FACTORS INFLUENCING THE SHI COSTS OF ORPHAN DRUGS AFTER THEIR RENEWED REGULAR BENEFIT ASSESSMENT DUE TO EXCEEDING THE SALES THRESHOLD IN GERMANY

Brückel S [1], **Reindl S** [1], **Tran TT** [2]

[1] **IGES Institut GmbH**, Nuremberg, Germany [2] **IGES Institut GmbH**, Berlin, Germany

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Objectives

Orphan drugs (ODs) have privileges in the early benefit assessment (EBA) and the subsequent price negotiation in Germany. The added benefit (AB) is acknowledged by law (§ 35a SGB V) and thus no appropriate comparator therapy (ACT) is defined by G-BA. If sales of the OD exceed a threshold of 30m€ (50m€ until Nov 2022), the product is subjected to a regular EBA, i.e. comparison against an ACT. The objective of this study was to evaluate factors influencing the change of yearly therapy costs (YTC) for the SHI between a drug's initial EBA as an OD and its subsequent regular EBA procedure.

Methods

All EBAs exceeding the sales threshold and completed price negotiations by March 2023 were analysed using the IGES ARA database. The analysis of change in YTC was carried out at active ingredient level. For this purpose, YTC of subpopulations were weighted by size of subpopulations according to the G-BA resolution. The following explanations are always based on a population-weighted analysis at the active ingredient level. A multiple regression was used to analyse the change in YTC in dependance of change of AB (cAB), patient numbers, availability of an active ACT (besides BSC) and quantifiable AB in OD procedure. A test for multicollinearity using a variance influence factor (vif) was performed to check whether several predictors in the regression correlate with each other. The variables change in YTC and cAB were calculated as follows:

- Change YTC:
  The YTC after the price negotiations of the exceedance procedure are extracted from the IGES ARA and constitute the YTC approximately one year after the procedure, when the effects of a price negotiation become visible in the prices. The YTC of the exceedance procedure are the YTC of the exceedance procedure published in the resolution. At this point in time, the influence of any previous procedures is already reflected in the price.

$$change_{YTC} = \frac{YTC\ after\ price\ negotiations\ exceeding\ procedure}{YTC\ exceeding\ procedure} - 1$$

- cAB:
  The population-weighted proportion of AB was determined for both the OD procedures and the exceedance procedures. Subsequently, the proportion of patients with an AB from the total population in the OD/exceedance procedure was determined. The AB was considered in a binary manner (AB: yes/no) and the extent of the AB was neglected. Due to the legally assumed AB, the proportion in the OD procedure was 100% for each active ingredient. If a drug was assessed in a subpopulation in the exceedance procedure with a non-quantifiable additional benefit, this represented a "verified" additional benefit. In this case, the classification of the non-quantifiable additional benefit is circumvented by the binary approach.

$$change_{AB} = AB\ after\ exceeding - 1$$

Results

A total of 17 drugs were analysed. In 3 of 17 cases, an AB was granted for the whole population of the OD after regular EBA. In 7 cases, there was an AB for at least one subpopulation and in the remaining 7 cases no AB was granted at all.
The YTC after sales exceedance changed between -43% and +14%. Change in YTC depending on change in AB is shown in Figure 1 for each active ingredient.
If an AB is granted for the whole population (= change in AB 0%) in the regular EBA, an increase of YTC between 5% and 14% is observed. For procedures in which the AB is maintained in at least one population (= change in AB >0-<100%), the change in YTC ranges between -2% and -43%. Procedures that could not prove an AB (= change in AB 100%) in any population in the regular EBA led to a change in YTC of -5% to -40% .

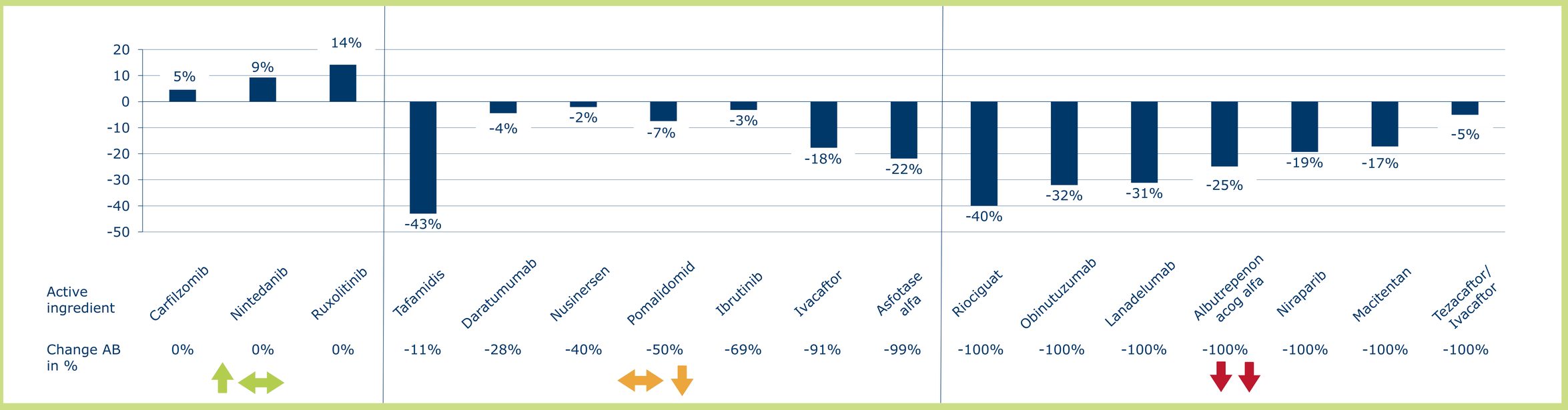
Table 1: Results of the regression analysis and the VIF test

	Model	
	(1)	(2)
Number of observations	17	17
Variable		
Intercept	-0.048 (0.114)	0.080 (0.111)
Change of patient numbers	0.002 (0.016)	0.228 (0.095)
Quantifiable AB in OD procedure	0.023 (0.095)	0.006 (0.014)
Availability of an active ACT	-0.136 (0.113)	-0.028 (0.084)
Change of AB	-	-0.085 (0.099)*
Test statistics		
R^2	0.103	0.392
Adjusted R^2	-0.104	0.189
F	0.177	0.151
VIF		
Change of patient numbers	1.027	1.037
Quantifiable AB in OD procedure	1.026	1.098
Availability of an active ACT	1.012	1.062
Change of AB	-	1.118

Standard errors are shown in parentheses.
+ p ≤ .10; * p ≤ .05; ** p ≤ .01; *** p ≤ .001; two-sided tests.

Results of the regression analysis in Table 1 reveal a statistically significant correlation between the change in YTC and the change in AB. Linear regression is able to explain 39.2% (adjusted R^2=19%) of the variation. Multiple regression analysis indicate that cAB had a significant influence on YTC (p=0.03) whereas the other variables (patient numbers, availability of an active ACT (besides BSC) and quantifiable AB) had not (p>0.05). The calculation of vif showed no multicollinearity.

Figure 1: Change in YTC in relation to the change in AB for each active ingredient



Conclusions

The results of the regression analysis reveal a correlation between the change in the additional benefit and negotiated reimbursement amounts resulting in a financial impact for SHI and pharmaceutical company depending on the actual result of the regular benefit assessment. The significant impact of cAB on YTC in Orphan drugs is in line with the objective of AMNOG, i.e. interdependence of reimbursement amount and additional benefit.

References

Gemeinsamer Bundesauschuss: Tragende Gründe zum Beschluss des Gemeinsamen Bundesausschusses über eine Änderung der Arzneimittel-Richtlinie (AM-RL), https://www.g-ba.de/informationen/nutzenbewertung/ (depending on respective EBA)

IGES ARA - AMNOG Resolution Analyzer (https://ara-info.iges.com/Home)

IGES Institut GmbH | Friedrichstraße 180 | D-10117 Berlin
www.iges.com