Parent Project Muscular Dystrophy

Overview

Ryan Fischer
Senior Vice President
Community Engagement
1994 - Questions and Missing Pieces

- What was the current state of research and care in Duchenne?
- What was the federal government investment in Duchenne?
- What data existed about the Duchenne population?
- How do we move the needle toward better care and treatments for Duchenne?

Critical Missing Pieces
- Care standards
- Natural history data
- Outcome measures
- Federal investment & coordination
- Companies
- Mobilized grassroots community
The Muscular Dystrophy Community Assistance, Research & Education Act (MD-CARE Act)

**MD-CARE Act 2001**
Established:
• Centers of Excellence
• MD STARnet tracking and surveillance
• MD Coordinating Committee
• MDCC to develop ‘MD Action Plan’

**MD-CARE Act Amendment 2008**
• Added the National Heart, Lung, and Blood Institute (NHLBI) to MDCC
• Enhancement of clinical research
• Expansion of MD-STARnet
• Duchenne Care Considerations – Develop and Disseminate

**Paul D Wellstone MD-CARE Act Amendment 2014**
• Expanded research to focus on Endocrine, Pulmonary, Cardiac & Transition into Adulthood
• Additional federal agencies on MDCC
• Sharing of data from MD-STARnet with community researchers
• Update of DBMD Care Considerations to include adults with
• Update of initial ‘MD Action Plan’ by MDCC
Results 2001 - Present

**Care**
- Care considerations published
- Care much more standardized (clinics)

**Research**
- Wellstone Centers of Excellence
- Animal Studies
- Basic and Translational Research Grants

**Data collection**
- Outcome measures
- Natural History Studies
- MD-STARnet Surveillance
- ICD Code

**Federal Coordination and funding**
- MD Coordinating Committee grows
- Research Plan for MD's
- 500+million in Duchenne Funding

**Drug Development**
- 2 Approved Therapies
- 45+ Companies
- Pipeline Full of Hope
• The only annual Advocacy Conference focused on Duchenne

• Focused on advancing MD-Care Act as well as regulatory policy

• Prioritize impacting:
  • Food and Drug Administration (FDA)
  • Center for Disease Control (CDC)
  • National Institutes of Health (NIH)
  • Department of Defense (DOD)
• Advocating and participating in the development of a **Standard of Care** (recently updated)

• Established the Duchenne **Certified Care Center Program**

• Focusing on **gaps that exist** in care sub-specialties
Advances in Research

- Invested over 50 million in research, leveraging $500 million in federal dollars and billions in private dollars.

- Have worked to establish a research infrastructure to enable drug development.

- 2 approved therapies with over 25 active treatment trials and over 40 companies in the space.

PPMD’s leadership in the Duchenne community created an inflection point, resulting in over 40 pharmaceutical companies now in the Duchenne space—companies who have invested approximately $8 billion in the fight to end Duchenne.
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<th>Pre-clinical</th>
<th>Phase I</th>
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PPMD building upon the work done to date

- **FDA Guidance for Industry**
- **PPMD Drug Development Roundtable** – pre-competitive consortium
- **Critical Path Initiative** – Understanding the progression of Duchenne
- **Newborn Screening** Consortium – treating as early as possible
- **Duchenne Registry** – Expansion into approved therapies (Sarepta partnership)
- **Platform Trials** – studying multiple drugs at once
- **Gene Therapy and Gene Editing** – PPMD investment in both

Encourage and Promote Collaboration among ALL Duchenne stakeholders
THANK YOU!