

25th Workshop of the EURORDIS Round Table of Companies (ERTC)

“Healthcare Companies & European Reference Networks: Expectations & Potential for Collaboration”

Tuesday 26 September, 2017 (09:00 to 17:00)
UAB - Casa Convalescència, Barcelona, Spain

CONCEPT PAPER

Introduction

The rare disease (RD) community is experiencing a paradigm shift in how we work together, due to the emergence and establishment of a **new central European infrastructure: European Reference Networks (ERNs)**. This new infrastructure revolutionise rare disease patient access to high quality care, through connecting patients, experts and hospitals across Europe. This will break the isolation of patients, foster collaboration among experts and researchers, and help reduce the current inequality in care, driving forward evidence based treatment and accelerating the development of innovation, research and therapies.

The successful development and recent launch of 24 ERNs in March 2017, has been the result of the growing groundswell of collaboration between Member States, connecting hospitals and creating a critical mass of patients, clinicians and researchers, not in their individual parts but as a newly completed whole.

European Reference Networks will pave the way for faster diagnosis and access to expert advice, diagnosis and treatment being spearheaded by the collective experience, expertise and knowledge of leading clinicians and researchers of over 900 expert centres, located in 300 hospitals, under 24 thematic grouped networks. ERNs are anchored in national healthcare systems and connect healthcare and research across 26 Member States.

This new infrastructure will hold most, if not all, of the caseload of rare disease patients, and will be supported by a common IT Platform. These networks will revolutionise the way we work, across the spectrum of stakeholders and throughout the lifespan of research and healthcare pathways. The platform offers a unique opportunity to support the linkage, sharing, storage and reuse of patient data between ERNs, with local hospitals and research groups.

As European Reference Networks make their first steps forward to implement their vision and formalise their networks, it is essential that all partners working in the RD field and specialised healthcare communities are engaged early, to share their hopes, expectations and experiences in our respective areas of expertise. Time is now to identify opportunities to harness our collective capacities and align processes to improve the quality of care, treatment and therapies for the 30 million people living with a rare disease in Europe.

EURORDIS role

EURORDIS, as an organisation focused on supporting people living with a rare disease, has played a key role in the conception of the idea of European Reference Networks, since 2006, first through its own network of members; then within the EU High Level Group on Healthcare Services; through its advocacy for the Directive on Cross Border Health Care with special articles on ERNs as well as on rare diseases; within the Rare Disease Task Force and later in the EUCERD or the CEG-RD with the support of its Joint Action; more recently as leading partner in the Consortium PACE-ERN tendered by the Commission and currently as a RD-ACTION partner. It is of utmost importance to the community EURORDIS represents that the establishment of ERNs in 2017 meets the needs of people living with a rare disease.

Rationale

The ERTC workshop builds on the collaborative spirit that successfully saw the launch of 24 ERNs earlier this year to give early input to help guide the paradigm shift that ERNs have set in motion, so we can unlock their full potential for the RD community.

With the establishment of this new ERN infrastructure, which will be central to all future RD activities and projects, the RD community needs to come together to map out the interfaces ERNs will have with all stakeholders.

In the context of this ERTC workshop, it is of particular relevance to look at how ERNs can contribute to the therapeutic development and the regulatory processes and what should be taken into consideration to ensure an optimal collaboration to generate new quality knowledge.

Workshop Aim

To share information and develop industry's understanding on how these networks can be of value across the research and healthcare pathway. To gather the needs and perspectives of ERTC member companies, regulators, competent authorities and patient advocates on the needs and perspectives of industry with its stakeholders, in order to share this with ERNs and the Board of Member States (BoMSs) in their early planning phase.

The workshop output will be an Industry-perspectives **Points to Consider / list of questions and key issues which** outlines an Industry-led 'wish list' on future collaboration and interaction with ERNs, specifically identifying the potential interfaces with ERNs and reflecting the needs and opportunities ERNs can have for all.

Delegated Decision, Operational Criteria and BoMS Guidance

The EC Delegated Decision and Implementation Decision form the legal based for European Reference Network to be established. This legalisation sets out the form and function each ERN is required to meet in detailed criteria, which have been further detailed in operational criteria for networks and their members.

European Reference Networks are required to engage with healthcare companies for a number of direct and indirect activities – please see ERN research and healthcare pathways in appendix 1, that maps these activities and potential interfaces with wider stakeholders.

The BoMS recognises the importance of healthcare companies in the development of clinical tools and therapies. It has highlighted the need for engagement between ERN members and industry, specifically in clinical trials and research projects. In its formal [Statement](#), the BoMS underlines the importance for ERNs to have independent governance structures as there is no legal provision for the involvement of external stakeholders in their operational delivery. It is necessary to generate operational procedures; furthermore, the BoMS Statement recommended that ‘a complete transparency policy should apply to the relationship between ERNs and industry’. A document of this kind (described as a ‘Code of Conduct’ or similar is presently being developed by the new Working Group under the ERN Coordinators Group.

The EC and BoMS have expressed that it would be of value to share the multi-stakeholder opinion on potential collaboration to inform the initial stage of development of ERNs.

Questions Outstanding:

- How do we want to shape collaboration between ERNs and Healthcare companies?
- What are the core common activities and interfaces between ERNs and Healthcare companies?
- What are the common requirements for multisystem data sharing across the research pipeline through to delivery of care?
- How can we maximize potential gains for all stakeholders?
- What are the big opportunities to drive forward quality of treatment and ultimately finding cures and therapies for all rare diseases?

Why an ERTC workshop now?

The European Commission has formed Working Groups (WG) composed of ERN clinical leads, reporting to the BoMSs. These WGs are expected to lead discussions on the development of specific topics, including:

- IT and data sharing
- Research
- Knowledge generation: Training, education, capacity building, guidelines
- Monitoring, assessment & quality improvement
- Cross-border healthcare, continuity of the ERN and communication
- Ethics & legal issues & relations with stakeholders (data protection, conflict of interest, informed consent)

These WGs are new and have only recently commenced operations; however, it is necessary to anticipate a ‘roadmap’ towards the adoption of a Code of Conduct to govern interactions between ERNs and Industry, to enable all stakeholders to benefit from this new opportunity.

Although the ERN coordinators have not yet had an opportunity to agree a joint position on hopes and expectations regarding collaboration with industry, it is an opportune moment to ascertain the hopes and expectations of healthcare companies and the biopharmaceutical industry, which can foreseeably enrich the next stage of stakeholder discussions (in particular the activities of the new WG on Ethics et. al. and a future RD-ACTION workshop).

The ERTC workshop is thus a timely opportunity to reflect on the needs of the healthcare industry, together with patients, competent authorities and clinicians, to identify critical questions and reflections around the activities that will involve healthcare companies and all stakeholders, e.g.: data generation from early pre-clinical research, through to meaningful real life end points for care.

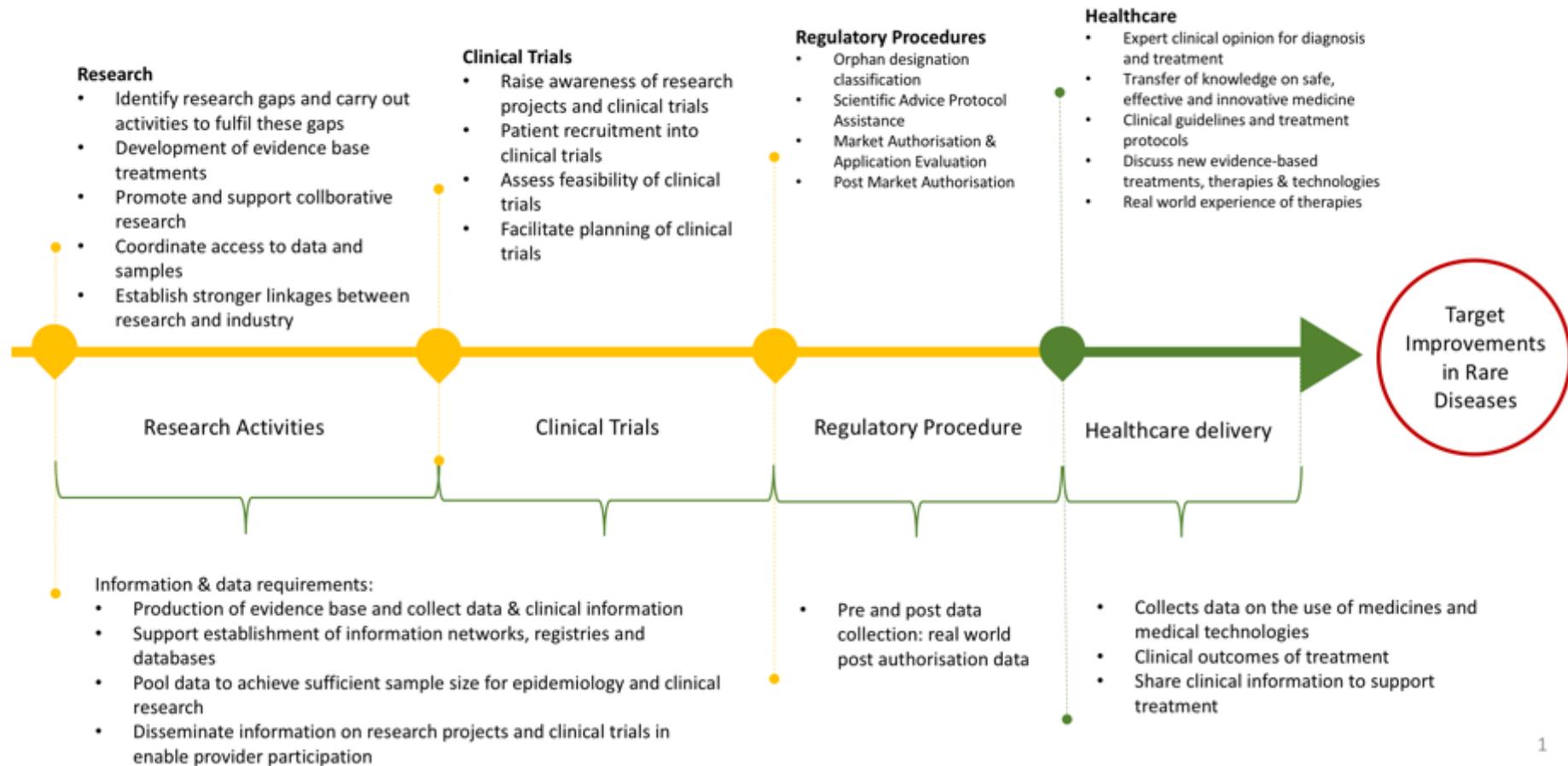
Structure of workshop

The workshop is structured in two sessions:

- **Morning session:** Developing an understanding of the aims and structure of ERNs and interfaces with the wider research and healthcare pathways and sharing experiences of previous multi-stakeholder collaboration, through brief interactive presentations, case studies and a panel discussion.
- **Afternoon session:** Three breakout sessions will explore the alignment of industry-perceived needs and opportunities around disease understanding and data generation, sustainability of the collaboration framework and exchange of expertise between ERNs and industry (early development, pre-launch and post-market authorisation touch points).

Appendix 1: European Reference Networks Core Activities¹

European Reference Networks Core Activities



¹ Source: EC ERN Assessment Manual for Applicants: Operational Criteria for the Assessment of Networks, 2016