EUROPEAN MEDICINES AGENCY
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Committee for Human Medicinal Products (CHMP)

# Concept paper on the revision of the guideline on the evaluation of anticancer medicinal products in man

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| Agreed by ONCWP | 10 October 2018 |
| Adopted by CHMP for release for consultation | 13 December 2018 |
| Start of public consultation | 14 January 2019 |
| End of consultation (deadline for comments) | 14 April 2019 |

The proposed guideline will replace 'guideline on the evaluation of anticancer medicinal products in man' (EMA/CHMP/205/95 Rev.5)

Comments should be provided using this template. The completed comments form should be sent to ONCWP@ema.europa.eu

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| Keywords | *Cancer, malignancy, biomarker, targeted drugs, pharmacogenomics* |

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# 1. Introduction

This concept paper introduces revision 6 proposing amendments and add-ons to the last version of the guideline on the evaluation of anticancer medicinal products in man. It is also proposed to amend the title to "the guideline on the clinical evaluation of anticancer medicinal products ~~in man~~" (currently EMA/CHMP/205/95 Rev.5).

The upcoming revision 6 proposes a review of the concepts related to biomarkers, which are increasingly used to define malignant diseases and develop new treatment strategies, improve description of the regulatory standards relevant for rare cancers and additional minor amendments to other sections.

# 2. Problem statement

Since the adoption of the guideline, the use of biomarkers in oncology and haematology has evolved and progressed considerably. This has resulted in novel development strategies as well as new definitions of therapeutic indications based on biomarkers. The current guidance does not adequately address these new aspects.

# 3. Discussion (on the problem statement)

The classical definitions of malignant diseases and of corresponding therapeutic indications are most often based on anatomy and/or histology. Meanwhile, treatments specifically targeting a pathological process linked to a biomarker have been shown to bring relevant benefits to patients suffering from tumours depending on biomarker status. Biomarker-based developments often focus on narrow subgroups identified within larger populations. These developments therefore often come closer to designs used for small populations. Another specific aspect of biomarkers in oncology is the possibility that the presence of a common pathological mechanism (detected by a biomarker) could predict the benefits associated to a treatment with an improved accuracy, across the traditional anatomy- or histology-defined diseases. New corresponding study designs (mainly basket trials and umbrella trials) are not discussed in the current version of the guideline.

Some other points deserve limited revision or adaptation (e.g., interim analyses, paediatrics).

# 4. Recommendation

The Oncology Working Party recommends revising the guideline on the evaluation of anticancer medicinal products by:

- Expanding the section on biomarkers to address the evolving scientific concepts, the place of biomarkers in the development pathway, and the main questions to be answered/solved during a standard development.
- Better describing the expected standards in the context of rare cancers.
- Presenting the main features and principles of new designs (mainly basket trials). Because regulatory experience is limited in this field for the time being, the guidance on this topic is only focusing on main aspects and principles.

## 5. Proposed timetable

Concept Paper to be released for consultation Q4 2018.

First draft of the Guideline to be released for consultation by Q2 2019.

## 6. Resource requirements for preparation

The resources needed for this addendum relate to the Oncology Working Party (ONCWP). The Biostatistics Working Party and the Pharmacogenomics Working Party will be consulted for comments during development of the draft guidance.

## 7. Impact assessment (anticipated)

The most important impact is expected to be on:

- The content of CHMP scientific advice.
- The content of regulatory submissions, including those to support anticancer agents based on biomarkers.

## 8. Interested parties

Healthcare professionals, pharmaceutical industry, patient organisations, EORTC, European learned societies involved in research in oncology.