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Science At PACE? A multi-stakeholder developed framework for Accelerated Patient Access to Cancer Care

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Background

The European Medicines Agency (EMA) has introduced accelerated pathways to enable regulatory approval to promising therapies where there is a high unmet need.

On the other hand, Health Technology Assessment (HTA) and pricing and reimbursement (P&R) processes have **not evolved consistently** in response.

Some countries have established dedicated pathways, which have led to **further fragmentation** at the European level.

As a result, patients often **do not receive timely access** to these treatments, with **significant inconsistencies persistent** across countries.

Aim

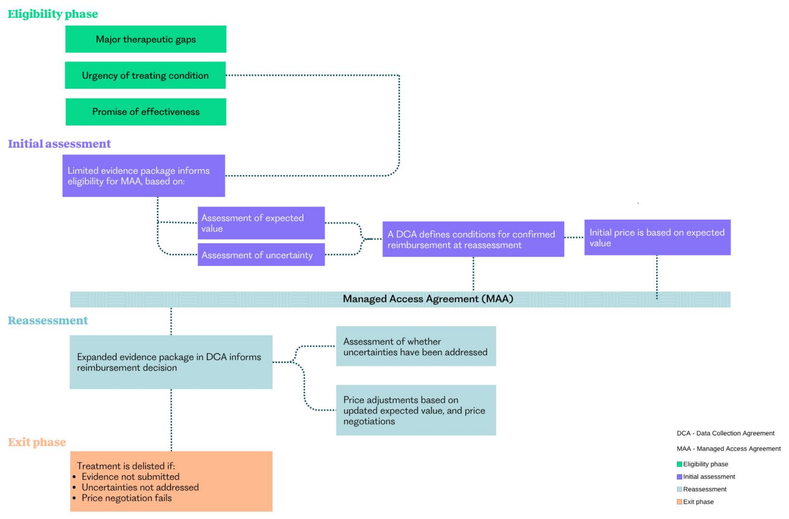
To develop a pan-European, stakeholder-endorsed framework for enabling accelerated access to promising oncology treatments (from hereon called eligible treatments), outlining key principles from regulatory approval through HTA to reimbursement.

Methods

A structured, multidisciplinary stakeholder engagement process was conducted to develop an Accelerated Patient Access to Cancer Care in Europe (APACE) framework, including two surveys and two roundtables with patient, regulatory, HTA, payer and industry representatives from Belgium, Italy, Norway, Spain, Sweden and the United Kingdom.

Results

FIGURE 1: A FRAMEWORK FOR APACE



Eligibility Phase

To assess whether a treatment meets the eligibility criteria to enter the process.

Key Principles

- There was strong agreement with the suggested eligibility criteria:
  - ➢ **Major therapeutic gaps:** ensuring only a limited number of treatments with specific characteristics enter
  - ➢ **Urgency of the condition:** ensuring early access is granted for severe conditions
  - ➢ **Promise of effectiveness:** ensuring treatment has potential to generate meaningful improvements in outcomes recognised by HTA

Points For Further Consideration:

- Participants recognised difficulties in setting quantitative eligibility criteria due to lack of clinical data inherent to eligible treatments. Suggested that qualitative deliberations are also required and should be transparent and accepted — e.g. using case histories.

Initial Assessment

To evaluate whether a treatment should enter a managed access agreement (MAA), and the conditions under which it should do so. This involves an assessment of the treatment expected value and of uncertainties that remain to be addressed.

Key Principles:

- **Limited evidence package** accepted due to difficulties generating RCT data.
- **Data collection agreement (DCA)** defined, based on explicit discussions about what **uncertainties** are and how to resolve them.
- Pricing aligned with payer's assessment of eligible treatments' **expected value**.

Points For Further Consideration :

- Some suggested initial assessment should have faster **timelines** than traditional assessments, but others noted large uncertainties require a thorough assessment.
- Some suggested that **Joint Clinical Assessment (JCA)** offers scope for faster timelines, but others questioned whether the JCA could meaningfully impact timelines in countries where additional evidence is required.

Reassessment

To evaluate whether the uncertainties set out in the DCA are addressed to a satisfactory level and new evidence changes the assessment of expected value.

Key principles

- **Expanded evidence package**, with Real World Evidence (RWE) when appropriate.
- Flexibility in the **reassessment point** but at a maximum of 5 years, to be agreed on a treatment-by-treatment basis as part of DCA.
- **Pricing** may be adjusted upwards or downwards in line with changes in expected value — upwards adjustments can incentivise evidence collection by the developer; downwards adjustments occur with rebates to ensure payers do not pay more for the eligible treatment than effectiveness suggests.

Points For Further Consideration:

- No consensus regarding the use of **non-OS outcomes**.
- Some felt dynamic pricing was not feasible, preferring a fixed pricing discounts approach due to its simplicity for payers.

Exit Phase

To detail the process to follow when an eligible treatment is delisted.

Key Principles:

The exit process achieved broad agreement across participants:

- If reimbursement is confirmed and price negotiated, eligible treatment exits the process and **enters the traditional reimbursement** pathway
- The eligible treatment **may be delisted** in cases where evidence is not submitted, fails to address uncertainties or price negotiation fails.

Points For Further Consideration:

- Concerns were raised over the **ability to enforce delisting**.

Discussion and Conclusion

- The APACE framework seeks to bridge the gap between regulatory approval and reimbursement by enabling conditional access alongside evidence development.
- There was broad stakeholder agreement with the proposed framework.
- However, some areas remain unsolved and require further dialogue including:
  - Definition of eligibility criteria
  - Acceptability of non-OS outcomes at the assessment phases
  - Support and implementation of dynamic P&R models
- To realise its potential, further policy work is needed to resolve outstanding issues and ensure consistent implementation across European Countries.

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