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Directorate

European Union - Japan orphan medicines cooperation

European Union (European Commission / European Medicines Agency) – Japan (Pharmaceuticals and Medical Devices Agency / Ministry of Health, Labour and Welfare) pilot terms of reference

1. Background

It is indispensable for the patient suffering from orphan diseases to have access to medicinal products for their diseases. However, the limited number of patients of orphan diseases hinders companies from research and development in such area, which creates the situation where active involvements by authorities are strongly required. Also, measures taken by authorities at post-market phase are especially important, considering the limited amount of safety information at the time of approval. EC/EMA and MHLW/PMDA has been implemented various measures to promote orphan medicines development. Under the confidentiality arrangements between the EC/EMA and MHLW/PMDA in the field of pharmaceutical affairs (herein after "Confidentiality arrangements"), exchanging experience and information would lead to improvement of measures taken by each authority in timely manner, as well as accumulation of supplement data, which would enable the balance between risk and benefit about orphan medicines to be evaluated in the comprehensive way.

2. Objectives (Aim)

To create an operational framework focused on a series of activities listed below:

- Creation of greater awareness of mutual orphan medicine designations submission process.
- Development of a system of exchange regarding the outcomes of orphan medicine designations in each Agency/Ministry.
- Development of a system of exchange regarding regulatory and licensing stage concerning orphan medicines.
- Development of a system of exchange regarding pharmacovigilance post-licensing activities associated with Orphan medicines.
- Development of a collaboration mechanism regarding small to medium size enterprises (within the range of confidentiality arrangements).

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3. Operations

All parties should implement an internal mechanism of identification which is considered of mutual high interest. This should be discussed at each authority unit or division in charge of Orphan products at meetings and high interest topics identified and flagged for future discussion in the bilateral quarterly telephone conferences.

Coordination of communication/ collaboration on these identified areas of mutual high interest areas should be performed via a designated contact person.

Initiating consultation and information sharing can be done if necessary via e-mail depending on the importance of the issue identified. For example orphan medicine unexpected events which may affect the licensing or availability of a product may need an urgent information sharing system as opposed to discussions of areas of high interest.

4. Activities

Under this cooperation, all parties will share the information according to the confidentiality arrangements in the areas of interest in the Regulatory process have been identified as outlined below.

This outline is not an exhaustive list and will be added other items on a proposal, when the proposal could be valuable for all parties.

4.1. Bilateral initiative to produce a framework for creating greater awareness of mutual orphan medicine designation submission processes.

- Identification of key documents for translation into Japanese or English.
- Increasing visibility and awareness of the documentation on mutual websites.
- Links via websites to mutual areas of interest.

4.2. Orphan medicine designation submissions stage:

The principle here is to exchange information on discussion of MHLW/PMDA and or COMP summaries following evaluation under the confidential arrangements.

- The name of the product, orphan condition, status of a submission in Europe, Japan or both. EMA to submit list of products processed and completed through the COMP (Committee on Orphan Medical Products) on a monthly basis. MHLW/PMDA to submit list of products processed and completed through the PAFSC (Pharmaceutical Affaires and Food Sanitation Council) on a quarterly basis.
- Exchange of reports or its outline in cases where there has been a divergence of opinion between Agencies/Ministry (conclusions by COMP and PAFSC) dependent on the interest of the topic and its implications for Orphan Designation. One of the key aims is to identify areas of overlap and difference in the process of generating an opinion.
- Information exchange of situations regarding to research and development stage where necessary.

4.3. Regulatory Licensing stage:

The principle here is to exchange information under the confidential arrangements.

- Information exchange regarding protocol assistance / scientific advices and outline of the discussion of authorization or refusal conclusion by PAFSC and COMP as well as review report, on a case by case basis
- Information exchange regarding those products where there are special considerations such as manufacturing issues supply and distribution
- Information exchange regarding final authorisation of the orphan medicinal product
- Special efficacy and safety data considerations on a case by case basis as is current practice between the FDA and EMA.

4.4. Post-Licensing/Pharmacovigilance stage:

The principle here is to exchange information under the confidential arrangements.

- Exchange of information regarding Pharmacovigilance and Risk Management Plans for Orphan Products on a case by case basis where there are serious expected adverse events which need to be monitored because of their added risk.
- Exchange of information regarding Serious Unexpected Adverse event exchange for Orphan medicines already on the market.

4.5. Collaboration regarding small to medium sized enterprises:

- Exchange of information regarding mechanisms in place to support small and medium sized enterprises to understand the regulatory environment concerning Orphan medicines within the responsibility of confidential arrangements.

5. Membership

Membership under this operation should be four parties, i.e., EMA, MHLW, PMDA and the European Commission (DG SANCO).

6. Meetings

Regular telephone conferences can be done on a Quarterly basis, at March, June, September, and December. A date of telephone conference is to be determined by the end of previous month of the month of the telephone conference.

Other than regular telephone conferences, the members will meet up according to necessity.