

Tuesday, May 2

8:00 Welcome Breakfast

8:50 **Mary Schwartz**, Director, PC Project
Welcome and PC Project Update

9:00 **Sancy A. Leachman, MD, PhD**
Introduction, Overview and Introduction of the Keynote Speaker
Chair, IPCC; Medical Director, PC Project; Associate Professor,
Dermatology, University of Utah

9:20 **Frances J.D. Smith, PhD** *Keynote Address*
PC Career Development Research Fellow
Human Genetics Unit, University of Dundee
9:45 Discussion

SECTION 1: PC RESEARCH PROGRESS

9:50 **Philip Fleckman, MD** *Moderator*
Professor, Dermatology, University of Washington

9:55 **Leonard M. Milstone, MD** *Sequence-Specific Gene Targeting*
Professor, Dermatology, Yale University School of Medicine
10:15 Discussion

BREAK

10:35 **W.H. Irwin McLean, PhD, FRSE**
Small Molecule Inhibitor Screening
Professor, Human Genetics Unit, University of Dundee
10:55 Discussion

11:00 **Dennis R. Roop, PhD** *A PC Mouse for Pre-Clinical Trials*
Professor, Molecular and Cellular Biology/Dermatology,
Baylor College of Medicine
11:20 Discussion
(Dennis Roop, PhD and Jiang Chen, MD)

11:25 **Robyn P. Hickerson, PhD**
Identifying Effective siRNA Inhibitors for PC
Senior Scientist, TransDerm, Inc.
11:45 Discussion

11:50 **Sancy A. Leachman, MD, PhD** *Summary Discussion*

WORKING LUNCH (discussion continues)

Section 1 (continued)

1:00 Moderator: Philip Fleckman, MD

1:05 **Sancy A, Leachman, MD, PhD**
Biological Endpoints for PC Clinical Trials
1:25 Discussion

1:30 **Mark J. Eliason, MD**
Development of a PC Severity Index Score
Post-Doctoral Research Fellow, Dermatology, University of Utah
1:50 Discussion

SECTION 2: FOCUS ON DELIVERY

1:55 **Roger L. Kaspar, PhD** *Moderator*
Founder and CEO, TransDerm, Inc.

2:00 **Devin Leake, PhD**
to be announced
Dharmacon, Inc.
2:20 Discussion

BREAK

2:40 **Bozena B. Michniak-Kohn, PhD**
Challenges and Approaches to Topical Drug Delivery
Assoc Prof, Ernest Mario School of Pharmacy, State Univ of NJ-Rutgers
Director, Drug Delivery Laboratory, NJ Center for Biomaterials
3:00 Discussion

3:05 **Jonathan B. Rothbard, PhD**
Transdermal Drug Delivery using Oligoarginine
Senior Research Scientist, Stanford University
3:25 Discussion

3:30 **PANEL DISCUSSION** *Choosing Delivery Strategies for PC Therapies*
Devin Leake, Bozena B. Michniak-Kohn, PhD; Jonathan B. Rothbard,
PhD

6:00 **SPECIAL EVENT AND DINNER**
Above and Beyond: A Rare Experience
A special Philadelphia Trolley will leave promptly at 6:00pm
from the Loews Hotel Lobby. Please don't miss it!
Sancy cell phone - 801-301-1423
Mary cell phone - 801-953-7553

WEDNESDAY MAY 3

8:00 Breakfast

9:00 Sancy A. Leachman, MD, PhD *Introduction and Overview*

SECTION 3: OVERCOMING OBSTACLES

9:10 Sherri J. Bale, PhD *Moderator*
Founder and CEO, GeneDx, Inc

9:15 Ervin H. Epstein, MD
Solving the Riddle: Obstacles to Translation Conference
Research Dermatologist; Clinical Professor of Dermatology, UCSF
9:35 Discussion

9:40 Stephen C. Groft, PharmD
Successful Advocacy for Rare Disorders
Office of Rare Diseases Research, OD, NIH
10:00 Discussion

10:05 To be announced
NIH/NIAMS
10:25 Discussion

BREAK

10:45 Peter L. Vaccari, RPh, RAC
Functions of the FDA's OOPD
Reviewing Pharmacist, Office of Orphan Products Development/FDA
11:05 Discussion

11:10 Roger L. Kaspar, PhD
Up, Up and Away: Navigating Rarefied Air
11:30 Discussion

11:35 PANEL DISCUSSION *Reaching Success in PC Therapy*
Ervin H. Epstein, MD; Stephen C. Groft, PharmD; Roger L. Kaspar, PhD; Peter L. Vaccari, RPh, RAC; NIAMS/NIH representative

WORKING LUNCH (discussion continues)

SECTION 4: PROMISING RESEARCH

1:00 Leonard M. Milstone, MD *Moderator*

1:05 Brian J. Nickoloff, MD, PhD
A Transdifferentiation Viewpoint for Psoriasis
Director, Oncology Institute; Director, Skin Disease Research Program
Loyola University of Chicago Medical Center
1:25 Discussion

1:30 Reinhard Windoffer, MD
In vivo Cell Imaging
Dept of Anatomy, Johannes Gutenberg-University Mainz
1:50 Discussion

BREAK

2:10 Irene Leigh, MD DSc FRCP
Focal Keratodermas
Lead, Centre for Cutaneous Research
Director of the Cancer Research UK Skin Tumour Laboratory
2:25 Discussion

2:30 Elizabeth L. Rugg, PhD *Promoting Protein Folding:
A Pharmacological Strategy for Treating Keratin Disorders*
Associate Researcher, Dermatology, University of California, Irvine
2:45 Discussion

2:50 Xuejun Zhang, MD, PhD
to be announced
Professor of Dermatology and Venereology
President of Anhui Medical University
3:05 Discussion

3:10 Summary Discussion *IPCC Vision for Success*
Sancy A. Leachman, MD, PhD

7:00 AWARDS BANQUET - *Celebrating the Vision*
Upstares at Varalli
231 South Broad Street
Philadelphia, PA 19103
215-546-6800