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 WEBINAR VERSION



NOVEMBER 2 AND 3, 2020 AT 10:00AM - 1:00 PM US EASTERN STANDARD TIME

**Zoom Agenda at a Glance 10AM EST-1PM EST November 2,3, 2020**

Day 1		Eastern Standard Time	Talk Length (Q&A)
Introduction & Greetings	PRF Executive Director <i>Audrey Gordon, Esq</i> Board Chair <i>Scott Berns, MD, MPH</i> Medical Director <i>Leslie Gordon, MD, PhD,</i>	10:00-10:13	13
<b>Moderator: Leslie Gordon</b>			
<i>Sammy, Amerigo and Laura Basso, Italy &amp; Alexandra, Cédric, and Esther Peraut, Spain</i>	The Journey From No Hope, to Clinical Trials, and Entering Young Adulthood – Living Life to Its Fullest	10:13-10:43	25 (5)
<b>Virtual Group Photo – Please make sure your video is on.</b>			
<b>Treatments and Trials on the Horizon</b>			
<b>Moderator: Mark Kieran, MD, PhD, Bristol-Meyers Squibb</b>			
<i>David Cory</i> Eiger BioPharmaceuticals, Inc., Palo Alto, CA	Rare Disease Pharma and the Journey Towards Lonafarnib Approval	10:44-10:59	10 (5)
<i>Monica Kleinman, MD*</i>	Current Treatment Trial Update	11:00-11:12	7 (5)
<i>Bum-Joon Park, PhD* and Dr. Minju Kim</i> Pusan National University, South Korea	Effect of Progerinin (SLC-D011) and Lonafarnib on HGPS: A Combined in Vitro and in Vivo Study	11:13-11:30	12 (5)
<i>Martin Bergö, PhD*</i> Karolinska Institutet, Stockholm, Sweden	Preclinical Development of ICMT Inhibitors for HGPS Therapy	11:31-11:48	12 (5)
<b>BREAK 11:48-12:03</b>			
<i>Colin Stewart, PhD*</i> Institute of Medical Biology, Immunos, Singapore	Breaking the LINC to Treat Progeria	12:03-12:20	12 (5)
<i>Giovanna Lattanzi, PhD*</i> CNR Institute of Molecular Genetics, Bologna, Italy	Improving the Quality of Life in Progeria: an IL-6 Receptor Antibody Murine Treatment Trial with Tocilizumab	12:21-12:38	12 (5)
<i>Vicente Andrés, PhD*</i> CNIC, Spanish National Cardiovascular Research Center, Madrid, Spain	Temporal, systemic and tissue-specific factors regulate disease progression and lifespan in a new HGPS mouse model	12:39-12:56	12 (5)
<b>Day 1 wrap-up – Leslie Gordon</b>			
<b>End of Day 1</b>			

<b>Day 2</b>			
Introduction and Framework of Meeting – Leslie Gordon		10:00-10:10	10
<b>Gene and RNA Therapeutics for HGPS Moderator: Francis Collins</b>			
<i>David R. Liu, PhD*</i> Howard Hughes Medical Institute, Broad Institute,	Base Editing to Correct HGPS in Patient Cells and in Animals	10:10-10:27	12 (5)
<i>Francis Collins, MD, PhD*</i> National Institutes of Health, Bethesda, MD	RNA and DNA Therapeutics for Progeria	10:28-10:45	12 (5)
<i>Tom Misteli, PhD*</i> National Cancer Institute, NIH, Bethesda, MD	RNA as a therapeutic target in HGPS	10:46-11:03	12 (5)
<b>BREAK 11:05-11:20</b>			
<b>Panel Discussion: Cause and Treatments: Cardiovascular Disease in HGPS and Ageing</b> <b>Moderator : Leslie Smoot, MD*</b> , Boston Children's Hospital, Boston, MA			
Introduction by Leslie Smoot, MD		Key Goals for Cardio-neurovascular Discovery in Progeria	11:20-11:24 4
<i>Ashwin Prakash, MD*</i> Boston Children's Hospital, Boston, MA	Echocardiography- diastolic dysfunction, AV stenosis	11:25-11:42	12 (5)
<i>Marie Gerhard-Herman, MD* &amp; Srinivas Mukundan, MD, PhD*</i> , Brigham and Women's Hospital, Boston, MA	New discoveries in vascular disease, collateral flow, and associations with aging	11:43-12:08	20 (5)
<i>Francesco Musumeci, MD</i> S. Camillo Hospital Roma, Azienda Ospedaliera San Camillo Forlanini, Italy & <i>Binny Shah, MD</i> , Brigham and Women's Hospital, Boston, MA	Aortic valve replacement in Progeria and Progeroid Laminopathy	12:09-12:34	20 (5)
<b>Key Lessons from Current Meeting and Looking Towards the 2021 Workshop</b>			
Tom Misteli, Judy Campisi, Monica Kleinman		12:35-12:55	<b>Inter-active</b>
<b>Leslie Gordon – Closing and Look forward to the 2021 Workshop in person!</b>		12:56-1:00	
* = PRF grantee			

## The 2020 PRF Progeria Workshop Organizing Committee

**Leslie B. Gordon, MD, PhD (Chair)** - *Medical Director, The Progeria Research Foundation; Professor of Pediatrics, Div. of Genetics, Hasbro Children's Hospital and The Warren Alpert Medical School of Brown University; Dept. of Anesthesia, Boston Children's Hospital and Harvard Medical School*

**Vicente Andrés, PhD** – *Professor, Laboratory of Molecular and Genetic Cardiovascular Pathophysiology, and Director Department of Basic Research, CNIC, Spain*

**Judith Campisi, PhD** – *Professor, Buck Institute for Research on Aging, and Senior Scientist, Lawrence Berkeley National Laboratory*

**Mark Kieran, MD, PhD** – *Clinical Trial Lead, Pediatrics Program, Oncology Clinical Development, Bristol-Myers Squibb*

**Audrey Gordon, Esq** – *President and Executive Director, The Progeria Research Foundation (PRF)*

**Lynn Doucette** - *Meeting Coordinator; Executive Administrative Assistant to the Medical Director, PRF*

**Madeline Schwartz, Kelly Edwards, Meghan Johnson** – *Technical Team; National Institute for Children's Health Quality (NICHQ)*

**ANA CLARA SAYS SEE YOU AT THE IN-PERSON WORKSHOP NEXT YEAR!**

