

25 MOMENTS IN PPMD HISTORY

■ Research
■ Advocacy
■ Care
■ Engagement

1994

PPMD founded and hosts 1st Annual Conference

1997

PPMD invests in first Duchenne Muscular Dystrophy Research Center

1998

Steroids become gold standard of care

2000

PPMD hosts 1st Annual Advocacy Conference

2001

MD-CARE Act signed into law (reauthorized in 2008, amended in 2014)

2003

PPMD funds first Duchenne Drug Discovery Program & begins convening consensus meetings that would cover topics like pulmonary, cardiac, and endocrine/steroid use

2005

PPMD's endurance program, Race to End Duchenne, participates in first race

2005

Provided seed funding for Preclinical Advancing Therapeutics Lab for the exploration and validation of compounds and biologics

2006

PPMD and CDC convene community thought leaders to develop Care Considerations

2007

The Duchenne Registry (aka DuchenneConnect) is born

2009

Care Considerations is published (updated in 2018)

2009

PPMD creates PPMD Connect program (aka FACES) uniting Duchenne families

2011

PPMD launches Cardiac Care Initiative which, to date, has invested over \$5 million

2012

PPMD leads way for FDASIA to be passed by Congress mandating regulatory flexibility for rare diseases

2013

PPMD announces two \$1 million RFAs for exon skipping and late stage preclinical/early stage clinical projects

2013

PPMD leads community in drafting guidance for FDA, presented to FDA in June 2014

2014

PPMD launches Certified Duchenne Care Center Program

2015

PPMD creates and convenes first Duchenne Drug Development Roundtable

2015

PPMD Adult Advisory Committee is formed to help guide the organization's mission

2016

PPMD launches Gene Therapy Initiative which, to date, has invested over \$4 million

2016

FDA approves EXONDYS 51 (eteplirsen) for treatment of Duchenne

2017

FDA approves Emflaza (deflazacort) for treatment of Duchenne

2018

New ICD 10 Code issued for Duchenne/Becker, resulting from PPMD-led evidence review & nomination

2019

PPMD launches Duchenne Newborn Screening in New York state

2019

PPMD announces 25th Certified Duchenne Care Centers and plans to certify adult care centers and mentor global certification program



H. Lee Sweeney, PhD, launches PPMD funded Preclinical Advancing Therapeutics Lab



Connecting Families — PPMD's first Annual Conference in Orlando, Florida 1994



PPMD's first national fundraising campaign — the Convoy of Hope — included families traveling across the country in WW2 vehicles