Manifesto
for a new approach for better medicine in Europe
Establishing Treatment Optimization
as part of personalized medicine development
(version 28 May 2019)

• Forewords

Personalized medicine refers to a medical model that tailors the therapy to the patient’s molecular profile and other individual information. The principles apply to medicines as well as other treatment modalities, including surgery and radiotherapy. The concept though has specifically emerged due to the increased number of drugs targeting specific proteins responsible for a specific disease. The commercial promotion of genome-wide analyses has led to an increasing expectation among patients.

On the other hand, there are numerous drugs authorized on the market, with limited knowledge on how to use them for dose, sequence, combination and duration of treatment. Sub-optimal administration of costly treatments may generate unnecessary toxicity for the patients and negatively affects national healthcare budgets. Thus, there is a need for investigating the optimal way to use medicines (applied research or “Treatment Optimization”)¹.

In Europe, most of the clinical research dedicated to therapeutic innovations aims primarily at regulatory approval. Once a drug enters the common market, each member state determines its real-world use based on its own criteria: pricing, reimbursement and clinical indications.

Such a regulatory approval-centred clinical research landscape may neglect patient-relevant issues in real-world setting, such as comparative effectiveness of distinct treatment options or long-term safety monitoring.

There is call for reforming the current system to a truly ‘patient-centred’ paradigm with systematically coordinated Treatment Optimisation in conjunction with drug development.² The purpose of this manifesto is to gain stakeholders support for making Treatment Optimization a standard step in medicine development in Europe.

This manifesto was prepared by the European Organisation for Research and Treatment of Cancer (EORTC).

• Direction for changes

1. Appropriate use of treatments is essential for optimizing patients’ health outcomes and preserving public health and healthcare budgets. Knowing the optimal dosage and duration of a treatment, used alone or in combination, is key for using it efficiently for the benefit of the patient and the society.

2. Treatment Optimization has to take place early. Treatment Optimization generating evidence for HTA, payers and clinicians should be initiated before the medicine’s full deployment on the market i.e. as soon as the safety and efficacy profiles are known but without delaying patients’ access to innovative treatments.

3. Treatment Optimization must cover all treatment options and modalities available for a given medical condition and should consider specific therapeutic needs of vulnerable subgroups such as infants and children. Research investigating the optimal way to use a treatment should comply with high scientific standards and be free of commercial consideration.

4. Member states and payers should support Treatment Optimization at a relevant scale. Research addressing medical practice oriented questions must be funded by public sources. Member states should pool their resources for supporting Treatment Optimization when doing so on an international scale is justified.

5. Treatment Optimization will benefit from new models of partnership. Pooling expertise and resources from clinicians, patients, HTA and industry is essential for conducting sound Treatment Optimization research.

6. Treatment Optimization will support industry competiveness and reputation. Optimised usage will improve medicines’ impact on health and justify fair price.

• Policy actions

- Europe in partnership with Member States should establish Treatment Optimization research as an official and mandatory step in the treatment access path to market, while ensuring this does not lead to further delays in patients’ access to innovative treatments.
- National legislation should include provisions allowing for publicly funded international research to address collective therapeutic challenges. Member States should agree on a framework for joint optimization research whenever there is need for an international approach.
- The EU’s next mission-oriented framework programme for research and innovation, Horizon Europe, should provide funding opportunities specifically aimed at supporting Treatment Optimization.
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