Duchenne muscular dystrophy: Project HERCULES

February 2019
Timeline for Translarna and timeline for Duchenne
Unique multi-stakeholder collaboration

- To allow pharmaceutical companies, charities, academics, patient organisations and experts to **work together to build the evidence base for DMD** required by Health Technology Assessment Agencies, such as the National Institute of Health and Care Excellence (NICE).

- To **generate, align and share** high quality disease-level evidence across an entire condition to enable an informed Health Technology Assessment (HTA) process for **more transparent and consistent** reimbursement decisions.
Project Hercules Steering Group

- **International patient organisations**
- **Academics**
- **Other partners**
- **Industry**: 9 pharma companies
- **Advisers**: clinical, health economics, EMA
- **Country experts**
- **HTA agencies**
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HERCULES Deliverables

Data analysis
- Mapping clinical trial endpoints and natural history to clinical outcomes - to populate transition states

Quality of Life Metric
- Critique of existing QoL metrics
- Bespoke utility metric for DMD

Burden of Illness study
- Broad measure of burden of DMD to inform economic model costs

Economic model
- Template economic model for companies to adapt for individual products

Additional HTA support
- Other support to HTA and reimbursement processes, including publications and stakeholder education
Key Project Hercules events/milestones 2019

• Critique of QoL metrics complete – January 2019
• Burden of Illness study starts – February 2019
• Stage 2 Quality of Life survey launched – February/March 2019
• Data analysis complete – May 2019
• Draft QoL metric available – May/June 2019
• Economic model complete – June 2019
• Quality of Life metric workstream completed – August 2019
• Burden of Illness study reports - tbc