



Duchenne
UK

Project HERCULES: A patient led paradigm in evidence generation for HTA in Duchenne Muscular Dystrophy

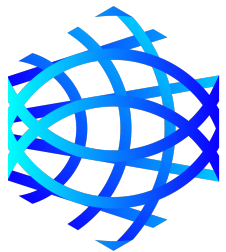
Fleur Chandler and Josie Godfrey
Parent Information Day, 2 October 2021



Fleur Chandler



Josie Godfrey



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Why Project HERCULES?

Regulators

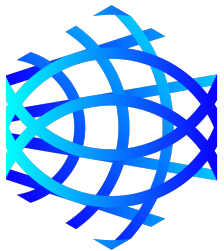
Is the treatment **safe**?
Does the treatment **work**?
Can the treatment be produced to a consistent high **quality**?

Health Technology Assessment

How **effective** is it?
Is it good **value for money**?
Is it **affordable**?

Payers

How **effective** is it?
Is it good **value for money**?
Is it **affordable**?



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SOLID
BIOSCIENCES

NICE
National Institute for
Health and Care Excellence



**Parent
Project
Muscular
Dystrophy**



Source
Your HEOR Partner



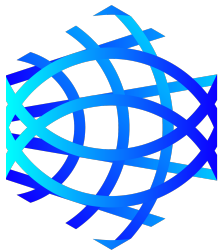
JGZEBRA
CONSULTING



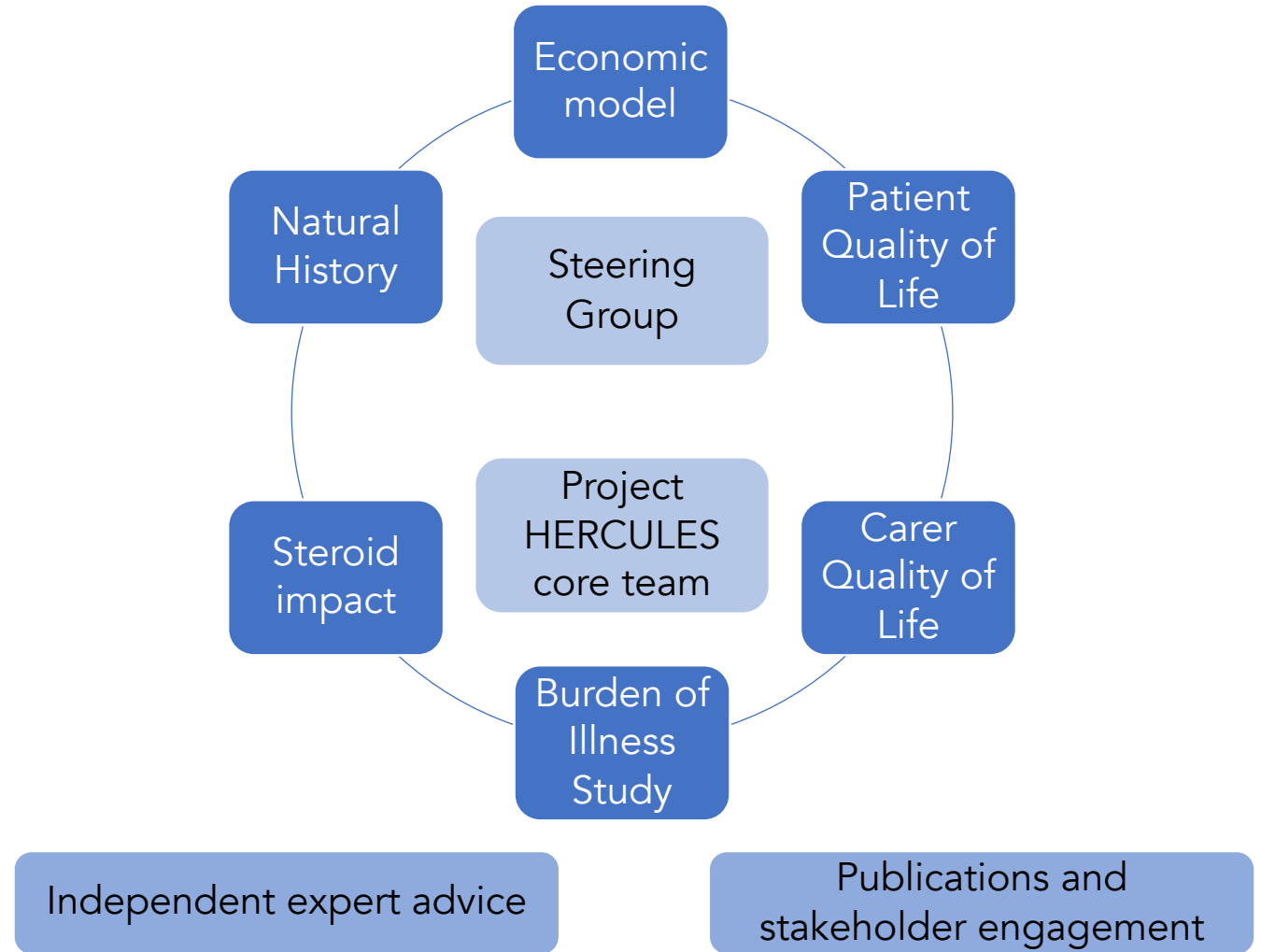
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What did we do?

- Patient leadership – Steering Group Chair, core team
- Patients at the centre of each workstream – defining and directing the work
- Iterative approach – work streams able to build on each other



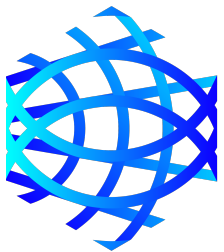
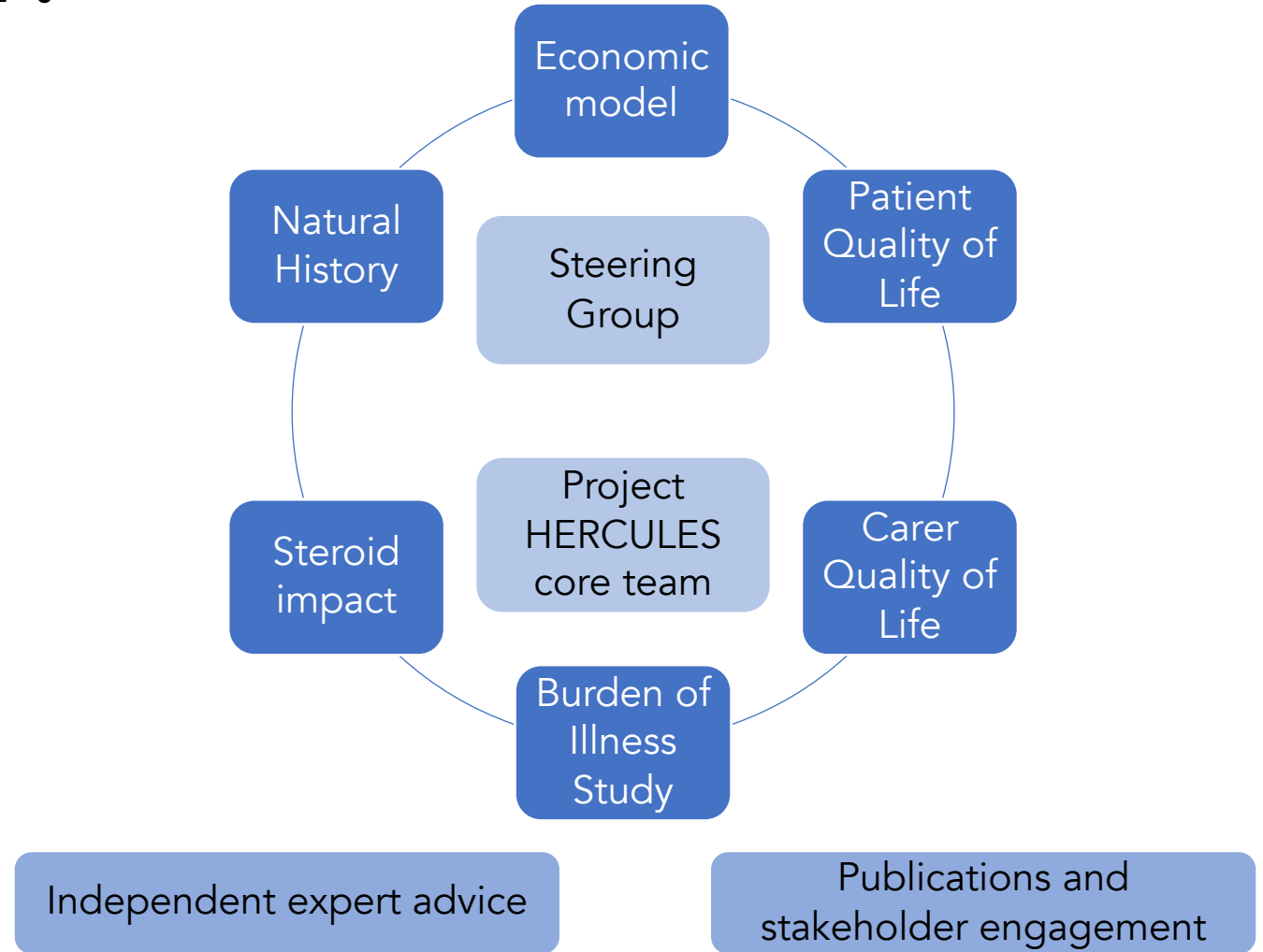
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What did we learn?

- Disease progression
- Quality of Life
- Carer Quality of Life
- Steroids



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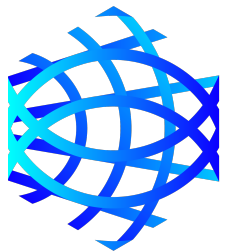
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What happens now?

Clinical
practice

Clinical
trials

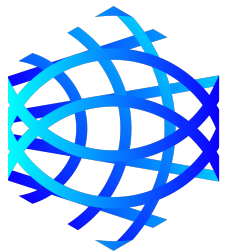
Health
Technology
Assessment



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Any questions?



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