



Flash report

Commission Expert Group on Rare Diseases 8th meeting, 28-29 November 2016, Luxembourg

INTRODUCTION

The meeting of 28-29 April 2016 focused on the new Framework for Commission Expert Groups, Research on Rare Diseases, Health Technology Assessment in the area of Rare Diseases, the European Platform on Rare Diseases registration and on the European Reference Networks.

NEW FRAMEWORK FOR COMMISSION EXPERT GROUPS

The Expert Group was informed that, in May 2016, the Commission has adopted new rules on the creation and functioning of the advisory expert groups which provide external expertise to help inform the policy-making process.

It was highlighted that the Decision provides a single set of rules and principles aimed at increasing transparency, avoiding conflicts of interest and ensuring a balanced representation of interests.

RESEARCH ON RARE DISEASES

The success of the ERA-NETs and EU funded research, together with the establishment of European Reference Networks, create momentum for closer collaboration and creation of a pipeline ensuring rapid translation of research results into clinical applications and uptake in health care for the benefit of patients.

An option for implementing such a programme in Horizon 2020 is the model of the European Joint Programme Cofund (EJP Cofund), as it is designed to support the implementation of a joint programme of activities ranging from research to coordination and networking activities, including training, demonstration and dissemination activities.

HEALTH TECHNOLOGY ASSESSMENT IN THE AREA OF RARE DISEASES

In October 2016 the Commission has launched a public consultation on strengthening EU cooperation on Health Technology Assessment (HTA).

The latest developments regarding the EU cooperation on HTA were presented and the main documented challenges for HTA in Europe were discussed.

A presentation on the patients' perspective on HTA in Rare Diseases was given, as well a presentation on the new Dutch approach on assessing orphan drugs (HTA) for reimbursement was given.

EUROPEAN PLATFORM ON RARE DISEASES REGISTRATION

The Expert Group was informed by the Joint Research centre about the developments of the Platform on Rare Diseases Registration. In the next months, the Joint Research Centre is going to organise interoperability workshops to agree registration standards. The next meeting on registries in European Reference Networks is going to be held on 30th November.

EUROPEAN REFERENCE NETWORKS (ERNS)

The Group was informed that, in response to the call for proposals in 2016, 24 networks applied, including 960 highly specialised healthcare units of 370 hospitals located in 26 Member States and covering a high number of disease groups. The ERNs will become operational in 2017. Regarding the approval of the ERNs, the Board of Member States will take the decision on 15 December based on the assessment by independent assessor. The launch of the Networks is foreseen during a major conference organised on 9 March 2017 in Vilnius.