Orphan Drug Assessments in Germany in Comparison with Other International HTA Agencies

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Objective

- Examine benefit assessment of Orphan Drugs conducted by the German Federal Joint Committee (G-BA) according to AMNOG criteria, and compare them with assessments from five other national HTA agencies.

Methods

- Analyzed all orphan drug assessments conducted by the G-BA between Jan 2011 and May 2015. Compared them with other HTA assessments from the EU (France, England, Netherlands, Scotland) and Canada. Data collection cut off was May 31, 2015.
- 20 assessments for 19 Orphan Drugs were completed by the G-BA by end of May 2015 and were comparable.
- Oncology Orphan Drugs i.e., all orphan drugs with both orphan drug designation(s) and approved.
- England’s NICE has the highest percentage (80%) of negative recommendations.
- Potential changes to the German drug assessment mechanism could impact this on definition and resulting evaluations.
- Stringent application of pharmacoeconomic criteria, such as cost-effectiveness and cost-utility, is associated with a significantly lower number of positive recommendations, especially in England (0%), Scotland (35%) and Canada (5%).

Results

Table 1: Possible Outcomes and Ratings of HTAs conducted by the G-BA and other HTA Agencies

Table 2: Overall HTA decision summary by country / agency for all Orphan Drugs

Table 3: Summary of results for ultra-orphan drugs

Table 4: Summary of results for Oncology Orphan Drugs

Table 5: Summary of results for Other Orphan Drugs

Conclusions

- Germany did not issue any negative recommendations for any of the 20 Orphan Drug assessments.
- Four other HTA agencies completed between 14 and 5 evaluations, with these differences seemingly due to local criteria for reviews and / or launch timing; e.g. NICE has not regularly reviewed ultra-orphan drugs.
- German G-BA (85%), Dublin (85%) and French HAS (85%) have the highest percentages of positive recommendations without restrictions.
- Canadian CADTH has the highest percentage (75%) of recommendations with clinical and economic restrictions.
- England’s NICE has the highest percentage (60%) of negative recommendations.
- 43% of the Scottish SMIC recommendations have a Patient Access Scheme and 36% took advice from a Patient and Clinician Engagement (PACE) group.

- Germany and France base their HTAs primarily on additional clinical value criteria, comparing the drug to existing treatment options, if any exist, or standard of care. As a consequence, there is substantial convergence in their recommendations.
- German G-BA and French HAS are the only agencies that reviewed all Orphan Drugs.
- Parameters used for gathering information were websites of the following HTA authorities: Federal Joint Committee (G-BA, Germany); National Authority for Health Technology Assessment (France); National Health Care Institute (ZIN, Netherlands); National Institute for Health and Care Excellence (NICE, England); Scottish Medicine Commission (SMC, Scotland); Canadian Agency for Drugs and Technologies in Health (CADTH, Canada).
- Sources used for gathering information were websites of the following HTA authorities: Federal Joint Committee (G-BA, Germany); National Authority for Health Technology Assessment (France); National Health Care Institute (ZIN, Netherlands); National Institute for Health and Care Excellence (NICE, England); Scottish Medicine Commission (SMC, Scotland); Canadian Agency for Drugs and Technologies in Health (CADTH, Canada).
- Collection cut off was May 31, 2015.
- Germany did not issue any negative recommendations for any of the 20 Orphan Drug assessments.
- In England, Scotland and Canada, pharmacoeconomic criteria such as cost-effectiveness and cost-utility have a greater weight in the assessments.
- Stringent application of pharmacoeconomic criteria, such as cost-effectiveness and cost-utility, is associated with a significantly lower number of positive recommendations, especially in England (0%), Scotland (35%) and Canada (5%).
- When comparing evaluation of Oncology Orphans in England and Scotland, there are significant discrepancies between the recommendations issued by the NICE and the SMC, with the latter issuing a higher number of positive recommendations, primarily as a result of greater importance attributed to the opinion of patients and clinicians (PACE groups).
- Providing evidence of low budget impact (Netherlands) and negotiating a price reduction or a Patient Access Scheme (Scotland, Canada) increases probability of positive or partially positive recommendation.