Outcomes of Benefit Reassessments of Orphan Drugs in Germany After Exceedance of the Lowered Sales Threshold of 30 Million Euros

Dr. Lydia Frick, Lukas Heinrich Schoppmeyer, Malte Glüsen, Heike Kielhorn, Univ.-Prof. Dr. med. Matthias P. Schönermark
SKC Beratungsgesellschaft mbH

ISPOR acceptance code: HTA208
Poster presented at ISPOR Europe 2024
17-20 November 2024 in Barcelona, ES.

OBJECTIVES

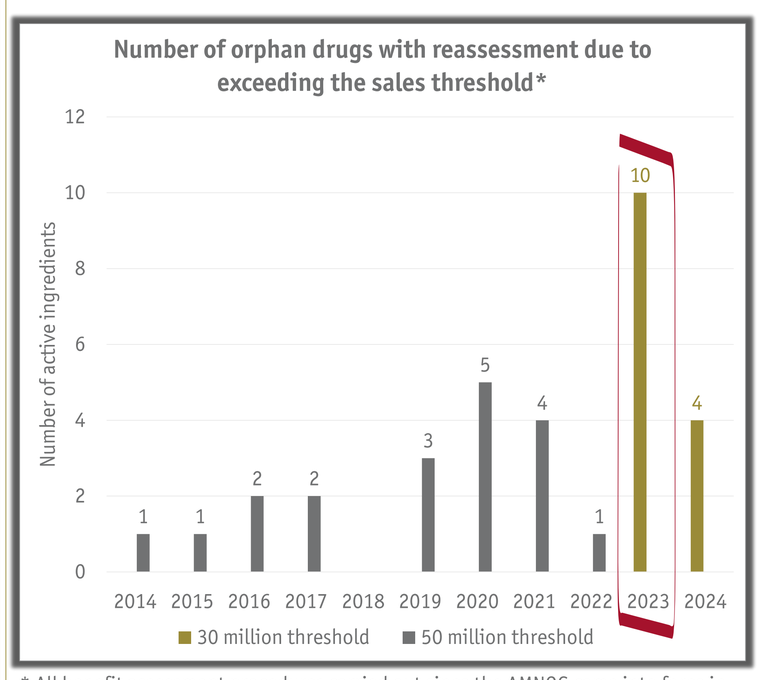
In German HTA, an added benefit is granted by law for orphan drugs until the drug's turnover exceeds a legally defined threshold within 12 months, leading to a reassessment against an appropriate comparator therapy (ACT). As part of the SHI-Financial Stabilization Act, this threshold was lowered from 50 million to 30 million euros. The objective of this study is to analyze the effects of this reduction on the benefit assessment outcomes and the price level of reassessed orphan drugs.

METHODS

Orphan drugs that have exceeded the 50-million-euro threshold were compared with those that have exceeded the 30-million-euro threshold by November 2024. The number of orphan drugs, the granted added benefit and the resulting price level compared to the ACT were examined.

RESULTS

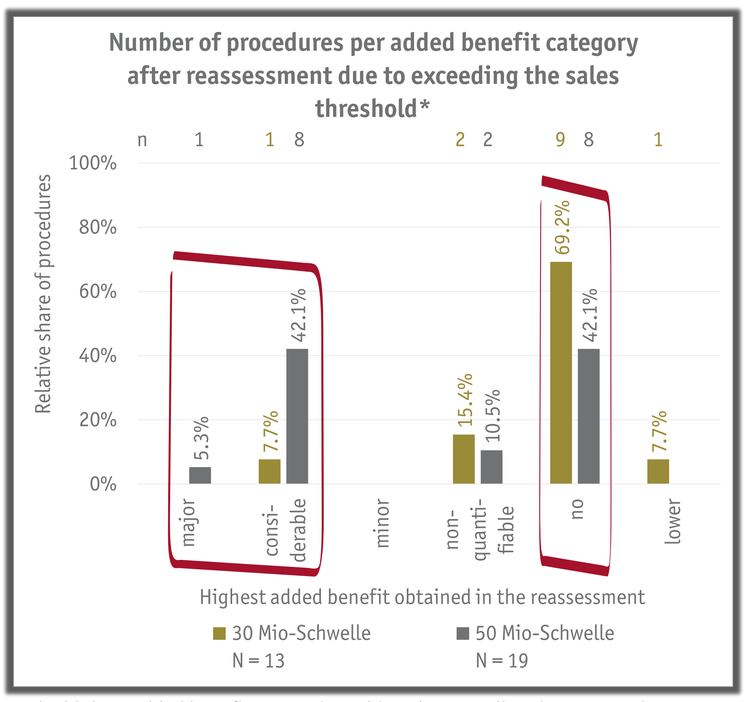
In 2023, there was a peak in reassessments with 10 orphan drugs affected by exceeding the sales threshold of 30 million euros.



- By 2022, 19 orphan drugs were re-evaluated in comparison to the appropriate comparator therapy (ACT) due to exceeding the sales threshold of 50 million euros valid until then.
- **In 2023, there was a peak in reassessments with 10 orphan drugs affected by exceeding the sales threshold of 30 million euros, which has been in force since November 2022.**
- In 2024, 4 orphan drugs have so far been affected by a reassessment due to exceeding the sales threshold of 30 million euros.

* All benefit assessment procedures carried out since the AMNOG came into force in 2011 due to the sales threshold for orphan drugs being exceeded were considered. In these procedures, **34 different orphan drugs** were evaluated, of which Kaftrio was excluded from the analysis because it immediately exceeded the sales threshold.
Status of the analysis: 01.11.2024

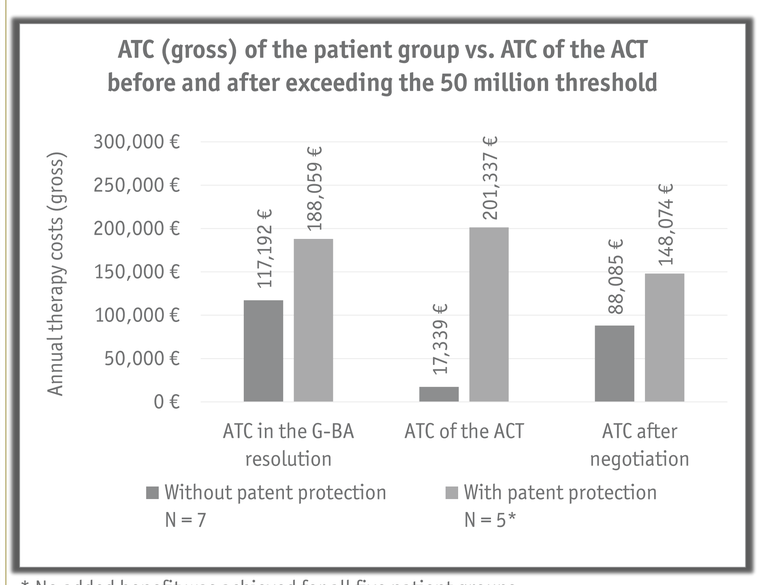
When the 30 million sales threshold was exceeded, it was more common for no added benefit to be achieved and less common for at least considerable added benefit to be achieved.



- In benefit assessment procedures due to exceeding the 50 million sales threshold, **an at least considerable added benefit was** achieved **in 47% of cases in at least one patient group**, including nusinersen/SPINRAZA with a considerable added benefit compared to BSC.
- If the sales threshold of 30 million euros is exceeded, **a considerable added benefit** has so far been awarded **in only one benefit assessment procedure** (tebentafusp/KIMMTRAK).
- In contrast, **in 69% of the procedures no added benefit** was achieved because the 30 million sales threshold was exceeded, **while in only 42% of cases no added benefit** was awarded if the 50 million sales threshold was exceeded.

* The highest added benefit categories achieved across all patient groups in a benefit assessment procedure are shown. **One active substance/ procedure is not included** in this analysis, as the reassessement procedure has been discontinued after exceeding the 30 million sales threshold.

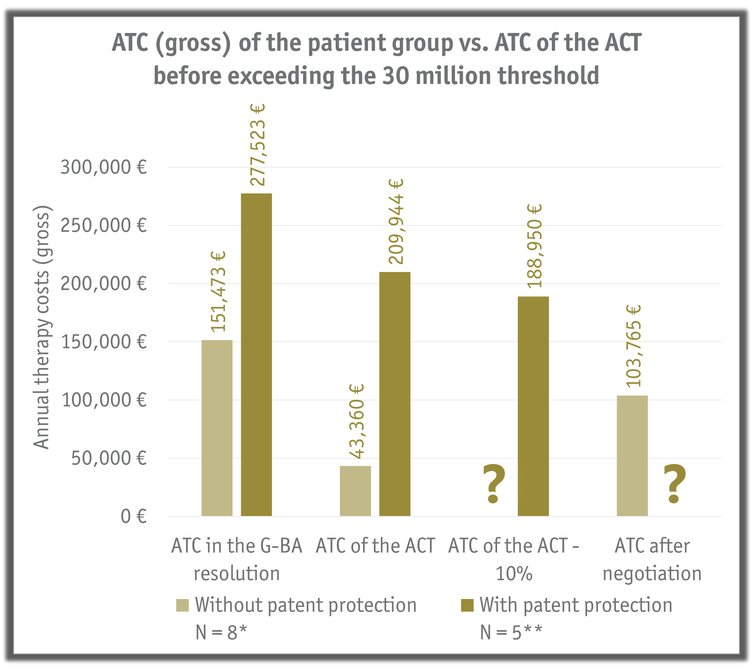
If the 50 million sales threshold was exceeded, there were price premiums for ACTs without patent protection and significant discounts for patent-protected ACTs.



- The annual therapy costs (ATC) of orphan drugs with an ACT without patent protection were significantly lower than for orphan drugs with a patent-protected ACT.
- As expected, the ATC of ACTs without patent protection were very low compared to ACTs with patent protection.
- The average **negotiated ATC of orphan drugs with an ACT without patent protection** after exceeding the 50 million sales threshold **exceeded the costs of the ACT many times over**.
- In the case of **orphan drugs with a patent-protected ACT, a significant decline in the price level** was seen after the renegotiation of the reimbursement amount for exceeding the sales threshold.

* No added benefit was achieved for all five patient groups.
The average ATC of orphan drugs with patent-protected ACT were already below the average ACT costs before the start of the renegotiation of reimbursement amounts.
Status of the analysis: 01.11.2024
Of 106 benefit assessment procedures due to exceeding the 30/50 million sales threshold, the patient groups of procedures with an at highest minor, non-quantifiable, none or lower added benefit, without best supportive care or watchful waiting as an ACT have been considered for the analysis. Kaftrio and Zolgensma have been excluded. In total 25 patient groups were included in the analysis.

The annual therapy costs of orphan drugs with patent-protected ACT were significantly higher than the ACT costs before the negotiations.



- The ATC of orphan drugs with an ACT without patent protection were significantly lower than for orphan drugs with a patent-protected ACT.
- As expected, the ATC of ACTs without patent protection were significantly lower than ACTs with patent protection.
- The average ATC of orphan drugs with patent-protected ACT were significantly higher than the average ACT costs before the start of the renegotiation of reimbursement amounts.
- Apparently the so called "shall"-rule has been applied to the three ACTs without patent protection in a similar way as it has been in the case if the 50 million sales threshold has been exceeded in the past.

* For the orphan drugs of only three patient groups with an ACT without patent protection there is a negotiated reimbursement amount.
** No added benefit was achieved for all five patient groups with a patent-protected ACT. There is no negotiated reimbursement amount today.
Status of the analysis: 01.11.2024

CONCLUSION

The risks of potentially exceeding the lowered 30 million sales threshold for orphan drugs should be carefully analyzed for each individual case. To this end, the possibility of exceeding the sales threshold should be taken into account in the initial pricing of the drug, but also in the intended area of application and the associated size of the target population of the orphan drug as all these factors can be determinants for whether the threshold will be exceeded resulting in a reassessment of the drug. The initial advantages of an orphan status in the context of the AMNOG benefit assessment must be weighed against the risk of reassessment due to the sales threshold being exceeded.

SKC is a strategic consultancy focused on the increasingly challenging market access environment of innovative drug products based in Germany. We support the successful market access both on a strategic and an operational level. For nearly 20 years, our highly experienced team has been supporting our clients in solving their strategically complex questions.

SKC joined the MAP group, thereby broadening further the group's European platform and combining crucial local expertise to drive our client's global success. The MAP Group is a pan-European specialist strategic consultancy for pharmaceutical and biotechnology that has established operations across the UK, Ireland and Benelux, and has served more than 200 clients in 20 markets.

All analyses have been generated by data from SKC's proprietary MAIS (**Market Access Intelligence System** = MAIS) database. This database contains and links information on completed and ongoing benefit assessments according to §35a SGB V of the German Federal Joint Committee (Gemeinsamer Bundesausschuss, G-BA). The MAIS-database records and evaluates relevant information from the dossier, the benefit assessment by IQWiG or the G-BA, the G-BA resolution as well as the Lauer-Taxe. It also contains an up-to-date overview of all procedures and their status.

schönermark kielhorn collegen

ACCELERATING PATIENT ACCESS

AxTalis

We are the market access special forces.

SKC Beratungsgesellschaft mbH | Hannover
www.skc-consulting.de | Email: frick@skc-beratung.de