

10th Anniversary Online Conference Little Steps Association for Duchenne-Becker Patients

Dec 7, 2020 * 6:00-10:15 p.m. Dec 8, 2020 * 4:00-10:15 p.m.

Monday, Dec 7, 2020

1st Session | 6:00-8:00 p.m.

6:00-6:15 p.m. Introduction	Eng*
Tali Kaplan, CEO, Little Steps Association Yoav Kisch, Deputy Health Minister Pat Furlong, Founding President & CEO, PPMD Film: A Decade of Activity. Little Steps	
6:15-6:35 p.m. The in-between: Why the Little Moments Matter More Than You Think.	Quality of Life Eng*
Christopher M. Jones	
6:35-6:50 p.m. Introduction to DMD Therapies	Research Eng*
Prof. Annemieke Aartsma-Rus, Leiden University Medical Center	
6:50-8:00 p.m. Clinical Trials Updates - Gene Therapy	
Chair: Prof. Yoram Nevo, Dr. Talya Dor	
Micro-Dystrophin Treatment Strategy: An Introduction	Research
Dr. Talya Dor, Head of Pediatric Neuromuscular Clinic Hadassah Medical Center Jerusalem Israel	
Micro-dystrophin Gene Therapy	Research Eng*
S Alexander Peter Murphy MBChB, MRCPCH, PhD Medical Director, Roche Product Development - Neuroscience	
Pfizer Gene Therapy Trial (PF-06939926) for Duchenne Muscular Dystrophy	Research Eng*
Prof. Yoram Nevo, Director, Institute of Neurology, Schneider Children's Medical Center Israel	
An IGNITE DMD Update, Solid Bio	Research Eng*
Annie Ganot (Founder & Head of Advocacy), Joel Schneider (Chief Technology Officer) and Cathryn Clarey (Acting CMO)	
DMD Genethon International Program: Natural History and Future Gene Therapy Trial	Research Eng*
Prof. Francesco Muntoni, Principal investigator Dubowitz Neuromuscular Centre, UCL Great Ormond Street Institute of Child Health & Great Ormond Street Hospital. London, UK	
7:40-8:00 p.m. Q&A panel	Eng*

*English spoken/ subtitled

Monday, Dec 7, 2020

2nd Session | 8:30-10:15 p.m.

8:30-8:35 p.m. Film	Eng*
Through His Eyes: Slamann's Story	
8:35-9:00 p.m. DMD Management Along the Disease's Lifespan	Treatment Eng*
Prof. Nathalie Goemans, Head of the Neuromuscular Reference Center, Department of Pediatrics and Child Neurology, University Hospitals Leuven	
9:00-9:15 p.m. Integrated Treatment Approach	Treatment
Dr. Maurit Beerli, CEO of Alyn Hospital and Ziva Goldschmiedt, Chair of Little Steps Association	
9:15-9:25 p.m. Between Participation, Environment, Movement, and Joy	Quality of Life
Nava Gelkop, BPT MSc, Chair of the Israeli Association for Child Development and Rehabilitation	
9:25-9:30 p.m. Stand Up Comedy	
Adi Goldschmiedt	
9:30-10:15 p.m. Clinical Trials Updates - Ambulatory Patients	
Chair: Dr. Liora Sagi, Dr. Sharon Aharoni	
A Safer Steroidal Anti-Inflammatory? Structural to Clinical Studies of Vamorolone	Research Eng*
Eric Hoffman, VP Research ReveraGen	
Italfarmaco Givinostat Development Program Update	Research Eng*
Ana Christensen MPH, Patient Science Liaison for Italfarmaco	
Sarepta RNA-Targeted Therapies	Research Eng*
Luigi Picaro, Senior Director, Medical Director, Italy, Israel & Greece	
10:00-10:15 p.m. Q&A panel	Eng*

*English spoken/ subtitled

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Tuesday, Dec 8, 2020 3rd Session | 4:00-5:30 p.m. | Adults

4:00-4:10 p.m. Film	Living Alongside Duchenne - Amir's Story	Eng*
4:10-4:30 p.m. Care of the Adult with Duchenne Muscular Dystrophy	Prof. Ros Quinlivan, Consultant in Neuromuscular Disease, MRC Centre for Neuromuscular Disease, UCL Institute of Neurology, National Hospital London	Treatment Eng*
4:30-4:40 p.m. Respiratory Treatment for Adult Patients	Dr. Erik Baltaxe, Institute of Pulmonary and Allergy Medicine, Rabin Medical Center	Treatment
4:40-5:00 p.m. Respiratory Aspects of Physical Therapy in DMD Adult Patients	Eran Lingo, Physiotherapist BPT Prolonged Mechanical Ventilation Unit, Bet-Hadar and Nurit Unit for Clalit Health Services, Home Respiratory Patients	Treatment
5:00-5:20 p.m. Q&A panel	Chair: Prof. Vivian Drory	Eng*
5:20-5:30 p.m. 10 Minutes A Day of Positive Thinking Practice	Shirley Ness-Berlin, Lecturer and Writer for Positive Thinking	Quality of Life

*English spoken/ subtitled

Tuesday, Dec 8, 2020 4th Session | 6:00-8:00 p.m.

6:00-6:05 p.m. Introduction	Meir Shpigler, CEO, National Insurance Institute of Israel Film: Eight Bundles of Joy: the Shwartzes	Eng*
6:05-6:20 p.m. The Power of Community	Michal Elyashiv, Social Worker and Director of the family support center, Little Steps Association	Quality of Life
6:20-6:40 p.m. The Impact of DMD and Steroid on Bone, Growth and Puberty	Jarod Wong, Senior Clinical Lecturer/Consultant Paediatric Endocrinologist, University of Glasgow	Treatment Eng*
6:40-6:50 p.m. The Art of Respiratory Care in Duchenne and Becker Muscular Dystrophy	Prof. Richard Shell, MD of Pediatrics Section, Chief Pulmonary Medicine, Nationwide Children's Hospital and The Ohio State University	Treatment Eng*
6:50-8:00 p.m. Clinical Trials Updates - Non-Ambulatory Patients	Chair: Dr. Mira Ginsberg, Dr. Sharon Aharoni	
Outcomes and Learnings from The Sideros Interim Analysis	Shabir Hasham, Global Program Lead, Santhera	Research Eng*
FibroGen DMD Program. Pamrevlumab LELANTOS Phase 3 Studies in Patients with DMD	Bassem Elmarkabadi, M.D., ABWS. Executive Medical Director, Clinical Development (Fibrosis / Oncology) Elias Kouchakji, M.D. Sr. VP of Clinical Development and Drug Safety	Research Eng*
ASP0367 Development Program Update	Gerard J Marek, MD, PhD, Executive Medical Director, Astellas Pharma Global Development Inc.	Research Eng*
Update on ATL1102 Program for Non-Ambulant Boys with DMD	Mark Diamond, CEO & Managing Director, Antisense Therapeutics Limited	Research Eng*
7:40-8:00 p.m. Q&A panel		Eng*

*English spoken/ subtitled

Tuesday, Dec 8, 2020 Poster session | Research | 8:00-8:30 p.m.

Tuesday, Dec 8, 2020 5th Session | 8:30-10:15 p.m.

8:30-8:45 p.m. Film	My Brother and Me - The Story of Duchenne & Becker Patients' Siblings	Eng*
8:45-8:55 p.m. The Heart Is a Muscle Too	Linda Cripe MD/Pediatric Cardiologist Professor of Pediatrics, Nationwide Children's Hospital, The Ohio State University Columbus, OH USA	Treatment Eng*
8:55-9:05 p.m. Health is Not Medicine - A Journey from a Rehabilitative Point of View	Dr. Mauri Beerli, Director General at Alyn Hospital Pediatric and Adolescent Rehabilitation Center, Jerusalem	Quality of Life
9:05-9:15 p.m. Film	Steps of Hope - The Story of Ziva, Adi Goldschmiedt's Mom	
9:15-9:25 p.m. The Importance of Physical Activity in Duchenne and Becker Muscular Dystrophy - a Physiological Research	Itai Goldfarb, Exercise Physiologist, Ph.D. student Tel-Aviv University Under the supervision of Prof. Yoram Nevo Director, Institute of Neurology, Schneider Children's Medical Center of Israel.	Research Eng*
9:25-9:40 p.m. CRISPR/Cas 9 for DMD	Prof. Annemieke Aartsma-Rus, Leiden University Medical Center	Research Eng*
9:40-9:50 p.m. Loaded Exosomes as a Novel Approach for Gene Therapy in Duchenne Muscular Dystrophy	Prof. Daniel Offen, Dept. of Human Molecular Genetics and Biochemistry, Sackler Faculty of Medicine Sagol School of Neuroscience, Tel Aviv University	Research Eng*
9:50-10:15 p.m. Novel Pathways and Therapeutic Approaches in DMD and BMD	Prof. Chaya Brodie, PhD. Faculty of Life Sciences & Mussel Therapeutics, Bar Ilan University	Research Eng*

*English spoken/ subtitled

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