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

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MEETING REPORT

Joint Meeting with all Interested Parties on a Continuity Policy for Orphan Medicinal Products in the European Union.

On 2-3 December 2002, the European Agency for the Evaluation of Medicinal Products in association with EBE/EFPIA, Europabio and EURORDIS held a two-day meeting with all Interested Parties on a Continuity Policy for Orphan Medicinal Products in the European Union. Orphan medicinal products are those products aimed to treat or prevent serious or life threatening diseases that occur very infrequently. The Committee for Orphan Medicinal Products, COMP, is an EMEA Committee composed of experts and representatives of patients' organisations which main task is to designate Medicinal Products as Orphan according to predefined criteria. This joint meeting is the result of the COMP-Working Group with Interested Parties' work, a COMP priority that reflects the importance of the collaboration between the stakeholders recognised by the COMP. It participates in one of the mission of the COMP, which is to advise the European Commission on orphan Policy.

The meeting was chaired by Mr Yann Le Cam (COMP vice-chairman, EURORDIS, a joint European patients' organisation) and Ms Paola Ricci (EBE/Europabio, Serono). On the first day a number of interventions on the achievements and obstacles of the European Orphan Medicinal Products Regulation implementation took place. In the afternoon five different workshops addressed the issue of "policy continuity" in the field of orphan drugs. Each of the workshops covered one specific area: drug development, orphan designation, protocol assistance, marketing authorisation and patients' access to orphan medicinal products. The conclusions of the five workshops were presented during the second day in a plenary meeting and the discussion and the proposals were discussed.

Mme Grossetête, member of the European Parliament, as well as members of various European Commission Directorate General, COMP and CPMP (Committee for Proprietary Medicinal Products) members, Patients' representatives, European Union (EU) experts from Academia and University Hospital, members of the Pharmaceutical Industry and representatives of regulatory authorities from the EU and candidate countries participated in the meeting. The participation of the US Food and Drug Administration (FDA) was also noted with the presence of Dr. Marlene Haffner, Director of the Office for Orphan Product Development (OOPD). On the first day 120 attendees participated in the meeting and more than 150 on the second day.

This is the first joint meeting with all interested parties (Patients, Pharmaceutical Industry, Learned Societies and Academia members and Regulatory bodies) held on Orphan Medicinal Products.

The experience accumulated in the European Union since the approval of the European Regulation on Orphan Medicinal Products is still limited but the work done by the Committee on Orphan Medicinal Products of the EMEA has been of great success. This has translated into 127 Orphan designations for Medicinal Products, and the Committee for Proprietary Medicinal Products of the EMEA has delivered positive opinion for marketing authorisation for seven Orphan Medicinal Products. In 2002 one third of the applications submitted for Marketing Authorisation corresponded to designated Orphan Medicinal Products. It is foreseen that this number will rise up to fifty per cent in 2003.

The integration of Patients in the evaluation of the criteria for designation of Orphan medicinal products for the first time in the European experience was pointed out as one of the most remarkable achievements after the Regulation implementation. This brings an innovative perspective to the evaluation of Medicinal Products, in the interest of Public Health.

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In the workshops it was stressed that the real availability of Orphan Medicinal Products and access to patients remain as one of the most important challenges for the success of the European Policy on Orphan Medicinal Products. A commitment from the European authorities responsible for pricing and reimbursement is seen as necessary to ensure a complete success.

In order to facilitate research, clinical investigation and evaluation it is necessary to develop further the European Expert Network as well as an European Clinical Trial Network in the field of rare diseases. These should be European collaborative initiatives.

All future projects and developments are limited by the available funding for research. It is felt that a substantial budgetary effort at an European level is necessary to maintain the activities aimed to develop the policy on Orphan Medicinal Products. This effort is still being made in the USA 20 years after the start of the Orphan Policy. Funding of research and development of orphan medicinal products are seen of paramount importance and necessary for the future development of the European Orphan Policy.

Information on current clinical trials and other forms of access to drugs for rare diseases should be made public. The general feeling is that a public database could be the most adequate way to provide this information. The current confidentiality concept has to evolve to enable authorities to provide additional information to the public.

Safety issues of Orphan Medicinal Products coming in the future will require new and innovative scientific approaches to deal with post-authorisation challenges. A coordinated effort in this direction, establishing the appropriate contacts and supporting networks has been already considered and started at the EMEA.

It was agreed that an integrated and consistent European policy on Orphan Medicinal Products is necessary and requires collaboration between all DGs (Directorate General) concerned as well as all stakeholders. Finally, constant communication between all interested parties is also necessary and was pointed out by all participants as a cornerstone for the future.