European Union Committee of Experts on Rare Diseases

EUCERD/EMA Workshop: Towards a Public-Private Partnership in the Field of Registries for Rare Diseases – 4 October 2011

Workshop Report Summary

On 4 October 2011, over 60 participants representing all stakeholder groups met at the European Medicines Agency (EMA) in London to discuss the subject of public-private partnering for rare disease registries. The workshop was organised by the European Union Committee of Experts on Rare Diseases (EUCERD) with the support of the EMA. It was the fruit of previous work on the subject of registries conducted by the EC Rare Diseases Task Force (RDTF) and Scientific Secretariat of the RDTF/EUCERD, including the RDTF reports on "Patient registries in the field of rare diseases" (2008/2011) and "Health indicators for rare diseases (2010, 2011)", as well as the Orphanet Report "Disease registries in Europe" (2011).

The objectives were to avoid the duplication of work in this field, to maximise the output, to discuss sustainability issues. The focus was on the burning issue of organising registries by disease and not longer by product, to better deal with the requests of regulators and payers to access data for the assessment of the clinical utility of new drugs for which registers are excellent source.

A consensus was established amongst the gathered stakeholders that it is imperative that fragmentation of data sources be avoided: public/private partnership is necessary and, although it cannot be made mandatory, it can be suggested by the EMA to companies that they should consider joining existing registration systems or establishing a new one in partnership with an academic team and patient organisations. It was also suggested that technical and methodological support be provided as should rules of conduct for such partnerships. Regulatory frameworks and standards must be assured. Open-access to data should be promoted. Management by academia was identified as a solution to ensuring long term sustainability with the financial support of the regulatory bodies and of the payers, jointly with the concerned companies.

Finally, it was proposed that a small working group of stakeholders be established to work on the next steps, in order to move forward and concentrate on future opportunities (i.e. HTA requirements for MS). Liaison with CAVOD plans should be assured with the EUCERD as right forum to discuss issues of clinical utility. The potential opportunities and/or threats posed by the revision of the data protection legislation must also be carefully considered.

The outcomes of this workshop will serve as the basis for the elaboration of a EUCERD recommendation in this field.

The full public report of this meeting can be found here :

http://www.eucerd.eu/upload/file/EUCERDWorkshopRegistries2011.pdf