Does HTA Process in Central and Eastern Europe Countries Improve the Availability and Accessibility of Advanced Therapies: A 3-Year Analysis for Bulgaria, Romania, and Poland

HTA342

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INTRODUCTION

- ✓ The **significant therapeutic potential** of the advanced therapies (ATs) has predetermined the increased interests in their development mainly in the context of rare diseases (1);
- ✓ Usually, public payers and health care systems cover the expenses of the treatment with ATs; However, there are still many **challenges** in front of the health insurance funds related to the cost-effectiveness and budget impact issues of these therapies (2);
- ✓ The **high prices** of gene therapies are a cause for rejection from the HTA institutions and payers;
- ✓ Since the ATs are **orphan drugs** and are used in **personalized medicine**, **the small number of patients** to whom these therapies apply and the **manufacturer price** which ranges from US$ 18,950 for a tissue-engineered product to US$ 1,206,751 for a gene therapy, are some of the reasons for the **extremely high cost** of treatment with ATs (2);
- ✓ **Unmet needs-driven reimbursement** could be an opportunity for improved access to innovative therapies. The framed risks could be used as part of the negotiation process and as a tool for reaching affordable prices (3).

OBJECTIVE

1. T**o analyze health technology assessment** (HTA) **process** and its specificities concerning advanced therapy medicinal products (ATMPs);
2. To **review the progress** in their market access over a 3-year period in three Central and Eastern European (CEE) countries: *Bulgaria, Romania and Poland.*

METHOD

- A specifically designed questionnaire was created;
- The questionnaire contains a section on the prices and reimbursement (P&R) policies and on the HTA process related to advanced therapy medicinal products (ATMPs) and their availability and accessibility;
- The questionnaire was sent to HTA experts from the countries of interest – Bulgaria, Romania, and Poland;
- The responses were collected at two different points of time, 2021 and 2023.

RESULTS

1. NUMBER OF APPROVED ATMPS IN EUROPEAN UNION TO 2023

In total **23 ATMPs** have been granted a marketing authorization in EU of which:

- ✓ Cell based therapies – **15 out of 23;**
- ✓ Gene therapies – **8 out of 23.**

2. HTA PROCESS AND HTA REPORT REQUIREMENTS

- ✓ The **HTA process is obligatory** for ATMPs, but **no specific guidelines are available** in the three countries Bulgaria, Romania and Poland;
- ✓ The **duration of HTA** assessment and appraisal for ATMPs is the **same as for the other medicinal products** – from 180 days in Bulgaria to 90 days in Romania;
- ✓ None of the three countries have established a **specific willingness to pay threshold for ATMPs**;
- ✓ In Bulgaria **payer perspective** of the HTA analyses is a requirement and in addition **public perspective could** be taken into account;
- ✓ In Poland **payer perspective** and **joint perspective** (payer and public perspective) is needed for HTA analyses;
- ✓ In contrast, in Romania **public perspective is not part** of the HTA analyses;
- ✓ In all three countries, **cost-effectiveness** and **cost-utility analyses** are preferred in HTA process.

3. AVAILABILITY AND ACCESSIBILITY OF ATMPS OVER A 3-YEAR PERIOD (2021-2023)

- ✓ In Romania **three ATMPs**, one cell-based gene therapy and two gene therapies, **were reimbursed** each with specific requirements for prescribing and administration.
- ✓ In Bulgaria only **one application** of a gene therapy was **submitted**, and the process is still ongoing.
- ✓ In Poland **three cell-based** gene therapies and **one gene** therapy were **reimbursed (Figure 1)**.

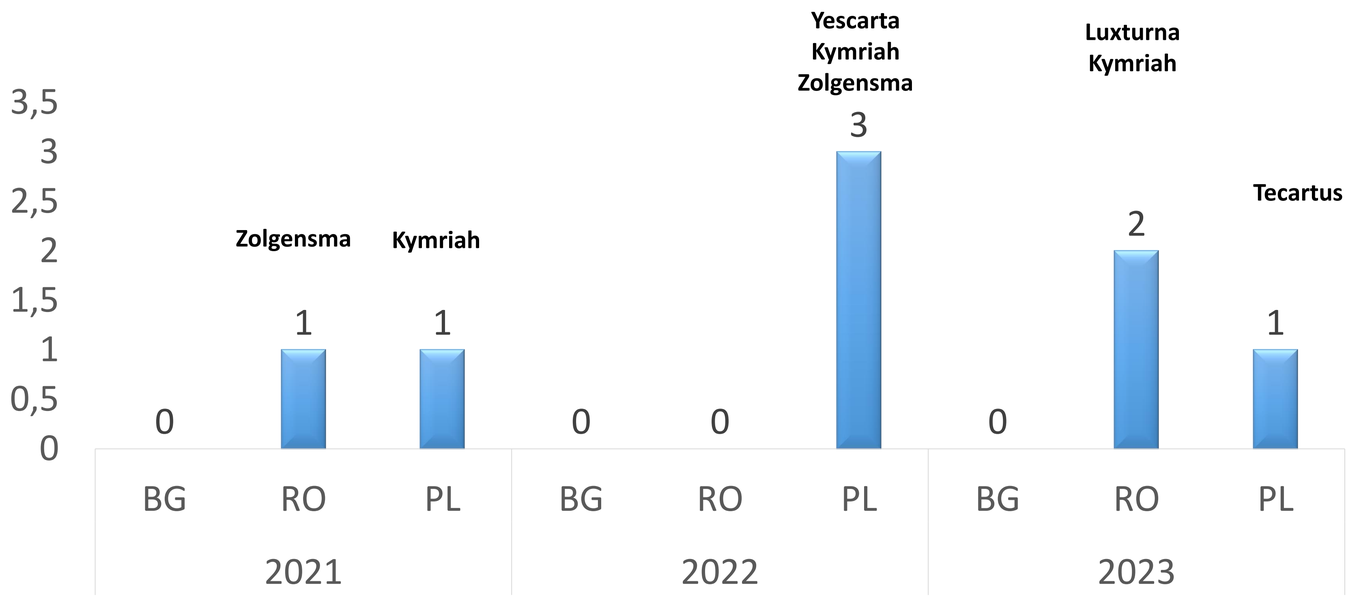

Figure 1. Number of ATMPs reimbursed in Bulgaria, Romania and Poland for a 3-year period (2021-2023)

CONCLUSIONS

- ✓ The main challenges such as **high treatment costs**, **uncertainty** in clinical **effectiveness**, and **inadequate HTA** methodological **approaches** for ATMPs, are also present in CEE countries;
- ✓ Reliable, nationally oriented **programs** for HTA and adequate **financial coverage** of ATMPs are **not yet implemented**;
- ✓ **No prioritization conditions** for the HTA of ATMPs have been identified in the selected countries;
- ✓ Over the 3-year period, **the HTA process** for ATMPs **has not changed significantly**;
- ✓ The number of ATMPs assessed and **available in Romania and Poland has increased**. However, **CEE countries lag behind Western European** countries in terms of the ATMPs availability for the patients.

REFERENCES

1.CARVALHO, Marta; SEPODES, Bruno; MARTINS, Ana Paula. Patient access to gene therapy medicinal products: a comprehensive review. BMJ Innovations, 2021, 7.1.

2.SEOANE-VAZQUEZ, Enrique; SHUKLA, Vaishali; RODRIGUEZ-MONGUIO, Rosa. Innovation and competition in advanced therapy medicinal products. EMBO molecular medicine, 2019, 11.3: e9992.

3.VREMAN, Rick A., et al. Unmet medical need: an introduction to definitions and stakeholder perceptions. Value in health, 2019, 22.11: 1275-1282.

4.KAMUSHEVA, Maria, et al. Do advanced therapies have a future in the low-and middle-income countries-The case of Bulgaria, Romania, and Poland. Frontiers in Public Health, 2021, 9: 729847.

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