Review and Evaluation of Health Equity Considerations in Health Technology Assessment in Cystic Fibrosis

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MAIN TAKEAWAY

There is a global need to improve consideration, incorporation, and reporting of health equity in health technology assessments in Cystic Fibrosis.

INTRODUCTION



Health technology assessment (HTA) bodies have the unique and influential role of evaluating health technologies evaluating health technologies to create policies that affect healthcare resource access and allocation.¹



Though many different health equity-related frameworks have been developed to support HTA bodies systematic incorporation of health equity into their decision-making structure, there is a lack of consensus on how to incorporate health equity measures.^{2,3}



A gap exists between considerations of health equity within health technology assessment and the implementation of HTA frameworks.⁴

OBJECTIVE

This review aims to describe the extent of health equity incorporation within global HTA evaluation of cystic fibrosis medications.

METHODOLOGY

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National HTA bodies were identified using the International Network of Agencies for Health Technology Assessment (INAHTA) List and independent assessment bodies (European Medicine Agency and Institute for Clinical and Economic Review).

Included HTA bodies had public reports in English on cystic fibrosis medications, identified using following search terms: cystic fibrosis, lumacaftor, elexacftor, tezacaftor, and ivacaftor.

Included submissions were reviewed utilizing the prespecified through targeted literature review,
Framework for Equity by Benkhalti et al. and the health equity key questions within each HTA evaluations were descriptively reported.⁴

- Some considerations included: stakeholders involvement, outcome measures and methodological approach.
- Additional data that does not fit within the scope of this identified framework will be qualitatively described and summarized.

RESULTS

- Of the 33 HTA bodies identified, 12 had at least one report on cystic fibrosis medication, but 9 were excluded due to the exclusion criteria 1) not public and/or 2) not in English.
- Three HTA bodies were included in the analysis, representing United States, European Union, and Canada.

Identification of HTA bodies via INAHTA (n=33)

Screening of HTAs via HTA bodies website search (n=12)

Excluded:
No website found

Excluded:
No public HTAs
No public HTAs
Not in English

Included from framework

Potential recommendations

based on excluded domains

from framework

(2020)Formal questionnaires and FDA reports

Institute for Clinical and Economic Review

- used for patient insight
 Engagement of patient/advocacy groups to illuminate impact to all aspects of life
- Technologies in Health (2018)

 Patient group submitted informati

Canadian Agency for Drugs and

- Patient group submitted information about outcomes and issues important to patients and caregivers
 - Advocacy group engaged
- Not evident: however, patient and

European Medicines Agency (2020)

- organization inclusion framework exists
- **DIMENSIONS OF EQUITY:** Equity concerns and goals using patient perspectives for different dimensions of health equity should be addressed in all phases.
- EQUITY ANALYSIS: Economic analysis should include health equity considerations.
- ACCESS & IMPACT: Medication access and impact on patient outcomes should be thoughtfully assessed and accounted for, when possible.
- TRANSPRENCY: As it is unclear whether considerations were given at different phases (scoping/priority setting, evaluation, recommendations/conclusion, etc.). If a general HTA body specific health equity guideline/framework exists and was utilized, it should be explicitly referred to in the document.
- ETHICAL & LEGAL: At all times, historic, present, and future ethical and legal contextualization should be carefully considered.
- Canada and US included patient engagement and advocacy considerations.
- There was variable to no consideration of medication access and its impact on outcomes.
- No health equity methodological considerations were identified.
- Among the identified HTA reports, there were significant gaps in the level of incorporation and implementation of health equity considerations.

CONCLUSION

- The study underscores the need to address gaps in the application and standardization of health equity principles and frameworks.
- This challenge is further exacerbated by variabilities amongst different countries in terms of their health equity guidance, if any exists.
- There is also a need for collaboration to improve health equity related data generation necessary for including in the HTA
- By shedding light on the challenges and opportunities specific to cystic fibrosis, this study provides valuable insights for enhancing the integration of health equity in HTA assessments, especially in diseases with high unmet needs.

LIMITATIONS

- Exclusion of non-English reports significantly reduced the sample size.
- Scope was limited to what is published publicly on respective HTA websites, so it is possible that documents addressing health equity measures may not have been included in this study.
- This study only evaluated CF, so the study finding may not be generalizable to other disease states.
- This study may not reflect recent advances in health equity in HTAs, as health equity improvements more recently gained rapid traction due to the COVID-19 pandemic highlighting stark health disparities.⁵

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DISCLOSURES

LYL, SV, and SS are employed by Rutgers University. ZMK is Director of Rutgers Center for Health Outcomes, Policy & Economics and Founder and CEO of Zebgene LLC.

Abbreviations CF: cystic fibrosis, HTA: health technology assessment, INAHTA: International Network of Agencies for Health Technology Assessment, COVID-19: coronavirus disease 2019