



## BioMed@TAU Rare Diseases Hub

## Rare Diseases: From patients to bench and back

**Tuesday May 29, 2018 | 9:00-14:30** 

Tel Aviv University, Sackler School of Medicine, room 927

9:00- 9:30 Coffee

9:30-9:40 Introduction

**Annick Raas-Rothschild**, Institute of Rare Diseases, Institute of Genetics, Sheba Medical Center, Tel Hashomer/ Department of Pediatrics, Sackler School of Medicine, TAU

9:40-10:25 **Keynote speaker** 

Pathophysiology and Therapeutic Developments in Progeroid Syndromes

**Nicolas Levy**, Département de Génétique Médicale et Biologie Cellulaire, Hôpital d'Enfants La Timone; Inserm UMR\_S 1251, Marseille Medical Genetics Center, Faculty of Medicine, Marseille, France

- 10:25-10:55 Mutation-Specific Computational Drug Recovery Initiative for Cystic Fibrosis Patients

  Ori Inbar, Director, Cystic Fibrosis Foundation of Israel
- 10:55-11:25 Artificial Intelligence to Aid the Medical Geneticist

**Ben Pode Shakked**, Institute of Rare Diseases, Institute of Genetics, Sheba Medical Center, Tel Hashomer/ Department of Pediatrics, Sackler School of Medicine, TAU

- 11:25-11:45 Teacher for Life- From a Personal Story to an Educational Philosophy Claudia Koby, Founder, "Krembo Wings"
- 11:45-12:10 The Usherhub

Vered Manny-Aframian, Usherhub Scientific Manager

- 12:10-12:45 Light lunch (provided)
- 12:45-13:15 Repurposing of Existing Drugs- A New Therapeutic Door Opens, Advantages and Challenges **Ronen Shemesh**, Lephtin Bio and Fertiprot Ltd.
- 13:15-13:45 ADA-SCID Deficiency- A Novel Platform of Drug Discovery for Metabolic Disorders **Dana Laor**, Gazit Laboratory, Faculty of Life Sciences, TAU
- 13:45-14:10 Fighting Neurodegeneration Starts from Your Skin: Lessons for Familial Dysautonomia **Miguel Weil**, Laboratory for Neurodegenerative Diseases and Personalized Medicine, Faculty of Life Sciences, TAU
- 14:10-14:30 The Future of Rare Diseases and Therapeutic Options

Yael Gruenbaum-Cohen, MEDISON Pharma

Organized by Prof. Annick Raas-Rothschild and Prof. Miguel Weil Pre-registration is requested <u>HERE</u> | For more information, contact <u>alanarhassan@tauex.tau.ac.il</u>