HTA329

# The Race to Improve Health Technology Assessment of Gene Therapies

## WHICH COUNTRIES ARE EMBRACING RECOMMENDATIONS?


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## Background

Gene therapies face significant barriers to timely patient access. In 2022, the Office of Health Economics and Pfizer published recommendations to revise health technology assessment (HTA) to facilitate improved HTA of gene therapies[1]. Specifically, the recommendations explore changes to **HTA methodologies** and **evidence-generation** activities that should be prioritised to enable HTA to reflect the full value of gene therapies. In 2023, we initiated additional research to explore the extent to which our recommendations are being achieved in nine European countries, Australia and Canada.

## Aim

- To identify HTA bodies currently achieving our recommendations
- To draw out examples of best practices to support countries looking to improve their HTA of gene therapies.

## Methods

We conducted **targeted literature searches**, including searches of grey literature and HTA agency documentation. Our findings are also a compilation of expert inputs during **roundtable discussions** and through **local country affiliates**.. These inputs enabled us to evaluate the extent to which written guidelines are implemented in practice.

## Which Countries are Achieving our Recommendations?

| | AUSTRALIA | CANADA | DENMARK | ENGLAND | FRANCE | GERMANY | ITALY | THE NETHERLANDS | SPAIN | SWEDEN | SWITZERLAND |
|---|---|---|---|---|---|---|---|---|---|---|---|
| **Recommendation 1:** Recognise lifetime benefits | | | | | | | | | | | |
| **Recommendation 2:** Operationalise additional elements of value | | | | | | | | | | | |
| **Recommendation 3:** Develop standards for use of real-world evidence and surrogate endpoints | | | | | | | | | | | |
| **Recommendation 4:** Include outcome or other value-based arrangements | | | | | | | | | | | |
| **Recommendation 5:** Expand data collection through registries and cross-border collaboration | | | | | | | | | | | |
| **Recommendation 6:** Enable early multi-stakeholder dialogue | | | | | | | | | | | |

Key:

| Category | Description |
|---|---|
| Recommendation achieved | The recommendation has been considered, and relevant guidelines/agreements are routinely implemented. However, this does not preclude further improvements. |
| Recommendation partly achieved | Steps have been taken to begin implementing the recommendation or implementation has begun, but uptake could be improved considerably. |
| Recommendation not achieved | No steps have been taken to begin implementation of the recommendation. |

### Summary

- Most countries in our sample recognise the lifetime benefits in HTAs and therefore achieve this recommendation.
- Only Italy is achieving the recommendation related to the incorporation of outcomes-based payment models.
- Overall, most work is needed around the recommendation relating to operationalising additional value elements. This recommendation is being achieved by England, but not by Canada or Switzerland, with the remaining eight countries assessed as only partly achieving this recommendation.

## How the recommendation can be achieved:

RECOMMENDATION 1
- HTA body or equivalent recommending the use of a lifetime horizon for models produced for economic evaluations.
- HTA body or equivalent providing guidance on the use of discount rates which includes allowance for the incorporation for the use of alternative discount rates for long-term benefits.

RECOMMENDATION 2
- HTA body or equivalent explicitly recognising additional elements of value in a consistent way across all technologies.

RECOMMENDATION 3
- HTA body or equivalent providing detailed guidance on surrogate endpoints and RWE, including details of the circumstances when the inclusion of these in HTA is deemed appropriate.

RECOMMENDATION 4:
- aving mechanisms in place for outcomes-based or value-based agreements to be negotiated and for these to be routinely implemented.

RECOMMENDATION 5:
- existence or development of national registries which demonstrate the presence of infrastructure for generating RWE.
- engaging in collaboration with any stakeholder in the development of international registries or by involvement of the HTA body (or equivalent) in national and cross-border collaborations linking registry data.

RECOMMENDATION 6:
- mechanisms/pathways in place facilitating early scientific dialogue, incorporating all relevant stakeholders, including patient representatives.

## Examples of best practice include:

**Time Horizon** – ZIN (the Netherlands) mandates the use of a lifetime horizon[2].
**Discount rates** – HAS (France) recommends a lower discount rate for costs and benefits after 30 years[3], and NICE (England) recommends a reduced discount rate for costs and benefits for technologies with benefits that are sustained over a long period of time[4]. ZIN (the Netherlands) recommend the use of differential discounting is particularly advanced[5].

NICE (England) has a willingness to incorporate a number of additional elements of value using methods that ensure these value elements are considered consistently across technologies[4].

CADTH (Canada) provides detailed guidance on surrogate outcomes and reporting[6]. NICE (England) provides an RWE framework[7].

Despite a recent decline in the use of outcomes-based agreement[8], many outcomes-based and economic risk-sharing agreements have been implemented in Italy. This includes staged payments linked to individual patient outcomes being used for two gene therapies.

**Registries**: Many countries have established registries including France (national database for rare diseases) and Denmark (nationwide hospital registry). Italy has a number of AIFA monitoring registries for routine collection of data on the use of products.
**Cross-border Collaboration**: NICE (England), Avalia-t (Spain), AIFA (Italy), HAS (France), ZIN (the Netherlands) and G-BA (Germany) all engage in EUnetHTA (European Network for Health Technology Assessment) post-launch evidence generation (PLEG) pilots.

Many HTA bodies partake in joint scientific advice such as the parallel advice offered by CADTH (Canada) and NICE (England) [9] and the joint consultations available through the EUnetHTA that include HAS (France), IQWiG (Germany), G-BA (Germany), AIFA (Italy), AEMPS (Spain), TLV (Sweden) and ZIN (the Netherlands).

## Conclusion

- Although progress is being made towards adjusting HTAs for gene therapies in many countries, there is still significant room for improvement.
- Further progress will accelerate patient access and enable the potential transformational benefits of gene therapies to patients, and the benefits for health systems and society to be realised.


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