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

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PRESS RELEASE

Orphan drug and paediatric clinical trials – EMEA workshop on methodological aspects of clinical trials for efficacy evaluation in small populations

Rare diseases and diseases in children both affect small numbers of individuals. Clinical trials for more common diseases have traditionally relied on the inclusion of large groups of patients – something that is not possible when developing medicines for rare diseases and children. The increasing number of orphan medicines seeking marketing authorisation in the European Union as well as the future EU legislation on medicines for children make it necessary to develop new ways and new methodologies to design reliable clinical trials appropriate to the size of these populations.

The EMEA took the initiative to organise a first workshop on this topic with attendance of experts, patients' organisations and regulators on 22 October 2002. Many renowned methodology experts from various EU Member States attended and shared their experience. As the most concerned by these trials, patients' organisations took an active part in the discussion on the advantages and limitations of both classical and alternative methods for such clinical trials. Positive conclusions were drawn showing the possibility of using alternative designs such as Bayesian, sequential designs or sequential analysis with an open mind but clear quality requirements.

This is an issue facing patients and regulators around the world and the EMEA was pleased to welcome the participation of Dr Marlene Haffner, Director of the Office for Orphan Product Development at the meeting. A number of other colleagues from the US Food and Drug Administration joined the meeting by video-link.

The EMEA intends to pursue this initiative to encourage the clinical development of orphan and paediatric medicines. More information is available in a preliminary meeting report and the proceedings will shortly be made available on the EMEA website *http://www.emea.eu.int*

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/

For further information, please contact:

Martin Harvey, EMEA press officer
Tel. (+44-20) 74 18 84 27,
E-mail: martin.harvey@emea.eu.int