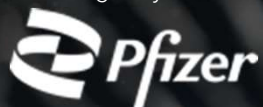


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as an agency on behalf of



OHE

ISPOR INTERNATIONAL

Health Technology Assessment of Gene Therapies: Are our Methods Fit for Purpose?

ISSUE:

- GTx represents a new era of medicine, offering the potential for transformational benefits for patients, health systems and society.
- Health Technology Assessment (HTA) of GTx can be challenging for a variety of reasons, including evidence generation; uncertainty; assessment of cost and affordability; and limitations of current value perspectives.
- The main objective of the panel will be to reconcile the various challenges facing value assessment for GTx, and put forward evidence-based recommendations to overcome these barriers.

Agenda



Session	Presenter / Moderator
Overview of the challenges associated with the HTA of Gene Therapies and potential solutions – report of OHE’s work with European experts	Adrian Towse <i>OHE</i>
Academic Health Economist Perspective	Louis Garrison <i>University of Washington</i>
Patient Perspective	Mark Skinner <i>Institute for Policy Advancement</i>
Industry Perspective	Patricia Dorling <i>Pfizer</i>
Discussion / Q & A	Adrian Towse <i>OHE</i>

Challenges of HTA of Gene Therapies



INITIAL ASSESSMENT OF CLINICAL EFFECTIVENESS

- **Generalisability** of clinical trial results
- **Trial design**: alternatives to RCTs
- Appropriate **outcome measures**
- Differing HTA body/payer **evidence requirements** increase the difficulty of designing trials



ASSESSMENT OF COSTS

- One/short duration treatments mean high **irrecoverable costs**
- Does / should **budget impact** affect the value-for-money rule used for reimbursement?
- Patient **portability** (insurance policyholders)



UNCERTAINTY REGARDING LONG-TERM OUTCOMES

- **Short-term follow up** (requires modelling)
- Uncertainty over whether **benefits sustained**
- Uncertainty regarding adverse events & **safety concerns**
- Need for appropriate HTA methods to handle uncertainty
- **Discount rates**



INCORPORATING ADDITIONAL ELEMENTS OF VALUE

- Are **curative therapies/life-changing** therapies valued more by society?
- Importance of **severity weighting**
- **Spillover effects** on family members/carers and society
- **Other elements**, including equity, insurance value, option value, etc.



We convened a roundtable to bring together **subject experts from different countries...**

...to work toward **consensus on the challenges** faced by gene therapies undergoing HTA evaluation...

...and to **find solutions** that could facilitate better patient access and improved patient outcomes.

Recommendations from our Roundtable Discussions

Challenges



INITIAL ASSESSMENT OF CLINICAL EFFECTIVENESS



UNCERTAINTY REGARDING LONG-TERM OUTCOMES



ASSESSMENT OF COSTS

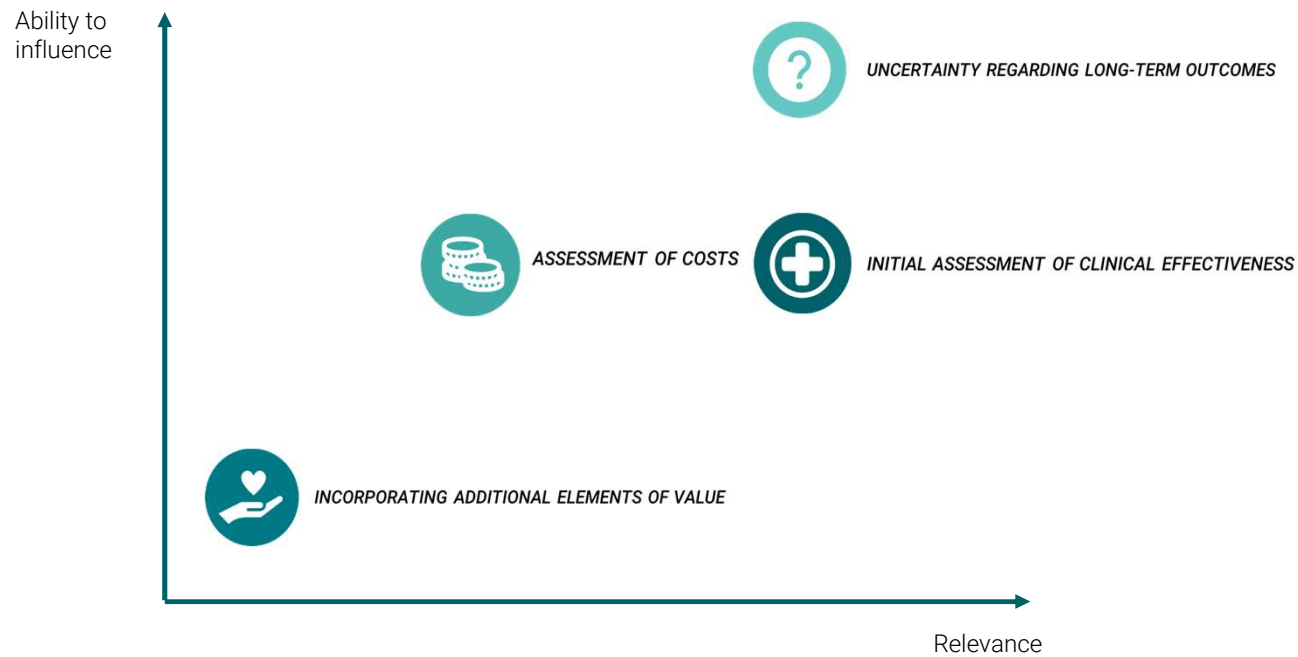


INCORPORATING ADDITIONAL ELEMENTS OF VALUE

Proposed solutions

- Early multi-stakeholder dialogue on study design pre- and post- launch
- Expanded data collection capability through registries with international collaboration
- Development of standards for the use of observational data and the validation of surrogate endpoints
- The use of novel payment models to help decision-makers reduce residual uncertainty
- Further research on the need to incorporate additional elements of value

Our Roundtable Experts Views of the Challenges



Agenda



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