International Collaboration in Value Assessment: The Hercules Experience

Poster Number 149

HERCULES.
DMD: HEALTH RESEARCH COLLABORATION UNITED IN LEADING EVIDENCE SYNTHESIS

DUCHENNE UK

Chandler, F[1,2], Crossley, E[2], Godfrey, J[3]*, Johnson, A[2]

[1]GSK, London, UK; [2]Duchenne UK, London, UK; [3]JG Zebra Consulting Ltd, London, UK;

What is Project Hercules?

In November 2017 Duchenne UK launched Project Hercules to support access to new treatments for Duchenne Muscular Dystrophy.

Project Hercules is an innovative international multi-stakeholder collaboration which aims to simplify the way evidence is generated for payers and Health Technology Assessment (HTA) Authorities such as NICE and HAS.

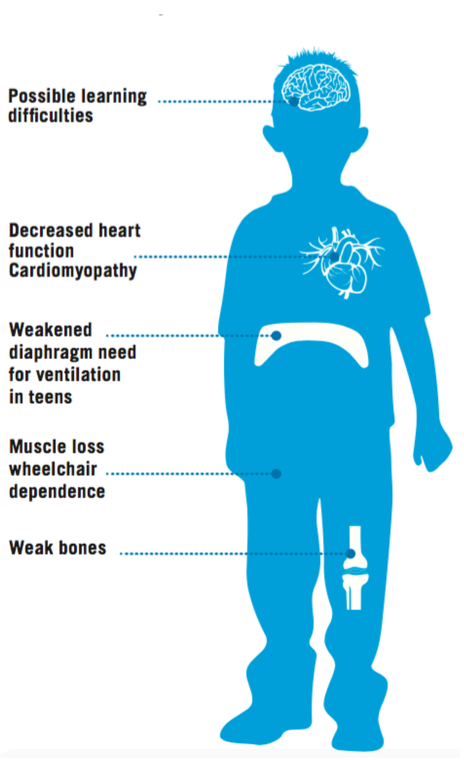
As for other rare diseases, the generation of robust data for Duchenne Muscular Dystrophy (DMD) treatments is hampered by the scarcity of affected patients and limited resources and Project Hercules is developing essential disease level evidence and tools to support international Health Technology Assessment and reimbursement decisions for new treatments for DMD.

Ensuring a high-quality evidence base that meets the needs of HTA agencies should result in more transparent and consistent decisions and fewer delays due to the lack of supporting evidence.

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

What is Duchenne Muscular Dystrophy?



Duchenne Muscular Dystrophy (DMD) is a genetic muscle wasting disease caused by the lack of the protein dystrophin. It affects the entire body.

DMD is the most common fatal genetic disease diagnosed in childhood. The disease almost always affects boy, and they tend to be diagnosed before the age of 5.

Children will be totally paralysed by their teens and they usually won't live beyond their 20s.

There an an estimated 2,500 patients in the UK and an estimated 300,000 sufferers worldwide.

Duchenne Muscular Dystrophy is classified as a rare disease.

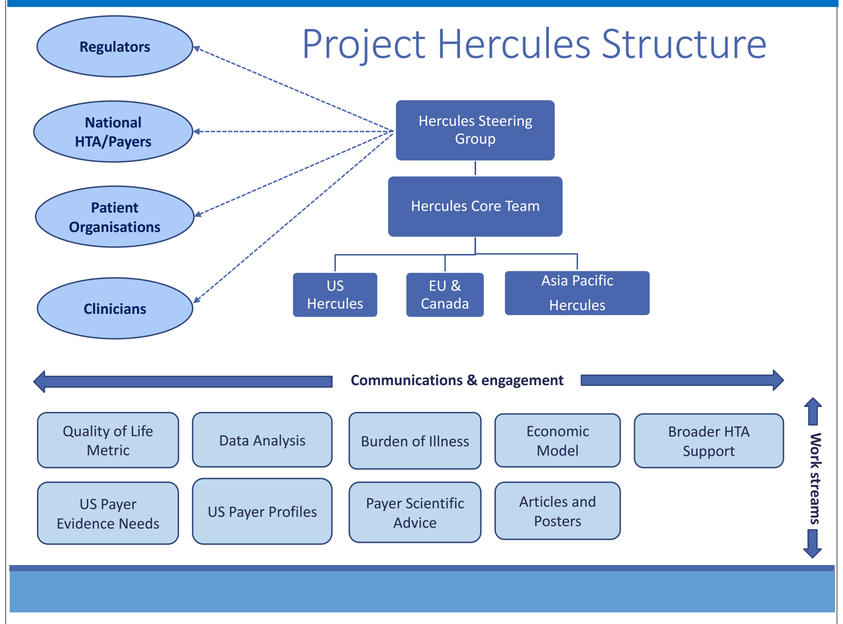
Why do we need this project?

Decisions about access to new treatments for DMD can take months, or even years. Some patients may no longer be eligible for treatment by the time it is available. For example, in England, reimbursement for Translana took 2 years from Marketing Authorisation.

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

2014		2015			2016		
JUL	AUG	MAR	JUN	OCT	JAN	JUL	AUG
Conditional Marketing Authorisation Granted EMA	Submit to NHSE	NICE request submission	Submit to NICE	Submit to SMC	Develop MAA	NICE approval	Use by NHS across devolved nations

How is Project Hercules run?

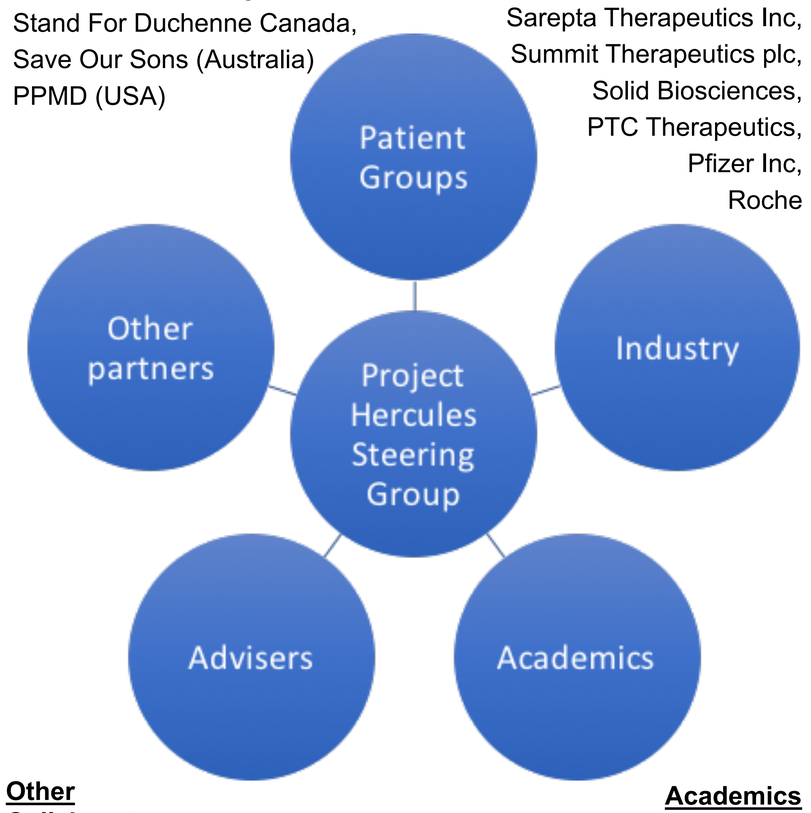


The project is led by Duchenne UK with support from Josie Godfrey, a former Associate Director at NICE. There is a quarterly multi-stakeholder Steering Group chaired by Fleur Chandler who works in Value Evidence and Outcomes at GlaxoSmithKline, and is a Duchenne parent who also sits on the Patient Advisory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

Patient organisations
World Duchenne Organization,
Stand For Duchenne Canada,
Save Our Sons (Australia)
PPMD (USA)

Industry Partners
Wave Life Sciences USA Inc,
Sarepta Therapeutics Inc,
Summit Therapeutics plc,
Solid Biosciences,
PTC Therapeutics,
Pfizer Inc,
Roche



Other Collaborators
Collaborative Trajectory Analysis Project (cTAP),
Source HEOR,
GSK

Advisers
Clinical expert, Clinical regulatory adviser, Health economics adviser, country experts

Academics
Sheffield School of Health and Related Research: the University of Sheffield,
University of Leicester

What will Project Hercules deliver?

Key deliverables include:

- A **critique of commonly used Quality of Life metrics**
- A **bespoke Quality of Life metric** for DMD, suitable for HTA
- **Data analysis** that maps available patient data against clinical outcomes that can be used for HTA
- A **gap analysis of available burden of illness evidence** and a **burden of illness study** that will measure the true burden of illness for DMD patients and their families
- A **core economic model** that can be adapted by individual companies
- An **analysis of US payer requirements** and development of **relevant disease level evidence**

What are the challenges so far?

Project Hercules has so far faced a number of challenges:

- Moving from the concept stage to fully signing up 7 industry partners. Duchenne UK were able to commit £200,000 to ensure work could start. This upfront commitment allowed sufficient progress to be made to encourage sign up and generate confidence in the project.
- Identifying potential data sources and accessing this data has proved challenging even with a broad multi-stakeholder collaboration.
- Individual workstreams and contractors need to come together to inform each others work. It has been important to create regular opportunities to share ideas, emerging findings and issues between different vendors.
- In the absence of a blueprint from a similar project, the initial proposal underestimated some of the complexities of delivery. The core project team are able to identify and respond to emerging issues such as:
  - The need for tailored work in the US and Asia-Pacific
  - The costs and time implications of seeking Scientific Advice from regulators and HTA agencies.

How could Project Hercules benefit other rare diseases?

- Current measures of Quality of Life are not suitable for many rare diseases, particularly degenerative conditions affecting children. A critique of these metrics and the development of DMD metric will be relevant to many of these diseases.
- A disease level evidence base for DMD will provide a more appropriate evidence base for other neurodegenerative conditions than those that are currently drawn from less relevant diseases.
- The disease level economic model itself could be replicable in other rare diseases, particularly those long term degenerative conditions.
- The Project Hercules approach demonstrates that collaboration in disease level evidence is possible and could be replicable across other conditions.

Conclusions

- Despite a number of good resources such as the NORTH STAR database, the current disease-level evidence base for Duchenne Muscular Dystrophy is not well suited to informing product value assessments and reimbursement decisions.
- Multi-stakeholder collaboration in disease-level evidence generation is both desirable and possible for DMD and other rare diseases.

References

Hatswell, A. and Chandler, F. (2017). Sharing is Caring: The Case for Company-Level Collaboration in Pharmacoeconomic Modelling. *PharmacoEconomics*, 35(8), pp.755-757.

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