

## SERVICES SPONSORED BY PC PROJECT

### Successful Research Programs

PC Project funded and sponsored a successful **FDA-approved Phase 1b Clinical Trial** for TD101, an siRNA drug. The trial proved siRNA is an effective means of ‘turning off’ a specific mutant gene.

PC Project has established an active partnership with TransDerm, Inc., a ciotech firm in Santa Cruz, CA which specializes in developing skin therapeutics and patient-friendly skin delivery technologies. A Phase 2 SBIR NIH grant has been awarded to TransDerm to further develop siRNA and develop micro needles for delivery of siRNA leading to a second clinical trial.

Several other small studies have been completed and preparations are underway for extended multi-center studies using drugs identified through drug screening grants.

### Program Services

The **International PC Research Registry (IPCRR)** which provides detailed patient data collection as well as physician consultations and **free Genetic Testing**.

The **International PC Consortium (IPCC)** and annual research symposiums for both scientists and physicians focused 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.

The website at [www.pachyonychia.org](http://www.pachyonychia.org) which provides 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 Needed for Research and Support!

For Awareness Day Events, every \$1 donated will be matched **\$2-for-\$1** and 100% of donated funds go to research and program services. All overhead costs are paid from the sponsors’ portion. Your donation dollars really multiply and make a difference at PC Project. Thank you for making our success possible.



## *Mission Statement* Find a Cure for Pachyonychia Congenita

**1000 PATIENTS**  
**50 COUNTRIES**  
**NO TREATMENTS**

### Pachyonychia Congenita Project

2386 East Heritage Way, Suite B  
Salt Lake City, UT 84109 USA  
Phone +1-877-628-7300  
Fax +1-877-628-7399  
Email — [Info@pachyonychia.org](mailto:Info@pachyonychia.org)

WEBSITE  
[www.pachyonychia.org](http://www.pachyonychia.org)

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Aug 2011



## Pachyonychia Congenita Project



**ALL DONATIONS  
FROM THIS EVENT  
WILL BE MATCHED  
\$2-FOR-\$1**

**Pachonychia 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 sometimes, they also have:
- **Cysts and bumps on their skin**

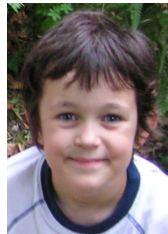


*"...if you have PC you get blisters all over the bottom of your feet (and sometimes on your hands). The blisters hurt a lot. It is kind of difficult to explain how hard it is to live with PC. You have to find a way to survive and live with pain basically. It's sad to say that this disorder sometimes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

*"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."*



### 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.



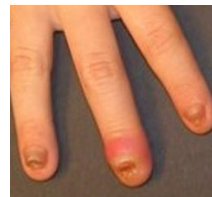
Encourage those with PC to do everything they can - but *listen* when they say they are in pain. PC pain varies and sometimes a person can run and run and another time, they may not be able even to stand up. Each person knows best how to handle their PC pain and PC kids are tough!



PC hurts - but 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.



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

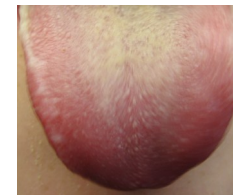


The thickened nails usually are not painful unless they are bumped, bruised, or become infected.



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.

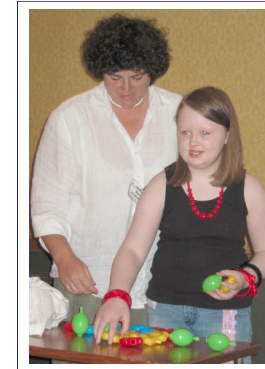
A white film on the tongue is also just extra keratin. This feature is not usually painful, although sucking as an infant may be difficult.



**PC IS NOT CONTAGIOUS.** PC is not caused by germs or a fungus, but 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.

*Dr. Robyn Hickerson uses toy connectors to demonstrate DNA and "filaments" with PC kids. (Patient Support Meeting, Dundee, Scotland, 2006)*

Men, women, boys and girls with PC live in every country in the world.



Because PC is so rare, they usually will never meet anyone else with PC unless they attend a PC Patient Support Meeting.



Even with all the challenges, most people with PC are very high achievers.

