Abstract 8160

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Challenges in Defining Target Populations: An Analysis of Orphan Drug Health Technology Assessments in Germany, 2024

M.Roll[1], B.Güntert[1]

[1] Institute of Translational Medicine, Faculty of Medical Sciences, Private University in the Principality of Liechtenstein (UFL), Triesen, Principality of Liechtenstein

BACKGROUND

Orphan drugs present substantial challenges in the Health Technology Assessment (HTA) process — particularly in estimating the size of the target population. Epidemiological data are critical to justify clinical benefit and economic impact, but methodological quality and transparency often vary. In Germany, manufacturers typically use prevalence or incidence estimates from various sources, which may not be systematically validated.

AIM

This study investigates the methods and data sources used to estimate epidemiological parameters (prevalence/incidence) for orphan drugs in German HTAs. A particular focus is placed on the type of data source, the quality of reporting, and the presence of critical appraisal by the IQWiG.

MATERIAL & METHODS

We conducted a cross-sectional review of all orphan drug HTA procedures completed in Germany in 2024. For each assessment, we extracted the type of data source used for epidemiological estimation (claims data, registries, literature) and whether methodological critique was raised by IQWiG. We also stratified by indication type (oncological vs. non-oncological). Descriptive statistics and Fisher's exact test were used.

CONCLUSION

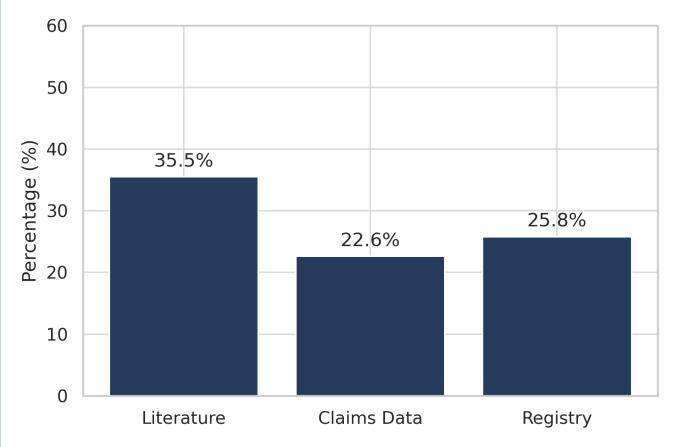
The analysis of orphan drug assessments in 2024 reveals substantial heterogeneity in how target populations are estimated in German HTA submissions. While different data sources are used, the decisive factor for methodological critique was not the type of source but rather the **transparency**, **validity**, and **reproducibility** of the estimation process.

Greater standardization and guidance are needed to improve the quality and comparability of epidemiological data in HTA dossiers for rare diseases.

RESULTS

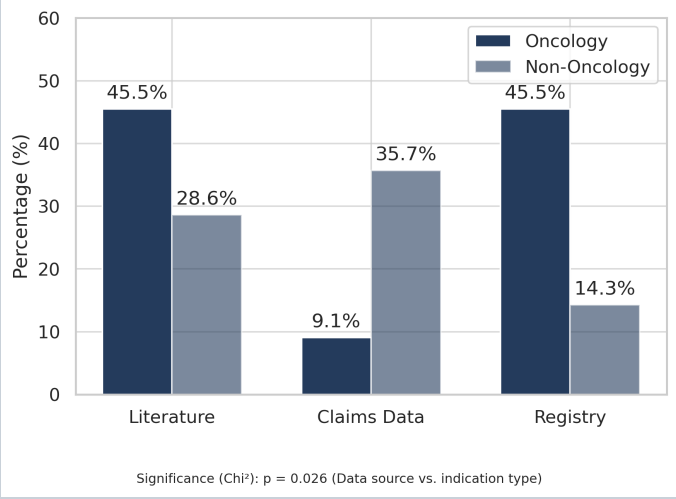
- AMNOG 31 orphan drug assessments in 2024, the most common data source for estimating target populations was literature (35.5%), followed by registries (25.8%) and claims data (22.6%).

Figure 1: Data Source Usage in 2024



- A significant association (p = 0.026) between indication type (oncology vs. non-oncology) and data source selection was observed.

Figure 2: Data Sources by Indication Type



- Additionally IQWiG raised methodological concerns in all cases, particularly regarding uncertain population estimates (77%) and lack of transparency (74%).

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