

THE VALUE OF PATIENT REPORTED OUTCOMES IN GERMAN AMNOG DOSSIERS

Kathrin Borchert, Julia Altevers, Sebastian Braun, Thomas Mittendorf

Xcenda GmbH, Hannover, Germany

BACKGROUND

- Today the measurement of Patient Reported Outcomes (PROs) forms an integral part of clinical trials on a regulatory level.
- The European Medicines Agency (EMA) as well as the Food and Drug Administration (FDA) currently issued guidance on how to measure and present PROs and particularly health related Quality of Life (hrQoL).¹⁻³
- The German Federal Joint Committee (G-BA) has suggested to actively integrate hrQoL as a main outcome in decision making, as missing data may have a negative impact on the benefit assessment.⁴
- Following the Act on the Reform of the Market for Medicinal Products (AMNOG) in 2011, hrQoL measures are already considered being a patient relevant outcome in the benefit assessment of medicinal products in Germany.⁵

OBJECTIVE

- Aim of this study was to examine how PROs are currently integrated in German AMNOG assessments and to assess their impact on the G-BA decision on added benefit.

METHODS

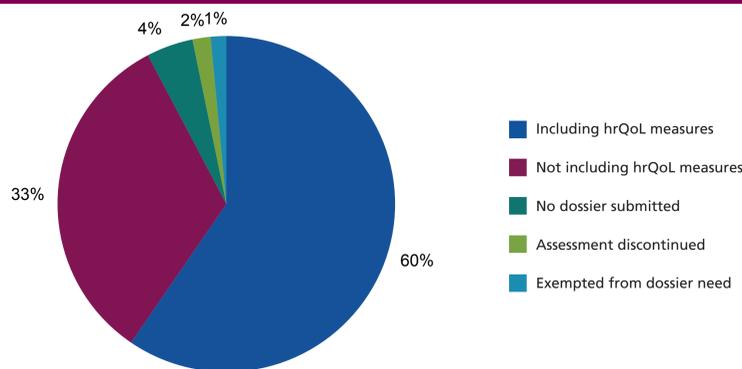
- AMNOG dossiers up until June 2016 were included in the analysis.
- They were screened based on the inclusion of hrQoL and type of questionnaire used to measure hrQoL as well as for the disease and orphan drug status. Questionnaires modified for specific indications were grouped and considered as one type of questionnaire (e.g. EORTC-QLQ-BR23 and EORTC-QLQ-C30 as EORTC-QLQ).
- In addition, the consideration and acceptance of these hrQoL measures by the G-BA within the decision making process as well as the added benefit assessment was evaluated descriptively.

RESULTS

SUBPOPULATIONS WITH HRQOL MEASURES

- In total, n=200 AMNOG dossiers comprising n=403 subpopulations were included. Dossiers comprise more than one subpopulation if the new drug will be launched in more than one field of application.
- PRO measures to assess hrQoL were utilized in n=240 (60%) subpopulations (Figure 1).
- Fifty-four different questionnaires were incorporated in AMNOG dossiers in the past.

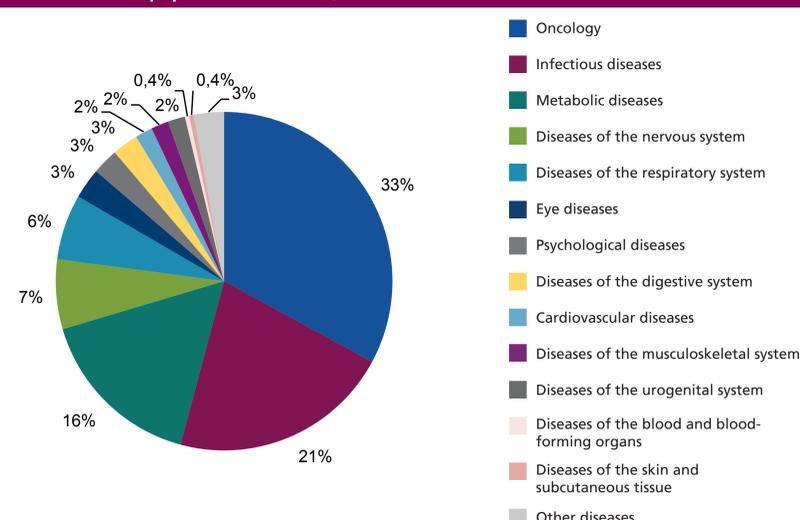
Figure 1: Subpopulations stratified by hrQoL measures



DISEASE AREAS

- One third of the subpopulations where hrQoL measures were incorporated were from oncology, 21% from infectious diseases, and 16% from metabolic diseases (Figure 2).

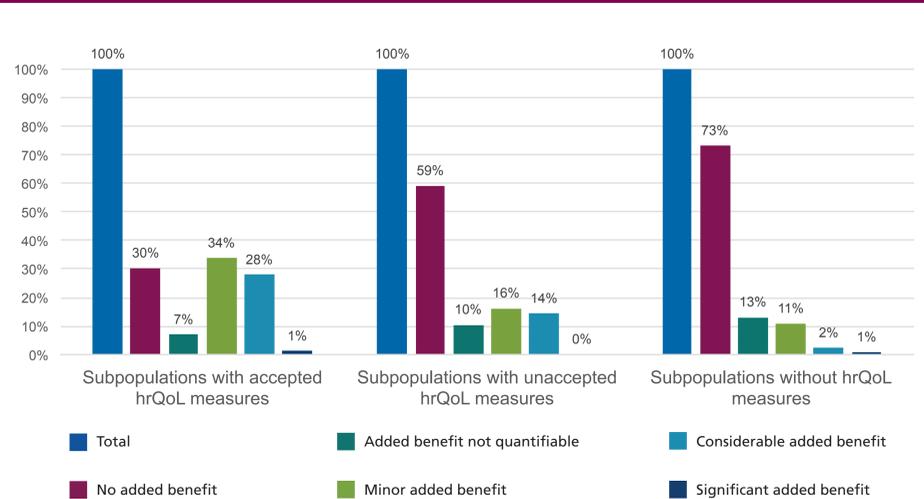
Figure 2: Disease areas of subpopulations with hrQoL measures



ACCEPTANCE & ADDED BENEFIT ASSESSMENT

- The G-BA accepted and took into account the results on hrQoL in n=86 subpopulations, representing 36% of all subpopulations incorporating hrQoL measures.
- Seventy percent of subpopulations with accepted hrQoL measures were granted an added benefit by the G-BA, compared to 41% in subpopulations with unaccepted hrQoL measures, and 27% in subpopulations without hrQoL measures.
- Subpopulations with accepted or unaccepted hrQoL measures were mostly granted a minor added benefit, whereas the added benefit in subpopulations without hrQoL measures was mostly rated as not quantifiable (Figure 3).

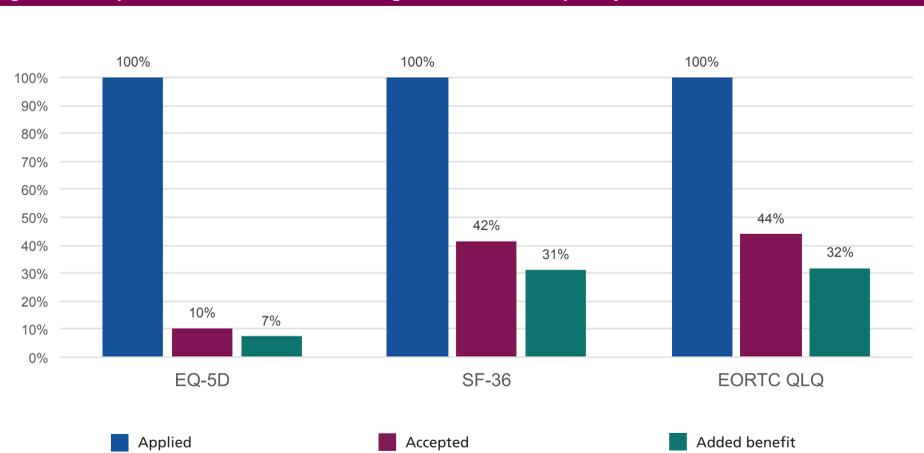
Figure 3: Added benefit assessments of subpopulations depending on hrQoL measures



MOST FREQUENTLY USED INSTRUMENTS

- The EQ-5D, SF-36, and EORTC QLQ were the most frequently used instruments to measure hrQoL (57%, 32% and 21% of subpopulations reporting hrQoL measures, respectively).
- A higher proportion of EORTC QLQ (44%) and SF-36 (42%) data was accepted and taken into account by the G-BA compared to data gathered with the EQ-5D (10%).
- An added benefit was granted in 32% of subpopulations with data from the EORTC QLQ, in 31% of subpopulations with data from the SF-36, and in 7% of subpopulations reporting EQ-5D data (Figure 4).

Figure 4: Acceptance and added benefit ratings for the most frequently used instruments



ORPHAN DRUGS

- Considering orphan drugs, hrQoL measures were reported in 67% of all orphan drug applications. Of these, 60% were accepted and at least taken into account by the G-BA.

CONCLUSIONS

- The integration of hrQoL measures is relatively common in the German AMNOG process and many different types of questionnaires are incorporated.
- Most common instruments for assessing the hrQoL in AMNOG dossiers are the SF-36, the EORTC QLQ, and the EQ-5D.
- Only one third of the hrQoL measures were accepted and taken into account by the G-BA. However, if accepted, the majority of subpopulations were being granted an added benefit.
- Without doubt, the integration of PROs in terms of hrQoL into the data package for German AMNOG dossiers may positively impact the rating for an added benefit by the G-BA.
- HrQoL data may also constitute a supportive tool for subsequent price negotiations potentially being of even greater importance in the future.

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