IMPACT REPORT 2019

Dear Friends,

As 2019 comes to a close, I'm proud to say we have scaled our organization to meet the needs of our family and scientific community. Our hallmark program initiative, the 2019 Family and Scientific Conference, was hugely successful with nearly 400 attendees. This incredible 5-day event would not have been possible without nearly 50 volunteers, numerous sponsors, and a committee laser-focused on providing meaningful conference sessions. In the past year, we have also improved our grant-making process under the direction of our Scientific Initiatives Committee. Through collaboration with our partners, The Wynne Mateffy Research Foundation and the Peter Hopkins Foundation, we have awarded a combined $462,000 since the beginning of 2018, with more grants slated to be awarded before the end of 2019. Our committees are the backbone of our foundation, and robust committee membership leverages our limited financial resources for enormous impact.

Our impact as an organization is only as great as the diversity of our community and there are many ways to serve. If you are interested in becoming involved in the work of the GFPD, as a volunteer or a committee member, please review the organizational chart on page 7 and contact me. What we have been able to accomplish in the last nine years has been a result of collaboration and teamwork. I invite you to join us and help keep our incredible momentum going.

With gratitude,

Melissa Bryce Gamble

Melissa Bryce Gamble
Mother of Ginny, Co-Founder and Executive Director

#PauseforPBD is Saturday, October 5!

An easy way to get involved is to join us in spreading awareness and raising hope for families facing peroxisomal disorders.

Use the back page of this report to write in why you're pausing for PBD and share on social media. Be sure to tag @TheGFPD so we can spread awareness to the world!

GFPD
GLOBAL FOUNDATION • for • PEROXISOMAL DISORDERS

Your donation funds research, champions scientific collaboration, and supports families facing peroxisomal disorders around the world.

See more GFPD journeys:

thegfpd.org

Webinars

In late 2018, the GFPD secured an online meeting system, providing increased access to technology to members of our community around the world.

Through the use of this platform, the GFPD hosted webinars and virtual roundtables during Q4 of 2018 and during 2019. Topics included self-care for caregivers, bone health in peroxisomal disorders, state and federal advocacy, navigating transition from pediatric to adult services, and educational advocacy.

Bereavement

Our bereavement support program is a unique component that sets us apart from other rare disease organizations.

In addition to hosting bereavement sessions at our Family and Scientific Conference, we send a wind chime and bereavement resources to family members after the death of an individual with a peroxisomal disorder. This fall, a small cohort of GFPD Families will utilize our online meeting system and complete the Sensing Connections bereavement curriculum developed by Molly Black of the Pennsylvania Deaf-Blind Project *(pictured left)* and Megan Cote of the National Center on Deaf-Blindness *(pictured right)*. Three GFPD families will be trained as facilitators for Sensing Connections, allowing us to offer this curriculum on an ongoing basis.

Support Groups

We now facilitate three support groups on Facebook: our Parent Support Group, Extended Family Support Group, and new this year, The GFPD Adult Self Advocate Patient (GFPD ASAP) Support Group. Due to increased genetic testing, we have had a huge influx of adult patients with a very mild phenotype.

The GFPD ASAP Group fills an unmet need for connection, sharing of experiences, and patient to patient support. Additionally, the GFPD has continued our monthly small group meetings via our online meeting system in collaboration with the Family-to-Family Communities project through the National Center on Deaf-Blindness, the National Family Association for Deaf-Blind and the State Deaf-Blind Project.

Family-to-Family Connections

As our patient and family community has grown, so has the need for direct support for families.

By providing access to trained family support facilitators, we meet families where they are to answer questions, listen to concerns, and educate about resources offered by the GFPD and other organizations. Families receive support through various modes of communication including phone, text, and email, with our facilitators averaging 45 instances of direct support each month.

State & Federal Advocacy

Upon the recommendation of NIH Director, Francis Collins, the GFPD expanded our advocacy initiatives this year to bring greater attention to the need for consistent funding for the NIH and FDA and expanded newborn screening (including screening for x-linked Adrenoleukodystrophy which also identifies babies born with a peroxisomal disorder).

We provided training to families to help them share their stories and shine a light on the needs of the rare disease community, and we took a team of seven advocates to Washington, D.C. to participate in Rare Disease Day on Capitol Hill in February.

$0 Projects

Since 2010, the GFPD has promoted and championed scientific collaboration through $0 research initiatives with a combined value of nearly $800,000.

Projects include mouse model development at Jackson Laboratories and rat model development at the University of Southern California. Patient and caregiver studies through the National Alliance on Caregiving, McGill University, Montclair State University, and the Sterol and Isoprenoid Research Consortium helped elucidate previously unknown symptoms of peroxisomal disorders and better understand the caregiver experience.

Grants + NCATS

In 2018 and 2019, the GFPD and our research partners, The Wynne Mateffy Research Foundation and the Peter Hopkins Foundation, supported research projects aimed at getting treatments from the laboratory bench to the patient's bedside as quickly as possible.

Funded research projects included: the development of a metabolomics resource, as well as a multi-year project to develop research resources for neurodegeneration and vision loss in peroxisomal disorders. We also continued to push ahead drug testing through the use of mouse models, as well as a high-throughput drug screening and assay development project at the National Institutes of Health/ National Center for Advancing Translational Sciences. Additionally, we funded two gene therapy projects and a project studying the correlation between mitochondria and peroxisomes.

***To find out more about our family and scientific initiatives, visit* thegfpd.org**

GFPD 2019 FAMILY & SCIENTIFIC CONFERENCE

Nearly 400 family members, patients, scientific researchers, physicians and professionals met in Washington, D.C. July 10th – 15th to share knowledge, experiences and to work together with the goal of pushing ahead meaningful treatments for individuals with peroxisomal disorders. This year, the GFPD offered sessions for caregivers, extended family members, siblings, professionals caring for individuals with peroxisomal disorders, as well as the scientific research community.

"It was encouraging because meeting all the other families going through the same thing has shown me that we can do it. Feeding tube? We got this! Seizures? We got this! And we have the whole GFPD family to help along the way."

- Natalie Clouse, mom of Gwendolyn
(pictured center with Jennifer Knox, mom of Charleston- left and Katina Rees, mom of Keely - right)

Scientists, Professionals and Families all come together

Feedback from our community highlighted a need for conference sessions targeted specifically to local patient medical and therapy teams, so the 2019 Family and Scientific Conference included sessions on Deaf-Blindness, physical, occupational, and speech therapy, and foundational information for new physicians and professionals about peroxisomal disorders (also known as Peroxisomal Disorders 101).

During the Scientific Advisory Board Panel session, caregivers shared their experiences with disease progression and symptom management. The message to parents from the Scientific Advisory Board to a packed conference room was unified: "YOU, the parents, are the experts. You're teaching us every time we meet. You help advance the field. Our research is based on what you observe in your children."

Individual family consultations at the 2019 Family and Scientific conference were a unique opportunity for members of our Scientific Advisory Board to interact one-on-one with patients and families in small groups. Families were grateful for the access to physicians and researchers that have expertise in peroxisomal disorders and were able to take back information to their local medical team.

The Meyers family meets with members of the Scientific Advisory Board to discuss treatment options for their son, Brian.

Sensory Experiences for All

From ball pits to tech devices to musical entertainment, the 2019 Family & Scientific Conference was full of opportunities for kids and adults to engage with the world using a wide range of senses.

In the childcare room, volunteers played with PBD Warriors and their siblings using a variety of toys that stimulated their senses. While the kids were having fun, the parents were able to try out communication devices for individuals with communication disorders. Attendees of all ages enjoyed interactive entertainment by Only Make Believe.

One of the unifying exercises that medical researchers, therapy professionals, and family members alike, were all impacted by an exercise to experience the world as an individual with vision and hearing impairments. During a demonstration by Krista Olsen of the South Carolina Interagency Deaf-Blind Project, Nancy Steele, an Educational Consultant, and Donna Carpenter of KY Deaf-Blind Project, participants donned special goggles that limited or obstructed their vision completely and wore ear plugs to muffle sounds. The older siblings then assisted in repeating the experiment for the younger PBD Warrior siblings so that everyone at the conference was able to experience the world as a PBD Warrior with vision and hearing impairment.

GFPD / By the numbers

39
COUNTRIES
Represented on patient registry

Patients on the GFPD Patient Registry

2,715 **Friends and Supporters on Facebook *(and growing!)***

557 **Members of GFPD Parent Support Group on Facebook**

21
Research collaborators/institutions spread throughout the world

2019 Family & Scientific Conference

NEARLY **400 TOTAL ATTENDEES**

35 ATTENDEES WITH PEROXISOMAL DISORDERS

Each year, the GFPD highlights a family's journey throughout the year to share their challenges and victories. In 2019, Archer's family has shown us how many voices help shape the decision making about treatments for peroxisomal disorders. From the Scientific Advisory Board to the Parent Support Group, everyone's knowledge and experience are needed when treating a rare disease. After many discussions with the GFPD community, Archer's family decided to move forward a feeding tube for Archer and has had great success! Your support gives continued funding for these programs so that families can find the answers they need to make difficult medical decisions.

"Peroxisomal disorders are complicated and unpredictable, but Archer teaches us to live in the moment and choose joy each day."

- Archer's mom, Ashley

REVENUE IN FISCAL YEAR 2018

Grants and Contributions
$157,659

Fundraising Events
$364,014

Other Income
$11,409

2018 Total Revenue
$533,082

SPENDING IN FISCAL YEAR 2018

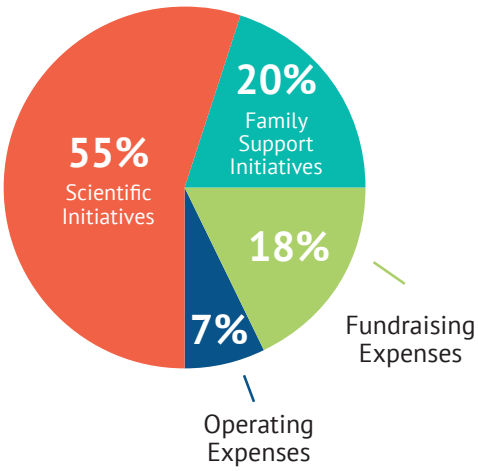
Scientific Initiatives:
includes research grants and the 2018 Scientific Conference
$296,528

Family Support Initiatives:
includes 2018 Regional meetups, webinars, bereavement gifts and equipment exchange
$103,258

Fundraising
$97,129

Operating Expenses
$37,897

Total Functional Expenses
Fiscal Year 2017
$534,813



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COMMITTEES OF THE GFPD

Community Advisory Council
Conference Planning Committee
Development Committee
Finance Committee
Governance Committee
Scientific Advisory Board
Scientific Initiatives Committee

*To get involved and join a GFPD Committee, please contact Melissa Bryce Gamble at **melissa@thegfpd.org***

SATURDAY, OCTOBER 5, 2019

#PAUSEFORPBD

I PAUSE FOR:

GFPD

GLOBAL FOUNDATION • for • PEROXISOMAL DISORDERS

FAMILY • RESEARCH • HOPE