RARE-Bestpractices Conference

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Session 1: HTA, guidelines and orphan drugs
Stakeholder perspectives

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Understand HTA & challenges with orphan drugs

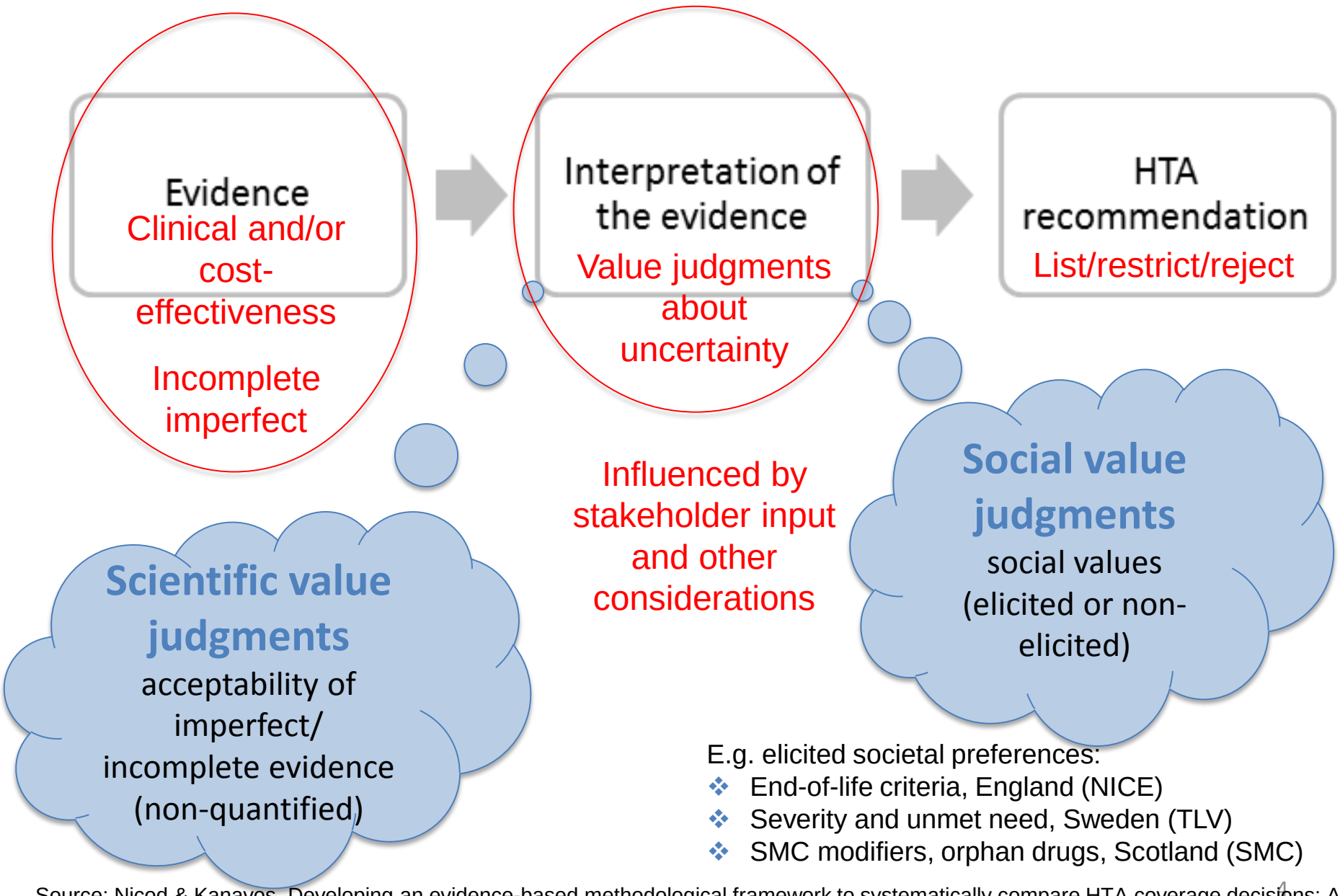
- Differences in HTA recommendations across countries
- Little differentiation of processes orphan and nonorphan drugs
- Uncertain cost-effectiveness of orphan drugs
- Rarity not sufficient to justify reimbursement

❖ ***Advance-HTA*** => systematically compared and identified the reasons for different HTA recommendations across countries

- Methodological framework
- 10 orphan drugs in England, Scotland, Sweden, France

SEVENTH FRAMEWORK PROGRAMME

Value assessment & reasons for differences

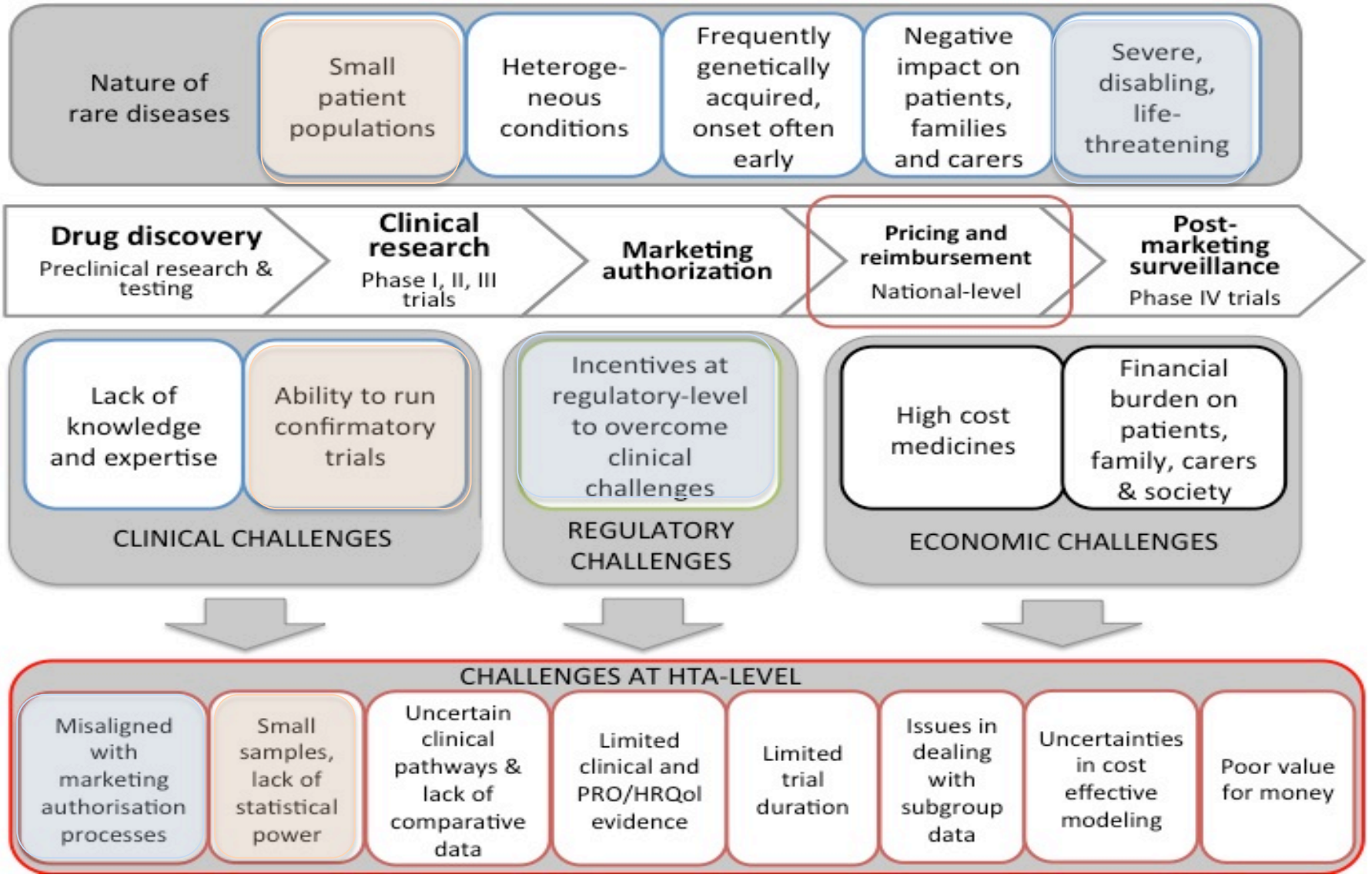


E.g. elicited societal preferences:

- End-of-life criteria, England (NICE)
- Severity and unmet need, Sweden (TLV)
- SMC modifiers, orphan drugs, Scotland (SMC)

Source: Nicod & Kanavos, Developing an evidence-based methodological framework to systematically compare HTA coverage decisions: A mixed methods study. Health Policy 2016

Conceptual framework of orphan drug development and assessment 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, 2016

Key messages

- Varying applications of HTA across settings
- HTA challenges with orphan drugs
- How rarity translates into challenges for HTA
- Impact on access to these drugs (differential access)

❖ What are the implications for clinical guideline development and best practices?

❖ Are best practices aligned with cost-effectiveness?

❖ How can we better align the steps made throughout the drug development pathway to ensure fair and optimal access to treatments? (=> life cycle approach)

THANK YOU FOR YOUR ATTENTION
Q&A

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