# BROADLY ENGAGING THE DEVELOPMENT PIPELINE

For over two decades, Parent Project Muscular Dystrophy (PPMD) has contributed to each stage of the drug development pipeline, awarding grants, filling in critical gaps, convening stakeholders, and redefining the clinical trial landscape.

Parent Project Muscular Dystrophy











## DISCOVERY & -

Exploratory research awards
Validation & replication

study services
Updated Duchenne Care
Consideration Guidelines
& Family Guide
Duchenne Newborn

Screening Program
The Duchenne Registry
ChildMuscleWeakness.org
an early diagnosis program
AAP motor delay tool

- ICD-10 code refinement

#### TRIAL READINESS/

#### PHASE 1

Certified Duchenne Care Center program & Clinical Trial Awareness program Duchenne Specialty Care Workshops PPMD / C-Path Duchenne Regulatory Science Consortium Duchenne Drug Development Roundtable engaging sponsors in pre-competitive space Partnering with federal agencies (MDCC, FDA, CDC, NIH, DoD, CMS, SSA) The Duchenne Registry trial readiness services Duchenne FDA Guidance

for industry

#### PHASE 2/3 & RECRU

### & RECRUITMENT

Trial education and recruitment
Duchenne community engagement
Leading creation of forward thinking expert publications, i.e.: Putting Patients First: Patients are Waiting, & numerous patient & caregiver preference study publications
Advisory Committee & IND meeting support

Leading passage of
 S federal bills, securing
 Duchenne-specific federal
funding, & supporting rare

disease legislation

## REGULATORY =

Clinical trial support
Drug development
research awards
FDA & regulatory
engagement
The Duchenne Registry trial

The Duchenne Registry trial recruitment services
Multichannel community outreach & education series
Clinical trial participant education

Expert consultation informing trial enrollment & design

#### POST-MARKET & ACCESS

Pioneering access, coverage, & reimbursement strategy

Decode Duchenne, free genetic testing

Patient engagement initiatives

Post-marketing strategy development

Payer engagement