European Medicines Agency

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COMMITTEE FOR MEDICINAL PRODUCTS FOR HUMAN USE (CHMP)

CONCEPT PAPER ON HAEMATOLOGICAL MALIGNANCIES

AGREED BY EFFICACY WORKING PARTY	January 2008
ADOPTION BY CHMP FOR RELEASE FOR CONSULTATION	24 January 2008
END OF CONSULTATION (DEADLINE FOR COMMENTS)	30 April 2008

This CP refers to a proposed new Appendix to The Notes for Guidance on the Evaluation of Anticancer Medicinal Products in Man (CPMP/EWP/205/95 rev. 3)

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1. INTRODUCTION

The Anticancer Notes for Guidance is a general guideline meant to cover conceptually all types of malignancies. However, this concept paper describes the reasons for considering an appendix covering haematological malignancies.

2. PROBLEM STATEMENT

Over the last few years we have seen a welcomed and much increased activity in the development of new medicinal products for the treatment of haematological malignancies, including rare disorders and small target populations. This activity has been reflected in submissions for marketing authorisation, but foremost in requests for scientific advice.

3. DISCUSSION (ON THE PROBLEM STATEMENT)

While the general guideline provides a conceptually valid basis also for the development of medicinal products for the treatment of haematological malignancies, there are some issues in need of special considerations such allogeneic stem cell transplantation and its consequences with respect to designation of primary endpoints, use of molecular techniques in response evaluations, treatments administered with curative or palliative intent, progression/relapse off-therapy, etc.

4. RECOMMENDATION

The Efficacy Working Party recommends to draft a new appendix to the anti-cancer guideline primarily focused the design of confirmatory studies in haematological malignancies. It is foreseen that certain major diagnosis are discussed separately such as the acute leukaemias, myelodysplastic syndromes, low and high grade lymphomas and malignant myeloma, while other rare conditions are covered conceptually.

5. PROPOSED TIMETABLE

- First discussion within the anti-cancer drafting group April 2008 (in the margin of the April EWP meeting: 7-8/04/08)

- First revised draft to anti-cancer drafting group May 2008.
- Written procedure in order to accomplish an agreed draft.
- Discussion of the consolidated draft at the July EWP (7-8/07/08)
- Adoption of a CHMP list of questions to the Oncology-SAG: July CHMP (21-25/07/08)
- Internally agreed draft to oncology SAG (10 September 2008).
- EWP (14/-15 October 2008).
- Release for 3-month consultation following adoption at CHMP (20-24 October 2008).

6. RESOURCE REQUIREMENTS FOR PREPARATION

It is foreseen that one drafting group meeting is needed. Otherwise no need for further extra meetings is foreseen.

7. IMPACT ASSESSMENT (ANTICIPATED)

Transparency and a consolidated EU regulatory position as regards the design of confirmatory studies in patients with haematological malignancies would be of interest for sponsors of drug development within this field and indirect for the haematological society.

8. INTERESTED PARTIES

EORTC, European Society of Haematology