Introduction

This is a joint European Medicines Agency (EMA), US Food and Drug Administration (FDA), and Japanese Ministry of Health, Labour and Welfare (MHLW) and Pharmaceuticals and Medical Devices Agency (PMDA) Orphan Medicinal Product Workshop.

The aim of the Workshop is to provide information to companies, as well as to academics, on the EMA, FDA and MHLW-PMDA systems for orphan medicinal product designation as well as on the grant programmes for development of orphan medicinal products that are available. These programmes aim to promote the development of new medicines for the treatment of rare diseases.

Programme

08:00 **Registration**

Morning session

09:00 **(1.a) Introductory opening**

Guido Rasi
Executive Director, European Medicines Agency (EMA)

09:05 **(1.b) Opening remarks**

Bruno Sepodes
Chair, Committee for Orphan Medicinal Products (COMP)

First plenary session: orphan designation around the World

09:15 ***(2.a) Europe – legal basis***
Agnès Mathieu
European Commission (EC)

09:20 ***(2.b) European orphan designation***
Stiina Aarum
European Medicines Agency (EMA)

09:45 ***(3) FDA orphan designation***
James H. Reese
Food and Drug Administration (FDA)

10:15 ***(4) MHLW/PMDA orphan designation***
Yasuko Inokuma
Ministry of Health, Labour and Welfare (MHLW)

10:45 ***Panel discussion***

11:00 **Coffee break**

Second plenary session: orphan designation incentives and grants around the World

(5) Grant frameworks:

11:30 ***(5.1) Situation in Europe and IRDIRC (Global)***
Irene Norstedt
European Commission (EC)

11:45 ***(5.2) Situation in USA***
Erica K. McNeilly
Food and Drug Administration (FDA)

12:00	***(5.3) Situation in Japan*** **Hirofumi Kusunoki** National Institute of Biomedical Innovation (NIBIO)
	(6) Incentives and regulatory considerations:
12:15	***(6.1) Situation in Europe*** **Segundo Mariz** European Medicines Agency (EMA)
12:35	***(6.2) Situation in USA*** **John Milto** Food and Drug Aministration (FDA)
12:55	***(6.3) Situation in Japan*** **Hiroshi Takeda** Pharmaceuticals and Medical Devices Agency (PMDA)
13:15	***Panel discussion***
13:30	**Lunch**

Afternoon session

14:30	**Three Agencies face to face consultations** Separate 40 minutes consultations as requested
16:30	**Break**
17:00	**Three Agencies face to face consultations** Separate 40 minutes consultations as requested
19:00	**End of the Workshop**

Practical information

Wi-Fi

To connect to Wi-Fi please log-in as follows:

Username: wifiguest

Password: Canary2014

Presentations

You will be able to download the presentations from the Agency's website approximately two weeks after the end of the workshop.

Catering

The Agency has a restaurant and a deli bar that offer a variety of food and drinks during the day. They both operate a cashless payment system. No cash or credit/debit cards are accepted.

You will be able to purchase a visitor card outside the meeting room during the coffee break at 11 o'clock and at the entrance of the canteen on 3rd floor at lunch time.

In addition, visitor card terminals are available in the 1st floor reception area and 3rd floor restaurant. The terminals accept both GBP and EUR. The terminals issue a card with the balance of cash received, less a £3 deposit for the card (i.e. if £10 is put into the machine, you will receive a card with £7 that can be spent in the restaurant and deli bar; the £3 will be refunded when the card is returned).

At the end of your visit, simply reinsert the card in one of the visitor card terminals and the deposit plus any account balance will be refunded. Please note that the machine refunds in **GBP** coins only. For this reason, we encourage you not to load it with more than £20. If visiting the Agency frequently, visitors may wish to retain the card for future use.

Media disclaimer

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By attending this meeting you consent to any recording or broadcast. The Agency will endeavour to inform attendees whether a specific meeting is being recorded or broadcast.

Visitor's pass

After the Workshop please return your visitor's pass to the ground floor reception.

Workshop secretariat

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