BioMed@TAU Research Hub Rare Diseases

A New Age for Treating Rare Diseases: Combining Genetics and Clinical Care at the Bench and Back to the Patient

Sunday, Dec 2, 2018 | 09:00-15:00
Sackler School of Medicine, room 100

9:00 Refreshments

9:30 Opening/greetings
Miguel Weil, Faculty of Life Sciences, TAU

9:35 Annick Raas-Rothschild, Sheba Medical Center & Sackler Faculty of Medicine, TAU
Patients with Rare Diseases: The Challenge

10:00 Orna Elroy Stein, Faculty of Life Sciences, Tel Aviv University
From Mutation to Drug: the Journey of eIF2B Leukodystrophy

10:25 Stephan Sanders, UCSF, USA
Challenges and Opportunities in Rare Genetic Disease Research

11:05 Tali Garin-Shkolnik, TiCure, Israel
GRIN2B Gene - GRIN to be Cured

11:30 Moran Rubinstein, Sackler Faculty of Medicine, TAU
Dravet Syndrome - New Early Diagnostic Tools and Treatment Options

12:00-12:45 Lunch

12:45 Daniel Aberdam, Inserm, France
Ectodermal Dysplasia Syndrome: from Patient iPSC to Drug Repurposing and Successful Patient Treatment

13:15 Aviva Fattal-Valvski, Sourasky Medical Center & Sackler Faculty of Medicine, TAU
The Role of Genetics in Pediatric Neurology

13:40 Yael Weiss, Ultragenyx, CA
Developing Therapies for Rare Genetic Diseases: Not a Rare Success

14:20-15:00 Open Discussion/Panel of Speakers, Moderator: Karen Avraham, Sackler Faculty of Medicine, TAU
What Will it Take to Make the Leap Needed?

Closing remarks

For questions contact mailto:alanarhasan@tauex.tau.ac.il
RSVP here
Organized by Karen B. Avraham, Miguel Weil, and Annick Raas-Rothschild