



OBJECTIVES

- Evidence across various categories, from disease burden to management, particularly for ultra rare diseases, is generally quite low, and healthcare technology assessment (HTA) bodies operate in a highly uncertain environment.
- Given evidence scarcity, there may be a higher reliance on qualitative data for value assessments and communication.
- As such qualitative evidence particularly experiences of patient, caregiver and clinicians are becoming increasingly important in HTA evaluations.
- We sought to understand how qualitative methods are used and how key HTA bodies like NICE, HAS, G-BA, and CDA (CADTH) consider such qualitative data presented in submissions.

METHODS

- We focused our examination on products developed for ultra-rare diseases and as such started with products that were evaluated by NICE under the highly specialised technology (HST) pathway.

20 products

Underwent NICE evaluations under HST pathway since 2020
**22 indications and evaluations*

NICE

HAS (TC)

G-BA

CDA / CADTH

HTA evaluations for these products by NICE, HAS, G-BA, and CDA/CADTH were considered, including any publicly available committee papers and submission / guidance documents.

- A systematic review was conducted to assess the use of qualitative data, data sources, and methodology; evidence categories in which these were used, and their considerations in the respective appraisals.

RESULTS

- For the 20 products identified, evaluations were available for:



22 evaluations



21 evaluations*



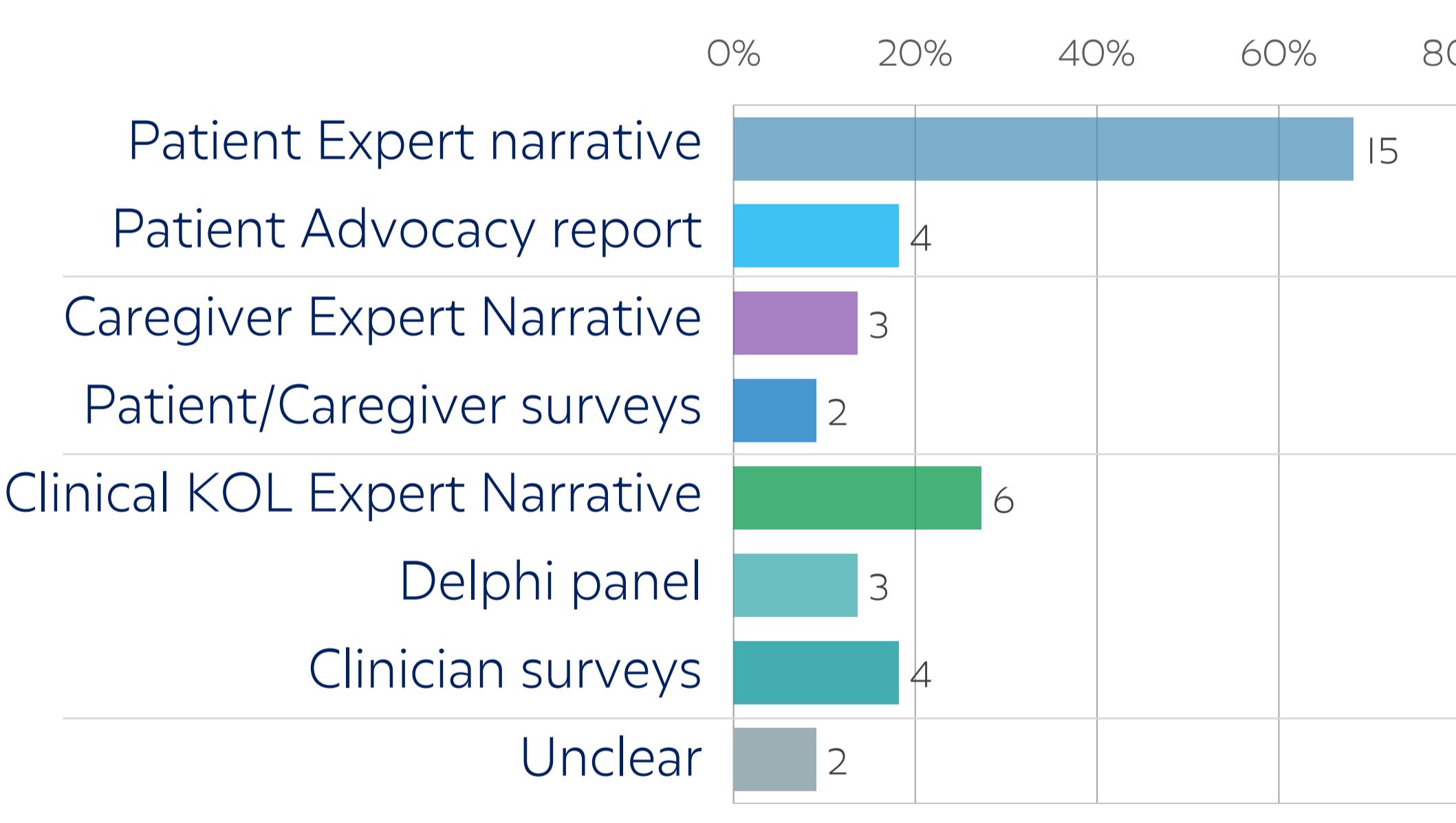
21 evaluations*



11 evaluations*

**Unavailable as no submissions were filed*

- Qualitative evidence was used explicitly in >90% of submissions, and unclear in 2 submissions, of all the submissions considered (N=22).
- Types of qualitative evidence submitted:



- Majority were to provide patient perspectives, mainly through Patient Expert or Caregiver narratives
- These were typically complemented with Clinician perspectives, through Expert narratives, surveys or Delphi panels

- Evidence presented pertained to illuminating aspects of disease burden, contextualising the benefit shown and in support of study design or economic model.



- Qualitative evidence in all submissions was considered by all four HTA bodies.

NICE consistently considered uncaptured benefits qualitatively; accepting a higher level of uncertainty in evidence and modelling assumptions.

The HAS (TC) and G-BA often accepted qualitative data in lieu of lack of robust HR-QOL data in their assessments and final decisions.

Qualitative data, particularly clinician and patient expert views were used to inform disease transition states and populating model parameters when robust quantitative evidence were not available

KEY TAKEAWAYS

- All 4 HTA bodies had flexibility in their assessment methodologies to incorporate qualitative data in their considerations, especially for ultra-rare conditions.
- Qualitative evidence in the submissions provided necessary context for value assessments, and shaped interpretation of clinical evidence and acceptance of the study design or economic model.
- It substantiated broader effects, such as impact on daily living, productivity loss, impact on families and caregivers, which were predominantly qualitative in these submissions and featured heavily in discussions around HR-QoL and overall value.

CONCLUSIONS

- While all agencies have formal procedures for themselves to collect patient perspectives at various stages of the HTA process, the influence on the HTA outcome was most pronounced when evidence was systematically gathered in the submission and clearly tied to decision criteria such as unmet need or quality-of-life impact
- Manufacturers can maximise opportunity, leveraging qualitative data to plug evidence gaps, contextualise clinical data, inform economic modelling and value perception.
- Submissions must include clear documentation of data collection and analysis methods, referencing best practice frameworks increasing acceptability.

POWERED BY



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