Minutes – Cancer Medicines Forum

March 7, 2023, 14:00 pm – 17:00 pm CET; WebEx

Chairperson: Denis Lacombe (European Organisation for Research and Treatment of Cancer, EORTC)

Co-chairperson: Francesco Pignatti (European Medicines Agency, EMA)

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), European Society of Paediatric Oncology (SIOPE)

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

Guests: Optimal Cancer Care Alliance (OCCA)

Introduction and adoption of the minutes of the previous meeting

SIOG and ESIP have now joined the CMF as observers.

During the last meeting, a decision tree was presented that could serve as a methodological guidance tool for the design of treatment optimisation studies. The tree was adapted based on the comments received from the participants and a new version was presented. This version will be revised further.

Learnings from the CMF so far on the occasion of its first anniversary

The first year of the CMF's existence was mainly dedicated to exploring the treatment optimisation landscape and to diagnosing the challenges associated with the conduct of treatment optimisation studies. Specific cases of such studies were discussed in prostate cancer, breast cancer and haematological malignancies. It was confirmed that despite its relevance for patients and healthcare systems, treatment optimisation falls into a vacuum, as highlighted by the fact that neither EMA's post-authorisation measures nor the new HTA Regulation offer approaches to address treatment optimisation questions.

Directions for the next year include setting up the basis for the deliverables of the CMF, communicating about the learnings, developing more case studies, and interacting with relevant stakeholders to act on the policy-related recommendations made by the CMF. The future meetings of the CMF will focus in particular on answering the questions of who should perform treatment optimisation studies, how these studies should be carried out, and when they should take place.

Discussion on the current HTA experience with real-world data

A recent publication co-authored by IQWiG was discussed. This publication describes the current HTA experience with real-world data (RWD) and explains how the use of such data for pre-licensing evidence generation, for example in the form of external control arms for single-arm trials, presents challenges for HTA agencies, since it means that these agencies often cannot properly evaluate how the effectiveness of new therapies compares to that of the standard of care. The paper also highlights that from an HTA perspective, evidence from randomised controlled trials (RCTs) in which the standard of care is used as the comparator is considered the most actionable type of evidence.

A potential solution to the problems HTA bodies face at present which is proposed in this paper could be to make randomised controlled trials easier, faster and cheaper to carry out by performing them in a standing European infrastructure in routine practice. According to the authors, an example to follow in this regard is the RECOVERY trial in the UK, which investigated treatments for COVID-19 and which produced robust data relatively quickly. During the discussion, it was stressed that such an infrastructure should ideally be run by an independent body, and that it would also allow for treatment optimisation questions to be tackled.

Discussion on the activities of OCCA

A presentation was given on the activities of OCCA, which aims to reduce the costs and the toxicity associated with cancer drugs by promoting the conduct of de-escalation trials. During this presentation, it was explored who would be best placed to undertake such trials, what the best location would be for running them, how they could be funded, and at what time they should be initiated in order for their results to be as impactful as possible. The general conclusion was made that you need the right place, the right people, the right drug, the right dose, the right funding mechanism, the right study design, the right level of evidence, and the right focus for these studies to succeed.

Next meeting

The next CMF meeting will be dedicated to preparing the first CMF workshop that will take place at the end of the year. This workshop will be open to the public. It will address some of the points raised here above and facilitate the development of a roadmap for the second year of the CMF.