HTA182

How Will Patient Registry Data Play a Role in Joint Clinical Assessment (JCA) of Rare Diseases?

Learnings From National Health Technology Assessments (HTAs)

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BACKGROUND

- From 13 January 2028, new orphan medicines submitted to the European Medicines Agency will undergo JCA as part of the European Union's HTA Regulation.[1]
- The JCA requires a single submission of evidence on clinical efficacy and safety of new medicinal products in Europe.
- Systematic identification of evidence will be required for the submission, including patient registry data.
- It is unclear whether or how patient registry data will be considered in view of the stated preference for evidence from randomised controlled trials.

OBJECTIVE

- To explore whether and how patient registry data have been used in national HTAs for rare diseases and to identify any potential learnings for JCA.

METHODS

- The INAHTA (International HTA) database was searched for published HTAs in rare diseases, focusing on muscular dystrophy, spinal muscular atrophy (SMA), and Duchenne muscular dystrophy (DMD).
- The search terms used were "Muscular Dystrophy" OR "SMA" OR "DMD."
- Articles were limited to English language.
- Assessment documents were reviewed to identify whether patient registry data were used, and if so, how.
- Data were recorded on country, year, disease of interest, and whether or how patient registry data were used.

RESULTS

Table 1. HTA Organisations From Each Country

Country	HTA Organisation	Number of Assessments Identified	Health Condition
Austria	Austrian Institute for Health Technology Assessment GmbH (AIHTA)	2	SMA
England and Wales	National Institute for Health and Care Excellence (NICE) National Institute for Health Research (NIHR) HTA Programme	7	DMD and SMA
Germany	Gemeinsamer Bundesausschuss (G-BA)	1	DMD
Republic of Ireland	Health Information and Quality Authority (HIQA)	1	SMA
Scotland	Scottish Medicines Consortium (SMC)	1	SMA

- Twelve HTAs were identified in muscular dystrophy, SMA, or DMD that were accessible.
- HTA organisations were from Austria, England and Wales, Germany, the Republic of Ireland, and Scotland **(Table 1)**.
- Six of the assessments did not include patient registry data (AIHTA, n = 1; NIHR, n = 1; NICE, n = 2; SMC, n = 1; G-BA, n = 1) (**Figure 1**).
- Of the remaining 6 that stated the use of patient registry data, 4 were for SMA and 2 were for DMD (**Figure 2**).
- Of the 6 included documents, 3 were original appraisals and 3 were reviews following a period of managed or other access to the intervention.
- In the 3 original appraisals, patient registry data were used for background information on symptoms and outcomes or for comparative efficacy for existing treatments.
- In the HTA reviews, data were used for long-term comparative efficacy or were considered alongside trial data for outcomes information.

Figure 1. HTA Assessments Including Patient Registry Data

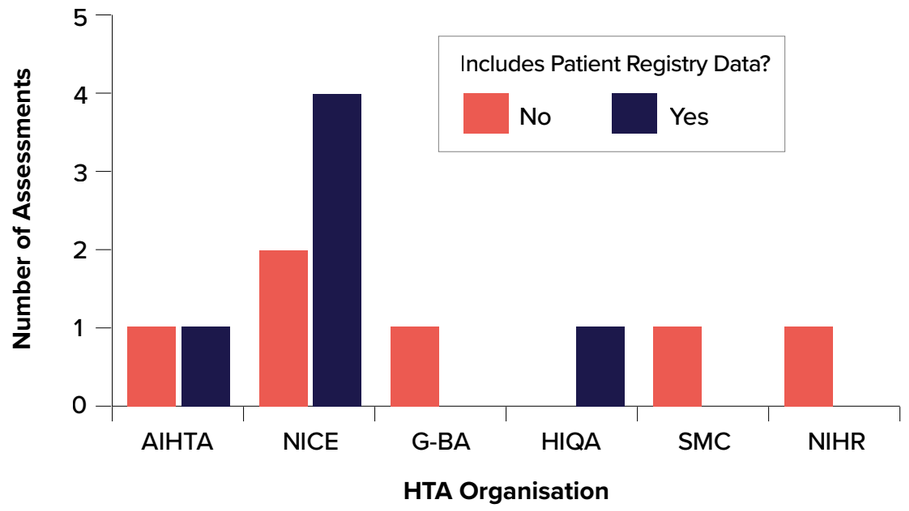
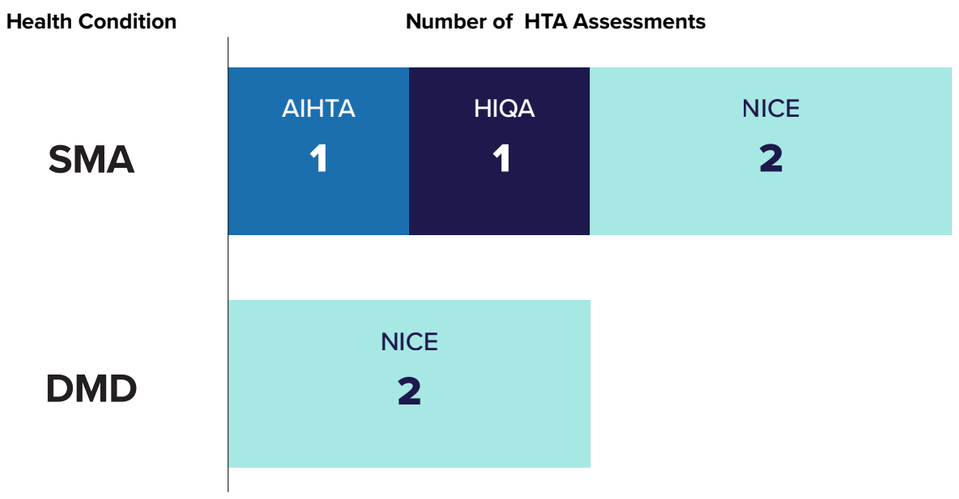


Figure 2. Health Conditions of the HTA Assessments Including Patient Registry Data



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1. European Commission, Directorate-General for Health and Food Safety. Joint clinical assessment of medicinal products: Submission of early information by health technology developers. 21 Jun 2024. https://health.ec.europa.eu/document/download/77476507-9ddc-47e3-ae0d-11c05f89169c_en?filename=hta_%20mp-jca_htd_en.pdf. Accessed 18 Sep 2025.

DISCUSSION

- Registry data were not often used in the identified HTAs of rare diseases.
- Where registry data were used, they provided relevant evidence for symptoms, outcomes, and comparative effectiveness for existing treatments.

CONCLUSIONS

Based on national HTAs, this study suggests that patient registry data may be valuable in JCA of rare diseases where trial data for existing therapies are limited and for use as background information on symptoms and outcomes.

Whilst the JCA may use registry data as evidence to support clinical effectiveness arguments, we found limited evidence of national HTAs using registry data in this way for the rare diseases of muscular dystrophy, SMA, and DMD.

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The value of **understanding**

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