SUMMARY REPORT

EMA- EuropaBio Information Day

15 October 2015, 10:00 - 17:00
De Vere Venues – Canary Wharf, 1 Westferry Circus, London

Introduction

The EMA-EuropaBio Information Day provides a forum for members of EuropaBio and EMA officials to meet on an annual basis to share information and discuss topics of mutual interest for the healthcare biotech industry. Industry representatives have the opportunity to discuss the latest regulatory and scientific developments and raise specific issues, illustrated with practical examples from their experience.

Previous Information Days held in 2013 and in 2014 have focused on topics such as HTA, Biosimilars, TTIP and Personalised Medicine. The topics identified for this meeting included: I) early access, II) orphan medicines and III) advanced therapy medicinal products. The detailed agenda and presentations have been made available on the EMA website. This report provides a brief recap of the discussions on the day and is not intended to be fully comprehensive. Please refer to the relevant slides for more details on each topic.

Session I - Early Access

Guido Rasi, EMA's Nominated Executive Director, opened the discussion with a short presentation of the work carried out by the Agency and the HMA Network. He summarised the main trends and challenges in the EU and outlined the four strategic goals of the European regulatory system to 2020, i.e.: operational optimisation, global convergence, transparent communication and access to innovation. With regard to the last goal, he highlighted the tools provided by the Agency to support drug development and the Agency's efforts to mainstream patients' input into all EMA activities. Following this, Jordi Llinares, Head of Product Development Scientific Support Department at EMA, provided a short overview of the new EMA scheme to support the development of priority medicines for unmet medical needs, called PRIority MEdicines (PRIME). The EMA launched a public consultation on a reflection paper describing PRIME on 26 October 2015 (more details can be found here).

Michael Berntgen, Head of Scientific & Regulatory Management Department at EMA, provided more in-depth information on two specific early access tools: accelerated assessment (AA) and conditional marketing authorization (CMA). He presented some highlights from medicines' evaluation experiences in 2015 and then described the main changes in the revised EMA guidelines on AA and CMA. He noted that the revision builds on the EMA experience with these guidelines over the last 10 years and is expected to be finalized by Q1 2016.

Martine Zimmerman, Vice President for Global Regulatory Affairs at Alexion, provided a case study of two products submitted for review via accelerated assessment. She provided the EMA with feedback on the company's experience of available tools and with some practical suggestions to further improve the procedural aspects related to accelerated assessment and authorization under exceptional circumstances.

Rob Hemmings, SAWP Chair and CHMP member for the United Kingdom, presented "adaptive pathways" as a concept exploring methodologies for evidence generation to

balance timely access for patients with the need to provide adequate evolving information on benefits and risks. After a short introduction to define the concept and the early access tools on which it builds, he provided an update about the on-going EMA pilot in this area. His introduction was followed by a presentation by Fabrizia Bignami, Head of Patient Engagement & Public Affairs, Global Medical Affairs Lead ADA-SCID at GSK Rare Diseases. Dr Bignami provided some feedback as a company participating in the pilot. Emily Crossley, Founder and Director of the Duchenne Children's Trust in the UK, provided the patient perspective on EMA's adaptive pathways pilot, by presenting how patients taking part in clinical testing with medicinal products included within such pilot, could benefit.

Session II - Orphan Medicinal Products

Stylianos Tsigkos, Scientific Administrator at the EMA Orphan Medicines office, set the scene by presenting highlights of 15 years of operation of the EU Orphan legislation, including the experience of the EMA in reviewing applications and the range of services offered by the Orphan Medicines office to guide sponsors towards an orphan designation.

Robert Morgan, Head of EU Regulatory Strategy at Shire, presented some of the challenges faced by companies in complying with certain EU guidelines for orphan drugs, especially as regards: a) obtaining initial orphan designation, b) demonstrating significant benefit at the time of MA application, and c) maintaining orphan status until final authorization.

Laura Fregonese, Scientific Administrator at the EMA Orphan Medicines office, elaborated on the previous presentation by explaining some of the challenges faced by the Agency in assessing the evidence to demonstrate significant benefit. A workshop on methodologies to assess significant benefit will take place at the EMA on December 7, 2015 and will go into further detail on this topic.

Session III - Advanced Therapy Medicinal Products

Rocio Salvador-Roldan, Policy Officer, DG SANTE, European Commission, described the background and key points of the EU targeted stakeholder consultation on the development of Good Manufacturing Practice for Advanced Therapy Medicinal Products pursuant to Article 5 of Regulation 1394/2007 (see more details here). Alec Orphanidis, Senior Vice President Global Commercial Operations at UniQure, the first company which marketed a gene therapy in Europe, provided some insight into the specificities of the manufacturing process for Advanced Therapies. He also explained that EuropaBio is working to collect input to respond to the EC consultation on GMP for ATMPs by the set deadline of 12th November 2015.

Metoda Lipnik Stangelj, Slovenian Member at the EMA Committee for Advanced Therapies (CAT) outlined the content of a new Q&A document for minimally manipulated ATMPs that the CAT is currently preparing (expected to be published in first half of 2016). The Q&A is meant to help applicants apply the Risk Based Approach, foreseen by the ATMP regulation, to the case of minimally manipulated ATMPs, which may be more difficult to characterize in terms of quality and of non-clinical/clinical development, due to the variability of starting material compared to other ATMPs.

Peter McArdle, Director Regulatory Affairs for Cell & Gene Therapies at Novartis, presented the perspective of a global manufacturer with regard to the challenges in the clinical development of ATMPs. He commented on the current state of ATMPs in Europe and called for further harmonization on this topic within the ICH region. In particular, he noted CTA processes for ATMPs can be challenging for sponsors and that public health authorities should be more flexible in assessing CT applications, with longer timelines.

Finally, Lisbeth Barkholt and Caroline Voltz from the EMA presented an overview of common questions raised during scientific advice/protocol assistance by ATMP

developers/applicants as well as the main kind of objections raised for such type of medicinal products/applications.

Conclusion

Melanie Carr Head of the EMA Corporate Stakeholders Department thanked all participants for their contributions. She noted how, in many areas, the Information Day had highlighted the need for industry to plan development strategies in a more prospective way and in particular to cater for more frequent, early interactions with regulators through the scientific advice process.

Robin Evers, EuropaBio Healthcare Council Chair & Senior Vice President Regulatory Affairs, Novo Nordisk, thanked the EMA for sharing up-to-date information with EuropaBio members. He also underlined that this annual meeting is an extremely productive platform to provide relevant feedback to the EMA on areas for improvement and further optimization of the framework, in line with scientific and industrial progress.