BACKGROUND

Utility data enable the standardised quantification of patients’ health-related quality of life (HRQoL). Developing utility data in rare diseases is challenging, as small and often paucidrome patient populations can mean limited numbers of eligible patients that are able to participate in utility collection studies. Utility data and collection methods are a crucial and well-considered component of health technology assessment (HTA) submissions, such as the National Institute for Health and Care Excellence (NICE) highly specialised technology (HST) appraisals, which consider treatments for very rare conditions.1,4

METHODS

A pragmatic literature review was performed to identify rare disease utility studies from EU5 countries (France, Germany, Italy, Spain, UK) published between 1 January 2014 and 26 May 2019. MEDLINE and Embase were searched simultaneously via the Ovid platform, aiming to capture disease and HRQoL search terms. Abstracts were screened by a single reviewer to include English language articles, narrative reviews, case studies and economic models were excluded. Included articles were classified according to study type(s).

The NICE website was searched (22 May 2019) to identify all available HST appraisals. Key details, such as study type(s) used, were noted using the utility data used in the economic model, were extracted from available project documents by one reviewer and checked by a second reviewer. Further details on surveys and vignette study methodologies, the two most commonly used methods outside of interventional/observational trials, were extracted for appraisals published in 2017 onwards. The information extracted included critique published as part of the appraisal.

RESULTS

Published rare disease utility studies

97/378 published articles identified through Ovid were included (Figure 1), of which 8.2% (9/97) used multiple methods to collect utility data. 38.1% (37/97) and 15.5% (15/97) collected utilities using observational and interventional trial methods respectively. Systematic literature reviews (SLRs), 11.3% (11/97), surveys 9.3% (9/97), tool development studies (2.1% (2/97)) and vignette studies (5.2% (5/97)) were also common (Figure 2).

Utility data in NICE HST appraisals

The majority of HST appraisals (87.5% (14/16)) included utility data collected in interventional studies. Of these, 78.6% (11/14) also included alternative methods such as surveys (35.7% (5/14)), vignette studies (21.4% (3/14)), 56.2% (8/14) and a registry study, observational or interventional study (7.1% (1/14) each). The two HST that did not collect utility data using an interventional study were used as part of the economic model. Surveys were defined as stating the use of a survey, online method or remote completion of a questionnaire; vignette studies were defined as mentioning the use or completion of vignettes under clinical or non-clinical circumstances. To characterise the methods used to collect utility data in NICE HST appraisals, categorised by utility data collection method (n=16), evaluations treated for very rare conditions.

Vignette and survey based collection of utility data in NICE HST appraisals

As shown in Figure 3, there was variation in the extent and nature of external involvement in the surveys and vignette studies. NICE often raised concerns over the use of survey or vignette-based data over trial-based data in HST economic models.

CONCLUSIONS

The survey of utility study types identified in both the published literature and HST appraisals point towards the challenges in collecting utility data within clinical trials. Technical concerns raised during NICE HST appraisals suggest that best-practice guidance on alternative utility collection methods, such as vignettes and surveys, may improve the rigour of these studies. Future research could further evaluate how methodologies found in the published literature compare to those screened in NICE HST appraisals, and whether they are subject to the same limitations that were identified by NICE.

References


Analysis of survey and vignette methods used in HST appraisals published in 2017 onwards (n=12)

4 HST appraisals included data from surveys (HST6, ID737, ID800, ID861)

Survey respondents included the general population (n=4), clinical expert (n=1), patient (n=2, ID737, ID800) and a survey completed by a public (ID861, discrete choice experiment ID943, with valuation by clinical experts rather than patients) with data over clinical study data (n=1; ID861). Surveys were defined as stating the use of a survey, online method or remote completion of a questionnaire; vignette studies were defined as mentioning the use or completion of vignettes under clinical or non-clinical circumstances. (draft guidance)