**WATCH TADEO'S VIDEO**
annualreport.octapharma.com/patients



# I WANT TO BE A SUPERHERO.

**ALEJANDRO AND SOFIA**
**PARENTS OF TADEO, 6**
**SALTA, ARGENTINA**

Our lives are marked by two major "before and after" moments. The first was when our oldest child Mateo, who is 13, was diagnosed with severe haemophilia A. The second was when our son Tadeo, who is six, was diagnosed with insulin dependent type 1 diabetes at two years old.

Tadeo's diagnosis with severe haemophilia A when he was two months old was not a surprise because of his older brother's condition. When Tadeo was born, our main fear was of the consequences of him being born prematurely and not of him having haemophilia like his brother. When he was diagnosed we were already prepared to face his haemophilia and we knew we were accompanied by the team at the Haemophilia Foundation of Salta.

From the moment Tadeo was diagnosed with type 1 diabetes, his haemophilia became second priority for us. Today, haemophilia is not limiting Tadeo's daily development in any way because his factor VIII concentrate, Nuwiq®, is performing well. Tadeo's treatment twice a week gives us peace of mind and reassurance that at least his factor VIII levels are stable. Mateo is also doing well on octanate®. He acts as Tadeo's protector by cherishing and protecting him against harm.

It is important to explain our children's haemophilia to others because there is so much misunderstanding and even misinformation in society. One of the major challenges we face is the ignorance of teachers at school. We counteract this by giving people clear and accurate information. We explain that Tadeo is not being harmed, his condition is not contagious, and we tell them where to call in an emergency situation.

"From the moment Tadeo was diagnosed with type 1 diabetes, his haemophilia became second priority for us. Today, haemophilia is not limiting Tadeo's daily development in any way."

The Foundation has had a significantly positive impact on our lives and on our kids' lives. Through it they have met other children with haemophilia. The Foundation has given us counselling, training and support, and without it we would not know what to do as parents. Whenever we have questions the Foundation welcomes us with open arms. Our kids feel empowered and protected with their continuous prophylactic treatment.

Tadeo's diabetes is one hundred times more powerful than haemophilia because it is more difficult to control. Our biggest challenge is to maintain stable glucose levels in his blood. Diabetes gives us no rest throughout the day because it is directly related to food intake, exercise, climate and the mood of the child.

Tadeo takes 16 units of insulin in the morning, and then at least eight on-demand doses of insulin as we monitor his blood glucose levels throughout the day. Tadeo manages his symptoms and knows when he has high or low blood sugar. He does not handle his medication or dosage; for that he depends on an adult with knowledge of his pathology.

Our advice to a family who has discovered that their child has haemophilia is: get support and educate yourself. Do not overprotect your child. Do not listen to advice from doctors who do not know this pathology. Do not go to healers who can supposedly cure haemophilia with natural preparations. Teach your child the importance of prophylaxis and how it is performed. Give your child tranquillity and a sense of security.

As parents we are free thinkers. We are committed to learn each day and to grow together with our children. We can make mistakes and we can try to learn from them. We instil joy, positivity and responsibility in our children. We share the most we can with them. Our goal is always to promote independence.

Tadeo never gets bored. He loves to play with other children and enjoys swimming and physical education at school. He loves superheroes and playing video games. Tadeo collects action figures of Dragon Ball, a Japanese manga series.

Tadeo's dream is to be a superhero.

# A DIAGNOSIS OF HAEMOPHILIA IS NOT THE END OF THE WORLD.

**DR MARIA SOL CRUZ,**
**PRESIDENT OF THE HAEMOPHILIA FOUNDATION OF SALTA**

The Haemophilia Foundation of Salta is dedicated to the diagnosis and comprehensive care of people with haemophilia. In the Salta province we have 63 people with haemophilia (45% of whom are children), 100 with Von Willebrand disease and 20 affected by other factor deficiencies. We offer diagnosis, treatment, multidisciplinary care and information about the diseases and how to improve quality of life.

In Argentina local healthcare professionals often do not have the knowledge or training to treat haemophilia. There has been insufficient understanding of how to take care of people with bleeding disorders, and often there were misunderstandings and mental barriers. The Foundation acknowledges and faces these challenges by developing educational programmes for healthcare professionals, as well as programmes educating patients and families.

The most important job we have is to demystify haemophilia. Prophylaxis means that children today do not have the same orthopaedic problems and joint problems experienced by adults who did not have prophylaxis growing up. Today families do not have to be afraid of this disease. A diagnosis of haemophilia is not the end of the world.

Our main role is to support families by providing tools, know-how and advice. Empowerment comes when the family understands the disease. Our families have direct connections with the Foundation, and access to a network of multidisciplinary physicians and healthcare professionals.

An early diagnosis is as important as getting the right treatment. In Latin America we are often lacking the tools and specialised laboratory staff to support diagnosis. The first challenge is proper diagnosis, and then providing proper treatment on time. One of our goals is to help physicians better understand the condition and the importance of treatment. Another goal is to inform and educate society, for example to expel the myth that children with haemophilia are prohibited from playing sports. To support families we have created guides for parents and teachers, and we go to the schools ourselves to educate them.

It has been a challenge to demonstrate the value of our work. Early attempts to establish a healthcare unit dedicated to haemophilia were initially rejected by the Ministry of Health. I was very glad when an Act was signed by the Ministry of Health in July 2016 formally acknowledging the importance of the Foundation's work.

After going to the World Federation of Hemophilia (WFH) World Congress in Melbourne Australia, in 2014, we made a legal request to use Nuwiq® before regulatory approval in Argentina. Authorisation was granted and currently we have four patients on compassionate prophylactic use of Nuwiq®. My experience of this product has been excellent. With the pre-filled syringe, it's easy to prepare, it's quick to infuse and the half-life is great meaning fewer infusions a week.

Tadeo's case is special because he has two diseases: severe haemophilia A and type 1 diabetes. It is unfortunate, but it gives us the opportunity to try to better understand the impact of having these two major diseases at the same time. We are learning every day how to improve things for him. I am very glad that Tadeo's parents are so engaged in understanding what they can to improve his quality of life.

I love working with families and having social interactions with many different people. I love taking care of people and seeing them improve each day. I travel internationally and nationally and am constantly learning. I am always eager to come home and apply and share the knowledge I have gained.

Mother Teresa said: "Not all of us can do great things. But we can do small things with great love." What we do may appear to be small, but when done with love it can have a huge impact.

**WATCH NATHAN'S VIDEO**
annualreport.octapharma.com/patients



# HAEMOPHILIA IS JUST A PART OF WHO MY SONS ARE, LIKE BEING TALL.

**DARRYL, FATHER OF NATHAN, 7**
**NEWMARKET, ONTARIO, CANADA**

My sons Nathan (aged seven) and Ben (aged nine) have lots of energy and are very funny and entertaining. They play basketball and want to play in the NBA when they grow up. I coach their basketball teams, and before they go to practice I infuse them with their factor concentrates. Nathan and Ben have severe haemophilia A. Ben loves all sports, and most of all he would love to play hockey; even today he still asks why he can't play. For my boys, hockey and American football are out of the question.

Strange bruises started appearing on Ben's body when he was 10 months old. He had really bad swelling and luckily our paediatrician was on the ball and recognised the symptoms of bleeding and referred us to the SickKids Hospital in Toronto. The diagnosis tore us apart. It was shocking. We were confronted with understanding what this condition would mean for our son, and figuring out how we were going to live with it. Our life trajectory changed in a huge way. We didn't deal with it well. We went through a grieving process, mourning what we thought our life was going to be. There was a lot of fear.

Eventually we came to realise that the diagnosis wasn't life *ending*, but it was life *changing*. Having the support of our healthcare system and working with nurses taught us a tremendous amount. We learned that if we give the treatments right our kids are going to be fine. Now we have found a very good path to live with the diagnosis. In fact, all the family have been very active in the bleeding disorder community, including grandparents, on the national board and at local level. We talk to newly diagnosed families and give them tools to help them face the diagnosis.

"God, grant me the serenity to accept the things I cannot change; courage to change the things I can; and wisdom to know the difference."

The kids get their factor concentrate every other day. We have been doing home treatment for seven years. You get into the routine. We have to get up early to do it. Infusing my sons every other day helps me to do my job as a father, and them to do their job, which is to be kids. We make the most of the time we spend doing the infusions. We talk; in fact we are getting really good at talking, and not all families take the time to do that.

Ben was 20 months old when he bit his tongue badly. The recombinant factor we gave him triggered an inhibitor. We had only been living with this condition for 10 months and suddenly the treatment no longer worked. It was a double blow. We quickly learned how to administer immune tolerance induction (ITI), which pushes the body into submission. Imagine infusing 3,000 IU of factor every day in a two year old; Ben's current dosage is half that. Ben was inhibitor free within a year, but it was a hard year. No kid wants to have to sit still for an hour. We used to lay him down and wrap him in a towel to hold him still. Even now we battle with trust issues because we had to do things that in the mind of a kid were traumatic. He just didn't understand, and I think that had an impact.

The haemophilia treatment centre (HTC) team worried that Nathan would develop an inhibitor like his brother. They recommended he use wilate®. First it was used on demand whenever he got a bleed, then at nine months old he started prophylactic treatment with wilate®. Thankfully the approach worked and Nathan did not develop an inhibitor.

It's hard when you are a little different but my sons' haemophilia does not define them; it's just a part of who they are, like being tall. This year our boys went to haemophilia summer camp for the first time. It's great for their confidence to go away for a week and be with other kids with haemophilia. It gives them a feeling of independence. Nursing staff are there to give factor if the kids don't know how. Ben is learning to self-infuse; he puts his hand on his nurse's hand, and is becoming more active. They should be in charge of how they live with their condition. Learning to self-infuse means they can decide when and if they infuse, and they are in charge of the consequences. We want them to own it.

Every time my cell phone rings I always check, even if I'm in a meeting, because it might be the school and my first thought is: "Oh my god, what's happened?"

Haemophilia has made them more compassionate and caring kids. We go to the SickKids Hospital twice a year and spend a lot of time in the same clinic as children fighting cancer. Being exposed to kids and young people with life-altering challenges has an impact. When compared with those kids, our boys just have to get a needle every other day. It allows them to be more supportive and empathetic. I am amazed by their poise when I give them their injections, and their perspective on life.

We are very fortunate with the healthcare system we have in Canada. We've got it good; but it could always be better and our goal should be to make it better. There have been great advances in treatment, and pharma companies continue to do research into how to make these conditions even easier to live with. As leaders in the community, we need to make sure we safeguard our fantastic healthcare system and ensure that we don't take it for granted. We also need to advance awareness globally to ensure that children in other countries are given access to treatment for this very treatable condition. It ought to be a human right not to live in fear of injury because of haemophilia.

I am not religious, but my mantra is the serenity prayer: "God, grant me the serenity to accept the things I cannot change; courage to change the things I can; and wisdom to know the difference."

# EVEN WHEN I'M IN PAIN, I FOCUS ON ALL THE POSITIVE THINGS.

**ERIC, 18**
**UNIVERSITY STUDENT, STUDYING HISTORY, WINNIPEG, CANADA**

I am grateful for everything I have in life. Living with severe haemophilia A has made me humble and has taught me a lot about how to cope with challenges. I don't think there is a lot of value in feeling down about yourself. Yes, the diagnosis sucks, but at some point you realise: this is my life, and feeling sad doesn't help me or anybody else. You always have something to be grateful for. Even when I am in pain, I focus on all the positive things; for example, at least I can still move my arms.

Even though things can get really bad, it does get better. For me, I have suffered a lot with chronic nerve pain and my inhibitor. I don't want to cheapen living with haemophilia, but haemophilia with an inhibitor is almost a completely different condition. An inhibitor is brutal. When I was 16 months old, I developed an inhibitor which means that my immune system identified my factor product as being non-self and developed antibodies to fight it. My body eliminated the factor immediately, so clots wouldn't form. As a small child I was put on immune tolerance induction (ITI). It's a tough regimen which involves daily treatment with large volumes. The treatment didn't work so I went on factor VIIa prophylactic treatment and also used it on demand.

In eighth grade I had what I thought were a series of bleeds in my knee, back and hip. I had a month and a half of bed rest where I could only lie on my side which was very frustrating. Eventually, after an MRI scan, they discovered there had been no bleeding and there was no physical damage. My body was simulating pain and inflammation. I was diagnosed with chronic nerve pain. We had been chasing phantom bleeds – I had gone through all that pain for nothing. I felt disillusioned. This had been draining on me, the family's resources and on the healthcare system. I eventually came out of that difficult situation through a combination of the correct medication and physiotherapy.

When I was 14 my haematologist suggested we give ITI another try. I was a teenager at this point, so the decision was mine to take. I committed to an 18-month trial and was tolerised in only three months; I was 15 years old and that was a huge turning point for me. I moved to treatment every other day with 10,000 units of wilate®. Now I can live my life the way I want to within my own limits. wilate® is how I control my quality of life by protecting me from bleeds.

Haemophilia has taught me how to deal with disappointment. Growing up, I got used to planned family holidays having to be cancelled because I got a bleed. It's disappointing for all the family, and although it's my fault I can't allow myself to feel super bad about it.

I've been talking about how complicated my life has been, but there are so many people around the world who do not have access to the treatment they need. Attending the 2016 World Federation of Hemophilia (WFH) World Congress in Orlando was a really humbling experience which blew me away. The major theme is "Treatment for All". Haemophilia is a manageable condition when you have access to factor concentrates, but 75% of people don't have access. For me, when I feel I am experiencing the beginning of a bleed, it is not a question of "if" I treat but "when"; I always have my factor available. Many people around the world don't have that luxury.

Haemophilia has shaped my interests and friends in many ways. As a kid I couldn't play sports. I played video games and board games. Having haemophilia means a lot of my interests are non-physical. I partake in what I call "mental athletics". In November 2016 I competed in the world championships of Netrunner, which is a two player card game set in a dystopian future. When I am playing competitive card games at the highest level I still feel the pain and the stiffness in my joints, but when I am truly involved in a game everything else falls away and I can just let go.

### CHRISTINE, ERIC'S MOTHER

Haemophilia is in our personal lives and is now my occupation as well. I am Executive Director of the Canadian Hemophilia Society's Manitoba chapter.

At 14 months Eric bit his tongue; it was a tiny little cut but the bleeding didn't stop for days. We took him into the hospital and he was diagnosed with severe haemophilia A. Eric's diagnosis was like an out of body experience. We were processing things three seconds after they were happening. It is devastating to discover that your perfect child has a chronic condition. You experience anger and denial. You think of all the things that won't be; I remember my husband Shane saying: "He'll never be a police officer."

Once you have been through the grief process, you realise that this little person has all these other fantastic things to offer. You learn to become good managers of the condition – it becomes your normal. There was no way I would have put Eric into day care. I sold my business so I could stay at home with him. I was lucky to be in a position to do that. He is so cute, so it's worth it.

My advice to newly diagnosed parents is to learn all you need to know, but not from the internet which is full of scary things. Whatever you dreamed up in your head about the reality of this condition, it might not be the reality. Educate yourself and reach out to the community. During that dark time after diagnosis we very quickly made a strong community connection. During our first community event we saw kids running around being normal children. It was a huge relief to see that these were not disabled children, and to meet other parents who had been through what we were going through.

"I am so proud to see Eric's positive approach to life. When he was little and expressed anger we would talk about all the good things that have come from his condition – all the life experience we have and the people we know. You have to remember to see all the silver linings."

As a small child Eric developed an inhibitor and was put on immune tolerance induction (ITI). We did treatments every day through a port-a-cath. We did that for a very long time, but we couldn't get his titre level to change. We discontinued ITI then began using another plasma product that we gave prophylactically every other day two or three times a week – and used a bypassing agent when he was bleeding.

When Eric had his inhibitor he experienced profound bleeding episodes. When he expressed that he had pain there was instant panic. The experience of living with the inhibitor was a significant part of his young life. Since he tolerised at 15 years old, I have watched him gradually let go of his anxiety. Now he can experience the body pain everyone experiences. Yes, he pays attention, but there is not that same level of fear and anxiety, and that's the case for me too. When we got the news that he had tolerised I felt a weight lift off me that I wasn't even aware I was carrying. The difference between life with the inhibitor and life free from the inhibitor is incredible.

After everything we've been through we have learned an important lesson: every challenge is temporary. During the tough times it may not seem temporary, but you have to persevere and dig into your internal resources. You do the best you can, knowing that it won't always be like this. Having the chronic nerve pain diagnosed was a huge improvement in Eric's life because he finally knew why he was in pain all the time. Pain is so little understood, and is often undermanaged in bleeding disorders.

Our nurses have become a part of our family. The care from the team has been amazing. These people have known Eric all his life. Our haematologist, who is Director of the Manitoba Bleeding Disorders Program, was a Fellow when Eric was diagnosed. I have experienced the healthcare professionals working in the haemophilia realm to be truly dedicated to the people they serve, not only in terms of the care they provide but also their advocacy. We have been very lucky.

Eric has a resiliency and maturity not typical of his age. As a child Eric was socialised to adults early on; he was much more mature than his peers. Some older gentlemen in the community, who had experienced bleeding in their childhood and could remember what it was like not to have product, showed Eric kindness because of what he was going through. They became friends. The social skills he built up being around adults all of the time have served him very well.

Eric was 16 when he joined the gaming community, which is a beautiful community of people who have welcomed him and boosted his self-confidence.

I am so proud to see Eric's positive approach to life. When he was little and expressed anger we would talk about all the good things that have come from his condition – all the life experience we have and the people we know. You have to remember to see all the silver linings.


KNIGHTS
FOOTBALL

**WATCH EDGAR'S VIDEO**
annualreport.octapharma.com/patients



# I NEVER LET HAEMOPHILIA DICTATE HOW I LIVE MY LIFE.

**EDGAR, 31**
**JACKSONVILLE, FLORIDA, USA**

The day I received my diagnosis of moderate haemophilia A at 10 years old was the worst day of my life. I was shocked and scared. My parents asked the same question every newly diagnosed family asks: "Is my child going to die?" It was like being in a tunnel and you looked ahead and saw no end. I felt like my life was being taken away from me. It was devastating. I grew up playing baseball and now I was told I couldn't play the sport I loved. At first it was hard to understand why. The hardest thing about haemophilia is knowing that there is no cure and for the rest of your life there are some things you can never do.

They say it takes a village to raise a child, and this is even truer when the child has haemophilia. After diagnosis I was referred to our haemophilia treatment centre (HTC) and to the Hemophilia Foundation of Greater Florida. Through education and networking events we met other families and gained a better understanding of the education materials and resources available. It sounds like a cliché but your haemophilia care team becomes like another family. When I was a paediatric patient my parents did most of the talking and they made the decisions; but as I grew older I had to learn how to communicate with the healthcare team and tell them how my treatment was working, for example if I thought I needed to increase my prophylaxis. I would advise the family of a newly diagnosed child to immediately surround yourselves with as much support as you can take in. Knowledge is power. The more you know, the better equipped you are and the better your quality of life will be.

"My parents asked the same question every newly diagnosed family asks: *Is my child going to die?*"

A great experience for me growing up was attending the weeklong summer camps for children with haemophilia at Camp Boggy Creek. I first went to camp when I was 11 and that's where I met a lot of my friends. In addition, one weekend a year there is a family retreat which is a great way for families to share experiences. It really helps to understand that you are not the only one. I went to camp every year until I was 16, and when I was 18 I volunteered for a few years to give something back and help young people see that having haemophilia is not the worst thing in the world.

Until you learn how to infuse factor VIII concentrate yourself you are always tied to a family member, HTC or nurse. You are always counting on them to take care of you. At first I didn't want to self-infuse because it's scary to stick a needle in your arm. However, I was trained by a nurse and began to self-infuse at 16. Once you learn to self-infuse you break away from the ball and chain of depending on others and your quality of life goes up. At the first indication of breakthrough bleeding or injury, you are able to infuse quickly, and in an emergency you don't have to wait. Self-infusion is a liberation.

When I was younger I was quite shy and kept myself to myself. Growing up and learning how to communicate and explain my haemophilia to adults means that today I am an open book. I use my life experiences to help other families. As Patient Educator for Octapharma, my role is not only to share my story but to listen to our community and guide them to resources that can help them. I am honoured to share my experience and the knowledge bank I have accumulated over the years – for example, teaching people how to educate employers, teachers and friends about

haemophilia. We have helped a lot of people understand that there is a solution to every problem that comes up.

I attended the 2016 World Federation of Hemophilia (WFH) World Congress in Orlando, which was truly eye opening. I met many patients from around the world and, despite differences in terms of availability of treatment, we shared many commonalities, such as physiotherapy techniques to use when you have a bleed. It was fascinating to find out that many countries are struggling to get medicine to patients or are not giving treatment to patients because of costs or availability of product. We are very blessed in the US.

My life has been enhanced because I know I am fully covered with my prophylactic treatment with Nuwiq®. I don't have to worry if I can do an activity because I know my factor levels are okay, and if something does happen I have the tools to take care of it. With my previous factor product I was having very uncomfortable adverse reactions, including dizziness, headaches and flushing. I switched to another product but went on to have 6–10 breakthrough bleeds a year, which is not the standard when it comes to prophylactic treatment. I happened to go to a Haemophilia A Consumer Roundtable meeting in Dallas, Texas and was amazed by the clinical data presented. From that day on I knew I wanted to switch to Nuwiq®. I did my research and started treatment initially on a free trial. I have been on the product since February 2016. I have not had any adverse reactions and zero breakthrough bleeds. I am ecstatic about my choice.

I stick to my infusion schedule very strictly, which comes from experience. Your teenage years are the most challenging time. You might not follow the prophylactic regimen properly because dealing with haemophilia is the last thing you want – you want to go to the movies or hang out with your friends. I am disciplined today because I know that my factor VIII product protects me. My daughter Amelia is one year old; since she was born I make sure I am taking care of myself because I want to be there throughout her life.

My life is very busy because as well as my job as a sales representative, and my role as Patient Educator, I coach high school American football five days a week. When you do what you love it's never work. I enjoy guiding young men and seeing them progress over the four years they are with me. Football teaches them valuable life lessons on how to stay calm and deal with difficult situations. It's great to see these young men evolve into valuable members of society.

I love knowing that I am making a difference in everything I do. I never let haemophilia dictate how I live my life. If I want to do something I always find a way to do it. With education and the correct support system I believe there is nothing you cannot accomplish.

# WE HAD A CHILD WITH TYPE 3 VWD WHO DIED AFTER A HEAD TRAUMA.

**DR FAISAL KHANANI, CONSULTANT PAEDIATRIC HAEMATOLOGIST ONCOLOGIST, TAWAM HOSPITAL, ABU DHABI, UNITED ARAB EMIRATES (UAE)**

Our centre provides comprehensive care to 225 bleeding disorder patients. We try to treat all patients who come to our hospital; however, we are unable to treat patients who do not have insurance. Haemophilia can lead to disability if not treated, so it is very difficult when we cannot support all the families who come to us.

As well as 100 patients with haemophilia, our centre treats six patients with the rarest form of Von Willebrand disease (VWD), type 3. This most severe form of VWD is characterised by a total or near-total absence of Von Willebrand factor (VWF) in the plasma and cellular compartments, leading to a profound deficiency of plasmatic factor VIII (FVIII). We use wilate® for the treatment of VWD and haemophilia A, and as second line treatment for inhibitors with immune tolerance induction (ITI). In wilate®, ratios of VWF to FVIII are close to physiological values (1:1), facilitating ease of dosing and monitoring.

If bleeding is left untreated, this can be serious. We recently had the case of an undiagnosed type 3 VWD child who died after a head trauma. I hope that in future the introduction of genetic testing will lead to early diagnosis and treatment.

Working in haematology and oncology, it is very satisfying when you see a child cured or feel better and begin to enjoy life. It is wonderful to see the children do well at school. Some of our patients develop a fascination with medicine. One of my patients is in medical school and another is studying pharmacy.

I believe that life is a gift of God. We have to make the world more beautiful by living positively, not only for ourselves but for the community.

# THE TAINTED BLOOD SCANDAL WAS HUGELY TRAGIC BUT VERY INSTRUCTIVE.

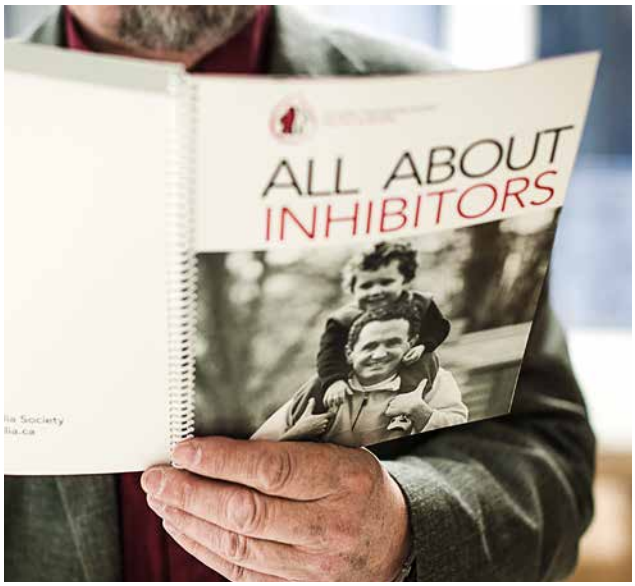
**DAVID PAGE, 64**
**NATIONAL EXECUTIVE DIRECTOR,**
**CANADIAN HEMOPHILIA SOCIETY**

I was born in 1952 and diagnosed with severe haemophilia B aged 11 months. My mother's uncles in England had died of haemophilia when they were young. In Montreal in 1953, a small group of haemophiliacs, their families (including my parents and grandparents) and physicians founded the Canadian Hemophilia Society.

In the 1950s and 1960s treatment for haemophilia B was fresh frozen plasma (FFP) administered intravenously which meant spending on average three to five days in hospital once a month. Each bag of FFP contained only small amounts of the missing clotting factor, so large volumes were needed to stop joint bleeding. FFP was not effective in serious surgery – a simple appendectomy, for example, was likely to be fatal.

It's a funny disease because one week out of a month you are incapacitated while the rest of the time you are almost normal. I missed a third of my school days, but it meant I had time to read. In the hospital I learned how to learn by myself, which is a tremendous life skill. In those days, kids were regularly staying in the hospital together and naturally you made friends. Today, because of home treatment children will only go into hospital once or twice a year for check-ups, so they don't foster those hospital friendships with other children with the same condition. These days, we manufacture those valuable connections through summer camps.

"My goal now is to pass on my knowledge to the younger generation. My message to young people is be knowledgeable and engaged in your own healthcare. This is your life."




In the early 1970s factor concentrates that could be infused at home became available. I learned quickly how to self- infuse and this changed everything. In 1972 I toured Europe by bicycle from England to Athens, which would never have been possible had I not had my concentrates with me.

Today we have kids who, because of the advances in treatment, are unable to identify bleeding when it occurs and don't know the consequences of bleeding. We need to educate these children about the causes of bleeding, how to identify bleeds and how to prevent them. Preventative treatment for children really makes a difference in the long run to the health of their joints. We want to raise kids so their joints are in good shape and they don't have to be repaired in later life.

Older people with haemophilia suffer with every step, because literally every step hurts. The damage was done in their youth. In addition to the pain, there is emotional suffering. Some feel socially isolated and unable to play a full role in society. Some might not be educated because they could not go to school. Some feel like a burden on their families. Young kids in Canada today will avoid almost all that pain and suffering. Yes, there are still frequent needles, but compared with the past, it is almost like night and day. Saying that, inhibitors are a parent's worst nightmare because when a child develops an inhibitor it's like travelling back in time 50 years.

By the early 1980s, the Canadian Hemophilia Society helped to build a network of haemophilia treatment centres (HTCs) across Canada to provide comprehensive medical care throughout the lifetime of patients. There are 25 HTCs in Canada. The patient is at the centre of a circle of care which includes a trained haematologist, a nurse coordinator, a physiotherapist for prevention and rehabilitation, a social worker, and a psychologist to support learning to live with a chronic disease.

The challenge for parents is finding the balance of not being over-protective, but equally not being under-protective and denying the disease. Some parents say: "My child is normal, he can do anything." That isn't quite true. I encourage people to learn about their child's condition and to be as open as possible with other people in terms of disclosure.

This has become a very treatable disease in the developed world, with life expectancy close to normal. In many countries, however, it is just like it was 50 years ago, with no access to factor VIII (FVIII) and factor IX (FIX) products, and lack of haemophilia expertise in the hospitals. Globally, 75% of people with haemophilia have inadequate or no access to treatment. Many people die before they reach 20, and if they do survive they are crippled. For the last 20 years the Canadian Hemophilia Society has run 10 successful twinning partnerships with developing countries to help them build their haemophilia organisations and support them to educate their members and advocate for care.

The infection of thousands of Canadians with HIV and hepatitis C was Canada's worst preventable public health disaster. It was a terrible time for everybody: individuals, families and healthcare providers. There are people still living with the consequences and some have been unable to rebuild trust in the healthcare system. The crisis was hugely tragic but very instructive. The Krever Commission (1993–1997) was set up to investigate allegations that the system of government, private and non-governmental organisations responsible for supplying blood and blood products to the healthcare system had allowed contaminated blood to be used.

Many of our members testified, including me. It was extremely hard to hear what had happened, and to learn that some of it could have been avoided. The Krever Commission led to many changes in blood systems worldwide and I am proud of what we achieved.

In Canada at the moment paid plasma donation is a hot issue. Our organisation takes the view that products from paid donors are as safe as those from unpaid donors. We need more plasma and the only way to get more plasma is through paid donors. This is heresy for some people because of the problems in the 1970s and 1980s. However people need to challenge their own preconceptions and look at the science and the facts. Things have really changed since the tainted blood scandal, and with all the scientific advances, lessons have been learned. The key issue is having a safe supply of product for patients.

We are a militant and demanding community. In general people with haemophilia tend to be obstinate. Once we get an idea we don't give it up easily, which comes from facing challenges as children and often dealing with medical people who didn't know what they were talking about. We knew which veins to use when we were four, and we were put in the hands of interns, so even as young children we had to assert ourselves to get the care we needed.

Fortunately, the younger people today don't have to go through what we went through in the 1980s. The Canadian Hemophilia Society is involved in lots of blood safety committees and medical conferences. We try to get the younger generation involved and allow them to take their proper place in decision making. My goal now is to pass on my knowledge to the younger generation. My message to young people is be knowledgeable and engaged in your own healthcare. This is your life.

**WATCH JANNIK'S VIDEO**
annualreport.octapharma.com/patients



# A HYPERACTIVE KID WITH HAEMOPHILIA IS A DANGEROUS COCKTAIL.

**JANNIK, 21**
**UNIVERSITY STUDENT, STUDYING JOURNALISM, MEMBER OF THE NATIONAL YOUTH COUNCIL OF THE GERMAN HAEMOPHILIA SOCIETY**

I was a very hyperactive child and that is hazardous when you also have haemophilia. I have attention deficit hyperactivity disorder (ADHD). When I was young I would constantly run through the house and jump around. I was always up to something. With hindsight I can admit that I was quite annoying. I had so much energy and was extremely active, which resulted in a lot of bleeds. Bringing up a boy like me was quite intense for my parents and it was especially difficult for my mother. To have a hyperactive kid with haemophilia is a dangerous cocktail.

As a kid I found it very hard to accept my haemophilia. I used to wish that I didn't have bleeds and didn't have to go to hospital so often. Sometimes I would stay in hospital overnight. It was tough, but occasionally I enjoyed the hospital stays because it meant I could watch videos.

The most challenging time for me and my mother was from 9–11 years old. I found it very difficult when I had to learn to inject myself with FVIII product. It is tough for a young lad to infuse himself. It was very hard for my mother to manage everything that was happening and to cope with having a little kid that doesn't want to look after himself.

"I would rather see more factor concentrate treatment available for people throughout the world than a pill which was only available in Europe."

I remember being alone in my room and crying. I felt so frustrated. I couldn't play football with my friends. I always wanted to play but it was just too dangerous, especially since my ankle was a target joint for bleeds. All my sports teachers knew about my condition, and that meant that I couldn't take part in some sports lessons. I was angry that I wasn't allowed to be normal.

I grew up believing I had moderate haemophilia, but recently my doctor told me that I have severe haemophilia. It seems that I don't have as much factor VIII activity as I used to. My older brother has mild haemophilia and he is just like a normal guy. Today he is doing his dream job – he is a train driver. I am happy for him. He didn't have the problems I had growing up. He was never under prophylactic treatment. He was also a much calmer child. I was the big challenge for my mother.

My grandfather had haemophilia and he was a great man. I know that he didn't have the chance to get the therapy he needed. If he was alive today he would be very happy to see the medicine that is available and all the advances that have been made. I think as young people we should appreciate all that we have today. Sometimes we don't appreciate it enough; I know I don't. I have experienced a lot of challenges, but I was never in a situation where I had a bleed and no access to therapy. Unfortunately I take my medicine for granted.

I should take my prophylactic factor product twice a week, but sometimes I don't have the time to do it or I just don't feel like doing it. Taking this medicine feels like a burden. It's a stupid attitude, I know. I understand that it is important to take it in order to stay protected, but when you are young you don't always look after yourself as you should.

Today I lead a nearly normal life, although I have target joint bleeds in my ankle. My foot swells from the inside and turns blue, and if it is really severe I can't walk. I am very involved with the German Haemophilia Society. I am a Youth Representative on the Youth Council of Germany. This work is very enjoyable and it has emotional worth for me. Spending such a long time in the community forms a deep and heartfelt connection. My involvement started at its summer camp, which I attended almost every year from age nine until my 18th birthday. You get to know a lot of kids with haemophilia and you realise that you are not alone. As I got older I became more involved in the running of the camp and was given more responsibilities. The summer camps were amazing; in fact, these were some of the best times of my life.

I attended the World Federation of Hemophilia (WFH) World Congresses in Orlando and Paris – these events offer a great experience. You get the opportunity to meet people from all over the world. I would love to see the majority of haemophilia patients having access to products. Today, only 25% of people have access. If I had the choice in the future I would rather see more factor concentrate treatment available for people throughout the world, than a pill which was only available in Europe.

I am a musician and have played guitar since I was 11 years old. Reading and writing fiction are also great passions of mine. I recently went hiking in the mountains with a friend whom I met at summer camp. The fact that two young guys with haemophilia can go hiking really shows what modern treatment can allow. I would say to a young guy with haemophilia that he should participate in the community because we need him. I would tell him that he shouldn't take his treatment for granted. Live the life you want, because the medication today gives you that freedom.

### SUSANNE, JANNIK'S MOTHER

When my sons were born I knew there was a chance they would have haemophilia; it is normal for us. Haemophilia has been in our family since the 1700s. My father had haemophilia and grew up during a time when no treatment was available and the disease was little understood. The doctors thought that vitamin C and eating lots of peanuts would help. Children were told they could not do sports and after injuries often they had to lay in bed for weeks. My father wanted to be a chef, but was told he couldn't because he would have to handle sharp knives. My father learned not to trust doctors. He believed that you were more likely to die in hospital than if you treated yourself at home. Even dentists were too scared to give him treatment. His brother lost a leg when he was young after cutting himself – they couldn't stop the bleeding. The first time my father was infused with a factor concentrate, in 1980, he contracted hepatitis C. I had convinced him to go to hospital for treatment to stop his nose bleeding. I felt guilty for a long time after that.

The connection between sons and mothers is usually very close and most mothers feel guilty about being a carrier of this disease. Even when you know you cannot change it and it was not your choice, in your heart you feel differently.

I was expecting my younger son Jannik to have mild haemophilia like my older son, but when he was born an inexperienced doctor said: "Don't let him cry, he will get bleeding in his brain." I was shocked. Despite knowing this was incorrect, he made me feel very insecure. I replied that it cannot be true because everyone in the family has always had mild haemophilia. Jannik was always considered to have moderate haemophilia. Only recently he has been shown to have severe haemophilia after all.

I didn't expect Jannik to have so many bleeds throughout his childhood. You could never predict what would happen next because of his hyperactivity. Being at home was sometimes like being in intensive care. It became too much for me – I had a lot on my shoulders. I felt destroyed. My husband could not help me that much, he could not see blood and he stood back. When Jannik was 10 years old I went with my two sons into rehabilitation for four weeks. I felt exhausted and during that time I asked the doctors to perform the injections. In the following year I realised it was finally time for Jannik to do his injections himself. But he said: "You do it or nobody will do it." When he was 11 years old, with the support of the home care service, Jannik learned how to infuse himself. What a relief!

In 2009 we built up a network for haemophilia families throughout Germany. The network consists of families with haemophilia who offer a helping hand or an open ear to other families. For three years I was a member of the Board for the German Haemophilia Society. I learned a lot during this time. In November I stepped down from the Board and now I am fully focussing my efforts on the network.

I am a Senior Home Care Specialist. We support patients and families with children with haemophilia with their therapies, and train them on home care treatment. Now we are seeing many refugees with haemophilia entering Germany. Some of these people have never had access to treatment before; some have subsequently developed inhibitors after treatment. It is very difficult for these people and there are some language barriers. We try to find a way through the chaos to support them.

We shouldn't grant this disease too much power over our lives. One can live a happy life despite having haemophilia. If one can accept help from others, many things become easier. I am really curious about the developments in the future and I hope that this disease can soon be treated even better.