

Inaugural Sanford Children's Health Research Center Scientific Symposium

Children's Diseases: Models and Therapy

Saturday, April 29th, 2023

Host: Hudson Freeze

Organizer: Sinead, sking@sbpdiscovery.org



Saturday, April 29th

Building 12 Auditorium

8:30- 9:00 am

Registration and Continental Breakfast

9:00-9:15 am

Introduction and Welcome Dr. Hudson Freeze, Dr. David Brenner, and Denny Sanford

Morning Session: Human Genetics and Sanford Children's Research Center Faculty
Chaired by David A. Brenner, M.D.
President and CEO, Sanford Burnham Prebys

9:15-9:40 am

José Luis Millán, Ph.D.

"Developing therapeutics for soft bones and ectopic calcification disorders"

9:40- 10:05 am

Duc Dong, Ph.D.

"From hope for few to drug for many – Why rare is precious"

10:05-10:30 am

Evan Snyder, M.D. Ph.D.

"A clinical trial using human neural stem cells for neuroprotection in perinatal asphyxia, a major cause of cerebral palsy in kids"

10:30-10:45 am

Coffee Break

10:45- 11:10 am

Anne Bang, Ph.D.

"Drug screens of human induced pluripotent stem cell (hiPSC) derived neuronal networks on multi-electrode arrays"

11:10- 11:35 am

Pamela Itkin-Ansari, Ph.D.

"Proinsulin misfolding in diabetes"

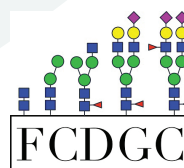
11:35-12:00 pm

Yu Yamaguchi, M.D. Ph.D.

"Multiple hereditary exostoses – From genetics to potential drug targets"

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- 12:00-12:25 pm **Hudson Freeze, Ph.D. & Joe Gleeson, M.D. Ph.D.**
"A complex coagulopathy: cause, consequences and treatment"
- 12:30-1:30 pm **Lunch**
- Afternoon Session:** Chaired by Hudson Freeze, Ph.D.
Professor and Director, Sanford Children's Health Research Center
- 1:30-1:55 pm **David Pearce, Ph.D.**
"From rare diseases to type-1 diabetes: Research that impacts children at Sanford Health"
- 1:55-2:20 pm **Eva Morava, M.D., Ph.D.**
"Clinical trials in Glyco-land"
- 2:20-2:45 pm **Ethan Perlstein, Ph.D.**
"Precision drug repurposing: patient avatar to pioneer study to Phase 3 trial"
- 2:45-3:10 pm **Lars Bode, Ph.D.**
"Human milk-based therapeutics and diagnostics to protect preterm babies from necrotizing enterocolitis"
- 3:10-3:35 pm **Stephanie Cherqui, Ph.D.**
"Hematopoietic stem cell gene therapy for cystinosis: Mechanism of action and clinical trial update"
- 3:35-3:45 pm **Break**
- 3:45-4:15 pm **Round Table Discussion: Opportunities**
Moderator: David A. Brenner, M.D.
Evan Snyder, M.D. Ph.D., Eva Morava M.D. Ph.D., Charlotte Hobbs, M.D. Ph.D., David Pearce, Ph.D.
- 4:15-6:00 pm **RECEPTION AND FAMILY RECOGNITIONS:**
Denny Sanford and Sanford Children's Health Research Center Faculty

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Directions to **Building 12**
Sanford Children's Health Research Center
(from I-5 North or South)

Building 12 is 1/3 mile east of the main Sanford Burnham Prebys campus.

1. Take the Genesee exit and head west.
2. Bear right onto N. Torrey Pines Road and head north.
3. Turn right onto Science Park Road.
4. Turn left onto Torreyana Road and then take an immediate right onto Road to the Cure.
5. Our parking lot is the first driveway on the left. Overflow parking is available in the dirt lot on the right.



Building 12

10905 Road to the Cure
San Diego, CA 92121
(858) 646-3100
sbpdiscovery.org

