



August, 2019

Proteus Syndrome Foundation Family Conference

October 25 - 26, 2019 Hyatt Regency, Bethesda, MD USA

Please join us for our Biennial Family Conference. Learn more about the clinical trials and the drug they are using, family support, surgeries, and more.

Conference Schedule:

October 24 - Thursday night: Meet & Greet with hotdogs and hamburgers. Register and pick up your conference packet at this time.

October 25 - Friday: All day conference. Breakfast and Lunch will be provided.

October 26 - Saturday: All day conference. Breakfast and Lunch will be provided.

October 26 - Saturday Night Dinner in ballroom of hotel.

October 28 - Monday: Capital Hill - This is the day we will be going to Capital Hill to talk to our new congress about rare disorders and Proteus syndrome. There will be a training session during the conference.

We will be announcing presenters and schedules for the conference in the upcoming months. There will be breakout sessions for smaller groups, sibling groups, and more. We will also have our Saturday evening dinner in one of the ballrooms for a relaxed evening of getting to know each other even better.

To register go to www.proteus-syndrome.org and download the registration form, fill it out and mail to the address on the form. *You can mail your registration check in with your form or you can go to the PSF website and pay on our PayPal which can be found on the home page - donate button.* Financial aid is available. If you would like to apply for financial aid fill out that form and send it in with your registration.

We have special room rates at the Hyatt Regency available until September 20. To reserve your room call 1-301-657-1234 and let them know it is for the PSF Conference.

If you have any questions or would like help with registration please contact Kim or Trish at kim@proteus-syndrome.org or trish@proteus-syndrome.org

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.

Dear Families and Supporters,

I hope this newsletter finds everyone doing well. My family is doing good. Cooper is in Atlanta working as an actor and Ian is working towards a career as a rap artist! I am not sure how this all came about but I am proud of them.

The last few years have been quite exciting in the world of Proteus. I know there has been frustration that things are not going fast enough, but things are moving forward now with the trials between ArQule and NIH so you will be hearing from me more often concerning trials, if you are interested. Please keep up with our web page for new information as it comes out.

We also have decided to use Constant Contact, which is an email program that we can use to send newsletters and updates. Some of you received a test email from us. I apologize for that, but we needed to figure out which emails were old and which are good. A link will be *added* to the website where you can sign up to get on this email list. It will be much more cost effective to email newsletters than to send them by mail. The cost to print and mail newsletters is fairly substantial which is why we don't do them as often as I would like. I look forward to seeing everyone at the PSF Family Conference this October. Lindsey's book, "Out of Breath," will be available for purchase as well as the opportunity to meet Lindsey.

Welcome Trish! With all this being said you can see that my duties for the foundation have increased a bit with family outreach and working towards getting everyone possible diagnosed and into the trials or at least into our foundation for support. I would like to introduce Trish Velez as my assistant in all things Proteus. She has been helping me with all duties of the foundation. Trish has jumped in feet first and is already a huge asset to me and the foundation. Some of you may have already heard from Trish concerning the conference, contact information or other things. Please know that she is working right by my side, and you can trust that any information she receives from you is confidential.

There will be complete updates at the conference on the trials, updates on results of what the trials have shown so far, and many other things. We will broadcast the conference live for those of you who cannot attend.

From my family to yours,
Kim

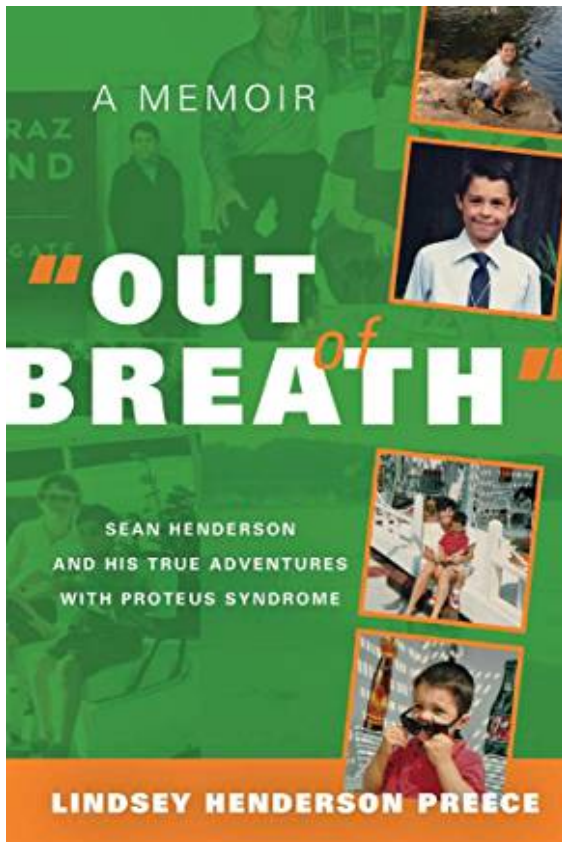
Welcome Cooper Hoag and Maddie Goodwin to the Proteus Syndrome Foundation Board of Directors!

They have both been involved with the foundation for years and will be amazing assets moving forward.



AVAILABLE ON AMAZON

Proceeds from "Out of Breath" benefit the Proteus Syndrome Foundation.



"Out of Breath" A Memoir: Sean Henderson and His True Adventures With Proteus Syndrome

by Lindsey Henderson Preece ~ Sean's Mom

Forward

I wrote this book from my journals that I kept during Sean's nine year war with Proteus Syndrome. It's taken me 15 years to finally let go of these pages. This isn't one of those feel-good books where the mother finds strength and purpose in her child's death. Frankly, I'm still a wreck. Sean's death blew a hole through my soul, through my very being. While I wrote about what I was going through and what was every mother's nightmare, I wanted to tell Sean's story of grace and incredible courage. While most people who knew us knew about all the surgeries and trips to the National Institutes of Health (NIH), most people had no real idea just what Sean went through those nine long years. An old soul and wise behind his years, he would plow through each challenge with his customary intelligence, analytical mind and wicked sense of humor. While it's sad what Sean went through, he would never want you to be sad or feel sorry for him. Ever. If you spent any time around us, you know how much we laughed and what a good time we had in spite of Proteus. Did you ever meet a person so present, so beautifully alive and joyous as Sean? When you remember Sean's sense of humor, quick wit, and his love of animals, I bet you smile.



FISHIN' FOR TREY!

Bass Fishing Team Tournament

Many of you know Trey Mills from our last family conference. Trey lost his battle with Proteus syndrome on August 10, 2018. Trey touched the hearts of everyone he met and always had a smile on his face. Trey loved bass fishing and watching his Georgia Bulldogs play.

The family decided to host the Fishin' for Trey fishing tournament in Trey's memory with the proceeds benefiting the Proteus Syndrome Foundation.

The Inaugural year the tournament raised \$3000!

"There is no footprint too small to leave an imprint on the world".

Trey Mills - 1/26/11 - 8/10/18



Mirsantab - What is this drug we keep taking about?

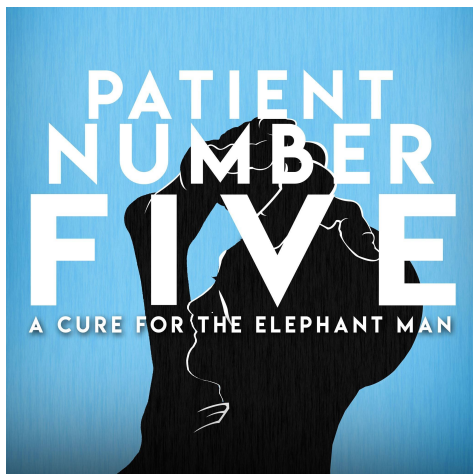
Overview/Mechanism of Action

Miransertib (ARQ 092) is an orally available, selective, pan-AKT (protein kinase B) inhibitor that potently inhibits AKT 1, 2 and 3 isoforms. Additionally, it binds both the active and inactive forms of AKT which directly inhibits and prevents membrane localization, respectively. Dysregulation of AKT has been implicated in a variety of rare overgrowth diseases and cancers; however, there are currently no approved inhibitors of AKT. AKT inhibitors, either as single agent or combination therapy, show significant promise in molecularly defined patient populations. We are currently initiating a registrational trial in both Proteus syndrome and PIK3CA-Related Overgrowth Spectrum (PROS) and further information related to these diseases and trials can be found in our orphan disease-focused presentation, PROS and PS Primer 2019.

Precision Medicine

The AKT1 mutation is believed to play a role in multiple cancers and overgrowth diseases, such as Proteus syndrome and PROS. The AKT pathway when abnormally activated is implicated in multiple oncogenic processes such as cell proliferation and apoptosis. This pathway has emerged as a target of potential therapeutic relevance for compounds that inhibit its activity, which has been linked to a variety of cancers and non-oncology indications. Preclinical research has demonstrated that miransertib suppresses PIK3CA/AKT1 mutant dependent kinase signaling and demonstrates anti-tumor activity in molecularly defined tumor models.

A landmark discovery by researchers from the National Human Genome Research Institute at the NIH demonstrated that a somatic mosaic mutation in the AKT1 oncogene is the underlying genetic alteration that causes Proteus syndrome. A spontaneous point mutation – a single-letter “misspelling” in the DNA of the genetic code — in the AKT1 gene during embryological development is responsible for activating the tissue growth characteristic of Proteus syndrome. The identification of the causal mutation of Proteus syndrome allows for the development of molecularly targeted treatments.



Patient Number Five, the documentary focused on the evolution of Proteus Syndrome treatment as well as the struggles of families affected, is currently in the editing phase. With such a vast amount of information provided by the NIH, patients, and ArQule in interview and day in the life footage, this will take some time.

The documentary team has just returned from overseas shoots in Australia and Italy, where principal photography for the feature film reached its conclusion, with the final stories being Sebastian and Dr. Kiara Leoni.

Both of these concluding stories will illustrate reasons to believe that the trial drug ARQ092, is well on its way to becoming an effective preventative measure in young children affected by Proteus, as well as a long term treatment for adolescents and young adults experiencing overgrowth.

Updates from Patient Number Five, can be found on the movies Facebook page.

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