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Price regulation through benefit assessment for rare diseases

In October 2022, the SHI Financial Stabilisation Act lowered the benefit assessment-relevant sales threshold for orphan drugs from 50 to 30 million euros. In view of the discussion on the pricing of orphan drugs, it therefore seems appropriate to examine the development of population-normalised treatment costs more closely. To this end, a study was already conducted in 2022 to analyse the treatment costs of orphan drugs (1), looking at the period before the introduction of the AMNOG (2005 to 2010) and the period after the introduction of the AMNOG (2011 to 2021). The calculations have now been validated as part of a study update, whereby the identical population was used. The update enabled an inflation adjustment based on the price index for 2022 (2) as well as seamless treatment costs after AMNOG assessment and price negotiation for all orphan drugs. As a result, the study results are confirmed. On average, the rising annual treatment costs of orphan drugs since the introduction of the AMNOG are more than compensated for by low patient numbers.

>> For orphan drugs, there are special features under EU Community law with the aim of promoting the research, development and marketing of suitable medicinal products by the pharmaceutical industry (3). Following on from this, special regulations apply to orphan drugs in the AMNOG procedure, which continue the objectives of the European regulation at national level (see Section 35a para. 1 p. 11 f. SGB V), but which are cancelled above a certain turnover threshold. The turnover threshold of 30 million euros stipulated by the SHI Financial Stabilisation Act (GKV-FinStG) refers, as did the previous threshold of 50 million euros, to the pharmacy retail price and a period of 12 months. This increases the likelihood of negotiating higher AMNOG discounts and the savings that can be realised in this way are estimated to be between 100 and 200 million euros per year in the medium term (4). In principle, the question arises as to whether and to what extent the AMNOG also fulfils its price regulation function for orphan drugs. In order to examine this, two working hypotheses were analysed: 1. the introductory prices of orphan drugs have not increased or have only increased slightly since the introduction of the AMNOG, 2. the AMNOG discounts granted result in savings compared to the situation before the introduction of the AMNOG.

Inflation-adjusted price development

Due to the long time series of 17 years, the prices have been adjusted for inflation. The adjustment relates to the year 2022, based on information from the Federal Statistical Office on the consumer price index. In addition to the price, the number of patients for whom the medicine has been authorised is crucial for determining the budget impact of a medicine: if a therapy is only indicated for very few patients, the total costs for health insurance are manageable despite high prices.

In order to take into account the influence of the number of patients, this was researched for the period 2005 to 2010 for each newly authorised orphan drug (Pubmed NCBI, Orphanet, European Medicines Agency). For the period since 2011, the information according to the G-BA resolutions was used with the help of the IGES ARA AMNOG Resolution Analyser© (6), which must be determined by the G-BA as part of the AMNOG process for each target population considered in the benefit assessment. The 'normalised treatment costs' were determined on the basis of price and number of patients by multiplying the annual treatment costs for each drug at the time of market launch by the SHI target population. This results in a meaningful figure that is suitable for comparing the costs incurred for orphan drugs in Germany in relation to the budget impact.

Market entry of 145 orphan drugs by 2021

In the period between 2005 and 2021, a total of 145 orphan drugs entered the market in Germany (Fig. 1). 133 of these drugs were included in the analysis. 12 medicinal products could not be included for the following reasons Exemption or suspension of the procedure, withdrawal from the market as well as missing price data, as the drugs were only used on an inpatient basis. For the period 2005 to 2010 (before AMNOG), this results in a quantity of 43 medicinal products, for the period 2011 to 2021 (after AMNOG) there are 90 medicinal products, for which the normalised treatment costs were also calculated after price negotiation.

Both working hypotheses are confirmed with regard to the normalised treatment costs. The costs for orphan drugs in Germany have not increased on average since the introduction of the AMNOG (minus 6%). If the negotiated AMNOG reimbursement amounts are taken into account, the normalised treatment costs after the introduction of the AMNOG are significantly lower than before (minus 25%) (Fig. 2). At 20.0%, the average discount after price negotiation for orphan drugs is only slightly lower than the figure for the market as a whole of 21.6% (7).

The analysis shows that the AMNOG also fulfils its function as a price regulation instrument for orphan drugs. Since the AMNOG was introduced, the inflation-adjusted normalised treatment costs for orphan drugs have fallen both in terms of the launch price and the reimbursement amount.

Decrease in treatment costs by 25%

The relationship between price and volume is crucial for the evaluation of the results. While the average annual treatment costs for orphan drugs increased from 101,000 euros (before AMNOG) to 253,000 euros (after AMNOG), the SHI target populations became significantly smaller - an average of 4,586 patients before the in-

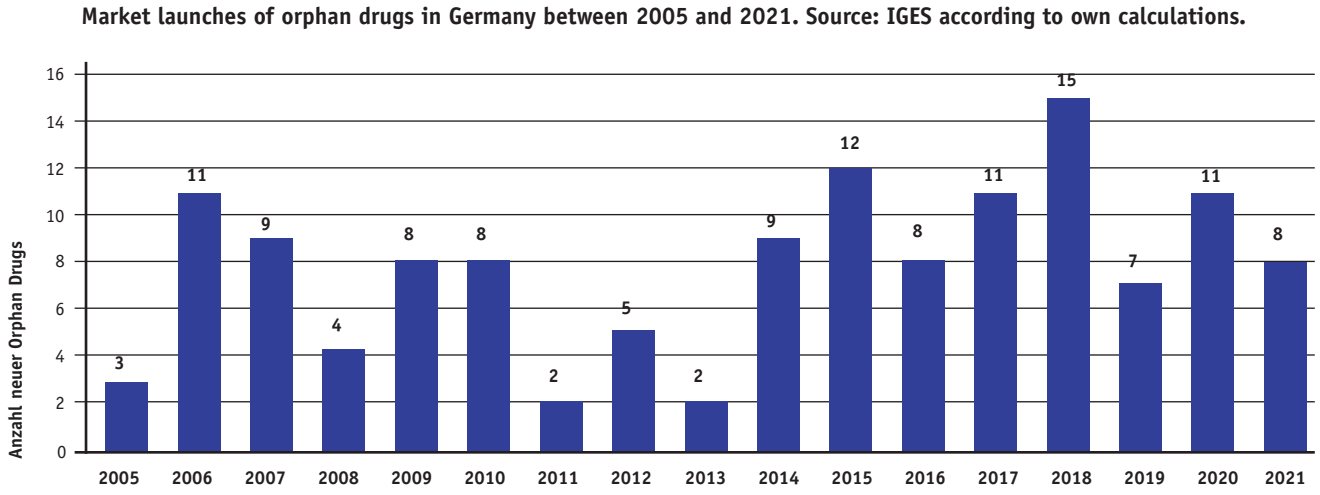


Abb. 1: *Markteinführungen von Orphan Drugs in Deutschland im Zeitraum 2005 bis 2021. Quelle: IGES nach eigenen Berechnungen.*

troduction of AMNOG compared to an average of 1,822 patients after the introduction.

In relation to the normalized treatment costs, both working hypotheses are confirmed. The costs for orphan drugs in Germany have not increased on average since the introduction of AMNOG (minus 6%). If the negotiated AMNOG reimbursement amounts are taken into account, the normalized treatment costs after the introduction of AMNOG are significantly lower than before (minus 25%) (Fig. 2). The average discount after price negotiations for orphan drugs is 20.0%, only slightly below the value of the overall market of 21.6% (7).

The analysis shows that the AMNOG also fulfils its function as a price regulation instrument for orphan drugs. Since AMNOG was introduced, the inflation-adjusted normalised treatment costs for orphan drugs have fallen both in terms of the introductory price and the reimbursement amount. <<

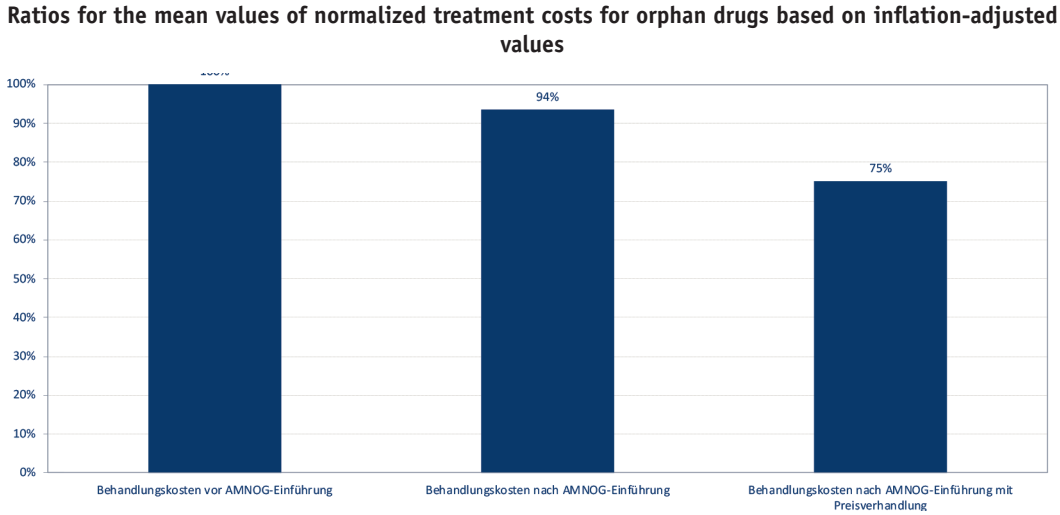


Abb. 2: *Relations for the mean values of normalized treatment costs for orphan drugs based on inflation-adjusted values Source: IGES according to own calculations.*

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Author's statement

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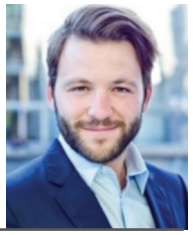
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