The Cancer Medicines Forum
An Industry Perspective

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Why Treatment Optimisation Matters (Insights)

- Optimal dose, duration, sequencing often unknown at approval.
- Registration trials do not reflect real-world clinical complexity.
- Optimisation requires clinical practice experience, patient, payer, system-level perspectives.
- Pragmatic and adaptive trials needed to evaluate real-world risk-benefit.
- Clear communication needed when dose modification is required.

Establishment & Purpose (Kick-off, March 2022)

- CMF launched by EMA + EORTC to address treatment optimisation gaps.
- Non-advisory, multi-stakeholder forum including academia, HTA, payers, and patient reps.
- Recognised uncertainties at authorisation: dose, duration, sequencing, target group.
- Goal: identify priority optimisation questions and enable collaborative research frameworks.
- Commitment to shared outputs and transparent evidence-driven recommendations. Initial focus on post-authorisation research challenges and feasibility.
- Drafted framework for when and how to run optimisation trials.
- Case studies illustrated benefits of de-escalation for toxicity and affordability.
- HTA bodies prefer RCT-based evidence over RWD comparisons.
- Need for sustained funding models for optimisation research.

Consolidation of Vision (Dec 2023)

- Agreed shared terminology and prioritisation logic.
- Optimisation positioned to begin during scientific advice pre-authorisation.
- Health systems expected to share responsibility for funding post-authorisation trials.
- April 2024 workshop planned to broaden engagement.
- Shift from analysing gaps → preparing implementation pathways.

Workshop and Prioritisation Exercise (2024,25)

- April 2024 workshop confirmed embedding optimisation across lifecycle.
- EMA integrating optimisation into scientific advice and post-authorisation planning.
- Focus shifting toward prioritisation and implementation structure.
- Proposed criteria for optimisation research question selection.
- Quarterly meetings continue to drive structured progress.
- Confirmed two goals: identify optimisation priorities and facilitate delivery.
- Observers redefined as advisors, increasing active contributions.
- Endorsed prioritisation pathway including public consultation.
- Acknowledged importance of multinational trial infrastructure and funding.
- Pilot prioritisation cycle planned for evaluation and refinement.

Potential Future Directions

- **Optimisation becoming part of the "expected" lifecycle — even pre-approval.**
  - Embed optimisation suggestions systematically into EMA scientific advice, HTA parallel consultation, and post-authorisation plans.
  - A prioritised pipeline of optimisation questions—open to consultation.
- **Greater use of pragmatic and post-licensing trials, with risk-proportionate approaches.**
  - Develop coordinated platforms for pragmatic, multinational optimisation trials
  - Create sustainable funding mechanisms involving payers, public agencies, and academic trial networks.
- **Earlier and deeper payer/HTA involvement to enable adoption and funding.**
- **Convergence with broader EU regulatory science and industrial-policy currents.**
  - Strengthen data interoperability and reporting standards to support real-world uptake and regulatory applicability.

Our View

- CMF is moving from **concept** to **execution**:
  - prioritised topics,
  - pragmatic EU trials,
  - payer-ready evidence,
  - earlier integration of optimisation into development.
- For industry, this is an opportunity to **de-risk labels**, **improve patient experience**, and **stabilise access** by aligning efficacy, safety, and affordability in the same evidence package.