

## 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**  
*Exploiting siRNA technology--  
enhancing delivery, increasing stability and minimizing off-target effects*  
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 for the Philadelphia Zoo where we will enjoy a Hot Air Balloon Ride to 'catch the vision' and a catered dinner in the rare animal exhibit hall.**

## 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** Carl Baker, MD, PhD  
*NIH/NIAMS: Funding Research for Rare Disorders*  
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