



28th Workshop of the EURORDIS Round Table of Companies (ERTC)

A New PARADIGM of Meaningful Patient Engagement in the Life Cycle of Medicines

Tuesday, 15 October 2019 (09:00 to 17:00)
Recinte Modernista – Barcelona – Spain

CONCEPT PAPER

Introduction

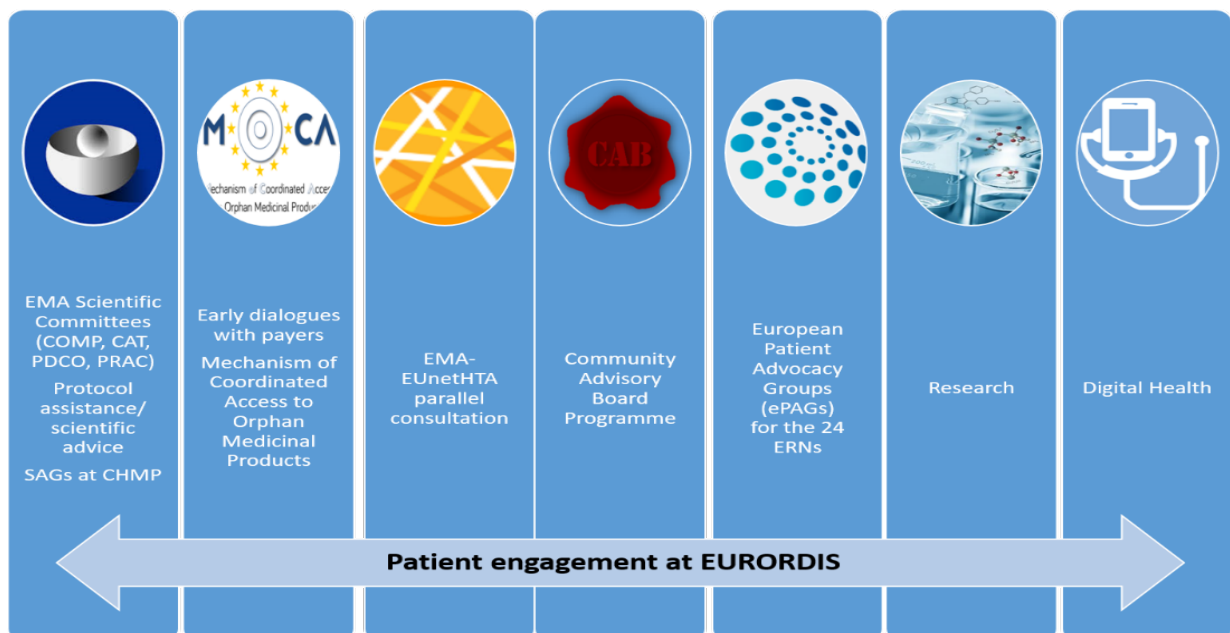
The patient voice has always been instrumental in advancing medical research and fighting access to treatment inequalities. In recent years, patient engagement in medicines R&D has been moving from tokenism to being consolidated as a key factor for successful product innovation within the medicine life-cycle.

The challenges faced by rare disease patients in terms of lack of research interest in their particular therapeutic area and the few or no treatments available are a trigger for the community to reach out to all stakeholders and be part of the decision-making. “Learning by doing” has helped both patients and developers (but also other relevant stakeholders, i.e. regulators, HTA bodies, payers, health care professionals) to acquire much needed capabilities through the process of engagement. For quite a few years, patient expert training programmes, such as the EURORDIS Summer School or EUPATI, have equipped patients with skills not only to engage and advocate for the development of and timely access to rare disease therapies, but also to lead their own projects in a diversity of areas from applying artificial intelligence to diagnosis and treatment to identifying new patient-reported outcomes and organising real-world data collection.

Global institutions such as the Patient-Centered Outcomes Research Institute (PCORI) or the International Rare Diseases Research Consortium (IRDIRC), have provided a framework and standards on how patient-centred outcomes research should be performed. The need for operational tools to perform patient engagement prompted the Patient-Focused Medicines Development (PFMD) consortium to develop the patient engagement quality guidance, a tool to guide the planning and monitoring of patient engagement activities. PFMD is also supporting projects to develop a fair market value for compensation of patients and a series of “reasonable agreements” between patients and the pharmaceutical industry, covering a void in patient engagement practices. Also, the Council for International Organizations of Medical Sciences’ (CIOMS) working group on patient involvement will develop pragmatic Points to Consider when engaging patients throughout the medicines life-cycle. The morning session of the present ERTC workshop will go over the most recent developments about these important topics.

Patient engagement at EURORDIS

The EURORDIS mission of improving the lives of people living with a rare disease is sustained by three pillars: advocacy, empowerment and engagement. Over the past few years, EURORDIS has set up a framework organised around the role of the Patient Engagement Manager (PEM). While the organisation as a whole is committed to patient engagement, its operationalisation is centralised in specialized roles covering engagement with the European Medicines Agency (engagement of patients in protocol assistance, scientific committees, scientific advisory groups), payers (the MoCA initiative), the health care industry (through the EURO-CAB programme), the European Reference Networks (through the European Patient Advocacy Groups), scientific research and digital health.



Thus, the PEM is the central contact point for both patients and the relevant stakeholder with regards to patient engagement in each of these fora. PEMs have a dual mission: 1) reaching out internally within their own organisations (or their constituency) and accompanying the patient along the journey of engagement and 2) liaising externally with the relevant stakeholder to optimize and ensure the right conditions for the engagement. This framework allows an iterative improvement in the process of engagement between patients and all other stakeholders.



IMI-PARADIGM

EURORDIS' role in IMI-PARADIGM has led the development of important deliverables such as recommendations to help stakeholders and their organisations identify the required capabilities for patient engagement. These recommendations aim at enhancing system readiness across stakeholder organisations and ease the path towards operationalizing meaningful engagement.

In addition, EURORDIS is involved in the co-development of a monitoring and evaluation (M&E) framework to evaluate patient engagement practices and towards demonstrating the return on engagement and the value that patient engagement brings to product development for developers, patients and society. This M&E framework is intended to be used by all stakeholders who can adapt it to their own specific needs. The EURORDIS EURO-CAB programme is serving as a case study to develop suitable metrics indicators for both patients and industry in a Community Advisory Board (CAB) context. Two breakout sessions will focus on how to apply the right metrics to CABs as well as to other patient engagement activities co-developed by and co-managed with the healthcare industry.

Among other co-designed resources for patient engagement, PARADIGM is working on guidance to raise awareness about the management of competing interests. Medicines R&D is a complex multi-stakeholder environment in which competing interests inevitably arise. The rare disease arena is particularly challenging as a reduced number of expert patients are recurrently involved in simultaneous activities with different stakeholders. While completely avoiding competing interests is close to impossible, their right identification and management will ensure that meaningful engagement still happens. A dedicated breakout session will delve into how best to identify and manage such conflicts.

PARADIGM's mission is to provide a unique framework that enables structured, effective, meaningful, ethical, innovative, and sustainable patient engagement and to demonstrate the 'return on the engagement' for all players for three key decision-making points: research priority setting, design of clinical trials and early dialogue. This workshop is especially important in raising the voice of highly vulnerable populations, like those with rare diseases, highlighting the background and specificities inherent to these conditions.