PP MD

How PPMD Engages the Pipeline

- Exploratory/bridge grants
- Duchenne Connect Registry
- NBS effort
- Care consideration guidelines
- Xcelerate Program
- Replication studies

- Corporate engagement program
- PPMD newsletter/blogs/webina rs
- Clinical trial support
- Input to FDA

- Connect Conference & Policy forums
- On-line education
- PPMD Parent Adv Council
- PPMD Adult Adv Council
- FACES & State Captains
- Combo Therapies program

Discovery & Preclinical

Trial
Readiness/
Phase 1

Phase 2/3

Regulatory Approval

Post-Market

- MD STARnet
- PPMD Certified Duchenne Care Centers
- Federal agency partnering (e.g., MDCC, NHLBI)
- TREAT-NMD TACT support
- PPMD/C-Path Duchenne Regulatory Science Consortium
- Duchenne Drug Development Roundtable
- Biomarker/Nat Hist funding

- Duchenne FDA draft guidance
- Benefit-Risk Survey
- Putting Patients First white paper
- Support for accelerated approval

ParentProjectMD.org