



**15<sup>th</sup> Workshop of the Eurordis Round Table of Companies (ERTC)**

***“Compassionate Access to Rare Disease Therapies”***

***Monday 21 November 2011  
Paris, France***

**Les Salons de l’Aéro-Club de France  
6, rue Galilée – 75116 Paris**

***Concept Paper***

In terms of access to a medicine, compassion applies to situations where a patient or his/her doctor knows that a new medicine might soon become available, but that its development, or the evaluation process, is not completed yet. Therefore, the patient’s health condition will seriously deteriorate before the medicine becomes authorised and available.

Typically, a patient with metastatic lung cancer having failed all therapeutic options and with a very bad prognosis cannot wait for the 180-day duration of the evaluation process for the marketing evaluation: it may be too late for the patient to wait until the product receives an approval. Compassion starts when society realises that there is nothing more distressing for a patient than dying with the knowledge that a new medicine is nearly there, but is not immediately available due to regulatory requirements.

**Compassionate use as envisaged by European legislation**

As defined in the EU REGULATION (EC) N° 726/2004 Title V, article 83.2, running a Compassionate Use Programme (CUP) consists in making a medicinal product 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 authorised medicinal product.

The legislation only addresses the case of groups of patients, when they can be defined, as opposed to nominative requests exclusively dealt with at national level. The severity of the disease, (chronically or seriously debilitating), or its life-threatening nature, needs to be justified, based on objective and quantifiable medical or epidemiologic data.

“Patients who cannot be treated satisfactorily”, according to Article 83 (2), means patients left without treatment options or patients whose disease does not respond, or who suffer a relapse with the treatments available, or for whom the treatments are contraindicated or inadequate. Whether patients can be treated satisfactorily or not, will be assessed by the CHMP/EMA who provides an opinion based on the review of diagnostic, preventive or therapeutic medicinal products authorised, and on the account that the medicinal products reviewed are not considered satisfactory for the treatment of the patients’ disease. This European centralised assessment procedure for compassionate use programmes is only triggered upon request of one, or more, Member States.

### **Compassionate use programmes in Europe**

As a result of the different implementations of the Directive 2001/83/EC, different schemes exist for compassionate use going from cohort studies to named patient basis programmes. Depending on the country, the costs of this pre-marketing access is covered by the Member State or by the sponsor. Finally, in some countries there is still no specific legislation addressing the needs of patients with life-threatening diseases.

Thus, while the intention of the legislator was to allow patients affected by serious and life-threatening diseases to have a last chance to test the efficacy of treatments still under development, in reality the lack of harmonisation following the implementation of the directive has resulted in an unequal access of European patients to potentially effective and life-saving treatments. This situation of inequality is even more acute in the case of rare diseases.

### **The 15<sup>th</sup> ERTC Workshop**

This 15<sup>th</sup> ERTC workshop aims to analyse how compassionate use programmes in Europe address the urgent needs of rare disease patients. In particular the participants will be invited to discuss:

- the achievements of the present legislations on Compassionate Use in Europe over the last years,



- the actions to integrate Compassionate Use Programmes into an improved orphan drug development model that would better serve the urgent health needs of rare disease patients while taking into account the limited amount of data available on the medicinal product under development.

The speakers of the morning session will present the recent experiences with Compassionate Use:

- current practices across Europe,
- experience at the European Medicines Agency's (EMA) level,
- how Compassionate Use Programmes are inserted in the general development of an orphan drug, and finally,
- experience of those companies who have engaged in Compassionate Use Programmes for their orphan medicinal products, now authorised in Europe. In particular, through a recent survey targeting orphan drug sponsors, EURORDIS has also collected comments and suggestions for improvement of the present Compassionate Use systems.

The afternoon session of the meeting will be fully dedicated to a discussion between the audience and the members of the panel on the specific points circulated before the meeting.

The general aim of this discussion is to explore how Compassionate Use for rare diseases could answer urgent public health needs while contributing to the development of orphan medicinal products in small and heterogeneous patient populations.