TRANSITION FROM ORPHAN DRUG TO FULL ASSESSMENT IN THE GERMAN ANMGO SYSTEM: KEY LEARNINGS FROM PIONEERS

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OBJECTIVES
- A specific feature of the German HTA process is the relevance of the orphan drug (OD) status. The additional medical benefit of orphan drugs, assessed by the German HTA body (FJC, Federal Joint Committee) is already acknowledged by approval. No head-to-head data are required.
- If the revenue per annum exceeds 50 million euros or OD-status is lost, reevaluation against an appropriate comparator is mandatory.
- The aim of this study was to reveal the consequences of reevaluation.
- Acceptance of study data, patient relevant endpoints and extent of additional benefit assigned by FJC and IQWiG (Institute for Quality and Efficiency in Health Care) of reevaluated former OD-dossiers were analyzed.

RESULTS

Figure 1. Trend of Extent of Added Benefit After Transition from Orphan Drug to Full Assessment

- Figure 1 displays the highest extent of added benefit, which was achieved at least in one subpopulation for each drug.
- The reassessment of two drugs (Brutinib and Macicentan) led to an increase in the added benefit in at least one subpopulation compared to OD-evaluation (Figure 1).
- Two drugs (Pomalidomid and Ramucirumab) could retain the extent of added benefit in at least one subpopulation after full evaluation (Figure 1).
- The full assessment of one drug (Macicentan) resulted in a downgrading of the added benefit in comparison to the Od-evaluation (Figure 1).

Table 1. Reassessed Orphan Disease Drugs in the German ANMGO System

Table 2. Brutinib - Details on Orphan Drug and Full Assessment

Table 3. Macicentan - Details on Orphan Drug and Full Assessment

Table 4. Pomalidomid - Details on Orphan Drug and Full Assessment

Table 5. Ramucirumab - Details on Orphan Drug and Full Assessment

Table 6. Ruxolitinib - Details on Orphan Drug and Full Assessment

CONCLUSIONS
- Following an OD assessment it is likely that FJC will split the label and define subpopulations with different appropriate comparators for the full assessment.
- FJC accepted the comparator used in the pivotal trial as appropriate in the full assessment (at least for one subpopulation) in four out of five dossiers.
- The extent of the added benefit from the OD assessment could be maintained or even increased during reevaluation (in subpopulations).
- It seems that the FJC still takes the OD-status into account during full assessment. The special medical need is a strong argument in the reevaluated dossiers.

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