NIH Director Francis Collins Returns as Speaker and Musical Guest at GFPD Scientific Conference

The Director of the National Institutes of Health, (NIH) will deliver an address at the Closing Dinner of 2018 Scientific Conference of The Global Foundation for Peroxisomal Disorders (GFPD) as well as share the gift of hope through music. This unique gathering of over 40 of the world's leading rare disease researchers, scientists and specialists as well as family participants at the GFPD Regional Family Meetup aims to facilitate collaborative research and accelerate treatments for patients suffering from peroxisomal disorders, a group of rare, genetic conditions that are fatal in childhood.

The GFPD announces the return of Dr. Francis Collins, Director of the NIH, as Closing Dinner Speaker and Musical Guest at the 2018 GFPD Scientific Conference June 2nd in Washington D.C. His address, "Rare Disease Research: Reasons for Hope," will highlight the progress, research and treatments still needed for peroxisomal disorders which are rare, genetic, multi-system diseases that are usually terminal in early childhood. Additionally, Dr. Collins, an accomplished guitarist, will perform several songs for the audience focusing on a message of hope.

"We are delighted to again prominently feature longtime friend of the GFPD, Dr. Collins, at our Scientific Conference and Regional Meetup this year," said Melissa Bryce Gamble, GFPD Executive Director and Co-Founder. "Dr. Collins brings a wealth of knowledge and experience with rare diseases, as well as stories of hope from other rare disease organizations. We know his presentation and musical performance will be meaningful for families and researchers alike."

Over forty of the world's leading rare disease researchers and scientists will focus on the development of therapies for peroxisomal disorders which cause a myriad of health challenges including adrenal insufficiency, neurodegeneration and demyelination of the brain, seizures, deafness, blindness, osteoporosis, failure to thrive and low muscle tone. Peroxisomal diseases are some of the over 7000 rare diseases that affect 30 million Americans. "Approximately 175 children are currently living worldwide with this disorder," said Gamble. "Research is grossly underfunded for our disease and thousands of other rare diseases as well."

"We're fortunate to be welcoming speakers from different scientific backgrounds who have successfully developed treatment in some area related to the disease," said Ms. Gamble "Having all these brilliant scientific minds together will foster collaboration, something crucial in all scientific endeavors, but even more so when dealing with rare diseases, like peroxisomal disorders."

To extend the GFPD'S influence and reach, the organization has partnered with the National Center for Advancing Translational Sciences (NCATS), as one of only nine groups in the country to have such a partnership. The collaboration with NCATS utilizes a "bench to bedside" approach which has helped accelerate the development of treatments for rare diseases like peroxisomal disorders. Dr. Di Wu, post-doctoral fellow at NCATS working on peroxisomal disorder drug screening projects, will also speak at the Closing Dinner, alongside Dr. Collins.

"This is a time for families —who don't see another human being dealing with similar issues any other time of the year—to come together," Gamble said. "Not only are we connecting families with each other, but we are also connecting scientists and physicians with patients, helping them put a face behind the disease they are studying."

"Connecting people is the power of this conference," she said.

To learn more about the Global Foundation for Peroxisomal Disorders visit: www.thegfpd.org

Interviews: Melissa Bryce Gamble, Executive Director and Co-Founder of the GFPD, Dr. Francis Collins, Director of the NIH, and Joseph Hacia, GFPD Medical and Scientific Advisor are available for interviews June 2nd, 2018 from 6:00 - 9:00 p.m. at Maggiano's Chevy Chase Pavilion 5333 Wisconsin Ave. N.W., Washington, D.C. 20015

About the Global Foundation for Peroxisomal Disorders
The mission of the GFPD is to fund and promote groundbreaking peroxisome disorder research and to assist families and professionals through educational programs and support services related to Zellweger spectrum disorders and the related single enzyme peroxisomal disorders. Visit www.thegfpd.org for more information.

For more information, please contact Ashlee Cabrera, Director of Development and Communications at ashlee@thegfpd.org or call 347-470-4373.
For more information online, please visit thegfpd.org