EARLY BENEFIT ASSESSMENT OF ORPHAN DRUGS IN GERMANY

METHODOLOGICAL CHALLENGES AND IMPLICATIONS ON REIMBURSEMENT

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BACKGROUND

Since the enactment of the Act on the Reform of the Market for Medical Products (AMNOG) in 2011, new pharmaceutical products in Germany are subject to early assessment of their additional benefit by the Federal Joint Committee (G-BA). In this process, new drugs are compared to the existing standard therapy as defined by the G-BA. The results of the early assessment in turn build the foundation of subsequent price negotiations with the National Association of Statutory Health Insurance Funds which ultimately result in the final reimbursement price of the new drug.

For orphan drugs special requirements apply during the early assessment as their additional benefit is considered to be proved by their marketing authorization. In addition, orphan drugs are not compared to the existing standard therapy as set by the G-BA but rather by the results of their pivotal marketing authorization studies.

OBJECTIVES

In this analysis we investigated the influence of the orphan drugs' study type (RCT vs non-RCT) on the assessment of the additional benefit in the German AMNOG process. In addition, a possible correlation between extent of additional benefit and negotiated discount was evaluated.

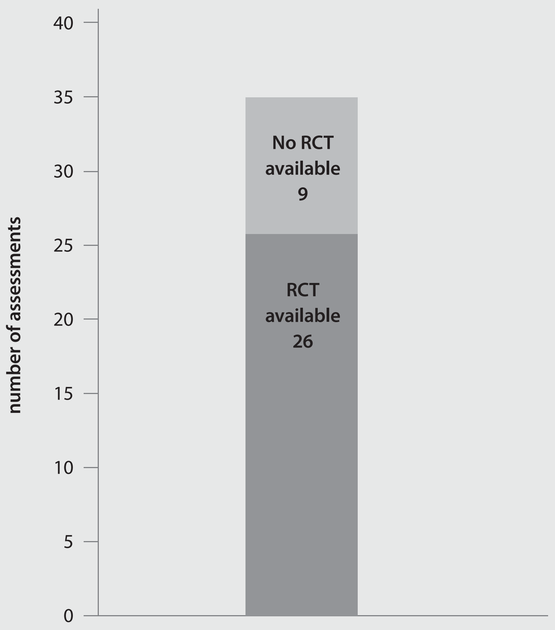
METHODS

We included any orphan drug assessment that was completed by 1 Apr 2016 and for which a final negotiated price was available*. It was investigated whether the presence of only non-RCT(s) resulted in a different extent of additional benefit then assessments with at least one RCT. In addition, the possible correlation between additional benefit and final negotiated discount was assessed by comparison of the initial price at market launch in Germany with the negotiated final price.

* the second criterion was only relevant for the analysis of correlation between additional benefit and price

RESULTS

Number of identified assessments
(as of : 1 Apr 2016)

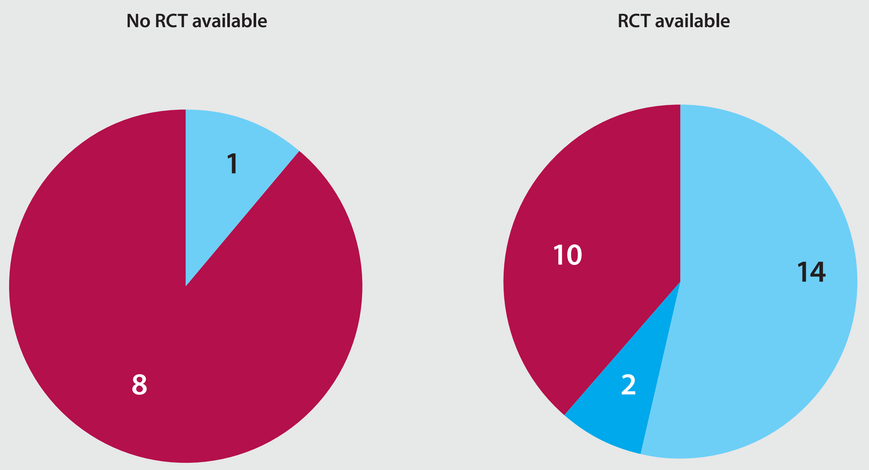


We were able to identify 35 assessments that had a final decision on additional benefit. Of those 35 assessments, 26 were based on the results of at least one RCT while in 9 assessments only evidence from non-RCTs was available.

15 of the 35 final assessments resulted in a minor and 2 in a considerable additional benefit; 18 assessments concluded with a non-quantifiable additional benefit. The highest category of additional benefit (major) has not yet been granted for an orphan drug.

A final negotiated discount was available for 28 of the 35 assessments with final decision, corresponding to 23 individual drugs.

Influence of available study type on additional benefit

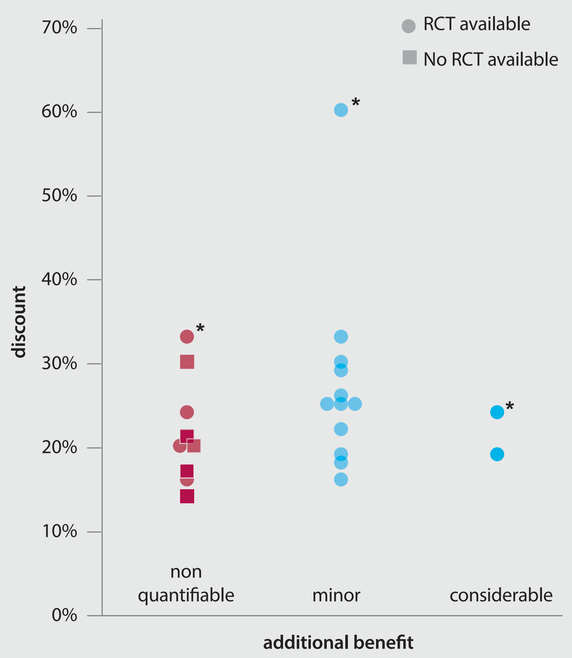


Of the 9 assessments in which only non-RCTs were available 8 assessments concluded with a non-quantifiable additional benefit while only in 1 case a minor benefit was granted.

In contrast, of the 26 assessments which were based on at least one RCT, the majority (16) concluded with a quantifiable additional benefit, i.e. either a minor (14) or a considerable (2) additional benefit. Only 10 assessments resulted in a non-quantifiable additional benefit.

Strikingly, 5 of the assessments in which a non-quantifiable additional benefit was granted despite the availability of at least one RCT, took place only recently (final decision adopted within 4 months prior to our analysis date). The reason congruently given for all 5 decisions was the absence of a clinically relevant effect in (G-BA defined) patient relevant endpoints compared to the study comparator. Such a trend was not evident in previous assessments which concluded with a non-quantifiable additional benefit.

Correlation between extent of additional benefit and negotiated final discounts



The negotiated final discounts ranged from 14–33 % (median: 20.0 %) for drugs with a non-quantifiable additional benefit (8) while it was 16–30 % (median: 25.0 %) for drugs with minor additional benefit (10) and 19–24 % (median: 21.5 %) for assessments with considerable additional benefit (2).

A correlation between extent of additional benefit and final discount did not become evident.

* discount set by Arbitration Board

Color code for assessments: minor additional benefit · considerable additional benefit · major additional benefit · non-quantifiable additional benefit

CONCLUSIONS

The results of our analysis clearly show a strong relationship between the presence of a RCT and a quantifiable additional benefit (minor, considerable or major) in the assessment of orphan drugs. Without a RCT a non-quantifiable additional benefit is most likely.
In contrast, there seems to be no obvious correlation between the additional benefit and the final reimbursement price. However, the additional benefit is only one of the components with a possible influence on the final reimbursement price. Other components include the orphan drug's price in other European countries as well as the therapy costs of comparable therapies (if existing).

REFERENCES

- Final decisions on early benefit assessment were taken from the G-BA website: www.g-ba.de/informationen/nutzenbewertung/ (English version [less recent]: www.english.g-ba.de/benefitassessment/resolutions/)
- Initial prices and negotiated final prices were taken from: ABDATA Pharma-Daten-Service der Werbe- & Vertriebsgesellschaft Deuscher Apotheker (WuV), ABDA-Artikelstamm, www.pharmazie.com/dacon32/global/infoseiten_eng/abdaartikelstamm.htm

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