European Union Committee of Experts
on Rare Diseases

4th Meeting of the European Union Committee of Experts on Rare Diseases

26-27 January 2012

Executive Summary

The fourth meeting of the European Union Committee of Experts (EUCERD) took place on 26-27 January 2012 in Luxembourg. The plenary was preceded by preparatory meetings by stakeholder groups, which resulted in fruitful discussions concerning the two main issues for discussion, the Clinical Added Value of Orphan Drugs (CAVOD) and European Reference Networks.

- **Clinical Added Value of Orphan Drugs (CAVOD)**

The EUCERD has started the elaboration of recommendations for a policy framework around the results of the report of the call for tender study on a mechanism of knowledge exchange on Clinical Added Value of Orphan Drugs (CAVOD) led by Ernst & Young. An initial draft of the EUCERD recommendations was discussed at the preparatory meetings and plenary session. A representative of EUnetHTA was present to reinforce collaboration with the EUCERD during the plenary session. Following discussion, it was decided that a set of tools is necessary to improve, ease and speed up the assessment of the Clinical Added Value of Orphan Drugs (CAVOD). The EUCERD favours focusing on the sharing of data between Member States to generate evidence for health technology assessment agencies and decision makers and recommends to entrust this task to the EUnetHTA, the European network of national health technology assessment agencies (http://www.eunethta.eu/). In conclusion, the EUCERD will issue recommendations to serve as a basis for discussion between the principal stakeholders at European level, including the relevant services of the European Commission. The final version will be submitted to the EUCERD for adoption at the next meeting in June 2012.

- **European Reference Networks (ERN) for rare diseases**

European Reference Networks (ERN) for rare diseases were another priority topic at this meeting : the EUCERD has started the process of elaborating recommendations on ERN to serve in the elaboration of the criteria for ERN to be established by the Committee on Cross-Border Healthcare. A workshop will take place on 19 June 2012 prior to the next meeting of the EUCERD, in the context of the EUCERD Joint Action, in order to discuss the draft recommendations. The adoption of these recommendations is scheduled for the sixth meeting of the EUCERD in November 2012. Member States were urged to work on the designation of centres of expertise at national level using the "EUCERD Recommendations on Quality Criteria for Centres of Expertise for Rare Diseases in Member States" adopted in October 2011, as this must be done before ERNs are established. The ERNs will be financed in the context of the 3rd Health Programme of the European Union.

- **New born screening practices in the European Union**

The EUCERD was presented the results of the tender on new born screening practices in the European Union by the contractors. Members gave their feedback on this report and expressed the opinion that best-practices in the field should be disseminated to share expertise between countries, especially for diseases where there is international consensus as regards new born screening (i.e. phenylketonuria and hypothyroidism). It was recommended to set up a collaboration between Member States to share data and knowledge on diseases where screening is technically possible in order to help Member States carry out their benefit-risk assessment. A workshop on newborn screening with the participation of the EU network of experts consulted in the course of the tender on newborn screening is planned for the first trimester of 2013.

- **European Partnership for Action Against Cancer (EPAAC) and rare cancers**

The European Partnership for Action Against Cancer (EPAAC) was presented to the EUCERD and discussion concerning the synergies between this initiative and actions in the field of rare diseases were discussed. It was decided that the members of the EUCERD implicated in the rare cancers activities of the EPAAC should explore the synergies between this initiative and the EUCERD Joint Action.

- **National plans and strategies for rare diseases**

The Member State representatives of the EUCERD each reported on the progress in their country towards the elaboration of a national plan or strategy for rare diseases, as recommended by the Council Recommendation on an action in the field of rare diseases for 2013. The elaboration or implementation of these plans or strategies seems to be gaining momentum; Member State representatives were asked to identify the person in charge of the elaboration or implementation of their national plan/strategy in order to constitute a network in the context of the work package of the EUCERD Joint Action that continues the actions of the Europlan project.

- **EUCERD Joint Action and events in 2012**

The events programmed for 2012 in the context of the EUCERD Joint Action (due to start on 1 March 2012) were presented. A Kick Off meeting is planned for associated partners on 12-13 March 2012 in Paris. A number of workshops have already been scheduled including: a workshop to elaborate ERNs on rare diseases on 19 June 2012, an expert workshop on the cross-referencing of terminologies is planned for 27-28 September 2012 in Paris, and a workshop on social services in the field of rare diseases in November 2012. The European Union Joint Research Centre in Ispra has offered to host a EUCERD workshop in Autumn 2012 on the genetic testing offer in Europe.

- **Elaboration of the 2012 EUCERD report on the “State of the Art of Rare Diseases Activities in Europe”**

The EUCERD were also updated on the schedule for the elaboration of the 2012 EUCERD report on the “State of the Art of Rare Diseases Activities in Europe” which will cover activities up to the end of 2011: Member States representatives received at the end of February 2012 their national data (up to December 2011) which they should update with the input of national stakeholders before 30 March 2012. The final publication of this report is scheduled for July 2012.

- **2012 European Conference on Rare Diseases and Orphan Products**

The programme of the 2012 European Conference on Rare Diseases and Orphan Products (23-25 May 2012, Brussels) was presented. Members were encouraged to attend the conference and participate in the « Meet the EUCERD members » networking drinks on 24 May 2012 and also to disseminate general information about the conference (www.rare-diseases.eu) and encourage national stakeholders to attend.

- **Rare Disease Day 2012**

EUCERD members also informed of a new awareness-raising video produced by EURORDIS to mark Rare Disease Day 2012 (29 February 2012) : members were encouraged to use the video widely (http://www.rarediseaseday.org/solidarity).

- **Other decisions and next meetings**

Finally, the decision was taken to produce executive summaries for each of the documents issued by the EUCERD in order to improve the dissemination of the results of the EUCERD's work.

The next meeting of the EUCERD will be held on 20-21 June 2012 in Luxembourg.