DISCLOSURE OF RELEVANT RELATIONSHIPS
Pachyonychia Congenita (PC) is a rare genetic skin disorder. There is no effective treatment.
<table>
<thead>
<tr>
<th></th>
<th>Pachyonychia Congenita (PC) is a ...</th>
</tr>
</thead>
<tbody>
<tr>
<td>Rare</td>
<td>ULTRA rare</td>
</tr>
<tr>
<td>(Orphan)</td>
<td>est 2,000-10,000</td>
</tr>
<tr>
<td>Genetic</td>
<td>autosomal dominant</td>
</tr>
<tr>
<td>Keratin</td>
<td>K6a/K16 or K6b/K17</td>
</tr>
<tr>
<td>Skin</td>
<td>= dermatologists!</td>
</tr>
</tbody>
</table>
Major Clinical Findings of PC

Thick (Pachy) Nails (onychia)

K17  K6a  K16

K6a  K16

Pachyonychia Congenita Project
Major Clinical Findings of PC

Blisters and Calluses

PC = Painful Calluses

Pachyonychia Congenita Project
Major Clinical Findings of PC

Cysts

K16

K6a

K17

Pachyonychia Congenita Project
Major Clinical Findings of PC

Follicular Hyperkeratoses
Major Clinical Findings of PC

Leukokeratosis

Pachyonychia Congenita Project
On-Line Bibliography

Full-text on-line bibliography

534 articles; 80 foreign language translations
Mission - ‘find a cure for PC’

Sancy Leachman, MD, PhD
University of Utah
Pachyonychia Congenita Project
International PC Consortium (IPCC)

Feb 2004 – Park City, Utah
May 2005 – Kansas City with SID
May 2006 – Philadelphia with SID
   (with a grant from NIH/NIAMS & ORD)
May 9, 2007 – Los Angeles with SID

If you are interested, you are welcome.
Contact us for further information.
Mission - ‘find a cure for PC’

- International PC Consortium (IPCC)
- Patient Registry (IRB approved) (IPCRR)
IRB-approved Patient Registry

IPCRR STATISTICS - 31 Jan 2007

389 PCers in 27 countries

133 questionnaires

125 consultations

Pachyonychia Congenita Project
Genotype results for 116 patients in 56 Families with 33 unique mutations

Pachyonychia Congenita Project
Find A Cure: Where Are We?

Drug development takes a community

Government

Research Scientists

Patients

Industry

Patient Advocates

Basic & Translational Research

Physicians

Pachyonychia Congenita Project
TransDerm is dedicated to developing therapeutics for skin disorders. In partnership with the International PC Consortium (IPCC) and PC Project, we are investigating different avenues for helping patients suffering from the very rare skin disorder, pachyonychia congenita (PC).
Find A Cure: Where Are We?

siRNA developed by TransDerm

Normal Cell
(WT K6a)
Find A Cure: Where Are We?

siRNA developed by TransDerm


...10 to 12 years at a cost of $10 – 12 million/billion

Pachyonychia Congenita Project
Find A Cure: Where Are We?

siRNA developed by TransDerm

✓ Orphan Drug Status
✓ Preclinical Animal Tox
✓ Pre-IND Request filed for
  Phase 1 Clinical Trials
So, Grandma, have you found a cure?

So, Grandma, have you made any progress?
Find A Cure: Where Are We?

PC Studies & Clinical Trials

- Moving to clinical trial on our mutation specific siRNA through partnership with TransDerm, Inc.

In addition, these other studies are underway

- One off-label study completed
- One off-label study currently in progress
- Proposed Botox multi-center clinical trial
Find A Cure: Where Are We?

We are moving forward... thanks to

PC Patients around the world

Sancy Leachman, MD, PhD
University of Utah

Roger Kaspar, PhD
CEO, TransDerm, Inc.

Members of the IPCC and MSAB members

And scientists and physicians like you! THANK YOU.

Pachyonychia Congenita Project