

The Impact of Rarity in NICE's Health Technology Appraisals

Sophie Clarke (✉ sophie.clarke@pfizer.com)

Pfizer Ltd <https://orcid.org/0000-0001-9236-9013>

Michelle Ellis

Pfizer Ltd

Jack Brownrigg

Pfizer Ltd

Research

Keywords: Orphan medicine, Rare disease, Population size, NICE, HST, STA, ICER thresholds

DOI: <https://doi.org/10.21203/rs.3.rs-226510/v1>

License:   This work is licensed under a Creative Commons Attribution 4.0 International License.

[Read Full License](#)

Abstract

In the absence of a framework designed to evaluate medicines for rare diseases in the UK, most orphan medicines are appraised by the National Institute for Health and Care Excellence (NICE) through the Single Technology Appraisal (STA) process. An analysis of STA appraisals of orphan and non-orphan medicines revealed that orphan medicines were subject to a significantly longer mean time in the NICE process than non-orphan medicines (370 days (n=44) vs 277 days (n=118), $p < 0.0001$). A higher proportion of orphan STAs required more than one Appraisal Committee Meeting (ACM) vs. non-orphan STAs, and orphan STAs were disadvantaged by worse outcomes with respect to positive recommendations than those orphan medicines assessed by Highly Specialised Technology evaluation (HST). The uncertainties inherent to developing orphan medicines may contribute to these disadvantages. Improved understanding of the challenges in drug development for orphan medicines and clearer guidance for decision makers on navigating uncertainty in the HTA process may promote greater equity in access to medicines across rare and common conditions.

Full Text

Due to technical limitations, full-text HTML conversion of this manuscript could not be completed. However, the manuscript can be downloaded and accessed as a PDF.

Figures

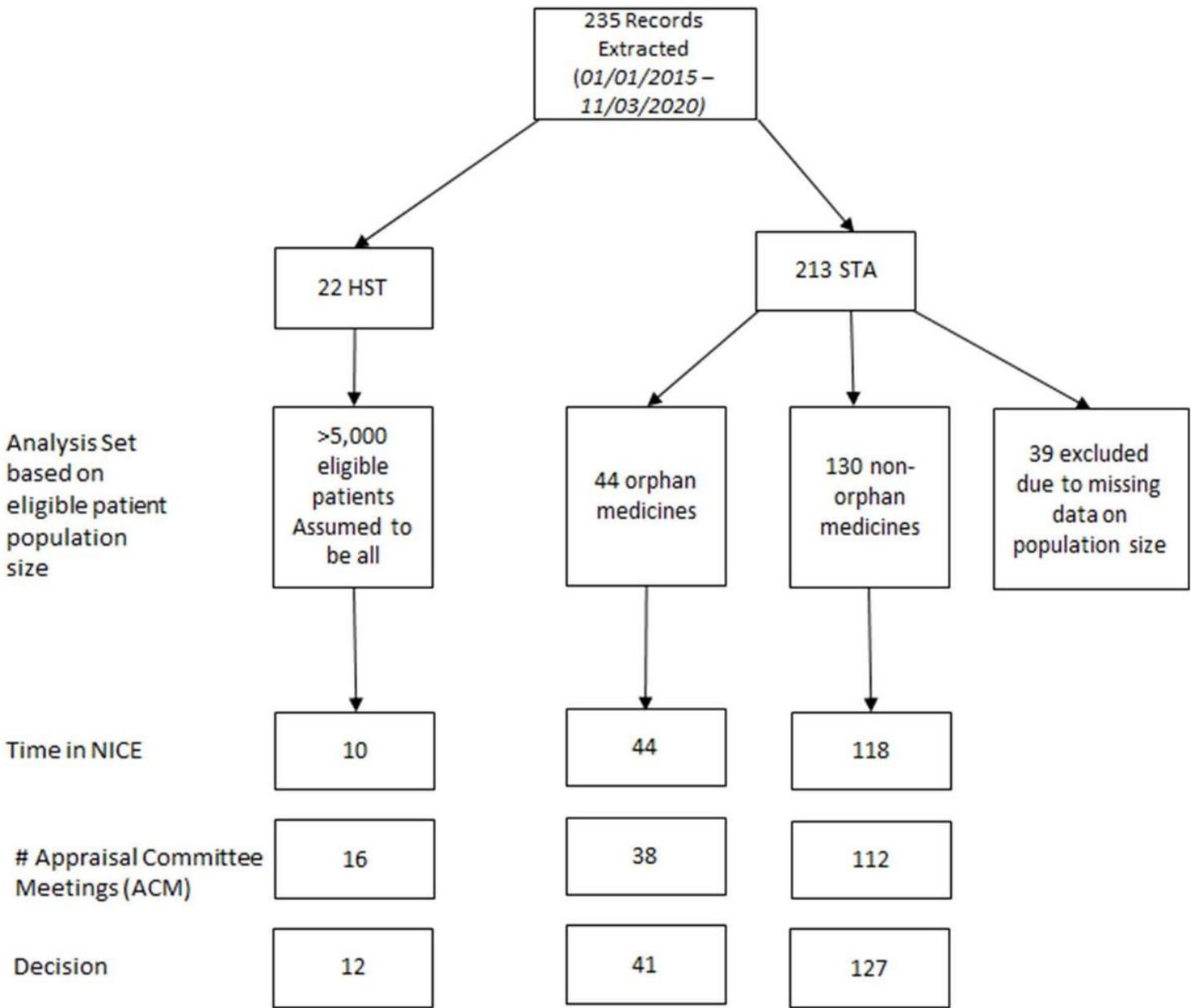
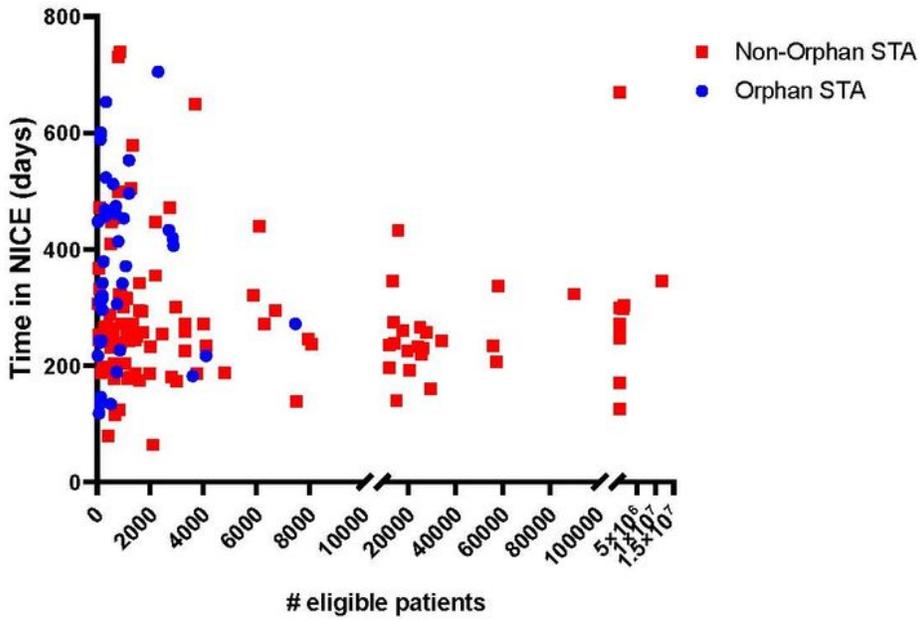


Figure 1

Prisma illustrating the analysis set of included STA and HST appraisals

A



B

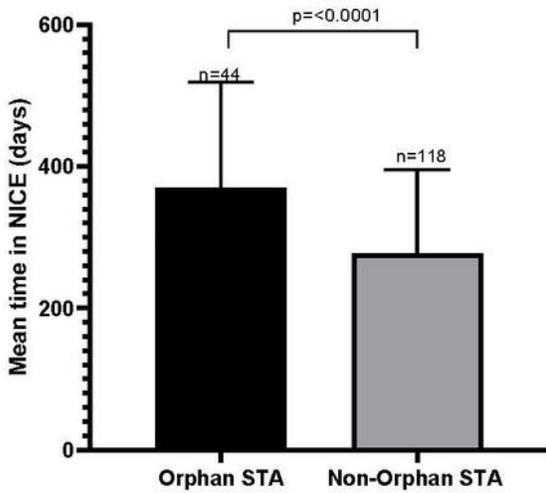


Figure 2

A: Time in the NICE process by eligible population size and orphan status. B: Mean time in NICE was significantly higher for orphan STA vs. non orphan STA appraisals.

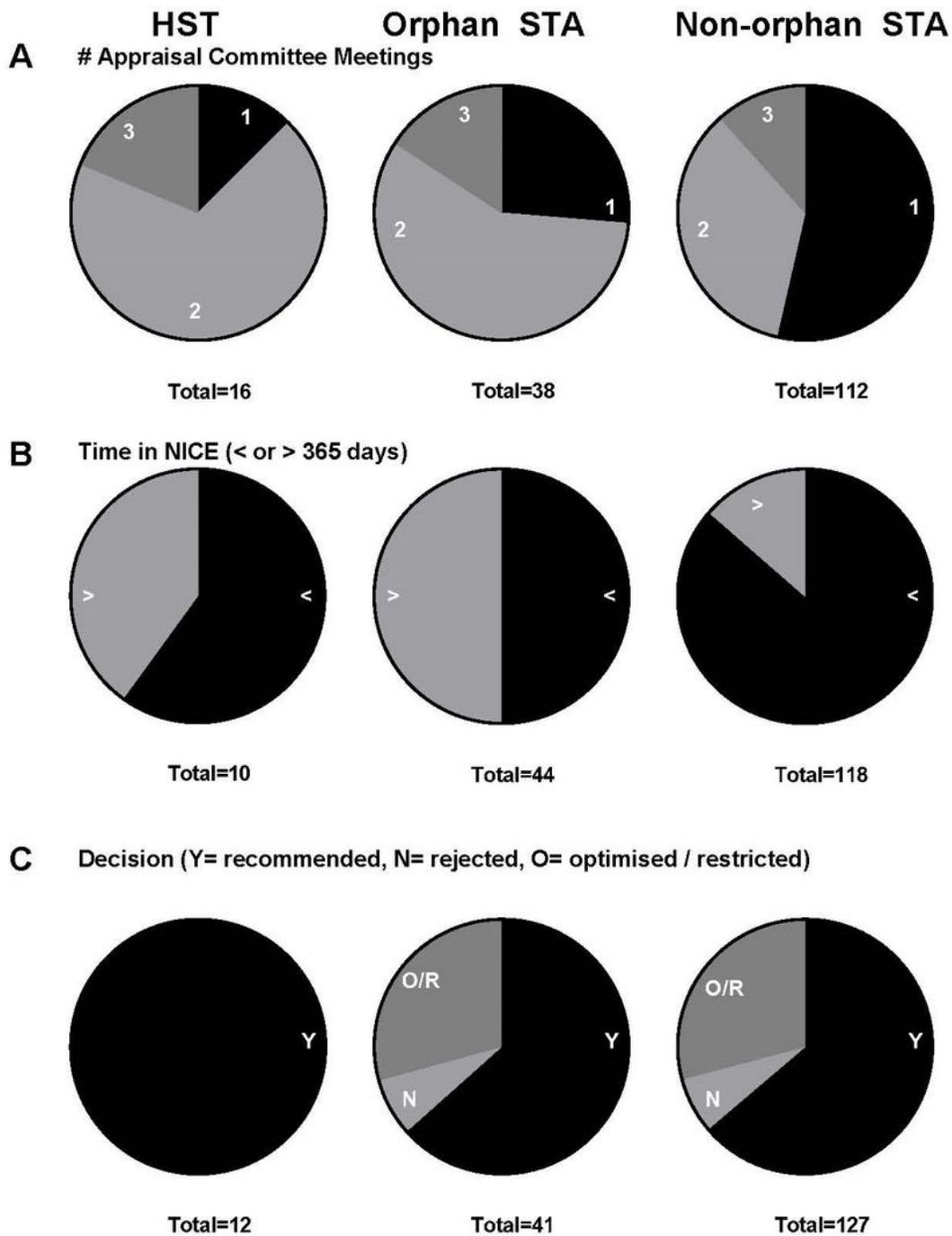


Figure 3

Descriptive analysis of HST, orphan STA and non-orphan STA appraisals A: Proportion of appraisal committee meetings, B: Proportion of appraisals with time in NICE < or > 365 days, C: Decision for completed appraisals