ORANDA THERAPEUTICS

Could improved competition in patent expired rare disease medicines improve patient access?

INTRODUCTION

- European legislation has successfully increased the number of orphan medicinal products (OMPs) approved over the past two decades.[1,2]
- However, access to EMA-approved OMPs remains suboptimal.[2]
- It has been widely suggested that following the loss of market exclusivity, society benefits from price reductions driven by the entry of generic alternatives and subsequent price competition.
- Previous investigations indicate that generics typically enter the market, on average, 14 years after the approval of the originator, resulting in substantial reductions in drug prices.[3,4,5]
- This pilot study aimed to assess whether this trend is observable for EMA-designated OMPs approved between 2001 and 2009.

METHODS

- All orphan medicinal products withdrawn from the European Community Register between 2008 and 2020 were identified and those approved between 2001 and 2009 were shortlisted for review.[6]
- OMPs withdrawn at the request of the sponsor were excluded from the analysis.
- The final dataset comprised 31 OMPs withdrawn from the register at the conclusion of their 10-year market exclusivity period (expired OMPs).
- Sales data from 2021 to 2023 and pricing data from October 2020 to September 2024 were collected to identify expired OMPs with commercially available generics in Germany.[7,8]
- These data were used to evaluate the impact of genericisation on the pricing of these expired OMPs in Germany.

RESULTS

- 31 expired OMPs were included in the analysis, and despite the loss of market exclusivity, only 9 had undergone genericisation by 2020[9] (Table 1):

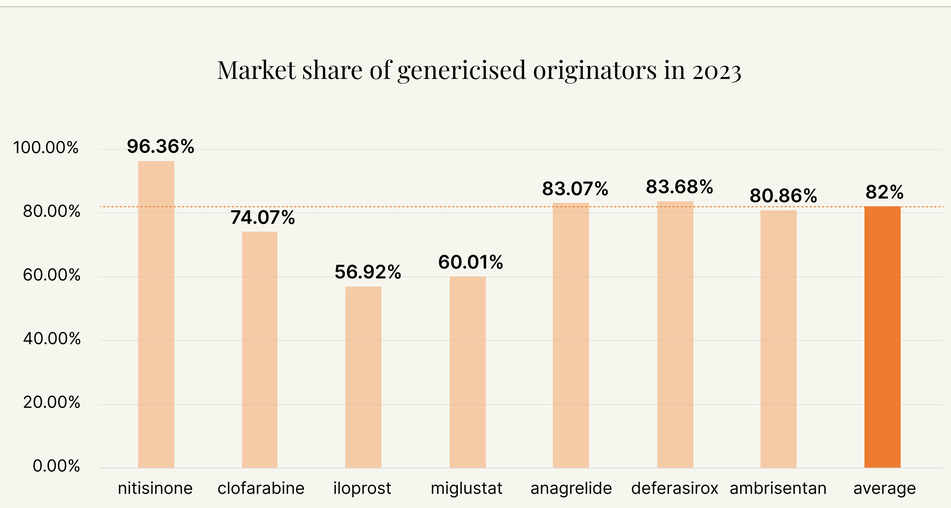
TABLE 1: OMPS APPROVED BETWEEN 2001 AND 2009 THAT HAVE SEEN GENERIC

Brand Name	International Non-proprietary Name
Orfadin®	nitisinone
Evoltra®	clofarabine
Ventavis®	iloprost
Trisenox®**	arsenic trioxide
Busilvex®*	busulfan
Zavesca®	miglustat
Xagrid®	anagrelide
Exjade®	deferasirox
Volibris®	ambrisentan

*The European Commission withdrew the marketing authorisation for Busilvex (busulfan) in the European Union (EU) in January 2023 at the request of the marketing authorisation holder, Pierre Fabre.[10] Busilvex® was excluded from further analysis given the lack of comparative data for the originator. "Generic' bulsufan continues to be available. ** Trisenox ® (arsenic trioxide) was excluded from analysis due to lack of available data.

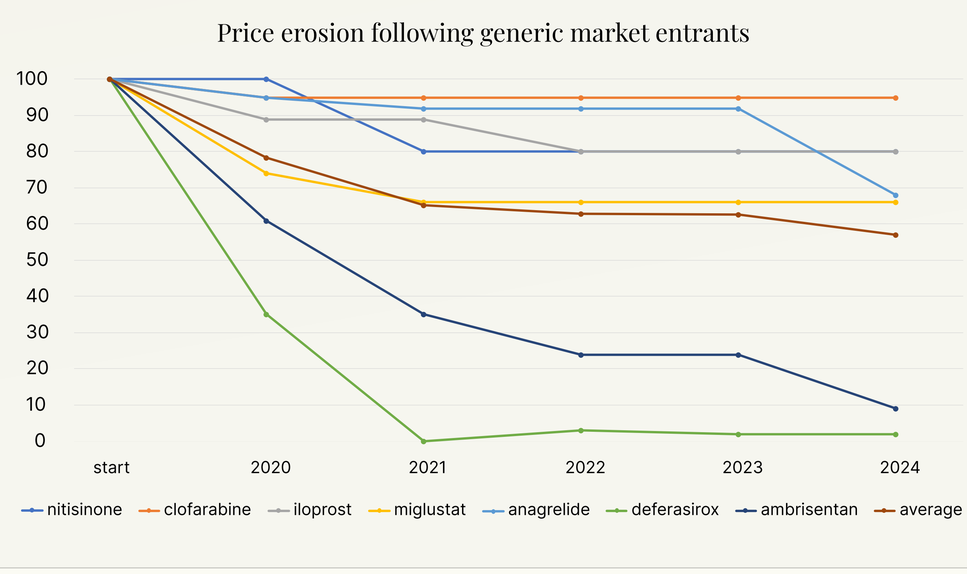
- Where generic competition was observed, generic version of the expired OMPs had on average achieved a market share of 82% by 2023 (highest 96% and lowest 57%)[9] (Graph 1):

GRAPH 1: MARKET SHARE OF GENERICISED ORIGINATORS IN 2023



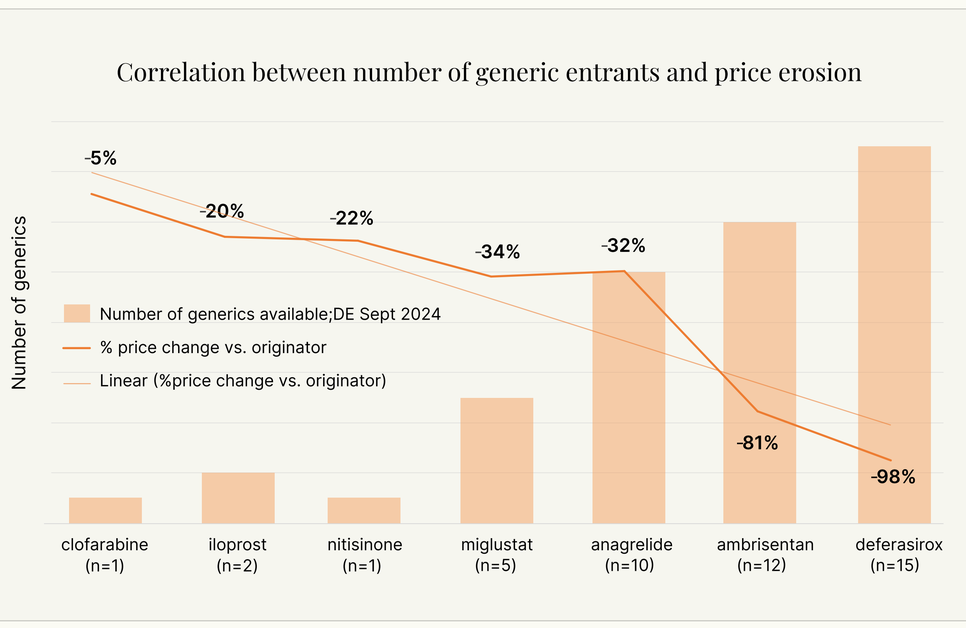
- Where generics are available, the average price demonstrated a significant decline over time, with observed reductions ranging from 5% to 98%, yielding a mean price decline of 37%[9] (Graph 2):

GRAPH 2: PRICE EROSION FOLLOWING GENERIC MARKET ENTRANTS OCT 2020 AND SEPT 2024



- The average number of generic entrants per expired OMP was 6, with a range from 1 to 15[9].
- A clear correlation was identified between the number of generic entrants and the magnitude of price reductions (15 generic entrants resulted in a 98% price decline vs. the originator, while 1 generic entrant corresponded to a 5% price decline vs. the originator), suggesting that increased competition facilitated greater price erosion.[9]

GRAPH 3: CORRELATION BETWEEN NUMBER OF GENERIC ENTRANTS AND PRICE EROSION



CONCLUSIONS

- These findings suggest that despite loss of exclusivity, only a minority of expired OMPs underwent genericisation within the observed timeframe.
- Where generic entrants become available, it can lead to substantial price reductions.
- The correlation between the number of generic competitors and price decline underscores the potential for increased generic competition to drive further cost savings.
- Encouraging the entry of generics for expired OMPs may play a role in addressing the ongoing challenges related to access of high-cost orphan medicines across Europe.

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