PORTRAITS OF PROGRESS

CSL Behring

Biotherapies for Life™

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ABOUT THE EXHIBIT

From the identification of hemophilia A and B in the 1940s to the potential of gene therapies on the horizon, the past 70 years have revolutionized our understanding and treatment of the disease.

As we look to the future, learn how hemophilia and the evolving treatment landscape has impacted the lives of this community.

This is Portraits of Progress – join us as we celebrate how far we've come.

RANKIN

ABOUT RANKIN

Rankin is a British photographer, publisher, and film director. He is best known for work that is on the cultural cusp and leading future trends, producing rule-breaking campaigns.

As a photographer, Rankin's portfolio ranges from portraiture to documentary. He has shot several high-profile musicians, actors, models and cultural icons.

Rankin's work has been published everywhere from his own exhibitions to *Elle, Vogue, Esquire, GQ, Rolling Stone* and *Wonderland,* and exhibited in galleries globally, including MoMA, New York, and the Victoria & Albert Museum, London.

BEHIND THE SCENES

GALLERY SHOW

ABOUT HEMOPHILIA

Hemophilia is a rare disorder in which blood doesn't clot in the typical way because it doesn't have enough blood-clotting proteins (clotting factors).

The two most common types of hemophilia are A and B.

Hemophilia A is the most common type of hemophilia and is caused by a lack of blood clotting factor VIII, whereas hemophilia B is caused by a lack of factor IX (FIX).

The more severe the condition, the less able a person is to form blood clots, making them more vulnerable to bleeding.

ABOUT GENE THERAPY

In general, gene therapies are an innovative approach to treating medical conditions by introducing a functional, or working, gene into the body or by turning off or changing the gene that is causing the condition.

Current treatments for hemophilia work to temporarily replace or supplement low levels of blood-clotting factor. But, gene therapy has the potential to restore near-normal blood clotting ability.

Through a one-time infusion, gene therapy aims to enable patients to create their own factor, potentially delivering lasting bleed protection for years. Gene therapy can potentially provide long-term benefits of sustained factor activity levels from a single administration of treatment, potentially reducing or even eliminating the need for regular, long-term prophylaxis.

PORTRAITS OF PROGRESS

DECADES OF DISCOVERY TIMELINE

1940s–1960s

1947

Dr. Alfredo Pavlovsky, a doctor in Buenos Aires, Argentina, distinguishes two types of hemophilia in his lab – A and B.

1952

Stephen Christmas is diagnosed with hemophilia B, which doctors colloquially refer to as Christmas Disease. Fresh frozen plasma is the only treatment option available, and the average life expectancy is less than 20 years old.

DAN

Dan has experienced the full treatment spectrum for hemophilia, from lyophilized plasma to factor. Now with a young grandson who also has hemophilia, he's amazed by how far we've come and is excited for where the science is headed with gene therapy.

DAN

Dan was diagnosed with hemophilia in 1953, shortly after his birth. He spent much of his childhood in the hospital, as there were no hemophilia outpatient or home treatments available – only lyophilized plasma. Because of this, he often missed events with friends and family, and the pain of the bleeds could at times feel unbearable.

His biggest childhood memory was an endless parade of medical students repeating his medical history – by the time he was eight, he knew more about hemophilia than they did.

By his teenage years, fresh frozen plasma became available, which allowed him to receive infusions as an outpatient. This was lifechanging – he could skip being booked into the hospital and was able to take up bike riding. Once he transitioned to factor concentrates, he could keep that in the fridge and take it with him, allowing him to travel and finally see the world.

Today, Dan marvels at how far treatment has come, allowing his grandson, who also has hemophilia, to experience a very different childhood from his own. He's also grateful for how much more united the hemophilia community is and the support that families have today.

MEET DAN

DECADES OF DISCOVERY

1970s

Factor concentrates in powdered form become available, allowing for home infusions and significant reduction in treatment time.

Researchers pioneer genetic engineering, creating the potential to treat genetic conditions by modifying a person's genes.

CHRISTOPHER

Christopher was told he wouldn't live past age 5 – but after years of health crises, he is hopeful for his future and that of his children due to the advancement of hemophilia treatments.

CHRISTOPHER

Christopher was born in 1975 and diagnosed with hemophilia in 1977 following a fracture to his skull from jumping on a loveseat in his living room. He had an older half-brother who also had hemophilia, so the condition wasn't new for his family.

Christopher was told he wouldn't live past the age of 5. Due to the lack of education on hemophilia in his community, he felt most people handled him with bubble wrap gloves and put restrictions on his activities. In school, he wasn't even allowed to use a stapler or scissors because of the misconception that he'd cut himself and bleed to death. Despite this, his parents tried their best to let him be a kid.

He began receiving fresh frozen plasma after his diagnosis, recalling it was a 12- to 24-hour process. As a result, his family felt handcuffed to a hospital, as there was no other way to get treatment.

A major turning point in Christopher's life came when a knee bleed didn't subside, and he went into multi-organ failure. As a last-ditch effort to keep him alive, he was given factor concentrates (an option his parents had previously decided against, preferring instead to source and control their own plasma donors). After that, he learned to self-infuse, which helped give him control over his own life, liberating him from constant hospital visits.

Christopher's purpose in life is now his family, and he hopes for better education on treatment options for people with hemophilia around the world.

MEET CHRISTOPHER

SUE

Sue, a now-retired nurse coordinator, spent the last three decades on the front lines caring for people with hemophilia.

SUE

Sue is a now-retired nurse coordinator who worked at a hemophilia and thrombosis center for 25 years, coordinating care on the front lines for people with bleeding disorders. Since retiring in 2013, she's continued to stay active in the hemophilia community, mostly working for nonprofits in patient education and volunteering at hemophilia summer camps.

Throughout her career, Sue has seen several changes in the treatment landscape, from fresh frozen plasma to self-infused at home treatments and how each new development has given people with hemophilia more and more independence. At hemophilia summer camps, she taught children how to administer their clotting factor themselves, allowing them to gain control over their own treatment protocol. Camp was an incredibly special time for everyone involved – kids could come together to not only learn self-infusion, but to finally let down their guard, be themselves, and be around others their age with hemophilia.

For Sue, being a part of the bleeding disorder community is incredibly special – it's like being part of an extended family. Even though she doesn't have a chronic illness, this community has taught her endlessly about courage, and she is honored to have shared in the lives of the men and women who have been through so many obstacles.

Moving forward, Sue believes there are only positive advancements on the horizon for the bleeding disorder community.

MEET SUE

DECADES OF DISCOVERY

1980s

Scientists successfully clone the Factor VIII and IX genes, paving the way for breakthrough synthetic factor therapies (not derived from human plasma) using recombinant technologies.

It is discovered that much of the human-derived clotting factor had come from blood donations contaminated with blood-borne diseases, such as HIV and hepatitis viruses.

YING POI

As a scientist working in gene therapy, Ying Poi has seen the many advancements of hemophilia treatments firsthand.

YING POI

Ying Poi leads a scientific team working on gene therapy. Her work has focused on the power and potential of gene therapy within HIV and hemophilia.

The cloning of the Factor VIII and IX genes in the early 1980s was a major breakthrough in science and essential to developing gene therapy. However, during this time, many people with hemophilia were infected with HIV and hepatitis due to infusions of infected blood. This pushed the scientific community even harder to develop new and safer treatment options.

Today, Ying Poi is excited about the future of hemophilia treatment and the potential of gene therapy to reduce or even eliminate bleeding and the need for regular, long-term prophylaxis treatment. She is hopeful that the hemophilia community will have access to more treatment options as a whole and is proud to have had a role in the advancement of science for hemophilia treatment.

MEET YING POI

DECADES OF DISCOVERY

1990s

The first recombinant FIX products are approved by the US Food and Drug Administration (FDA) and European Medicines Agency (EMA) to treat hemophilia B, leading the way for prophylactic treatment, which dramatically improve patients' lives.

Hemophilia patient life expectancy improves to 67 years old.

ADAM

A child of the 90s, Adam felt comfortable engaging in most activities with family and and friends thanks to the availability of prophylactic hemophilia treatments.

ADAM

As a baby, Adam tended to bruise easily, and he was diagnosed with hemophilia at three months old. However, he wasn't put on a prophylactic regimen until the first grade, when he tore a calf muscle after a fall. He considers himself lucky as he has mild hemophilia and has been able to participate in most activities. He credits his prophy regimen with his ability to live a comfortable life – noting that his treatment helps keep his bleeds at bay and helps him recover from bleeds.

Adam does however recall missed days outside due to painful joint bleeds, which he describes as just a "normal part of life."

Now married with a baby on the way, Adam looks forward to additional advances in treatment, including gene therapy and its potential to create one less worry for the hemophilia community.

MEET ADAM

DR. KLAMROTH

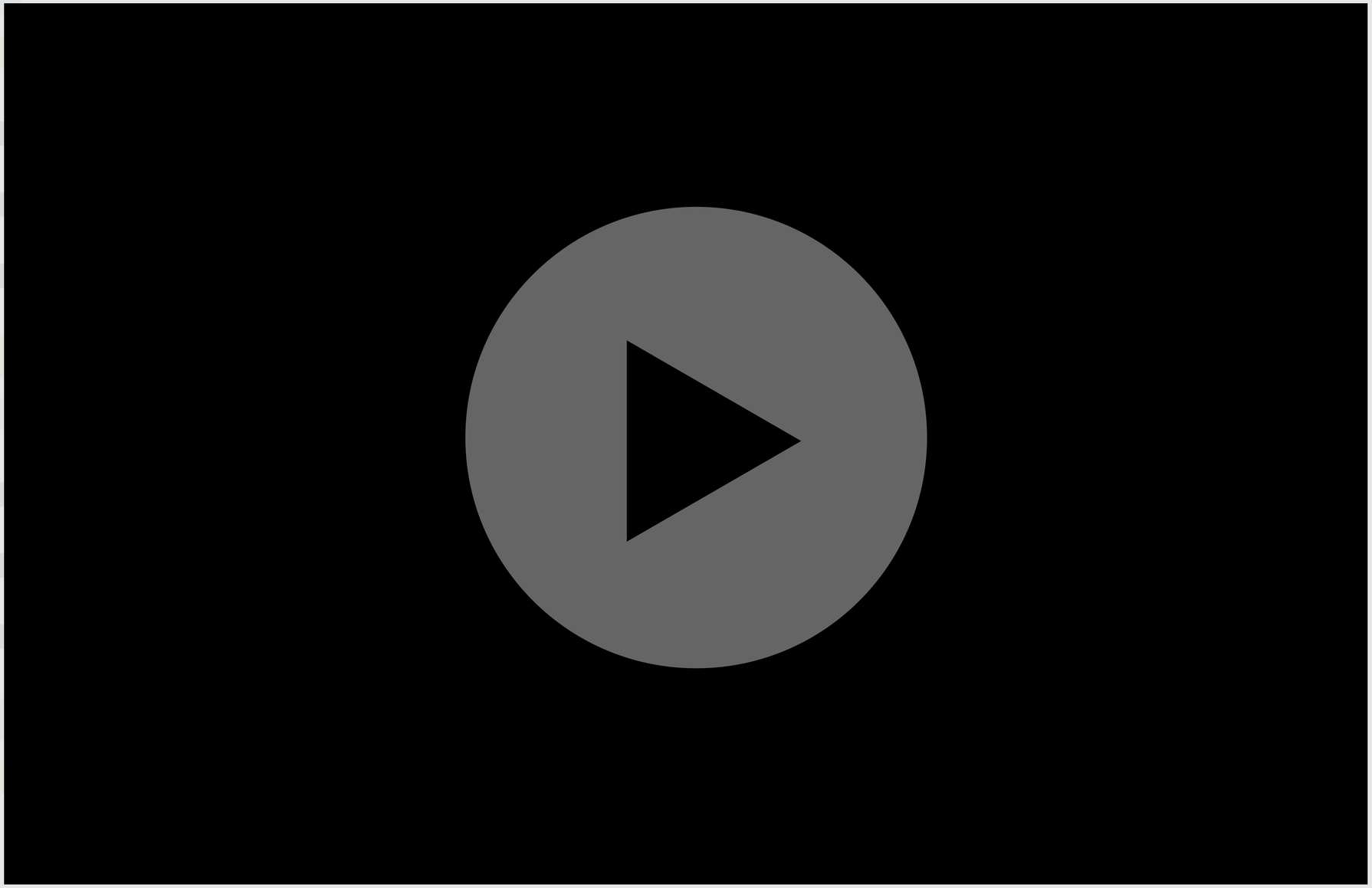
A leader in the hemophilia medical community, Dr. Klamroth has witnessed several major treatment advancements over the past 25 years and looks forward to what the next 25 years will bring.

DR. KLAMROTH

Dr. Klamroth is the head of the Hemophilia Center in Berlin, Germany, as well as the head of the European Association of Hemophilia and Allied Disorders. He has worked in hemophilia for 25 years and saw his first patient in 1996.

At the Center, he works with adults and children with hemophilia and is involved in several clinical studies. The current standard of treatment for hemophilia at his clinic is prophylaxis independent of age, starting very early in childhood at 10 to 12 months, with the goal to prevent bleeding and atrophy in the future. These treatments have made it possible for those with hemophilia to be active in their community, which Dr. Klamroth said wasn't even possible just 25 years ago.

Dr. Klamroth believes we are now in the "golden age" of hemophilia treatment as patients can now choose treatment options that not only manage their disease but also fit into their lifestyles. He also expects that in the next 25 years, factor treatments will continue to improve, and new treatments such as gene therapy, which has the potential to allow people to produce their own clotting factors, will be available.



MEET DR. KLAMROTH

DECADES OF DISCOVERY

2000s

Evidence grows for the potential of adeno-associated virus (AAV) vectors to deliver safe, effective, and lasting genes that produce blood-clotting factor into the body.

JERON

Though held back from playing sports as a child, Jeron has channeled his energy into being an advocate in the hemophilia community, traveling the world and inspiring others with the condition.

JERON

Jeron was diagnosed with severe hemophilia shortly after birth in 1996. Growing up, he couldn't participate in physical activities, and often felt held back. No matter where he went, he had to ensure he always had the right amount of medication with him, which was stressful and something that kept him from feeling "normal."

Jeron recalls that as a child he once snuck out to baseball tryouts and got hit with the ball during practice. He needed to be infused right away, a moment which truly drove home the dangers of his condition.

Still, Jeron was determined to make the most of his diagnosis, and he became an advocate, camp counselor and mentor. He has traveled the world meeting other community members with severe hemophilia, growing a family of people who understood what he is going through.

Jeron believes that there will continue to be advancements in hemophilia treatment that will offer a better way to live and better mobility. He believes gene therapy has the potential to do wonders for the community at large by giving them more options than ever before.

MEET JERON

NATHAN

Nathan credits his happy and full childhood to his mother and his prophy regimen. Now a college student, Nathan's journey with hemophilia has inspired him to pursue a medical degree.

JENNIFER

Having a child with hemophilia added another layer of worry to being a mother, but Jennifer raised her son with resilience, strength, and a dash of gratitude.

NATHAN

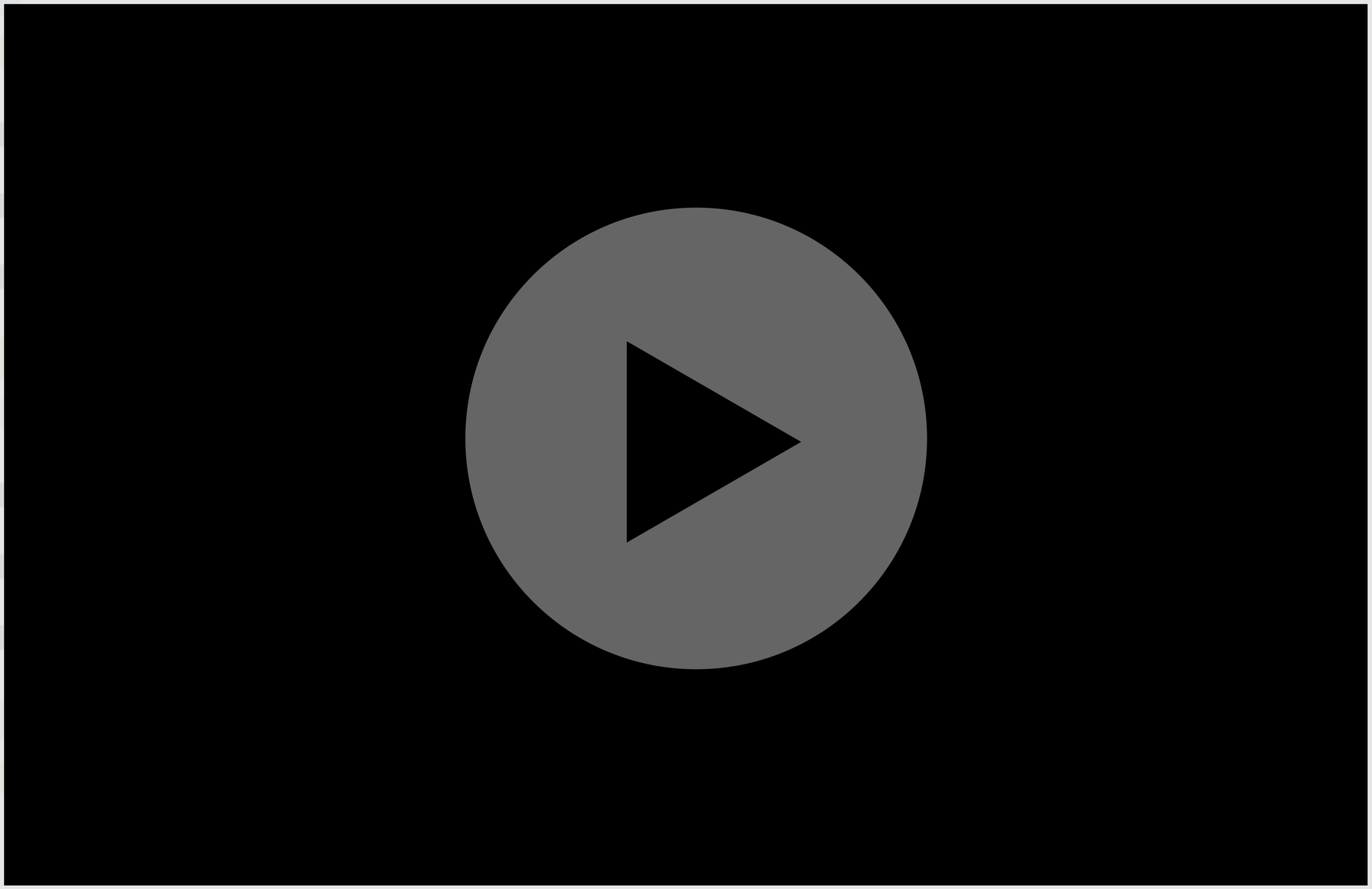
Nathan was diagnosed with severe hemophilia when he was 3 weeks old. Growing up with hemophilia, he remembers sitting on the couch every Monday and Thursday while his mother, Jennifer, infused him.

He learned to infuse himself at camp when he was seven years old and began infusing by himself by the time he was 14, a huge step in gaining his independence. He credits his mom for helping him learn the various steps – mixing and infusing properly, bringing the materials, hitting the vein correctly – along with being his number one teacher and cheerleader.

Nathan feels blessed that he lives in this time period – he notes that if he lived even 50 years ago, he may not have made it to adulthood. He's thankful for treatments like prophy that have allowed him to stay healthy and live a relatively "normal" life.

Nathan is currently in college, and because of his experience with hemophilia, he wants to go to medical school. Growing up, he learned about gene therapy at conferences – and hearing about such a revolutionary treatment further excited and motivated him to pursue this career path.

He looks forward to seeing firsthand how treatment for hemophilia will continue to evolve.



MEET NATHAN

JENNIFER

Jennifer's son, Nathan, has severe hemophilia, and was diagnosed at three weeks old; his heel stick shortly after birth didn't stop bleeding for over 12 hours, and the doctors immediately knew something was wrong. The diagnosis was terrifying – but she knew she was strong enough to handle it.

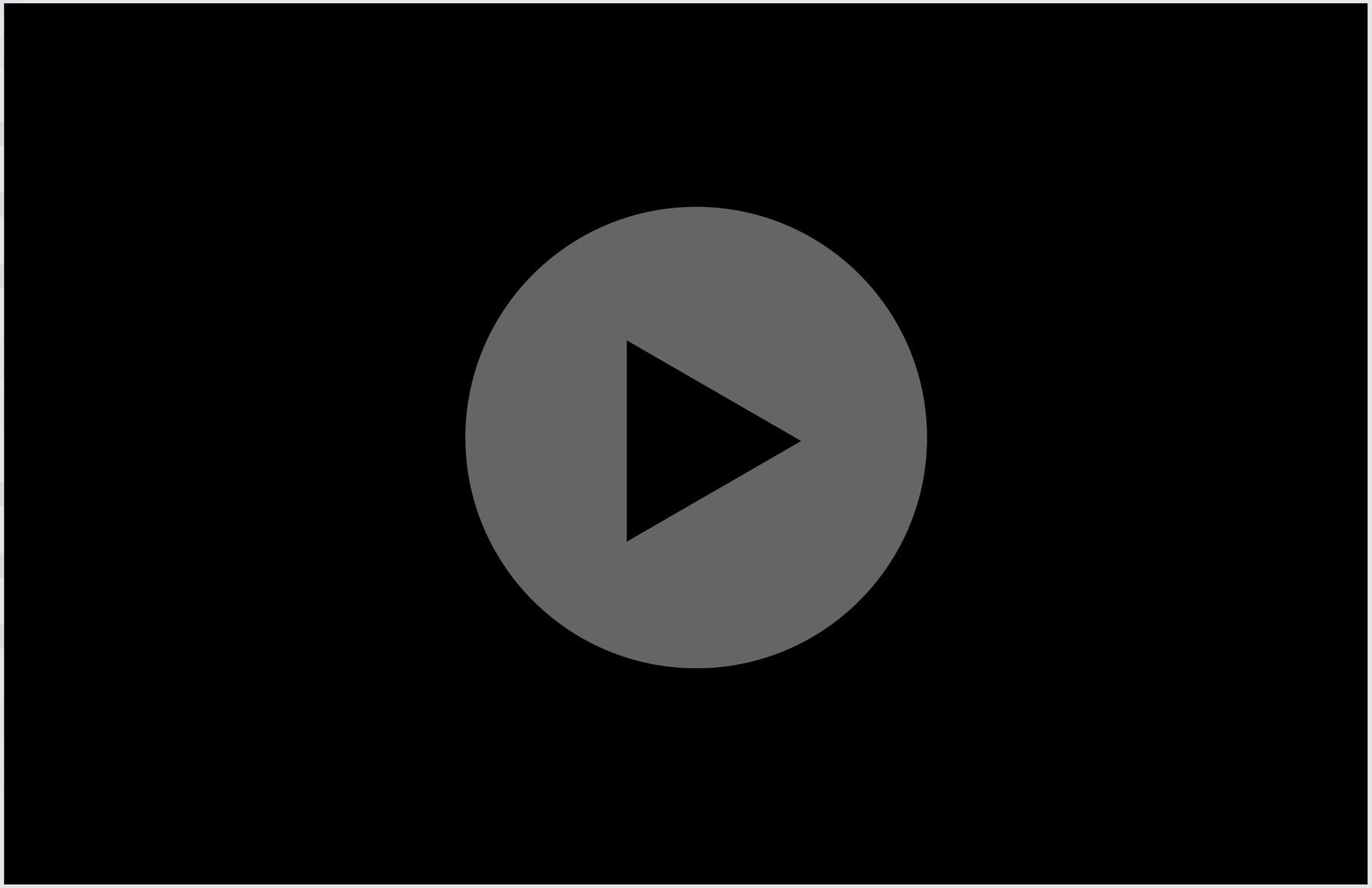
As soon as she received Nathan's diagnosis, she set out to find others who were dealing with the same issues. She found her local hemophilia community, and learned that life could be normal, despite the constant infusions and trips to the hospital.

She recalls the first time she infused Nathan at home was Christmas day – she and her husband stayed up all night, not only putting presents under the tree, but worrying and practicing how to infuse correctly. For her family, being able to infuse at home was life changing.

Jennifer fostered independence in her son, allowing him to speak up with doctors and helping him learn to infuse himself by the time he was 14 years old. Despite how nerve-wracking the infusions and bleeds were, she forced herself to put on a brave face for her son – a skill he learned as well. She felt it was her mission to teach Nathan how to live with hemophilia, how to accept it and how to live safely with it.

Jennifer heard from her doctor about the prospect of gene therapy shortly after Nathan was diagnosed, but it wasn't something her family discussed while Nathan was growing up. Now that gene therapy is on the horizon 20 years later, she is excited for what this treatment could potentially mean for her son.

Jennifer's advice to parents of children with hemophilia? Make their environment as safe as possible, and then let them live in it. Her advice is tried and true – Nathan is now at college, with a bright future ahead.



MEET JENNIFER

DECADES OF DISCOVERY

2010s

Long-acting recombinant factor therapies are available offering the hemophilia community additional treatment options.

Late-stage trials for AAV-based gene therapy in hemophilia begin.

STORMY

As a woman with hemophilia and a caregiver to a son with hemophilia, Stormy has made it her mission to educate others, advocating for men and women alike in the hemophilia community.

STORMY

Stormy's son, who is now 23, was diagnosed with hemophilia when he was 3 years old, after excessive bleeding from a tonsillectomy. As he grew up, he dealt with severe knee bleeds, and he learned to infuse himself by age 10. Things as simple as a dentist's appointment required a series of hoops to jump through – her son could never just lose a tooth on his own due to the risk of severe bleeding.

Despite his diagnosis, Stormy wanted her son to live a normal life, and always encouraged him to be open with friends and family about his condition. Stormy made it her mission to learn everything she could about hemophilia and got involved with a local chapter – but she didn't realize the impact of the wider community until she herself was diagnosed with hemophilia seven years ago.

Hemophilia is a condition that primarily appears in men, with women traditionally being carriers. Stormy knew she was a carrier – but recalled instances throughout her life where she needed blood transfusions for things as simple as a routine laparoscopy. However, her diagnosis wasn't as easy as her son's, due to lack of awareness among the healthcare community regarding women and hemophilia.

Stormy's hope for the future is that girls will be diagnosed early and receive proper treatment. She is thrilled to see the prospect of gene therapy within her lifetime, and credits the hemophilia community with getting her and her son through the difficult moments.

MEET STORMY

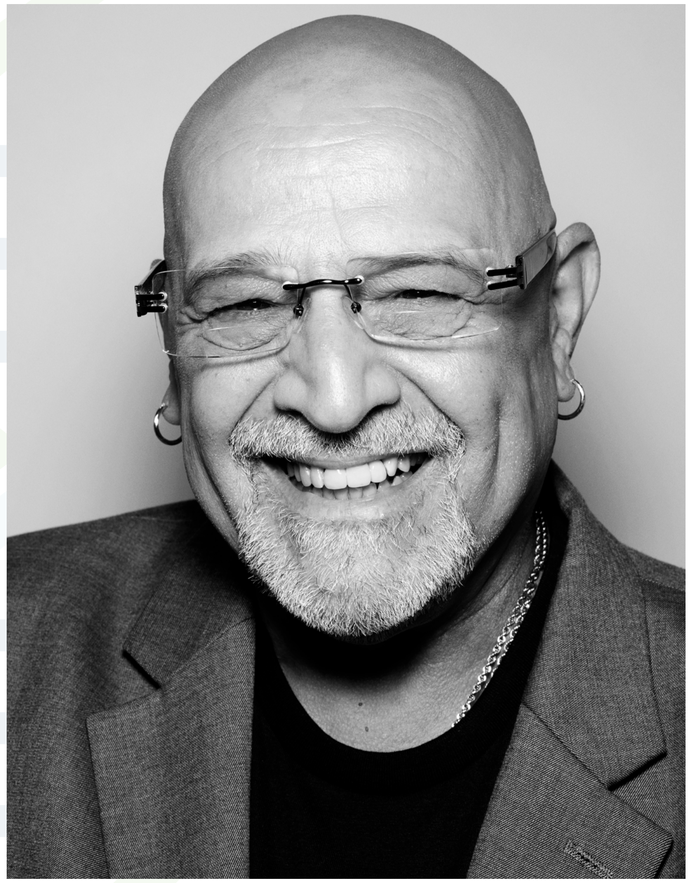
DECADES OF DISCOVERY

2020s

Gene therapy trials in hemophilia continue and show promise in potentially decreasing annualized bleeding rates and use of replacement therapy.

WAYNE

As an advocate for hemophilia for over 40 years, Wayne has experienced the monumental advancements in hemophilia treatments and has committed his life to ensuring a better world for today's hemophilia community.



WAYNE

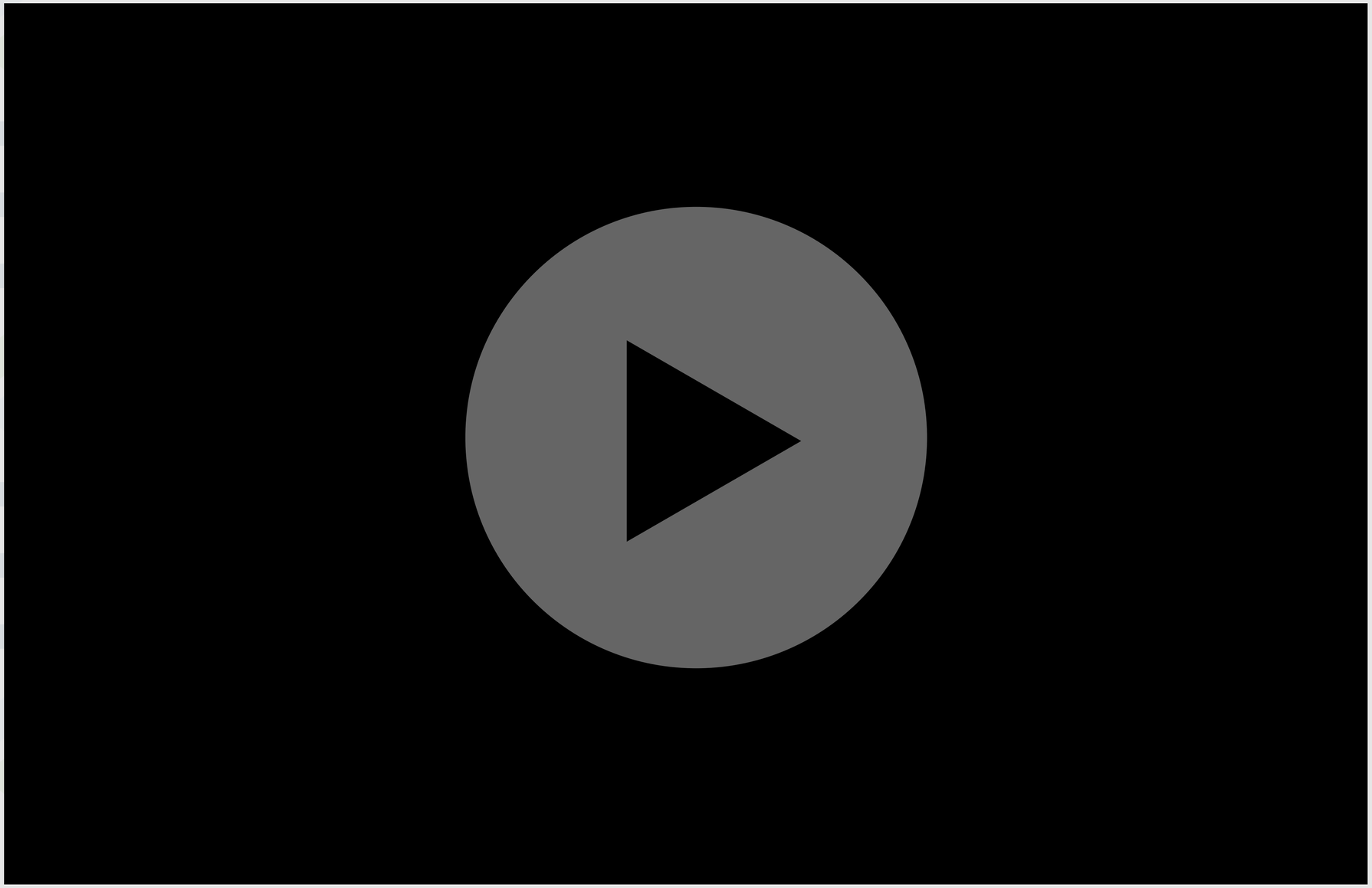
Wayne was five years old when he was diagnosed with hemophilia. At the time, no one in his family knew what the disease was – but then two of his cousins were diagnosed shortly after, with one passing away not long after his diagnosis.

In the early 1960s, Wayne's family was told that the life expectancy rate for the disease was 20 years of age. At the time, he and his family didn't receive much education on the condition – he just knew he had a bleeding disorder and had to go to the hospital to take fresh frozen plasma whenever he got a bleed.

Wayne was introduced to factor in 1985 after complications from a tonsillectomy. He then began to educate himself and joined the board of a local hemophilia chapter. From there, he made advocacy his life's mission, working to pass legislation for the community, including the first bill in New York state governing the treatment of people with bleeding disorders in emergency situations and their transportation.

His advocacy work has come full circle: he now has young grandsons with hemophilia who live active, happy lives. He's noticed that life at hemophilia camps is much different now than in the past – before, children would be covered head-to-toe with helmets and protective pads, but now they're able to run and jump and play sports without fear. Wayne credits the evolution of treatments for this milestone – and is overjoyed that he is able to see so many children become healthy adults, including his own.

MEET WAYNE



BTS VIDEO