Developing The Next Generation of Therapeutics: The Role of Early Model-Based Cost-effectiveness Analysis

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BACKGROUND

- What are Next Generation Therapeutics (NGTs)?
  - Transformative therapies in the prevention, management and cure of disease.
  - Advancement of technical methods, advanced delivery systems, or discoveries of new materials that aim to treat diseases more precisely and effectively, or for previously untreatable conditions (Figure 1).
- What is Early Model-based Cost-effectiveness Analysis (CEA)?
  - Economic evaluations to inform technology developers about a range of decisions relating to market assessment, reimbursement, go/no go, trial design and research prioritization.
- Aim of this review: to clarify the practical role of early economic evaluation in accelerating the development, optimisation, and informed decision-making for NGTs.

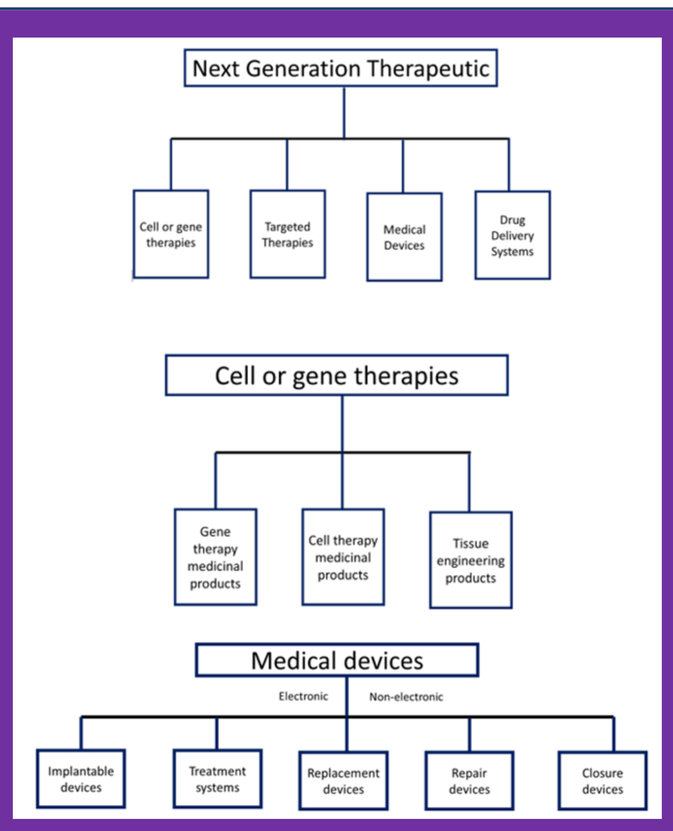

Figure 1: Types of NGTs

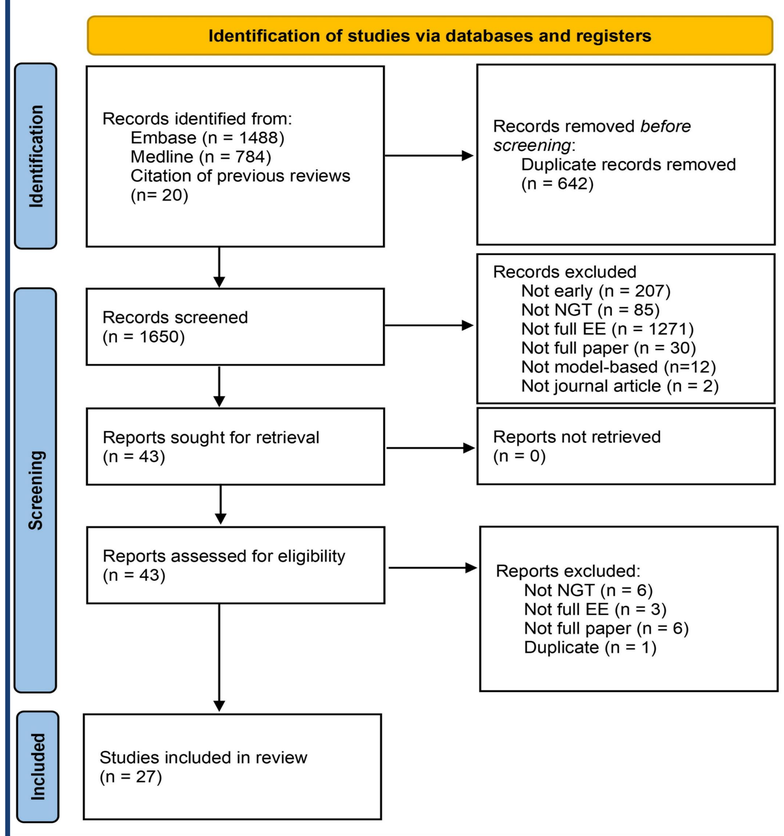

Figure 2: PRISMA Diagram

METHOD

- Search databases: Medline and Embase (PRISMA diagram see Figure 2).
- Time: Jan 2012 - Feb 2024.
- PICOS:
  - Population: any individual (adult or child)
  - Intervention: NGTs as defined by 11 topic experts
  - Comparator: relevant current or standard practice
  - Outcomes: costs, health consequences, incremental cost-effectiveness ratio, headroom, value of information
  - Study types: early model-based cost-effectiveness analysis.
- Narrative synthesis.
- Review protocol is available at PROSPERO: CRD42023425881.

RESULT

- Number of studies included: in total twenty-seven, 14 medicines and 13 devices (Figure 3).
- Where are they conducted: The Netherlands (10), UK (4), Germany (3), US (2), Italy (2), Australia (1), Canada (1), Denmark (1), Singapore (1), Spain (1) and Sweden(1).
- How to deal with novelty and uncertainty in clinical and cost evidence of NGTs:
  - Clinical effectiveness: non-structured expert elicitation, extrapolation form animal studies, earlier-generation devices, idealised effectiveness.
  - Cost: prices of similar interventions, innovator expectations and author assumptions.
- Quality of the studies:
  - Generally good as assessed by the Consolidated Health Economic Evaluation Reporting Standard (CHEERS) (Figure 4).
  - Limitation: lack of subgroup analysis and distributional effects, lack of engagement with patients in design.

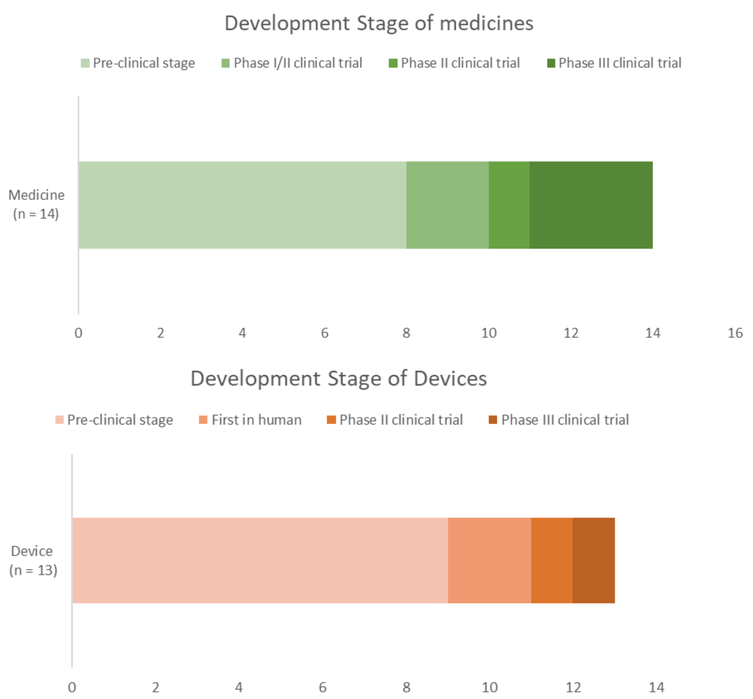

Figure 3: Development Stages of the NGTs Being Studied

DISCUSSION

- How can early model-based CEA help NGTs development?
  - Recommendation on follow-up time of future clinical trials based on one-way sensitivity analysis;
  - Prioritised parameter collection based on sensitivity analysis.
  - Recommendation on the potential value of future clinical trials based on Value of Information analysis (VOI);
  - Headroom of pricing.
- How to better use early model-based CEA for NGTs development in the future:
  - Early collaboration between innovators and health economists;
  - Transparent justification for model inputs and structured expert elicitation;
  - Conduct VOI following the CHEERS-VOI recommendations;
  - Regarding early model-based CEA as part of an iterative process throughout NGTs life cycle.

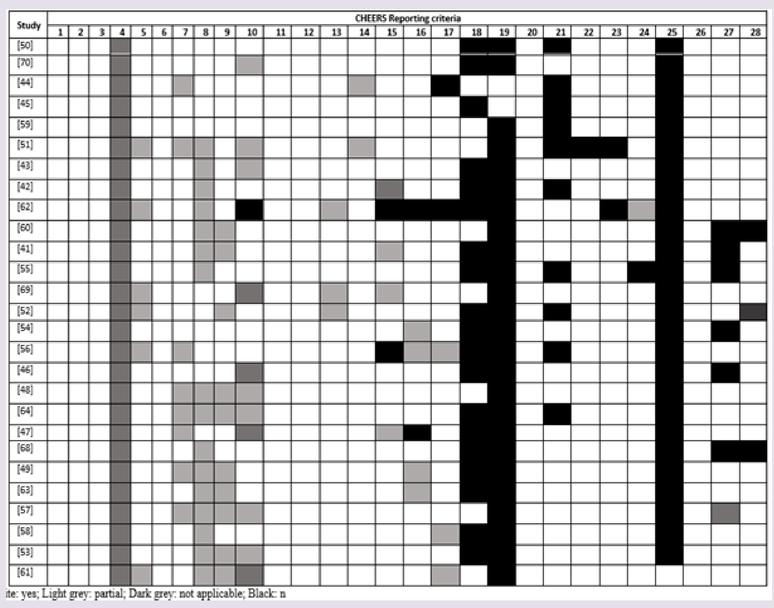

Figure 4: CHEERS Reporting Quality of Included Studies

CONCLUSION

Early model-based CEA contribute to subsequent development of the NGTs by identifying key uncertainties and informing future research priorities to help resource allocation decision-making. Sensitivity analyses and especially VOI techniques are essential in shaping future research and development by pinpointing the high-impact parameters needing additional evidence generated by specifically designed future research studies.

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