst he was having a bleed and advised to manage it with Calpol. At that time I didn't feel confident enough to challenge the doctor due to the lack of my own experience, however I would most definitely do this now."



Harry's transition to prophylactic self-care

Harry, aged 24 was one of the first childen to receive...

prophylactic treatment with recombinant factor VIII, as part of a clinical trial. Harry's grandfather had haemophilia A, as does his younger brother. As a child, Harry was under the care of Great Ormond Street Hospital for Children (GOSH), which encouraged him to take a self-empowered approach.

"I've been self-treating since age 7," explains Harry. "Up until last year I played professional cricket and I still run and go to the gym. Great Ormond Street pushed me to play sport and gave me confidence. I remember the nurse teaching me about the biological side of haemophilia A."

Personal education helped boost transition

In learning to manage his haemophilia as an adult, Harry received educational support from Haemnet, which is an association that supports health and social care professionals to improve care for those with bleeding disorders. Harry found the educational programme useful but says: "There's no national standard way of transitioning teenagers with haemophilia; that's usually left up to the parents. This lack of education is a big hole for some people."

Don't drop off the radar

"A lot of haemophilia teenagers drop off the radar as they grow up – they have all this treatment as a kid, and then become an adult, and don't learn to continue this or look after themselves. Help is really needed for young people here. When I was younger I had some bleeds, so I knew exactly what it felt like and wanted to avoid them. That helped me become proactive with my treatment." Harry now takes prophylactic factor VIII daily. "I'm quite aggressive with it – if I feel a bleed coming on, I just double or triple the amount of factor – I basically treat it like a bruise." When Harry met his fiancé online the subject of haemophilia came up before they actually met in person, when they talked about family illness. "I've never been one to shout about haemophilia, but I also don't mind telling anyone," he recalls.

I don't see haemophilia as a problem

"I'm happy with my current treatment. I don't see haemophilia as a problem at all – I deal with it every day and can live life normally. Some patients have an issue doing the daily injections, so longer-lasting treatment would be good. My sister has just had a child with haemophilia and I know that she might not handle it in the same way I have."

Mothers still carry the burden

Parents of children recently diagnosed with haemophilia often experience feelings of denial, confusion, sadness, anger, guilt and fear for the future. These psychosocial effects can impact the whole family, including siblings. [21]

Bernadette's son aged 3 was diagnosed with haemophilia A at 7 weeks. She had no prior history of the disease in her family. She first took him to hospital at 5 weeks old, after he started screaming in pain when he couldn't move his right elbow. "Initial X-rays and blood tests showed no clear diagnosis so the obstetrician put it down as a soft tissue injury, but a week later he was still screaming," explains Bernadette.

After returning to the hospital for ultrasound and further testing the obstetrician told her he suspected haemophilia and sent her home with strict instructions not to google anything because the diagnosis was not confirmed. "I knew nothing about haemophilia. I thought it was just something you treated once and it went away. I was in shock and guilt when I found out online what it really meant," she recalls.

Bernadette now transfuses prophylactic treatment for her son every other day through an intravenous portline. "Although I use numbing cream, this injection is uncomfortable and undesirable for us both at times. I went through a few weeks of training with a nurse from the hospital where we had his portacath inserted, as well as with professionals from a local hospital before injecting him myself. I cried after giving him the first one, it was a very emotional experience."

"The good news is he's had no bleeds since starting this prophylaxis treatment," adds Bernadette, who is now more confident about managing the condition. When her son began nursery, a nurse came in to train the teachers; now Bernadette trains every new teacher he has.

A mother's advice

Remain positive and objective...

"Learning how to manage my son has been a real journey for all of us as a family. I've had to train myself to be emotionally and mentally strong," explains Bernadette. "Telling me not to google haemophilia on the internet was bad advice from doctors.

Of course you'll do your own research to find out how to help your child, but the results can be frightening." Her own advice to other parents of young children with haemophilia is: "Remain objective and positive. My son has haemophilia A but apart from that, he's still normal."

Learn more about haemophilia society events

The Haemophilia Society runs weekends away for parents with newly diagnosed children, which Bernadette recommends. [22] "We went for a couple of years and learnt a lot. Last year, after my son got the port line, we could share our experiences and reassure people who were very nervous because their sons had just been diagnosed," she says. "Because he hasn't had any recent bleeds, I'm now more in contact with various Facebook groups for any moral support, rather than the CCC staff."

Listen to the expert patient

Since haemophilia is a complex disease and can be very unpredictable, patients often become the experts about their own care. However, in many cases, some doctors do not listen to these opinions.

“With a disease you’ve got for life, you might as well become an expert to manage your own health, so I wish doctors would listen to me,” says Jay, “I might have had five bleeds since I last saw the doctor, but I won’t tell them because I don’t trust them, and know they won’t pass on this information to other haemophilia centres I visit.”

Inform young people of career choices

Many men with haemophilia say they have experienced employment-related challenges due to frequent time off because of bleeds or pain. They are also faced with a lack of understanding by employers about their condition. In some young men, the presence of psychosocial issues related to their haemophilia, such as personal anxiety, immaturity, lack of trust, and parental attachment, may also create barriers for them in accepting responsibility for building a career. [2]

With advances in treatment, PAGs and researchers have highlighted the importance of educating teenagers about training options and jobs, to find employment roles that are suitable for them. [2] The need to contribute to society by working is also important for adults with haemophilia and can have a positive impact on their self-esteem.

Roger, aged 69 said "I applied for various technical jobs associated with government when I finished college but experienced rejection after rejection because of my haemophilia. Employers just weren't willing to take the risk then which was incredibly frustrating, especially as I had the right qualifications and had access to good quality care via a Comprehensive Care Centre. Eventually I became a TV engineer and am still working now but back then I think many people with haemophilia faced the same barriers.

With better attitudes and treatments today there are more choices for young people empowering them to take control of their own futures."

New hope for paediatric patients and families

New advances in preventative haemophilia treatments are...

offering new hope for families with affected young children. “Two-thirds of boys under 12 with haemophilia A are still not bleed-free so there’s an urgent need for effective paediatric treatment.” explains Dr Daniel Hart, Trustee and Lead Clinician at Little Bleeders Patient Group.

“Improving paediatric treatment in haemophilia means more than just reducing bleeding. It’s about unshackling children and families from the risk of bleeds and joint damage to allow kids to lead a more normal life into adulthood,” notes Dr Hart.

“Ongoing clinical trials indicate that some potential new agents can significantly reduce the number of bleeds over time, compared to no prophylactic treatment, in children with haemophilia A,” he reports. These new treatments may also help to ease some of the burden of managing haemophilia for children and parents.”

Shake off the stigma

Starting in the 1960s haemophilia replacement therapies were originally made from human blood (plasma-derived products). Although they were effective, these blood products were not routinely screened for blood- borne infections at that time. In the late 1970s up to 1986, this lack of screening caused many people with haemophilia to become infected with HIV and hepatitis C. [3]

Many patients today still face the legacy of contaminated blood products. However, the routine use of advanced, synthetic replacement factor treatments, which are not developed from human blood plasma, has practically eliminated the risks of haemophilia treatment.

"You have to decide whether to let haemophilia define you or not," says Mark. "People still think haemophilia is akin to AIDS. There should be more public awareness that this is not true. This myth is holding young people back."

As such, PAGs and clinicians are calling for increased haemophilia education for employers, career counsellors, teachers and the public.

Empower yourself

With significant advances in the medical treatment and management of haemophilia, UK teenagers and young people with this condition can live long, healthy lives. Furthermore, by actively educating themselves and others about haemophilia, and taking control of their treatment during their transition to adulthood, young people can also empower themselves to seek out new opportunities and live more normal, productive lives.

Active education: key to better care for young people

With the right treatment, the right multi-disciplinary care, and the right education, haemophilia has become a more manageable condition over the past 30 years. The Haemophilia Society believes these various advances now present the opportunity for a **180°** shift in society's focus on haemophilia – from the negative outcomes of the past to a positive, hopeful future for young people.

Improved, active education and awareness programmes appear to be the key to producing and managing better overall outcomes in haemophilia care. These can help society in general and UK policy makers to understand the current facts, challenges and prospects for teenagers and young people with haemophilia.

> Improved, active education and awareness programmes appear to be the key.

Glossary

Factor: when followed by a Roman numeral e.g. factor VIII – describes one of the proteins of the body's clotting pathway.
Gene therapy: replacing, manipulating or supplementing a dysfunctional gene with a functional one.
Intravenous injection or infusion: delivering a medication directly into a vein using a needle or tube.
Plasma: the fluid component of blood, which carries the red blood cells, white blood cells and platelets, containing clotting factors in solution.
Port line (portacath): a small medical appliance that is installed beneath the skin, and can be used to give medicines, fluids, nutrients, or blood products over a long period of time. A catheter, which is a long, thin, flexible tube, connects the port to a vein.
Prophylactic/prophylaxis: a medication or a treatment designed and used to prevent a disease from occurring. In haemophilia, prophylaxis involves the regular infusion of clotting factor concentrates to prevent bleeding.
Recombinant clotting factors: genetically engineered clotting factor products, made in a laboratory using genetically engineered DNA technology. They do not contain human blood plasma.
Sporaic mutation: a condition that occurs in individuals with no history of the disorder in their family.
Trough level: is the lowest concentration of a medication in a patient's bloodstream before the next dose is administered; often used in therapeutic drug monitoring.

Abbreviations

AIDS (acquired immune deficiency syndrome): the syndrome that leads to a number of potentially life-threatening infections and illnesses that occur when your immune system has been severely damaged by the HIV virus.
CCCs: comprehensive care centres.
GOSH: Great Ormond Street Hospital for Children.
HTCs: haemophilia treatment centres.
HIV: (human immunodeficiency virus): a virus that damages the cells in the immune system and weakens the ability to fight infections and disease.
LGBT: lesbian, gay, bisexual and transgender.
PAGs: patient advocacy groups.
PROMs: patient-reported outcome measures.

Patient advocacy groups

Dedicated PAGs in the UK work to improve the lives of people with haemophilia, and are calling for better education, support and treatment.

The Haemophilia Society are the only UK-wide charity for everyone affected by an inherited bleeding disorder, made up of a community of over 6000 members, supporters and healthcare professionals. They provide trusted, evidence-based information and support at all stages of life. From childhood and adolescence, leaving home, right through to older age. They influence national policy to ensure the care and treatment of people and families with bleeding disorders is consistent, effective and accessible to all. They empower their community to be active in decision making about their lives, to ensure their voice is heard. http://www.haemophilia.org.uk/
Haemophilia Scotland are a charity that connects people with haemophilia and other bleeding disorders to each other for support. They provide a united representative voice when speaking with the medical profession, government, and Scottish society, and when connecting with people with haemophilia worldwide. https://haemophilia.scot/
Haemophilia Wales are a volunteer-run charity: all the trustees are affected by the condition. They provide information, support and advocacy and their mission is to ensure access to comprehensive care and to new treatments. They campaign for an acknowledgement and compensation for those people who were affected by contaminated blood from their NHS treatment. http://www.haemophiliawales.org/
Little Bleeders are a patient advocacy group established by professional British cyclist Alex Dowsett, who has haemophilia A. They provide guidance to young people and their families. They promote the message that, despite having a bleeding disorder, young people can still live active lives and participate in sport, particularly swimming and cycling. The charity recognises that sport helps builds self-confidence and self-esteem. They use the message: 'Move more, be more' to encourage this. http://www.littlebleeders.com/

Haemophilia care centres in the UK

Haemophilia care centres across the UK provide specialist care, diagnosis, treatment, support and information to haemophilia patients and families. Haemophilia treatment centres (HTCs) provide care for bleeding disorders, while comprehensive care centres (CCCs) provide resources to cope with more complex treatment issues, as well as routine care. HTCs are usually smaller than CCCs.

Haemophilia Treatment Centres

- NHS Grampian Haemophilia Centre, Aberdeen
- Abergavenny Haemophilia, Abergavenny, Monmouthshire
- Ashford and St Peter's Hospital NHS Trust, Chertsey, Surrey
- Alaw Unit, Bangor, North Wales
- North Devon Haemophilia Centre, Barnstaple, Devon
- Bournemouth and Poole Haemophilia Centre, Bournemouth
- Bradford Haemophilia Centre, Bradford
- Royal Sussex County Hospital, Brighton
- Chichester Haemophilia Centre, Chichester
- Colchester Haemophilia Centre, Colchester
- UHCW Haemophilia Centre, Coventry
- Royal Derby Hospital, Derby
- Dundee Haemophilia Centre, Dundee
- Eastbourne Haemophilia Centre, East Sussex
- Exeter Haemophilia Centre, Devon
- Medway Maritime Hospital Haemophilia Centre, Gillingham, Kent
- Hammersmith Hospital Haemophilia Centre, London
- Hull Haemophilia Treatment Centre, Hull
- Haemophilia Centre, Ipswich
- Raigmore Haemophilia Centre, Inverness
- James Paget University Hospital NHS Foundation Trust, Great Yarmouth
- Haemophilia Centre, Kettering, Northamptonshire
- Lancaster Haemophilia Centre, Lancaster
- Lewisham Hospital Haemophilia Centre, South London
- Lincoln Haemophilia Centre, Lincoln
- Luton and Dunstable NHS Foundation Trust, Luton
- Northampton Haemophilia Centre, Northampton
- North Midlands Haemophilia Centre, Stoke on Trent
- Norfolk and Norwich Haemophilia Centre, Norwich
- Haematology Department, Peterborough City Hospital, Peterborough
- Plymouth Haemophilia Centre, Devon
- Portsmouth Haemophilia Centre
- Salisbury NHS Foundation Trust, Wiltshire
- Shrewsbury & Telford Hospital (SATH), Shrewsbury
- St George's Healthcare NHS Trust Haemophilia Centre, South London
- Swansea Haemophilia Centre, South Wales
- Taunton & Somerset Haemophilia Centre, Taunton
- Truro Haemophilia Centre, Cornwall
- University College London Hospital
- Royal Wolverhampton Hospital Trust, West Midlands
- York Teaching Hospital NHS Foundation Trust, York

Comprehensive Care Centres

- Northern Ireland Haemophilia Comprehensive Care Centre and Thrombosis Unit, Belfast
- Children's Haematology Unit, Belfast
- Birmingham Children's Hospital Haemophilia Unit, Birmingham
- West Midlands Adult Comprehensive Care Haemophilia and Thrombosis Centre, Birmingham
- Bristol Haemophilia Comprehensive Care Centre, Bristol
- The Haemophilia Comprehensive Care Centre, Cambridge
- Kent Haemophilia and Thrombosis Centre, Canterbury
- Arthur Bloom Haemophilia Centre, Cardiff
- Haemophilia and Thrombosis Centre, Edinburgh
- Paediatric Haemophilia Comprehensive Care Centre, Glasgow
- Haemophilia & Thrombosis Centre, Glasgow
- Great Ormond Street Haemophilia Centre, London
- Leeds Haemophilia Centre (Children), Leeds General Infirmary, West Yorkshire
- Leeds Haemophilia Centre (Adults), St James's University Hospital
- Leicester Haemophilia Centre, Haemostasis & Thrombosis Unit
- Liverpool Paediatric Haemophilia Centre
- The Roald Dahl Haemostasis and Thrombosis Centre, Liverpool
- Manchester Haemophilia Comprehensive Care Centre, Adults'
- Manchester Haemophilia Comprehensive Care Centre, Children
- Newcastle Haemophilia Comprehensive Care Centre
- North Hampshire Haemophilia, Haemostasis & Thrombosis Centre
- Nottingham Haemophilia Comprehensive Care Centre
- Oxford Haemophilia & Thrombosis Centre
- Katharine Dormandy Haemophilia and Thrombosis Centre, North London
- The Royal London Haemophilia Centre, Whitechapel, London
- Department of Paediatric Haematology, Sheffield
- Sheffield Haemophilia and Thrombosis Centre, Adults'
- Southampton Haemophilia Comprehensive Care Centre
- Centre for Haemostasis and Thrombosis, Guy's and St Thomas' NHS Trust, Central London

References

1. The Haemophilia Society Annual Report (2016). Year ended 31st March 2016.
2. Brand B, Dunn S, Kulkarni R. Challenges in the management of haemophilia on transition from adolescence to adulthood. Eur J Haematol. 2015 Dec;95 Suppl 81:30-5.
3. Carr ME, Tortella BJ. Emerging and future therapies for haemophilia. J Blood Med. 2015 Sep 3;6:245-55.
4. Witmer C, Young G. Factor VIII inhibitors in hemophilia A: rationale and latest evidence. Ther Adv Hematolol. 2013; 4:59-72.
5. National Heart, Lung, and Blood Institute. How Is Hemophilia Treated? Available at: https://www.nhlbi.nih.gov/health/health-topics/topics/hemophilia/treatment. Last accessed March 2018.
6. Morfini M, Benson G, Jiménez-Yuste V, et al. Tailoring care to haemophilia patients' needs: which specialty and when? Blood Transfus. 2015;13:644-50.
7. World Federation of Hemophilia. About Bleeding Disorders. How do you get hemophilia? Available at: https://www.wfh.org/en/page.aspx?pid=644. Last accessed March 2018.
8. The Haemophilia Society. Understanding Haemophilia. Available at: http://haemophilia.org.uk/wp-content/uploads/2017/04/Understanding_haemophilia_WEB.pdf. Last accessed March 2018.
9. United Kingdom Haemophilia Centre Doctors' Organisation. Annual Report 2017 and Bleeding Disorder Statistics for 2016/2017. December 2017.
10. World Fedeation of Hemophilia. Carriers and Women with Hemophilia. Available at: http://www1.wfh.org/publication/files/pdf-1471. Last accessed March 2018.
11. NHS England. Haemophilia Service Specification 2013/14. Available at: https://www.england.nhs.uk/wp-content/uploads/2013/06/b05-haemophilia.pdf. Last accessed March 2018
12. NHS Choices. (2017). Haemophilia Treatment. Available at https://www.nhs.uk/conditions/haemophilia/treatment/.Last accessed March 2018.
13. European Haemophilia Consortium. Haemophilia. Available at: https://www.ehc.eu/bleeding-disorders / haemophilia/. Last accessed March 2018.
14. Young G, Mahlangu JN. Extended half-life clotting factor concentrates: results from published clinical trials. Haemophilia. 2016 Jul;22 Suppl 5:25-30.
15. National Hemophilia Foundation. Future Therapies. Available at https://www.hemophilia.org/Bleeding-Disorders/ Future-Therapies. Last accessed March 2018.
16. Imperial College London. World Haemophilia Day: Q&A with Professor Mike Laffan. Available at https://www.imperial.ac.uk/news/185746/world-haemophilia-day-qa-with-professor/. Last accessed May 2018.
17. European Haemophilia Consortium. Haemophilia. Inhibitors. Available at: https://www.ehc.eu/bleeding-disorders/inhibitors/. Last accessed March 2018.
18. The Haemophilia Society. What to expect from your treatment centre. Available at: http://haemophilia.org.uk/bleeding-disorders/treatment-centres/expectations-treatment-centre/. Last accessed March 2018.
19. van Os SB, Troop NA, Sullivan KR, Hart DP (2017) Adherence to prophylaxis in adolescents and young adults with severe haemophilia: A quantitative study with patients. PLoS One. 2017 Jan 19;12(1):e0169880.
20. Canadian Hemophilia Society. Standards of Physiotherapy Care and Assessment. http://www.hemophilia.ca/en/ care-and-treatment/physiotherapy/standards-of-physiotherapy-care-and-assessment/. Last accessed March 2018.
21. Cassis. FR. Psychosocial care for people with hemophilia. Treatment of Hemophilia. ResearchGate. No. 44.December 2007.
22. The Haemophilia Society UK. Home page. Available at: http://haemophilia.org.uk/. Last accessed March 2018.

Haemophilia 180

Taking control

A better future for younger people with haemophilia

We believe advances in haemophilia now present the opportunity for a 180° shift in society's focus on haemophilia – from the negative outcomes of the past to a positive, hopeful future for young people. By actively educating themselves and others about haemophilia, and taking control of their treatment during their transition to adulthood, young people can also empower themselves to seek out new opportunities and live more normal, productive lives.

Haemophilia 180 - 8 calls to action for haemophilia care in 2018

Improvements in educational services:

1. Setting new haemophilia goals – people with chronic diseases are more likely to reach goals that they pick themselves. Patient groups and Healthcare Professionals (HCPs) can help and encourage young people to select their own haemophilia goals, such as learning to self-infuse their treatments, educating their family and friends, getting physiotherapy advice, reducing their risk of bleeds, and using treatment checklists.

2. Providing education at first diagnosis and puberty – better information and education can give families and young people more confidence about managing haemophilia. This should also include better sexual education for young people in the lesbian, gay, bisexual and transgender (LGBT) community.

3. Improving education for healthcare professionals – continuing to learn more about haemophilia is important to help GPs and non-specialist doctors improve diagnosis. Better education can also help to make sure patients are referred to regional CCCs for holistic treatment.

4. Empowering young people – encouraging teenagers to take control of their haemophilia care as they become an adult can empower them to seek out new opportunities and career paths, and live full, productive lives.

Improvements in treatment services:

5. Access to expert care – the specialist care provided at regional comprehensive care centres (CCCs) is especially important when a child is first diagnosed and when teenagers are learning to self-care for their condition. So, all families and patients should have equal access to their CCCs.

6. Multidisciplinary care – haemophilia care should include advice from physiotherapists and psychological counsellors to manage physical and emotional problems. Experts now encourage people with haemophilia to take part in non-contact sports. Regular exercise can help to prevent bleeds and protect joints, as well as maintain a healthy body and mind.

7. Increasing access to treatments – haemophilia treatments can help to prevent a bleed in the first place, rather than treating a bleed after it occurs. These preventative treatments can also reduce the risk of internal bleeding and minimise the development of premature arthritis and disability problems.

8. Listening to patients and carers as the experts – haemophilia is a complex disease and can be very unpredictable in each individual. People often become experts on their own condition, which means it's essential for HCPs to ask each patient/carer about their opinion when deciding upon haemophilia care.

The Haemophilia 180 report was fully funded and developed by Roche and Chugai, with support from The Haemophilia Society, Little Bleeders, healthcare professionals (HCPs) and people living with haemophilia. No compensation was paid by Roche and/or Chugai to the patient organisations or individuals who contributed to the report. The report is a valuable resource for anyone living with or working in the area of haemophilia. The personal views set out in the report are those of the contributors and do not necessarily reflect those of Roche and Chugai. The report can be downloaded from the websites of The Haemophilia Society and Little Bleeders.

This document was prepared in March 2018. Due to ongoing research and development the information in this report may change. To ensure you have the latest version of this document, contact the Roche PR team.
Job number: RCUKCOMM00263
Date of prep: March 2018

Roche UK PR team:
09:00-17:30: welwyn.pr_pharma_uk@roche.com
01707 367807
For after office hours: 01707 366000

Haemophilia 180