## The Progeria Research Foundation 9th International Scientific Workshop





Thursday, September 20 Registration and Poster Set-up		
2:00-4:00 PM	Registration in the West Tower, level two, Foyer in front of Grand Ballroom	
2:00-4:00 PM	Poster Set-up: All poster presenters (Sessions 1 and 2) are asked to set up their posters at this time in the West Tower, level two, Skylines A-E	

Thursday, September 20 Day 1 Kickoff Session: 4:00 PM – 9:30 PM, Grand Ballroom Dinner Included					
4:00-4:30 PM	Introduction & Greetings: Medical Director Leslie Gordon, MD, PhD, PRF Executive Director Meryl Fink, Esq; Board Chair Scott Berns, MD				
4:30-5:30	Children with Progeria and their Parents	Patient and parent perspective: Living with Progeria, participating in clinical trials			
5:30-5:40	Francis Collins, MD, PhD National Institutes of Health	Plenary speaker Introduction			
5:40-6:20	Eric S. Lander, PhD - Harvard Medical School; the Broad Institute of MIT and Harvard	New genomic approaches for understanding disease			
6:25	DINNER IS SERVED				

Friday, September 21 Day 2: Breakfast Buffet / 7:00 AM – 7:55 AM, Grand Ballroom All Posters should be set up by start of morning session.				
Day 2 Morning Session				
Molecular and Cellular Mechanisms in HGPS and Aging (I)  Moderator: Vicente Andrés García, CNIC				
8:30-9:15	Meeting Program Overview & Intro. To Progeria	Leslie Gordon		
9:15-9:45	Juan Carlos Izpisúa Belmonte The Salk Institute for Biological Studies, La Jolla, CA	CRISPR/Cas gene editing in HGPS mice		
9:45-10:15	Nicolas Levy University of Marseille, Inserm, France	MG132-induced progerin clearance is mediated by autophagy activation and splicing regulation in Progeria		
10:15-10:45	BREAK			
10:45-11:15	Vicente Andrés García, PhD Centro Nacional de Investigaciones Cardiovasculares Carlos III, Madrid, Spain	Molecular and cellular mechanisms governing cardiovascular disease and aging in HGPS		
11:15-11:45	Isabella Saggio – Dept. Biology and Biotechnology "C. Darwin" Sapienza University of Rome, Italy	The lamin-interacting telomeric protein AKTIP in HGPS		
11:45-12:15	Selected Posters – Short Oral Presentations			
12:15-12:45	Lightening Poster Session – Odd numbered	1 minute & 1 slide per poster presenter		
12:45-1:45	LUNCH			
	Day 2 Afternoon Session			
	Cardiovascular Disease in HG Moderator: Mark Kieran, Dana Farber Cancer			
1:45-2:45	Ashwin Prakash; Boston Children's Hospital, Boston Srinivasan Mukundan; Brigham and Women's Hospital Marie Gerhard-Herman; Brigham and Women's	Clinical Cardio-Neurovascular Panel: echocardiographic, Brain, and Peripheral Vascular manifestations in Progeria, and effect of Lonafarnib on disease		
2:45-3:15	Richard K. Assoian Institute of Translational Medicine and Therapeutics, University of Pennsylvania	Extracellular Matrix remodeling and arterial stiffening in HGPS: lessons from the <i>LMNA</i> G609G mouse		
3:15-4:00	7 <sup>th</sup> INNING STRETCH and Ne	tworking Time		
4:00-4:30	Roland Foisner Max F. Perutz Laboratories, Medical University, Vienna	Progerin expression in endothelial tissue impairs nucleocytoskeletal coupling and causes cardiovascular Pathology		
4:30-5:00	Lightening Poster Session- Even numbered	1 minute & 1 slide per poster presenter		
ADJOURN UNTIL DINNER AND EVENING SESSION				
6:00-8:00 PM	6:00-8:00 PM Buffet Dinner/Informal Conversation, Grand Ballroom			
Day 2 Evening Session: Formal Poster Session #2 - Skylines A-E				
6:30-8:00	Even-Numbered Posters Formally Presented			
8:00-9:30	Odd-Numbered Posters Formally Presented			

Saturday, September 22						
Day 3: Breakfast Buffet / 7:15 AM – 8:25 AM, Grand Ballroom  Day 3 Sunrise Session: PRF's Newly Revised Granting Structure						
	or: PRF Medical Research Committee Chair Bryan To	· ·				
B	committee Members: Bryan Toole, Vicente Andres, Ted crown, Judy Campisi, Maria Eriksson, Tom Glover, Leslie Bordon, Christine Harling-Berg, Monica Kleinman, Tom Misteli, Marsha A. Moses	PRF's Revised Granting Structure: How to Optimize Applications for Success				
	Day 3 Morning Session					
Current and Future Treatments and Trials Moderator: Tom Misteli, National Cancer Institute, NIH						
8:30-9:00	Monica Kleinman – Boston Children's Hospital	Phase 1 Results: Ongoing Everolimus plus Lonafarnib clinical trial				
9:00-9:30	Ralph D'Agostino, Sr. and Joe Massaro Boston University, Boston, MA	Statistical challenges in trial design and outcome measures for Progeria				
9:30-10:00	Leslie Gordon Boston Children's and Hasbro Children's Hospitals	Biochemical biomarker development in Progeria				
10:00-10:30	10:00-10:30 BREAK					
10:30-11:00	Francis Collins National Institutes of Health, Bethesda, MD	Progeria: from basic science insights to therapeutic opportunities				
11:00-11:30	Tom Misteli National Cancer Institute, NIH, Bethesda, MD	RNA as a therapeutic target in HGPS				
11:30-12:00	Silvia Ortega Gutiérrez Medicinal Chemistry Laboratory Av Complutense, Madrid, Spain	New Isoprenylcysteine carboxylmethyltransferase (ICMT) Inhibitors for the Treatment of Progeria				
12:00-12:30	Ricardo Villa-Bellosta Fundación Jiménez Díaz Univ. Hospital Health Research Institute (FIIS-FJD, Madrid, Spain)	Therapeutic strategies to recover the normal pyrophosphate homeostasis in HGPS				
12:30-1:30	LUNCH					
	Day 3 Afternoon Session					
Molecular and Cellular Mechanisms in HGPS and Aging (II)  Moderator:						
1:30-2:00	Toren Finkel- Aging Institute, Pittsburgh, PA	Vascular autophagy and Hutchinson- Gilford Progeria				
2:00-2:30	Carlos Lopez-Otin Universidad De Oviedo, Spain	NF-κB signaling as a driver of Progeria and ageing				
2:30-3:00	BREAK					
3:00-3:55	Summation of Meeting and Key Findings	Judy Campisi,PhD: Professor of Biogerontology, Buck Institute, Tom Misteli, Mark Kieran				

## Thank you for coming!

The 2018 PRF Progeria Workshop Organizing Committee

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

Tom Misteli, PhD – Director, Center for Cancer Research, NCI, NIH

Vicente Andrés García, PhD – Professor, Laboratory of Molecular and Genetic Cardiovascular Pathophysiology,

Centro Nacional de Investigaciones Cardiovasculares Carlos III

Mark Kieran, MD, PhD – Clinical Trial Lead, Pediatrics Program, Oncology Clinical Development, Bristol-Myers Squibb Meryl Fink, Esq – President and Executive Director, PRF

Maria Sullivan – Continuing Medical Education Director, The Warren Alpert Medical School of Brown University Donnalynn Gibson (Meeting Coordinator) – Executive Administrative Assistant to the Medical Director, PRF