CureDuchenne

CureDuchenne is a national nonprofit based in Newport Beach, Calif., that raises awareness and funds research to find a cure for Duchenne muscular dystrophy. Duchenne is a progressive, muscle-wasting disease that impacts one in every 3,500 boys. CureDuchenne’s mission is its name…to cure Duchenne. Our vision is to save this generation of Duchenne boys and our goal is to fund research to find a cure for all boys impacted by the disease. CureDuchenne focuses on high-impact research projects and helps accelerate them through the clinical trial process.

Innovative Research

CureDuchenne is leading the charge to find a cure, and to date, seven research projects have made their way into human clinical trials with our support. Despite the disease’s devastating impact on its sufferers, Duchenne research is severely under-funded compared to other pediatric diseases. The funds we have raised have been used wisely and have made a tremendous impact within a short period of time.

CureDuchenne funded two companies early on, Prosena and Sarepta, that are on the way to becoming the first to seek FDA approval for drugs to treat Duchenne. These first drugs will only treat 15% of Duchenne boys, but with the success of these initial drugs, we are more motivated than ever to fund the research that will treat all Duchenne boys. The potential exists, therefore, to make a marked difference in the quality of life in Duchenne patients, and there is hope, for the first time ever, for curing this destructive disease affecting 300,000 boys worldwide.

CureDuchenne has also funded the Center for Duchenne Muscular Dystrophy at UCLA for the past five years. The CDMD at UCLA supports muscular dystrophy related translational research and clinical care at UCLA.

CureDuchenne has funded four research projects in 2012: the MDX Duplication Mouse Model with duplicated exons; Spironolactone, a study with currently approved drugs that may help treat critical cardiac issues; Summit Corporation plc. which is testing the reformulation of the utrophin upregulator SMT C110; and Halo Therapeutics which is developing an anti-fibrotic drug.

Very few health-related nonprofits have been as successful and as focused as CureDuchenne in helping bring potential life changing treatments into human clinical trials.

Leadership

Scientific Advisory Board

CureDuchenne’s Scientific Advisory Board includes the leading scientists and innovators in the world working on Duchenne muscular dystrophy. The Board plays a critical role in setting research strategy, sharing knowledge and collaborating to find a cure.

Chief Scientific Officer

CureDuchenne has a Chief Scientific Officer on staff. Dr. Michael Kelly, a senior pharmaceutical executive with more than 25 years’ experience in drug discovery and development. Dr. Kelly is helping to advance drug development programs and identify new drug targets that exhibit potential to transform the treatment of Duchenne muscular dystrophy by treating the whole disease.

Board of Directors

The CureDuchenne Board of Directors is comprised of talented and successful individuals around the country who bring a wealth of knowledge and expertise to the organization. The Board Chair is Jeffrey A. Goffman, a business leader from Corona del Mar, California.
CureDuchenne Events
In 2012, CureDuchenne hosted three major, national events that raised a total of more than $1 million and had more than 1,400 attendees who gave generously to support our mission. In addition, countless parents and Duchenne advocates created local events and fundraisers across the country to help support CureDuchenne and its efforts to find a cure.
From national spokesperson Super Bowl Champion Clay Matthews of the Green Bay Packers to NHL All-Star Ryan Getzlaf, captain of the Anaheim Ducks, celebrities helped us increase awareness of Duchenne.

CureDuchenne Impact
• Seven research projects have made their way into human clinical trials with support from CureDuchenne.
• The most advanced project Prosensa/GSK has a drug in Phase III clinical trials that, if approved, will be the first novel drug available for the treatment of Duchenne.
• Sarepta’s drug has shown positive results and will be applying for the next phase trial early next year.
• CureDuchenne’s research investments have leveraged pledges of more than $100 million from government agencies and pharmaceutical companies to fund research leading to a cure.
• Reached more than 100 million people worldwide through awareness campaigns.
• CureDuchenne has seen an average of 25 percent in year-over-year revenue for the last three years.

**Based on current scientific knowledge, Duchenne could become treatable for this generation of boys. We just need the funding to make it happen now. Help us CureDuchenne Now, donate at www.cureduchenne.org.**