EURORDIS
ROUND TABLE OF COMPANIES

22nd Workshop

EURORDIS Round Table of Companies (ERTC)

"Rare Diseases: Going Global!"

Wednesday 25 February, 2015 (9:30 to 16:30) - Le Plaza Hotel Brussels, Belgium

CONCEPT PAPER

The EURORDIS Round Table of Companies is dedicating its first workshop to international strategies and initiatives. This workshop, organised the day after the EURORDIS Policy Event *"Rare but Real:Talking Rare Diseases"* and the EURORDIS *Black Pearl* Gala Dinner celebrating Rare Disease Day, is not about scientific, regulatory or technical aspects, but rather about strategy, trends & environment changes and public affairs.

The objective is to discuss (a) the current strategic initiatives to structure the internationalisation of rare disease research, information and patient advocacy (b) the spearheading projects to increase awareness, promote access to information and facilitate international connection of patients (c) the reality, challenges and ambition of the pharma & biotech companies operating internationally (d) and to increase synergies and mutual support between all stakeholders.

We are living the historic and pioneering time of the globalisation of rare diseases!

When looking at key areas where collaboration between industry and patient groups, together with other stakeholders, can make a major contribution to improving the environment by bringing more, better and faster innovative therapies to patients, international policy and collaboration comes as one of them.

Academic researchers, in rare diseases as in all life sciences, have always collaborated and competed internationally. This is how research, excellence, advancement of sciences work. However, in the past, their action was not supported by a common framework of policy and tools.

Regulatory agencies, FDA first and now also EMA, have played a key role in promoting orphan product regulations or policies in different countries around the word (e.g. Australia, Singapore, South Korea) and new countries are in the process of adopting policies incentivising the development or access to orphan products (e.g. Brazil, Canada, Mexico, Turkey). Both the FDA and EMA collaborate regularly on orphan designations, providing an experience base to foster guidelines, and potentially scientific advice and marketing authorisation in the future. All stakeholders should look at processes which would streamline and accelerate the development and approval of orphan products at an international level.

Pharmaceutical and biotech companies engaged in rare disease therapies have long been pioneering actors both in conducting challenging international clinical trials for a few patients scattered around the world, or in introducing their products to national markets corresponding to the patients' needs. Unfortunately, each company acts in isolation, there is no international pharma & biotech collective voice and few national collective voices, when indeed a lot of collaboration on pre-competitive aspects could take place.

Rare disease patients groups are broadly connected to other patients groups for the same disease (s) elsewhere in the world. Only a few rare disease patient advocates are in regular contact with each other. The vast majority is connected only to their national community with a degree of European networking, therefore little international collaboration takes place. The fact that rare diseases are a challenge to people everywhere in the world has been recognised and mentioned by all rare disease umbrella patient organisations being created over the years but actions have been postponed in order to focus on the national level.

Rare diseases are not yet recognised as an international public health issue, and surely not as a national public health issue in the vast majority of countries. It does not exist yet in the UN System, at WHO or elsewhere.

Times are changing. Structuring strategies help rare diseases to emerge!

Patients are getting together within *Rare Diseases International* to connect the rare disease advocacy movement around the world, speak with one strong, legitimate and credible voice at global level, support the expansion of the movement in all regions, and partner with stakeholders at international level. This is the beginning of a long march, a challenging but necessary one.

EURORDIS is already very much engaged and experienced in international activities, from its coverage of 48 countries across the European continent, to its communication in English, French, German, Italian, Portuguese, Spanish and Russian and its partnerships with major patient groups around the world. It takes an important new initiative such as *Rare Diseases International* to create a new common good with its own governance engaging as equal partners the rare disease organisations from Europe, the USA, Canada, Japan, Russia, Latin America, Australia, China, India, and possibly also Israel, New Zealand, South Africa and Taiwan. We need to act at local, regional and international level. Acting at international level across countries and across rare diseases will make each member stronger locally, or for their own disease.

Whereas *Rare Disease International* is the global organisation for rare diseases, connecting patient groups, *Rare Connect* is the platform to enable individual patients, families and professionals to connect with online global communities for their disease, or around a specific topic. At the core of both initiatives, and at its interface, rare disease patient advocates will increasingly connect and work together globally on a regular base. A Working Group of the EURORDIS' Council of National Alliances organises the public and policy maker awareness annual campaign *Rare Disease Day*. These three initiatives are autonomous to enhance their performance, strategically consistent, already reinforcing each other and will continue to grow.

The International Consortium for Research on Rare Diseases (IRDiRC) initiated in 2010 is half way towards its 2020 objectives, and already sure to reach the targets of 200 new rare disease therapies and diagnostic means for most rare diseases. The coordination of public-funding bodies and private actors based on common policy and strategic actions is essential to make the most of limited resources and scarce knowledge. The consortium generates specific recommendations to funders, promotes priority innovative research and catalyses coordinated actions, identifies platform or tools to be recommended, brings regulators into the research agenda. The consortium is an accelerator of research collaboration and of dialogue between all stakeholders at international level. It is also a flagship to hold on to.

The international classification of rare diseases is another structuring strategy for rare diseases. Being classified and the classification being used, rare disease people become visible in the healthcare systems, more research is feasible, a common language facilitates collaborations. Here too, it is a complex and long-term adventure, but already quite well-advanced with OrphaCode and in ICD 11 and SNOMED. It is a strong vector, a game changer.

Besides its work related to the nomenclature and classification of rare diseases, over the years, Orphanet has developed and enriched its database of information on relevant resources. Since its creation, the Orphanet website has always been widely consulted beyond the national boundaries of EU Member States. Now, the Orphanet Consortium is expanding towards new languages and new territories.

Conferences have an important function in promoting the cause of rare diseases, the current and most innovative messages, the networking between all stakeholders to share a common vision, language and culture. The European Conference on Rare Diseases & Orphan Products is in itself an international event bringing together 500 participants from all over Europe, but also 100 participants from around the world. The US Conference NORD Rare Diseases and Orphan Drug Breakthrough Summit also involves some international speakers. The World Orphan Drug Congress organised in the USA, Europe and now Singapore reinforces awareness and networking primarily among the industry. The International Conference on Orphan Drugs & Rare Diseases (ICORD) puts the spotlight in different parts of the world such as Japan, Latin America / Argentina and Central America / Mexico.

Towards a concerted strategic approach?

Why go global now? What are the drivers, opportunities and challenges? Where could these initiatives take us by 2020?

How is industry embracing and leading the globalisation? What are the risks and opportunities for a company? What is needed for a better environment for rare disease therapy development? What could the game changers of tomorrow be?