Reflection paper on the use of Patient Reported Outcome (PRO) measures in Oncology Studies

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On behalf of the Oncology Working Party
The oncology working party was set up by the CHMP in order to carry out specific tasks related to the field of oncology.

The working party is composed of European experts selected from or associated with the national agencies, with an expertise in oncology.

Working Party's tasks include:

- Preparing, reviewing and updating of guidelines and concept papers
- Contributing to Scientific Advice Working Party activities upon request
- Contributing to product-related assessment following specific CHMP requests
- Preparing specific position papers and question-and-answer documents following specific CHMP requests
- Interacting with stakeholders under the supervision of the CHMP
The European Medicines Agency publishes scientific guidelines to help Applicants prepare marketing-authorisation applications for human medicines.

The guidelines provide a basis for practical harmonisation of how the EU Member States and the Agency interpret and apply the detailed requirements for the demonstration of quality, safety and efficacy.

In the area of oncology, the main guidance document is ‘Evaluation of anticancer medicinal products in man’.

This document was recently revised by the ONCWP, coming into effect in July 2013.

Among the new text and sections, the executive summary of the guideline states that:

- ‘A planned appendix 2 will focus on the use of patient reported outcome (PRO) measures and health-related quality of life (HRQoL) from a regulatory perspective’
PRO reflection paper

- In order to assist the WP in determining the scope of the HRQL paper, a workshop was held, where views were canvased from a variety of stakeholders including:
  - Regulators
  - HTA bodies
  - EORTC
  - Clinical experts
  - Patient organisations
  - Pharmaceutical industry

- There were ‘open floor’ discussions in relation to:
  - Clinical trial design
  - HRQL instruments
  - Statistical methodology
  - Clinical importance and added value
  - Patient related outcome measures

- Using the discussions of the workshop as a basis for the paper, the ONCWP has drafted a reflection paper on the use of patient reported outcome measures in oncology studies.
PRO reflection paper

- The draft paper contains the following sections:
  - Important definitions
  - 1. Background
  - 2. Scope
  - 3. Legal basis
  - 4. Patient reported outcomes
    - 4.1. Health Related Quality of Life (HRQL)
  - 5. Clinical trial design
    - Frequency and duration of assessment
    - Data collection
    - Statistical methods and missing data
  - 5.1 Instruments
    - Selection of an instrument
    - Carer / proxy input
  - 5.2 Special patient populations
    - Paediatric
    - Elderly
    - Palliative setting
  - 6. Symptom PRO measures
  - 7. Clinical importance and added-value

References
Summary

• A new reflection paper on the use of PRO measures in oncology studies was released by the CHMP for public consultation in May 2014

• The reflection paper builds on the CHMP paper on regulatory guidance for the use of HRQL measure in the evaluation of medicinal products (2005)

• The PRO paper covers a number of areas, recognising the importance of the patient’s view on their health status

• The paper aims to spur an open discussion on the value of PRO data in the development of oncology medicinal products, whilst acknowledging that PRO methodology is developing and evolving

• Comments on the paper can be sent to the ONCWP Secretariat (consultation ends 30 November 2014)