HOW THE RESULTS OF THE AMNOG EARLY BENEFIT ASSESSMENT (EBA) IN GERMANY HAVE DEVELOPED OVER TIME

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Objectives

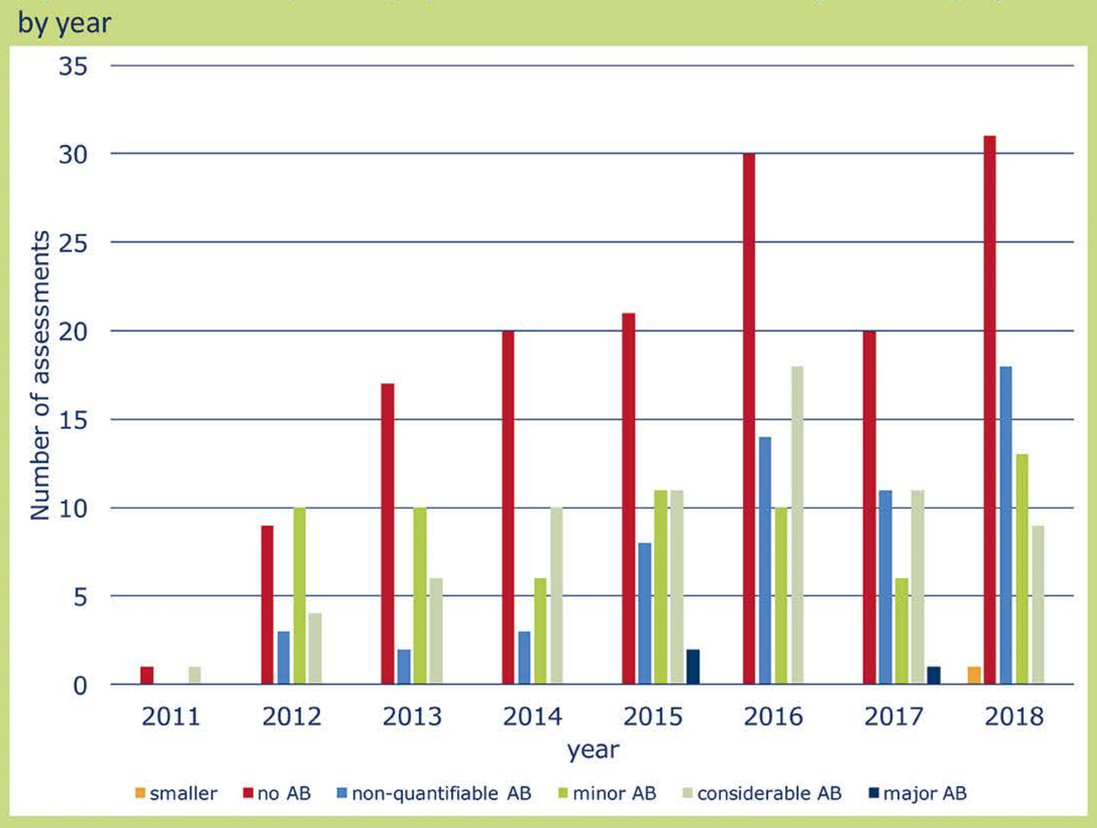
Since 2011, newly marketed drugs undergo an EBA in which the Federal Joint Committee (GBA) assesses the drug's additional benefit (AB) compared to an appropriate comparator. A post-hoc segmentation of a therapeutic indication into patient populations (PP), e.g. by pre-treatment or disease severity, is possible. Specific requirements regarding study design and data analysis often constitute obstacles for pharmaceutical companies that submit a dossier in order to prove the AB. This research aims to quantify as to how the success rate of EBAs has evolved over time.

Methods

All 366 EBAs finished until December 31st, 2018 were considered in this analysis. The number of PPs awarded with the highest category of AB was counted.
The number of positive vs. negative results as well as the respective proportions were summarized and descriptively analyzed with regard to category of AB and year.
Positive results (defined as at least "non-quantifiable" AB) were separated with regard to the category of AB. Negative results (defined as "no" or "smaller" AB) were not presented separately.

Results

Figure 1: Number of patient populations awarded with the highest category of AB by year

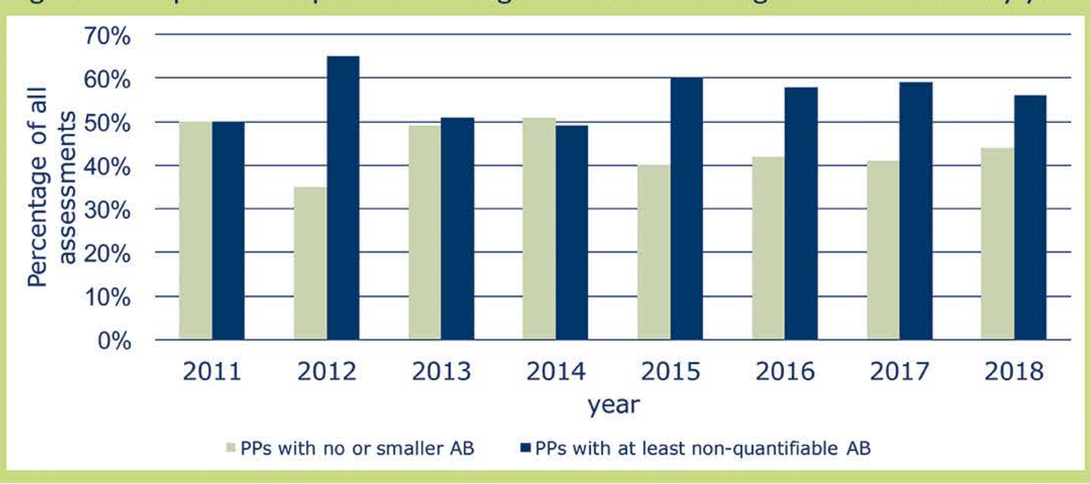


The figure shows in detail the distribution of AB for every EBA referring to the particular PP with the highest AB category (Figure 1). Of note, the proportion of anti-cancer drugs being assessed has increased substantially over the years (e.g. 2012: 21%; 2017: 56%; Figure not shown). Those tend to be associated with higher categories of an AB.

Figure 2: Number of positive vs. negative results among all assessments by year

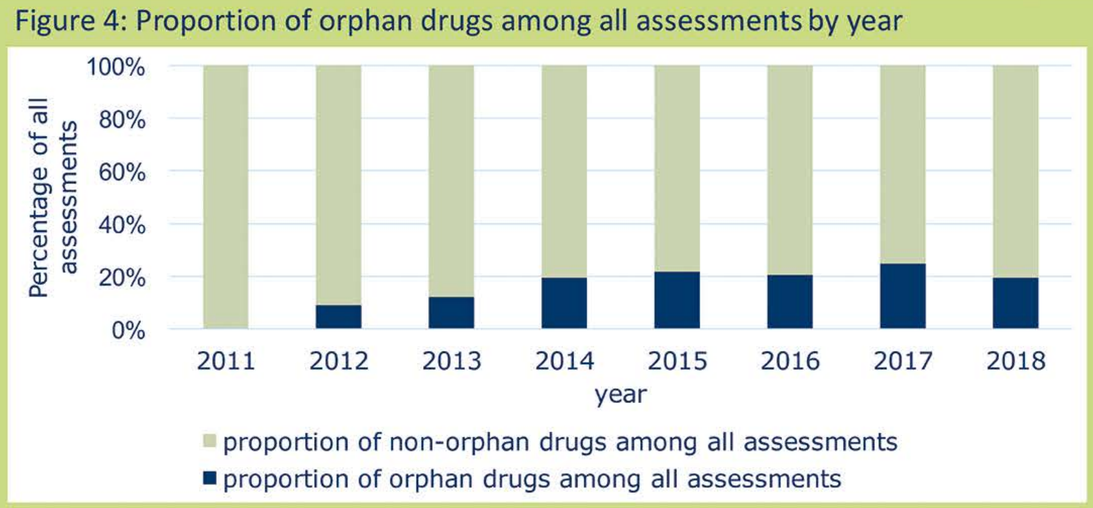
Over the years an increase regarding both positive (at least non-quantifiable AB) and negative results (no or smaller AB) was determined which is attributable to the rising number of EBAs per se (Figure 2).

Figure 3: Proportion of positive vs. negative results among all assessments by year



Between 2011 and 2014 the proportion of PPs with a positive result vs. a negative result was roughly evenly distributed. However, from 2015 onwards a rise in the proportion of positive vs. negative results became apparent (Figure 3).

Figure 4: Proportion of orphan drugs among all assessments by year



In addition to the impact of anti-cancer drugs, the increase of positive results among all assessments might also be partly due to a higher percentage of orphan drugs being assessed (Figure 4).

Figure 5: Distribution of positive AB categories among all positive results by year

The proportion of PPs with a non-quantifiable AB among positive results has risen during the years whereas the proportion of PPs with a minor AB has declined. There is no clear picture regarding the other positive AB categories (Figure 5).

Conclusions

With increasing positive EBA results, a better understanding of the methodological issues of the EBA can be assumed. However, the increasing number of marketing authorizations for orphan drugs which entail a positive EBA result by law might have played a relevant role. The observed increase in positive results may also partly be due to an increase in the proportion of anti-cancer drugs being assessed and the higher probability for an additional benefit compared to drugs e.g. for chronic diseases. EBA-tailored study designs and early coordination with the GBA in this respect will likely increase the chances of positive EBA results.

Reference: Gemeinsamer Bundesausschuss: http://www.g-ba.de/informationen/nutzenbewertung