TEL AVIV UNIVERSITY

BioMed@TAU

BLAVATNIK CENTER for Drug Discovery
מרכז בלווטניק לפיתוח תרופות

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