

**The DMD-QoL: A new quality of life tool designed in collaboration with patients to help support access to life changing treatments for boys and men living with Duchenne muscular dystrophy**

**What is Duchenne muscular dystrophy (DMD)?**

DMD is the most common fatal genetic disease diagnosed in childhood. Children, who are almost exclusively male, born with DMD cannot produce the protein dystrophin which is vital for muscle strength and function. Muscle weakness starts in early childhood. Many use a wheelchair by around the age of 12. As deterioration continues it leads to paralysis and early death, often in their 20s. In the UK there are around 2,500 boys affected and around 300, 000 worldwide.

**What is the DMD-QoL?**

The DMD-QoL is a short survey completed by a patient or caregiver that captures changes to quality of life over time. This can be used to assess disease progression or the impact of a therapy. Unlike traditional DMD quality of life tools this includes social and psychological aspects in addition to physical, giving a more rounded and realistic overview of the quality of life.

The DMD-QoL was developed through research with patients and their families and has been validated through robust scientific processes.

**How does the DMD-QoL support access to new treatments?**

Health technology assessments (HTAs), are the processes which evaluate the clinical and cost effectiveness of health interventions and are used to inform policy, such as whether a new treatment should be funded by the NHS. These assessments use established measures such as QoL to judge the impact a new treatment could have. If the HTA process does not judge that a treatment can make a difference against established measures, it can lead to significant delays or may prevent patients from accessing treatments that could make a real difference to other aspects of their life such as mental health or the ability to socialise.

Historically, the QoL measures available for people living with DMD have inadequately captured the full reality of living with the disease. The DMD-QoL is different as it is a much better reflection of all the aspects of quality of life affected by DMD that are most important to patients and their families. This gives regulators a new tool to evaluate new therapies against, and by using this tool they can clearly see how much of an impact the treatment can really make.

**Is the DMD-QoL already being used?**

The DMD-QoL is already being used by some pharmaceutical companies as part of their clinical trial process to assess potential new DMD treatments. Duchenne UK is now working with all other Project Hercules members, researchers and regulators to try and expand usage within the clinical trial and drug assessment pathways.

### **How was the DMD-QoL Developed?**

Duchenne UK, a patient-led charity with a mission to find new treatments and ultimately a cure for DMD, led the development of the DMD-QoL through their innovative collaboration, Project HERCULES in association with researchers at the University of Sheffield led by Drs. Jill Carlton and Philip Powell.

The questionnaire was developed in compliance with FDA guidance on the use of Patient Reported Outcome Measures (PROMs) in medical product development. It looks at all aspects of the disease, beyond the physical symptoms, to include the impact on mental health and socialising. Project HERCULES involved patients at every stage of development of the new QoL measure, from initial interviews about what mattered most to them, through to the final selection of items for the questionnaire.

### **What is Project HERCULES?**

Project HERCULES (HEalth Research Collaboration United in Leading Evidence Synthesis) is a groundbreaking multinational collaboration set up by Duchenne UK to develop tools and evidence to support Health Technology Assessments and reimbursement decisions for new treatments for Duchenne muscular dystrophy (DMD). It brings together patient organisations, clinicians, academics, leading pharmaceutical companies, Health Technology Assessment agencies and other advisers to build a better evidence base for DMD.

### **What additional work is planned for the DMD-QoL?**

Development work at Sheffield continues and the developers of the DMD-QoL are now researching a preference-based measure (using the DMD-QoL) that can be used to generate quality adjusted life years (QALY) to inform resource allocation decisions within HTA's.

### **How can the DMD-QoL be accessed?**

Project HERCULES is partnering with the Clinical Outcomes team at Oxford University Innovation to ensure that anyone undertaking research in DMD will be able to use the DMD-QoL. This will include companies undertaking clinical trials as well as charities, universities and other researchers. Companies who have developed new treatments for DMD will be able to use the DMD-QoL to measure QoL when they submit evidence to drug approval agencies, such as NICE in the UK. The DMD-QoL can also be used as a tool by clinicians making an initial assessment of a patient's quality of life.

Licences to use the DMD-QoL in non-commercial academic research and publicly funded healthcare are provided free of charge as part of our commitment to supporting the DMD-QoL for the benefit of the Duchenne community.

Further information on the DMD-QoL can be found on the Clinical Outcomes website along with the facility to request use of the DMD-QoL and support materials, including access to the rapidly growing library of available translations.