EURORDIS
ROUND TABLE OF COMPANIES

20th Workshop

EURORDIS Round Table of Companies (ERTC)

A 10 Year Anniversary Workshop!

Unlocking Europe's Potential in Rare Disease Therapies

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CONCEPT PAPER

The 2008 *Commission Communication Rare Diseases: Europe's Challenges*, the 2009 *Council Recommendation on an action in the field of rare diseases*, and the 2011 *Directive on Patients' Rights in Cross-border Healthcare* complement the EU Regulation on Orphan Medicinal Products (EC) No. 141/2000, establishing a comprehensive and integrated EU strategy for rare disease research, diagnostics, treatment and care.

2014 is a turning point for the next decade.

After 15 years of actions promoting rare diseases as a public health and research priority, 2014 opens a new phase for actions on rare diseases in Europe: a more structured, systematic approach with a few clear common objectives optimising synergies and outcomes and ensuring a long-term impact.

National plans for rare diseases will be consolidated and increasingly integrated into common European approaches. European Reference Networks for rare diseases should progressively bring together Centres of Expertise and national experts as well as diagnostic laboratories; all sharing common missions of data collection, creation and maintenance of registries, best practices for diagnosis and care, telemedicine, and clinical trials. This vision is clear and outlined in the respective EUCERD Recommendations.

The challenge lies in ensuring that this vision is well-conceived and properly funded in ongoing calls, in order to ensure that European Research Infrastructures on Rare Diseases will be integrated and geared toward development of diagnostics and/or therapies. This will permit a comprehensive and sustainable leading European platform of world-class potential to be developed.

While conceived as information systems to manage patients' data, European Reference Networks and European Research Infrastructures provide additional opportunities to translate research into innovative treatments, and information technology into knowledge generation, ultimately leading to better patient care.

- EU Programmes for 2014-2020 *Health for Growth* and *Horizon 2020* are expected to support actions to achieve these objectives.
- Innovative Medicines Initiatives (IMI 2) – will increase its multiyear budget and duration and recognises that rare diseases are a strategic area for more pre-competitive collaboration and public-private partnership, aiming for progressive patient access to medicines.
- International Rare Disease Research Consortium (IRDiRC) – aims at developing diagnostic tools for most rare diseases and 200 new therapies for rare diseases by the year 2020. The consortium and its widely shared aims provide an opportunity to synergise the potential of main public and private funders around the word through common policies.

In this context, a few stakeholders including some Member States' payers, niche pharmaceutical companies, and patient groups have criticised the EU Regulation on Orphan Medicinal Products. They claim that the Regulation is responsible for the high prices of some orphan medicines, the market exclusivity incentive blocks entry of innovative products, and that there is a need to fragment ultra-rare from rare diseases. Most, if not all, of these criticisms stem from misconceptions or short-sightedness.

However, there is overall a broad and robust consensus across the majority of patients, industry, academia, Member States, and the European Commission that there is no need to revise the EU EU Regulation on Orphan Medicinal Products. The rare disease and orphan medicine community needs stability, predictability and clarity in order to deliver better outcomes.

Nevertheless, there is a well-recognised need to address issues such as: insufficient scientific and medical knowledge for most rare diseases; insufficient knowledge on natural history and a lack of a wider range of endpoints or patient relevant outcomes; low success rate (or bottleneck) from designation to market authorisation; high R&D costs of rare disease therapies due to lack of innovative design or statistical methods and rigid regulatory pathways; gaps between EU centralised regulatory procedures and national health technology assessments or pricing and reimbursement decisions; poor access to new medicines for patients as well as outstanding delays and discrepancies across Member States resulting in a 'life lottery' for millions of rare disease patients in Europe.

We know how to address these challenges. Innovative solutions have been developed over the last five years through intense multi-stakeholder dialogue, public consultation, mandated experts groups and consensus opinions. These now need to be piloted, supported, and, if positive, expanded.

This 20th Workshop of the EURORDIS Round Table of Companies aims at "Unlocking Europe's Potential in Rare Disease Therapies".

The knowledgeable and experienced chairpersons, speakers and panellists from the European Commission, European Medicines Agency, pharmaceutical industry and patients' representatives participating in this forum will provide an opportunity for stakeholders to discuss:

- the most recent opportunities and developments within the EMA and COMP, IRDiRC, Horizon 2020, IMI 2
- the multi-stakeholder common vision shaped by the industry OMP Task Force in consultation with EURORDIS, the European Commission Expert Group on Rare Diseases (formerly EUCERD) members, EMA, HTA and Payers
- the vision on how the Regulation can be best used to stimulate therapeutic innovation
- the vision on how patients can contribute even more to therapeutic innovation

The three sessions of the workshop will be debated by a panel of five company representatives to engage in interactive discussion with all participants.