#### THE PROGERIA RESEARCH FOUNDATION 12TH INTERNATIONAL SCIENTIFIC WORKSHOP



October 29–31, 2025 Boston Marriott Cambridge Cambridge, MA

## **AGENDA**

Wednesday, Oct	ober 29, Check-In and Poster Set-Up		
3:00-5:00 PM	Registration Open in Grand Ballroom Foyer (Second Floor)		
3:00-5:00 PM	Poster Set-Up in Ballroom Salon 5 – Please check in at Registration for set-up instructions		
	ober 29, Day 1: Kickoff Session		
5:00-5:15 PM	Introduction & Greetings from PRF	Audrey Gordon, Esq Scott Berns, MD, MPH, FAAP Leslie Gordon, MD, PhD The Progeria Research Foundation	
5:15-5:25 PM	PRF Medical Research Remarks	Leslie Gordon	
5:25-6:25 PM	"Coming of Age" Conversations: Young Adults with HGPS as Research Partners	Moderator: Merlin Waldron, USA Kaylee Halko, USA Amber Vandeweert-Wielemans and Michiel Vandeweert, Belgium Riccardo Zanolli, Italy	
6:25-6:35 PM	Musical Kickoff	Francis Collins, MD, PhD	
6:35-6:45 PM	Group Photo – All Attendees!		
6:45-8:00 PM	Dinner & Collaborative Interaction Time		
Thursday, Octob	per 30, Day 2: Workshop Sessions		
8:00-9:00 AM	Bridging the Bench Mentor Session		
9:00-9:30 AM	Breakfast – All posters should be set up by the sta		
	Morning Session: Current Treatments and		
	Moderator: Mark Kieran, MD, PhD, Day One		
9:30-9:50 AM	*New Trial: Disease Progression in Progeria	Leslie Gordon, Boston Children's Hospital and PRF	
9:55-10:15 AM	*Phase 2a Progerinin Clinical Treatment Trial for Progeria	Monica Kleinman, MD Boston Children's Hospital	
10:20-10:40 AM	*Tools for Pivotal Science and Medicine in Progeria: Biomarker Assay Development, Qualification and Reagent Generation	Barbara Natke, PhD The Progeria Research Foundation	
10:45-11:05 AM	*Late-Stage Treatment of Aortic Stenosis in HGPS	Ashwin Prakash, MD Boston Children's Hospital	
11:10-11:30 AM	Break		
11:35-11:50	Late Breaking Presentation: A novel progerin- producing nonclassic HGPS phenotype: What progeria might look like with effective therapy	Linda Bulmer, MD Sahlgrenska University Hospital Gothenburg, Sweden	
11:55-12:20 PM	Poster Lightning Round	Poster Presenters	
12:20-1:30 PM	Lunch and Collaborative Interaction Time		
	Afternoon Session: Cardiovascular Disease Moderator: Maria Eriksson, PhD, Karolinska		
1:30-1:50 PM	*New Autoptical Pathological findings Revealing HGPS Pathophysiology	Andrea Porzionato, PhD University of Padova, Italy	

2:20-2:40 PM 2:45-3:05 PM	Landscape of Arterial Cells in Progeria	
		Lara Garcia Merino, MSc
2:45-3:05 PM	*Evaluation of Coronary Artery and Aortic	Sunil Ghelani, MD
2:45-3:05 PM	Calcium in Progeria by Computed Tomography	Boston Children's Hospital
	*Angiopoietin-2: A Potential Therapeutic Target	Lizzy Izydore, BS (for Kan Cao, PhD)
0.05.0.05.514	for Vascular Protection in HGPS	University of Maryland
3:05-3:25 PM	Break	
3:25-3:45 PM	*Bridging the gap: Translating CVpathology to	Keri Shafer, MD & Sheila Hegde, MD
	clinical assessments and research study design	UT Southwestern Medical Center
3:50-4:10 PM	*Progeria and Vascular Calcification: Diet and	Ricardo Villa Bellosta, PhD
	Treatments	Center for Research in Molecular
		Medicine and Chronic Diseases (CiMUS),
		Spain
4:15-4:25 PM	Poster Elevation: LRRK2 Knockdown Rescues	Lukas Mann
	Progerin-Induced Premature Aging	Institute of Molecular Biology Mainz,
		Germany
4:25-4:35 PM	Poster Elevation: National Interview Survey for	Yuko Okawa, MD
	Patients with Hutchinson-Gilford Progeria	Oita University, Japan
	Syndrome and Progeroid Laminopathies and	
	Their Families in Japan	
4:35-5:30 PM	Adjourn Until Poster Session & Dinner	
	Evening Session: Posters and I	Dinner
5:30-6:30 PM	Poster Session Part 1	Even Numbered Posters*
6:00-7:00 PM	Dinner and Collaborative Interaction Time	
7:00-8:00 PM	Poster Session Part 2	Odd Numbered Posters*
	1, Day 3: Workshop Sessions	Gad Nambered 1 osters
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7:30-8:30 AM	Breakfast & Collaborative Interaction Time	
	Morning Session 1: Progeria Treatment Discove Moderator: Leslie Gordon	ery for a Healthy Future
8:30-8:50 AM	*Al-driven Therapeutic Antisense Oligonucleotide	Ning Shen, PhD
	for HGPS	Zhejiang U. School of Medicine, China
8:55-9:15 AM	*Development of Gene Silencing (RNAi) & Gene	Nizar Saad, PhD
	Editing Therapeutics for HGPS	Nationwide Children's Hospital
9:20-9:45 AM	*Mini Plenary: Next Generation Therapeutic	David Liu, PhD
	Genome Editing	Broad Institute, MIT
	*ProSPER: Progeria Streamlined Platform Gene	
9:50-10:10 AM	1 1001 Ett. 1 10gcha Oli carrillinea i latiorni Oche	David Liu
9:50-10:10 AM	Editing for Rare Diseases	David Liu Francis Collins
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9:50-10:10 AM	<u> </u>	Francis Collins Leslie Gordon
	Editing for Rare Diseases	Francis Collins Leslie Gordon Meirui An, PhD, Broad Institute Mike Erdos, PhD, NHGRI
9:50-10:10 AM 10:15-10:35 AM	*First-Generation PROTACs for Improving	Francis Collins Leslie Gordon Meirui An, PhD, Broad Institute Mike Erdos, PhD, NHGRI Silvia Ortega Gutierrez, PhD
10:15-10:35 AM	*First-Generation PROTACs for Improving Progeria Phenotype	Francis Collins Leslie Gordon Meirui An, PhD, Broad Institute Mike Erdos, PhD, NHGRI
	*First-Generation PROTACs for Improving Progeria Phenotype Break	Francis Collins Leslie Gordon Meirui An, PhD, Broad Institute Mike Erdos, PhD, NHGRI Silvia Ortega Gutierrez, PhD Univ. Complutense de Madrid, Spain
10:15-10:35 AM	*First-Generation PROTACs for Improving Progeria Phenotype Break  Morning Session 2: Endocrine and Gl Disc	Francis Collins Leslie Gordon Meirui An, PhD, Broad Institute Mike Erdos, PhD, NHGRI Silvia Ortega Gutierrez, PhD Univ. Complutense de Madrid, Spain
10:15-10:35 AM	*First-Generation PROTACs for Improving Progeria Phenotype Break  Morning Session 2: Endocrine and GI Disc Moderator: Vicente Andrés, P	Francis Collins Leslie Gordon Meirui An, PhD, Broad Institute Mike Erdos, PhD, NHGRI Silvia Ortega Gutierrez, PhD Univ. Complutense de Madrid, Spain
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# Thanks for Coming and See You Next Time! The 2025 PRF Progeria Workshop Organizing Committee

Leslie B. Gordon, MD, PhD (Chair) - Medical Director, The Progeria Research Foundation; Professor of Pediatrics, Hasbro Children's Hospital, Boston Children's Hospital, and Harvard Medical School Audrey Gordon, Esq — President and Executive Director, The Progeria Research Foundation Maria Eriksson, PhD — Professor, Department of Medicine, Huddinge, Karolinska Institutet, Sweden Vicente Andrés, PhD — Professor, Laboratory of Molecular and Genetic Cardiovascular Pathophysiology, and Director Dept. of Basic Res., Centro Nacional de Investigaciones Cardiovasculares Carlos III (CNIC) Mark Kieran, MD, PhD — VP Clinical Development, Day One Biopharmaceuticals Darrien Marazzo, BA — Meeting Coordinator, The Progeria Research Foundation Elizabeth Maynard, CMP — Workshop Planner, Executive Director, Etre Partners, LLC



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