

HERCULES.

DMD: HEALTH RESEARCH COLLABORATION UNITED IN LEADING EVIDENCE SYNTHESIS

Presented by Emily Crossley

FINAL PIECE OF THE JIGSAW: Reimbursement

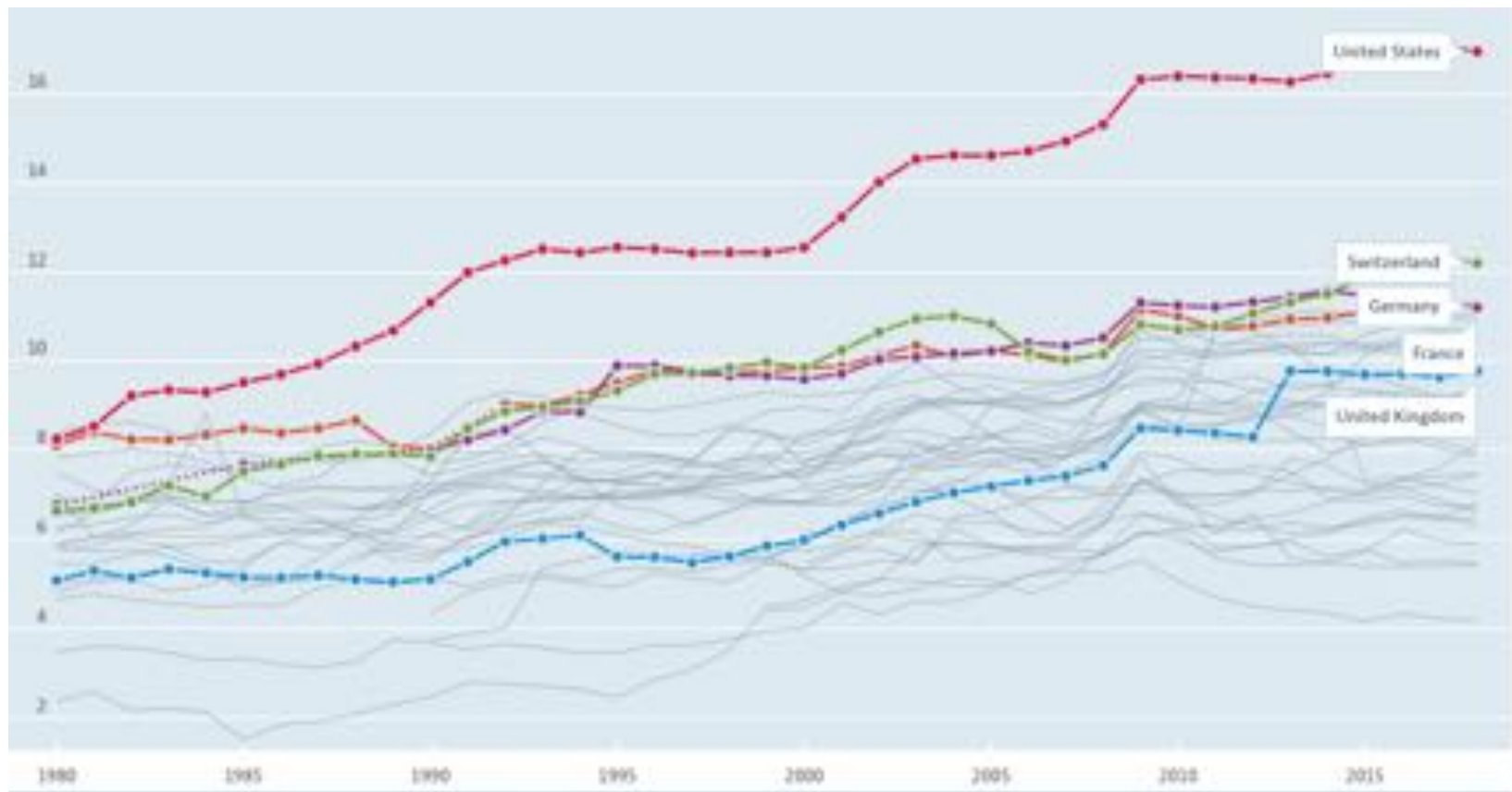


RISING HEALTHCARE COSTS:

Need for economic evaluations and assessment

Health spending Total, % of GDP, 1980 – 2018

Source: Health expenditure and financing: Health expenditure indicators



Why do we need Health Technology Assessments?

RESOURCES ARE SCARCE



DEMAND EXCEEDS CAPACITY TO TREAT

The Payer's View



Professor Michael Rawlins
 Ex-Chairman of NICE
 Chairman of MHRA

“The industry has to accept that just because new drugs are licensed does not mean that the health service wants or needs to buy them.”

Why it is so challenging in rare diseases?

- Small patient population sizes so no large scale data or information
- If medicines are given accelerated or conditional approval we lack the long term data to show how the drug works over time
- Difficult to quantify true burden and cost of progressive disease

Cost-effectiveness explained

Direct costs (incurred by health service)

- Monitoring
- Medication
- Administration
- Treating adverse events

Wider costs (incurred by society)

- Lost work time
- Costs incurred by carers
- Travel (and travel time)



Health outcomes

Patient reported outcomes

Quality of life measures –
EQD5

The Challenge of Reimbursement

Case Study: Translarna/Ataluren – PTC Therapeutics

Timeline for reimbursement of Translarna, a treatment for nonsense mutations in DMD:

2014

JUL

Conditional
Marketing
Authorisation
Granted EMA

AUG

Submit to
NHSE

2015

MAR

NICE
request
submission

JUN

Submit to
NICE

OCT

Submit
to SMC

2016

JAN

Develop
MAA

JUL

NICE
approval

AUG

Use by NHS
across devolved
nations



UNIVERSITY OF
LEICESTER



The
University
Of
Sheffield.



Source
Health Economics



HCD
ECONOMICS

HERCULES.

DMD: HEALTH RESEARCH COLLABORATION UNITED IN LEADING EVIDENCE SYNTHESIS

An innovative collaborative project to help companies prepare better for interactions with HTA bodies.



HERCULES.

DMD: HEALTH RESEARCH COLLABORATION UNITED IN LEADING EVIDENCE SYNTHESIS



Company Award for Patient Engagement



The HERCULES Project (Pfizer Inc, PTC Therapeutics, Roche, Sarepta Therapeutics, Solid Biosciences, Summit Therapeutics, Wave Life Sciences and Duchenne UK)

For the collaborative effort of all the companies involved in the HERCULES Project, bringing together patient organisations and industry to support access to new treatments for Duchenne Muscular Dystrophy (DMD).