



# Leave No Value on the Table

Addressing Health Inequities in Rare Disease HTA

# **What are HTA bodies saying about equity?**

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# Health equity in HTA

**NICE** National Institute for  
Health and Care Excellence

## B. 1. 4. Equality considerations [HST company evidence submission template]

*Provide an assessment of whether the use of this technology is likely to raise any equality issues.*

# Health equity in HTA

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## B. 1. 4. Equality considerations [HST company evidence submission template]

*Provide an assessment of whether the use of this technology is likely to raise any equality issues.*

## B. 1. 4. 3. [company evidence submission user guide]

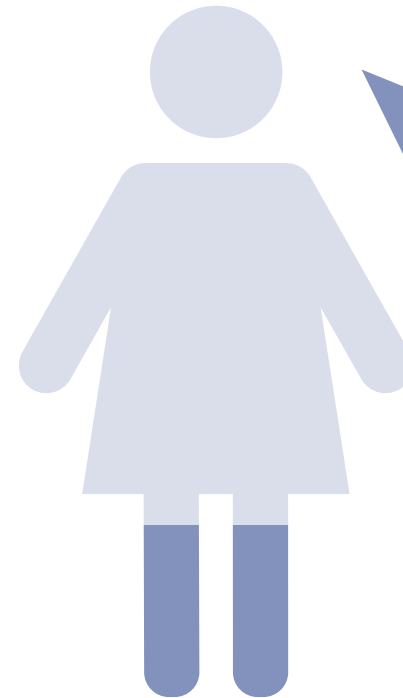
*Please provide any evidence that would enable the committee to identify and consider the impact of equality issues. State how the analysis has addressed these issues.*

# Is equity even important in rare disease?

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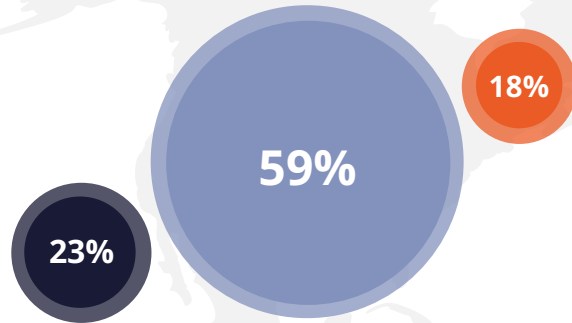
# Why address inequity in HTA? Does it even matter?

Do health inequities actually impact reimbursement decisions?



Isn't most inequity in rare diseases due to factors that payers and manufacturers have no control over?

# Equity considerations across HST appraisals

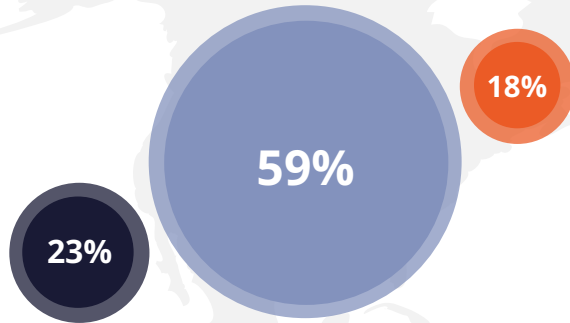


## EQUITY IN HST APPRAISALS\*

- Most (59%) had equity considerations noted at scoping or consultation
- 23% had equity issues raised **only** by external stakeholders
- 18% had **no** equity issues raised by committee or stakeholders

01

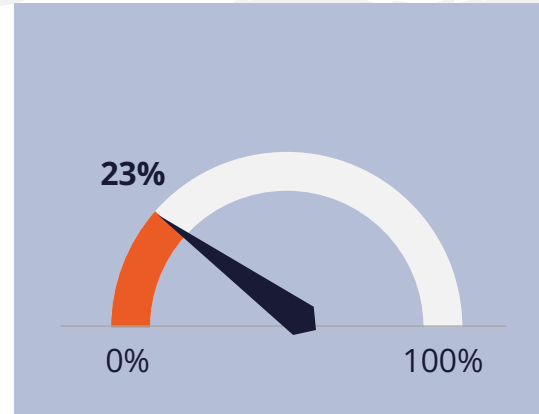
# Equity considerations across HST appraisals



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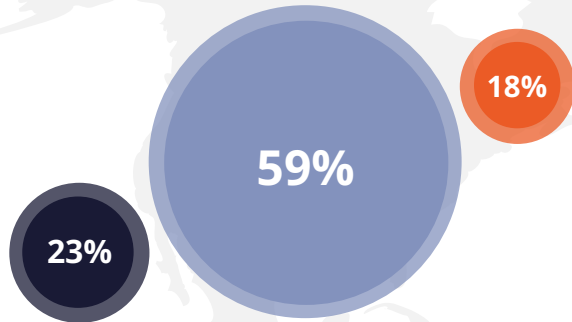
## EQUITY THAT MOVED THE NEEDLE

- 23% of HST appraisals\* noted that decision-making was impacted based on an equity consideration

02



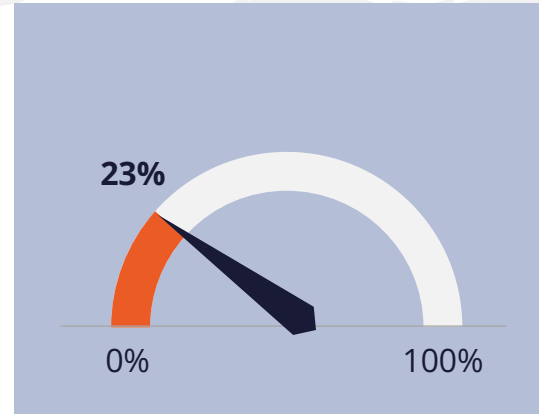
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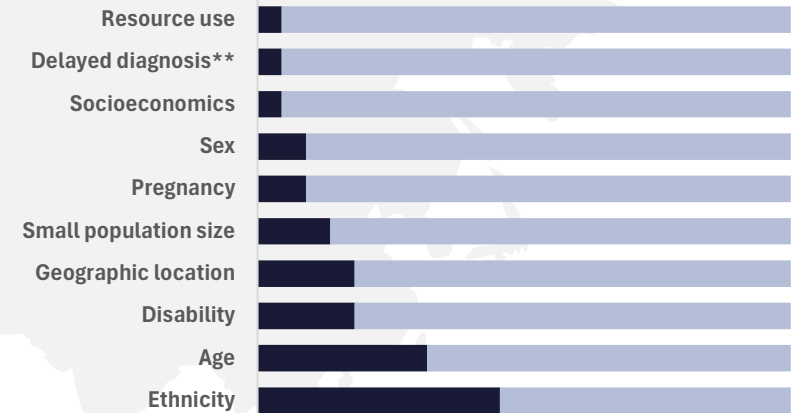
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## EQUITY THAT MOVED THE NEEDLE

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02



## EQUITY ISSUES

- Most **common** equity issues raised were:  
**Ethnicity Age**  
**Disability Geographic location**
- Most **effective** equity issues raised were:  
**Age Disability**  
**Resource use Delayed diagnosis**

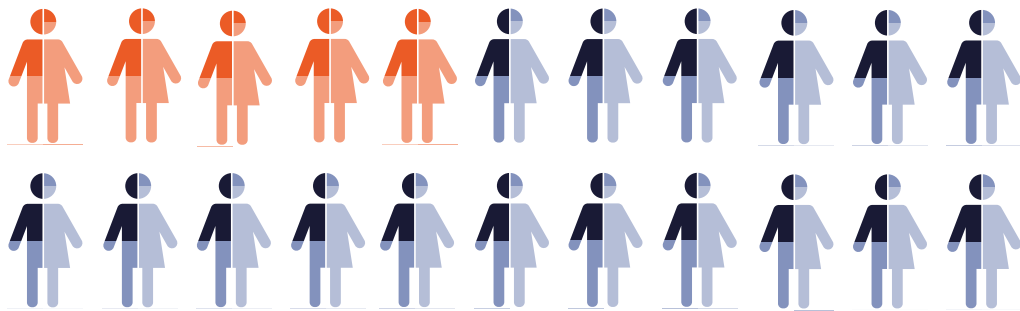
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# Stakeholders who moved the needle



5

HST appraisals were influenced by equity issues in the target population



3 of 5 had equity issues identified by the committee at scoping/consultation

2 of 5 had equity issues identified by external stakeholders

External stakeholders can move the needle

Company    Clinical experts    Patient groups

# How do you contextualise and quantify equity?

# How do you contextualise equity?

## **Early patient and stakeholder engagement**

- Ensuring their perspectives are reflected in the economic value story

## **Equity messaging throughout the submission**

- Potential impact on disadvantaged populations, the inclusivity of the clinical trial design, and the expected outcomes in terms of reducing health disparities
- Acknowledge any data limitations, such as small sample sizes or lack of representation of certain groups, and discuss how these limitations might affect the assessment of health equity



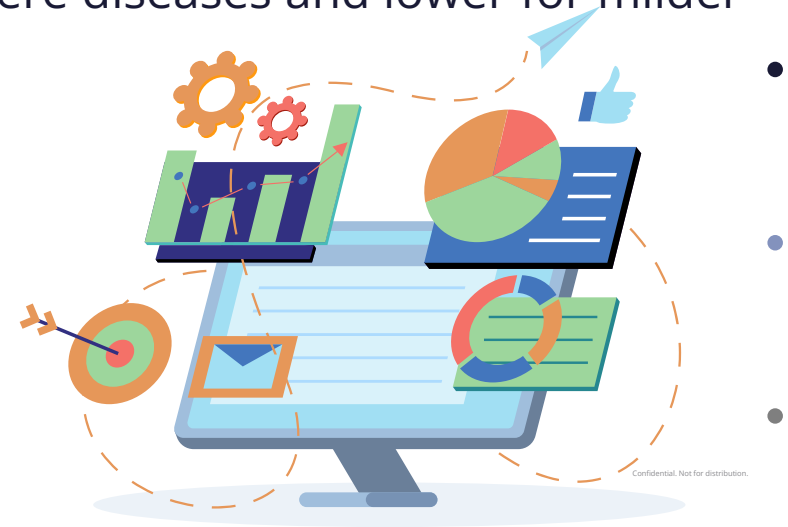
# How do you quantify equity?

## Equity-informative economic evaluation

- Distributional Cost-Effectiveness Analysis (DCEA)
  - Helps to identify interventions that not only improve overall health but also reduce health disparities
- Generalized Risk-Adjusted Cost-Effectiveness (GRACE)
  - Adjusts cost-effectiveness thresholds higher for severe diseases and lower for milder ones

## Scenario and subgroup analyses

- Identifying populations where the new intervention is more efficacious or
- Broader societal perspective, e.g. travel costs



# How do you move the needle?

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# How to integrate equity into your access plan



## Early strategic planning

- ▶ Data capture (effectiveness, resource use) via:
  - Compassionate use programs
  - Studies in patient populations of interest
  - Landscape assessments & literature reviews
- ▶ Ensure trials include relevant outcomes in population of interest
  - Be aware of equity issues for patients (eg, do studies need to capture disability data, specific AE data, or PROs?)



## Stakeholder engagement

- ▶ Needs assessments with patient advocacy groups
  - Identify specific AEs
  - Patient subpopulations with substantial unmet need
  - HRQoL burden
  - Level of disability
- ▶ Clinician surveys & disease awareness tools



## Health economics

- ▶ Equity-informative economic evaluations
  - Distributional Cost-Effectiveness Analysis (DCEA)
  - Generalized Risk-Adjusted Cost-Effectiveness (GRACE)
- ▶ Scenario analyses
  - Subgroups
  - Broader societal perspective



## Value strategy

- ▶ Publications:
  - Patient and caregiver vignettes
  - Resource use studies
- ▶ Patient journey infographic
- ▶ Consistent messaging
  - Highlighting assumptions and limitations due to inequities



**Do you know which inequities are impacting your current products?**

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