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Data Availability and Modelling Approaches in Health Economic Evaluation of Cystic Fibrosis: A Systematic Literature Review

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INTRODUCTION

- Cystic fibrosis (CF)is a hereditary, life-limiting condition most prevalent in Europe.
- CF is a rare genetic disease caused by a defect in the CFTR gene, leading to thickened mucus and lung damage.
- Although CF treatments have improved clinical symptoms and disease management, there is limited information on health state utility (HSU) associated with CF, including adverse events and disease severity changes.
- This systematic review aims to summarize existing HSU-related research in CF and identify research gaps and propose recommendations for future research in this area.

OBJECTIVE

- This study aimed to review the current state of health economic modelling in cystic fibrosis(CF), focusing on data availability and modelling approaches.
- The objective was to identify gaps and propose recommendations for future research in this area.

METHODOLOGY

Literature Search:
A systematic search was conducted on PubMed, Embase, and Google Scholar to identify relevant studies on health economic modeling in cystic fibrosis (CF).

Search Terms:
Various search terms, including "cystic fibrosis," "data availability," "health economic modeling," and "modeling approaches," were used to identify pertinent studies.

Review Inclusion:
A total of 153 studies were retrieved from the databases, and the final review focused on 22 studies that explored CF-specific health states, including pulmonary exacerbations, comorbidities (e.g., diabetes and liver disease), and health state utilities (HSU).
Additionally, the Preferred Reporting Items for Systematic Reviews and Meta-Analyses (PRISMA) guidelines were adhered to as a reporting standard during the process.

RESULTS

- 153 studies were obtained from the database, with the final review focusing on 22 studies exploring CF-specific health states.
- These aspects included pulmonary exacerbations, comorbidities such as diabetes and liver disease, and health state utilities (HSU). The methods and limitations of the identified studies were analyzed to determine the current landscape of CF health economic modelling.
- All included studies predominantly focused on a narrow range of CF health states while lacking comprehensive data on comorbidities and long-term complications.
- The inconsistent measurement of HSU and lack of consensus on the ideal instrument hindered comparability.
- The review exposed data gaps and the need for improved modelling in CF health economics.

Identified Gaps	Recommendations
Limited HSU Data	Collect Longitudinal HSU Data
Population Diversity	Utilize CF Trust Registry
Selective Clinical Trials	Mapping Studies
Inadequate Measurement of Adverse Events	Consensus on Measurement Tools
Lack of Consensus on Measurement Tools	Patient-Centered Research
Limited Mapping Studies	Comply with NICE Guidelines

CONCLUSION

- In conclusion, this study emphasizes the urgent need for improved access to data and more comprehensive modelling methods in CF health economic modelling.
- Future research should aim to incorporate CF-specific health states, comorbidities, and long-term complications in epidemiological models
- Exploring CF-specific instruments for measuring HSU and implementing mapping techniques to improve the availability of HSU can be considered.
- Addressing these research gaps will facilitate more accurate and informative cost-effectiveness evaluations of interventions for CF patients, there by supporting healthcare decision-making processes.

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CONFLICT OF INTEREST

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