

3rd Workshop

Eurordis Round Table of Companies

*“Rare Disease Clinical Trials:
Ensuring Fruitful Collaborations between Sponsors & Patient Organisations”*

November 21st 2005- Barcelona, Spain

CONCEPT PAPER

Eurordis priorities are to create a strong rare disease patient movement across Europe with an effective voice to advocate in strategic European policy areas. Because Eurordis recognises that little can be achieved in isolation, most of its efforts in recent years have been geared at building a European rare disease community, inclusive of all interested parties, as well as developing action programmes which can potentially help improve the quality of life of people living with rare diseases. To that purpose, Eurordis encourages and supports strong synergies between all interested parties in the field of rare diseases.

More than in any other health domains, patients, researchers, physicians and sponsors concerned by rare diseases share the same expectations: the rapid acquisition of quality scientific data and the development of effective and safe treatments.

In this context, Eurordis has launched a reflection process with a number of patient associations involved in clinical trials, initially with the Alliance Maladies Rares, (French National Alliance for Rare Diseases) and through a series of meetings with clinical researchers, pharmaceutical companies, biotechs, legal and ethical advisors, health quality and evaluation experts. This process highlighted the pressing need to define a common framework for collaboration between associations and sponsors, and resulted in the preparation of the “Draft Charter for Clinical Trials in Rare Diseases”. This document, which is currently under consultation, has been developed by Eurordis in order to bring together the efforts and expertise of both patients and sponsors, and to establish and clarify their mutual roles in the context of a clinical trial.

The 3rd Eurordis Round Table of Companies (ERTC) workshop to be held on November 21st 2005 in Barcelona on “Rare Disease Clinical Trials: Ensuring Fruitful Collaborations between Sponsors and Patient Groups”, will be the opportunity for an in-depth discussion on this new initiative. This workshop will be an essential step in a period of long-term consultation.

The expectations and fears that such an innovative experience will raise, at least at the beginning, will of course, be addressed at this meeting, as well as all possible implications for the work of the national and European regulators.

Patient groups and clinical trial sponsors have, on occasions, fruitfully collaborated, for example in the fields of HIV/AIDS, cancers and rare genetic diseases, providing real lessons on how to improve such relationships. This valuable knowledge will be shared during the workshop and will benefit the rare diseases community.

Due to their unique knowledge of the disease, rare disease patient associations are clearly the legitimate partners of clinical trial sponsors, and as all stakeholders, they have an active part to play in clinical trials.

Eurordis and its members are fully aware that a concrete and trustful collaboration can only be achieved when interested parties voluntarily commit themselves in a transparent manner, on the sound basis that there are mutual benefits at stake for each party.

Already, rare disease patient associations are preparing themselves for this new and important commitment: Eurordis and Inserm have developed a pilot training session for patients on “How to Read a Clinical Trial Protocol”. Ten pilot training sessions were held in 2004 for 97 patient representatives in France and three in 2005 for 66 patient representatives, as well as one in Spain in collaboration with the Fundació Doctor Robert. More training sessions will be organised in other European countries. The importance of patients being well-informed and well-prepared will be outlined during the workshop.

Sponsors are aware of the expertise of patient associations, calling on them to facilitate the participation of patients in clinical trials and, on occasions, consulting them in order to validate the documents designed for clinical trial participants. Unfortunately, this collaboration often starts too late, during the period of marketing, and is often limited to patient recruitment rather than other important aspects, sometimes taking place only after the emergence of problems that may jeopardise the continuation of the clinical trial (e.g. when unexpected and serious events occur).

The upcoming November ERTC workshop seeks to encourage dialogue between all interested parties concerned by rare disease clinical trials, and will establish the basis of a unique collaboration in a domain that has never been, and is unlikely to ever be, regulated by any national or super-national legislation, while respecting the present regulations on clinical development (EU Clinical Trial Directive 2001/20/EC for example) and the role of parties such as national agencies and ethical committees.