Patient Advocates Capacity Building Workshops

EURORDIS Membership Meeting 2012
Brussels, 23 May 2012

6. Compassionate use programmes for orphan drugs (Timeline: 14-17:30)

- ***Moderator: Michele Lipucci Di Paola (Member Committee Advanced Therapies/EMA, Vice-President ,Italian Association for Thalassemia, Italy)***

- **- How do we define such programmes and why are they needed? François Houÿez, Eurordis, FR**

- **- Compassionate use programmes in France: the A.T.U legislation and its impact on orphan drug**
  Chantal Belorgey, Afsssap, FR

- **- Recent attempt to create a compassionate use in Romania,**
  Etelka Czondi, Romanian Prader Willi Association

- **- Survey on recent compassionate use programmes for orphan drugs. François Houÿez, Eurordis, FR**

- **- Conclusions of the ERTC workshop on compassionate use (21/11/2011) and proposals to move forward. Arielle North, FR**

- **Workshop exercises:**
- **- 3 to 4 groups, each group receives a short text proposing to organise a compassionate use for a**
- **product for a given indication, and to think about all the aspects of the implementation.**

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"Compassionate use" Art. 83 n.2 of the Regulation no. 726/2004 of the European Parliament and of Council

DEFINITION: as "… making *a medicinal product* belonging to the categories referred to in Article 3 (1) and (2) *available for compassionate reasons to a group of patients with a chronically or seriously debilitating disease or whose disease is considered to be life-threatening,and who cannot be treated satisfactorily by an authorized medicinal product.* The medicinal product concerned must either be the subject of an application for a marketing authorization in accordance with Article 6 of this Regulation or must be undergoing clinical trials."

- ***National compassionate use programmes*, making medicinal products *available either on a named patient basis or to cohorts of patients*, are governed by individual Member States (MS) legislation.**

- ***Compassionate use implementation remains the competence of a MS***

- **Article 83 of Regulation (EC) No 726/2004 on compassionate use is complementary to national legislations and provides an option to MS who wish to receive a CHMP opinion *regarding the conditions* for compassionate use *of a specific medicinal product which falls within the scope of Article 83(1) and 83(2).***

- **Recital 33 of Regulation (EC) No 726/20042 states that *"In order to meet, in particular, the legitimate expectations of patients (…) a common approach should be followed, whenever possible, regarding the criteria and conditions for the compassionate use of new medicinal products under MS' legislation"***

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"Compassionate use"
Questions

- ***When do you suggest to start a compassionate use programme ???***

- ***Who have to initiate the compassionate use programme ???***

- ***How do you suggest to run the compassionate use programme ???***