MSR89

Criteria and methods applied to decision-making to embark on late-stage clinical trials with a multi-stakeholder perspective

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Quinten Health

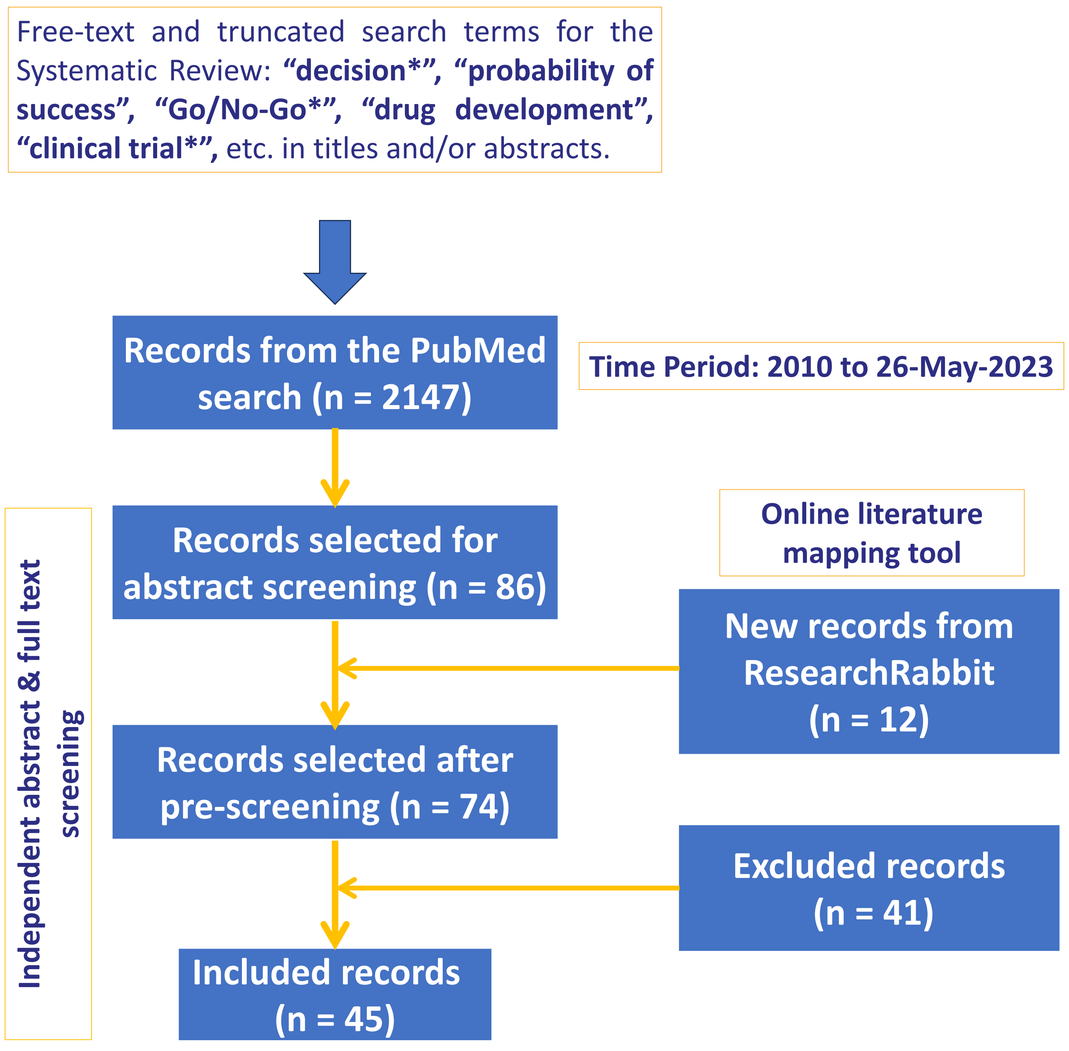
Introduction

- Registry data may be used to improve exploratory (pre-licencing phases) trial design across all phases of drug development.
- The improvements involve quantitative understanding of outcomes, modelling (natural) variability in disease course, **and identifying subgroups that may especially benefit from the experimental treatment.**
- Analysis of registry data can support in generating the evidence required for the decision-making to restrict or target a specific subgroup.
- Decision-making is based on mathematical modelling and simulation studies results.
- Additional criteria encompass economic, development, business, legal, health systems, patients' health outcomes, and scientific perspectives come into play.
- **Objective**: Identify criteria and methods for Go/No-Go decisions on whether to proceed with late-stage (phase III) trials considering multiple stakeholders' perspective.

Methods

- This research is part of the **More-EUROPA project**:
  - 15 public and private organizations from 7 EU countries.
  - Aims to **establish the value of registry-based RWD** in augmenting RCT data and **enabling effective and ethical use of registry data** to support patient-centered regulatory and HTA decision-making.

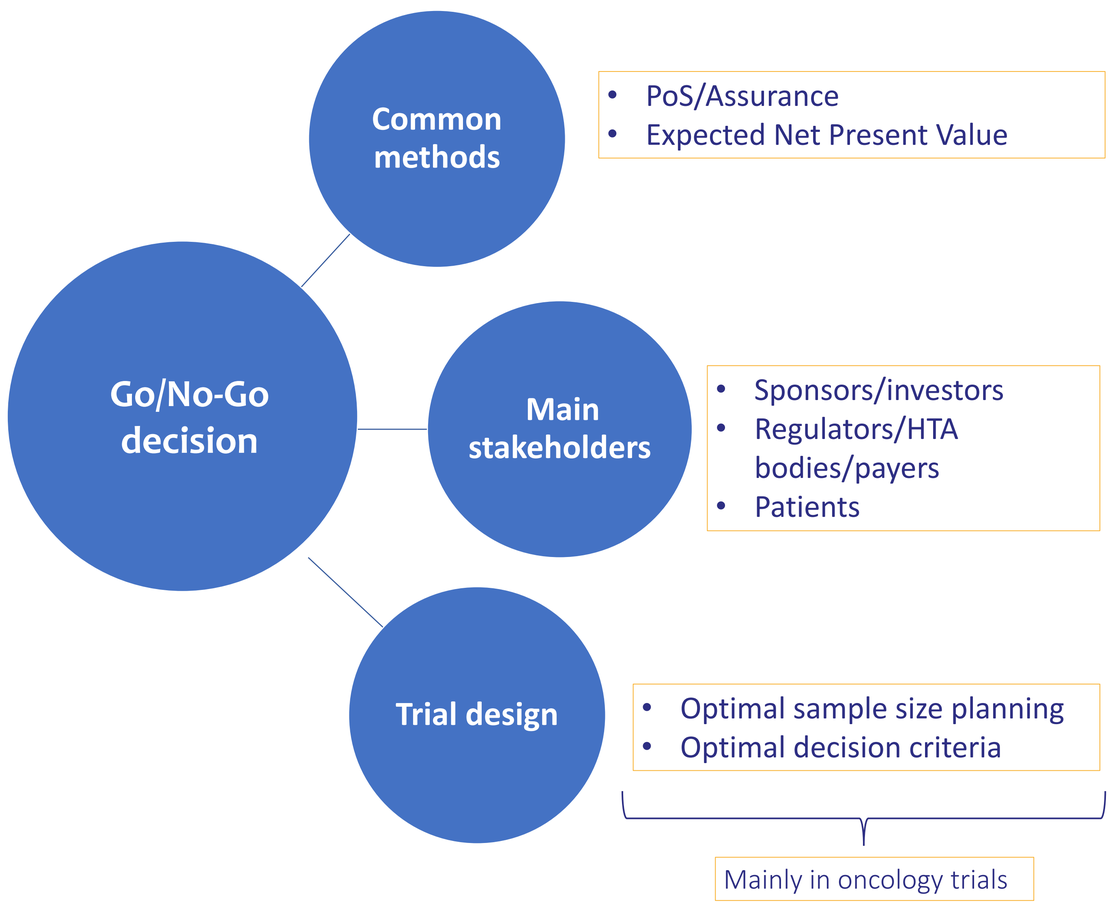
Figure 1: PRISMA diagram of the Systematic Review



Results

- **45** articles selected for full-text reading.
- A **limited number** of papers presenting the decision-making framework from a **comprehensive multi-stakeholder** perspective.
- **Lack of generalisation** for most of the identified papers: one or two stakeholders' perspectives considered with sometimes specific disease domain and/or trial setting.

Figure 2: Main pillars for Go/no Go decision-making



- Several **sources of uncertainties of the drug development programs** with different level of importance by **multiple stakeholders.**
- **Growing trend towards the use of registry data** and **machine learning** methods to support quantitative decision-making before pivotal trials.

Conclusion

- **The methods and criteria for evaluating the level of evidence and uncertainty to facilitate quantitative decision-making before late-stage trials are presently ambiguous, given the increasing employment of RWD and more complex trial designs.**
  - Identify **relevant methodologies** to support decision-making before late-stage trials from a **multi-stakeholder perspective.**
  - Need to include **more stakeholders' perspectives.**
  - **Trial design frameworks** in terms of optimal sample size planning and decision rules principally based on oncology setting with **potential extensions** to be developed.
  - All sources of uncertainty factors of the drug development programs should be considered when making the Go/No-Go decision.

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This project has received funding from the European Union's Horizon Europe Research and Innovation Actions under grant no. 101095479 (More-EUROPA). Views and opinions expressed are however those of the author(s) only and do not necessarily reflect those of the European Union nor the granting authority. Neither the European Union nor the granting authority can be held responsible for them.

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