HTA370

PATIENT VOICES IN RARE DISEASE HEALTH TECHNOLOGY ASSESSMENT (HTA)

RECENT TRENDS IN THE USE OF QUALITATIVE RESEARCH

Lauren C. Powell, Jessica S. Dunne, Evelyn Griffin, Matthew Martin, Shelagh M. Szabo

Broadstreet HEOR, 300 – 177 West 7th Ave, Vancouver BC Canada

BACKGROUND

- Calls for incorporating patient voices into drug development and decision-making have been made by regulatory, HTA, and health organizations.[1-4]
- Qualitative methods are one approach for deriving in depth patient-based evidence and may be particularly well-suited for rare diseases due to small populations.
- However, for HTA submissions to the National Institute for Health and Care Excellence (NICE) there are no minimum requirements for use of patient-based, qualitative research.[4]
- Therefore, the extent to which manufacturers are utilizing qualitative research in rare diseases, and the specific aspects of HTA submissions that are being informed by such work, is unclear.

OBJECTIVE

To document the use of qualitative methods for deriving patient-based evidence in recent rare disease HTA submissions.

METHODS

GUIDELINE REVIEW

- Current NICE documents related to patient-based research and patient voice were reviewed, and guidance around use of qualitative work was summarized (**Text box 1**).[1,5,6]

DATA COLLECTION

- Published HTA submissions for rare disease treatments from 06/2021-03/2024 were accessed on 07/06/24 from the NICE website.
  - Submissions that were terminated early were not included, as the committee papers for these were unavailable.
- Rare diseases were defined as conditions affecting <1/2,000 as listed on orpha.net; oncologic diagnoses were excluded from the current study due to inherent differences in their reimbursement review.

DATA EXTRACTION and SYNTHESIS

- Data on the use of qualitative methods (interviewing or focus groups with minimum sample size of 5) were extracted and synthesized.
- Data extraction included: disease area and drug type, qualitative research study design, section of the company submission that utilized qualitative research, and reimbursement decision.
- Qualitative research was categorized using a published framework.[1]
- Quality was assessed using the CASP checklist for qualitative research (https://casp-uk.net/casp-tools-checklists/qualitative-studies-checklist/).

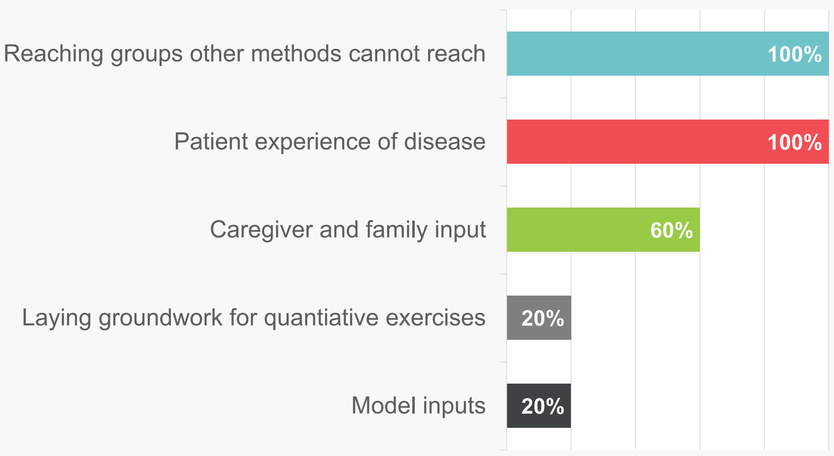
RESULTS

- Ten NICE HTA submissions in rare disease were identified and reviewed (**Table 1**).
- Five described patient-based and carer-based qualitative evidence obtained via new research undertaken by the manufacturer (interviews and focus groups; **Table 1**).
- Two of the five reported sufficient detail to assess quality of the qualitative research which scored 9/10 (full publication, TA804) and 5/10 (poster only, TA912) on the CASP criteria.
- Qualitative methods were primary used to understand patient experience of disease (n=5), reach groups other methods could not reach (n=5), solicit caregiver/family input (n=3), and less frequently, to inform subsequent quantitative work (n=1) and model inputs (n=1; **Figure 1**).
- One submission (TA912) used patient-based qualitative research to create health state vignettes **(Table 1),** which were then valuated by the general population to inform patient utilities in the model.
- In addition to within the pre-defined categories (**Figure 2**), patient-based qualitative evidence was used to assess the validity of a patient-reported outcome measure.
- Though not meeting our criteria for patient-based qualitative research, several submissions conducted Delphi panels and ad-boards among health care professionals to inform cost-effectiveness analyses. There was insufficient detail provided to assess the quality of these studies.
- Nine of the ten drugs were reimbursed.

Table 1. Summary of patient-based qualitative research in 10 rare disease HTA submissions to NICE

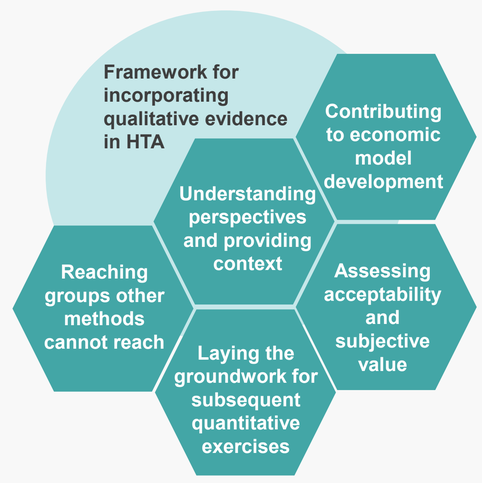
TA number	Initial publication date	Disease area	Treatment	*De novo* patient-based research?	Description of patient-based qualitative research	Drug reimbursed by NICE?
TA955	13-Mar-24	Prurigo nodularis	Dupilumab	**Yes**	**Qualitative interviews** to characterize **patient burden**, lived experience, challenges with current treatment	No
TA937	20-Dec-23	Primary immunoglobulin A nephropathy	Targeted-release budesonide	No		Yes
TA912	15-Aug-23	Late-onset Pompe disease	Cipaglucosidase alfa + miglustat	**Yes**	**Semi-structured** interviews to characterize **patient burden.** **Interviews** with patients, to develop **health state vignettes;** health state valuation was then conducted using a general population sample.	Yes
TA882	03-May-23	Lupus nephritis	Voclosporin	No		Yes
TA915	25-Jan-23	Fabry disease	Pegunigalsidase alfa	No		Yes
TA825	21-Sep-22	Granulomatosis with polyangiitis or microscopic polyangiitis	Avacopan	No		Yes
TA821	24-Aug-22	Pompe disease	Avalglucosidase alfa	**Yes**	Pompe PROM study **qualitative interviews** with patients and carers, to assess impact on **mental health**	Yes
TA808	08-Jul-22	Dravet syndrome	Fenfluramine	No		Yes
TA804	30-Jun-22	Short bowel syndrome	Teduglutide	**Yes**	**Semi-structured qual interviews** to characterize challenges with **current treatment**	Yes
TA959	23-Jun-21	Systemic amyloid light chain amyloidosis	Daratumumab SC	**Yes**	Two online **focus groups** to understand the **psychological and emotional impact** of amyloidosis	Yes

Figure 1. Use of qualitative evidence in rare disease HTA*



*Qualitative evidence derived from patients, carers, and family members

Figure 2. Framework for incorporating qualitative evidence in HTA



Text box 1. Summary of NICE guidance on use of qualitative work in HTA

- Several relevant documents were reviewed that discussed the use of qualitative work in NICE submissions.
- However, specific guidance on use of patient-based evidence in HTA submission is currently lacking.

NICE HTA manual[5]

Outlines topics that can be addressed by qualitative evidence, and considerations more generally for incorporating real world evidence.

Currently there is no guidance on how qualitative evidence is to be integrated with clinical and cost effectiveness data, or minimum requirement for use of qualitative evidence.

Booth 2020 DSU report[6]

Acknowledges the value of a rapid framework for qualitative evidence synthesis (QES); feasibility assessments are ongoing to understand how this could fit within the HTA program at NICE.

Recommends that NICE examine the feasibility of conducting rapid QES as explored by Canada's Drug Agency and Health Information Scotland.

Public and patient involvement (PPI) initiative[1]

The PPI initiative is one way that NICE ensures patient voice is represented in the HTA process. However, this is distinct from having a clear mechanism for integrating qualitative evidence into decision making.

Furthermore, it's unclear how or if this has any impact on reimbursement decisions.

LIMITATIONS

- There was lack of detail on the quality of qualitative research undertaken by manufacturers to support HTA submissions, as three of the five identified were *data-on-file.*
- It was not possible to review committee papers for submissions that were terminated early.
- The impact of including high quality patient-based research on reimbursement decisions cannot be determined from this work and requires further investigation.

CONCLUSIONS

- Qualitative methods are well-suited for deriving patient-based evidence; however, these findings suggest that there is still a gap in their application in HTA, despite several HTA agencies, regulatory bodies, and health organization calling for more patient voices in drug development.[1-4]
- In rare disease HTA submissions, qualitative methods were most often used to contextualize patient experiences; yet, over half of HTA submissions reviewed did not incorporate *de novo* patient-based research. This may be due to time and cost barriers for conducting such research, and lack of guidance on how best to incorporate qualitative evidence into HTA submissions.

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CONTACT: lpowell@broadstreetheor.com

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