



# A dedicated evaluation framework for value added medicines

Value added medicines (VAM) are medicines that are developed based on existing therapies, aiming to bring additional benefit to patients, carers, or the healthcare system. However, for these medicines to deliver on their potential, a fit-for-purpose, more patient-centric approach to assessing their value needs to be developed.



## VALUE DOMAINS FOR VALUE ADDED MEDICINES

A **core evaluation framework**<sup>1</sup>, which can serve as a basis for a dedicated approach to assess these medicines, was developed by Petykó and colleagues. The process started with a systematic literature review identifying the various healthcare benefits these medicines bring and was further strengthened through an iterative consultation with policy makers and health economists from different EU countries.

This resulted in 11 value domains grouped into 5 clusters (Fig 1). While unmet medical need or gains in efficacy and safety form an essential part of current evaluation frameworks, other value clusters, such as patient reported outcomes, the burden on the healthcare system or the household, play a reduced role or are not recognised in the existing evaluation, indicating a gap in the current system.

In addressing public health need, any approach to value evaluations needs to encompass not only the traditional domains, but also patient-centered ones, such as quality of life, adherence or acceptability and ease of treatment. Moreover, from a societal perspective the burden on households or the healthcare system plays an important role in the economic impact of disease, therefore integrating this in the evaluation process is essential to develop a holistic approach.

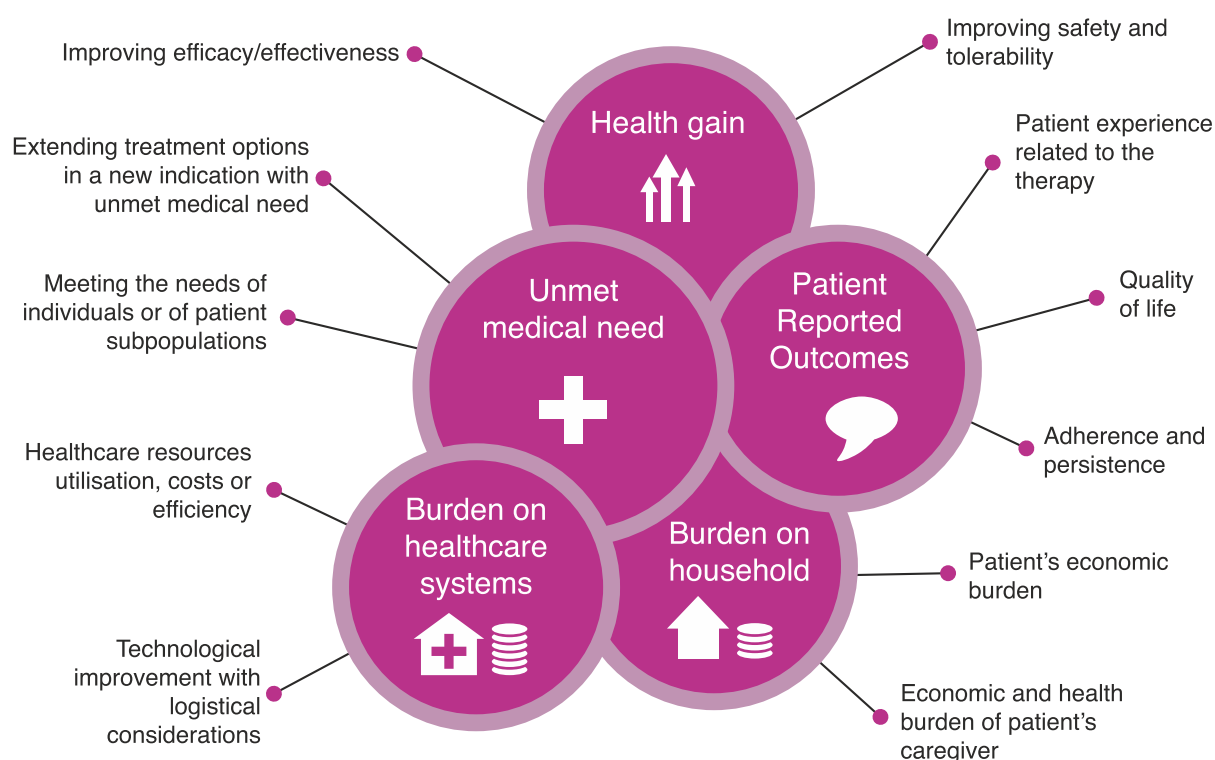


Figure 1 – The 11 value domains and 5 value clusters identified for value-added medicines

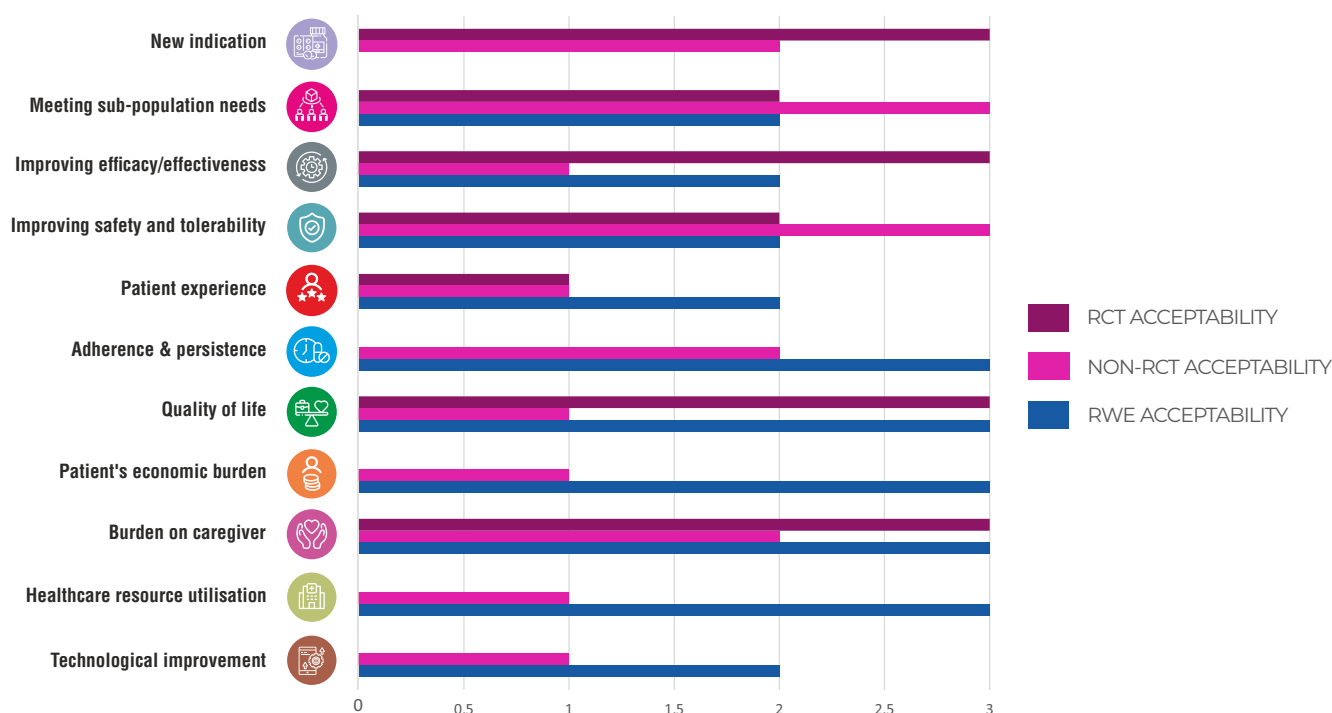


## SOURCES OF EVIDENCE FOR THE ASSESSMENT OF VALUE ADDED MEDICINES

Building on the framework, the research also assessed the perceived **acceptability and complexity of evidence<sup>2</sup>** sources for each value cluster. While there are areas where randomised clinical trials (RCTs) would be the most acceptable tool (such as unmet medical need or health gain), the research also highlighted that certain benefits, such as improved adherence, reduced resource utilization and treatment costs, are better measured through observational studies, patient registries or real-world evidence (RWE).

Value added medicines are built on the existing body of evidence, and data is generated to fill in the missing gaps. This approach can result in reduced development time and costs, resulting in more affordable innovation. However, this approach implicitly leads to a very different dataset than in the traditional new medicines development. Therefore, it is even more important that value evidence requirements and evaluation should be streamlined in an early scientific and payer dialogue. Alternative, complementary sources of data should be considered and balanced against the claimed benefit and the final cost of innovation in play.

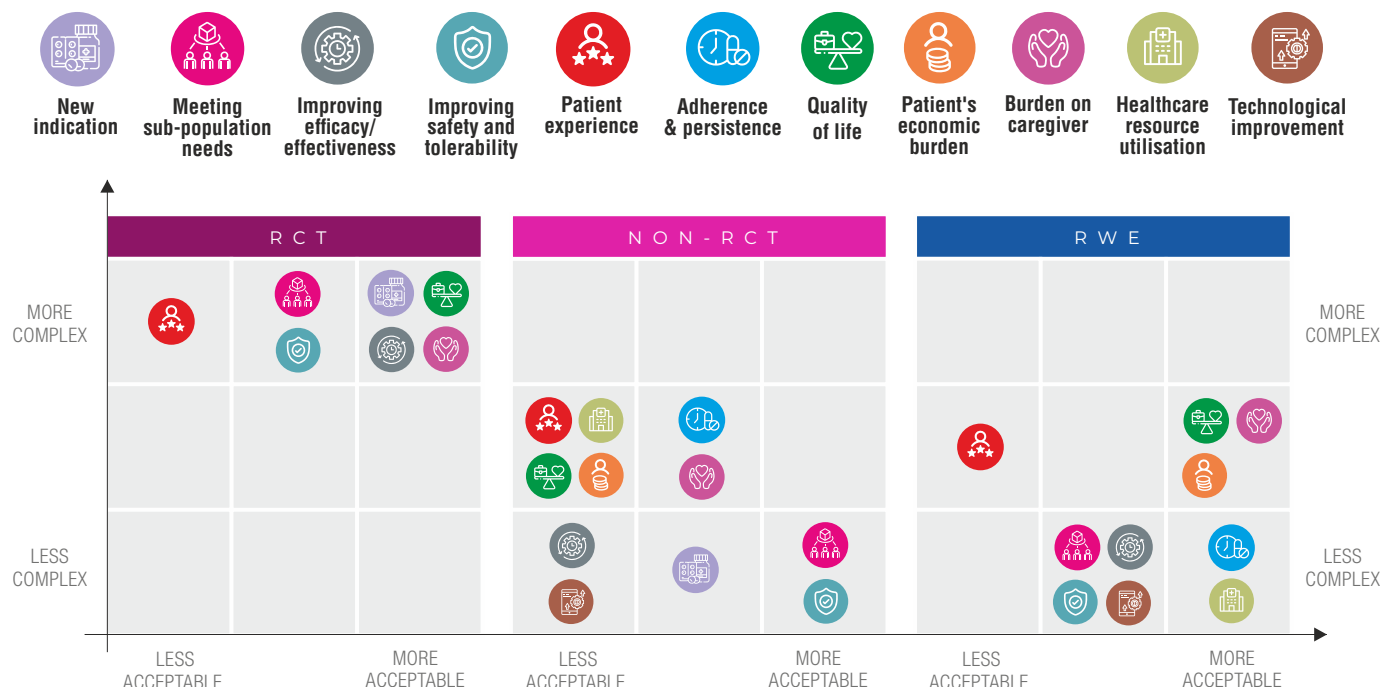
Where additional data is needed, the cost and complexity for generating it should be considered (see Figure 3). If acceptability is similar, (e.g. for Quality of life), it would be desirable that the least complex and costly evidence type is employed, as that would free up resources and incentivise investment in additional projects.



**Figure 2 – RCT data supports assessments for traditional value domains, while RWE is crucial for the evaluation of novel value domains.**



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**Figure 3 – Where acceptability is similar, using the least complex evidence source would be desirable.**

## RECOMMENDATIONS AND FUTURE STEPS

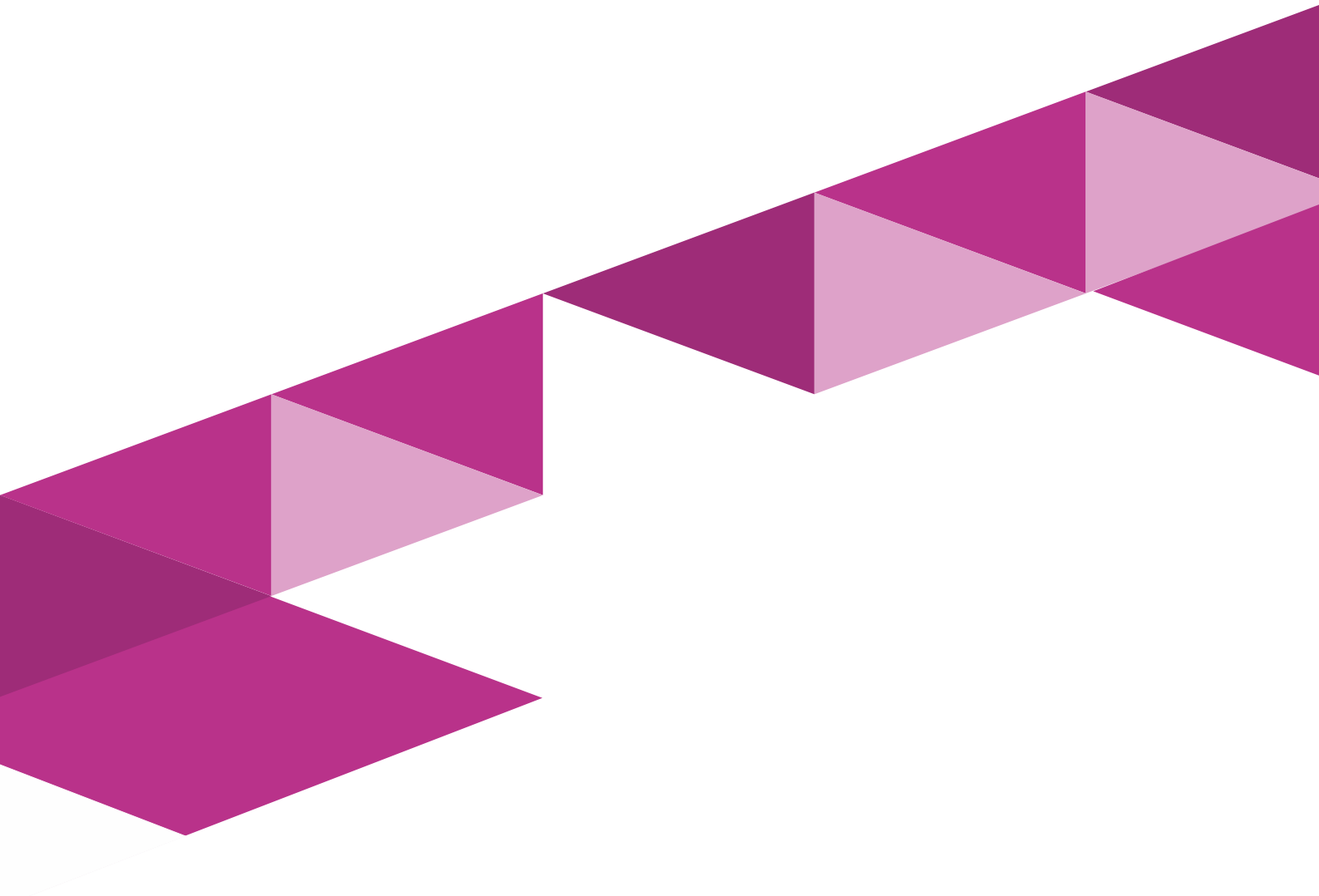
To support the development of national dedicated frameworks for value added medicines, in line with practices employed for other particular types of health technologies (e.g. digital technologies), this core evaluation framework can be used as a basis.

National frameworks should be developed and validated in consultation with a wide range of stakeholders, including governments, payers, healthcare providers, patients and the pharmaceutical industry. Governments should also consider the suitability of various evidence sources, with good practices particularly for the collection of RWE explored. Where appropriate, post-approval evidence generation should be considered in a pragmatic manner, according to the type of VAM and the claimed benefit.

Overall, as these reports show, for value added medicines to deliver on their potential, regulators should take a holistic approach in assessing their benefit.

1.Petykó, Z.I., Kaló, Z., Espin, J. et al. Development of a core evaluation framework of value-added medicines: report 1 on methodology and findings. Cost Eff Resour Alloc 19, 57 (2021). <https://doi.org/10.1186/s12962-021-00311-6>

2.Kaló, Z., Petykó, Z.I., Fricke, FU. et al. Development of a core evaluation framework of value-added medicines: report 2 on pharmaceutical policy perspectives. Cost Eff Resour Alloc 19, 42 (2021). <https://doi.org/10.1186/s12962-021-00296-2>



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