Parent Project Muscular Dystrophy

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LEADING THE FIGHT TO END DUCHENNE

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Mission

Parent Project Muscular Dystrophy's (PPMD) mission is to end Duchenne muscular dystrophy. It works to do so by accelerating medical research, advocating in Washington, demanding equal and unsurpassed care for all young men living with Duchenne, and raising awareness in the global community.

Strategy/Approach

PPMD has five primary focus areas:

- **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 its investment in Duchenne.
- **Advocacy**
  - To ensure the patient's voice is heard in Washington, 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 (NIH) 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 healthcare 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.

Research Portfolio

PPMD invests in research that will impact every boy touched by Duchenne, regardless of age, stage of progression, or mutation. The organization's funding portfolio highlights new treatments and therapies at various stages of development.

Types of grants include:

- **End Duchenne Grant Award** – Program in partnership with NIH, provides bridge funding for promising translational research projects that receive scores beyond the current funding paylines of NIH.
  - *Up to $150,000 for one year*
- **Global Investment For Therapeutics to End Duchenne (GIFTED): Drug Development Corporate Grant** – Awarded to a biotechnology or pharmaceutical company developing a therapy that can be tested in and applied to Duchenne muscular dystrophy.
  - *Bridge Funds: $50,000 to $300,000*
  - *Pre-IND projects: $500,000 to $1 million*
  - *Phase I, II studies: $1 million to $1.5 million*
- **Cardiac Initiative:** Parent Project Muscular Dystrophy is starting a $2 million cardiac initiative to cover the bases from the identification of new drugs to better, more proactive cardiac care.
  - *Identifying New Cardiac Drugs: $250,000*
  - *Repurposing Existing Drugs to Treat Cardiomyopathy: $750,000*
- **PPMD Investigator Award** – For academic investigators doing translational research that is highly relevant to therapy development for Duchenne.
  - *Contingent on availability of funds for one to two years*
- **PPMD Meeting Support** – Provided to support meetings directly relevant to research, education, or services for Duchenne.
- **PPMD Exploratory Funds** – For investigators seeking to develop preliminary data for new ideas or studies that are highly relevant to Duchenne muscular dystrophy therapy development.
  - *Contingent on availability of funds for one year*

Partnership Practices

PPMD partners with academic institutions and investigators, policymakers, and industry to advance progress against its mission.

PPMD partners with biotech and pharmaceutical companies. Through the GIFTED Fund, PPMD invests in companies developing new therapies that can be applied to Duchenne. This program is designed to put better drug

candidates into clinical testing and to decrease failure rates. Examples include:

- **Dart Therapeutics**—A partnership between PPMD and Dart Therapeutics for the development of a set of clinical trial tools and sites to be utilized for any clinical study aiming to reduce cost and time of DMD clinical trials.
- **Summit plc**—A $250,000 grant for the manufacturing of clinical grade utrophin upregulator (SMT C1100) to enable the start of Phase I clinical trials sooner than aniticpated.

Financials

PPMD is a 501(c)(3) charitable organization. The most recent financial information available is from 2012.[1]

Year ending 12/31/2012:

- Revenue: $4,543,284
- Net Assets: $2,885,159
- Gifts received: $3,274,324
- Expenditures: $6,223,199

Key Accomplishments

- In October 2012, PPMD reported that Summit PLC has been able to achieve sufficient plasma levels of its reformulated utrophin-upregulating drug to justify a Phase I/II trial in Duchenne.
- PPMD has helped to advance seven compounds into preclinical and clinical development.
- PPMD's investment in Project Catalyst led to follow-on funding for PTC Therapeutics of $15.4 million from NIH and $3 million from the private sector to advance its research findings.
- PPMD convened a meeting with leaders of the Food & Drug Administration's drug review center to seek ways to accelerate the review process and to ensure the patient voice is included when key decisions are being made.
- Pat Furlong, the founding president and CEO, has been recognized as The New Yorker Magazine 2010 World Changer and WebMD Health Hero 2010, and received the 2008 Research!America Advocacy Award.
- Launched Decode Duchenne, an innovative genetic testing program for patients with Duchenne or Becker muscular dystrophy with support from DuchenneConnect Registry and Sarepta Therapeutics.
- Awarded nearly $1 million in funding from PCORI to be one of the 29 members of PCORnet

Leadership

- PPMD is led and advised by a **Board of Directors**, **Scientific Advisory Committee**, **Drug Development Advisory Committee**, and **TREAT-NMD's Advisory Committee on Therapeutics**.
- **Founding President and CEO**: Pat Furlong, Pat@parentprojectmd.org
- **Vice President, Research**: Sharon Hesterlee, PhD, Sharon@parentprojectmd.org
- **Chief Operating Officer**: Kimberly Galberaith, Kimberly@parentprojectmd.org
- **Vice President, Outreach and Education**: Holly Peay, MS, CGC, Holly@parentprojectmd.org

[1] Information obtained from PPMD Financials http://www.parentprojectmd.org/site/PageServer?pagename=About_financials July 2013