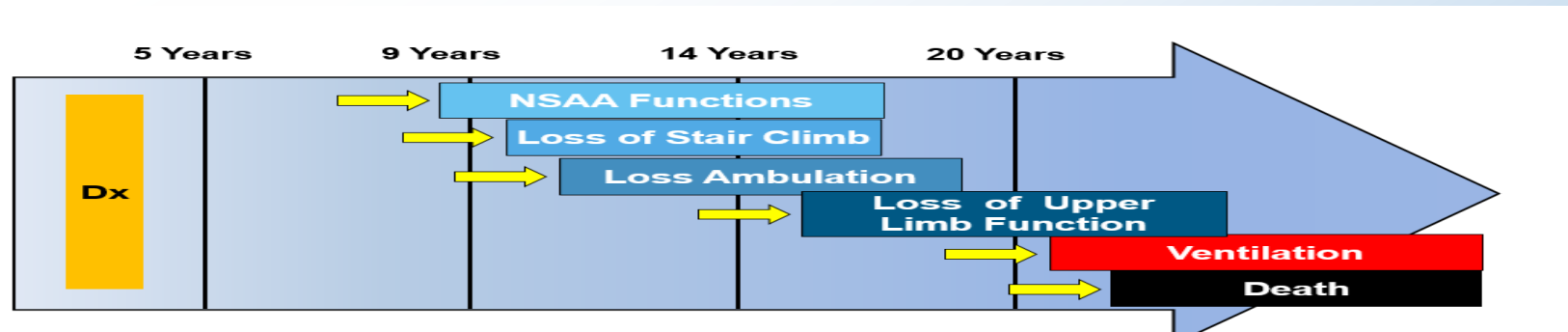
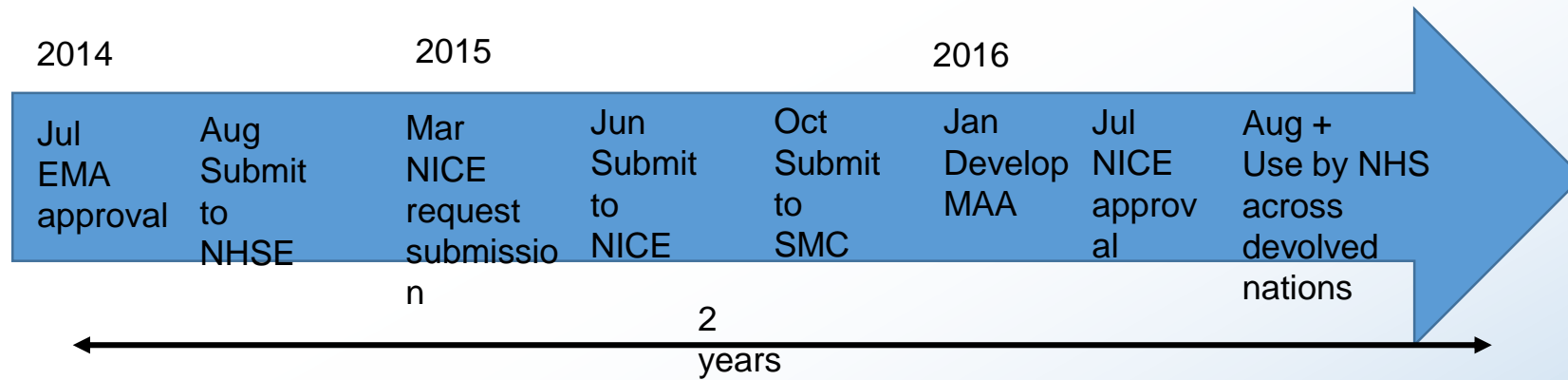




Duchenne muscular dystrophy: Project HERCULES

February 2019

Timeline for Translarna and timeline for Duchenne



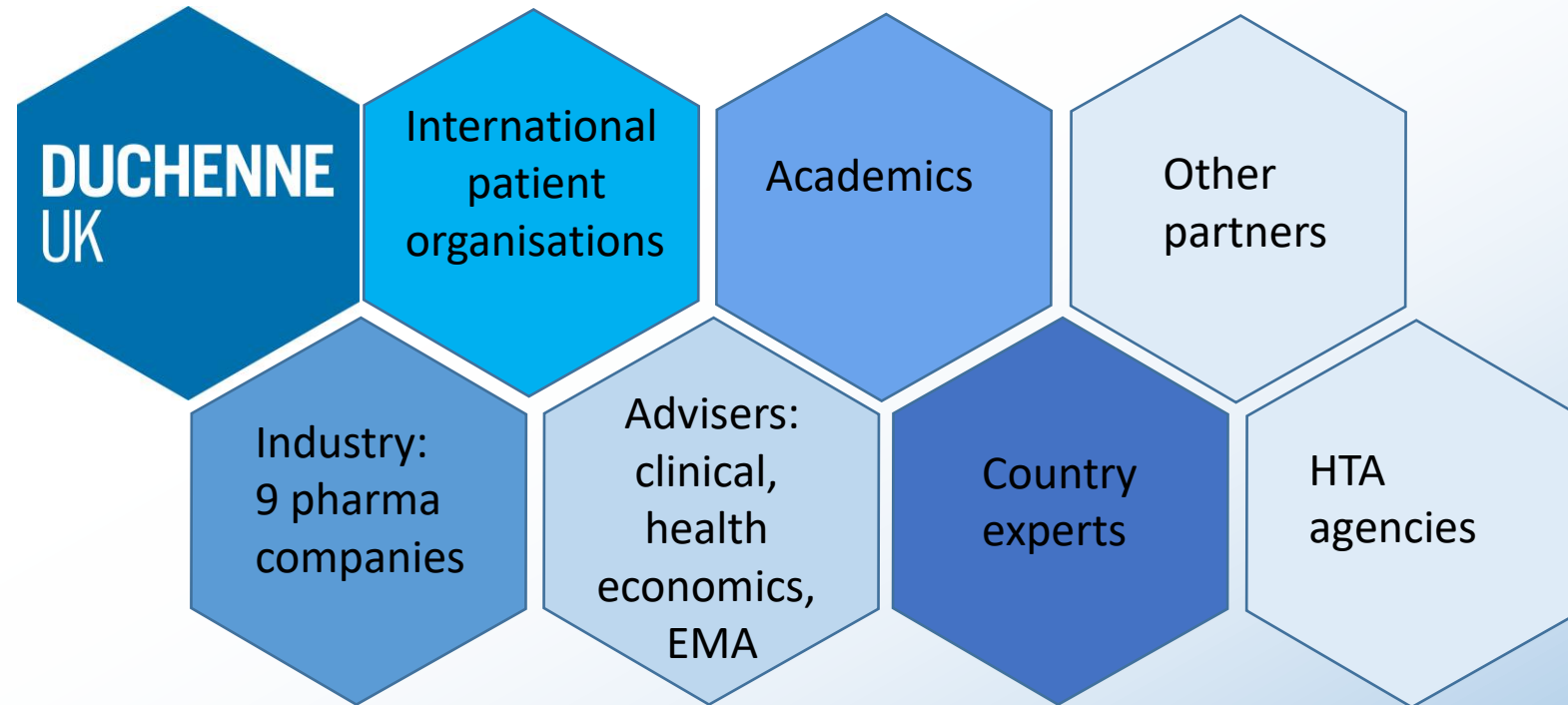
HERCULES.

DMD: HEALTH RESEARCH COLLABORATION UNITED IN LEADING EVIDENCE SYNTHESIS

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



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