



16th Workshop
Eurordis Round Table of Companies (ERTC)

***The value of partnering in RD therapies:
Benefits of working with patients along the treatment lifecycle in the areas of clinical trials,
regulatory affairs and Health Technology Assessment***

Thursday 1st March 2012
Brussels, Belgium

Concept paper

Today building upon experience acquired and some advances made, EURORDIS focuses on expanding the areas where collaboration already exists and must be further encouraged.

Patients' representatives already participate in decision-making processes at the European Medicines Agency and work closely with some national regulatory agencies. In addition, Regulators and HTA experts have recently started to harmonise their respective methodologies in order to avoid redundancy and inconsistencies in their respective opinions. However, systematic involvement of patients with HTA agencies is only beginning.

In 2005, EURORDIS held an ERTC workshop inviting all stakeholders to reflect on the need to define a common framework for collaboration between patients' associations and sponsors in the context of clinical trials. The purpose of that workshop was to clarify the expertise and respective roles of both patients and sponsors as well as to address some of the associated reticence and fears. The conclusions were that early dialogue, commitment and transparency were important to ensure fruitful collaboration and success. Since then, pilots have been conducted and states of mind have matured.

More than in any other health domains, rare disease patients, regulators, sponsors and -now hopefully- HTA agencies share the same expectations: the rapid collection of quality data, the development of effective and safe treatments and the rapid access for patients to these treatments including reimbursements.

The first part of the morning session of the 16th Eurordis Round Table of Companies (ERTC) Workshop on “*The value of partnering in RD therapies: Benefits of working with patients along the treatment lifecycle in the areas of clinical trials, regulatory affairs and Health Technology Assessment*”, will be dedicated to the experience of a patients’ group involved in the elaboration of clinical trials protocols with industry and conversely the experience of the industry member of working with the patients’ groups will be presented. Two presentations relating to collaborations of patient groups and clinical trial sponsors in the fields of HIV/AIDS and rare cancers will follow. The session will be completed with the regulatory perspective with respect to Scientific Advice and Protocol Assistance and the contribution of patients to this important process.

The second part of the morning will begin with the perspective of the European Medicines Agency (EMA) on the involvement of patients in regulatory processes and will emphasise the importance of these roles as documented in:

- EMA Reflection paper on the Further involvement of Patients and Consumers in Agency’s activities
- Role of Patients as members of the EMA Human Scientific Committees
- Procedure for Review of Information on medicinal products by patients’ and consumers’ organisations.

Two presentations by patients’ representatives at EMA, the first in the Committee for Orphan Medicinal Products (COMP) describing the role and activities of patients in a scientific committee followed by new activities planned with respect to pharmaco-vigilance, risk assessment and patient-reporting of adverse drug reactions.

In order to be prepared to be involved in these important processes, patients’ advocates need to be informed regarding the terminology, the sequence of events and the areas where they can act. EURORDIS - in conjunction with INSERM, the French public research body- began pilot training sessions for patients on “How to Read a Clinical Trial Protocol” in 2004 and 2005. In 2008, EURORDIS extended this training via the EURORDIS Summer School for rare disease patients’ advocates – in conjunction with EMA, University Autonomous Barcelona, DIA - to include the drug development process, with experts invited to explain clinical trials including ethics, statistics, issues of applying these practises to small populations such as rare diseases and children. The training also includes a description of the scientific committees at the European Medicines Agency and the demonstration of the role of patients’ representatives on these committees. To take this initiative to a higher level, an on-line learning tool has been developed that is available on the EURORDIS web site.

In 2012, a new initiative was launched, the European Patients' Academy on Therapeutic Innovation (EUPATI) an IMI-funded patient-led project involving 29 partners including industry members, academics and patients' associations. This training will be extended to all European patients and aims to raise awareness and interest in clinical trials and the complexity of developing new treatments and patients' access to them. The material will be targeted to patients, patients' organisations and lay people and will be in at least 6 languages. Courses will be developed to communicate, teach and inform about therapeutic innovation and research and development in medicines. The importance of patients being well-informed and well-prepared will be outlined during the workshop.

The workshop will conclude with the afternoon session focused on collaboration with all stakeholders in health technology assessment processes (HTA). From the patient/consumer viewpoint, collaboration in HTA at the EU level can contribute to the reduction of inequalities in access to treatment between and within member states as it encourages a common approach to HTA decisions. Both initiatives EUnetHTA and HTAi incorporate the different stakeholder viewpoints into their mission.

From the regulatory viewpoint, EMA is progressively developing its collaboration with HTA into early scientific advice and protocol assistance procedures. In this session the perspectives of an HTA agency working with patients and a patient working with an HTA agency are presented. The final session of the workshop is the relevance of the current HTA practises to rare diseases.

The upcoming ERTC workshop seeks to encourage dialogue between all interested parties, demonstrate the value of collaboration and illustrate the important role that patients already play in the drug development process from clinical trials, regulatory affairs and HTA.