

2013 Progeria Research Foundation's 7th International Scientific Workshop



Hand in Hand
Basic & Clinical Science
Working Together
Toward the Cure
 April 24-26, 2013 • Bethesda, MD

A record **180 Progeria clinicians and researchers** gathered from **18 countries** to set the stage for the next round of progress in translating bench research into treatment. **39 talks** and **56 poster presentations** inspired future collaborations and disseminated important scientific findings. Many of the hot topics discussed during the workshop had both a basic and translatable clinical component. *This exchange between basic and clinical investigators will be crucial on the road to success, when we not only cure children with this fatal disease, but also translate discoveries in Progeria to our understanding of heart disease and aging.*

The Meeting Agenda at a Glance

- ✓ Living with Progeria – Patient Perspectives
- ✓ Presentation by Francis S. Collins, NIH Director
- ✓ Current Treatment Trial Discoveries: US & French Teams
- ✓ What Progeria Can Teach Us About Cardiovascular Disease and Aging
- ✓ Lightning Young Investigator Panel Presentations
- ✓ Boston Children's Hospital Clinical Presentations
- ✓ The Biochemistry and Pathophysiology of Lamins
- ✓ The Next Phase: Drug Development, Genetic Therapy

The stage was set during the first evening with a family panel entitled **Living with Progeria – Patient Perspectives**, moderated by Academy and Emmy Award-winning documentary filmmakers **Andrea Nix Fine** and **Sean Fine**, directors of *Life According to Sam* (see page 10). Researchers had a unique chance to meet some of the people their work could help: **Meghan Waldron** and her parents,



Tina and **Bill**; **Devin Scullion**, along with his mom **Jamie**; and **Megan Nighbor**, with her parents **Sandy** and **Steve**. They talked to a captivated audience about what it's like to live with Progeria, and thanked the researchers for the work they're doing to help find a cure.

Day two kicked off with a session entitled **Current Treatment Trial Discoveries**. Clinical treatments with the farnesyltransferase inhibitor lonafarnib, pravastatin and bisphosphonate were discussed with regard to their potential impact on Progeria and aging. **Nicolas Lévy** (University of Marseilles) presented new findings from a Progeria clinical trial of pravastatin and zoledronate. Discoveries on the clinical implications of calcium dysfunction in Progeria and aging were presented by **Catherine Gordon** (Brown Univ., Harvard) and **Catherine Shanahan** (King's College London), while cardiologist **Marie Gerhard-Herman** (Brigham & Women's Hospital, Harvard) and neurologists **Michelle Silvera** and **Nicole Ullrich** (Boston Children's Hospital, Harvard) gave new insights into cardiovascular disease and stroke in Progeria and aging. Finally, **Mark Kieran** (Dana Farber Cancer Institute, Harvard) guided a discussion on Challenges and Strategies for Clinical Trial Design in Progeria.

PRF added a new twist to this year's meeting, incorporating two **lightning panel sessions** intended to maximize cross-talk between basic and clinical researchers.

Panel 1 was packed with PRF-funded scientists who provided cutting edge findings and their implications for a better understanding of the biology of disease in Progeria, CVD and aging. **Kris Dahl** (Carnegie Mellon Univ.), **Jason Lieb** (Univ. of North Carolina), **Kan Cao** (Univ. of Maryland), **Yue Zou** (E.Tennessee State Univ.), **Tom Glover** (Univ. Michigan), **Jan Lammerding** (Cornell Univ.), **Karima Djabali** (Technical Univ. Munich), **Thomas Dechat** (Medical Univ. of Vienna), **Maria Eriksson** (Karolinska Inst.) and **Samuel Benchimol** (York Univ.) displayed new work ranging from stem cell studies, to cell treatment with rapamycin, to genome instability and cellular aging in Progeria.



Dr. Lévy



Dr. Cao

In **Panel 2**, physicians from the Boston Children's Hospital clinical trial team representing every area of clinical disease in Progeria shared their latest insights. **Monica Kleinman** (Critical Care), **Leslie Smoot** (Cardiology), **Ashwin Prakash** (Cardiac MRI), **Marilyn Liang** (Dermatology), **Guangwei Zhou** (Audiology), **Brian Snyder** and **Ara Nazarian** (Orthopedics), and **Jessica Spratt** and **Annette Corriea** (Physical and Occupational Therapy) are finding brand new ways to measure treatment effect through a deep understanding of how Progeria affects different body systems in the children.



Dr. Prakash

The final day of talks left no stone unturned. National Institutes of Health Director **Francis Collins** started the day with insights into Progeria's overlaps with generalized aging, followed by perspectives on the overlaps with atherosclerosis of aging and Progeria from the basic perspective, **Vicente Andrés** (CNIC, Madrid) and a pathologist's perspective, **Richard Mitchell**, (Brigham & Women's Hospital).



Dr. Collins presents "Progeria as a Model of What's Right with Medical Research"

In a session on the **Biochemistry and Pathophysiology of Lamins**, **Robert Goldman** (Northwestern Univ.) detailed studies on the importance of B-type lamins on disease, **Colin Stewart** (Inst. of Medical Biol., Singapore) presented studies on induced pluripotent stem cells, and **Kan Cao** (Univ. Maryland) showed cellular effects of the drugs everolimus and rapamycin.

The afternoon session delved into **The Next Phase for Treating Progeria**. **Tom Misteli** (National Cancer Inst.), **Jeff Chamberlain** (U. Washington), and **Carlos López-Otín** (Univ. of Oviedo, Spain) discussed genetic treatment strategies and their potential for Progeria. In a fitting end to the meeting, **Brian Kennedy**, (Buck Institute), **Rafael de Cabo**, (National Inst. On Aging) and **Monica Kleinman** spearheaded a **Future Treatments Panel**, where they debated approaches to treatments with resveratrol and everolimus for children with Progeria.



Drs. Kennedy and de Cabo



Congratulations to this year's Poster Winners! **Ricardo Villa-Bellosta** from Centro Nacional de Investigaciones Cardio-vasculares in Madrid won best basic science

poster for studies on vascular calcification in HGPS; clinical poster winners were Boston Children's **Isabella Chase** for Dental and Craniofacial studies, and **Nicole Quinn** for her study on Energy Intake, Energy Expenditure and Body Composition in HGPS.

Overwhelmingly Positive Feedback!

With a 100% approval rating overall, the most common remarks from attendees reflected excitement about data sharing and collaboration leading to new ideas for future work. Here are a few attendees' comments:

"These workshops aid in moving the field of Progeria research along at a faster pace as a result of the collaboration, networking, sharing of new ideas and personal interaction."

"I gained a greater understanding of the disease process and what/how we are trying to treat/cure the condition, and how these impact research into normal aging."

"This meeting has given me new ideas and access to new resources by building contacts. I plan to start a new research project on Progeria."

"The greatest strengths of this workshop were the cross discipline and from bench to bedside review of what we've learned, what is going on and what the future brings - all very exciting!"

Many thanks to our Workshop Organizers...

Leslie B. Gordon, MD, PhD, Medical Director, The Progeria Research Foundation

Frank G. Rothman, PhD, Professor and Provost Emeritus at Brown University.

Carlos Lopez-Otin, PhD, Professor of Biochemistry and Molecular Biology, Medical School of Oviedo University, Oviedo, Spain.

Tom Misteli, PhD, Director of the Cell Biology of Genomes Group at the National Cancer Institute, NIH.

...and our Supporters



BROWN
 Alpert Medical School

Thank you to Brown University for providing Continuing Medical Education credits.



National Center
 for Advancing
 Translational Sciences



National Institute
 on Aging ■ ♦ ★ ✱

The Max and Victoria Dreyfus Foundation



"Every year the number and quality of publications in the field grows exponentially. It is the people sitting in this room that are pushing this field forward, making all the difference in finding treatments and cure."

— Opening remarks by PRF Medical Director Dr. Leslie Gordon