The European Agency for the Evaluation of Medicinal Products
Pre-authorisation Evaluation of Medicines for Human Use

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PRELIMINARY MEETING REPORT

Workshop on Methodological Aspects of Clinical Trials for Efficacy Evaluation in Small Populations

On 22 October 2002, the European Agency for the Evaluation of Medicinal Products held a one-day workshop on methodology of clinical trials in small populations. Such trials are those performed in particular in patients affected by rare diseases and in children.

This first workshop organised by the EMEA on the subject was co-chaired by Prof. Josep Torrent-Farnell, Chairman of the Committee for Orphan Medicinal Products (COMP) and Dr. Daniel Brasseur, Chaiman of the Committee for Proprietary Medicinal Products (CPMP).

COMP and CPMP members, as well as European Union (EU) experts from academia and university hospitals, patients representatives and representatives of regulatory authorities from the EU and candidate countries participated to the meeting. The US Food and Drug Administration (FDA) participated with presence of Dr. Marlene Haffner, Director of the Office for Orphan Product Development (OOPD) at the FDA and through video-links with several sites in Washington DC.

This event is a contribution to the development of orphan medicinal products in the EU as well as comes in anticipation of future developments in favour of paediatric medicinal products. Since the implementation of the EU Regulation on orphan medicinal products in 2000, 115 orphan medicinal products have been designated following positive opinions from the EMEA COMP and 7 applications for marketing autorisation of orphan medicinal products have received positive opinions from the EMEA CPMP.

The objective of the meeting was to initiate a reflection on alternative designs and statistical methods to evaluate efficacy of medicines where recruitment of patients is limited and classical methods would require large sample sizes. Advantages and limitations of these methods (e.g. Bayesian methods, sequential designs and sample-size re-assessment) for both sponsors and regulatory agencies were discussed, using relevant practical examples. Pragmatic approaches were confronted to classical randomised, parallel groups clinical trials.

Patients' representatives supported the need to obtain high quality data for the evaluation of medicines with optimal numbers of patients included in the trials, in particular to take into account the difficulty of recruitment of volunteer eligible patients in small populations.

This very positive experience will encourage the EMEA to renew such event in the future.

NOTE: This Press Release, together with other information about the work of the EMEA, may be found on the internet at the following location: http://www.emea.eu.int/

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