Background and purpose

- Countries appraise treatments to determine their added clinical benefit and/or value for money in their pricing and reimbursement systems that affect the access patients have to medicines. Traditional appraisal and reimbursement approaches may be unsuitable for RDTs.
- Often standard appraisal processes are used for RDTs, but this is challenging because RDTs come with a small evidence base and high cost, leading to substantial uncertainties in the determination of value.
- This raises the question as to whether supplemental processes with specific features as used by some countries are needed. These processes might affect the evidence submissions, critical assessment, wider appraisal and pricing and reimbursement.
- The objective of this research is to illustrate the impacts and contrast the influence on decision-making between countries with and without supplemental processes through evaluation of two specific cases.

Methodology

- Case studies were chosen that had been appraised in 2018-2019, and had generated substantial discussion in the clinical, patient and payer communities – Spinraza for Spinal Muscular Atrophy (RDT) and Luxturna for Inherited Eye Disorders (ultra-RDT).
- Countries with a variety of RDT appraisal processes were selected.
- Public appraisal reports were retrieved from Belgium, England, France, Germany, Netherlands, Norway, Scotland, Sweden, U.S., and BeNeLuxA used (no Luxturna reports for Belgium or BeNeLuxA). Both RDTs were approved in all selected countries.
- Information from reports was extracted into pre-designed templates for systematic comparison across countries, following the appraisal process structure (evidence submissions, critical assessment, appraisal, pricing/reimbursement).
- Analysis aimed to identify whether supplemental processes made a difference in the appraisal and decision.

Conclusions and practical application

These case studies enabled illustration of how some features of supplemental processes facilitated the appraisal of RDTs, specifically related to issues around uncertainty and high price. In particular, these features included broader consideration of value, more lenience around interpretation of clinical and economic evidence, more formal criteria for accepting uncertainty, different willingness to pay, and risk sharing to split the risk of high cost medicines. Findings suggest a need for adapted approaches for RDT appraisal, as they facilitate better management of specific challenges associate with RDTs and more consistent decision-making.

**References**


**Abbreviations**

- RDT = rare disease treatment
- HST = highly specialised technology
- STA = standard technology appraisal
- QALY = quality adjusted life year

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