International overview of appraisal systems for rare disease treatments

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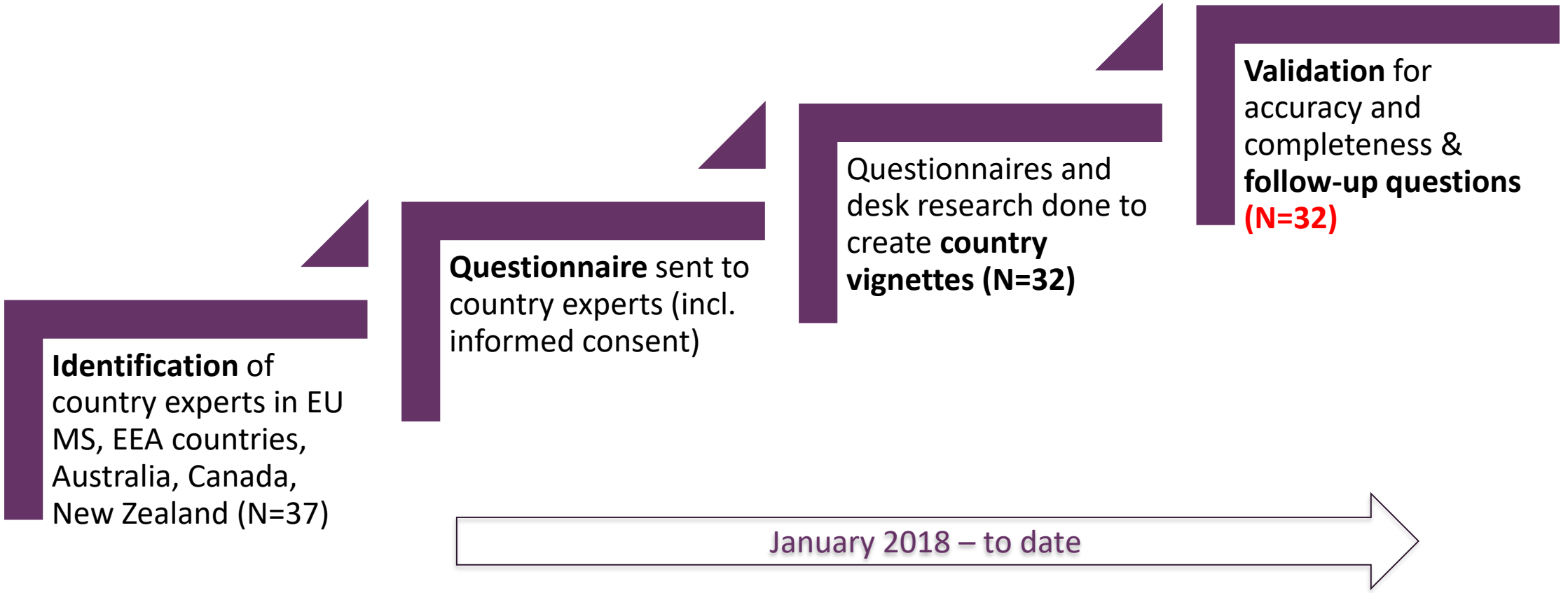
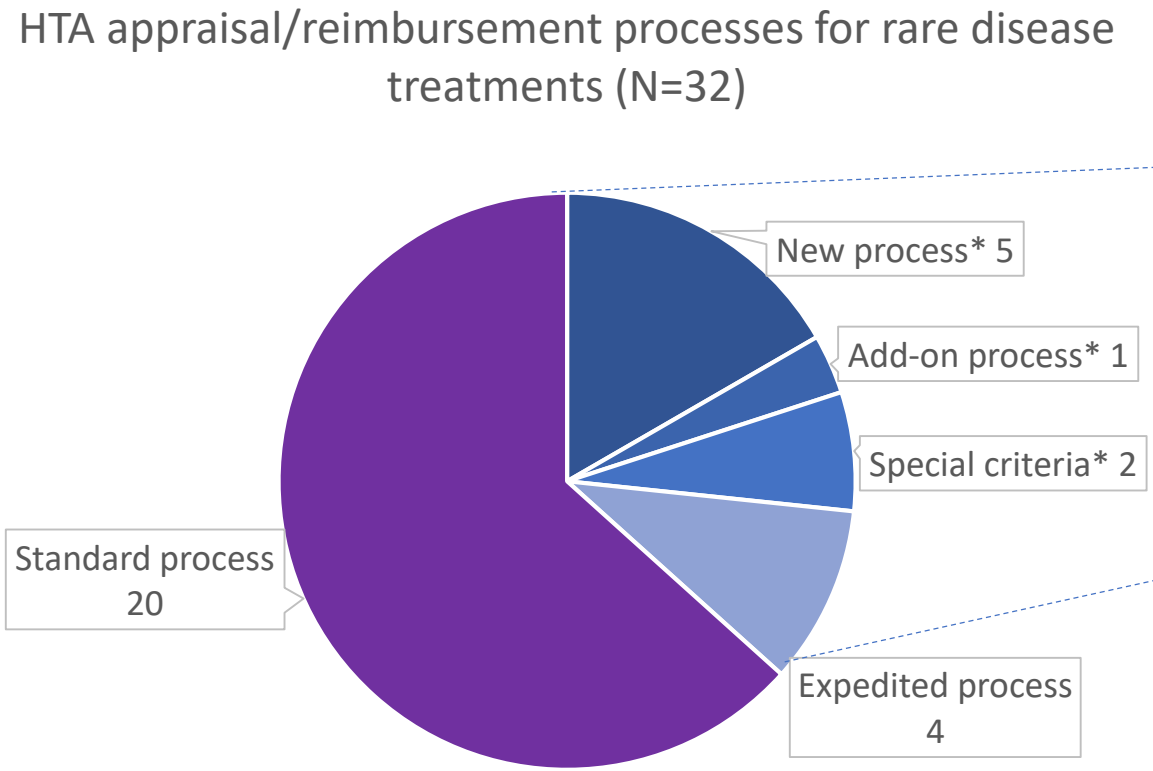
Objectives and process

Objectives: documentation of international rare disease appraisal and reimbursement processes



HTA appraisal/reimbursement processes for medicines to treat rare diseases in all EU Member States, EEA countries, Canada, New Zealand and Australia



	Rare	Ultra-rare
Special criteria		2/2
Add-on process	1/1	1/1
New process	5/5	3/5

* Referred to as **special processes** in this presentation

NB. Many countries have named-patient programmes, but this is out of the scope of this research,

(**Work in progress: results may change)**

Overview of country special processes for rare disease treatments

Ultra-rare conditions

ENGLAND
- Highly specialised technology : ultra-orphan drug framework, managed access agreements, higher willingness to pay and greater uncertainty

GERMANY
- Special reimbursement status (benefit automatically proven)

LITHUANIA
- Special reimbursement status (special positive list, therapeutic value not graded, special committee)*

ROMANIA
- Reimbursement based on points system (extra points for orphan drugs)

SLOVAKIA
- Greater willingness to pay based on points system (extra points for orphan drugs)

(Work in progress: results may change)

* Awaiting validation from country expert

Overview of country special processes for rare disease treatments

Ultra-rare conditions

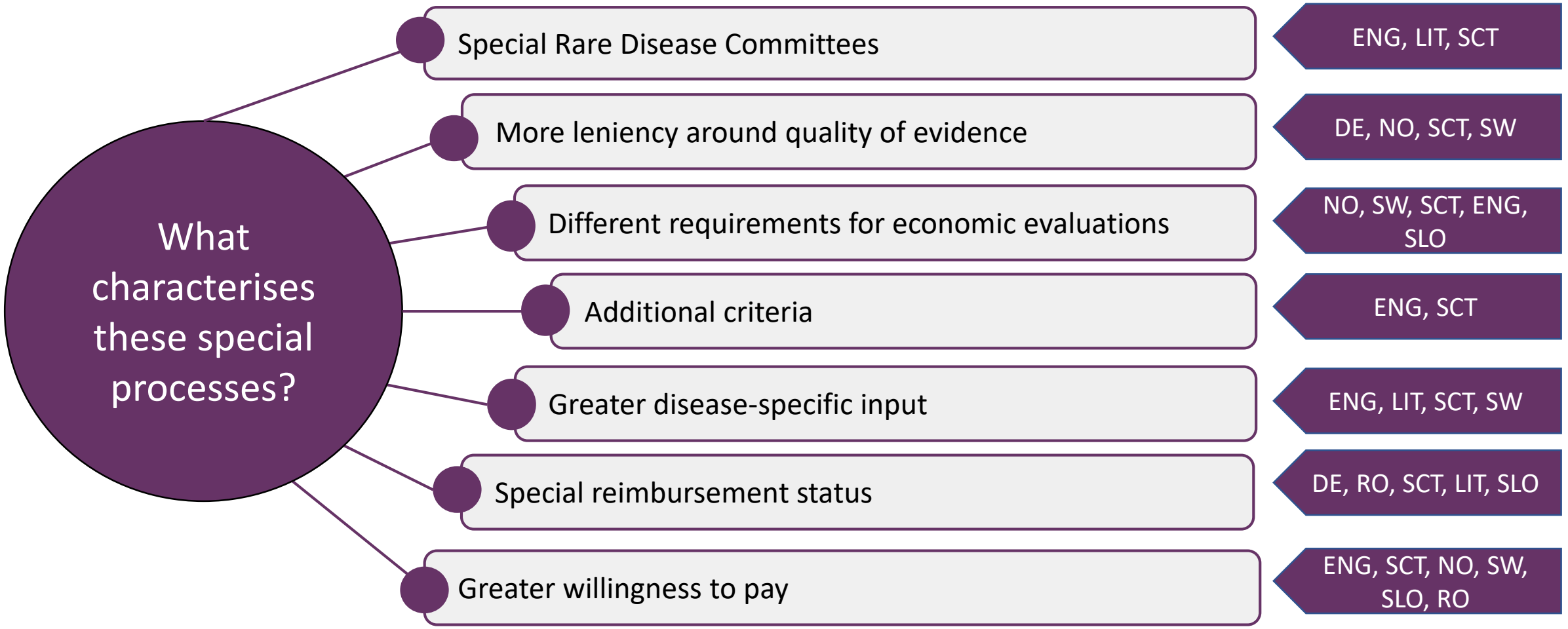
NORWAY & SWEDEN
- Higher willingness to pay and greater uncertainty

SCOTLAND
- Patient and Clinician Engagement process for rare and end of life treatments

- Ultra-orphan drug framework
- New ultra-orphan drug process (April 2019): 3 year monitoring

(Work in progress: results may change)

Overview of main differences with standard processes



(Work in progress: results may change)

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Challenges in appraising OMPs

CHALLENGES

- Lack of good quality clinical data (5)
- Lack of real world data
- Justifying value for money (unacceptably high ICERs)
- Monitoring treatment efficacy
- Managing budget impact
- Lack of criteria and transparency
- Making arrangements work for all stakeholders
- No long term meaningful outcomes

Stated impacts (countries with special processes)

- More OMPs applied for are reimbursed (5)
- Allows reasonable reimbursement considering limitations (2)
- OMPs privileged in reimbursement process (1)

IMPACT HTA

Thank you!

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