CureDuchenne

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Mission

CureDuchenne is a grassroots effort that has one purpose – to cure Duchenne muscular dystrophy (DMD). The organization aspires to save this generation of boys who have Duchenne. CureDuchenne will consider its mission achieved if it is out of business in the next 10 years. It was founded by the parents of a son with DMD.

Strategy/Approach

CureDuchenne focuses its efforts on bringing together leading scientists worldwide to help determine the most viable research projects, accelerate the clinical trial process, and bring potential life-saving drugs to help this generation of Duchenne boys. It works to identify research that has the most likelihood of making it to clinical trials and then provides the financial bridge that will take it from the lab and into human trials.

These strategies include:

- Gene delivery therapy aimed at reintroducing a functional recombinant version of the dystrophin gene
- Tricking the gene into producing dystrophin, such as modification of the dystrophin pre-mRNA, commonly referred to as exon skipping
- Read-through strategies for nonsense mutations
- Cell-based therapies
- Approaches that compensate for the lack of dystrophin, such as utrophin upregulation, myostation inhibition/HDAC inhibition, and IGF-1
- Pharmacologic solutions using FDA-approved products, either alone or in combination
- Anti-inflammatories/anti-oxidants

It also organizes regular fundraising and advocacy events, including galas, climbs, auctions, and local advocate outreach sessions to raise money for research and provide updates on scientific progress.

CureDuchenne maintains the eDystrophin database, containing 209 different non frame-shifting BMD mutations from 945 patients. The database is a resource for researchers, parents, and patients and contains information useful for designing new therapeutic strategies.

CureDuchenne launched CureDuchenne Ventures, a new initiative to identify and fund a pipeline of therapies to treat Duchenne. The goal of this program is to provide substantial funding to several projects over the next three years that will serve as a catalyst to drive investment from biotech and pharmaceutical companies and venture philanthropists.

Research Portfolio

CureDuchenne is funding several ongoing research projects, including clinical trials. Seven CureDuchenne-funded **research projects** have made their way into human clinical trials.

Research includes:

- Viral Vector Mediated Gene Therapy
- Adeno-Associated Virus Therapy
- RAAV Gene Transfer that has achieved clinical trial status
- Enhancing Gene Transfer and Reducing Immune Rejection
- Mediated Exon Skipping
- Development of Animal Models
- Read-Through Strategy for Nonsense Mutations
- Stem Cell Transplantation
- Compensatory Proteins
- Pharmacology
- Anti-Inflammatories/Anti-Oxidants Supplements

Partnership Practices

CureDuchenne partners with biotech and pharmaceutical companies through its **research projects**. It supports the research of companies such as PTC Therapeutics, AVI Biopharma, GlaxoSmithKline, and Summit Corporation. It was also the first U.S. organization to support Prosensa, an exon skipping biotech company that leveraged CureDuchenne's investment to secure an additional $650 million, which it is using to bring new drugs to market.

Financials

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

Year ending 12/31/2012:

- Revenue: $1,769,444
- Net Assets: $2,121,132
- Grants: $565, 457
- Gifts received: $1,345,852
- Expenditures: $1,988,523

Key Accomplishments

- Currently facilitating four Phase I, four Phase II, and two Phase III clinical trials.
- Seven of the research projects that it has supported are in human clinical trials.

[1] Information obtained from GuideStar, www.guidestar.org, March 2014

- Funding 15 research projects and helps run the Center for Duchenne Muscular Dystrophy at University of California, Los Angeles.
- In September 2010, it was selected as Cadillac's featured charity for Super Bowl XLV.
- Debra Miller, founder and CEO of CureDuchenne, participated in the Department of Defense Duchenne Muscular Dystrophy Research Program.

Leadership

CureDuchenne is primarily led by a **Board of Directors** and a **Scientific Board of Advisors**.

- **President and CEO**: Debra Miller, debra@cureduchenne.org
- **Chief Scientific Officer**: Michael Kelly, michael@cureduchenne.org
- **Senior Director of Development:** Drew Hoyer, drew@cureduchenne.org