

How accessible are orphan medicinal products in Slovakia?

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INTRODUCTION

- Pricing, reimbursement and national health policy are crucial for providing patients access to orphan medicinal products (OMP). Slovakia is a small country with approximately 5.5 million inhabitants. It has a National plan as well as an action plan with an assigned budget. However, the availability of OMP products is limited. In the last 3 years, there were several important legislation changes. We present some supportive data, to call for action.

METHODS

- We performed an analysis of MP for rare diseases which were launched to Slovak market in time period 012018 – 022020. The information about the authorisation of OMP products comes from the EMA website. The information about the drugs directly available in Slovakia comes from the website of the Ministry of Health (01.02.2020). Orphan status was evaluated in time, when the drug was launched at the national market in Slovakia. The data are presented as mean±SEM. The relationship between the time to launch of OMP vs nonOMP was tested by independent T test, $p < 0.05$.

RESULTS

- In the time period 2011 – 2017 almost no orphan medicinal product (OMP) reached the national market. OMP were provided at the patient individual basis. At the beginning of 2020 (22020) 29 OMP out of 105 OMP (28%) were directly available for Slovak patients. Out of them 22 OMP (79%) entered the Slovak market due to the changes in 2018, that means in last 2 years. The Slovak legislation doesn't pay special attention to the orphan status in the process of pricing and reimbursement of drugs. This process takes into account low prevalence, defined as the prevalence ≤ 1 in 50 000. In the time period 12018-122019 resp until 22020 22 OMP and 29 medicinal products for rare disease without orphan status were launched in Slovakia. The average time to be launched in Slovakia was almost half of the market exclusivity of the OMP thus almost 5 years. There was no statistical difference in the average time of launching drug in Slovakia between the two groups: OMP (4.13 ± 0.78 , [0.78 – 16.87]) vs medicines for rare diseases without orphan status (5.74 ± 0.73 , [0.78 – 16.92]). Together 3 OMP (ELAPRASE, ORFADIN, FIRDAPSE) were launched after the market exclusivity expired and other four (XYREM, ILARIS, LYNPARZA, VENCLYXTO), after the orphan status was withdrawn. LATRUVO the OMP which was withdrawn from the European market, managed to reach the Slovak market before the withdrawal.

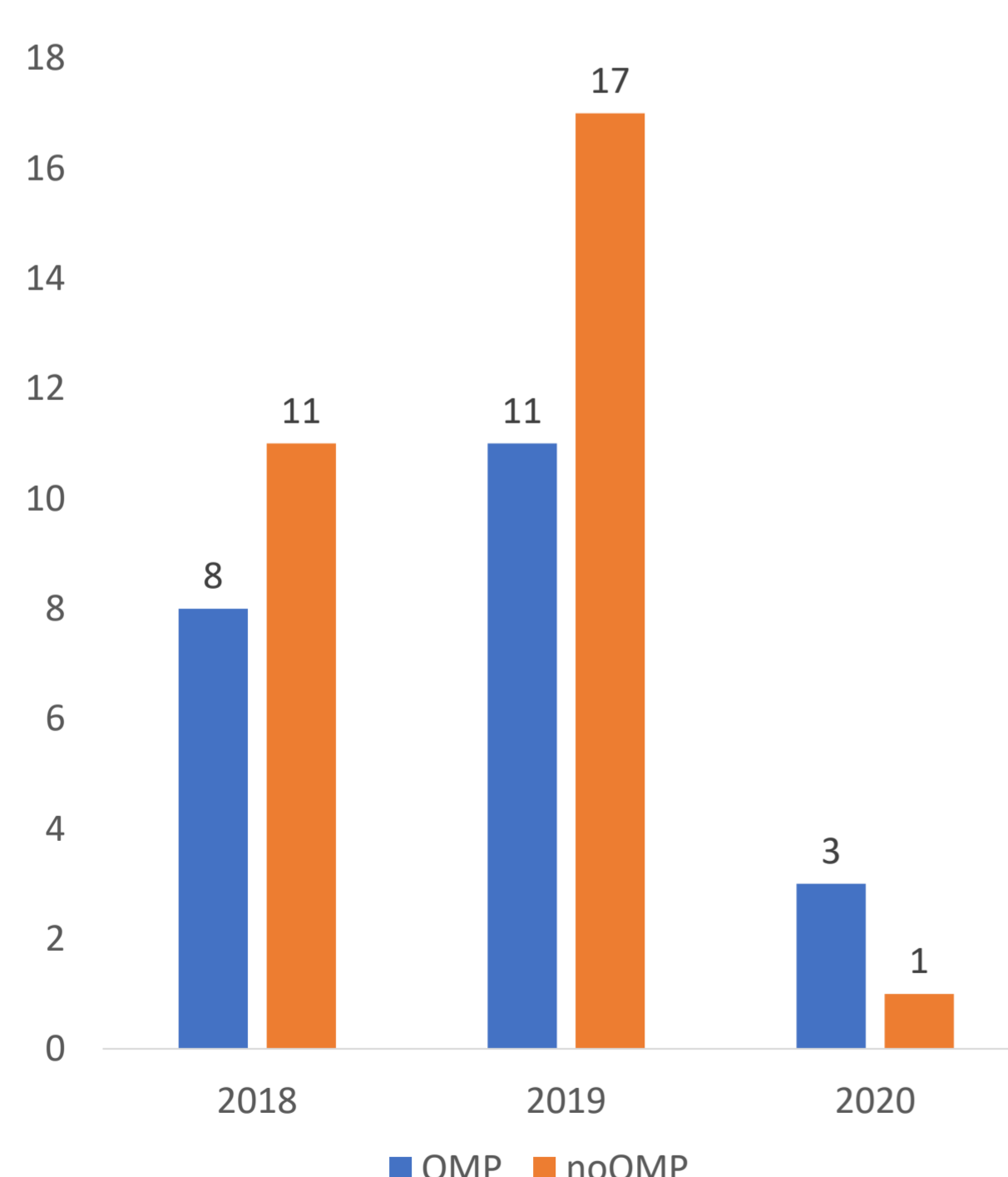


Fig. 1 Launching MP for rare diseases after the change of legislation

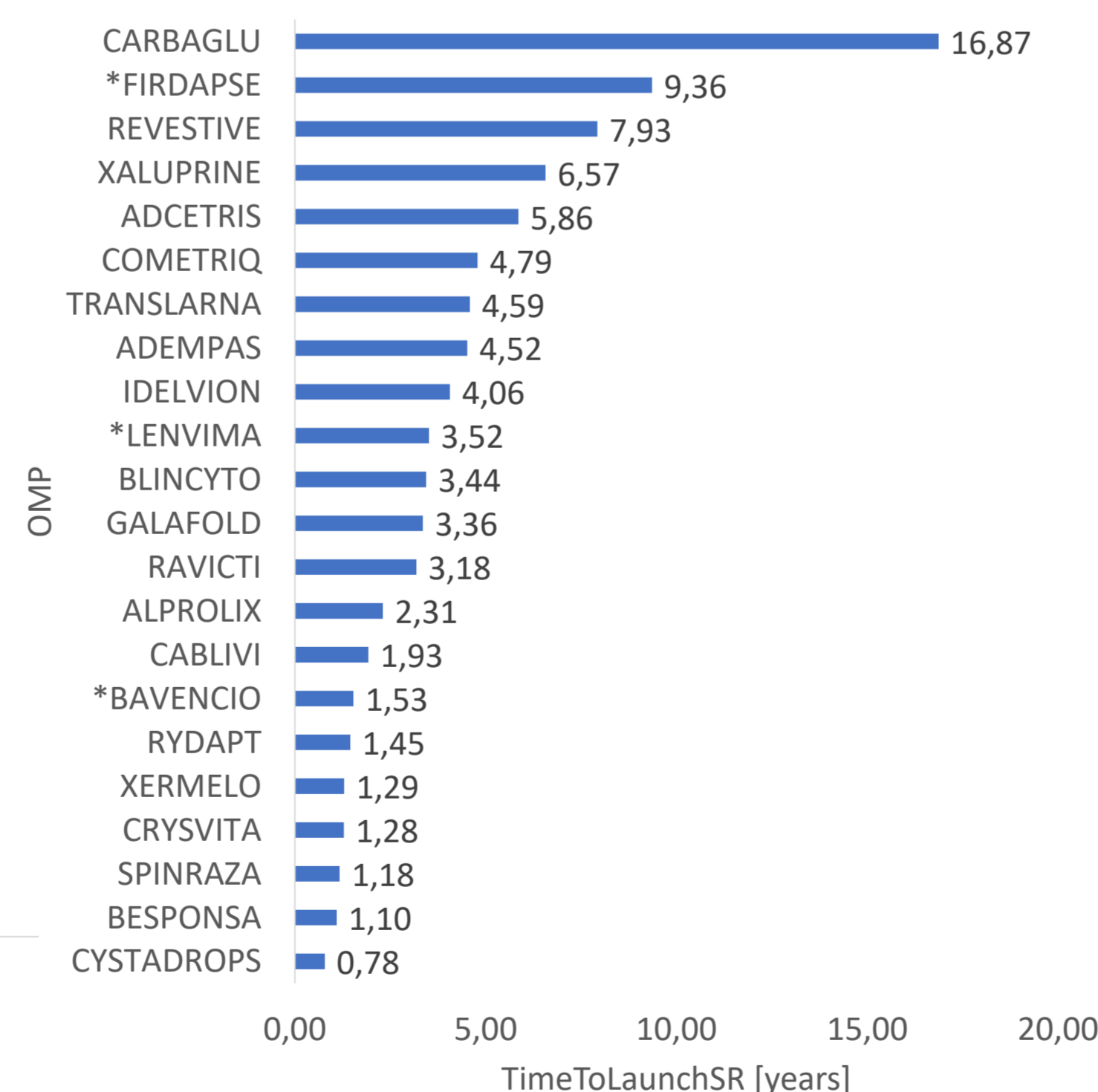


Fig. 2 Time to reach the Slovak market

*were OMP at the time of launching SR, afterwards status lost/removed

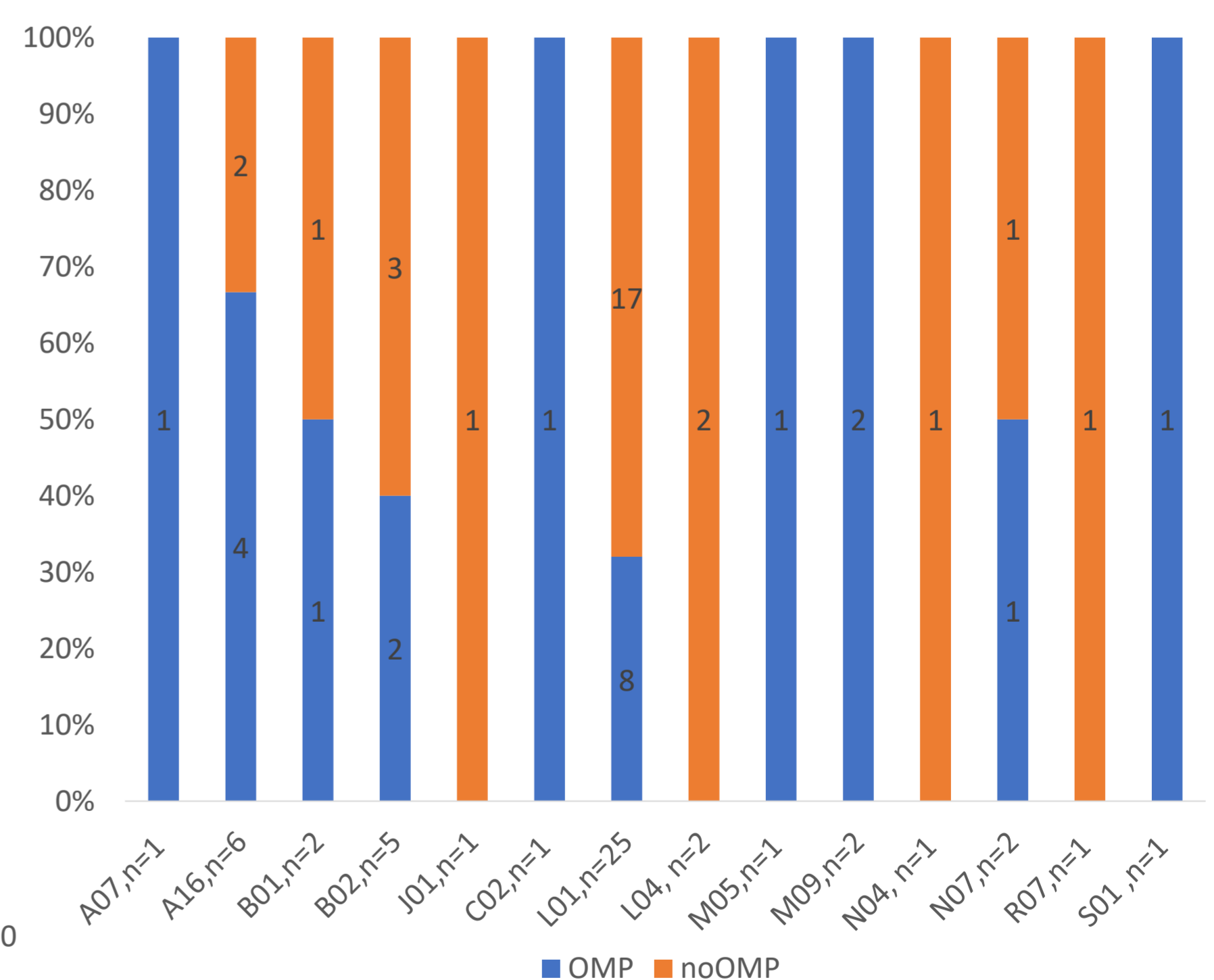


Fig. 3 MP for rare diseases after the change of legislation according to ATC class

CONCLUSION

- Although a lot of effort is given R&D as well as accessibility of OMP at the European and international level, patients are lacking the advantages of existing treatments and it is also depending on the country they live. In Slovakia 73% OMP are missing at the national level. The relative moderation in the legislation beginning 2018 changed the situation, however orphan status isn't meaningful advantage, when launching the OMP in Slovakia. There was no significant difference between OMP and MP indicated for rare diseases without orphan status (OMP vs no OMP: 4.13 vs 5.74 yrs). In Slovakia the impact of orphan status on pricing and reimbursement is low, even taking low prevalence 1:50 000 into account, doesn't change the situation. This indicates a big gap in treatment accessibility as well as quality of care for Slovak rare disease patients.