COMP 2016 Work Plan

What is it about?
On 21 January 2016, the Committee on Orphan Medicinal Products adopted its work plan for 2016.

Why is it important?
The COMP puts a strong emphasis on the subject of significant benefit, which is mentioned in the great majority of its priority activities for 2016. This reflects the current revision of the European Commission guideline on the topic, which will most likely make the assessment of significant benefit of orphan medicines stricter. The COMP activities will partly respond to a necessary adaptation to these new rules.
The pharmaceutical industry will be given the opportunity to share feedback on the role of the COMP and on the development of orphan drugs. This will be done via meetings with trade associations.

Perspective
Each year, each committee and working party of the European Medicines Agency sets up its respective goals and activities, including the various guidelines they plan to create or review. These work plans give an idea of the topics on which each regulatory group plans to focus during the year, reflecting the subjects of importance in the current external environment.

News summary
1. Evaluation activities for human medicines
1.1. Pre-authorisation activities
1.1.1. Designation of orphan medicines
Key objectives
· Ensure consistency of answers to questions on significant benefit in Protocol Assistance and allow time for in-depth discussions within the plenary, with the aim of clarifying the requirements for the demonstration of significant benefit
· Ensure consistency, transparency, quality and detail of the grounds of opinions given by the COMP on 'significant benefit' at the time of marketing authorisation in view of its impact on the post-marketing life of the orphan products
Activities in 2016
· Publications of an article on non-clinical models
· Work through quarterly TCs with Rarecare and Orphanet with a focus initially on prevalence issues associated with orphan designation and rare diseases to:
- continue to build on the observations of increasing prevalence in certain oncological conditions;
- explore if changes are under-reported in non-oncological conditions;
- discuss prevalence methodology and concerns specific to Europe.
· Decide whether the document "Points to Consider on the calculation and reporting of the prevalence of a condition for orphan designation" (COMP/436/01) should be updated based on the outcome of the discussions.
· Systematically involve patients' representatives in COMP discussions on significant benefit based on major contribution to patient's care.
· Establish a working group on Protocol Assistance that will meet monthly to discuss any topics on significant benefit and prepare recommendations to the COMP on these questions. Operational considerations involving COMP members and EMA members are to be explored to maximize involvement of members on both sides.
· Publication of a comprehensive article that summarises the conclusions of the Working

Group on Significant Benefit.

· Publication of a report on the outcome of the Significant Benefit Workshop.

· Update the "COMP recommendation on elements required to support the medical plausibility and the assumption of significant benefit for an orphan designation, EMEA/COMP/436/01" based on the outcome of the Workshop on significant benefit, the work of the Working Group on significant benefit and the updated EC Communication.

2. Horizontal activities and other areas

2.1. Committees and Working Parties

2.1.1. Additional objectives and activities

Key objectives

· Establish a COMP-CAT working group to optimise the use of the two Committees in discussions on orphan ATMP.

· Increase the visibility of the COMP within the regulatory system and make sure there is a common understanding on significant benefit when used for orphan designation.

Activities 2016

· Participate in the joint CHMP-PDCO and COMP working Group on significant benefit and report back to COMP. The key objectives with this inter-committee collaboration are:

- Clarify the different concepts of "significant benefit"
- Review of consistency in assessment of how "significant benefit" is applied across different legislative provisions
- Improve interactions with CHMP on significant benefit and clinical superiority.

· Collaborate actively with PRIME and other early dialogue initiatives and assess the implications for orphan medicines

2.2. Partners, stakeholders and transparency

2.2.1. Interactions with partners

Activities in 2016

· Participation in the European Commission Expert Group on Rare Diseases (CEGRD) meetings organised by the Commission.

· Invite stakeholders (patient and industry associations) to meet the COMP and share reflexions regarding the role of the COMP and development of orphan drugs

2.3. International activities

2.3.1. Exchange of information

Key objectives

· Increase awareness between EMA and PMDA/MHLW (Japan) regarding the orphan drug designation process.

· Facilitate global development by increasing transparency on differences and similarities of the orphan designation process in US, Japan and Europe

· To further develop interactions with the Office of New Drugs, Center for Drug Evaluation and Research at FDA to discuss post designation issues

· Strengthen the exchange of information with Health Canada

Activities in 2016

· Contribute to a book on the development of medicines for rare diseases in US, Japan and EU

· Article on international communication to be published

· Within the sustained committee policy on publications, to prepare joint abstracts with FDA and PMDA/MHLW to be presented at the Euro Congress on Rare Diseases (May 2016).

Related Information

· EMA workshop "Demonstrating significant benefit of orphan medicines: concepts,

methodology, and impact on access"