



The Progeria Research Foundation 10th Anniversary Workshop on Progeria
"From Bench to Bedside in a Decade"

April 11-13, 2010 • Seaport Hotel and World Trade Center • Boston, MA

DRAFT AGENDA

*The workshop begins at 5 pm Sunday, April 11
and ends at 4 pm Tuesday, April 13.*

DAY 1 – Sunday evening, April 11

5:00 - 8:00 pm
Dinner Included

Introduction and Greetings

Leslie Gordon and Frank Rothman - Conference Organizers
Audrey Gordon - PRF Executive Director

Plenary Session

State of the Field: Stunning Insights from a Rare Disease

Francis Collins – NIH Director

Living with Progeria – a Patient Perspective

Round Table Q & A with Children and Parents living with Progeria
Moderator: *Amy Dockser Marcus*, Pulitzer Prize-winning science writer for
The Wall Street Journal

DAY 2 Morning session:– Monday, April 12

Clinical Trials for Progeria

Moderator: *Leslie Gordon*

Design for Phase II Progeria Clinical Trials — FTI-only Trial and Triple Treatment Trial

Leslie Gordon – PRF Medical Director, Brown University and Harvard University

Design for Phase II Progeria Clinical Trials — FTI-only Trial and Triple Treatment Trial

Mark Kieran - Harvard University and Dana Farber Cancer Institute

Vascular Abnormalities in Progeria and Effect of FTI Treatment on Disease

Marie Gerhard-Herman - Harvard University and Brigham and Women's Hospital

Growth, Bone Health, and Endocrine Aspects of Progeria and Effect of FTI Treatment on Disease

Catherine Gordon - Boston Children's Hospital

Phase II Treatment Trial for Progeria and Laminopathies with Pravastatin and Zoledronic Acid

Nicolas Lévy - University of Marseilles

Natural History of Neurovascular Disease

Nicolle Ullrich – Boston Children's Hospital and Harvard Medical School

LUNCH PRESENTATION:

Progeria and PRF as a Case Study for Translational Medicine

Leslie Gordon

DAY 2 Afternoon session – Monday, April 12

Aging, Cardiovascular Disease, and Progeria

Moderator: *George Martin*

Some Unanswered Questions Regarding the Pathogenesis of the Vascular Pathology Associated with Normative Aging and Progeria

George Martin - University of Washington

Peripheral Vascular Disease in Progeria — From Mouse Model to Human Disease

Elizabeth Nabel - Brigham and Women's Hospital

Endothelial Cell Influence on Disease in Progeria and Normal Aging

Michael Gimbrone - Brigham and Women's Hospital

Extracellular Matrix Integrity in Vasculature and Other Tissues of Progeria Mouse Models and Human Pathological Specimens

Thomas Wight - University of Washington

Human Gene Editing with Engineered Zinc Finger Nucleases

Fyodor Urnov - Sangamo Biosciences

Discoveries on HGPS in the Context of Aging

Judith Campisi – Buck Institute for Age Research

DAY 2 Evening session – Monday, April 12
Poster Session and Open Evening Meeting Time

DAY 3 Morning session – Tuesday, April 13
Lamins – Biochemistry and Pathophysiology
Moderator: Robert Goldman

Nuclear lamins: Essential Building Blocks of Nuclear Architecture
Robert Goldman - Northwestern University

Structure and Assembly of Wild-Type and Disease Variants of Human Lamins A/C
Ueli Aebi - University of Basel

Quantification of Progerin Binding to Membranes
Kris Dahl - Carnegie Mellon University

Cell Cycling Effects of Progerin Expression
Maria Eriksson - Karolinska Institute

Drug and Genetic Manipulations of Prenylation and Methyl Esterification in *C. Elegans*
Yosef Gruenbaum – Hebrew University of Jerusalem

Day 3 Afternoon session* – Tuesday, April 13
Cutting Edge Strategies for Research and Discovery
Moderator: Thomas Misteli

Future Treatment Tools and Strategies
Tom Misteli - National Cancer Institute, NIH

Stem Cells and iPS Cells in Progeria and Laminopathies
William Stanford - University of Toronto

Presentation by Poster Competition Awardees

Closing Comments and Wrap-Up
Meeting Organizers and Advisory Board Members

****Final session ends at 4:00 pm***