Medicines and Healthcare Products Regulatory Agency

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






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Oncology Working Party

Medicines and Healthcare Products Regulatory Agency

- 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

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- 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'

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PRO reflection paper

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- 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

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PRO reflection paper

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- 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

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Summary

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- 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)
- http://www.ema.europa.eu/ema/doc_index.jsp?curl=pages/includes/document/document_detail.jsp?webContentId=WC500168852&murl=menus/document_library/document_library.jsp&mid=0b01ac058009a3dc

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