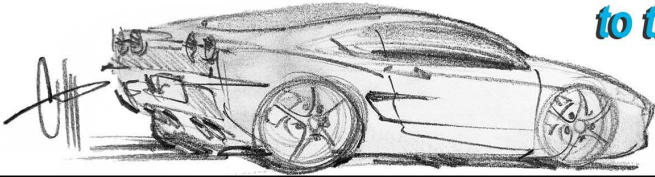


RACE Progeria to the Cure

November 2-4, 2022
Cambridge, MA



Detailed Workshop Agenda

Wednesday, November 2 Check-In and Poster Set-up

2:00-4:00 PM	Check-in located 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, Skyline A-E

Wednesday, November 2 Day 1 Kickoff Session: 4:00 PM – 8:00 PM, Grand Ballroom Dinner Included

4:00-4:30 PM	Introduction & Greetings: Medical Director Leslie Gordon, MD, PhD, PRF Executive Director Audrey Gordon, Esq; Board Chair Scott Berns, MD	
4:30-5:20	In-person - Sammy Basso, Carlos Luiz Silva, Meg Waldron By Zoom: children from around the world	Meet the Children Around the World Living with Progeria: Inspiring Presentations by Patient Families
5:20-5:40	Francis Collins National Institutes of Health, Bethesda, MD	Progeria Musical Celebration
5:40-6:00	Leslie Gordon The Progeria Research Foundation, Brown University, Hasbro Children's Hospital, Boston Children's Hospital, and Harvard Medical School, Boston, MA	The Lonafarnib Journey to Approval – Lessons for Future Treatments and the Cure
Group Photo		
6:15	DINNER IS SERVED	

Thursday, November 3
Day 2: Breakfast Buffet / 7:25 AM – 8:25 AM, Grand Ballroom
All Posters should be set up by start of morning session.

Day 2 Morning Session

8:25 **Progeria Clinical Trial Development Programs**
Moderator: Francis Collins, MD, PhD, National Human Genome Research Institute (NIH)

8:30-8:40	Leslie Gordon The Progeria Research Foundation, Brown University, Hasbro Children's Hospital, Boston Children's Hospital, and Harvard Medical School, Boston, MA	Hello and Meeting Program Overview
8:40-9:10	David Liu* Merkin Institute of Transformative Technologies in Healthcare, Broad Institute, Howard Hughes Medical Institute, Harvard University, Boston, MA	Plenary Talk: Base Editing and Prime Editing: Precision Gene Correction Without Double-Strand DNA Breaks
9:10-9:30	Francis Collins David Liu John Evans – Chief Executive Officer, Beam Therapeutics, Cambridge, MA Leslie Gordon	Panel Presentation: Base Editing to Rescue Progeria – Where Are We Now, and Where Do We Need to Go?
9:30-9:50	Francis Collins Leslie Gordon Ray Skwierczynski - Skwierczynski Consulting, MA Emma McCullagh – Preclinical Toxicology Specialist, Domain Biotechnologies, Austin, TX	Panel Presentation: Update on Progress in Development Program for SRP-2001 HGPS-specific RNA Therapy
9:50-10:10	Bae-Hoon Kim PRG Science and Technology, Korea	Update on Progress in Development Program for Progerinin, Inhibitor of Progerin
10:10-10:30	Monica Kleinman Boston Children's Hospital and Harvard Medical School, Boston, MA	Everolimus Plus Lonafarnib Clinical Trial Outcomes
10:30-10:50	<i>BREAK and Group Photo</i>	
Progeria Biomarkers and Progerin Turnover Across Tissues		
10:50-11:10	Abigail Buchwalter* Cardiovascular Research Institute and the Department of Physiology at the University of California, San Francisco, CA	Combined Proteome-Wide Measurements of Protein and Cell Turnover Across Tissues Accounts for The Tissue Specificity of Progeria

11:10 -11:30	Leslie Gordon	Plasma Progerin Changes with Lonafarnib Therapy - Association with Drug Effect and Extended Survival
11:30-11:50	Mario Cordero* Biomedical Research and Innovation Institute of Cadiz (INIBICA), Cadiz, Spain	Biochemical Biomarker Development in Progeria
Day 2 Afternoon Session Moderator: Maria Eriksson, PhD, Karolinska Institutet, Huddinge, Sweden		
Basic Biology of Progeria, Poster Elevation Talks and Lightning Poster Session		
11:50-12:10	Selected Poster Presentation Colin Stewart ASRL, Singapore	Disrupting the LINC complex as a therapeutic route to treating HGPS
12:10-12:30	Selected Poster Presentation Magda Hamczyk Universidad de Oviedo, Spain	Progerin expression in endothelial cells does not provoke premature aging or atherosclerosis in atheroprone mice
12:30-1:30	LUNCH	
1:30-2:00	Lightning Poster Session	1 minute & 1 slide per poster presenter
2:00-2:20	Maria Eriksson	Single-cell RNA analysis of the murine Arterial Wall During HGPS Disease Progression
2:20-2:40	Giovanna Lattanzi* CNR Institute of Molecular Genetics, Bologna, Italy	Adipocyte-Specific Effects of Drug Treatment Prospects in HGPS
2:40-3:00	John Cooke Houston Methodist, Houston, TX	DNA Damage in Progeria, and its Reversal by Telomerase
3:20-4:50	Formal poster session #1: Even-Numbered Posters – Skyline A-E	
ADJOURN UNTIL DINNER AND EVENING SESSION		
6:00–8:00 PM	Buffet Dinner/Informal Conversation, Grand Ballroom	
6:30-8:00	Formal poster session #2: Odd-Numbered Posters – Skyline A-E	

Friday, November 4
Day 3: Breakfast Buffet / 7:15 AM – 8:25 AM, Grand Ballroom

Day 3 Sunrise Session: Round Tables with Leaders In Progeria and Aging (Table Sign-up Required)

8:00-8:30	Vicente Andrés Sammy Basso Francis Collins Leslie Gordon Monica Kleinman Tom Misteli- National Cancer Institute, Bethesda, MD	Mentors will be assigned to a table where investigators will have significant personal exposure to senior leaders and a patient who can provide guidance and inspiration. Sign up here
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Day 3 Morning Sessions

8:40 Progress in Cardio-Neurovascular Treatment Outcomes in HGPS
Moderator: Marie Gerhard-Herman, MD , Brigham and Women’s Hospital, Boston MA

8:40 – 8:50	Marie Gerhard-Herman	Overview of Ongoing CV/NV Progress at BCH and BWH and Summary of CVIS Meeting
8:50-9:10	Srinivas Mukundan Reveal Pharmaceuticals, Cambridge, MA	New Stroke and Brain Vascular Natural History Findings in Progeria
9:10 -9:30	Marie Gerhard-Herman* Brigham and Women’s Hospital, Boston MA	Pulse Wave Velocity Portends Survival Risk in HGPS
9:30-9:50	Pedro del Nido Boston Children’s Hospital; Harvard Medical School, Boston, MA	TAVR and Apico-Aortic Conduit Surgery for Critical Calcific Aortic Stenosis in HGPS
9:50-10:05	Sammy Basso Sammy Basso’s Italian Association for Progeria, Italian Network for Laminopathies, Italy	Patient Perspective on Quality of Life 3 years Post-Aortic Valve Intervention
10:05-10:25	BREAK	

Cardiovascular Pathobiology of Progeria

10:25-10:55	Jay Humphrey Department of Biomedical Engineering Yale University, New Haven, CT	Evolving Vascular Phenotypes and Cardiovascular Consequences in a Mouse Model of HGPS and Its Treatment
10:55-11:15	Vicente Andrés* Centro Nacional de Investigaciones Cardiovasculares (CNIC), Madrid, Spain	Smooth Muscle or Endothelium: Which is to Blame for Precocious Vascular Disease and Death in HGPS?
11:15-11:35	George Truskey Duke University Durham, Durham, NC	Effect of Adenine Base Editing on the Function of a Tissue-Engineered Vascular Model of HGPS
11:35-11:55	Roland Foisner Max F. Perutz Laboratories, Medical University, Vienna, Austria	Endothelial and Systemic Upregulation of miR-34a-5p Fine-tunes Senescence in Progeria
12:00-1:00	LUNCH	

Day 3 Afternoon Session**1:00**
New Mechanisms of Disease in Progeria
Moderator: Karima Djabali
Technical University of Munich (TUM) School of Medicine, Munich, Germany

1:00-1:20	Chiara Lanzuolo* Institute of Biomedical Technologies (ITB)-CNR, Istituto Nazionale di Genetica Molecolare, Milan, Italy	Monitoring genome structure in Hutchinson Gilford Progeria Syndrome progression
1:20-1:40	Valerio Orlando King Abdullah University of Science and Technology, Kingdom of Saudi Arabia	Repeatome RNA: From Junk to Therapeutics for Premature Aging and Tissue Regeneration
1:40--2:00	Elsa Logarinho* Instituto de Biologia Molecular e Celular (IBMC), Porto, Portugal	Small-Molecule Correction of Microtubule- Dependent Genotoxic Stress in HGPS Cells Bypasses Progerin-Driven Cellular Phenotypes
2:00-2:20	Karima Djabali*	Modulation of JAK-STAT Signaling in Hutchinson-Gilford Progeria Syndrome

2:20-3:00
Break and Prep for Summation Session

3:00-3:55	Mark Kieran Day One Biopharmaceuticals, Brisbane, CA Kan Cao University of Maryland, College Park, MD	Summation of Meeting and Key Findings
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*Current Progeria Research Foundation Grantee

Thank you for coming!

The 2022 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*

Judy Campisi, PhD – *Professor, The Buck Institute; Senior Scientist, Lawrence Berkeley National Laboratory*

Vicente Andrés, PhD – *Professor, Laboratory of Molecular and Genetic Cardiovascular Pathophysiology, and Director Department of Basic Research, Centro Nacional de Investigaciones Cardiovasculares Carlos III (CNIC)*

Mark Kieran, MD, PhD – *VP Clinical Development, Day One Biopharmaceuticals, a company dedicated to developing novel treatments for children*

Justine Maestranzi, BA – *Meeting Coordinator, The Progeria Research Foundation*

Joanne Morris – *Workshop Administrator, Joanne Morris Events*

