With the landscape for hemophilia undergoing tremendous changes that bring significant hope for improved treatment and perhaps eventual cure, it’s perhaps time to take the opportunity to look back at the history of this condition and the quality of life of persons with hemophilia through the years. What better way to do this than to walk with a person through his lifetime of experiences with a bleeding disorder? And it is so wonderful to share his experience with a real Game Changer.

Brian Johnston is a truly inspirational person in many ways. When I interviewed him recently, I was touched by his honesty and openness as he related his life story with its many challenges. Through it all, he considers himself very lucky. That’s because Brian is an optimist, always looking at the glass as half-full. He has never understood why people get angry. In his mind, it never helps.

Brian is now 51 years old but he was only 2 days old when he was diagnosed with severe Factor VIII deficiency or Hemophilia A. His mother, Nancy, was a nurse and she was never aware of any family history of hemophilia. But she and her husband, George, accepted his condition and sought treatment. Then at the age of 11 months, Brian developed an inhibitor, an antibody in the blood that fought against the factor that he needed to promote his blood to clot. In those days, there was no known method to eliminate inhibitors. Treatment plans only included many infusions of bypassing agents, along with multiple hospitalizations and missed school for the rest he needed and the constant pain he endured.

For persons with hemophilia and their medical providers, inhibitors have always been of great concern. In addition to the emotional and physical consequences of the many bleeds, the cost of the complicated treatment was, and remains, exorbitant. Years ago, many persons with hemophilia and inhibitors reached their insurance maximum of coverage due to the high cost of the products used. Today, the coverage of products and treatment is still of great concern.

In those early years, Brian’s family, which included three older brothers who did not have hemophilia, moved several times for their father’s job opportunities. Eventually, they were glad to settle near Boston where Brian was able to receive his care at the Boston Hemophilia Center (BHC) which included Boston Children’s Hospital and Brigham and Women’s Hospital.

Brian grew up with frequent visits to Boston Children’s Hospital. Many of them were outpatient visits but some involved lengthy complicated inpatient hospitalizations.

Along the way, Brian’s parents decided that they would treat him as “normally” as possible. But sometimes, his life didn’t seem quite “normal”. When he was 6 years old, Brian was running when he slipped and fell on the kitchen floor. He suffered a lacerated liver but the real danger was presented by a clot on his spine that damaged his spinal cord. He wound up being paralyzed from the waist down. Brian and his parents admit that this was a tough time but they steadfastly maintained their positive attitudes and determination for a successful recovery. After a year of factor treatment and with the care of his family, he regained most of his feeling and movement. Brian gives a lot of the credit for his wellbeing to his brothers who supported him through those difficult childhood years. They have strong bonds now as adults.

Brian tells his life story with sincerity, honesty and humor. He is very positive in his outlook and feels that this helped him succeed and survive throughout the trials and tribulations of his life. At about the age of 13, Brian would need these strengths when he was diagnosed with HIV which had been transmitted through the factor and the blood supply at the time. Once again, Brian felt very lucky to have done well through the course of that medical complication and has not had many physical setbacks because of it.

Later, Brian also faced another medical challenge when he was diagnosed with hepatitis C. With his characteristic good
At one point, Brian faced the biggest challenge of his life when, after an illness, he wound up in the hospital’s Intensive Care Unit (ICU) where he was on a ventilator which breathed for him for 50 days. His organs began to shut down but he eventually responded to the multiple intravenous antibiotics he received. With his typical humility and optimism, Brian expresses much gratitude and gives credit to the physicians and staff for saving his life once again.

In recent years, a wave of clinical trials for the newly-developed hemophilia products developed. Dr. Parnes encouraged Brian to consider entering a trial of ACE 910. If it was the right match, the new medication could prove to be a life-changer, or a Brian describes it, a Game Changer. He met the requirements of having severe hemophilia A with an inhibitor. So in August, 2016, Brian decided to enter Clinical Trials of ACE 910 at Boston Children’s Hospital under Stacy Croteau, MD, Associate Director of BHC.

ACE 910 is also known as Emicizumab and is marketed as Hemlibra. Emicizumab was recently approved for use by the Federal Drug Administration (FDA) and the ACE 910 trial will be ending in a couple of months.

So, how did Brian fare? The change in his quality of life was remarkable. First of all, Emicizumab is administered subcutaneously so there is no longer an issue of finding veins and scarring skin on his hands and arms. The product increased the half-life of his factor from about 12 hours to 30 days so he only needed injections once a month. As a result, he has had no spontaneous bleeds in 20 months, a time of relief that he has not experienced in his entire life. He did break his leg while on the trial but it was the result of falling out of his wheelchair while turning awkwardly. Brian felt that it was not related to the factor product or trial and, amazingly, he did not need additional factor during this period.

These days, Brian is very happy – but then again, he has always been very happy. He doesn’t know what the future will bring but he enjoys his life even more now.

Brian ends with: “You’re given what you’re given. So you deal with it. I don’t consider myself special.” But for those of us who have the pleasure of knowing Brian, we indeed feel that he is very special. He deserves this Game Changer.

Disclaimer: This article details the life story and clinical trial experience of a patient of the Boston Hemophilia Center. It is not an endorsement of any particular hemophilia product. Patients are advised to talk with their physicians to find a product or clinical trial that meets their needs.
Kudos… To Kristen Benya

Kristen Benya, the physical therapist for the Boston Hemophilia Center’s (BHC’s) adult hemophilia team at Faulkner Hospital, was recently presented the Partners in Excellence award from her Director, Jean Flanagan Jay. Kristen received this honor for her work over the past two years towards achieving the status of Senior Physical Therapist in her department.

This Partners in Excellence award resulted from Kristen’s work in the Senior Ladder Program that requires a certain number of “points” in 3 sections of career development: professional development, clinical education and research, and projects and committees. After two years of accumulating the required number of points she presented her portfolio of work to her directors for review. It is expected that Kristen will continue her personal continuing education while remaining a clinical instructor for students from the surrounding colleges and universities. She will also continue her work in the Hemophilia Clinic and personal projects to help create a robust department that continues to provide the highest level of patient care.

Congratulations to Kristen in successfully reaching this advanced step of her physical therapy career at Faulkner Hospital. It takes a tremendous level of skill and motivation to receive his honor and we feel very fortunate that Kristen is a member of our adult bleeding disorder team.
New Staff at BHC

**BROOKE FORTIN** earned her B.S. in Biology and a B.A. in Economics at Tufts University before being hired as a Clinical Research Coordinator for the BHC team. Outside of her studies, she played soccer for the Tufts University Women’s Varsity Soccer team for four years and served as the Team Impact Liaison for the team. In addition, she was the Director for Tufts Tutoring, a program that provided free after-school tutoring to children who were in kindergarten through 12th grade in the Medford/Somerville area.

Brooke was born and raised in New Hampshire, and now calls Boston home. She is very excited to work in research at the BHC and hopes to pursue a career in Medicine. In her spare time, she enjoys cycling, hiking, reading, and travelling. Brooke can be reached at (617) 525-8038.

**TIEN HUA** joined the BHC team as a Clinical Research Coordinator in August, 2017, after graduating from Brown University, where she received her Bachelor’s Degree in Health & Human Biology. She is dedicated to eliminating educational and health disparities in and beyond her community. For this purpose, Tien served on the executive board of the Brown University Best Buddies chapter, as a mentor for QuestBridge Scholars and as a volunteer at the Rhode Island Free Clinic.

Tien was born in Vietnam, but grew up in the United States, living in California and Alabama before calling Massachusetts her home. Outside of her studies and work, Tien is an avid lover of languages, tennis, and travelling. She looks forward to working with our patients and families and further enriching her experiences! She can be contacted at (617)-919-6407.