Implementing Combination Treatment Cost-Effectiveness Solutions Beyond the Legal Challenges - What Else Needs to be in Place?

The overarching challenge: Demonstrating value

In 2014, the Decision Support Unit (DSU) for the UK National Institute of Health and Care Excellence (NICE) published a working paper that outlined the circumstances in which health technologies that are demonstrated to be effective may nevertheless be deemed not cost-effective even at a zero price. Since the overall cost of the treatment combination includes all components, the add-on component often faces cost-effectiveness barriers.

Objective

The aim is to develop a framework that could lead to the successful implementation of a combination treatment solution that aligns with current NICE appraisal, and NHSE commercial methods and doesn't overstep the bounds of competition law. With collaboration and communication at its heart, the Framework aims to outline the critical success factors, relevant policy constructs and enabling platforms that could support a successful launch and provide insight into future-proofing the solution to reflect policy changes.

What is a combination treatment?

A combination treatment combines two or more individual component treatments to treat a single disease.

A "backbone"

A treatment or treatment combination that is already available to patients.

Now

- The "backbone" treatment's market share and use in clinical practice is well-established before it is combined with another treatment and is often the existing standard of care for a given disease.

One or more"add-on" treatments

A treatment or treatment combination, that is added to the existing backbone treatment

Why we need combination treatments?

- Using multiple treatments in combination can simultaneously target numerous pathways that drive a complex disease to improve patient survival and quality of life.

How is a combination treatment evaluated?

In cost-effectiveness markets like the UK, combination treatments are usually currently appraised, via cost-effectiveness analysis (CEA) methods, as an entire treatment, therefore all costs for the backbone are included in the appraisal of the add-on.

Tackling the value challenge: A two-part approach

Value attribution methods are being explored to attribute value across all components of a combination and also different stakeholders are looking at ways in which manufacturers may be able to interact given the bounds of competition law.

Implementing the approach

As with the Attribution of Value and Arbitration Frameworks development, Takeda UK has taken a multi-stakeholder collaborative approach to design this Framework to ensure its core elements are implementable and transactable.

The Framework takes a whole systems approach:

Earliest point where a potential combination cost-effectiveness issue could be flagged to allow maximum possible resolution time to data collection within the health system.

The critical points in the system where the combination medicines' cost-effectiveness issues and the proposed solutions could and should be flagged.

Horizon Scanning and Early Advice (UK PharmaScan, NIHR Innovation Observatory, The Innovative Licensing and Access Pathway)	All stakeholders, especially clinician/HCP professional groups and patient organisations, to implement horizon scanning programmes as part of their strategic plans.
The Medicines and Healthcare products Regulatory Agency (MHRA) (Project Orbis)	**Raise the issue through multiple touchpoints:** The Early Access to Medicine Scheme (EAMS) can be utilised by the MHRA to give a scientific opinion on the benefit/risk balance of the medicine based on the data available when the EAMS submission is made. **Two-step evaluation process for scientific opinion:** 1. Promising Innovative Medicine (PIM) designation. A PIM designation indicates a product may be eligible for the EAMS based on early clinical data. The PIM designation will be issued after an MHRA scientific meeting and could be given several years before the product is licensed. 2. Early Access to Medicines Scientific Opinion. The scientific opinion describes the risks and benefits of the medicine based on data gathered from the patients who will benefit from the medicine. The opinion supports the prescriber and patient to decide on whether to use the medicine before its licence is approved.
NICE & NHSE	**Raise and discuss issues within NICE and NHSE:** This relies on early engagement and technology processes including: NICE Office or Market Access, Nice Early Scientific Advice, NICE Decision Support Unit, Technology Appraisal Processes, Methods and Guidance, NHSE Commercial and Pipeline Surgeries and NHSE Commercial Framework.

Steps to Implementation:

- Pilot study
- NICE/NHSE webinar briefing
- VPAS
- Pilot Study
- NICE/NHSE communication to system partners

Critical success factors

1. Willingness to trial available solutions

Stakeholders and decision makers, including NICE Appraisal Committees, EAGs and manufacturers need to be aligned and willing to implement solutions while remaining flexible to accommodate any required adaptations and providing constructive feedback.

2. A commitment to review and give feedback on learnings on solution methods

Develop a learning environment through constructive feedback and case studies to help companies to use the solution optimally.

3. Robust data collection

Having a robust data collection platform is critical if companies are to have faith in potential solutions being implemented in the health system.

4. Non uniform pricing

Where combination treatments are introduced, non-uniform pricing will be required to ensure that in competitive markets no advantage or disadvantage occurs with the introduction of a treatment as a component of a combination that is available at any other line of treatment or in any other indication. This will require the system to be open to flexible pricing options.

5. Support from patient advocates, patient organisations and clinicians

Support will be needed from all stakeholders including patient advocates, organisations and clinicians. To highlight the value of the combination, leveraging their voices on why the system should come together and explore these methods.

6. Enabling platforms

The following enabling platforms will be leveraged to play a key part in the successful implementation by identifying potential gaps and system trip hazards: VPAS, CMA guideline, Data Platform

Conclusion

The successful implementation of this framework hinges on the involvement and collaboration of every key stakeholder in order to collectively optimise the process and develop a solution that overcomes the key challenges of the combination treatment landscape to benefit patients now and in the future.

Authors:
Helen Taylor, Dominic Cameron, Eric Low

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Helen Taylor[1], Eric Low[2], Dominic Cameron (1. Takeda UK Ltd. London, UK | 2. Eric Low Consulting. Edinburgh, UK)

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