Duchenne Muscular Dystrophy
What a Community Can Do

Elizabeth Vroom
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Disclosure of Interests

President Duchenne Parent Project Netherlands
Chair United Parent Projects MD
Duchenne MD

‘Paralyzed’

Progressive

Severe deformities

Fatal

Ventilation
Stakeholder cooperation to overcome challenges in orphan medicine development: the example of Duchenne muscular dystrophy

A collaborative and constructive dialogue between patients’ representatives, academics, industry, and regulators can facilitate and accelerate treatment development for rare diseases.
For rare diseases, development and implementation of standards of care to decrease variability is crucial for multicentre trials.
Take home messages

Functional and molecular outcome measures should be developed in collaboration with patients’ representatives and regulators.
High-quality data for natural history and outcome measures are crucial for clinical trial design and regulatory approval; ideally, data should be obtained before or in parallel with potential treatments.
Developing a treatment for a rare disease such as Duchenne muscular dystrophy should be a global effort. Regulatory requirements should be aligned and communication should be continuous between regulatory bodies in different global regions with respect to guidelines for treatment development and biomarker qualification.