

OPMD – Emerging Opportunities for Research and Cure of STR-opathies

Tuesday, May 16, 2023 • Beautiful Israel Complex, Rokach Ave. Tel Aviv, Israel

We are honored to invite you to this unique conference in which the scientific part will deal with Oculo-Pharyngeal Muscular Dystrophy (OPMD) as a model for the development of potential treatments and cure for degenerative diseases that stem from Short Tandem Repeats mutations (STRopathies) and in the second part in the afternoon we will provide the relevant information for OPMD patients and their families.

The scientific part of the conference is intended both for clinical researchers from fields that treat diverse manifestations of degenerative diseases and for researchers whose field of activity is related to the search for a cure for rare genetic diseases.

The conference will be an important landmark by providing an opportunity to create research collaborations between clinicians and basic science researchers for development of treatments for OPMD and other STRopathies.

We hope and expect interesting conference and subsequent collaboration.

Alex Zvulunov and Amir Dori, Co-chairs.



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Prof. Zohar Argov MD

Dr Argov is Professor (Emeritus) of Neurology at the Hadassah-Hebrew University School of Medicine in Jerusalem, Israel and an Adjunct Professor at the Department of Neurology/Neurosurgery at the Montreal Neurological Institute, Montreal, Canada. He also serves as a Member of the Neuromuscular Panel at the European Academy of Neurology and member of the Editorial Board of Neuromuscular Disorders.

Prof Argov graduated from the Hebrew University- Hadassah Medical School and finished his training in Neurology at the same Institute. He had two fellowships: A WHO Neuromuscular Fellowship at the Muscular Dystrophy Laboratory in Newcastle upon Tyne (UK) and MDA Fellowship in Biochemistry and Biophysics Laboratory of the University of Pennsylvania, Philadelphia (USA).

Prof Argov was a Chief Medical Officer and later a Special Medical Advisor to the CEO of BioBlast Pharma and an Ad Hoc consultant of Ultragenyx Pharmaceutical. Today he is an ad hoc consultant for Seelos Therapeutics.

His main research and academic fields of interests are: GNE myopathy, clusters of hereditary neuromuscular disorders, iatrogenic neuromuscular disorders and metabolic myopathies.



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Dr. Jerel Banks

Jerel Banks, MD, PhD, received a doctorate from Brown University, an undergraduate degree from Princeton University and a doctorate from The Warren Alpert Medical School.

Presently, Jerel A. Banks occupies the position of Chairman & Chief Executive Officer for Benitec Biopharma, Inc., Executive Chairman & Chief Executive Officer of Benitec Biopharma Pty Ltd. and Chief Executive & Financial Officer & Secretary at Tacere Therapeutics, Inc. (both are subsidiaries of Benitec Biopharma, Inc.) and Chief Investment Officer at Nant Capital LLC. Jerel A. Banks is also on the board of Genos Research, Inc. and Aardvark Therapeutics, Inc.

Benitec is a biotechnology company developing a proprietary therapeutic technology platform that combines RNA interference with gene therapy for the goal of providing sustained, long-lasting silencing of disease-causing genes from a single administration.

Benitec is using the technology, called DNA-directed RNA interference, to develop product candidates in chronic and life-threatening human disease areas including: Orphan disease: Oculo-Pharyngeal Muscular Dystrophy (OPMD) and Infectious disease: Hepatitis B Virus (HBV).



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Prof. Sergiu Blumen, MD

Dr. Blumen is Associate Clinical Professor (Emeritus) at the Rappaport Faculty of Medicine at The Technion, Haifa and senior neurologist at The Hillel Yaffe Medical Center, Hadera, Israel.

Prof. Blumen graduated from the Hebrew University – Hadassah Medical School and finished his training in Neurology at the Sourasky Tel Aviv Medical Center.

Since 1986, professor Blumen is active in the field of Oculopharyngeal Muscular Dystrophy (OPMD) both as a clinician and in epidemiologic, clinical and genetic research. He has identified the existence of a large OPMD cluster among Uzbek (Bukhara) Jews in Israel. In 2009, together with professor Braverman, he has founded and directed, at the Hillel Yaffe Medical Center, the first Israeli OPMD national service.

Together with collaborators from Israel and abroad, prof. Blumen has published several articles on OPMD in journals like Annals of Neurology, Neurology and others. He is one of the authors of the 1998 paper in "Nature" that reports the PABP2 expansion mutation producing OPMD.

Prof. Blumen's other fields of interest and research includes: motor neuron diseases, inherited peripheral neuropathies, familial spastic paraparesis and the interference between neurology and philosophy.



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Dr. Lior Greenbaum

Dr. Lior Greenbaum, MD PhD, graduated from the Hebrew University of Jerusalem. He specialized in neurology and medical genetics at Sheba Medical Center. Dr. Greenbaum deals in the field of neurogenetic diseases in adults, youth and children. He coordinates the neurogenetic clinic at the Sheba Genetic Institute, is a senior lecturer in neurology at Tel Aviv University and has participated in writing over 70 scientific articles.

A significant part of his work is devoted to the diagnosis of neuromuscular diseases, including neuropathies, myopathy, myotonia, motor neuron diseases and more. So far, Dr. Greenbaum has helped hundreds of patients in this type of situation in finding a genetic diagnosis for their condition, in a wide age range: from children to adults in their seventh and eighth decades of life.



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Dr. Ben M. Maoz

Dr. Maoz is a faculty member at the Sagol School of Neuroscience and the Department of Biomedical Engineering at Tel Aviv University. Dr. Maoz did his Ph.D on nano-optics in the School of Chemistry at Tel Aviv. During his post-doctoral studies, at Harvard University, in Prof. Don Ingber and Kit Parker, he developed Organ-on-a-Chip platforms for studying human relevant physiology.

Dr. Maoz received number of prestigious fellowships, awards and honors, such as the Harvard-Wyss Technology Fellowship, Azrieli Fellowship for Academic Excellence and Leadership, ERC grant, recently he was chosen by "The Marker" as the most promising 40 under 40 and he gave a talk in the first metaverse TedX

More information on the MaozLab can be found in: https://www.maozlab.com/



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Dr. Grant W. Mitchell

Grant W. Mitchell, MD, MBA, is a physician-entrepreneur in the biotech space focused on innovative healthcare delivery models and products. He is Co-Founder and Chief Executive Officer of Every Cure.

Previously, Grant was the Principal in Analytics at Quantum Black, and Engagement Manager at McKinsey & Company.

Grant received his B.A. in Economics from New York University and his MD/MBA from the University of Pennsylvania.



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Prof. Yaakov Nahmias

Prof. Yaakov "Koby" Nahmias is a bioengineer and innovator, whose breakthroughs ranged from the first 3D printing of cells to the first commercial human-on-chip technology. He is a Magna Cum Laude graduate of the Technion, Israel Institute of Technology, and is the founding director of the Grass Center for Bioengineering of the Hebrew University of Jerusalem.

Nahmias is a recipient of a NIH career award, two European Research Council (ERC) grants, the Kaye Innovation Award and the prestigious Rappaport Prize in Biomedical Research. He is the first scientist outside Britain to win the Rosetrees Trust Prize.

Nahmias is the co-founding director of BioDesign-Israel. An entrepreneurship program that educated over 120 fellows since 2013, launching 10 startup companies including Guide In Medical, CardioVia and SwiftDuct.

Nahmias is the founder and CSO of two biotechnology startups, including Tissue Dynamics that is developing a groundbreaking human-on-chip instruments for drug development, and the industry leading Future Meat Technologies focusing on the cost-effective production of cultured meat.





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Prof. Vered Raz

Vered Raz is an associate professor at the department of Human Genetics in Leiden University Medical Center, The Netherlands. Her research focuses on RNA biology's role in skeletal muscles and in adult-onset neuromuscular dystrophies.

She grew up in Kibbutz Beit Hashitta and obtained her Ph.D. from the Weizmann Institute of Science (cum laude), Israel. She obtained the Fulbright for a post-doc at the University of Pennsylvania, Philadelphia, US. With EMBO and Marie Currie fellowships she then carried out research in the Netherlands. Since 2009 she heads the OPMD and muscle aging research at Leiden University Medical Center. Her research is funded by European funding, governmental funding, patient organization, and companies.

Her group investigate the molecular mechanisms and molecular signatures for adult-onset muscle weakness using multidisciplinary omics approaches in cell and animal models as well as in muscles from patients. Omics results are then translated to functional causes, which opens opportunities for therapeutical developments.

She organized the first ENMC-OPMD meeting.

She heads the Dutch RNA network and the protein aggregation network.

She contributes to educational programs outside the academy, including secondary schools, adults, and patient organizations.



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Prof. Jean Lacau St Guily

Jean Lacau St Guily (Paris France) is MD, Professor of Otolaryngology Head Neck Surgery in Paris Sorbonne University & Faculty of Medicine. Previously head of the OL-HNS in Paris-Tenon University hospital, he works now in the Department of Otolaryngology-HNS, in Rothschild Foundation Hospital in Paris.

Past-President of the French Society of Oto-Rhino-Laryngology- HNS (2020-2022), he is a member of the French national academy of surgery, and an active researcher in the Myology laboratory, UMRS 974, Myology Institute G.H. Pitié-Salpétrière. Moreover he co-directs the research group of biomedical ethics in Bernardins center in Paris. His involvement in swallowing disorders from neuro-muscular origin and in OPMD, has been a whole life-story, in both clinical and scientific aspects; he published numerous international papers in the field, including a cellular therapy protocol in the human; he plays now a key role in a gene therapy protocol in the human which is hoped to begin within a short time.



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Prof. Capucine Trollet

Capucine Trollet, PhD, is Research Director DR2 INSERM - leader of the team OPeRA: Cellular and molecular orchestration in muscle regeneration, during ageing and in pathologies (OPMD, Regeneration and Aging). She received the PhD degree in the Chemical and genetic pharmacology laboratory directed by Daniel Scherman, Univearsity of Paris Descartes, Paris, France. Study, regulation, and application of in vivo gene transfer by electrotransfer. Her Post-doctoral training in the school of biological Sciences directed by Pr. George Dickson, Royal Holloway, university of London, United Kingdom in development of gene therapy strategies for muscular dystrophies.

Her team focuses on molecular and cellular actors involved in human in skeletal muscle regeneration, during ageing and in muscle dystrophies, particularly oculopahryngeal muscular dystrophy (OPMD) and Duchenne muscular dystrophy (DMD). She has been working on OPMD for 15 years now. Her approaches aim at better understanding RNA metabolism, muscle regeneration, muscle stem cells and fibrosis in order to develop therapeutic strategies.