Community Matters

Autumn 2022

The Haemophilia Society

More Haemfest 2022 photos inside

The future of ageing

Plus a closer look at pain, gene therapy, and more

News on your delivery

We know just how much many of you rely on the timely and efficient delivery of treatment to your home, and how much stress and anxiety can be caused if this service goes wrong.

This is why we were heartened that the Haemophilia Society (THS) was invited to join a panel of senior decision makers to help represent the patient's voice in scrutinising the tenders of the companies who bid to perform this home delivery role on behalf of your local centre, hospital or trust.

We are pleased to report that from 1 July 2022, a wider range of home delivery service providers has now been listed for centres, hospitals and trusts to choose from. Your healthcare organisations will have the information to allow them to easily compare providers and they will also be in a better position to switch providers if they don't feel the company is delivering for their patients, which has happened in the past.

Do keep us informed, but hopefully this will ensure a better service for everyone. •

The thorny issue of waste

We are often contacted by members who have unused treatment or equipment that they don't want to see wasted.

Unfortunately, this is a difficult issue to solve. Factor should never be shared directly with someone else to use. Clinicians need to know how much factor you are taking and this must be accurately monitored and recorded through Haemtrack. If there was a batch recall, clinicians need to know who has taken the treatment for their safety. There are also health risks, for example someone taking donated factor could have a life-threatening anaphylactic reaction.

Donating factor to developing countries in desperate need is something that THS has tried very hard to organise, but at present this is unworkable. Exporting prescribed medicines involves a huge amount of paperwork and regulations, including being able to guarantee that they are stored correctly, at the right temperature for the entire journey.

Like you, we hate the thought of wasting factor and are still working to find a solution, but currently our best advice is to contact your centre about how to dispose of unneeded medication or equipment. •

World Federation of Hemophilia World Congress 2022

The World Federation of Hemophilia World Congress returned in May 2022, after nearly four years, in Montréal, Canada. Hundreds of representatives from national member organisations and the global healthcare and scientific research community reunited to share experiences and to learn about the latest developments in bleeding disorders treatment and care.

THS sent a small group to attend the congress in person. Over the course of seven days, we took part in important discussions around women and girls and ageing with a bleeding disorder, as well as attending seminars on best practice treatment and managing stress and anxiety alongside a bleeding disorder. Our Chair, Clive Smith (pictured below with our Youth Ambassdor Jess Page) and Chief Executive, Kate Burt, also presented an update on the Infected Blood Inquiry.

Commenting on the event, former THS trustee, Barry Flynn, says, 'My main takeaways are that we are still years away from a safe, permanent cure for haemophilia A, that social and religious taboos in various regions around the world are huge barriers to adequate diagnosis and treatment of people with bleeding disorders and to quality of life, but that humanitarian aid programmes like Cornerstone are vitally important in low-income countries and do work.'

As ever, we are inspired to continue working as hard as we can to ensure that everyone living with or affected by a bleeding disorder is fully supported and has the access to safe and effective treatment that they need to live life to the fullest. •

Don't stop me now

Kelan received his Motorsports UK driving licence on his 18th birthday.

This was no small feat, as Kelan has severe haemophilia A, but he and his dad Mark were surprised by how accommodating Motorsport UK were, once they'd discussed the issues. Having gone through quite a lot growing up, Kelan is mature for his age, and is ready to take the next steps to pursuing his long-held passion, rally car racing.

Now aged 19, Kelan is a busy man, studying graphic design, working at MacDonald's to finance himself, and being sponsored as a competitor in sophisticated car simulator rallies, with the equipment stationed in the family's conservatory, in Gloucestershire.

Kelan already has experience as a co-driver and driver in real life too (see Kelan and his dad, Mark, above) and hopes one day to be racing as part of a team, with his car's livery branded with a reference to his haemophilia. He says, 'My condition is just part of me and my identity, whether I want it or not, so there's no point shying away from it.' He's even designed a tattoo of an infusion kit for his forearm, which he blackly jokes might come in handy if he's ever unconscious!

Of course, some risks are inherent in rally driving, but Kelan's family have weighed them up, and work around race days with factor in advance. Kelan infuses every other day, but because he had inhibitors for 18 months as a child, he is wary of new treatments. Mark says, 'Despite the big appeal of less frequent treatments, at the moment, we know where we stand. Kelan has looked after himself and is brilliant, never ignoring his care.' Kelan agrees, saying that although some days it's a drag, and his dad helps, he's used to it.

Kelan and Mark say that meeting fit, cool, and very successful sports people with haemophilia like champion cyclist Alex Dowsett has really inspired them to think big. 'For me', says Kelan, 'it's about pursuing a dream and not letting haemophilia stand in your way.' Good luck, Kelan! •

New look AGM & events

This year our AGM will be different and we hope more beneficial to you.

An AGM is a good opportunity to reflect on the year and future plans. However, it's becoming increasingly difficult to meet everyone's diverse interests in the afternoon sessions. Additionally, attendee numbers have been declining over the years, with the pandemic accelerating this trend.

As a result, our AGM this year will solely focus on more formal aspects, such as reviewing last year's financial results, and voting in new trustees. The AGM will be on 19 November in London. You are very welcome to attend, or you can watch and ask any questions online via Zoom.

To replace the afternoon sessions, we are planning four, separate, day-long events around the UK. They will provide more opportunities for people to meet, and more relevant, in-depth talks.

If this works well, we intend to rotate events across the UK, making it easier for members to come along and feel part of the bleeding disorders community, wherever you happen to live.

Please get in touch if you have any comments, and we hope to see you at one of the events soon. •

Events

Haemophilia Live – 1 October 2022, Nottingham. News, discussion and debate for people of all ages affected by haemophilia.

VWD Live – 28 January 2023, Southampton. A day devoted to von Willebrand disorder (also known as von Willebrand disease or VWD).

Rare & Unclassified Bleeding Disorders – 25 February, London. A chance to get together and also hear expert speakers.

Talking Red Live – 4 March 2023, Oxford. Bringing together women and girls with a bleeding disorder. Partners and family members welcome.

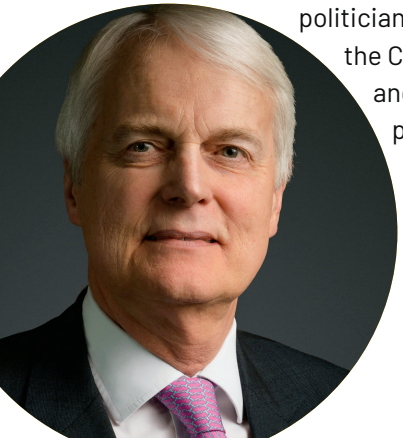
Infected Blood Inquiry

Final stages of inquiry hearings

The Infected Blood Inquiry is now entering the last stages of taking evidence.

There are six weeks of oral evidence remaining, beginning on 12 September, followed by three weeks of submissions from legal representatives on what conclusions their clients believe inquiry chair, Sir Brian Langstaff, should reach. We don't know how long it will take Sir Brian to write his final report, but it seems likely to be published in mid-2023.

This year, evidence has focused on the technical side of blood fractionation as well as on political decision makers, including civil servants and politicians from both the Conservative and Labour parties. The inquiry has taken evidence from nine former health secretaries, spanning the period 1981 to 2021, as well as former Prime Minister, Sir John Major.



The inquiry has explored the issue of missing Department of Health (DoH) documents, particularly relating to former health minister Lord David Owen's papers on decisions relating to self-sufficiency in blood products in the 1970s as well as the destruction of documents from the Advisory Committee on the Virological Safety of Blood between 1989 and 1992 – the period in which the DoH faced legal action from people with haemophilia infected with HIV.

Remaining witnesses include former health ministers, a journalist who broke the story of 'killer blood' in the UK in May 1983 and civil servants from DoH.

If you have any questions about the inquiry, contact our team at publicinquiry@haemophilia.org.uk. •

Interim compensation will be paid

Interim compensation of £100,000 will be paid to everyone currently registered on a UK infected blood support scheme.

After decades of campaigning from the Haemophilia Society (THS) and many others, the government in the UK has for the first time accepted that compensation should be paid for infection and suffering caused by contaminated blood and blood products.

The payments will go to those infected and bereaved partners who are registered on a UK support scheme. We hope this is the first step towards full compensation for everyone who has suffered devastating loss.

The issue of compensation dominated the Infected Blood Inquiry this summer, resulting in its Chair, Sir Brian Langstaff, making an early recommendation that interim compensation should be paid 'without delay'. In August the government announced that it would implement Sir Brian's recommendation.

Kate Burt, our Chief Executive, said: 'While welcoming this news, there is still a long way to go in ensuring that all those who have endured devastating loss and suffering – such as bereaved parents and children – are recognised and compensated. Steps must be taken now to set up a workable scheme which can deliver full compensation quickly and fairly.'

In June, the government published a report it commissioned by Sir Robert Francis QC, pictured, which looked at a framework for compensation. Sir Robert concluded there was a 'compelling case' for interim compensation to be paid to the infected. We continue to wait for the government's response to this report. •

> **'Steps must be taken now to set up a workable scheme which can deliver full compensation quickly and fairly.'**
> **Kate Burt, Chief Executive**

Bridge Walk is back

If you want to get to know a city properly, see it on foot. And if you really want to get to know a city, walk across all of its bridges!

Our three Bridge Walks take place this September in the late summer sunshine (hopefully!) and are a unique day out. The atmosphere, walking with others for a common cause, is so warm and friendly. You will also bask in the glow of knowing that you are raising money all the way.

This year, as well as bridge walks in London and Glasgow, we have added Derry in Northern Ireland. What Derry lacks in quantity of bridges, will surely be made up for by beautiful views of the River Foyle!

So why don't you join us on one of the walks? We can give you lots of encouragement as well as fundraising tips. For example, you don't have to ask for sponsorship, you can hold a cake sale or car wash instead. Or would your employer match whatever you raise?

Big Red Bridge Walk London – 17 September

Spend a memorable afternoon crisscrossing eight of London's (the world's?) most iconic bridges. The route map covers around 14km but you can choose your own distance, pace and route.

Big Red Bridge Walk Glasgow – 24 September

Another eight-bridge challenge, weaving your way back and forth across the Clyde on charming bridges which reveal the river's fascinating history. The route map covers around 14km but you can choose your own distance, pace and route.

Big Red Bridge Walk Derry – 1 October

Just three bridges to cross, but the route takes in the Derry Walls, Guildhall and St Columb's Park. Around 14km, if you choose the full distance.

See haemophilia.org.uk/events/events-calendar/ •

If these set dates aren't for you...

...you can work at your own pace by taking part in the 36 Fitness Challenge throughout September. Choose your own challenge in the spirit of '36', to represent the 36,000 people in the UK with a bleeding disorder. For example, walk 3.6km or do 36 press-ups a day.

A priceless walk in the park

Lockdown meant that lots of children with bleeding disorders weren't able to benefit from mixing and sharing experiences with other families as usual.

Deciding to do something about it when circumstances allowed THS Trustee, Natalie Lawson, a paediatric nurse at Birmingham Children's Hospital, and Amy Owen-Ward, THS Trustee and local parent, organised a fantastic family event in Cannon Hill Park, Birmingham. Based on the Race Around the World fundraiser, families completed laps around the lake, picnicked, and played sports together.

Apart from raising a smashing £2,000, the aim of the event was to end the isolation that many families had been feeling and the day was a success on this front too, with many families coming down to meet each other and have fun. One little girl told her mum before the event that she hadn't known that there was anyone else 'like her' with her condition, making the day absolutely priceless.

A special mention

Every year the Warren family from Devon gather friends and family to do a running challenge to raise money and awareness, which is all the more incredible as they have had lots of challenges managing their four-year-old daughter's type 3 (severe) VWD.

This year they organised a 'bleeding marvellous half marathon' with 12 people setting off, and nine running the full distance (including two who only planned to run half the route but who were pulled to the finish by the atmosphere!). Amazing work, thank you. •

A closer look at pain

It is increasingly recognised that pain, how we experience it and how it affects our lives, is very individual.

Lifestyle, physical and emotional trauma, current or past, can all come into play when assessing someone's pain. It takes time and effort, but this whole-patient approach is what we should aspire to, argues Anna Wells, Advanced Practice Physiotherapist in Haemophilia, at Hampshire Hospitals NHS Foundation Trust.

'Our understanding of pain is always evolving', continues Anna. 'We know that severe physical changes on an MRI scan does not automatically mean that someone has high pain levels, and someone else with less severe changes may experience higher levels of pain.

Pain is a multidimensional experience. For people with bleeding disorders, pinpointing joint damage as the single cause of pain is too simplistic. It won't be the only thing affecting that person's experience of pain.

'It's really important to look at not just how someone's pain affects their life, but how their life affects their pain. What are the aggravating factors? When do they feel better? We know that if someone has high anxiety or depression, they will likely feel increased pain levels, so taking both a physical and psychological approach will give a better understanding of pain.

‘What does living well look like and how can we get more of this?’

‘Exercise is absolutely very helpful, but it can be quite off-putting to be told to exercise if you are in pain. It doesn’t have to be swimming or walking though, it is about gradually increasing your activity. Specific exercises might help if the goal is to build up strength, so that you can do the activities that you really enjoy and live well.

‘I’d encourage people to take control of their healthcare. You are the expert. Work in partnership with your healthcare team. Look at the strengths and positives. What can help with the pain? What skills do you have for managing pain? What does living well look like and how can we get more of this? What are your goals?

‘As a first step, ask to speak to a member of your team about your pain. There may be alternatives to pain killers, surgery and injections. Physios and psychologists can be helpful to dig deeper into the causes of your pain, so find out what’s available to you.’

It’s clear that we need to see a different approach, involving more support, more tailored care, and more options on how to cope with pain, alongside medication. Activity, physical and psychological therapies, and support groups which can stop people feeling isolated, can all play a part. Pain is complex and is best managed with the support of a multidisciplinary team, with the patient right at the centre. •

Tell us your views...

...about your care, so that we can better campaign on your behalf.
Visit www.haemophilia.org.uk/PIS

Community Matters

Haemfest 2022


The Haemophilia Society
Lincolnshire & East Midlands
Together for life
We are the only UK-wide charity for everyone affected by a genetic bleeding disorder. Our community is here for you.
Find out more at haemophilia.org.uk
Contact us on 020 7939 0780
Or email info@haemophilia.org.uk
Being there matters
When times are tough, we are each other's anchor
We had a wonderful time at Haemfest 2022 in Derbyshire, with lots of conversations, games and laughter. Thank you all for coming and making it such a great weekend.

The future of ageing

Average life expectancy in the UK is 79 for men and 83 for women. For people with bleeding disorders and adequate treatment, this should in theory be no different.

'The bleeding disorders population is expanding and growing older, as in a sense, we have become victims of our own success. Yet how will haemophilia centres meet the challenge of managing larger total numbers of patients, especially those who are older? Managing the consequences of ageing in this community is a vastly under-researched issue,' explains William McKeown, our Access and Service Improvement Ambassador.

William is a doctor specialising in the care of the elderly and stroke medicine. He also has severe haemophilia A.

'We know that issues associated with growing older, such as falls and poor mobility, are best managed by multidisciplinary teams which include not just doctors and nurses, but also physiotherapists, occupational therapists, and social workers. Unfortunately, right now access to these essential services varies, with patients facing a postcode lottery. All centres need dedicated multidisciplinary teams in place soon if the effects of ageing in people with bleeding disorders are to be properly managed. Many people ageing with haemophilia also have the additional complications of living with hepatitis C and/or HIV.'

‘Unfortunately, right now access to these essential services varies, with patients facing a postcode lottery.’

‘Of course, there are many common health issues that affect people with bleeding disorders and the general population equally, such as heart disease and strokes. Yet when someone with a bleeding disorder is referred to, for example, a cardiologist, the consultant is less likely to have specialised knowledge about bleeding disorders. A particular issue with heart conditions and strokes is that they are usually treated with blood thinners, which are a risky prospect for people with bleeding disorders. This puts a lot of pressure on the patient to advocate for themselves. For me, it’s critical that haemophilia care centres should pair with other individual specialists, such as in cardiology, so that knowledge is pooled. We see this already in areas such as obstetrics, but this approach should be expanded.

‘Many people also worry how they will manage their treatment as they get older. Veins get worse, cognition and hand dexterity may decrease…all these things may make self-infusion difficult. We need to consider how we facilitate this if the person doesn’t have a family to help or if they are in a care home, for example. A line can be used, but this may bring its own problems. Emicizumab is another option, although we don’t have as much evidence about how this works in an older population.

‘Soon we will have a large elderly population with haemophilia or other bleeding disorders, and we don’t yet know how best to manage them. As a community, we need to be on the ball, use our patient voices, and encourage stakeholders and researchers to look at this challenge, as currently our ageing is being managed less well than other populations.’ •

In the meantime…

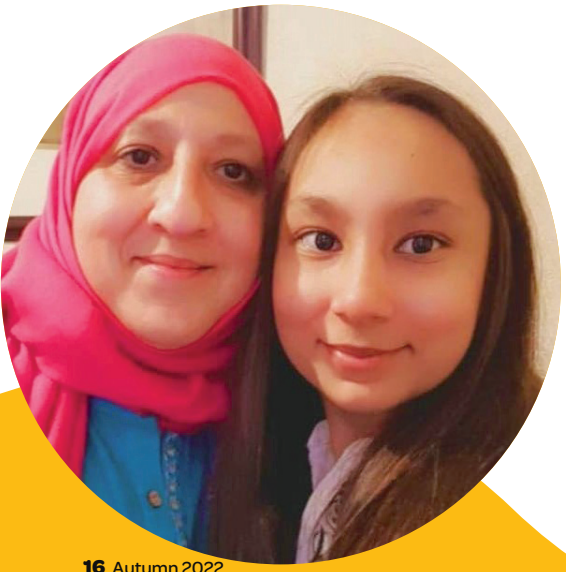
…the advice for successful ageing in people with bleeding disorders remains similar to that for the general population; a healthy diet, limit alcohol, don’t smoke, and exercise. For good joint health, it’s especially important to exercise or keep active, if possible, as we know strength around the joints can prevent bleeds.

This is real life not a text book

When Zaynab was diagnosed with factor VII deficiency at birth 21 years ago, life for her family changed forever.

The youngest of three children, Zaynab is the only one of her siblings to have a bleeding disorder and her diagnosis was a complete shock.

She suffered with a lot of nosebleeds and needed treatment at hospital three or four times a week until her mum Azra learnt how to inject Zaynab when she was a toddler. Azra had to rely on extended family so she could give Zaynab the extra support she needed, which included attending most of primary school together, where she became an additional teachers' support in the classroom so she could be present to help Zaynab.



Azra said: 'Zaynab became my priority. Primary school was an amazing support. All the children knew about Zaynab and were very caring, they would come and get me if there were any problems.'

But when Zaynab moved from care at her local paediatric centre to the adult centre four years ago, she was unprepared for how different it would feel.

She said: 'Before, all the nurses and consultants knew me and my family, but now that I'm at the adult centre no one is really interested in all that. I don't feel my consultant knows me as a person – I'm just results on a piece of paper for him.'

Both Azra and Zaynab say they have had to learn to be thick-skinned at times,

> **‘There needs to be a mutual understanding and the knowledge of parents and carers should be listened to.’**

in trying to ensure clinicians listen to their views.

Azra would like to see more value placed on the experience of parents and carers, with the introduction of parent forums and better support for people who do not have English as a first language.

She said: ‘The clinicians are the professionals, and I’d never put myself above them, but there needs to be a mutual understanding and the knowledge of parents and carers should be listened to. This is not a textbook, this is real life and it’s important consultants understand their patient’s world.’

Zaynab, who is studying biomedical science at university, felt especially isolated because she didn’t know any other girls with a bleeding disorder. When she attended Talking Red Live this year in York, which is an event organised by THS to bring together women with bleeding disorders, she finally met others in the same position. She said: ‘At Talking Red it was great to meet other women with haemophilia. I talked to one person about how to self-inject, which I’ve always struggled with, and she gave me some really useful advice.’

Although Zaynab now takes control of her own care, Azra can’t see her support role ending any time soon. She said: ‘Zaynab knows I’m on board 24/7. She is my priority, and always will be.’ •

Get involved!

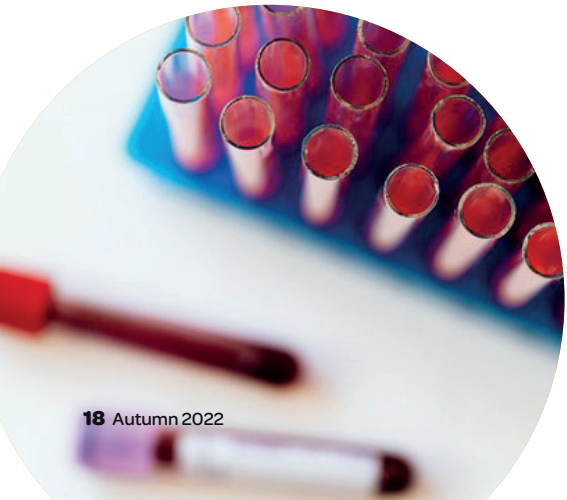
Talking Red is the Haemophilia Society’s campaign to raise awareness about women’s bleeding disorders and to bring together women to share experience and knowledge. Join the debate on our Talking Red Community Facebook page.

Gene therapy progress report

Gene therapy for haemophilia works by using a viral vector to deliver a transgene to your liver cells. This allows them to start making the missing clotting factor you need.

The first gene therapies for haemophilia A and B are expected to be licensed within the next year, but it is still unclear how, when or even if they will be made available to people with haemophilia in the UK through the NHS.

CSL Behring/Uniqure's haemophilia B Gene Therapy EtranaDez (etranacogene dezaparvovec) has already begun the process of being considered by NICE, while BioMarin's haemophilia A gene therapy, Roctavian (valoctocogene roxaparvovec) is not far behind. They are likely to be the first gene therapy treatments for haemophilia licensed in the UK and available to be considered for commissioning by the NHS. Over the next few years, more will follow. Here we will take a look at what we know so far from the latest trials of these and other products.



Factor Levels and Variability

EtranaDez has recently published results which show average FIX levels of 39% after six months, 41.5% after one year and 37% after 18 months. However, the results after one year showed some variability between people on the trial. While most people had levels between 30% and 50%, results ranged between 10% and over 100%.

Other haemophilia B

gene therapies on the horizon include products from Pfizer/Spark, Freeline, another UniQure product and Sangamo.

Durability

The clotting factor level achieved with some gene therapies, particularly those for haemophilia A, appear to drop off over time. For example, the Biomarin Roctavian Phase 1/2 trial showed that median factor levels dropped from their 60% peak in year one to 26% in year two, 17% in year three, 14% in year four and finally 8% in year five of the study. Similarly in the phase 3 study, the latest data showed the median % at the end of year one was 24% and at the end of year two was 12% as measured by chromogenic assays.

Early data on SPK8011, Roche/Spark's haemophilia A gene therapy, showed an average Factor VIII level of 7% after a year but this varied greatly within the trial population. Two of the 18 participants lost factor expression entirely.

Other haemophilia A AAV gene therapies currently in trials include Pfizer/Sangamo, Bayer, Spark and UCL/St-Jude.

Side Effects

Short term infusion reactions can occur but of greater concern are elevated liver ALT levels. Most trial participants needed treatment with corticosteroids for six months or more which can have serious side effects. Escalating doses of gene therapy can lead to liver toxicity which can have an impact on factor expression as well as liver health.

Exclusion factors

Most trials have excluded children, people with previous inhibitors or ongoing inhibitors, liver damage, HIV infection and/or antibodies to the AAV vector. So, these treatments, even if licensed and commissioned by the NHS will not be available for everyone. •

Whether gene therapy is right for you is a very personal decision. It will vary based not only on your personal circumstances, but also the factor levels you can achieve, the durability of the expression of the treatment, how effective it is in improving outcomes compared to your current treatment options, and considering all the potential risks and side effects.
Later this year we will be publishing a guide to gene therapies in haemophilia to help you talk to your friends, family, and haemophilia centre team about gene therapy and to help you make that decision.

Dates for your diary

Go to **haemophilia.org.uk** for details as well as the latest information on all events. Or you can email us at **info@haemophilia.org.uk** or telephone **020 7939 0780** for more information.

September
36 Fitness Challenge
Your challenge, your time – it could be 36 press-ups or 3.6 miles!

17 September, London
Big Red Bridge Walk London
Walk eight of the capital's bridges.

24 September, Glasgow
Big Red Bridge Walk Glasgow
Get to know Glasgow on foot.

1 October, Derry
NEW Big Red Bridge Walk Derry
Stride out with us in Northern Ireland.

1 October, Nottingham
NEW Haemophilia Live
A day to focus on the big issues.

14 – 16 October, Essex
Newly Diagnosed Weekend
Support for young families.

29 October, London
Service of Remembrance and Thanksgiving
For those infected and affected by the infected blood scandal.

19 November, London
AGM
Join in person, or online.

6 December, London
Carol Service
An uplifting service in St Botolph's Church.

28 January, Southampton
NEW VWD Live
A day dedicated to VWD.

25 February, London
NEW Rare & Unclassified Bleeding Disorders Day
A chance to get together and also hear expert speakers.

5 March, Oxford
Talking Red Live
Conversations about women and girls with bleeding disorders.

This issue of Community Matters has been sponsored by Novo Nordisk: novonordisk.co.uk

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