# Minutes – Cancer Medicines Forum

June 17, 2025, 2:00pm – 5:00pm CET; Teams Meeting

Chairperson: Francesco Pignatti (European Medicines Agency, EMA)

Co-chairperson: Denis Lacombe (European Organisation for Research and Treatment of Cancer, EORTC)

Scientific coordinator: Caroline Voltz-Girolt (EMA)

Cancer Medicines Forum members: European Organisation for Research and Treatment of Cancer (EORTC), European Society of Medical Oncology (ESMO), European Haematology Association (EHA) and International Society of Geriatric Oncology (SIOG)

Observers: Organisation for Economic Co-operation and Development (OECD), HTA body (Institut für Qualität und Wirtschaftlichkeit im Gesundheitswesen, IQWiG), patient representative (Patvocates), industry representative, European Society of Paediatric Oncology (SIOPE), International Association of Mutual Benefit Societies (AIM) and European Social Insurance Platform (ESIP)

Guests: Zorginstituut Nederland, The Netherlands; Clinical Trials Coordination Group, EU

**Welcome and adoption of the minutes of the previous meeting**

The Chairs welcomed the members, observers and the guests. The minutes from the previous meeting were adopted without comments.

**Collection/analysis of safety data - information needed for optimisation/clinical decision**

The EMA provided an overview on the concern that the safety data collected through the Common Terminology Criteria for Adverse Events (CTCAE) reporting system may not sufficiently capture the information required to support clinical decision-making. This is particularly relevant for new classes of medicines, which often exhibit toxicity profiles that differ significantly from those of classical agents. Furthermore, trial populations often differ from patients encountered in routine clinical practice, underscoring the need to balance safety data collected during trials with information obtained post-authorisation.

To address these concerns, safety data collection is a point of attention of the ongoing revision 7 of the EMA guidelines on anticancer medicines. Specifically on cardiovascular safety follow-up, guidance is being developed jointly by the EMA's Cardiovascular and Oncology Working Parties.

**Report of the immunotherapy adverse event working group**

The EMA provided an overview of the work developed by the Cancer Immunotherapy Adverse Events Working Group, composed of international clinicians, researchers, methodologists, and biostatisticians, outlining the current challenges in the reporting and analysis of adverse events (AEs) in clinical trials.

While immunotherapies have transformed oncology treatment paradigms, their toxicities are particularly complex. Clinical trial data on these AEs represent a valuable source of information that can inform both pre- and post-marketing settings. Nonetheless, challenges remain, particularly regarding the reporting and causality attribution of AEs, with unexpected toxicities potentially being overlooked. Moreover, the tools currently available to describe and quantify AEs are limited, as crude incidence rates often lack temporal context.

Addressing these challenges was recognised as essential. Potential solutions include improving access to comprehensive and granular AE data, with the establishment of a unified repository for AE data representing a potentially meaningful advancement in this field. During the discussion, stakeholders reinforced that centralised repositories, supporting agnostic analytical approaches, could enable more robust detection of unexpected safety signals and a better understanding of potential causal relationships between treatments and AEs.

Furthermore, the relevance of treatment optimisation in the context of safety data generation was discussed. Given the increasing number of available treatment options, comparative assessments were highlighted as essential to support clinical decision-making, and randomised studies were emphasised as the most robust method to generate such evidence.

**Update on the study determining the optimal treatment duration of an antibody-drug conjugate in patients with previously untreated advanced urothelial cancer**

The EORTC provided an update on the treatment optimisation study in bladder cancer, which aims to determine the optimal treatment duration of an antibody-drug conjugate in patients with previously untreated advanced urothelial cancer. To support discussions with potential funders, a return-on-investment analysis has been conducted using a tool designed to demonstrate the potential financial benefits of this de-escalation study. Currently, funding discussions are ongoing with different EU healthcare systems, with the aim of launching this study as a pilot. This model would involve financial contributions from healthcare systems toward trial execution, with the anticipated return including access to the study results and dataset.

During the discussion, stakeholders raised the possibility of applying risk-proportional approaches, with proportionality in trial design and execution, aligned with the trial's objectives, presented as an opportunity for optimisation.

**STARTBUST program: Challenges in Defining Clinical Complete Response to Systemic Therapy in Muscle-invasive Bladder Cancer: Insights from the EORTC STARBURST Project**

The EORTC provided an overview of STARBURST, an initiative aiming to develop strategies for treatment adaptation in muscle-invasive bladder cancer.

The STARBURST platform seeks to refine methods to assess treatment response prior to radical intervention. The project will explore the integration of several aspects, including clinical and imaging data, to better identify patients achieving a complete response, focusing on developing robust predictive signatures and evaluating new tools to optimise response assessment. The aim is to adapt treatment strategies, offering active surveillance to those with no residual disease while escalating therapy in patients who do not respond adequately to neoadjuvant therapy.

While this approach offers the potential to spare patients unnecessary radical treatment, it also challenges traditional clinical trial endpoints, namely those assuming bladder removal. During the discussion, it was highlighted that early engagement with regulators and developers on acceptable endpoints in trials pursuing bladder preservation will be essential.

**Upcoming CMF workshop – 14 November 2025**

The EMA provided an overview of the draft proposal for the upcoming CMF workshop, scheduled for 14 November 2025.

The first session will explore how to facilitate academia-led optimisation trials in the EU, with an emphasis on study design and conduct considerations that reflect real-world clinical practice, clarifying stakeholder roles, regulatory requirements, and promoting collaborative approaches. The aim would be to have a dedicated closed session with the Clinical Trial Coordination Group and the CMF to find practical and constructive solutions, with a particular focus on oncology.

The second session, which will be a public workshop, will examine optimisation strategies in drug development, specifically aiming to balance innovation and optimisation, identify best practices for incorporating optimisation into development programmes, and foster an environment between academia and industry that encourages such approaches.

During the discussion, stakeholders highlighted that applying risk proportionality in regulatory requirements for such trials is of utmost importance. The discussion could help reach a common understanding on how to adapt trial requirements proportionally, enabling more pragmatic clinical trial designs and conduct.

**Update on the new mandate for the CMF**

The EMA provided an update on the draft mandate for the CMF, developed under the Healthcare Professionals Working Party (HCPWP) and the Patients and Consumers Working Party (PCWP). The draft mandate has been prepared and will be discussed at the upcoming meetings of the HCPWP and PCWP. It will then be circulated to member organisations and observers for review and feedback.

**CMF 2024 workshop: report and publication update**

The EORTC provided an update on the manuscript reporting on the CMF Workshop 2024. The final draft is currently under review and will be submitted to the *Journal of Cancer Policy* thereafter.

**Next CMF meetings**

The CMF will meet on a quarterly basis and below an overview of the remaining dates for 2025:

- 4 September at 2pm CET
- 2 December at 2pm CET