

SERVICES SPONSORED BY PC PROJECT

Successful Research Programs

PC Project has established an active partnership with TransDerm, Inc., a biotech firm in Santa Cruz, CA which specializes in developing skin therapeutics and patient-friendly skin delivery technologies.

PC Project has supported two successful **FDA-approved Phase 1b Clinical Trials** with TransDerm, Inc. The first, for TD101, an siRNA drug proved siRNA is an effective means of ‘turning off’ a specific mutant gene. The second, for TD201, proved safety in administration of topical sirolimus (rapamycin).

More than 15 other small studies have been completed using drugs identified through drug screening grants.

Program Services

The **International PC Research Registry** (IPCRR) which provides a detailed patient data collection, with images and physician notes and **free Genetic Testing**.

The **International PC Consortium** (IPCC) and annual research symposiums for both scientists and physicians focuses on educational outreach and collaborative research.

An active **Research Grant** program to fund research on specific gene inhibitors, small molecule drug screening, microneedle development, and other innovative research to meet our mission goals.

The website at www.pachyonychia.org provides extensive and detailed information and support for patients, researchers and clinicians.

Annual **Patient Support Meetings** where PC patients meet others with PC as well as consult with experts from the medical and scientific fields.



Donations Welcome!

Your donation dollars really multiply, accomplish a great deal and make a real difference at PC Project. Thank you for making our success possible.



Our Mission

Fighting for a cure.
Connecting and helping patients.
Empowering research.

Pachyonychia Congenita Project

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PACHYONYCHIA CONGENITA (PC)
is a very rare and painful skin disorder.

Those with PC have:

- **Painful blisters and calluses on their feet and sometimes on their hands**
- **Thickened fingernails and toenails** and may also have:
- **Cysts and bumps on their skin**
- **A white tongue and mouth**

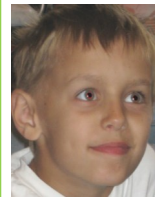
HOW TEACHERS, FRIENDS AND OTHERS CAN HELP

- ♥ Make it comfortable to talk about PC and reassure classmates, parents, fellow workers and others that PC is not contagious.
- ♥ Encourage those with PC to do everything they can - but *listen* when they say they are in pain. PC pain varies and sometimes a person can run and run and another time, they may not be able even to stand up. The person with PC knows best how to handle their own PC pain and PC kids are tough! Let them guide you to what they can/cannot do on any given day.
- ♥ PC hurts. Teasing and name-calling are also very painful. Help by being aware of unkind actions towards those with PC. Take action to correct the problem if someone with PC is being teased, picked on or attacked.



"...if you have PC you get blisters all over the bottom of your feet (and sometimes on your hands). The blisters hurt a lot. It is kind of difficult to explain how hard it is to live with PC. You have to find a way to survive and live with pain basically.

It's sad to say that this disorder sometimes pushes you away from people you love."



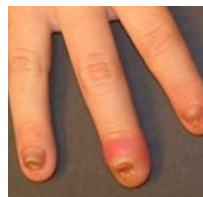
There is currently no effective treatment for PC.



A person with PC cannot take a drug or medicine, or use a lotion or cream to change the PC gene and stop their PC pain.



Blisters and calluses on PC feet are really painful. Cold sometimes helps the pain and patients may wear sandals — even in winter. Others may want to keep their feet covered — even in the summer.



The thickened nails usually are not painful unless they are bumped, bruised or become infected.

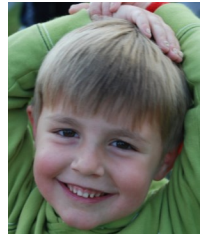


Extra keratin also may create bumps around each hair follicle or may sometimes cause cysts. The bumps and cysts are often very uncomfortable but are not anything someone can catch.

A white film on the tongue is also just extra keratin. This feature is not usually painful, although sucking as an infant may be difficult.



PC IS NOT CONTAGIOUS. PC is not caused by germs or a fungus. It is a genetic disorder caused by a change in just one keratin gene.



Genetic messages tell cells what to do to keep our body working well. Every person has from 25,000 to 40,000 genes and everyone is born with some genetic variations.

The one small mistake in the PC genetic message code means the 'filaments' needed for skin and nails do not join together properly, causing the skin to blister and thicken.



Boys and girls, men and women with PC live in every country in the world. Because PC is so rare, they usually will never meet anyone with PC unless they attend a PC Patient Support Meeting.



In spite of all the challenges and pain, most people with PC are very high achievers.

"I'm eight years old and I'm in 2nd grade. I like the color blue. I love to play chess and checkers. I like to do mazes. Sometimes people run away and think I'm gross because I have PC. I feel sad and lonely - I just get this feeling that I'm alone in the world. When this happens, I just go and find something to do, and I've got this friend I've had since Kindergarten and I just go play with him if I feel really sad and we go have fun."

