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Opportunities and Challenges for Value Added Medicines: Results of a Scoping Research Based on Expert Interviews

Zsuzsanna Ida Petykó[*1,2], András Inotai[1,2], Jaime Espin[3], Derek T O'Keeffe[4], James F O'Mahony[5], Marcin Czech[6], Zoltán Kaló[1,2]

[1] Syreon Research Institute, Budapest, Hungary; [2] Semmelweis University, Center for healthcare Technology Assessment, Budapest, Hungary; [3] Andalusian School of Public Health, Granada, GR, Spain; [4] HIVE Lab, Lambe Institute, School of Medicine, National University of Ireland, Galway, Ireland; [5] Trinity College Dublin, Dublin, Ireland; [6] Institute of Mother and Child at Warsaw University of Technology, Warsaw MZ, Poland

INTRODUCTION

Value-added medicines (VAMs, also known as repurposed medicines) provide a wide range of important benefits to patients, healthcare professionals and healthcare systems by repositioning, reformulating or combining known molecules. Additionally, innovation of mature pharmaceuticals is more affordable, carries lower risks and allows for a faster time to market than de novo innovation. However, several barriers complicate the market access of VAMs. The evaluation criteria of new medicines applied by health technology assessment (HTA) bodies are limitedly adaptable for VAMs with a less abundant evidence base of their differential value. Consequently, their benefit is often not recognized by decision-makers [1,2].

Recently, an increasing number of ongoing and new EU-level initiatives, including Commission Expert Group on Safe and Timely Access to Medicines for Patients (STAMP) [3], Horizon Europe [4] and many others, recognize the importance of VAMs from a societal perspective. In addition, the COVID-19 pandemic highlighted the need to change the European policy environment to be more receptive to VAMs [5-7].

A core evaluation framework has been developed to support the value assessment of VAMs [8,9], containing 11 value domains in 5 main clusters, including unmet medical need, health gain (measured by healthcare professionals), patient-reported outcomes, burden on households, and burden on the healthcare system.

Encouragingly, there are sporadic cases in certain countries where the benefit of VAMs is already acknowledged or VAMs are differentiated from other off-patent pharmaceuticals. Moreover, specific national policy frameworks in other areas with potentially relevant elements for VAMs may be starting points for creating a more favourable policy environment in each country. Still, academic researchers, policymakers, payers and industry representatives may have divergent opinions on the current barriers and realistic opportunities for integrating VAMs into European healthcare systems in the near future.

This scoping research aims to identify the opportunities, challenges and relevant value elements related to the adaptation of VAMs in Ireland, Spain and Poland.

METHODS

To identify the main market access barriers for VAMs in the target countries, structured, in-depth interviews were conducted with health policy experts and market access managers. The interviews covered three main topics: 1) the legal and policy environment for differentiating VAMs, 2) the current and future relevance of value domains [8,9], and 3) the barriers to VAM adaptation and potential policy solutions. Barriers were grouped in major policy areas as the following: regulatory, national HTA, institutional HTA, price setting, reimbursement, national formulary listing, institutional formulary listing, national-level purchasing, institutional-level purchasing and patient-level purchasing.

A standardized interview guide was consistently used during the interviews both with health policy experts and market access managers, to identify the relevance of value domains and country-level barriers. All interviews were carried out in a virtual video conference format. Country reports were drafted based on the inputs of interviewees and were developed in an iterative process as shown in Figure 1.

RESULTS

The report is based on 13 in-depth interviews conducted with 5 health policy experts (2-2 from Ireland and Spain and 1 from Poland) (5x2 interviews) and 6 market access managers (2-2 from each participating country) (3 interviews).

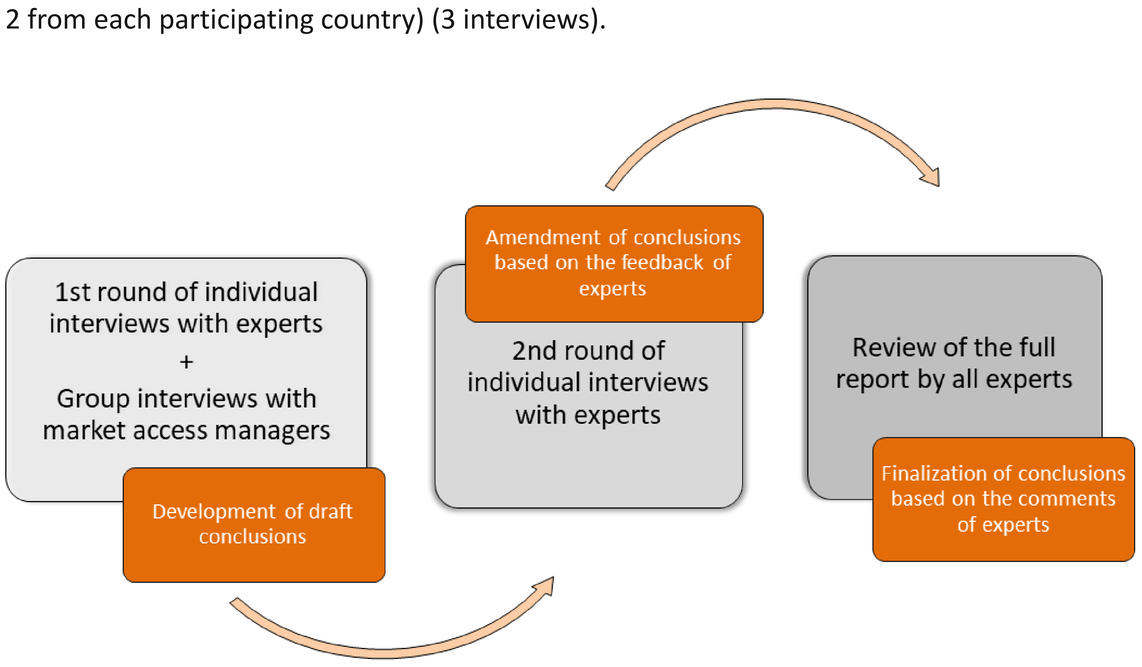

Figure 1 - Iterative process of recommendation development

High level conclusions

Large heterogeneity in how different stakeholders within each country perceived different value elements prevented the research team from forming conclusions on which value domains could be accepted in each country. However, patient experience (PEx) and the financial burden of households are limitedly considered or not taken into account at all in the value judgement process of new pharmaceuticals. Sporadic reference cases (when VAMs are already differentiated from other off-patent medicines) and specific policy frameworks (with potentially relevant elements for VAMs) could be the starting point for national policy frameworks for VAMs in all three countries.

Repositioning of off-patent pharmaceuticals to new indications with high unmet need is perceived desirable in all three countries. However, practice is limited regarding the differentiation from generic medicines in clinical guidelines, special reimbursement status or exclusion from pharmacy substitution, all of which are disincentives for investing in this type of innovation. On the other hand, reformulated or combined off-patent medicines may not be subject to pharmacy-level substitution and their differentiation in clinical guidelines is possible.

Proposed action points

Despite some similar high-level conclusions, policy actions should remain specific to each country. VAM innovators (including public and private financial investors, pharmaceutical companies, start-ups, universities or hospitals) at the national level should:

- study and collect existing reference cases and policy frameworks to facilitate a specific national framework and incentive system for VAMs
- identify potential partners who may be interested in investing in the R&D of VAMs or the integration of VAMs into the healthcare system (e.g., Ministry of Industrial Affairs, National Research and Development Agency, HTA office, Drug Regulatory Agency, medical universities and other academic centers, hospital pharmacists and many more)
- build a national communication strategy - including clarifying or establishing the scientific and legal definitions of VAMs - to reach key stakeholders
- build national consensus around a scientific manuscript or policy white paper
- quantify and present the unmet medical need for incremental innovation (especially in rare diseases, pediatric diseases and oncology)
- explore good practices of relying on real-world evidence and invest into improving the credibility and pragmaticism of real-world data collection
- promote the acceptance of PEx and the burden of households as potential benefits considered in value assessment (in association with patient organisations and other industry partners)
- work with hospital associations to explore legal opportunities and incentives on how to focus on the added value instead of purchasing the lowest price medicines

Limitations

* Since the scoping research relied on interviews as the main sources of information, only the reference documents and relevant websites recommended by interview participants were studied in detail.
* The small number of expert viewpoints (3-4 per country) is not representative at a country-level; however, the in-depth interviews conducted with eminent health policy experts provided an opportunity for strong interaction and a thorough understanding of the details.
* It was challenging to consolidate the heterogeneous viewpoints of different stakeholders (and even experts of the same stakeholder group) within each country.

CONCLUSION

Despite of the multiple, ongoing international initiatives in the value-added medicine and drug repurposing arena, the heterogeneity of countries in various aspects neccecitates that the content of policy documents and other policy actions should remain specific to each country.

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*Presented by **Zsuzsanna Petykó MD MSc** *correspondence: zsuzsanna.petyko@syreon.eu*

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