



23rd Workshop

EURORDIS Round Table of Companies (ERTC)

“Patient Relevant Outcome Measures & Patient Reported Outcomes”

29 September, 2015 (9:00 to 16:00) - UAB - Casa Convalescència, Barcelona, Spain

CONCEPT PAPER

The focus of the 23rd Workshop of the EURORDIS Round Table of Companies is on Patient Relevant/Centered Outcome Measures (PROMs) and Patient Reported Outcomes (PROs).

Patient-centeredness in clinical research is a concept that is now leading the way (Patient-Centeredness in the Design of Clinical Trials VALUE IN HEALTH 17 (2014) 471 – 475).

Patient-centeredness means that the clinical studies are designed to be the most relevant to patients, the most adequate to generate proofs showing that a therapy will improve their lives. The more appropriate the chosen endpoints and outcomes are; the least time and efforts are wasted in not enough-conclusive trials. The more useful and right to the point the clinical trials are, the better the chance for therapeutic development to be successful.

Patient-centeredness means also that patients are not subjects in clinical research anymore, but active participants, and even full partners in some cases. Patients' expertise and knowledge about their disease has to be taken into account with regards to the design of a trial, to the assessment of the benefit-risk of their outcomes and to value assessment

It is essential to collect robust data along the life cycle of a product development, through a good and standardised methodology allowing the generation of tangible and appropriate outcomes. Patient-Reported Outcomes are one way of obtaining such results. Those are measurements based on data provided by patients (self-report or interview) regarding their health condition without amendment or interpretation of the patient's response by a clinician or anyone else. Such as the other types of Patient Relevant Outcomes Measures, they have to be convincing to satisfy the requirements of both regulators and HTA during the assessment of a product.



Why this workshop, now? – When everyone knows about the concepts, when everyone is conscious of what are the needs, why do we need to talk about it?

A lot has already been done, several initiatives are looking into Patient Relevant/Reported Outcome Measures, regulators (FDA and EMA) are providing guidance documents, pharmaceutical companies and patient organisations are looking into it and developing some of them. However, appropriate and validated outcome measures of disease activity, or disease progression, still do not exist for the vast majority of rare diseases, even diseases for which medicines are already approved, or for which therapies are under development.

This ERTC workshop is meant to be a forum of discussion between all the stakeholders represented so as to reach a common understanding of PROs and PROMs. It is also timely aligned with the work done within the IRDiRC Consortium (www.irdirc.org). One of the three Scientific Committees of IRDiRC, on Therapies, has issued recommendations highlighting the importance of Patient Relevant Outcomes Measures and Patient Reported Outcomes. These recommendations have led to the creation of a Task Force gathering top experts to consider what has to be done specifically in the field of rare diseases.

The morning session will give us an overview of the State-of-the-Art of Patient Relevant/Reported Outcomes with the participation of different stakeholders and using different formats (presentations, interviews and case studies), while the afternoon aims to be a constructive discussion leading to better understanding and common approaches.

One of the major initiatives contributing to the PROMs field is named PCORI - Patient-Centered Outcomes Research Institute (www.pcori.org). It is a validated and recognised approach on how to develop PROMS. PCORI's mandate is to improve the quality and relevance of evidence available to help patients, caregivers, clinicians, employers, insurers, and policy makers make informed health decisions. Specifically, they fund comparative clinical effectiveness research, or CER, as well as support work that will improve the methods used to conduct such studies.

Another very interesting initiative, COMET – Core Outcome Measures in Effectiveness Trials (www.comet-initiative.org) brings together researchers focused on developing and applying validated and standardised sets of outcomes. These 'core outcome sets' represent the minimum criteria to be measured and reported in clinical trials and other forms of research on a specific condition. The existence or use of a core outcome set does not imply that outcomes in a particular trial should be restricted to those in the relevant core outcome set. Rather, there is an expectation that the core outcomes will be collected and reported, making it easier for the results of trials to be compared, contrasted and combined as appropriate; while researchers continue to explore other outcomes as well. COMET aims to collate and stimulate relevant resources, both applied and methodological, to facilitate exchange of ideas and information, and to foster methodological research in this area.

Mapi (mapigroup.com) is a private company taking a phased approach to Patient-Centered Outcomes research and who will share with us its experience so far.



Keywords in this area are validation, qualification and standardisation. Developing standardised core sets of patient-centered outcomes for different conditions would improve the quality of clinical studies and increase homogeneity between clinical trials. Additionally, in order to meet regulatory approval, early discussions should be conducted with the regulatory agencies to ensure that appropriate PRO measures are applied and compatible with regulatory standards.

Through the two interviews, we will see which approach EMA has taken with the PROMs and what is the experience of the Agency until now, but also besides the qualification of the new methodologies, what type of data is required by regulators when assessing a dossier.

Finally, through the three case studies, we will have concrete examples of how to integrate PROs in the clinical development from a developer's point of view, as well as patient perspectives and initiatives in two medical areas, Duchenne Muscular Dystrophy and Cystic Fibrosis.

In the end, all these initiatives could expand to rare diseases and benefit a maximum of patients, with guidances and standards to be adopted by all stakeholders involved, regulators, developers, payers, etc. That is why we have invited the participants to prepare questions ahead of the workshop, in particular three principal benefits and barriers of PROs they are faced with in their activities.