The use of real-world data in health technology assessments of medications for rare diseases

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BACKGROUND

Assessing the risks and benefits of new therapies for rare diseases is challenging:

- Difficult to assess safety and efficacy in controlled trials;
- Costly or infeasible to collect rigorous comparative data;
- Other important aspects of disease burden such as caregiver impact and patient quality-of-life (QoL) may be infrequently reported.

RWD are data collected outside of randomized controlled trials, including cohort and natural history studies, electronic health records, disease registries, and claims databases.

Both health-service researchers and decision-makers are advocating for considering real-world data (RWD) in health technology assessment (HTA) of medications.

How such data are being presently used across different HTAs is unclear, as is whether their inclusion impacts decision-making.

OBJECTIVE

To characterize the use of RWD in HTA of medications for rare diseases from the United Kingdom (UK) and Canada, and to examine whether the inclusion of RWD was associated with a favorable HTA recommendation.

METHODS

All diseases with a prevalence of 10 to 50 per 100,000 persons were identified from Orphanet:

- Those with a prevalence of <10 per 100,000 were excluded to focus on diseases that would be more likely to have ≥1 published technical appraisal.

Technical appraisals from both the National Institute for Health and Care excellence (NICE) and the Canadian Agency for Drugs and Technology in Health (CADTH) published between January 2009 to July 2019 were selected for review, data extraction and analysis.

The following features of technical appraisals from the two HTAs were reviewed and compared:

- The percentage of appraisals including RWD:
  - Characteristics of the appraisals with and without RWD, including whether the submission was for a cancer or non-cancer medication;
  - Whether submissions with RWD received favorable recommendations;
  - The types of RWD submitted for appraisal;
  - The percentage of RWD provided as primary (i.e. clinical or cost-effectiveness) vs. ancillary evidence.
- Trends in use of RWD overtime (2009 to 2013 vs. 2014 to 2019).
- The number of appraisals reviewed by both HTAs, and the:
  - Proportion that included RWD;
  - Proportion that were for cancer medications.

RESULTS

- Of 205 rare diseases from Orphanet, CADTH reviewed appraisals for 23 diseases and NICE reviewed appraisals for 24 from 2009-2019.
  - 37% (NICE) and 67% (CADTH) of appraisals included RWD as part of the evidence submission.
  - Of these, 72% (NICE) and 81% (CADTH) were published from 2014 to 2019.
- A higher percentage of cancer medication appraisals reviewed by both HTAs included RWD compared to non-cancer medication appraisals in both time periods (Figure 1):
  - NICE: 57% vs. 43% in 2009-2013 and 67% vs. 33% in 2014-2019;
  - CADTH: 88% vs. 13% in 2009-2013 and 76% vs. 24% in 2014-2019.
- Across both HTAs, compared to appraisals without RWD, those that included RWD had a higher likelihood of being recommended in both time periods (Figure 1):
  - 2009-2013: RWD vs. without RWD - 86% vs. 70% (NICE); 75% vs. 44% (CADTH);
  - 2014-2019: RWD vs. without RWD - 100% vs. 97% (NICE); 85% vs. 67% (CADTH).
- A higher percentage of RWD included in NICE submissions was included as part of primary evidence compared to CADTH (48% vs. 29%). A higher percentage was included as ancillary evidence in CADTH (79% vs. 60%).
- 31 appraisals were reviewed by both HTAs, of these, 81% (CADTH) and 35% (NICE) of submissions included RWD as part of the evidence submission.
  - Across the 31 appraisals reviewed by both HTAs, there were 9 for which RWD was used in both submissions;
    - 78% (7/9) of these were first reviewed by CADTH and then NICE
    - 67% (6/9) of these were for cancer medications.

Figure 1: Characteristics and outcomes of appraisals with & without RWD, over time
The majority of NICE and CADTH appraisals for rare diseases with RDW between the 2009-2013 period were recommended, and this proportion appears to be consistently higher than those without RWD and to be increasing over time; although more pronounced in NICE appraisals.

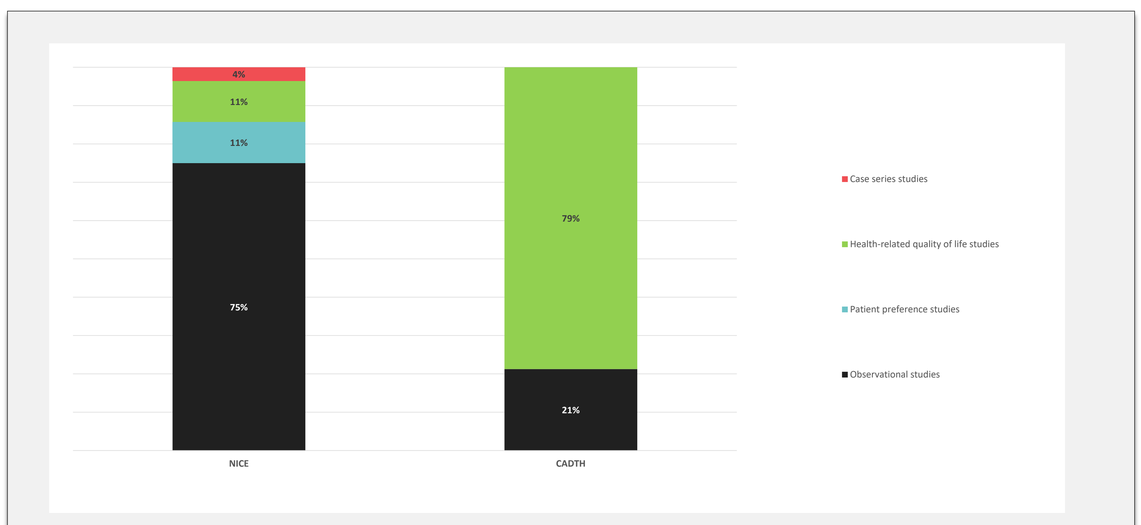

Figure 2: Types of RWD included in appraisals from 2009-2019
The majority of the RWD included in appraisals between the period of 2009 and 2019 were observational studies in NICE and HRQoL studies in CADTH.

DISCUSSION

- Overall, the likelihood of a positive appraisal was higher for appraisals including RWD compared to those without RWD in both NICE and CADTH
- The inclusion of RWD has been increasing over time in both jurisdictions.
- The inclusion of RWD for cancer medications was more frequent in appraisals reviewed by CADTH compared to NICE, this may be due to HTA requirements in each of these jurisdictions.
- There were differences in how RWD were described between the HTAs that complicated assessment of the impact of those RWD in Canada vs. the UK. As efforts to formalize and standardize how RWD are incorporated in to HTA increases, between-country comparisons should become easier.
- While the incorporation of RWD in HTAs for rare diseases has increased over the past decade in both the UK and Canada, whether this trend is observe in other jurisdictions warrants further examination.

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