



Navigating Methodological Challenges and Informed Consent in Paediatric Unmet Medical Needs Research: a Rapid Literature Review

Contact: charlotte.vanisterdael@kuleuven.be

Charlotte Van Isterdael^{1*}, Zilke Claessens^{1*}, Isabelle Huys¹

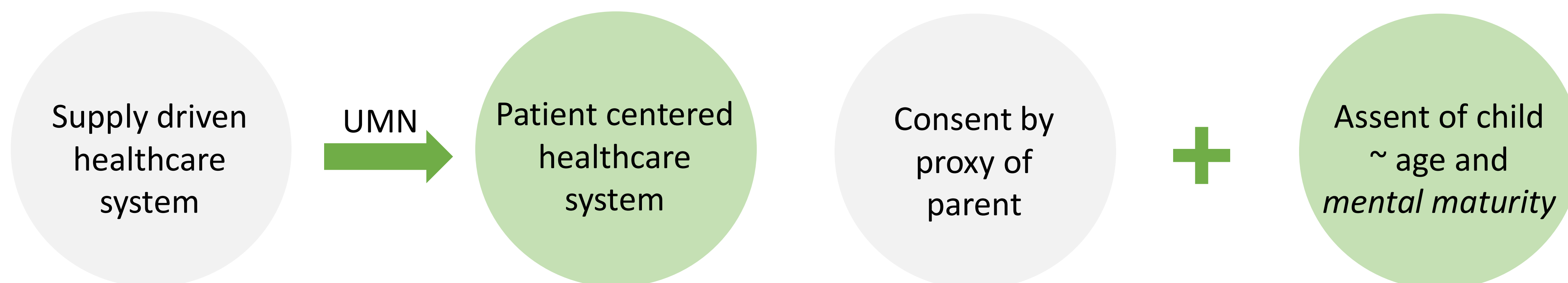
¹Clinical Pharmacology and Pharmacotherapy, Department of Pharmaceutical and Pharmacological Sciences, KU Leuven, Leuven (Belgium)

*Shared first authors

Background

Growing importance of unmet medical needs (UMN) in pharmaceutical R&D

Legal basis for informed consent (IC): Clinical trial regulation (Regulation (EU) No 536/2014)



Lack of insight into methodologies for UMN research in children and related informed consent practices

Investigators lack guidance on how to decide if the child is mature enough

Objectives



To investigate methodological challenges regarding UMN research in paediatric patients



To study informed consent (IC) models used in paediatric patients

Methods

Rapid literature review following Cochrane guideline on rapid reviews
Scientific databases: Pubmed and Embase

Concepts (combined with AND)

- 1 Methodologies
- 2 Unmet needs
- 3 Paediatric population
- 4 Blood cancer

Eligibility criteria

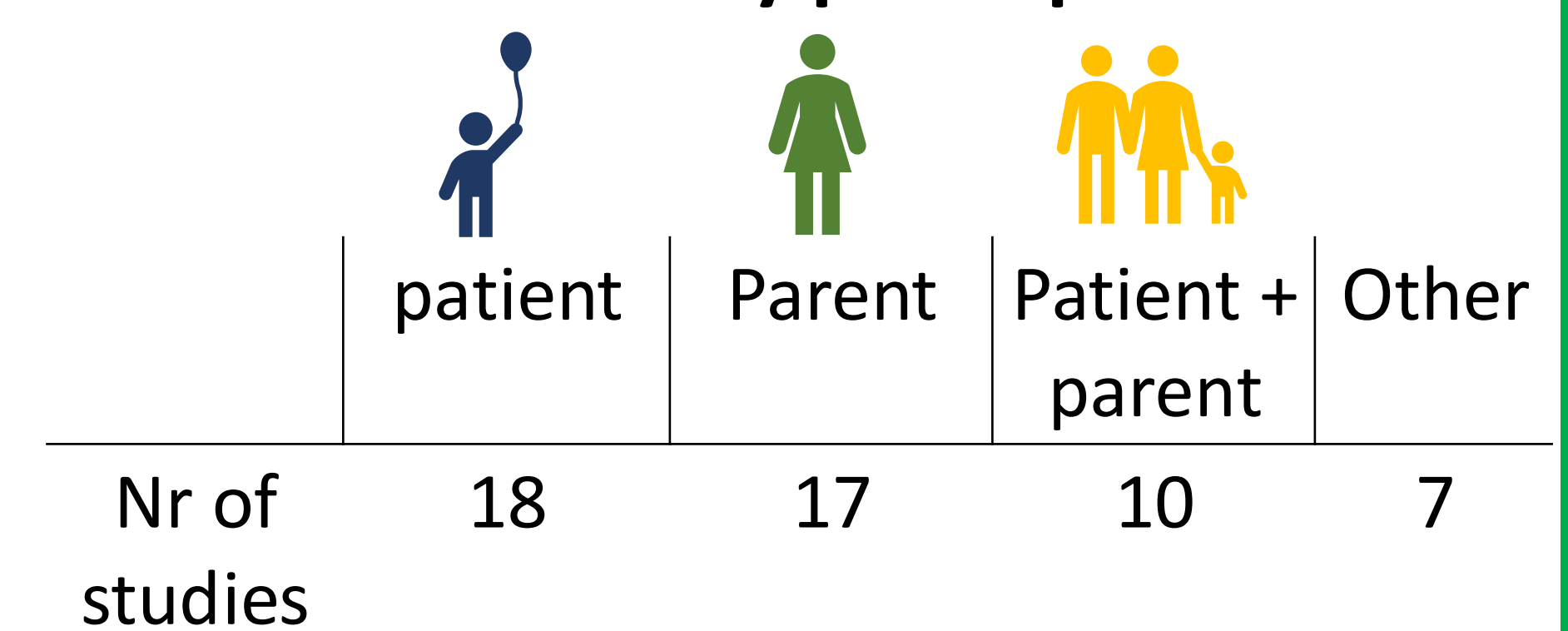
- Publication type
- Higher-income countries
- Blood cancer in children
- Methodological nature
- Unmet need assessment

Descriptive results

Research stage	Nr. of articles
After de-duplication	2727
After title/abstract screening	111
After full text screening	52

Method	Nr. of articles
Interview	26
Focus group	2
Questionnaire	16
Literature review	2
Mixed methods/other	5

Study participants



Methodological challenges and mitigation



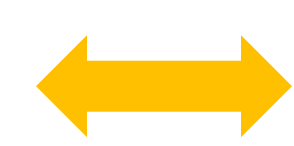
Difficulties in obtaining an age-representative sample: majority of participants in adolescent- and young adult (AYA) studies is older than 18 years



Limited value of parent-proxy alone without including the patients' view



Include patient with presence of proxy to complement the patients' view



Parental dominance during interview with patients

Increase awareness of situation, for both investigator, patient and parents

IC models in studies assessing needs of children

IC model

IC model	patient	Parent	Patient + parent
IC parent	N = 1	N = 11	N = 3
IC parent + assent patient	N = 2	/	N = 1
IC parent + IC patient	N = 4	/	N = 3
IC patient	N = 3	/	/
Not mentioned	N = 5	N = 6	/

! Age did not determine the differences in IC models across studies that include the same age groups

Conclusions

- 1 Many researchers still report challenges with conducting research in the pediatric population
- 2 There is a lot of variance in IC forms **across** studies involving paediatric patients within the same age ranges
- 3 **Within** a study, a single informed consent model is used, confirming that investigators find it hard to consider the mental maturity of participants

Additional guidance is needed to support researchers in the conduct of studies assessing paediatric unmet needs

- i. To enhance inclusion of younger participants
- ii. To generate consistency of methodological approaches (including IC)

Acknowledgement

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List of abbreviations

AYA: adolescents and young adults, IC: informed consent, UMN: unmet medical needs