Comparative analysis of legislative requirements about patients' access to biotechnological drugs for rare diseases in Central and Eastern European Countries

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Objectives: The aim of the study was to make a comparison between the access of patients with rare diseases (RDs) to biotechnological drugs in several Central and Eastern European (CEE) countries. We focus on the legislative pricing and reimbursement requirements, the availability of biotechnological orphan medicinal products (BOMPs) for RDs and on their reimbursement expenditures. **Methods**: A questionnaire-based survey was conducted among experts from ten CEE countries: Bulgaria, Croatia, Estonia, Greece, Hungary, Poland, Romania, Slovakia, Serbia and Macedonia. The legal requirements about reimbursement and pricing of BOMPs were collected. All BOMPs and medicines without prior orphan designations were extracted from the European List of OMPs, 2017. The reimbursement status of these medicinal products for 2017 in the included CEE countries as well as their expenditures and the share of their costs from the total public pharmaceutical budget for the period 2014-2016 were defined. **Results**: Our survey shows that some differences in the legal requirements about pricing and reimbursement of BOMPs exist. All European Union countries have developed and implemented pharmacoeconomic guidelines with or without some specific reimbursement requirements for OMPs. Cost-effectiveness analysis, cost-utility analysis, Markov models, meta-analysis and discount levels of cost and results are required only in Bulgaria, Poland and Hungary. The number of reimbursed BOMPs and biotechnological MPs

for RDs without prior orphan designation is the highest in Hungary (17 and 40, respectively) whereas the results in Bulgaria, Serbia, Slovakia and Poland are similar to each other: around 4 and 30, respectively. Patient-based reimbursement schemes are available only in Hungary for 11 out of 17 BOMPs. Total pharmaceutical expenditures, reimbursed by the local funds are the highest in Poland and Greece as their average values are approximately 214 million and 180 million euro, respectively for the observed period 2014-2016. The share of pharmaceutical expenditures on the reimbursed biotechnological medicinal products for RDs for the observed period 2014-2016 in Bulgaria (8%, 17% and 19%) and Slovakia (16.8%, 16.6% and 17.6%) accounts a significant percentage. An increasing trend is also revealed for Poland, Serbia and Macedonia. **Conclusions**: The non-European Union CEE countries have a significant delay in the legal implementation of a pharmacoeconomic guideline for assessment of BOMPs. The access to BOMPs is similar among the observed CEE countries as it is the highest in Hungary and Greece. The influence of the BOMPs expenditures on the budget in the individual countries is significant.

Key words: CEE countries, biotechnological orphan medicinal products, reimbursement, rare diseases