

# International Collaboration in Value Assessment: The Hercules Experience

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## 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 boys, 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 are an estimated 2,500 patients in the UK and an estimated 300,000 sufferers worldwide.

Duchenne Muscular Dystrophy is classified as a rare disease.

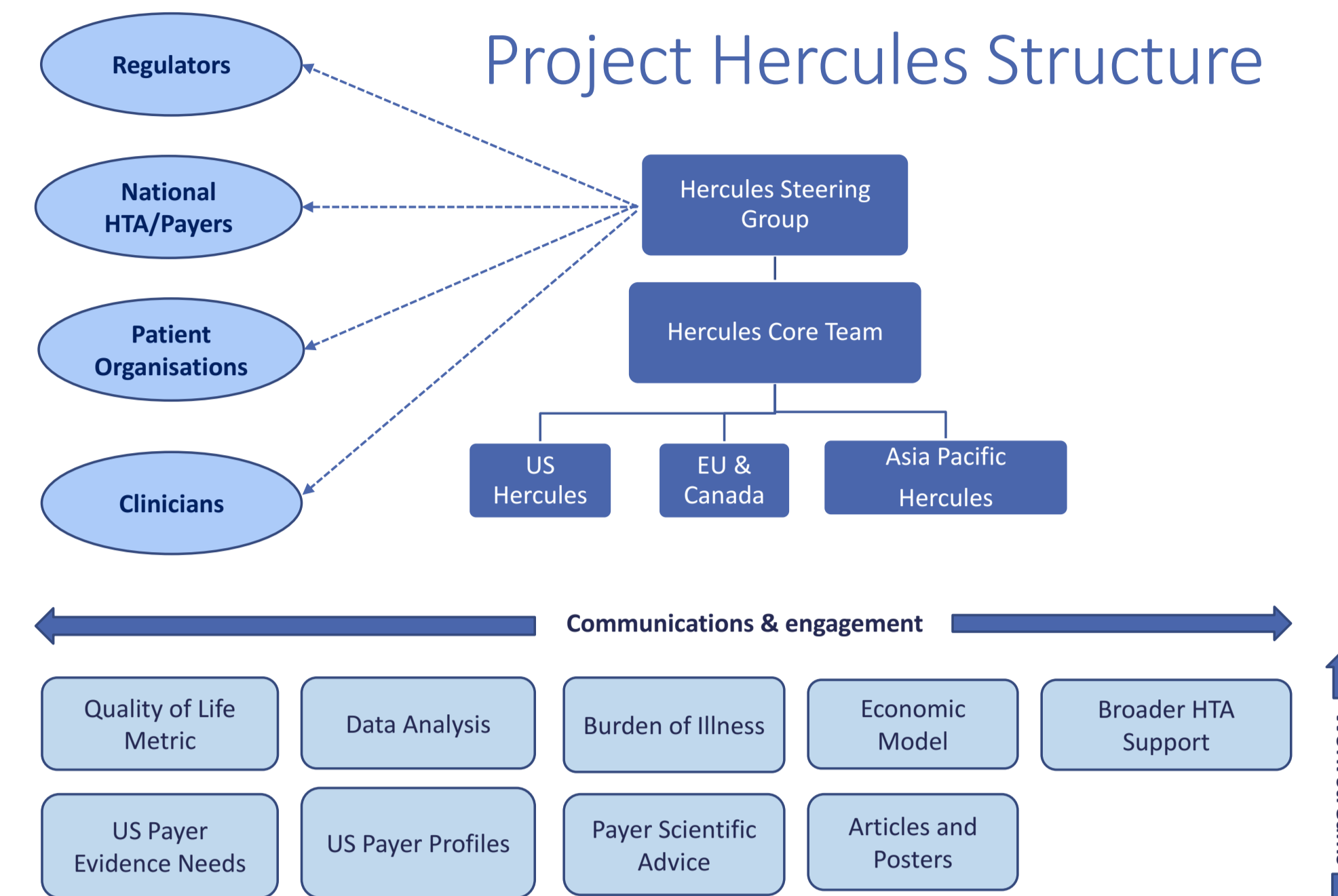
## Why do we need this project?

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 Translana a treatment for nonsense mutations in DMD:

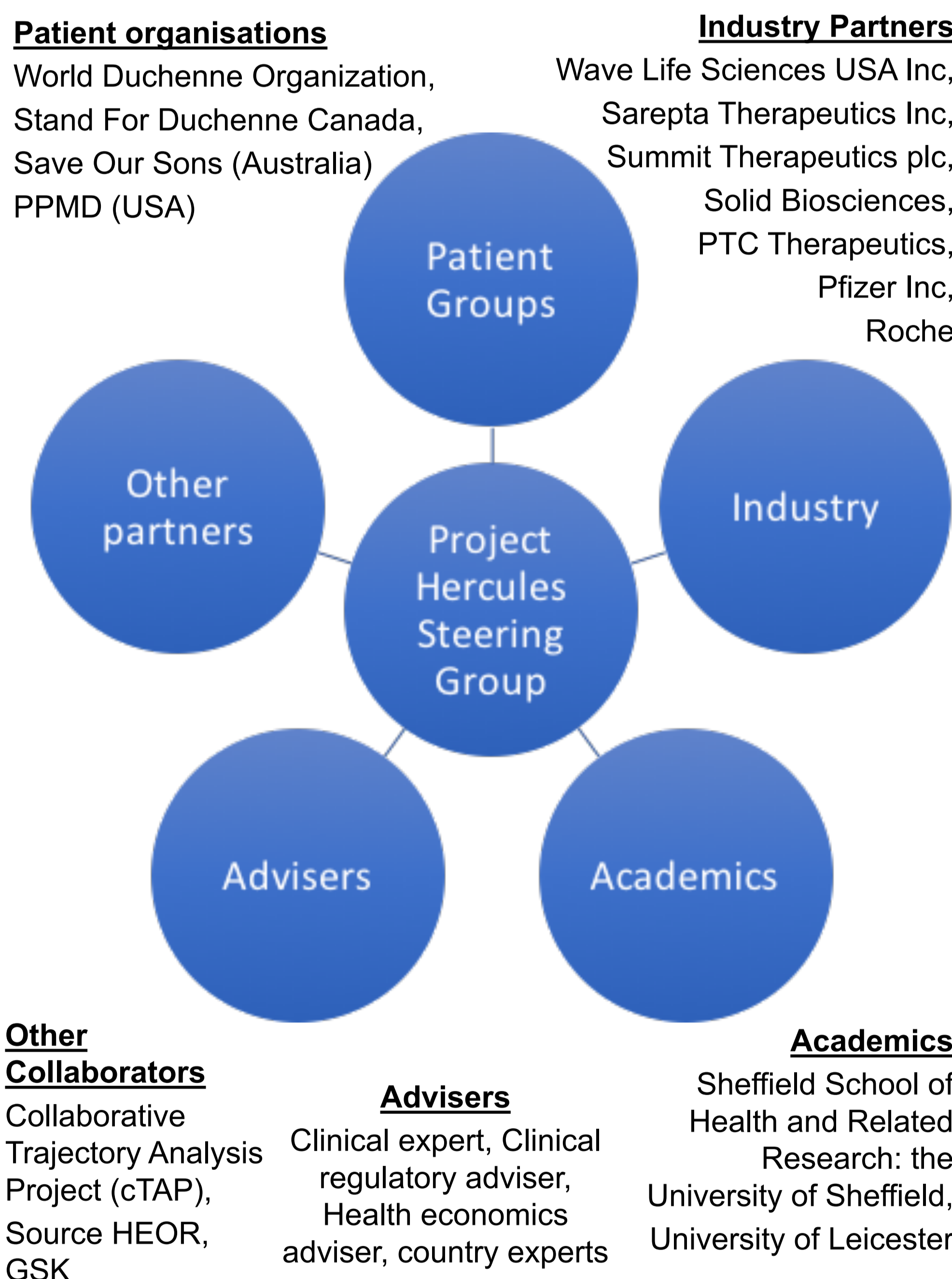


## How is Project Hercules run?



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?



## 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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