GFPD 2023 Vision- Draft for approval by the GFPD Board 4/20/2020

Description- The GFPD is a global leader as a patient advocacy group and as a grassroots issue-driven nonprofit public charity. In the 13 years since our incorporation, the foundation has improved the lives of individuals with peroxisomal disorders (and their families and caregivers) by funding research, championing scientific collaboration, and empowering families and professionals through educational programs and support services. We are also a model for other rare disease patient advocacy groups in best practices for searching for emerging treatments and engaging in results driven advocacy.

Financial- Our financial planning over the past 3 years has led to a sustainable process to continue to fund our family and research initiatives, without being overly reliant on our families to host local fundraisers. We have utilized a grant writing contactor who secures 10-20% of our annual budget in grant funding to maintain operations. We have initiated a $1,000,000 endowment campaign to annually fund research grants without dipping into operational funds. We have established a telemedicine program to provide our families with consistent access to specialists with a peroxisomal disorder or rare disease specialty, which also provides sustainable funding for the organization.

People/Board- The GFPD has continually recruited between 2 and 4 candidates annually (50% family members, 50% Tulsa community members) to fill the Board of Directors and has diversified our Board in accordance with our Board Matrix- specifically filling slots to address age, race, and political diversity. The Board has a sustainable succession plan each year so that the Board Chair and Chair Elect both serve 2 consecutive ONE-year terms. Our staffing model includes an Executive Director, an assistant Executive Director (with goal for current Executive Director to retire in 2024), CFO, Administrative Assistants, and contractors/staff devoted to Family, Research, and Advocacy programming initiatives. We have instituted a diversity committee to encourage diversity in our hiring process, our board recruitment, and in our family engagement and educational initiatives.

Family Support- With awareness of the difficulty to find a comprehensive CURE for all PEX mutations and mutations causing single enzyme disorders, the GFPD has focused on providing credible, accurate treatment and management of peroxisomal disorders to families as well as high impact culturally competent family and community engagement to build relationships and partnerships. Our resources include our Family Resource Guidebook, our website, monthly webinars/ virtual roundtables, direct caregiver support and referral to additional resources.

Research- The GFPD has instituted a Clinical Advisory Board to include subject matter experts (ENT, Vision, Gastroenterology, etc.) in addition to our Scientific Advisory Board. The GFPD manages a physician mentorship program to leverage and retain the decades of institutional knowledge that resides with our Scientific Advisory Board members with the goal to train the next generation of physicians and scientists. The GFPD annually awards research grants to promising young investigators focused on management or treatment of peroxisomal disorders. Additionally, we have completed regulatory and scientific requirements to begin treating a subset of patients with Diosmetin.

Advocacy- In 2019, only 14 states in the United States actively tested newborns for peroxisomal disorders. Through our collaboration with related patient advocacy groups, between 2020 and 2023, 10 new states have begun testing or are in the final stages of implementation. GFPD families have become excellent advocates both at the state and federal level for issues impacting families in our community including newborn screening initiatives and continued and consistent NIH/FDA/CDC funding.