**Update on VALO Clinical Trial**

We are excited to report the Palvella PC program has had a "last" and two "firsts" over the past few weeks. The last patient in the primary phase 2/3 VALO trial for patients with K6a, K6b and K16 completed the final treatment period and moved into the follow-up phase. Results of the study will be available in Q4, only a few months away!! In addition, a few other “firsts” have occurred in September 2020 - the first patient has entered the VALO K6c/K17 sub-study and the first patient has entered the extension study.

Patients who participated in the phase 2/3 VALO trial may be eligible to enroll in the extension study if they, and their study doctor, think the study medication improved their PC symptoms.

Next up, recruitment will soon begin for a PK sub-study. A PK study is an essential part of routine drug development and required for potential FDA approval of the study medication. Please stay tuned for information to come about recruitment efforts for this study as we will need your help in participating to make it a success!

Throughout the COVID-19 pandemic, Palvella’s team has successfully overhauled the entire study to a home health trial to ensure patient safety and the continuation of the trial. Since March 13, 2020, date of the national emergency declaration, over 100 remote study visits took place and approximately 38 patients completed the study.

PC Project is grateful for the Palvella team and for PC patients who have selflessly participated in the different aspects of this trial on behalf of all patients.

**NEW! PC Project’s Legacy Society**

Pachyonychia Congenita Project (PC Project) has been recognized since 2004 as a public charity in the USA. With limited staff and a small budget, PC Project connects patients, researchers, and physicians throughout the world in a united effort to help those with Pachyonychia Congenita.

Our new Legacy Society preserves and sustains our important work, while also recognizing those donors who make a planned gift to PC Project. Legacy Society membership is one of the simplest ways to show your commitment to the future of Pachyonychia Congenita Project.

PC Project’s Legacy Society welcomes all individuals and couples who have generously included PC Project in their estate plans and/or as beneficiary of a charitable trust, retirement plan, or life insurance policy.

Visit the website at the following link to learn more about the PC Project Legacy Society and all the Ways to Give [pachyonychia.org/legacy/](http://pachyonychia.org/legacy/)
The Legacy From a PC Patient’s Great Uncle, Robert Hansen

Robert Hansen, the great uncle of PCer, Allison Block, passed away in 2019. When Mr. Hansen’s estate distributed in 2020, Allison’s grandparents, Robert and Marilyn Bramer (sister of Robert Hansen) generously gifted a significant portion of their inheritance to PC Project. Mr. Hansen was always a great supporter of PC Project and now shares his legacy for patients like Allison who very much need treatments and a cure for PC.

PC Project is incredibly grateful for the Hansen, Bramer, and Block families for their generosity and their trust in our important cause.

My PC Experience

By: Dhara Leite Lopes
(In Portuguese and English)

Portuguese

Meu nome é Dhara Leite Lopes, sou brasileira. Atualmente tenho 21 anos e por muitos anos da minha vida não tinha um diagnóstico para o meu caso, fui “tratada” diversas vezes de maneira desnecessária. Apenas em 2007 que uma dermatologista, de um hospital renomado em Minas Gerais, me informou sucintamente sobre a PC, que de acordo com o fenótipo apresentado eu era portadora dessa doença.

Porém, sempre fui muito curiosa, então comecei a pesquisar sobre isso, mas infelizmente as informações em português são pouquissimas, o que dificultou muito. Foi então que em 2013 minha mãe resolveu fazer uma publicação sobre mim em sua rede social, pedindo ajuda das pessoas para que indicassem profissionais interessados em pesquisar sobre PC. Foi a partir dessa publicação que alguém me indicou o PC Project, onde me permitiu conhecer um pouco mais sobre essa doença. Porém, apenas em 2015 que fiz o teste genético e fui diagnosticada com PC pelo PC Project.

Diante disso, vejo que ainda há uma escassez de informações sobre PC em português, na verdade, de uma maneira geral, para nós brasileiros, que nem sempre tem um entendimento aprofundado em outro idioma e sobre o assunto. Por isso acho tão importante a divulgação sobre PC, a possibilidade de
conhecimento para outras pessoas, que talvez estejam passando pelas mesmas coisas que eu.

Assim, venho pedir que você, em especial brasileiro, que esteja lendo meu relato, divulgue a PC, explique para todos que você tem contato, seja canal de informação, isso faz toda diferença.

**English**

My name is Dhara Leite Lopes and I am Brazilian. Currently I am 21 years old and for many years of my life I did not have a diagnosis for my case. I was “treated” several times in an unnecessary way. It was only in 2007 that a dermatologist, from a renowned hospital in Minas Gerais, briefly informed me about PC and that according to the presented phenotypes, I had this disease.

However, I was always very curious, so I started to research about it, but unfortunately the information in Portuguese is very little, which made it very difficult. It was then that in 2013 my mother decided to make a publication about me on her social network, asking for help from people to refer professionals interested in researching PC. It was from this publication that someone referred me to the PC Project, where I was able to learn a little more about this disease. However, it was only in 2015 that I did the genetic test and was diagnosed with PC by the PC Project.

In all these years, practically every time I went or I go to a doctor, I have to explain in a simple way what PC is, because many are unaware. That’s because I know and study about it. But who doesn't have this information?!

Given this, I see that there is still a shortage of information about PC in Portuguese. In fact, in general, for us Brazilians, who do not always have a thorough understanding in another language and on the subject. That is why I think the dissemination about PC is so important - the possibility of knowledge for other people, who may be going through the same things as me.

So, I come to ask that you, especially the Brazilian, who is reading my report, divulge the PC, explain to everyone that you have contact, be an information channel. That makes all the difference.

**Note:** PC Project invites all patients or their family members to share their PC experience (in any language) with our PC community. Please send to: info@pachyonychia.org

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**From PC Project’s Social Media:**

**A Rollercoaster Achievement**

Posted by Kerry Briggs-Evans, mom of a spontaneous PCer:

This is my son Tony. From the day he was born (with teeth) we knew there was something wrong. By 5 weeks old, his nails were so thick, we couldn’t cut them. At 8 months old, doctors told us he may have a condition called Pachyonychia congenita and he would most likely be deaf and “mentally retarded”. If I’d believed them, I would have been devastated, but I didn’t, I knew my little boy. He hit all his milestones and would wake up at the slightest noise... I took him to a different doctor and found the PC project website (my godsend).

Here he is at 12—he has K17, he’s as bright as a button and certainly not deaf; this kid can hear the words ‘pocket money’ from 3 rooms away.
I wanted to share one of Tony’s achievements, it may be trivial to some, but not to him. This week he completed the big 7 at Alton towers theme park here in the UK. It’s something he’s wanted to do for a long time, but couldn’t, because the walk around the park was too much for his feet to cope with.

But he finally did it; he walked around the entire park and waited in line for every ride. I’m so proud of him, I’m thrilled he got to achieve his goal, but I’m proud because he overcame his fear of the pain. His feet have blistered terribly and he’s resorted to crawling around the house because he can’t put his feet down, but he’s still got the smile on his face as he shuffles past me saying, “It was all worth it mum.”

**FACEBOOK BIRTHDAY FUNDRAISERS**

Happy Birthday and great thanks to Charity Oswald, Brittiney Steinnmeyer, and Mary Cook for creating fundraisers to support PC for their birthdays. These individuals successfully invited their family and friends to donate to PC Project.

These fundraisers raise awareness about PC and support the mission of PC Project.

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**PCer in “Small For Good” Campaign**

Congratulations to beautiful Luiza who has PC! She will be in a magazine for a “Small for Good” campaign. She will also be in a children’s clothing store. Luiza’s mom, Kamila, writes, “She and so many others can do anything! Self-esteem, good humor, necessary care, a lot of faith, and everything is flowing.”

Kamila wishes to bring encouragement, happiness and positivity to all with PC. She believes that one day there will be effective treatments.