2020 France Patient Support Meeting

The European Patient Support Meeting (PSM) will be held in Roissy, France (near Paris), June 4-6, 2020. This is the only PSM to be held in 2020. (The 2021 meeting will be in the United States, location to be determined.) The France meeting will be held in English and French with translation. Reduced fees apply before April 1. Please register as soon as possible at pachyonychia.org/2020psm/

Many thanks to Le Coeur Au Pied for their help and support in organizing and planning this meeting with PC Project staff.

VALO Study Update

Do you know some treatments for diseases never get approved because there aren’t enough patients for the required clinical trials?

This is not the case for the PC patient community! The Palvella team has continually been impressed by the responsiveness of PC patients who have been willing to try this topical gel on behalf of PC patients worldwide.

This primary study will soon close enrollment. If you are interested in participating in the study, especially if you have already completed genetic testing and know your mutation, please indicate your interest immediately at surveygizmo.com/s3/4854812/Phase2-3 or Pachyonychia.org/valo/

A Letter About the Clinical Trial from a Mother & Wife

Dear participants of the current clinical trial and those who are eligible to participate in the trial but haven’t yet enrolled,

First off, this is a letter of gratitude. Thank you from the top to the bottom of my heart to all those PC patients who are giving up their time to participate in the VALO trial. As I said goodnight to my 12 year old son this week, after a particularly hard and painful day on his PC feet, he asked me if there would ever be a cure. As you all probably know, seeing someone we love in so much pain and being unable to help, is one of the hardest things to have to experience. It’s awful!

However, that night, I was able to explain to him how PC Project was recruiting patients into a study trying out a new treatment. When he asked when the results would be known, I explained not for a while as the study still needs people with PC to sign up and participate. Immediately he said he’d do it. Of course, he can’t as he’s too young, but as we also live in the UK, neither my husband nor any other PC sufferers from around the world can enrol. The trial is only open to those of you in the US and Canada.

In all the years of knowing about PC Project, I think this trial is the closest we’ve ever been to having a chance of finding something that may stop the pain. I ask any of you who are eligible and are considering, to please go for it! To do it on behalf of us who can’t and to do it for the future generations! Yes, being involved in research can be scary and takes time, but your help is desperately needed to understand what works and what doesn’t. Data from studies like these will be key to get companies to help us!
So, as we watch for news of the trial anxiously from across the pond and from the eyes of someone who is too young to participate, we want to thank all of you who have taken the leap and gotten involved. To those that are considering being involved, we encourage you, and most of all, thank you to PC Project for continually driving forward in the search for a way to stop the pain.

Sincerely,
Julie Peconi, wife & mother of two PCers

**NEW DATA ABOUT PC**

Two issues many PC patients deal with that aren't typically associated with PC are **deep itching** and **neurovascular-like structures** (as seen in the below photos) in the calluses.

As new patients join the registry and share their experiences, PC Project is ever-learning about PC and how it affects patients.

In October 2019, PC Project surveyed its genetically confirmed patient community with an IRB approved registry addendum. With 350 responses, **69% of patients reported they experience deep itching**, an itch that is under the calluses that is difficult to reach.

Furthermore, **62% of patients have neurovascular-like structures**. In truth, what they are exactly is not known. What is known is they significantly increase pain and make it difficult for patients to trim their calluses.

These two symptoms of PC are not typically talked about in PC discussions but are clearly a part of life for a significant portion of the PC population. Data from the series of questions about these issues are being studied and will be published.

Thanks to all patients who participated in the questionnaire addendum to help researchers broaden their understanding of PC.

**PARTNERING WITH A DOCTOR FOR THE BEST PC CARE**

Because PC is ultra-rare, finding a dermatologist or primary care physician who knows a lot about PC is not nearly as important as finding a doctor who is willing to learn more about PC, work with, understand and listen to the patient, and connect with PC Project if needed. This is especially important when a patient needs a doctor's care for infections or other PC-related concerns.

Some of the PC patients in our registry have cultivated positive relationships with their doctors. Click here or read the story below for an example of a dermatologist who partners with her PC patients:

**MEDICAL COLLEGE OF GEORGIA PHYSICIAN HONORED FOR CARE OF FAMILY WITH RARE GENETIC CONDITION**

The American Academy of Dermatology has honored board-certified dermatologist Loretta Davis, MD, FAAD, as a Patient Care Hero for her role treating a family with a rare genetic disorder. The condition, known as pachyonychia congenita, makes nails grow abnormally thick and causes painful callouses and blisters on the soles of the feet. This can make seemingly ordinary tasks, like standing, walking, holding items, or even breathing, uncomfortable and challenging for many patients living with pachyonychia congenita.

Buff Farrow of North Augusta, S.C., was diagnosed with pachyonychia congenita at age 16 by dermatologist Dr. Jack Lesher, who had
also diagnosed her father, before Dr. Davis took over their care upon Dr. Lesher’s retirement. Farrow’s son was diagnosed at birth.

Dr. Davis helps the Farrow family effectively manage the condition and limit its effects on their daily lives. Because taking extra steps increases pain and causes blisters to form, the dermatologists helped the family acquire special accommodations, such as handicapped parking.

“Growing up with such a rare condition is challenging. The physical effects can be debilitating, but the psychological aspects of it are just as bad,” said Farrow. “While there is no cure for pachyonychia congenita, Drs. Davis and Lesher have helped us manage it as best we can.”

The genetic disorder is a result of mutated keratin genes that prevent skin cell filaments from properly forming. Less than 1,000 patients are on the International Pachyonychia Congenita Research Registry worldwide, but despite its rarity, the Farrow family credits the dermatologists for helping increase awareness of the condition.

“The doctor-patient relationship is always special, but it is especially rewarding to care for three generations of such an inspiring family,” said Dr. Davis. “Our dermatology division has learned just as much from Buff and her family as they have from us. They have helped improve our knowledge about managing pachyonychia congenita, which we’ve been able to share with our dermatology colleagues.”

The AAD created the Patient Care Heroes program to recognize physicians who transform patients’ lives by utilizing their expertise and collaborating with other physicians to treat serious skin disease.

“Living with a chronic condition—especially one that is rare like pachyonychia congenita—affects physical, mental and social well-being,” said board-certified dermatologist George J. Hruza, MD, MBA, FAAD, president of the AAD. “Every day, dermatologists like Dr. Davis help patients live life to the fullest while managing painful, serious, and often life-threatening conditions.”

To learn more about Dr. Davis’s work with Buff Farrow, visit https://www.aad.org/skinserious/stories-buff-farrow.

Congratulations, Dr. Davis, and thank you for caring about PC patients.

Birthday Fundraiser

Happy Birthday to Jessica White, who raised money for PC by creating a Facebook fundraiser in honor of the special day she was born. Thanks to Jessica and her supporters!

PC Superpower

Kerry Evans-Briggs, the winner of last fall’s meme-making contest requested her PC t-shirt prize to be for her son, Tony, who has PC.

Kerry wrote, “I honestly cannot put into words how much Tony loves his t-shirt. He wore it to school back to front, underneath his school shirt, just so when he unbuttoned his shirt he could rip it open like superman does. Then he flashed his friends the PC logo and declared that’s his superpower!

“Because he’s running with the idea that PC is his super power, this is his pop art project.” Thank you, Tony, for sharing your PC power.
All the PCers in the International Pachyonychia Congenita Research Registry (IPCRR) are superheros to us and to future generations! Thank you for participating!

You are helping to expand PC knowledge and are the reason why researchers and drug developers are interested in finding treatments. The summary graphs shown on this page are available online at pachyonychia.org/pc-data/

If you or your family members have PC and have not completed the online IPCRR questionnaire, consent form, and sent photos, please complete the forms online at https://registry.pachyonychia.org/s3/IPCRR or print a copy to fill out and mail at pachyonychia.org/patient-registry/

Each person who registers is vital to our progress. If you have questions, please contact PC Project at info@pachyonychia.org or at 801-987-8758.
2020 France Patient Support Meeting

There are currently 56 people registered (including 21 PC patients) to attend the European Patient Support Meeting which will be held in Roissy, France (near Paris), June 4-6, 2020.

The France meeting will be held in both English and in French, with translation services. For reduced fees, apply before April 1.

Please register as soon as possible at pachyonychia.org/2020psm/

Valo Phase 2/3 Clinical Study is Fully Enrolled

In only eight months, the Phase 2/3 study investigating PTX-022 (QTORINTM rapamycin) for pachyonychia congenita has now been fully recruited. Enrollment for this primary study has closed.

All patients who have participated in the study, in any way, are true medical heroes. Palvella looks forward to sharing top line study results at the end of this year.

Because of the trial design, some patients are screened out at different points. This is a normal part of the process. All information gathered from each person is extremely valuable.

The importance of each patient who joined the clinical trial cannot be stressed enough. This PC community is making its mark for being comprised of patients who are willing to sacrifice time and energy to be in a trial to establish the effectiveness of this or any other potential treatment for PC.

With the close of recruitment for the main study, recruitment will soon begin for additional studies needed for FDA approval. PC Project will continue to share news about each of these steps.

For more information on the VALO trial, please visit pachyonychia.org/valo/ or clinicaltrials.gov.

It’s All About Love

February is the month of Valentine’s Day in many countries, a time when people express their feelings of love for others. PC Project was established because of love and continues to run because of the service and goodwill of many people.

Here are just a few examples of the selfless, loving acts we’ve observed this month:

♥ Members from the PC Project Board of Trustees continue to sacrifice time, energy and resources to support PC Project and it’s mission.

♥ Trustees from PC Project Europe have selflessly worked to keep our European organization running and compliant, not a small task.
A mother of a PC patient assisted PC Project staff in translating and speaking with a mother of a PC baby who doesn’t speak English. This baby is a spontaneous case and the new parents needed support and sound care advice.

A PC advocate translated important documents for disability status from English into her native language for another patient.

An employee at Jet Blue, through volunteer service, earned vouchers for four one-way flights and donated them to PC Project. Two of these vouchers will fly a scientist to and from our upcoming IPCC Symposium where he will present his PC research.

PC patients continue to support and encourage one another in the private PC Facebook Chat. The level of kindness and love shown in that group is heartwarming.

The IPCC Genetics Team continues to donate their time monthly in reviewing cases, genetic testing, and answering questions.

On Valentine’s Day, supporters donated over $260 through a social media meme. In addition, our 62 PC Love Builders continued their monthly support.

These are only some of the many acts of kindness we see expressed on an almost daily basis. Love is what keeps PC Project running, until PC patients can someday literally run!

**FACEBOOK BIRTHDAY FUNDRAISERS**

The February love continued with four individuals who created fundraisers for their birthdays. The following individuals successfully invited their family and friends to donate to PC Project:

- **Kaili Bloedorn**—PCer
- **Hilary Misiano**—Mom and wife of PCers
- **Edward Pyner**—PCer
- **Olin Yingling**—Friend of PCers

Hilary’s description on her fundraiser included the words, “It’s my birthday in a couple of days and all I want is to raise a boatload of money for this AMAZING organization who has helped us more than I can ever say. It’s an inherited condition and it can be extremely difficult. It’s painful. No doctor ever knows what it is or how to help. We were in the dark trying to figure out how to manage it until we found PC Project.”

Thanks to each of you—and Happy Birthday!

**PC COMMUNITY SKILLS SURVEY**

Because PC Project has a limited staff and a small budget, we rely heavily on the volunteer services of patients, family, friends, scientists, physicians, and other professionals to keep our work moving forward.

We invite you to complete this short survey to help us gather a standby list of skilled people we can call upon when we have a need in a specific area.

[pcproject.skills.sgizmo.com/s3/](pcproject.skills.sgizmo.com/s3/)

We are a small but mighty PC community, and PC Project would like to learn more about your skills, talents and your interest in helping move our mission forward.
In addition, if you know someone who specializes in any of the areas, please encourage them to also complete the form.

pcproject.skills.sgizmo.com/s3/

Thank you for helping us reach our goals as we continue to do more with less.

**PC TownHall Meeting**

On Thursday, March 19 at 5:30pm PT (6:30pm MT, 7:30pm CT, 8:30pm ET), PC Project will host a townhall style webinar. This first–ever townhall meeting will tell about PC Project’s mission, explain PC, including the burden of living with it, and current research efforts.

Anyone interested is invited to register for the free meeting.

pcproject.townhall.sgizmo.com/s3/

This web meeting will last approximately 45 minutes. You can join by computer, smart phone or tablet, or you can join the listen only mode by calling from any telephone. Questions can be submitted in advance or after the meeting, but live questions will not be taken during the live meeting due to time constraints.

The webinar will be recorded so that those in other parts of the world can watch and listen at a more convenient time for them. If there is enough interest in this townhall meeting, additional ones will be scheduled in order to keep the global PC community updated.

**Shopping To Support PC**

Don’t forget to select Pachyonychia Congenita Fund as your charity when you shop. You can also shop in the amazon app and add the items to your cart, then login to amazon smile and purchase the items in your cart.

smile.amazon.com/
smile.amazon.co.uk/

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**IN MEMORY OF MARGARET MAIER**

The great-grandmother of PC patient, Allison Block, recently passed away.

Donations to PC Project were given in Allison’s honor and in memory of Mrs. Maier. PC Project expresses sincerest condolences and wishes the family peace and comfort.

**PALVELLA’S BOARD COMES TO UTAH**

During the month of February, Palvella gathered their board members in Salt Lake City and invited the PC team to a dinner and to present about PC and PC Project at their board meeting. While Palvella has been invested in learning about PC patients from day one, this was a special opportunity for PC Project to directly talk with Palvella Board members, answer questions about PC and PC Project, and share the accomplishments of PC Project and the burden of living with PC.

Because of the love and stories from the PC community that were shared, Palvella’s board felt they truly understood PC patients and the mission of PC Project. One board member even sent an encouraging note the next day to PC Project with a personal donation of $1,000.

These pictures feature only some of the Palvella board members and staff who joined PC Project in February.
HOPE in Uncertain Times

Because PC Project serves patients in over 50 countries, we are acutely aware that the COVID-19 pandemic is affecting our entire PC community. This is an unprecedented time, not only in our PC Project history, but in our personal histories as well. You are ever in our thoughts and our hearts.

Please be reassured that although these are uncertain times for all, PC Project continues to carry on its mission. We recognize some of our biggest annual events are scheduled for May and June. We are carefully monitoring the overall situation, discussing plans with our PC Medical and Scientific Advisory Board, and will follow government regulations in the various states and countries where we serve and plan to hold events.

Above all, PC Project will be good global citizens and will always put first the safety and health of our PC and our broader communities. We will keep you notified and appreciate your patience as we deal with this unprecedented situation.

And while there is uncertainty and even fear surrounding COVID-19 and the ramifications of a global pandemic, we are optimistic that we will get through this challenging time. If we’ve learned anything from our community, it’s that PCers know how to adapt, how to do hard things, and how to valiantly keep going, even when it hurts.

One of the quotes PC Project strives to live by is, “Life is meant to be enjoyed, not just endured (Gordon B. Hinckley).” This statement is particularly applicable when living with a rare, painful disease, but it also can apply to the current pandemic. While we are not downplaying the seriousness of the situation in any way, our sincere wish is that within this newsletter and in your own lives, you will find snippets of encouragement, joy and hope.

A Silver Lining: From the PC Facebook PC Patient Chat

The question was asked: “Hello PC friends. These are unusual times for most of us. How is everyone doing, PC or otherwise?”

PCers had some interesting remarks to share:

“Hello, I am holding on so far. Still working and thankful for it. I hope everyone is doing alright in this troubled time.”

“It’s actually doing my feet a favour as I’m not having to commute to work and wear work appropriate shoes.”

“Our household is in self isolation. Stuck indoors, feet up, doing our part to save the human race.”

“Keeping busy, kid doing school work, house chores and art!”

“Isolated at home with 2 of my 3 sisters. We’re all doing OK so far. Staying off my feet, which has been wonderful. Getting our groceries (minus TP) through delivery from Walmart & Publix. Beautiful weather here.”

“Great, doing what I do best - sitting.”
“Working at home with husband, college student and high school student. My feet like the slow pace.”

“Home office for me too! Less stress, less cysts! I’m doing good.”

“Jo bare hjemme, er noe ute å prøve å gjøre litt hagearbeid, men har så mye smert, men skal vel ikke klage så mye i disse tider (It’s just home, it’s something outside to try to do some gardening, but I have so much pain, but I guess I shouldn’t complain so much in these times.)”

“At last some rest from proper shoes. Never had enough time to make some long lasting blisters dry. And the best, spend all day quality time with my first 10-month baby daughter. Blessings to all PCers. Stay safe.”

“Noticed the other day that my feet have not been hurting since I have been working from home so that is a plus. But I went and got groceries last week and decided not to take the scooter and be “normal” for once.”

“Isolating right now. Feet are great, hardly any walking!”

**PC Featured in British Journal of Dermatology**

The March 2020 Rare Disease issue of the British Journal of Dermatology contains numerous articles on Pachyonychia Congenita. This strong emphasis on PC in an accredited journal is the result of collaborative efforts of members of the International PC Consortium that are comprised of scientists and physicians worldwide who work together to advance PC related research.

A number of the articles were made possible because of the information provided by PC patients who are part of the powerful International PC Research Registry such as this one:


Thanks to the dedicated scientists who care about PC and thanks to the PC patients who have joined the Registry in order to advance PC research efforts. These will be posted in the Research Articles section of the PC website.

**A Disabilities Champion**

Max Aaron Morris, a PC patient, was spotlighted recently in the Jewish News.


Here is an excerpt from the article:

“For the first year of secondary school, Max felt too self-conscious to use his wheelchair, hiding the pain he felt walking.

“Now the 15-year-old with pachyonychia congenita, a rare skin disease, is a proud disabilities champion, having successfully promoted Visibility for Disability week for the past three years.

“Activities include talks, inclusive sports events and a sign-language workshop. Praised as “engaging, optimistic and enthusiastic”, Max has empowered students and teachers to talk about their own disabilities and strive for better acceptance within JFS and wider society.”

Thank you, Max for inspiring others with your courage and for raising awareness about PC.
**PC Represented at Rare Disease Week**

PC Project took part in Rare Disease Week in Washington, DC February 25-28, 2020, joining more than 800 advocates to brainstorm Capitol Hill in support of greater federal funding for rare diseases. PC Parent and Board Member, Barbara Feinstein (her daughter Audrey is PC K-17), briefed members of Congress and their staff in the House and Senate on Pachyonychia Congenita and pressed legislators to increase federal funding in support of a cure or treatment for PC.

PC Project also displayed a poster about the burden of Pachyonychia Congenita and Barbara joined, Kathy Goin, a representative from Palvella Therapeutics, in speaking with researchers, clinicians, and rare disease representatives at the National Institutes for Health (NIH) about PC.

During the Hill Day, Barbara, Feinstein, second from the right, had meetings with members of both the Utah and Maryland Congressional delegations, including a Member meeting with Rep. Jamie Raskin, whose district includes the NIH; as well as staff-level meetings with Rep. Paul Sarbanes, who sits on the Energy and Commerce Committee, with legislative jurisdiction over the NIH and HHS, and Sens. Romney (R-UT), Lee (R-UT), and Cardin (D-MD).
Raise Funds For PC While Shopping

As online shopping becomes more convenient, please don’t forget that many of the stores you shop at will give a portion of the sale to PC Project. Check out the following sites and see if your favorite stores are on the list:

Use iGive

Or, Give As You Live in UK/Europe
www.giveasyoulive.com/join/poproject-eu

If you shop on Amazon, please remember to make your purchases through AmazonSmile and select Pachyonychia Congenita Fund as your charity when you shop.

smile.amazon.com/ or smile.amazon.co.uk/

Facebook Birthday Fundraisers

Happy Birthday and great thanks to those who created fundraisers to support PC for their birthdays. These fundraisers raise awareness about PC, support the mission of PC Project, and have no Facebook fees because PC Project is an accredited charity.

The following individuals successfully invited their family and friends to donate to PC Project through a Facebook Fundraiser:

Amanda Soderstrom—PCer
Jillian Hubbard Robertson—Family member of PCers

We hope you had a wonderful birthday!

PC Town Hall Meeting

On Thursday, March 19, 2020 PC Project hosted a townhall style webinar. This first-ever townhall meeting shared the basic fundamentals of PC, what living with PC means, an update of the VALO trial and other potential PC studies.

One PC patient who watched wrote, “Thank you for all you do on our behalf. I was moved to tears a number of times when watching the town hall meeting by how much care, love, commitment and enthusiasm you bring to the pc community. Your work is truly inspirational.”

If you missed the meeting, you can watch it here: pachyonychia.org/pachyonychia-congenita-town-hall-may-19-2020/

In Memory of James P. Mages

A generous donation was given to PC Project in memory of James P. Mages, who fought a two year battle with pancreatic cancer. His cousin, Bob Bramer and Bob’s wife, Marilyn, chose to honor James by donating to PC Project. Bob and Marilyn’s granddaughter has PC.

With the donation were the words, “Thanks to all of you at PC Project who work so hard on the mission to find a treatment/cure for PC. We pray this will help the mission of PC.”

PC Project expresses sincerest condolences to the family of Mr. Mages and wishes them peace and comfort.

Fast Enrollment for VALO Study Makes the News

In only eight months, the Phase 2/3 study investigating PTX-022 (QTORINTM rapamycin) for pachyonychia congenita has now been fully recruited. This rapid enrollment is an extraordinary feat for a rare disease and was possible because of the participation of patients in the International PC Research Registry.

Please click here to enjoy the press release about this impressively fast enrollment.

Also, while many PC patients are completely through the trial, Palvella has been working closely with clinical trial site coordinators to ensure continued study participation in the midst of the COVID-19 pandemic.
Awareness for Understanding PC

PC patient Stephanie Jones created car decals for others to read when they see her park in a handicap spot and don’t understand why she needs that closer parking spot. Because this patient was in so much pain from walking, he was able to brush off the mean words. Still, it was not a nice note to receive, especially for a young man who has always wanted more than anything to have the ability to walk, run and play sports, and who would gladly park at the far end of a parking lot and walk long distances if he could.

Stephanie is doing something proactive for herself to help raise awareness about PC.

Stephanie is not alone in her need to have others understand the invisible battles PCers face. Many patients have experienced a lack of understanding from others. One PC college student has a handicap placard for his car, which he rarely uses because he is embarrassed about needing an accommodation for his PC. One day, however, a store parking lot was very full and his feet hurt terribly. This young man parked in the handicap stall with his doctor-endorsed, government-approved handicap parking pass. When he returned to his car after shopping, he found a nasty note on his windshield telling him, among other things, that he shouldn’t be using a handicap spot when he didn’t need to.

VALO Trial Continues Amidst COVID-19 Social Distancing

Because of the outstanding efforts of Palvella staff and participating PC patients, the current VALO clinical study to test a treatment for PC is still ongoing, in spite of COVID-19 challenges.

The Palvella team was anticipating and preparing for coronavirus long before the pandemic got as serious as it is now. Palvella’s foresight and commitment to patient safety has resulted in the seamless running of the trial which is utilizing tele-health options. Current FDA guidance appears to be supportive of clinical trials continuing remotely.

In addition, PC patients who are still in the study are impressively staying compliant with trial guidelines, applying the medicine as directed, and doing all that’s required to capture accurate data. (Some clinical trials for other diseases have been cancelled because patients did not want to focus on participating in a trial at this time.) Thanks to committed PC patients and to the dedicated Palvella team for your diligence.
PC Project Still Thriving
Thanks To Generous Supporters

Many nonprofit organizations are struggling and even closing due to the fallout of COVID-19. But because of you, that is not the case for PC Project. Your generosity, especially during the giving season at the end of 2019, has enabled our programs and services to continue during the pandemic. Additionally, each time we receive a donation, especially from the monthly PC Love Builders, our hearts overflow with deep gratitude for your support.

Please be assured that because of you, PC Project not only remains solvent at this challenging time, but the staff continues to work hard each day (while physically distancing) to further the mission of PC Project.

Admittedly, we were disappointed at the need to cancel the annual International PC Consortium (IPCC) Symposium where we had invited researchers from all over the world to come together to share and collaborate regarding PC-related research. Because of travel restrictions and our desire to be good global citizens, this meeting could not take place.

However, PC Project has connected with each person who had planned to attend. These are researchers who care very much for PC patients. Their hearts are still with us, they are eager for the next meeting to be held during safer times, and they are continuing their work in their respective labs and clinics throughout the world.

This facilitation of research and all the other work we do as the only organization in the world for PC patients, doctors, researchers and drug developers is only possible because of your continued support. Thank you for keeping PC Project a strong organization, even in the midst of a global pandemic.

The Power of One Teenager

Twelve year old French patient, Paul Duquesne, shared on Facebook about the pain and difficulties of PC. This was to help his friends and relatives learn and care about this disease. Paul then invited them to make a donation to support PC research. Paul would like to be cured one day and have a normal life. He’s also grateful for PC Project’s mission to empower research and find a cure.

As a result of his efforts, Paul raised 500 euros and taught a lot of people about PC!

Warmest thanks to Paul and his loved ones for their support and enthusiasm. Paul has hope for the future and so do we! Special thanks to Le Coeur Au Pied for their continued support for French PC patients and PC Project.

France Patient Support Meeting
Postponed Until 2021

Due to the COVID-19 pandemic, PC Project has cancelled the June 4-6, 2020 France PC Patient Support Meeting.

After talking with our physicians, scientists and representatives from Le Coeur Au Pied, PC Project felt this was the best decision under the circumstances. In addition to travel restrictions and economic hardships, COVID-19 is affecting many of your in very personal ways. We feel
your safety and well-being is the most important priority right now.

The next patient support meeting will still be in France, but until the pandemic calms down, we are reluctant to schedule any sort of in-person meeting. We plan to hold a special PC web meeting, most likely on June 4th at 6pm in the France time zone (5pm UK, 12pm ET, 10am MT). Stay tuned for more details.

If you made your own hotel room reservation, please contact the Golden Tulip and cancel your reservation by phone 01.34.29.03.80 or by email: seminaire.roissy@goldentulipcdgvillepinte.com

If you had previously paid the fees for this meeting, please contact info@pachyonychia.org.

IN MEMORY OF PCER HELEN SCOVILLE

PC Project extends sincerest condolences to the family of Helen Scoville, a patient in the PC registry and a supporter of PC Project. Helen has a number of PC family members who are also part of the registry.

When Helen died from complications due to COVID-19, her family was unable to have a traditional funeral and had a private family graveside service. To honor her, some family members gave generously to Helen’s favorite charity, PC Project, including Helen’s brother, Solon Horn and Helen’s niece, Victoria VanHorn.

We are grateful to have associated with Helen. She and her family members have been strong supporters of PC Project for many years. We send her family our love and wish them comfort and peace as they cherish their happy memories of Helen.

Click here to read about Helen’s remarkable life.

JOIN PC PROJECT ON SOCIAL MEDIA

Please like, subscribe, and connect with us on all our platforms:

- PC Website https://www.pachyonychia.org/
- YouTube Channel https://www.youtube.com/user/Pachyonychia
- Facebook Organizational Page https://www.facebook.com/pachyonychia/
- Instagram Profile https://www.instagram.com/pachyonychia/
- Twitter Profile https://twitter.com/Pachyonychia
- Linkedin Company Page https://www.linkedin.com/company/pc-project

Having you be active on PC Project social media by liking and sharing PC posts raises awareness and provides features to assist us in getting more information to you. For example, we currently have 229 YouTube subscribers. If we get 1,000 subscribers, we will be able to live stream from a mobile device to our YouTube channel. This is a feature we wish to use at future patient support meetings. Please subscribe and help us reach 1,000 subscribers before the next meeting in 2021.

And, if you have ideas for us about content or how to enhance our social media presence, please let us know.

RAISE FUNDS FOR PC WHILE SHOPPING

Don’t forget that many online stores will give a portion of the sale to PC Project when you use the following sites to get to the store.

- igive.com/welcome/lp4/cr39.cfm
- smile.amazon.com/
- smile.amazon.co.uk/
- giveasyoulive.com/join/pccproject-eu
**Pedaling for PC**

PCer, Nathalie Kilchoer and her family organized their 9th annual spinning marathon on November 17, 2019 to support the PC mission.

Once again, the marathon was a great success and raised 5,500 Swiss francs which was given to the French PC Association “Le Coeur Au Pied.”

The concept was that each person could choose to pedal one hour or more. In all, there were more than 250 people who pedaled for six hours on 80 bikes.

The Kilchoer family plans to organize their 10th spinning marathon on November 8, 2020 with hopes that the coronavirus pandemic will allow this event to still happen.

Thanks to Nathalie and her husband, Phillip, along with their children, Jade (10) and Dylan (14), for their hard work and dedication to the PC cause. They are fabulous examples of PCers who are striving to make a difference.
**GET READY FOR PC AWARENESS MONTH**

June is PC Awareness Month. We hope each of you will join us as we share information about PC with our family, friends and others. Watch for emails in June for ideas on how to participate. Hint: You’re going to need sidewalk chalk!

**VALO TRIAL**

Palvella is happy to announce the VALO Phase 2/3 clinical trial remains on track with anticipated completion this fall. Because of dedicated PC patients, supportive caretakers, excellent clinical trial coordinators, and the commitment of lead principal investigators, the VALO study has continued in spite of the challenges of COVID-19.

In the next few months, several smaller studies will be conducted, which are needed for FDA approval of this investigational therapy. In addition to these smaller studies, the extension trial will also open.

Thank you for all of your efforts!

**IPCRR DATA PRESENTED AT VIRTUAL SID CONFERENCE**

The Society for Investigative Dermatology (SID) Annual Meeting was held virtually this year. Included in the meeting were presentations from two young scientists who used de-identified data from the International PC Research Registry (IPCRR) to study specific symptoms about PC.

Because their findings are not yet published, the presentations are not currently available for public viewing. However, the most important thing for PCers to know is that while joining the registry helps many patients find a name and a mutation for their disease, their information about their PC is also de-identified and used by researchers to learn more about PC and to advance drug development. Thanks to all patients who are part of the IPCRR!

Below are the titles of the presentations with messages from each of the speakers about their experience:

**Novel Distinctions in the Profiles, Impact, and Management of Nail Disease in Pachyonychia Congenita Subtypes**

Presenting: Albert Wu, MS, New York Medical College—Mentor: Sheri Lipner, MD, PhD, Weill Cornell Medical Center

“Thank you for allowing me to record the presentation and share Dr. Lipner’s and my project with the people at the SID. Thank you for giving us the data, inviting us to the conference, for setting up the Webex session and making it such a simple process. It really was a more emotional and educational journey than I would have ever thought.”

“As I continue my studies, I hope to have the chance to keep pursuing research of some form in PC. The PC project is filled with such passionate, kind, and knowledgeable people. I will definitely look out for the PC gatherings in the future!”
Neurovascular Structures on Feet in Pachyonychia Congenita

Presenting: Xiang Li Tan, MBBS, BSc(Hons), Queen Mary University of London
Mentor & Session Chair: Edel O’Toole, MD, PhD, FRCPI, FRCP, Queen Mary University of London

“PC was not taught in medical school so I was fascinated to learn about PC through this project and am very grateful that I had the opportunity to be involved. I hope that one day I can contribute more towards helping PC patients and hope that more treatments will become available for PC! I will always keep the PC Project in my mind when I see patients and I will always be happy to be involved in any way.”

Thanks to Albert Wu (Shari Lipner) and Xiang Li Tan (Edel O’Toole) for expanding the collective PC knowledge, especially at such a prestigious conference.

Graduating Strong with PC

Congratulations to all PCers who are graduating from various schools at this time. PCers are amazing; in spite of their challenges, they find ways to adapt, fight through the pain, accomplish their goals, and live extraordinary lives.

Jennifer Marcinko shared her thoughts as a mother of a son who has PC:

“My son, Patrick, graduated from Virginia Tech today! It’s been a long road and he is one tough soul! Time just flew by! To all the PC moms out there, these PC kids are strong, tough, and resilient! I worried and worried about him. I remember dropping him off at college thinking, “Oh my, did he pick a school that was too big? Would there be too much walking? How will he handle the pain?” Well, he pushed through and made it! PC does not and never will define him! Stay strong PCers! And for the moms that worry and worry like I did, they will make their way!”

Jen also shared that Patrick overcame many obstacles when he went to college: “His feet flared up so badly in the first month that he couldn’t walk. We got him a bike to use around campus. Then, someone stole it outside of the library! There were definitely some crazy hurdles, but he came out strong. Virginia Tech has a disabilities office that offered plenty of support. We put that in place in case we needed it but Patrick did not use it much. I don’t think any of his professors ever knew he had PC. I do want to reach out to Virginia Tech - to thank them for supporting students who need accommodations. I want them to know how impactful their efforts are. I think it is so important to acknowledge those that supported our PC community so more will follow suit.”

Facebook Birthday Fundraiser

A huge thank you and best birthday wishes to PCer Linda Harvey for supporting PC Project in May with a Facebook birthday fundraiser. These fundraisers have no fees because PC is an accredited charity and raise awareness about PC.

Shopping for PC

Don’t forget that many online stores will give a portion of the sale to PC Project when you use the following sites to get to the store.

- igive.com/welcome/lp4/cr39.cfm
- smile.amazon.com/
- smile.amazon.co.uk/
- giveasyoulive.com/join/pccproject-eu

Join PC Project On Social Media

Connect with us and follow us on YouTube, Facebook, Instagram, Twitter & Linkedin.
PC Awareness Month

This past month the global PC community celebrated our annual PC Awareness Month by sharing their PC stories, donating to PC Project, and participating in the first-ever #PCSidewalk Challenge.

If you didn’t have a chance to participate, you can spread PC awareness any time of the year! In the meantime, please enjoy the samples of awareness month in this issue of the news brief.

Campaign Launched by PC Family

Thanks to 3 year old, PCer Wyatt and his mom, Jennifer for launching the campaign and encouraging PC patients everywhere to make art and share their PC stories around the world.

Jennifer wrote: “As mom to Wyatt, a little boy with Pachyonychia Congenita, I have a special relationship with the month of June ... otherwise known as PC Awareness Month.

“...and I’m especially aware when I remember there is still no cure for PC.

That’s why PC Project is so important. It’s the only organization in the world that is 100% focused on helping people with Pachyonychia Congenita and working to find a cure in our lifetime. PC Project helped me learn how to deal with my son’s nails and found the PC mutation for our family. This month, we honor their important work.”
SPECIAL VIDEO FOR PC AWARENESS

Kerry Briggs-Evans, mom of a PCer, created an exceptional video about PC which was shared over 70 times on Facebook!

Click here to watch this her labor of love.

TONY—ENGLAND UK

Tony’s sidewalk was torn up so this PCer made a window board instead of a chalk project. He placed it in his front window so people passing by can see it. Many people have topped to look at it and one person specifically asked about PC. We love his slogan “little steps lead to big things.”

HICKMAN CHILDREN—ENGLAND UK

A group of young PC siblings from England made their PC Awareness art inside while it was raining outside. And we agree their message written on their art project that rain or shine, “The pain of PC on the feet is real.”
**PC Family—Wales UK**

A PC family working on their art for the #PCSidewalkChallenge.

**Greg—California USA**

The following message and photos were posted on Facebook and NextDoor by Greg Van Krugel, who drew this art of his neighbor who has PC:

“This little boy has a presently incurable condition called Pachyonychia Congenita (PC for short). He is one of the most energetic, sweet, happy little kids I’ve ever met. He erroneously calls me “gammpa” because he heard our family call me that. If you look into this boy’s condition a bit, you’ll discover that he has painful hands and feet at times among other problems associated with the condition. Few people worldwide have this condition so little is known or done about it but research IS going on and I support that effort wholeheartedly.

This month is PC Awareness Month. To bring attention to the condition and those who suffer with it the proponents of that research have put out a call for sidewalk art to support their effort. This is my contribution. It comes from our love for this little pal who someday will play HIS harmonica for US.”
**Kaelyn—Illinois USA**

PCer Kaelyn Rittle drew this picture (below) of our PC Project Board of Trustees for the PC Sidewalk Challenge. Our board lovingly volunteers hours and hours of their time and expertise to our cause.

They really are our heroes!

As for Kaelyn, even though she experiences pain every day, this talented person is headed to Purdue this fall to study engineering!

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**Mike—New Jersey USA**

“From the time I was born, my mom and dad never tried to put the brakes on me in terms of walking, running, or playing games. We had a neighborhood full of kids who ran through the streets and parks all day and night. My dad had the same disease all his life. My grandmother took him to see many doctors when he was growing up to see what caused his condition. There were never any answers. Hey, it was the mid to late 1930s. Dad passed away in July of 2001, never knowing what caused his feet to feel so bad.

Up until the age of eleven, my dad would trim my feet. I was ticklish, so his iron grip was necessary, both to keep my foot still and maintain control. So he could use the single-edge blade effectively without slicing anything important off. After that, he allowed me to trim my own feet.

I knew I could never have a career in the military or serve as a fireman or policeman. At eighteen years old, I discovered a beautiful thing. We played street hockey with roller skates. I remember the skates bothering me because there was lots of vibration which impacted the calluses. But our game plan was to try ice skating. I still remember going to the ice rink in Center City, Philadelphia. We rented skates and tried our best for about an hour. We had figure skates that were not particularly comfortable, but the sport was fun, and I wanted to pursue playing the game. I purchased twenty dollar ice hockey skates. All bets were now off. Not only were these skates solid with great support, but they felt so comfortable. I remember skating non-stop at the University of Penn Ice rink for close to two hours. What a rush that was.

In truth, I played competitive ice hockey for 23 years. I was as close to normal as I ever imagined. I could compete with the best players, and we won ten championships in the years that I played.”
You may be asking yourself, how is that possible? Consider this: your foot is locked into a solid skate, having no movement. So your foot doesn’t bend at all. There’s no pressure on the calluses. It’s all distributed through the skate blade, which sits in the center of the skate. There’s no torque on your feet, you just glide on the ice. I could stop quickly and even jump up and down without pain. The skates must be tight to work correctly, and you need to wear a thick sock, but boy did this open up my eyes.

Yes, this disease is a handful, but look at all we need to be thankful for. Our community is moving forward, supported by warriors like PC Project and all the doctors who are searching for an answer. We now have clinical trials and companies that are researching to find medicines that may provide relief and possibly a cure. It’s all good!

Click here to read Mike’s full story.

**Alicia—Minnesota USA**

“The strongest people I know have PC. They don’t let anything hold them back or stop them as they take each painful step with calculation to achieve their goals.

“Here is a picture of my husband, Jack, and daughter, Ella, who both have PC K16. Thank you for all you do for the people I love the most!” - Alicia, wife and mom to two awesome PCers!

**Roxy—California USA**

Dear PC family, I live in Santa Cruz, CA, and just did a podcast about my career as a bicycle designer. While I’m not very vocal about my PC, I want to share this with our community because I know there are a lot of us who struggled with figuring out our future career paths.

As a designer, I have the flexibility to work sitting or standing. Riding bicycles I design, I don’t have to be on my feet, but pedaling is much better for me. Because I mountain bike and have to test what I design, I wear gloves to protect my hands and pretty durable shoes to protect my feet, but this also allows me not to hurt my nails and I can pretty much feel free to play.

I’m very, very blessed to live where I do and work as a creative. When I was younger, I didn’t know about “industrial design” as a potential career, but it’s creative and analytical at the same time. I have mild PC symptoms, but have struggled with it all my life and I know I strayed away from some career paths that I would have loved to do just because they might draw attention to my PC in a negative way.

I know there are a generation of young PC'ers who might learn about a job they might not have known existed or what we have done happily and successfully.

With love, Roxy

Click here to see more pictures and read more about Roxy’s very interesting career.
**Barbara—Maryland USA**

“In honor of PC Awareness Month, I am sharing this ‘recreation’ of the PC Project Logo created in sand by our 7 year old spontaneous PC-K17 daughter and her 9 year old sister. In their minds, P.C. stands for ‘People Care.’ Let’s prove them right.” - Barbara, PC mom

**Father’s Day PC Tribute**

During PC Awareness Month, we paid tribute to the dads with PC and dads with PC children.

This father of two kids with PC learned to help feed both of his boys who struggled with eating as infants, one of the challenges some of our young PC patients experience.
Christine—Wisconsin USA

This mom of a PCer wrote, “Allison cooling off her PC feet at a waterfall during a little break during a mountain bike ride.”

Lots of PCers enjoy cooling off their hot and hurtful feet.

Kelsie—Utah USA

This PC supporter drew the first submission for the #PCSidewalkChallenge

You Bring Hope

Hope, the word that embodies not only PC Awareness Month, but everything we do at PC Project. All the love and effort we put forth is based on that one powerful word - hope. This hope is what unites us all across the world.
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/
**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
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

**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 Steimmeyer, 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.

**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 magazine for 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.