COURAGE AND DETERMINATION

IN THE FACE OF UNCERTAINTY: LIVING WITH IMMUNE THROMBOCYTOPENIA AS A YOUNG ADULT

By Jennifer DiRaimo

With a deep passion for life and a strong motivation to help others live life fully, Haley shares her story to continue to inspire those living with immune thrombocytopenia (ITP).

Turning sixteen usually marks a year of celebration for most teens as they honor their coming of age, and the freedoms that come with it transitioning from childhood to young adulthood. For Haley Agius, turning 16 and receiving a diagnosis of ITP meant learning to live life with restrictions and uncertainty as she adjusted to living with her new diagnosis, putting aside her current goals and dreams to face her new reality head on.

Growing up in St. Thomas (Ontario, Canada), Haley envisioned herself playing professional hockey, a sport she loved and had been playing since the age of four. In addition, she dreamed of following in her grandfather's footsteps to become a firefighter. Haley played competitive soccer and enjoyed being part of a team, and the comradery associated with it. Athleticism is a big part of Haley's life, and her diagnosis could not have come at a worse time. In the middle of tryouts for a AAA Junior hockey team Haley started to notice she was developing random bruises that she initially attributed to intense physical training. However, the bruises kept appearing. One morning she woke up covered in petechia all over her arms and legs and had a mouth full of blood blisters. Not understanding what was going on with her body, Haley was anxious. Shortly after learning her platelet count was only 2,000, she was admitted to the pediatric floor of her local hospital and wasn't allowed to leave her bed. "I was told I definitely couldn't finish hockey tryouts and that I probably wouldn't be able to play any sport ever again. This news devastated me!" Haley remained in the hospital for over a week. It was not the sweet 16th birthday she had imagined.

Haley sits on the patient panel during PDSA's Externally-led Patient-Focused Drug Development Meeting (EL-PFDD)

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As if processing that news wasn't bad enough, Haley then had to endure the painstaking process of finding a medical treatment that would stabilize her platelet counts and bleeding. This resulted in having to deal with side-effects from trialing treatments and experiencing a roller coaster of emotions hoping a treatment would work and then feeling sad when it did not. First, Haley tried two rounds of IVIG, the second one coupled with prednisone, but her platelets didn't come up. Then she tried Anti-D therapy which made her very sick and did not increase her platelet counts at all. "The scariest night was when, after the Anti-D, I started to throw up violently, and then my nose started to bleed, and it wouldn't stop." Over the next four months, Haley was given high-dose steroids to stop her nose bleeds that would happen multiple times in one day, along with bleeding in her mouth and gums. "The steroids worked at first, but within 48 hours after taking them, my platelets would drop back down near zero and I would start bleeding again." During this time, Haley didn't participate in any activities, instead she watched and cheered her soccer team from the sidelines. "I hated how the steroids made me feel and look. Just a few short months ago I was healthy, fit, active, and happy. Now my face was all swollen and I felt so alone, depressed, and cried all the time."

Labeled as a 'conundrum' by her hematologist because her ITP was not responding to first line therapies in the 'typical' way, Haley and her family were determined to find something that worked. Haley's medical team suggested starting romiplostim (Nplate®). Unfortunately, at that time it had not yet been approved for use in children, and it was not covered financially through the province of Ontario in Canada. "My parents explored every avenue to get coverage or assistance for the cost of the medication but were denied over and over." Her parents even advocated to local government officials but to no avail. It was a very frightening experience for Haley and her family, especially since her parents had excellent private health insurance through their employers. When her bleeding started to get worse, her medical team gave her the option of doing four-day pulse steroid treatments, at higher doses. Worried about the long-term side-effects from the steroids, Haley decided to refuse steroid treatment. At that point her parents insisted Haley's medical team order Nplate and said they would find a way to pay for the treatment. To help pay for the cost of the treatment, Haley's soccer team, the St. Thomas Scorpions, organized a 6K walk/run led by her coach and team manager. Over 400 people attended from as far away as Ottawa city. Having a strong support system is one of the priceless benefits sports has given to Haley.

Haley saw an improvement in her platelet count in a few weeks using Nplate, however her platelet counts would fluctuate from as high as 70,000 to as low as 11,000. When they were under 30,000 bleeding episodes occurred. And as with most medical treatments, Nplate was not without side-effects. Once a week, Haley would haul her laptop and homework to the Children's Hospital in a neighboring city just to receive her injection of Nplate. Being a conscientious student, she did not want to fall behind in her schoolwork. "I missed a lot of school, and I know my mom missed a lot of work to bring me and support me at all my appointments." Again, Haley persevered. She learned to take a nap where she could and dealt with the side effects head on. Unfortunately, despite showing great strength while enduring her treatments, after eight months her platelet counts dropped and remained low. In total, Haley received over 60 Nplate injections.

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Haley and her hockey team

Haley and her family at her fundraiser organized by her soccer team

Luckily, eltrombopag (Promacta®, Revolade®) had just become available for use in children. While this medication was much more convenient to take compared to having to visit the hospital for an infusion, it came with rigid dietary restrictions and some unpleasant side effects. “You can’t take it with calcium, so I took mine at 10:30 every night. I also experienced extreme pain in my chest which ended up being acid reflux from the Revolade, so I take lansoprazole every day.”

In order to gain access to this drug, since it was also not covered through public funding, Haley and her parents had to reach out to patient support through Novartis who then advocated to her parents’ private insurance companies to provide funding for this medication. “I have to remain a dependent under my parents’ insurance, which means I need to be in school full-time, and I have to be under 25 years of age.” Scheduled to graduate at 22, Haley is worried she will need to stay in school longer incurring more student debt just to have continued access to her current treatment. She also worries about what will happen if she is unable to get a job right away after graduating and cannot obtain private insurance due to her pre-existing condition. And then there is the worry about the possibility of her parents losing their jobs one day which would mean losing their private insurance currently funding her Revolade medication.

Revolade has been successful at stabilizing Haley’s platelet count which now sits at about 100,000. Haley is thankful that Revolade has allowed her to live her life in a more normal way and has provided her with an opportunity to re-engage in sports while her levels are elevated. “To be able to be well emotionally, I know I have to have sports in my life.” Haley’s journey with ITP has not been easy, and she describes it as a wild ride of ups and downs. She describes her greatest fear as “…having a brain bleed” and fearing her immune system could one day become resistant to Revolade like it did with Nplate.

As a strong young lady, Haley refuses to let ITP steal her happiness away. She continues to look for ways to still live her life staying true to who she is. She is now in her second year of university studying kinesiology with the goal to work in the area of rehabilitation. She loves teaching others about proper healthy eating habits, cooking, and spending time with her dog Wrigley and her friends and family. When Haley was asked what her strategy is for dealing with stress, she stated “My best way of handling stress is working out. It gives me motivation … and helps me feel better about myself.” She mentioned it is also essential to have friends who can help you through the hard days and enjoys listening to inspirational podcasts.

Haley is a strong advocate for herself and for others living with ITP. Earlier this year, alongside a few other patients living in Ontario, Haley shared her story with the Canadian Deputy Premier and Minister of Health Christine Elliott. In sharing her story, she helped educate politicians on why it’s important to have access to innovative treatments and to show the hardships she and her family endured to obtain life-saving treatment within a country that prides itself on universal health care. Haley also shared her ITP journey at PDSA’s Canadian Regional Meeting (May 2019) and the Externally-led Patient- Focused Drug Development Meeting (July 2019). While she was nervous to share her story in front of such a large group of people, she wanted to inspire others as an older child/youth living with ITP, and wanted to remind others with ITP to look after their mental health and to find ways to continue to do the things they love. “For me, I really struggled the first few months as everything I loved I was told I could no longer do, but I found that as I started to understand what I was going through with ITP, I could find ways to live my life and still do some of the things I love to do... I just had to be more careful.” Team Haley is also a proud supporter of PDSA’s Pump It Up for Platelets annual walk/run fundraiser.

When Haley was asked to describe some life lessons she had learned from living with a rare disease, she said, “I have learned that we take our health for granted....we don’t realize how lucky we are to be healthy and to be able to do things until we are no longer able to do them. I also realized how little people know about or understand rare diseases and how frustrating that is when trying to explain your illness and try different medications to see what works. I thought the doctors would have answers for me, but many of them had little knowledge of ITP, and everyone with ITP can be quite different.”

Haley continues to be an inspiration to all through her determination to succeed, her unwillingness to let her disease define her, and her kindness towards others and desire to help others navigate life living with a rare illness. ■