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Human Medicines Research & Development Support

# Worldwide Orphan Medicinal Designation Workshop, 10 March 2014

The European Medicines Agency (EMA) held the first Worldwide Orphan Designation Workshop on the 10 March 2014 where the US Food and Drug Administration (FDA), Japanese Ministry for Health, Labour and Welfare (MHLW)/Pharmaceuticals and Medical Devices Agency (PMDA) and EMA presented their respective designation systems as well as the respective grants and post designation incentives available in their regions. This later topic was introduced as a new topic within the framework of the morning sessions to enhance the understanding of the opportunities and incentives available in the framework of each region.

The European Commission presented the basis of the orphan legislation in Europe as well as the grants available within the European Horizon 2020 and the International Rare Disease Research Consortium (IRDiRC). IRDiRC teams up researchers and organisations investing in rare diseases research in order to achieve two main objectives by the year 2020, namely to deliver 200 new therapies for rare diseases and means to diagnose most rare diseases. The FDA discussed its grants system for rare diseases and associated ones within the framework of the National Institutes of Health (NIH) and the Japanese authorities presented the grants system under the National Institute of Biomedical Innovation (NIBIO).

Regulatory incentives also presented included reduced fees for scientific advice, variations on marketing approval submissions specific to orphan medicines and support for research in children for example. In the afternoon the three agencies organised face-to-face meetings with sponsors who presented products which they wished to submit for an orphan designation. During these face-to-face meetings the adequacy of the data available in the proposed submission was discussed as well as some of the incentives available.

The morning session meeting was restricted to 100 participants in person and was broadcast live on the internet. Two hundred and fifty people accessed the broadcast live during the morning session. The audience comprised pharmaceutical companies, academic centres, patient organisations and clinical research organisations.

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