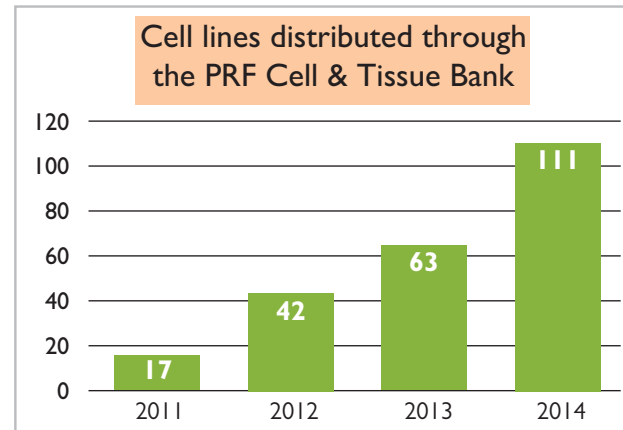


PRF 2014 Annual Report More growth, more progress, more planned to get us to the cure!

PRF's programs are thriving and expand annually with increasing numbers of children identified, researchers involved, and awareness raised. All of PRF's activities work cohesively to provide the core structure essential not only to drive Progeria awareness and research forward, but also to discover what Progeria can tell us about heart disease and aging.

From 2013 to 2014, our program growth continued at a remarkable pace. This includes a 12% increase in the number of known living children and a 76% increase in cell lines distributed to researchers – a testament to PRF's global awareness efforts and the ever-growing interest in Progeria research.

For more details on PRF's programs and services, check out **PRF By The Numbers** at progeriaresearch.org/prf-by-the-numbers and an article published in *Expert Opinion* authored by Executive Director Audrey Gordon and Medical Director Leslie Gordon, wherein the two PRF leaders discuss PRF's history, goals and accomplishments, and how PRF's programs have been pivotal in the journey from obscurity to treatment. progeriaresearch.org/whats_new_in_progeria_research



In recent years, there has been an exponential increase in the number of cell lines distributed to research teams: 553% from 2011 through 2014!

2014 Highlights:

Longer lives for children with Progeria: PRF, Boston Children's Hospital and Brown University teamed up on a major study of lifespan in Progeria. The May 2014 study, published in the prestigious American Heart Association journal *Circulation*, estimated that lonafarnib – the drug tested in the PRF-funded Progeria clinical trials – increased lifespan by at least 1.6 years, over the 6-year testing period. Time will tell if the life extension is longer than 1.6 years; The longest time any of the children were on drug was 6 years, so the researchers will re-evaluate after children are on the drug for a longer period of time. This is the first evidence of treatment influencing survival for this fatal disease – an historic finding in our quest to improve health and extend the lives of children with Progeria.

*Gordon, LB et al., Impact of farnesyltransferase inhibitors on survival in Hutchinson-Gilford progeria syndrome, *Circulation*, 2014, 130 (27-34)

The extension and expansion of the lonafarnib-only trial continued in 2014, with record numbers of children arriving at Boston Children's Hospital throughout the year: 46 patients from 23 countries speaking 16 languages! As of 12/31/14, a total of 63 children had participated in the trial, 18 of whom are new and taking lonafarnib for the first time. With the historic discovery that lonafarnib improves some disease features, including the cardiovascular system and longer estimated lifespan, PRF is making it possible for children to have access to lonafarnib while we continue to search for even more effective treatments and ultimately the cure.

"Flying out to Boston from Mumbai! Can't thank enough my dearest friends, beloved family, and all from PRF for making this possible!"

– December 2014 Facebook post from Nihal's father, on route to his son's first trial visit. All trial expenses are covered by PRF, including travel, lodging, food, interpreters and testing.

Night of Wonder 2014 sets a new bar With a record, sold-out crowd of nearly 500 people, **Night of Wonder 2014 – Lights, Camera, CURE!** was a special evening as we celebrated the success of HBO's documentary *Life According to Sam*, and the life of Sam Berns, the remarkable boy who captured the hearts of people around the world with his extraordinary personality and inspirational view of life. The event raised \$500,000, which is being used to fund the clinical trial expansion and PRF's other research-related programs.

Many thanks to all of you for making such tremendous advancements possible. With your steadfast support, we will work to ensure that 2015 and beyond brings us ever-closer to curing children with Progeria. Together, we WILL find the cure!



We're a 4-Star Charity!



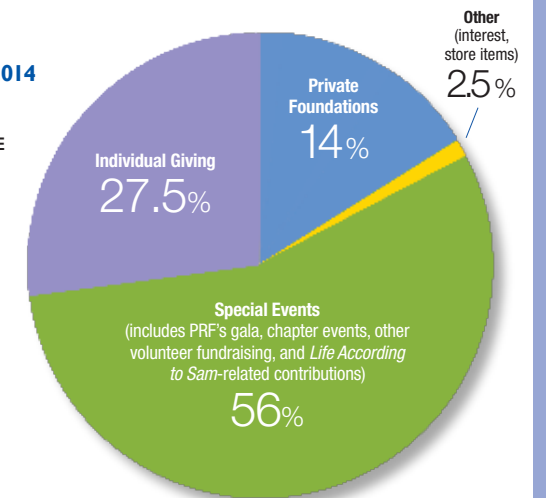
(Left to right) Megan and Meghan, both 14 years old, were the first to enroll in the lonafarnib trial in June 2007. Their most recent trial visit was November 2014.

10 Core PRF Activities That Support Our Mission

2014 ACTIVITY	PRF PROGRAM	CUMULATIVE ACTIVITY Through 12/31/14
15 children from 12 countries	International Patient Registry	208 children from 51 countries and all continents
4 children diagnosed from Brazil, Honduras, India & South Korea	Diagnostic Testing Program	115 children diagnosed
111 cell lines sent to 27 teams in 12 countries	Cell & Tissue Bank	194 cell lines from 197 donors; cells sent to 80 teams in 17 countries
6 children enrolled; 20 treatment guidelines sent to 8 countries	Medical & Research Database	132 enrolled; 466 treatment guidelines sent to 39 countries
Funded Triple Treatment Trial Expansion (Enrollment ongoing)	Clinical Trial Funding & Co-ordination	3 clinical trials funded since 2007
5 grants awarded	Basic Research Grant Funding	55 grants to 41 labs in 12 countries; funding total >\$6.1 million
Initial planning for 2015 & 2016 meetings begins	International Scientific Meetings	11 meetings: 7 general workshops and 4 subspecialties
50 events & over 250 new volunteers	Volunteer Program	Nearly 4,250 volunteers worldwide have helped raise awareness and funds
2 languages added: Indonesian & Swedish	Translations Program	28 languages
15 new children identified and 309 million people reached through media	Public Awareness & Find The Other 150 Campaign	125 living children from 39 countries; over 1.96 billion* people exposed to Progeria and PRF's work *figure based on media impressions

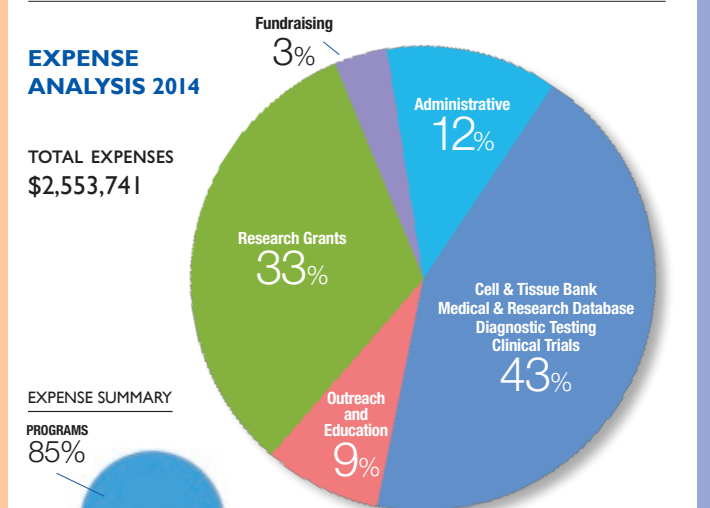
INCOME ANALYSIS 2014

TOTAL REVENUE \$3,004,264

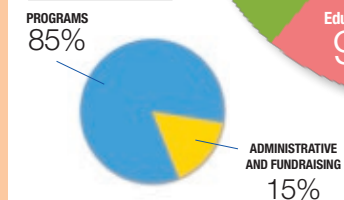


EXPENSE ANALYSIS 2014

TOTAL EXPENSES \$2,553,741



EXPENSE SUMMARY



12/31/14 Net Assets: 4,482,530*
*Much of these funds are designated for upcoming clinical trials and drug development costs