IMPACT HTA

Improved methods and actionable tools for enhancing HTA

Do we need special processes to appraisal rare disease treatments?

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HTA conference

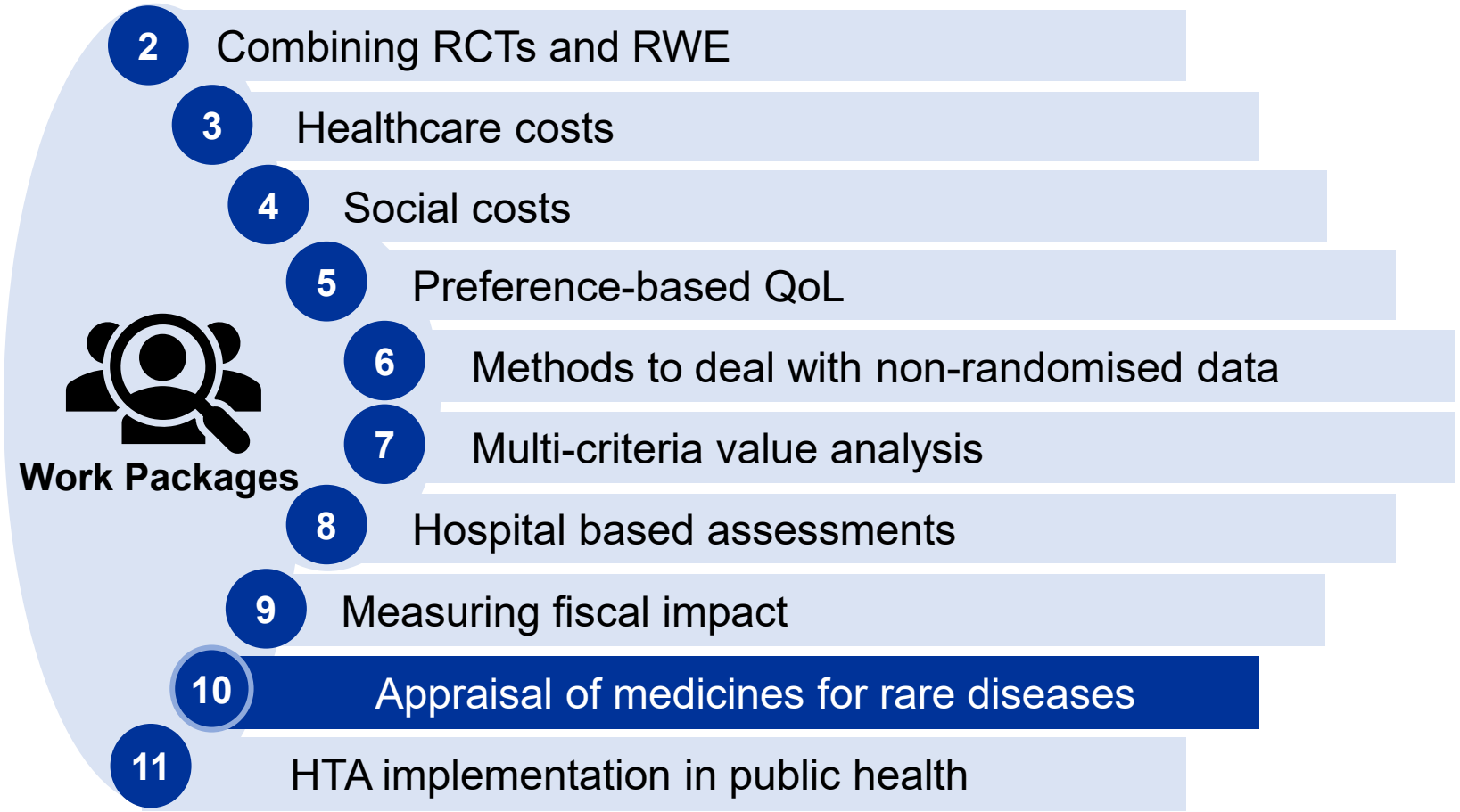
The role of HTA in the era of radical changes & disruptive innovation

Session 2: Spotlight session on the future of HTA

23 March 2021

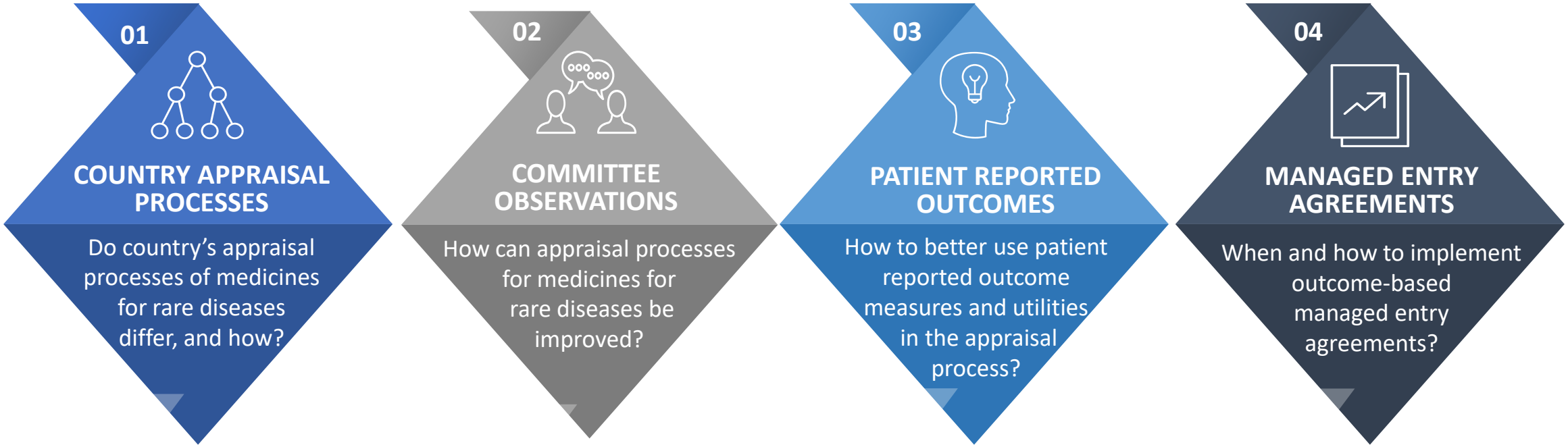
Overview of IMPACT-HTA and its Work Packages

- EC funded H2020 project under the call for improved methods for economic evaluation
- Developing ***methods, tools and guidance*** for decision-makers in the context of HTA and health system performance



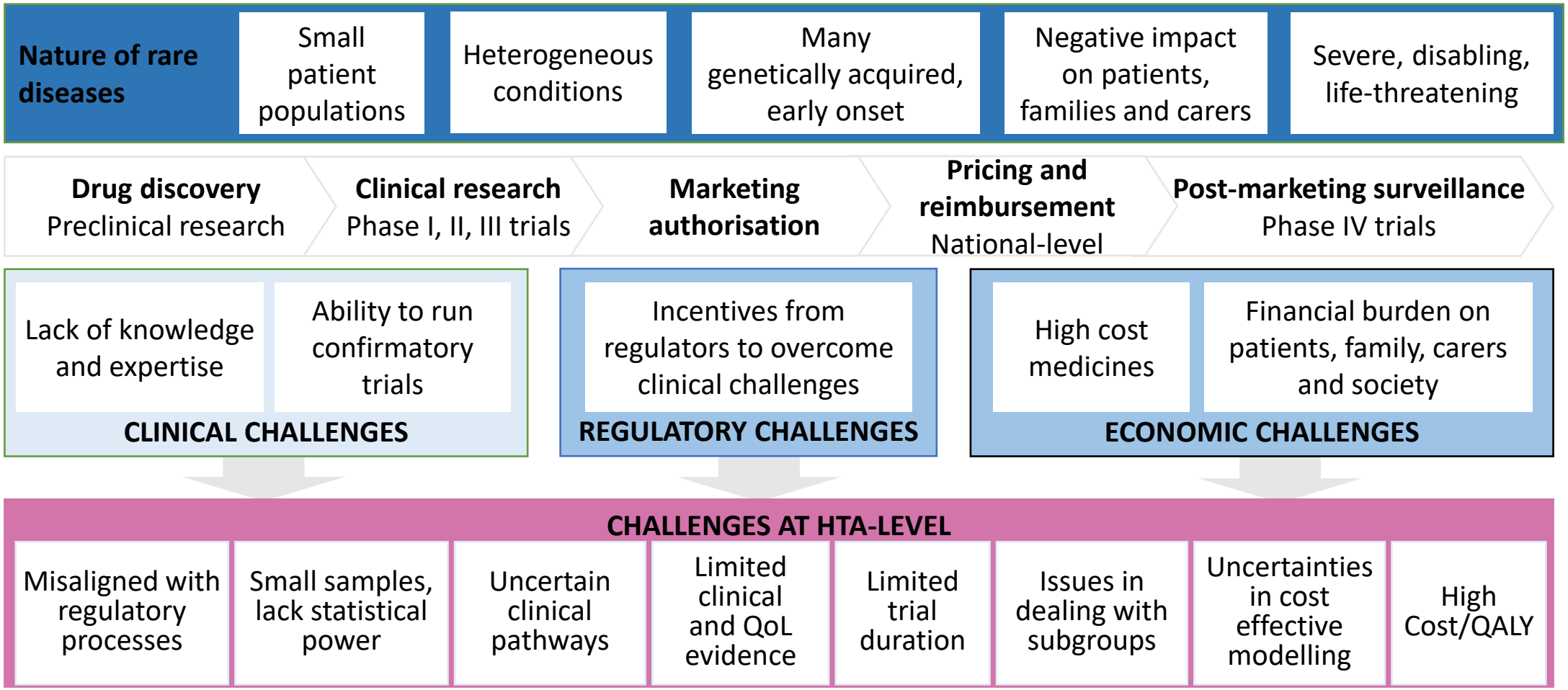
WP 10 on appraisal of rare disease treatments is developing an **Appraisal Framework** with associated toolkits from four different workstreams

Focus of this presentation



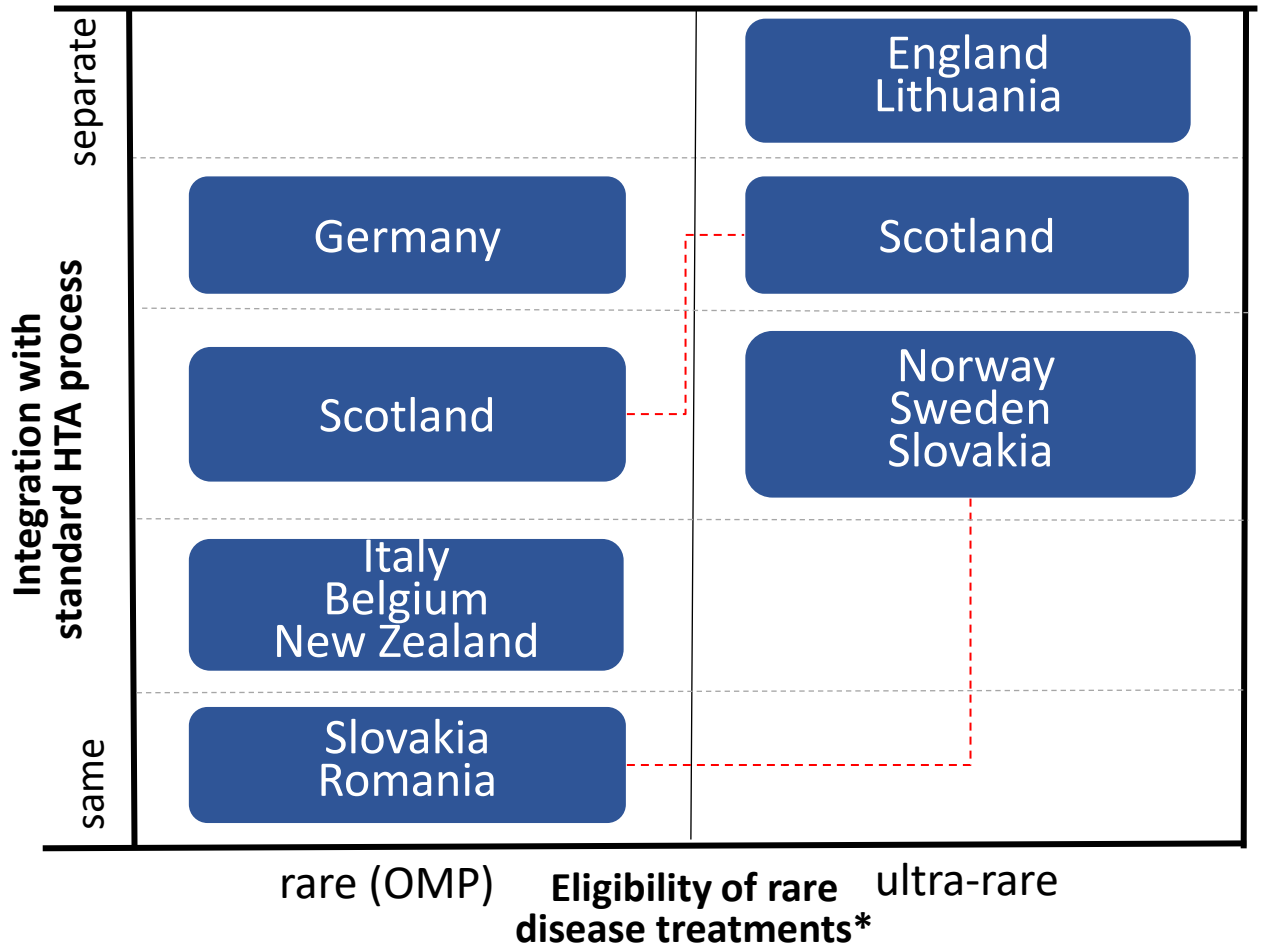
Publications + vignettes	Publications	Publications	Toolkits + Publications
https://www.impact-hta.eu/country-vignettes	Soon to be published	Published + soon to be published	soon to be published

The difficulties to develop medicines for rare diseases lead to HTA challenges



Source: Nicod E, Annemans L, Bucsics A, Lee A, Upadhyaya S, Facey K. HTA programme response to the challenges of dealing with orphan medicinal products: Process evaluation in selected European Countries. Health Policy, 2019

13 (41%) of the 32 participating countries have some form of special HTA appraisal process informing routine use of medicines for rare diseases



Separate

Partially separate

Adapted

Expedited

Rarity weighted

Exempt from HTA
Latvia, Bulgaria

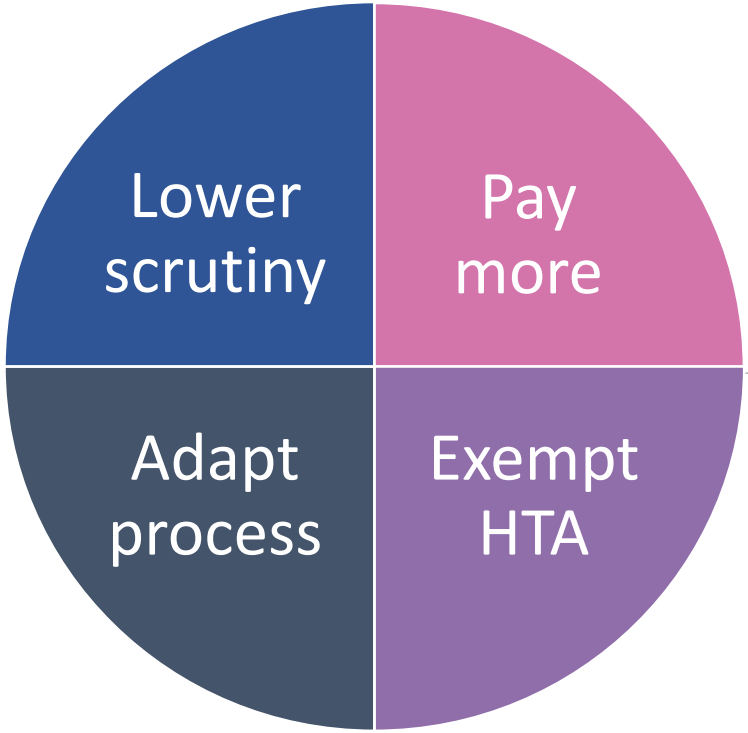
32/37 countries included:

- 41% special process
- 53% standard process
- 6% other programs applicable to rare disease treatments

* Rare disease treatment with orphan designation from European Medicines Agency ("Orphan Medicinal Product", OMP); ultra-rare disease treatments defined by individual country definitions

Source: Nicod E, Whittal A, Drummond M, Facey K. Are supplemental appraisal/reimbursement processes needed for rare disease treatments? An international comparison of country approaches. Orphanet Journal of Rare Diseases, 2020; 15:189
Country vignettes: www.impact-hta.eu/country-vignettes

These special processes include features allowing to better deal with the common challenges in rare diseases around uncertainty, price and delayed access



Decision modifiers

UNCERTAINTY

More leniency around quality of evidence

More flexibility in economic modelling

Different (appraisal) committees

Broader consideration of value

Disease-specific input to inform appraisal

Conditional approval

Earlier start*

PRICE

Different willingness to pay

Alternative reimbursement rules

Different formulary listing*

Different budget*

Alternative reimbursement rules*

Different requirements for clinical submission

Different requirements for economic submission

***DELAYED ACCESS**

Vignettes and results are published and publicly available


Nicod et al. Orphanet Journal of Rare Diseases (2020) 15:189
https://doi.org/10.1186/s13023-020-01462-0

Orphanet Journal of Rare Diseases

RESEARCH Open Access

Are supplemental appraisal/reimbursement processes needed for rare disease treatments? An international comparis country approaches

Elena Nicod[1*], Amanda Whittal[1], Michael Drummond[2] and Karen Facey[3]

Abstract

Background: There is increasing recognition that conventional appraisal approaches may be value rare disease treatments (RDTs). This research examines what supplemental appraisal/rein RDTs are used internationally and how they can be characterised. A qualitative research desig documentation of country appraisal/reimbursement processes for RDTs via questionnaires, de interactions with country experts to produce country vignettes, and (2) a cross-country analys identify and characterise features in supplemental processes for RDTs, and compare t supplemental processes.

Results: Thirty-two of the 37 invited countries participated in this research. Forty-one supplemental processes for RDTs. Their level of integration within standard processes high, characterised by whether they are separate or partially separate from the stand accelerated standard processes, or standard processes that may be applied to RDTs. features implemented throughout the appraisal process. These features are mechanis of different standards to assess the value of the medicine, support to the appraisal/d overcome the issues of lack of cost-effectiveness, or exempt from part of/the full ap process. They increase the likelihood of reimbursement by adjusting and/or foregoin process, or accepting to pay more for the same added benefit as for common cond of countries with standard processes include one or more of these features (formally discussing potential changes in their systems.

Conclusions: Results suggest revealed preferences to treat RDTs differently than con Some of the challenges around uncertainty and high price remain, but supplementa support decision-making that is more flexible and consistent. Many of these process continue to adjust as they gain experience.

Keywords: Rare disease treatment, Orphan medicine, Ultra-orphan medicine, Apprais to treatments, Supplemental processes, Health technology assessment

Results published in the **Orphanet Journal of Rare diseases**

Publicly available country vignettes hosted on the I**MPACT-HTA** and **Orphanet** websites

iMPACT HTA

Medicines to treat rare diseases benefit from adaptations to the appraisal process in order to better deal with rare diseases specificities

- **Revealed preference** to treat medicines for rare diseases differently (41% of 32)
- **Process features*** to better deal with challenges common in rare diseases in a **structured and consistent way**
- All issues not being dealt with via special processes, many are new and continue to be adjusted as **experience is gained**

*The next phase examined the key considerations to account for within the appraisal process to allow **decision-makers to make the best possible decision –** alongside possible implementation of these features identified*

*We do not go into the technicalities of these features (e.g. which modifiers, type of adjustment to WTP), but the paper does depict how these have been implemented across countries

WP10 published work

- Nicod E, Whittal A, Drummond M, Facey K. **Are supplemental appraisal/reimbursement processes needed for rare disease treatments? An international comparison of country approaches.** Orphanet Journal of Rare Diseases. 2021; 15:189
- Whittal A, Nicod E, Drummond M, Facey K. **Examining the impact of different country processes for appraising rare disease treatments: a case study analysis.** Int J Technol Assess Health Care. 2021 (under review)
- Country vignettes available at: https://www.impact-hta.eu/country-vignettes
- Meregaglia M, Whittal A, Nicod E, Drummond M. **'Mapping' Health State Utility Values from Non-preference-Based Measures: A Systematic Literature Review in Rare Diseases.** Pharmacoeconomics. 2020 Jun;38(6):557-574.
- Meregaglia M, Nicod E, Drummond M. **The estimation of health state utility values in rare diseases: overview of existing techniques.** Int J Technol Assess Health Care. 2020 Oct;36(5):469-473
- Whittal A, Meregaglia M, Nicod E. **The Use of patient-reported outcome measures in rare diseases and implications for health technology assessment**. The Patient. 2021 Jan (open access, in press)
- Facey K et al. **Implementing Outcomes-Based Managed Entry Agreements for Rare Disease Treatments: Nusinersen and Tisagenlecleucel.** PharmacoEconomics. 2021 (under review)
- Xoxi E, Facey K, Cicchetti M. **The evolution of AIFA registries to support Managed Entry Agreements for orphan medicinal products in Italy.** PharmacoEconomics. 2021 (under review)