HPR81

Shining a Light on Unmet Health-related Needs: A Generic Framework to Advance the Identification and Assessment of Unmet Needs Tailored to Rare Diseases

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INTRODUCTION AND OBJECTIVES

Current healthcare system is primarily **supply driven**

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To move towards a more **needs driven** healthcare system, the Belgian Health Care Knowledge Centre (KCE) has developed the **NEED Framework** as a tool to identify and assess needs over three main domains (i.e., impact on patients, society, and the future)

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NEED Framework applicable to rare diseases?

→ To provide an overview of the **most pressing unmet needs** in the context of rare disease

→ To identify **methodological challenges** in research assessing unmet needs in rare diseases

METHODS

Rapid review

- Between May-June 2023
- PubMed and Embase
- 204 peer-reviewed articles were included

→ **Evaluation of existing criteria and methods to identify unmet needs in rare diseases**

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Expert meetings

- Two 2h workshops (N1=23, N2=33) between July and August 2023
- Stakeholders: patient and research organisations, HTA bodies, industry experts, policymakers, HCPs
- Thematic framework analysis

→ **Complement insights from rapid review**

UNMET NEEDS IN RARE DISEASES

Experienced by patients

- Timely and accurate diagnosis to prevent disease worsening
- Emotional and psychological support for patients' anxiety and depression
- Information about the RD, prognosis, and treatment options
- Integrated and holistic care with multidisciplinary approach
- Social support, such as peer support groups

Experienced by caregivers and family

- Appropriate healthcare to cope with uncertainties and burden of caregiving responsibilities
- Financial support due to changes in employment
- Information about the prognosis, treatment options and patients' complex care needs
- Emotional, psychological and social support for caregivers

Experienced by HCPs

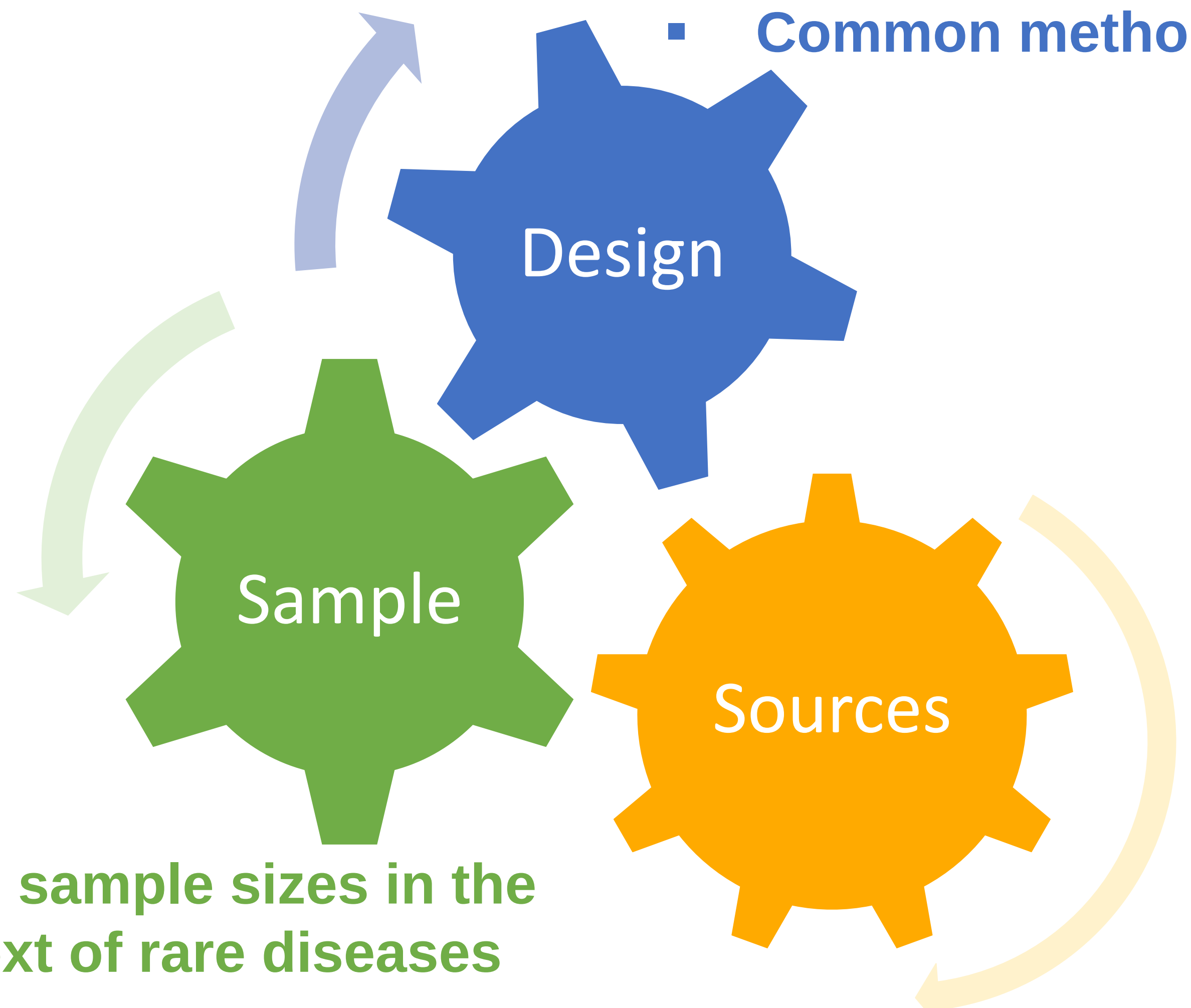
- Information about the RD, mechanism of action and treatment options
- Tools for an accurate value for money assessment

Experienced by society

- Awareness, understanding, and familiarity with RD

METHODOLOGICAL CHALLENGES

- **Memory bias or recall bias**
- **(Non-)Response bias**
- **Funding or sponsorship bias**
- **Common method bias**



- **Small sample sizes in the context of rare diseases**
- **Heterogeneous sample**

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Lack in generalisability

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Accurate recruitment strategy is necessary

- **Limited literature**
- **Limited disease-specific databased or rare diseases included in generic databases**

List of abbreviations

RD: Rare Disease, KCE: Belgian Healthcare Knowledge Center, NEED: Needs Examination, Evaluation, and Dissemination, HCP: Healthcare Professional, HTA: Health Technology Assessment, SOC: Standard of Care

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CONCLUSION

Patients, caregivers, HCPs, and society over various rare diseases report similar criteria to identify unmet needs

Research in rare diseases is challenging and accurate strategies towards design, sample and sources must be taken

Findings can guide future needs assessments in the context of rare diseases