The Parent Project for Muscular Dystrophy Research Inc

GuideStar Nonprofit Profile Charting Impact Report * Last Updated on 08.06.2014

This report represents The Parent Project for Muscular Dystrophy Research Inc's responses to Charting Impact, a joint project of BBB Wise Giving Alliance, GuideStar USA Inc, and Independent Sector. Charting Impact uses five simple yet powerful questions to encourage strategic thinking and help organizations share concise information about their plans and progress toward impact.

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Mission:
Parent Project Muscular Dystrophy's mission is to end Duchenne. We accelerate research, raise our voices in Washington, demand optimal care for all young men, and educate the global community.
The content of this Charting Impact Report is the sole product and responsibility of The Parent Project for Muscular Dystrophy Research Inc. This report does not in any way represent an endorsement from Independent Sector, BBB Wise Giving Alliance, or GuideStar, nor does it represent fulfillment of the BBB Wise Giving Alliance’s Standards for Charity Accountability. For more information on Charting Impact, visit www.guidestar.org/chartingimpact
1. What are we aiming to accomplish?

Research *To identify and aggressively fund the most promising near and long-term Duchenne research and cutting-edge therapies. *To stimulate new research to ensure that the therapeutic pipeline is rich with opportunity. *To encourage and support the pharmaceutical industry to maximize their investment in Duchenne. Advocacy *To ensure the patient’s voice is heard in Washington, DC, so policies lead to improvements in the lives of families affected by Duchenne and reflect the needs of the whole community. *To work with the National Institutes of Health and other agencies to ensure that Duchenne research and clinical trials remain a high priority. *To work with federal regulatory agencies so they address Duchenne-specific concerns in their decision-making. Care *To identify gaps in care for young men with Duchenne and work toward solutions. *To work with clinicians and other health care professionals across the globe to ensure all Duchenne patients have access to optimal care. Community *To provide a supportive environment in which people affected by Duchenne can share needs, concerns, and common experiences. *To work collaboratively with stakeholders who make up the Duchenne population and to participate actively and effectively in the international Duchenne community. Education *To increase recognition of muscle weakness among healthcare professionals and promote early diagnosis. *To share up-to-date information about treatment and care options with all members of the Duchenne community. *To raise awareness about Duchenne and provide educational materials to the global community.

2. What are our strategies for making this happen?

* Maintain funding and federal support of the Wellstone Centers of Excellence by continuing to show the effectiveness of these centers amongst the Duchenne community. * Lead and develop first of its kind patient initiated draft guidance on Duchenne for the FDA and Industry, ultimately to inform the FDA about the current landscape in Duchenne and to encourage them to write an official Duchenne guidance. * Decreasing the time and cost of clinical testing for new Duchenne therapeutics through support of clinical infrastructure. * Improving the chances that a drug entering clinical testing for Duchenne will result in an approved therapeutic. * Identify gaps in care and services for individuals and families living with Duchenne. * Identifying and addressing the current cardiac needs of people living with Duchenne. * Address the outstanding educational needs of patients and families. * Understand the perspectives and preferences of patients and families related to managing Duchenne.

3. What are our organization's capabilities for doing this?

* Passage of the Amendments to 2001 MD-CARE Act in the House, schedule to be voted on by Senate in Fall 2014 with PPMD advocates urging Senators to support with home visits during recess. * PPMD develops process and methodology for creating draft guidance, formalizes structure, (steering committee, working groups, professional writer, project manager) and creates timeline. Develops Community Advisory Board to oversee and participate in the process. * Development of biomarkers and new clinical endpoints for Duchenne muscular dystrophy. * Funding multiple therapeutic strategies to treat Duchenne through a unique and rigorous review process. * Creation of the Certified Duchenne Care Center Program to certify centers across the US capable of providing comprehensive care and services to individuals living with Duchenne; communicate this information to the Duchenne Community at large. * Proposed meeting organized in conjunction with NHLBI to address contemporary cardiac issues in Duchenne. * Conduct analysis of needs and gaps related to outstanding educational needs of patients and families. * Conduct research to evaluate perspectives and preferences of patients and families related to managing Duchenne.

4. How will we know if we're making progress?

* Continued support and momentum of Amendments in both Houses thanks to efforts of PPMD advocates. * Draft guidance
formally submitted to FDA in June 2014. FDA to review and respond by end of year. * Favorable comparison to average time and costs of drug development in other fields as appropriate. * First new drugs for Duchenne approved in next 2-3 years and combination drug therapies developed within next 5 years. * Continued community interest and enthusiasm for the certification program. Regular inquiries from centers curious about/interested in applying for certification. * Two day meeting organized by NHLBI, supported by PPMD, of cardiac thought leaders, to discuss gaps in Duchenne cardiac care and research. * Fewer outstanding educational needs of patients and families. * Ability to inform programmatic and advocacy decision making with the perspectives and preferences of patients and families related to managing Duchenne.

5. What have and haven't we accomplished so far?

* MD-CARE Act Amendments are approved by both Houses of Congress, and signed into law by the President. * FDA writes official draft guidance on Duchenne, accepting majority of content submitted by community. PPMD sets precedent for other rare diseases. * Number of trials of novel therapeutics for Duchenne has increased from 1 in 2004 to 16 in 2014. * PPMD-funded pilot study of a drug approved for another purpose led directly to a 300 person phase III clinical trial for Duchenne by the original drug developer. * Seven Certified Duchenne Care Centers to be named this year. * Small groups organized to address areas of need in cardiac care and research: development of cardiac care guidelines in Duchenne, standardization of operating procedures in animal research, organizing infrastructure that would further facilitate human research. * Evolve to responding to new, emerging and anticipated (within the next ~12m) educational needs of patients and families. * Perspectives and preferences of patients and families related to managing Duchenne become integrated into regulatory decision making.