Relevance of the Orphan Drug-Regulation within AMNOG for Patient Care

An analysis based on a retrospective market simulation

Translated version - original study publication accessible in German language.

Introduction & research question

Orphan Drugs (ODs) are therapies developed to treat life-threatening or chronically debilitating rare diseases for which there are no satisfactory therapy options available. Their development is challenging as knowledge about these diseases is often limited, and there are typically only few patients available for clinical trials. As a result, the development of ODs is encouraged by the EU-regulation 141/2000.

Building on this EU-regulation, in Germany, a special protection mechanism applies for ODs (OD-regulation), which supports pricing and market access within the AMNOG-framework. For ODs, the added benefit is deemed to be proven with approval until the revenue threshold of €30 million per year is exceeded.

In light of the politically desired increase in the number of ODs in healthcare provision and the associated pharmaceutical expenditures, recurring health policy debates arise regarding the abolition or restriction of the OD-regulation (Staeck, 2022; GKV-Spitzenverband, 2022). Given the well-known challenges in the clinical development of ODs, this raises risks for the G-BA benefit assessment, pricing, and the potential to economically market an OD. Any restrictions of the OD-regulation must therefore be carefully evaluated regarding their impact on the potential market availability of these therapies.

This study investigates the risk of market withdrawals in the event of a hypothetical abolition of the OD-regulation by analyzing the consequences for the G-BA benefit assessment and the corresponding pricing implications, particularly with respect to the potential to economically market an OD.

Methodology of the study

The retrospective simulation includes market launch procedures of 76 ODs whose benefit assessments were completed between 2017 and 2023. For these ODs, the hypothetical benefit assessment and the associated price potential without OD-regulation were simulated. Subsequently, the discount applied to the actual reimbursement price negotiated under OD-regulation was used to analyze the potential to economically market the OD without OD-regulation. Based on this simulated discount, the risk of market withdrawal was classified.

(i) "Maximum" risk of market withdrawal due to generic price level
The average price discount upon generic entry is -73% on the price valid before patent expiry (Pro Generika e.V., 2022). If an OD has to accept an even greater discount on the price negotiated under OD-regulation after market entry, it can be assumed with near certainty that the OD will be withdrawn from the market.

(ii) "Very high" risk of market withdrawal due to additional discount of unprecedented level
The highest discount ever observed for an OD during its initial re-negotiation without OD-regulation is -42%. If an OD faces an even larger discount on the price negotiated under OD-regulation after market entry, a very high risk of market withdrawal needs to be assumed.

(iii) "Individual" risk of market withdrawal due to additional discount of previously observed level
Any additional discount on the price negotiated under OD-regulation bears a potential risk of market withdrawal. Discounts of up to -42% have already been observed. Up to this level of discount, an individual risk of market withdrawal must be assumed, which can, however, jeopardize market availability already depending on case-specific circumstances.

(iv) "Low" risk of market withdrawal due to no additional discount
If an OD does not have to incur any further discount on the price negotiated under OD-regulation (at which it was available at that time), it can be assumed that there is a low risk of market withdrawal.

Results

In the simulation of the benefit assessment without OD-regulation, only 21% of ODs demonstrate an added benefit, while 79% of ODs do not achieve any added benefit. This results in average discounts of -52% on the price already negotiated under OD-regulation and -62% on the freely set launch price. Consequently, 45% of ODs are exposed to a maximum risk of market withdrawal, as their price declines to generic price levels (see Figure 1). Furthermore, 12% of ODs face a very high risk of market withdrawal due to unprecedented discounts of more than -42% on the price negotiated under OD-regulation. Further 20% of ODs are subject to an individual risk of market withdrawal with discounts of up to -42%, while only 24% of ODs are considered to be at low risk, as they do not incur any additional discounts on the price negotiated under OD-regulation.

Additional analyses of market withdrawal risks for different therapeutic groups indicate that soloists and non-soloists, as well as oncological and non-oncological therapies, and pediatric and non-pediatric therapies, are exposed to very similar market withdrawal risks. Novel therapies (Advanced Therapy Medicinal Products, ATMPs), which include highly innovative gene and cell therapies, face an elevated risk of market withdrawal.

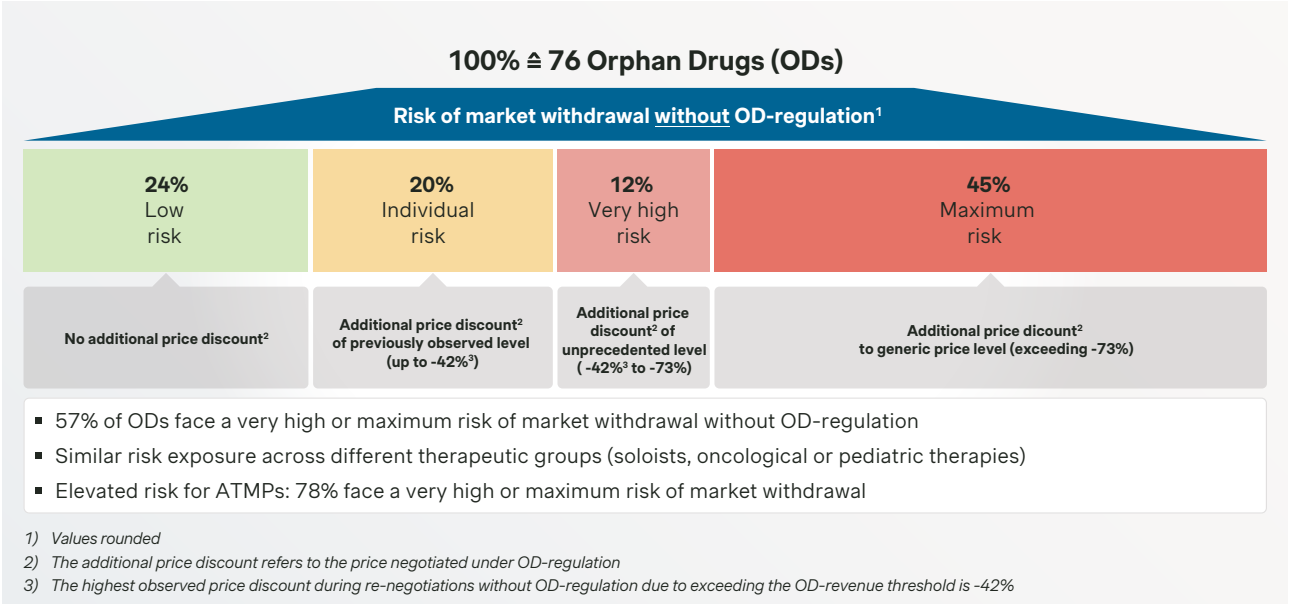

Figure 1: Summary of study results

Conclusion

The simulated abolition of the OD-regulation from the German market indicates that a significant proportion of ODs would exhibit no added benefit, resulting in a substantial decline in price levels. More than half of the ODs face a very high to maximum risk of market withdrawal due to their inability to demonstrate an added benefit, exposing them to dramatic price reductions. The risk exposure is consistent across various therapeutic groups (soloists/non-soloists, oncological/non-oncological therapies, and pediatric/non-pediatric therapies), with ATMPs facing an elevated risk. Additionally, a considerable number of ODs are subject to individual market withdrawal risks, further amplifying the overall risk potential of ODs. Only about a quarter of ODs remain at low risk.

These findings highlight the fundamental importance of the OD-regulation within the AMNOG-framework in ensuring the broad care for patients with rare diseases. Abolishing the OD-regulation or restricting it to individual therapeutic groups would not be feasible without far-reaching negative consequences for patient care with ODs in Germany.

List of references

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Background of the study

This study was conducted by Simon-Kucher on behalf of the Verband forschender Arzneimittelhersteller (vfa).

About Simon-Kucher

Simon-Kucher is a global consultancy focused on driving measurable growth for clients through optimized commercial strategies in product, price, innovation, marketing, and sales. With 2,000+ employees across 30+ countries, we leverage deep customer insights to unlock better growth. For nearly 40 years, Simon-Kucher has been a leader in healthcare and life sciences consulting, helping pharmaceutical, medical technology, and consumer healthcare companies maximize the value of their innovations. Since AMNOG's inception, we have supported pharma clients in strategic planning, market entry, and reimbursement negotiations.

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