

# 2018 HOLIDAY LETTER

## Happy Holidays from the Proteus Syndrome Foundation

[www.proteus-syndrome.org](http://www.proteus-syndrome.org)

HELLO from my family to all of you! Here is a brief update for 2018 and a quick look into the near future for the PSF. This past July we had the 7th Annual Swing for Sunshine at the Cheyenne Mountain Resort in Colorado Springs. This year we broke all fundraising records for the tournament and brought in \$37,000! Thank you to our sponsors RMC Distributing, Coors Light, Miller Lite, ArQule, Pacifico, Bristol Brewing Co. and of course Cheers Liquor Mart. Thank you also to all the golfers, hole sponsors, and volunteers. We have the BEST volunteers!

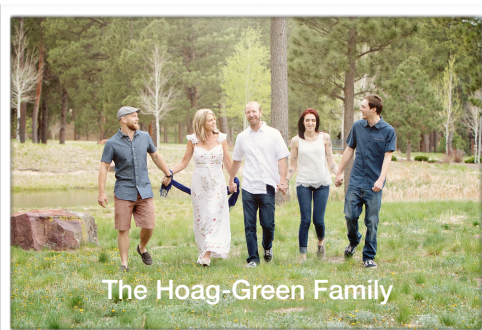
This year we lost a very special little boy. Trey Widner lost his battle with Proteus syndrome in August. Everyone who was at the last family conference was touched by this audacious, sweet and amazing little boy. If you didn't get to meet Trey and his family they are featured in the upcoming documentary Patient Number 5. My son, Cooper was very close to Trey and wrote a piece about him which you can read in this letter. Please send your prayers to this precious family. We have been working to solve this syndrome since 1991. It is absolutely devastating that even with the medical/pharmaceutical advances, we are still losing our precious children. The fight will go on until we defeat this syndrome.

In 2019 you will see the Proteus Syndrome Foundation move forward with our world wide outreach. We must get to these patients and let them know that there is support for them. So often families come to us or to NIH when they are 12, 18, 30+ years old. *Where have they been? Why didn't they know about us?*

There are several ways we are planning on expanding our reach. The first is the documentary Patient Number 5. It is getting close to completion and it tells a complex human story of the struggle not only from the families but from the doctors to find the cause and cure for this syndrome. Once this is finished we are hoping (with fingers crossed) that it will be picked up by Netflix and shown to worldwide audience.

The PSF UK & PSF USA are combining our websites to work towards one message from both of us. Tracey will still be working on her end as will we in the US but we believe that it will be less confusing to families if there is **one** place they can find all the answers. This is in the works now and should be complete by the end of the year. Our website address will remain the same for the USA.

Our parent portal on the website is up and running. This is a place where families can communicate with each other, compare doctors, surgeries, etc.. This is a password protected part of the website for families only. If you would like access and you are a family please go to our website and sign up.



**And last.... we will be having a PSF Family Conference in the fall of 2019!! No details yet. As soon as we have the date we will make sure to let our families know so you can clear your schedule.**

We wish you all love. Kim Green & The Proteus Syndrome Foundation Board of Directors.

## Trey Widner by his friend Cooper Hoag

When I first met Trey Widner, it was at the Children's Inn at the NIH. I was there to film his meeting with Julie Sapp and Dr. Biesecker, with the help of Alex Fahl and Ian Brander for the documentary, Patient Number 5. At first, Trey was a bit shy but always very polite. During this, we were just filming from a background standpoint, and the following day is when we got to sit down with Summer, Troy, and Trey to do a proper family interview for the documentary. It was during this interview that we learned about the social struggles Trey went through, about how kids would tease him or always ask him questions about what happened with his leg or his hands. His response to these kids would be, in that southern drawl he had, "hush your mouth," or "a shark bit it off". He had a sense of humor! Trey was a little spitfire.



Now, during this interview, Trey started to get a little uneasy and started to play with his mom Summer a bit with some funny jokes and then he told us how he would wrestle Troy, his stepfather, at home. Since we were filming an interview, it was the obvious choice that, at that moment, Trey decided it was time to challenge Troy to a wrestling match. Troy declined the invitation to wrestle, so naturally, Trey pointed to me, a person he really didn't know and said, "how about you, big boy?" He waved me over saying, "you can't beat me." ...so I did what any 26 year old, former college wrestler would do, I wrestled the one legged Trey in the middle of the interview and the kid showed me a thing or two! It was that moment, that I knew I loved this kid. He had a heart of gold, he could tease and trick you,

and laugh so hard that the whole room would smile and laugh with him.

Since I moved out to Atlanta about two months after meeting Trey, I was able to go visit him and his family every so often in Harlem, GA. While I was out there at the house, Trey and I would play basketball, play Madden or Call of Duty on the X-Box, and we also had a giant nerf gun war around the house. Trey even sent me a pretty sweet freestyle rap video for Christmas.

Trey was surrounded by an absolutely amazing and loving family. His step sisters, Bailie and Sophie, are the most loving and compassionate sisters I have ever met. For being so young, they understood so much about Trey's condition and were very mature about it. Every time I saw them, they were always positive, happy, loving and able to live in the moment with Trey.

My own mom has dedicated her life to finding a cure for Proteus syndrome my brother, Alex, died in 1999 due to complications from this syndrome Proteus has surrounded me my entire life. And now here is Trey in 2018 sick from lung tumors that completely compromised his breathing...all because of Proteus syndrome. The drugs are arriving with hopes they will stop the syndrome, but they are not good enough yet.

*On Friday, August 10 at 9:31am Trey passed from complications of Proteus syndrome.*

Trey's passing has devastated first and foremost his family, the entire Proteus community and me. I miss his laugh, his smile, his sense of humor, his rapping, everything about that kid is missed.

Rest in peace sweet Trey. You will never be forgotten. Love, Cooper.



## **‘Are we there yet?’** *An update on treatment trials by Dr. Leslie Biesecker and Julie Sapp.*

We are on the road to an effective treatment for Proteus syndrome and this is encouraging and reason for great hope. All of us have feelings of frustration, however, at how long this journey seems to be – like being on a long car ride, we too are eager to arrive at our much-anticipated destination. There is good reason to be optimistic about treatment for this devastating disorder – we have seen encouraging evidence of benefit from just the first few patients. At the same time, it is very unlikely that the first trial, or even the first several trials, will address all of the treatment needs of a disorder that can affect so many different organs and tissues in such remarkably variable ways. To help patients and families to understand the complexities of the efforts that are underway, this article will lay out some of our thinking on this complex topic.

The most important thing to keep in mind is that research participant safety is first and foremost in the minds of our team. Patient safety, thankfully, is also a major concern for others who evaluate our research, such as our ethics board and the FDA. It is very challenging, but essential, to be cautious and careful with everything that we do. No one wants this process to go slowly. At the same time, caution sometimes requires that we don’t speed ahead to minimize the chances of harm and to carefully consider every decision that we make. While it may seem attractive to rush ahead, a single major avoidable adverse event from an experimental drug can set back clinical trials for years. This harm is felt immediately and directly by the participant in whom it occurs, but also it can delay further research for all other patients.



The second thing to keep in mind is that there are several different ways to evaluate treatments for Proteus syndrome. The NIH group is focused on developing an approach to long term, low dose, minimal risk treatment that will prevent manifestations of Proteus syndrome from developing and/or worsening. Others are focusing more on shorter term, higher dose, higher risk approaches to try to ameliorate some of the symptoms in patients with more advanced disease. Both strategies are important and useful – we would like to think that all patients in the future will be diagnosed early (and benefit from low dose, long term treatment) but the unfortunate truth is that patients are still being discovered by us and others, late in the course of their disease, where shorter term, higher dose therapy is the only option. This is not ideal as we are not optimistic that treatment later in the disease will be as effective – it is going to be difficult or impossible to reverse severe manifestations. We strongly believe that the best hope for future patients is to prevent severe manifestations from occurring in the first place. An added benefit of this approach may be that it is safer and could allow us to experiment with a dosing schedule that allows for occasional drug-free intervals in a child’s or adolescent’s life.



We have finished our first low dose trial with one full year of treatment and are working hard to start the next trial soon. Again, this will be a long-term trial and will include an opportunity for higher dosing than our first trial. Even this higher dose will be lower than what others are considering – our goal is to provide years of treatment with minimal side effects. We will enroll younger patients than in our first trial (possibly as young as 5 years old) and we have been working hard on a design that allows all enrolled patients to receive drug (a non-placebo study). Conversations with important agencies such as the FDA and the NIH ethics board have been promising and finalizing the design and execution of this study remains our top priority. We are optimistic that these multiple approaches to this

difficult disease will open new routes to the destination we all hope for, which is an effective treatment that will yield a normal lifespan with improved outcomes for our patients and your loved ones.

## Jordan Whitewood-Neal by Tracey, Jordan's mom



Hello everyone – I thought you might like an update on Jordan and what he has been up to over the last few years. Following his amputations, Jordan then had spinal surgery as his spinal cord was being compromised by bony overgrowth and was starting to impact on his everyday life. Unfortunately the surgery did not go well and caused partial paralysis and Jordan ended up in the Spinal Unit for three months. All the time and effort to get Jordan walking on prosthetics was effectively wasted. At the same time the doctors discovered Jordan had a Chiari malformation and this had caused a bubble in his spinal cord. The day before planned brain surgery the doctors found that the bubble had disappeared and Jordan was no longer getting the

horrendous headaches so the operation was cancelled. The problem came back once more and disappeared once more!



Not one to let anything stop him in his tracks, Jordan went on to get his first class honours in Architecture. He also learned to drive a car. Jordan had a year working in London, commuting for four hours a day on the train. Jordan also now has an amazing girlfriend Jenny and they have been together for over a year. Jenny's family attended the recent UK Proteus conference and Jordan and Jenny did a talk together on their travels to Berlin. Jordan is a lot more widely travelled than me and he visited three countries in 10 days with his Uni friend Ethan, as well as Barcelona, Paris and Denmark!

Jordan has recently embarked on his Masters degree in Architecture at the University of Brighton – he lives unaided in student accommodation, helped somewhat by the fact that Jenny lives in Brighton! His health is pretty good these days although he is on life long medication as a result of two pulmonary embolisms and the spinal injury.

In conclusion, sometimes it can be difficult to hear about the struggles, but they are real. As a foundation we support each other tremendously.

Thank you for your support of the PSF.

We appreciate you!

For more information about the Proteus Syndrome Foundation:

[www.proteus-syndrome.org](http://www.proteus-syndrome.org) or find us on Facebook.

For more information about the documentary Patient Number 5

[www.patientnumberfivemovie.com](http://www.patientnumberfivemovie.com)

## Happy Holidays from the Proteus Syndrome Foundation!

The Proteus Syndrome Foundation, a 501c3 not-for-profit organization, is dedicated improving the lives of Proteus patients by funding AKT1 research. We focus on providing family support in the form of education and networking individuals living with Proteus syndrome with other families and medical professionals.