

# NCATS Research & Development Day

Novartis Institutes for Biomedical Research • Cambridge, MA September 12, 2013

## AGENDA

- 9:00 A.M. Welcome Address**  
*Joan Mannick* — Director, New Indications Discovery Unit, Translational Medicine, Novartis Institute of Biomedical Research
- 9:10 A.M. National Center for Advancing Translational Sciences (NCATS) Introduction**  
*Christopher Austin* — Director, NCATS
- 9:30 A.M. Division of Preclinical Innovation (DPI) Introduction**  
*John McKew* — Acting Scientific Director, DPI, NCATS
- 10:00 A.M. [Aes-103 for the Treatment of Sickle Cell Disease](#)**  
*Stephen Seiler* — Founder and CEO, AesRx
- 10:20 A.M. [PB-01 for the Treatment of Severe Sickle Cell Disease](#)**  
*Susan Perrine* — Phoenicia BioSciences, Inc
- 10:40 A.M. BREAK**
- 11:00 A.M. [DEX-M74 for the Treatment of Hereditary Inclusion Body Myopathy](#)**  
*Selwyn Yorke* — Business Development Manager, New Zealand Pharmaceuticals
- 11:20 A.M. [BMP Inhibitors for the Treatment of Fibrodysplasia Ossificans Progressiva](#)**  
*Kenneth Bloch* — Professor, Harvard Medical School
- 11:40 A.M. [VBP15 for the Treatment of Duchenne Muscular Dystrophy](#)**  
*Edward Connor* — President and CEO, ReveraGen Biopharma
- 12:00 NOON [LUNCH ON YOUR OWN](#)**
- 1:25 P.M. “Public-Private Partnerships” – Collaborating with NCATS**  
[Lili Portilla](#) — Director, Office of Strategic Alliances, NCATS  
[Bruce Trapnell](#) — Professor, University of Cincinnati
- 2:25 P.M. [Auranofin for the Treatment of Chronic Lymphocytic Leukemia](#)**  
*G. Sitta Sittampalam* — Senior Scientific Officer, NCATS
- 2:45 P.M. [Cyclodextrin for the Treatment of Niemann-Pick Type C1 Disease](#)**  
*Elizabeth Ottinger* — Senior Project Manager, NCATS
- 3:05 P.M. BREAK**
- 3:20 P.M. [Cyclocreatine for the Treatment of Creatine Transporter Deficiency](#)**  
*Rick Hawkins* — President and CEO, Lumos Pharma
- 3:40 P.M. [Long-acting Parathyroid Hormone for the Treatment of Hypoparathyroidism](#)**  
*Michael Mannstadt* — Assistant Professor, Harvard Medical School
- 4:00 P.M. [VT-1129 for the Treatment of Cryptococcal Meningitis](#)**  
*William Moore* — CSO, Viamet Pharmaceuticals
- 4:20 P.M. Closing Remarks**  
*Christopher Austin* — Director, NCATS
- 4:30 P.M. NETWORKING RECEPTION**  
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