



Workshop Rare Diseases, Tel Aviv, June 1st, 2016

Italy-Israel Day

Rare diseases: state of the art for a bilateral alliance

Venue: Renaissance Tel Aviv Hotel

Agenda

8.30 -9.00

Registration & refreshments

Opening remarks

9.00- 10.40

Session I: Public and private players, the frontline of research

The role of public and private players, a thriving business model for patients and investors: why pharmaceutical companies are entering in this space? Opening for an Italian-Israeli bilateral collaboration

Dr. Paolo Barbanti, (CEO, *Pharma & Biotech Advisors srl, Milano*)

Targeted (epi)genome editing for therapeutic applications

Prof. Angelo Lombardo (*Group Leader San Raffaele Telethon Institute for Gene Therapy, SR-Tiget, Unit of Epigenetic regulation and targeted genome editing, Milano*)

Using big data for personalized medicine

Prof. Noam Shomron (*Director of Rare Genomics Institute Israel, Sackler Faculty of Medicine, Tel Aviv University, Tel Aviv*)

New approach in the road towards drug personalized medicine of rare diseases

Prof. Miguel Weil (*Cell Screening Facility for Personalized Medicine, Laboratory for Neurodegenerative Diseases and Personalized Medicine, The George S. Wise Faculty of Life Sciences, Tel Aviv University, Tel Aviv*)

10.40-11.00

Coffee break

11.00-13.00

Session II: From bench to bedside

When a rare disease meets a prevalent disease: the association between Gaucher disease and Parkinson disease

Prof. Mia Horowitz (*Dept. of Cell Research and Immunology, Sagol School of Neurosciences, The George S. Wise Faculty of Life Sciences, Tel Aviv University*)

Mast cells from allergy to mastocytosis: can we use the same cellular targets for therapy?

Prof. Francesca Levi Schaffer (*Chair Immunopharmacology, Pharmacology and Experimental Pharmacology Unit, School of Pharmacy, Institute for Drug Research, Faculty of Medicine, The Hebrew University of Jerusalem, Ein Kerem Campus*)

Rare diseases: team therapy

Vivi (Reviva) Einy MsC (*Genetic Counselor, Project Manager Orphanet Israel, Institute for Rare Diseases, Sheba Medical Center, Tel Hashomer*)

Gaucher disease and Parkinson: from a rare inherited disease to drug development for common neurodegenerative disorders

Prof. Ari Zimran (*Associate Professor of Medicine, the Hebrew University of Jerusalem, Shaare Zedek Medical Center, Jerusalem*)

Q & A

13.00-14.00

Lunch

14.00-15.30

Session III: The role of Companies

Teva, at the heart of pharmaceutical innovation

Dr. Ralph Laufer (*Senior Vice President, Head of Discovery and Product Development, Teva, Petach Tikva*)

Mechanism of action of placenta-derived PLX-R18 stromal cells in mitigating radiation induced hematological deficiencies

Racheli Ofir, PhD (*VP Research and IP, Pluristem Therapeutics, Haifa*)

Metabomed: Targeting orphan cancer pathologies by a synthetic lethality approach

Dr. Simone Botti (*CEO, Metabomed, Yavne*)

15.30-15.50

Coffee break

15.50-16.30

Session IV: Investing in Rare Diseases

Title: tbc

Merck Serono Incubator, (Yavne)

Title: tbc

Dr. Nissim Darvisch (*Senior Managing Director, Orbimed, Herzliya*)

Q & A

16.30

Conclusions

An agenda for a fruitful Italy-Israel win win collaboration, the next step, the way forward

Dr. Paolo Barbanti (*CEO, Pharma & Biotech Advisors srl, Milano*)

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