What is Project Hercules and what does it aim to do?

In November 2017 Duchenne UK launched Project HERCULES to support access to new treatments for Duchenne Muscular Dystrophy (DMD). Hercules is a unique international multi-stakeholder collaborative project that is working to develop tools and evidence to support Health Technology Assessments and reimbursement decisions for new treatments for Duchenne Muscular Dystrophy (DMD). It brings together leading pharmaceutical companies, academics, patient organisations and advisers to develop and build a better evidence base for DMD.

What are the objectives of Project Hercules?

- 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 Assessments (HTA) process for more transparent and consistent reimbursement decisions.

Although Project HERCULES focuses on DMD, it paves the way for similar approaches in other rare diseases, and has the potential to better demonstrate the impact of neurodegenerative conditions and the value of medicines in this field to help improve their chances of receiving positive HTA decisions.

There is one thing worse than having no drug licensed, and that is having one that’s been approved for use but that is sitting on a shelf because no-one has agreed to pay for it.

Emily Crossley, Co-Founder and CO-CEO, Duchenne UK

How is the project run?

The project is led by Duchenne UK with support from Josie Godfrey, a former Associate Director at NICE. There is a quarterly multi-stakeholder Steering Group chaired by Fleur Chandler who works in Value Evidence and Outcomes at GlaxoSmithKline, and a Duchenne parent who also sits on the Patient Advisory Board of Duchenne UK. The Steering Group is also attended by the pharmaceutical companies, patient organisations, advisers and academics.

Who is involved in the project?

- The seven pharmaceutical partners for the project are:
  - Pfizer Inc
  - PTC Therapeutics International Ltd
  - Roche
  - Sarepta Therapeutics, Inc
  - Solid Biosciences
  - Summit Therapeutics plc
  - Wave Life Sciences USA, Inc

- Academics - the University of Sheffield and the University of Leicester have been commissioned to develop the quality of life metric and data analysis.

- cTAP

- International DMD patient organisations including World Duchenne Organization, PPMD, USA, Save Our Sons, Australia, medical advisers and experts in aspects of HTA.

- GSK are providing support to the HERCULES project through sharing materials and expertise.
**HERCULES.**

**DMD: HEALTH RESEARCH COLLABORATION UNITED IN LEADING EVIDENCE SYNTHESIS**

### What are the outcomes of the project?

- A critique of the existing measures of Quality of Life (Summer 2018).
- Analyses of available data that will include mapping clinical trial endpoints to clinical outcomes, to be used in disease modelling and evidence that are meaningful for HTA and reimbursement decisions (Winter 2018/19).
- A core economic model for use in the appraisal of new treatments for DMD which individual companies can adapt to their products (Spring 2019).
- A new Quality of Life metric that will better capture important elements of DMD and which will generate a utility measure for use in QALY generation (Summer 2019).
- A Burden of Illness study to provide a comprehensive measure of the impact of DMD on patients and their families, and in the social and healthcare sectors. (Spring 2019)

### How is the project funded?

Duchenne UK is investing £200,000 in the initial stages of the project. The seven pharmaceutical companies are also investing in the project.

### Timeline for reimbursement Translarna a treatment for nonsense mutataions in DMD:

<table>
<thead>
<tr>
<th>Year</th>
<th>Month</th>
<th>Event</th>
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</thead>
<tbody>
<tr>
<td>2014</td>
<td>JUL</td>
<td>Conditional Marketing Authorisation Granted EMA</td>
</tr>
<tr>
<td>2014</td>
<td>AUG</td>
<td>Submit to NHSE</td>
</tr>
<tr>
<td>2015</td>
<td>MAR</td>
<td>NICE request submission</td>
</tr>
<tr>
<td>2015</td>
<td>JUN</td>
<td>Submit to NICE</td>
</tr>
<tr>
<td>2015</td>
<td>OCT</td>
<td>Submit to SMC</td>
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<tr>
<td>2016</td>
<td>JAN</td>
<td>Develop MAA</td>
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<tr>
<td>2016</td>
<td>JUL</td>
<td>NICE approval</td>
</tr>
<tr>
<td>2016</td>
<td>AUG</td>
<td>Use by NHS across devolved nations</td>
</tr>
</tbody>
</table>

### Why do we need Project HERCULES?

Decisions about whether or not to pay for new medicines for DMD, as for other rare diseases, can be challenging in the absence of comprehensive disease level data to inform these decisions. This can prevent or delay patients gaining access to effective new treatments. Organisations such as NICE in England run HTAs to determine whether new medicines offer value for money and should be paid for, known as reimbursement, by the NHS. For NICE to recommend that the NHS should fund for their medicines, companies are required to develop a comprehensive value dossier which includes data collection, economic modelling and quality of life measurements, all of which can be difficult, time consuming and expensive. Through Project HERCULES, we are developing a common disease level evidence base containing all the elements required for HTA that companies can adapt for their products. This should lead to a broader evidence base that better captures the impact of DMD on patients, families and society, improved economic modelling, lower costs and management time and better use of patient input.

### Further Reading

Please read this article in Pharmaco- economics, by Anthony Hatswell and Fleur Chandler:

*Sharing is Caring: The Case for Company- Level Collaboration in Pharmacoeconomic Modelling*

### How can I get involved or find out more?

If you would like to know more, please contact the project team at hercules@duchenneuk.org or visit: hercules.duchenneuk.org