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Press Office

Press release

Ten years of orphan medicines legislation in Europe – European Medicines Agency reviews success and looks ahead

10 years of Orphan Regulation in Europe Conference, 3-4 May 2010

On 3 and 4 May 2010 the European Medicines Agency held a two day conference to mark the 10th anniversary of the Orphan Regulation in the European Union. The Agency brought together representatives from the European Parliament, the European Commission, international and European regulatory agencies, members of the Committee for Orphan Medicinal Products (COMP), patient groups, health professionals, and pharmaceutical industry to review the impact of ten years of orphan medicines legislation and to look ahead at future opportunities and challenges.

Ten years since the orphan legislation came into force in April 2000, the Agency has received more than 1100 applications. Out of these, 720 orphan designations have been granted to date, a success rate of 65 %. A total of 62 orphan designated medicines have now been approved for use in the EU, giving treatment options for 53 different rare diseases.

The COMP was the first of the Agency's scientific committees to include representatives from patients' organisations. "The collaboration in the assessment of applications for orphan designation has led to a process of cross-fertilisation between regulators and patients, and ultimately to improved decision-making by the Committee as a whole" said Kerstin Westermark, chair of the COMP. The Paediatric Committee (PDCO) and the Committee for Advanced Therapies (CAT) have since followed the successful COMP model and include patients' representatives as committee members.

The future of orphan medicines – challenges and opportunities ahead

The continued interest in the orphan designation process shown by the pharmaceutical industry indicates that orphan-designated medicines will keep coming to the market at a steady rate offering new treatment options for patients with rare diseases. Over the next few years the period of market exclusivity (10 years) will expire for the first authorised orphan medicines, opening up the market for older orphan-designated medicines for competition, while new orphan medicines continue to be protected by market exclusivity. The Agency expects that an increasing number of new marketing

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authorisation applications will relate to complex, innovative medicines, such as advanced therapies medicinal products (gene therapy, somatic cell therapy or tissue engineered products). With the combined expertise of the COMP, CAT and the Committee for Medicinal Products for Human Use (CHMP) as well as a network of experts from across the EU, the Agency is in a good position to tackle the challenges coming from new scientific developments.

While the outlook for authorised orphan-designated medicines is promising, there is some concern that not all patients, who need to, have access to these medicines in the European Union because of the high costs of treatment. European Medicines Agency Executive Director Thomas Lönngren said: "We are looking at how to increase transparency of our scientific decision-making so that those bodies within the EU Member States, who decide about the pricing of orphan medicines can also use our knowledge when making their decisions." The Agency is already collaborating with Health Technology Assessment (HTA) bodies to identify the best ways to share information.

Making more and better treatments available for patients with rare diseases will require global collaboration. The European Medicines Agency and the US Food and Drug Administration have been working together for several years now to reduce red tape for sponsors who want to develop orphan medicines for European and for US patients. The workshop participants agreed that other aspects such as post-marketing safety surveillance studies or handling of supply issues would also benefit from increased multi-national cooperation and should be tackled at a global scale.

The orphan legislation in Europe has been a success so far. The workshop participants all agreed that adequate funding from the EU budgetary authority for the incentives provided by the orphan designation and for clinical research will be imperative for the continued success of the orphan initiative.

Notes

1. Information about the conference is available here: http://www.ema.europa.eu/meetings/conferences/03may10.htm
2. An overview of 'Ten years of orphan legislation in numbers' is available here: http://www.ema.europa.eu/pdfs/human/comp/27960110en.pdf
3. Information about orphan medicines and rare diseases is available here: http://www.ema.europa.eu/pdfs/human/comp/29007207en.pdf
4. More information about the Agency's activities in relation to orphan medicines is available here: http://www.ema.europa.eu/htms/human/orphans/intro.htm
5. This press release, together with other information on the work of the European Medicines Agency, can be found on the Agency's website: www.ema.europa.eu

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