Charley's Fund

635 Main Street, Suite 3
Great Barrington, MA 01230
(877) 436-3363
413.528.0400
www.charleysfund.org

Mission

Charley's Fund directs money into the hands of researchers who have the best shot at developing a treatment or cure for Duchenne muscular dystrophy (DMD). Its sole mission is to move scientific research from the laboratory into human clinical trials so that it can have a measurable impact on boys who suffer from DMD now.

Strategy/Approach

Historically, most of the funds raised for Duchenne have subsidized the tremendous cost of caring for children with the disease, not research to cure it. Several established organizations provide helpful services for families struggling to provide for their DMD-afflicted children. In contrast, Charley's Fund's sole mission is to fund a cure or treatment for Duchenne. Charley's Fund invests its resources in translational and clinical research.

Research Portfolio

Initially, Charley's Fund's focus was translational research – research that moves the science from the laboratory into human clinical trials. More recently, the organization began funding clinical research directly. This is a clear indication that hopeful therapies are moving through the drug development pipeline. Through innovative partnerships with biotechnology companies and clinicians, Charley's Fund is attempting to impact the speed of drug development for Duchenne muscular dystrophy.

The fund focuses its research funding on several therapeutic areas:

- Exon skipping
- Novel small molecules
- Biological
- FDA-approved drugs
- Convergent medical devices
- Stem cells
- Drug therapy

Supported by Charley's Fund, Pilot Trials Now is an innovative program that supports small clinical trials for FDA-approved drugs that show promise as treatment for DMD.

Partnership Practices

Charley's Fund partners with pharmaceutical companies worldwide that show a commitment to aggressive Duchenne's research. For example:

- **DART Therapeutics** – Charley's Fund partnered with other nonprofits to found and run this biotech company devoted to finding therapies for DMD.
- **AVI Biopharma** – partnered to optimize the company's backbone chemistry for a suite of exon skipping drugs. AVI's lead compound, eteplirsen, is currently in Phase II trials in the U.S. and Europe.
- **Galapagos** – Collaborated with this Belgian biotech company to develop its selective androgen receptor modulator drug as a Duchenne treatment.
- **Halo Therapeutics** – Together with 11 other nonprofit foundations, invested in Halo's efforts to expedite a Phase II study of HT-100, an orally available small molecule drug candidate being developed to reduce fibrosis and promote healthy muscle fiber regeneration in Duchenne patients.
- **Sarepta Therapeutics** – Charley's Fund has partnered with Sarepta Therpaeutics to optimize the company's backbone chemistry for a suite of exon skipping drugs. Sarepta Therpaeutics' lead compound, Eteplirsen, is currently in Phase II trials in the United States and Europe. They are exploring a partnership to expedite development of additional compounds so more boys can be treated faster with this promising therapeutic approach.

Financials

Charley's Fund is a 501(c)(3) tax-exempt nonprofit organization. The most recent financial information available is from 2011.

Year ending 12/31/11:

- Revenue: $1,615,320
- Assets: $3,783,996
- Grants paid: $259,000
- Gifts received: $1,580,560
- Expenditures: $636,751

Key Accomplishments

- Since 2004, Charley's Fund has directed more than $18 million into medical research to expedite the development of treatments for Duchenne muscular dystrophy.
- In 2005, Charley's Fund made its first major investment in a biotechnology company; Duchenne boys in many countries are now being treated with Prosensa's lead compound in Phase III clinical trials. Prosensa's solid results prompted Charley's Fund to enter into a partnership with GlaxoSmithKline.
- Co-owns DART and Halo Therapeutics with the Nash Avery Foundation. Halo was founded to acquire and develop a powerful anti-fibrotic drug that shows promise as a treatment for Duchenne and other orphan diseases. The DART model joins patient foundation assets, pharmaceutical industry expertise, and investor capital to speed development of new therapies.

- Charley's Fund has been instrumental in getting three promising treatments into human clinical trials, including PRO-051, Sildenafil (repurposed), and IGF-1 (repurposed).

Leadership

Charley's Fund is governed by a Board of Trustees and counseled by a Scientific Advisory Board.

- **President and Co-Founder**: Benjamin D. Seckler, MD, info@charleysfund.org
- **Co-Founder**: Tracy Seckler, tracy@charleysfund.org