

## How has RD-ACTION supported the conceptualisation and implementation of ERNs?

RD-ACTION (2015-2018) inherited the mandate of the previous Joint Action for Rare Diseases (the EUCERD Joint Action) – i.e. supporting the rare disease (RD) field in adding depth and clarity to the concept of an ERN and preparing the way for implementation of the Networks. Through its Policy and Integration WP (6 - led by Newcastle University) in particular, RD-ACTION has supported the ERNs by organising meetings and workshops and seeking to create policies and guidance *with* the Networks, *for* the Networks.

### HANDS-ON SUPPORT TO ENSURE COLLABORATIVE AND NON-COMPETITIVE ERN PROPOSALS

- In preparation for the first Call for ERNs, RD-ACTION organised a major workshop (Summer 2015) to build capacity amongst field leaders and assist the RD community in organising itself around the 21 broad thematic groupings identified by this same team and adopted by the Commission Expert Group on Rare Diseases as the backbone for ERN structure and scope.
- As many experts expressed a desire for a means of identifying Healthcare Providers (HCPs) interested in setting –up/joining an ERN within the same disease area, the RD-ACTION team designed a ‘**Matchmaker tool**’. Launched in December 2015, the Matchmaker ran until May 2016. This tool allowed the specialists to make contact and align intentions. In total, the Matchmaker had received **801** responses across the **21** thematic groups.
- Where several candidates were interested in coordinating the same ERN, the RD-ACTION team sought solutions and helped the consortia move forwards, partnering in this crucial work with DG Sante and EURORDIS colleagues to ensure a single comprehensive and robust application for each ERN.

### ENSURING MEANINGFUL PATIENT INVOLVEMENT IN ERNs

- The European Patient Advocacy Groups (ePAGs) were conceived by EURORDIS, as part of its contribution to RD-ACTION. The purpose is to ensure patients are fully integrated in the development, governance and operations of the ERNs.
- From 2016 onwards, open elections were held to agree formal permanent representatives for each of the 24 ERNs. Over 100 individuals have been appointed as ePAG members, tasked with representing all patients under their broad Thematic Grouping (e.g. rare cardiac), and serving on ERN Boards, committees and task forces.

### POLICY SUPPORT FOR ERNs

The ERNs are a great opportunity to embed good practices and disseminate these into broader healthcare systems. RD-ACTION’s vision was that, as ERNs were established & evolved, dedicated guidance would be important to support but also to ensure a baseline compatibility and interoperability (at many levels) *between* the ERNs. RD-ACTION partners thus developed a workplan for the years 2016-2018, designed to capitalise on the lessons learned in the broader RD field and ‘pilot’ Networks and bring these to the ERN stakeholder community, to agree together how to address shared challenges. To-date, 3 major workshops have been delivered (each co-organised with DG Sante) each addressing a particular policy area in which consensus building was deemed important

### Recent Workshops:

*Exchanging data for virtual care in the ERN framework – 27-28 September 2016, Brussels:* This topic was selected because at the heart of the ERN concept is the opportunity to provide healthcare virtually, enabling expertise to travel as opposed to patients or physicians, where possible and appropriate. With over 55 participants, this workshop:

- ✓ Agreed practical advice to enhance the efficiency and utility of virtual consultations;

- ✓ Clarified the legal issues around data protection -especially in view of the new General Data Protection Regulation- and the legal, ethical and social issues relating to consent for the sharing of data in the ERN framework;
- ✓ Brainstormed how patients will enter/ be 'referred' to the ERN for virtual care
- ✓ Shared experiences on the standardisation of data in the RD field, to identify good practices which should be embedded in the ERNs

**Using standards and embedding good practices to promote interoperable data sharing in ERNs – 26-27<sup>th</sup> April 2017:** 68 participants (a mixture of ERN Coordinators, EURORDIS and Orphanet partners, data/eHealth specialists, Coding experts, Phenotype ontology experts, data linkage experts, and DG Sante representatives) met to agree how to capture data collected in the ERNs for care purposes.

- ✓ After previously identifying the value of using the ORDO and the HPO -deemed most sensitive and appropriate ontologies for RD- participants had expressed a desire to learn more about how to use these sorts of tools practically, to optimise the use and re-use of data collection in the ERN context.
- ✓ Hands-on demonstrations were organised, to illustrate the power of these tools.
- ✓ This was followed by a session on linking data, especially through the concepts of FAIR data and the 'PPRL'.
- ✓ The workshop generated a list of recommended good practices, to enhance the use and reusability of data collected in the operations of ERNs, and created 'tool-kits' on how to practically use the most relevant of these data resources/practices.

**Indicators and Outcomes for ERNs (1-2 June 2017).** This workshop united 40 participants to work closely on issues related to the impact and monitoring of ERNs. It allowed the ERN community to:

- ✓ better understand terminology around indicators and become acquainted with the different types of indicators (structure, process, outcome) and their use in health systems
- ✓ to discuss an initial set of indicators, common to all ERNs, for the purposes of monitoring impact, and isolate the definitions needed to advance this selection
- ✓ to identify key issues and challenges on data collection and reporting
- ✓ to elucidate the challenges in selecting clinically-oriented indicators in the rare disease/highly specialised healthcare field

### **Future workshops**

Future workshops are planned -before May 2018- on the topics of **Clinical Practice Guidelines; Strategic Collaboration for orphan therapy development and accessibility;** and **Provision of Integrated, holistic care in rare diseases.**

### **SAMPLE OUTPUTS:**

The workshops are a key part of the project's activities in the ERN sphere, but WP6 has been active in uniting and supporting the Networks in other ways, as evidenced from the following sample of outputs:

- [Concept paper on convergence of eHealth and Rare Disease initiatives \(2016\)](#)
- Report on [1st meeting between the ERN Board of MS and the future Coordinators \(Sept '16\)](#)
- [Highlights and conclusions from Workshop on Exchanging data for Virtual Care](#) (Sept '16)
- [Identifying ERN requirements for an IT platform](#) (Aug '16 exploration of IT needs and correlation to published CPMS Tender)
- Canvassing of ERN plans and perspectives re. Registries (Nov '16) and [Analysis of the key issues concerning ERNs & Registries](#)
- [Report on Activities of the JA Task-Force on Interoperable Data-sharing in the framework of the operations of ERNs](#)
- [Canvassing perspectives on ERNs and Research](#) for Maltese Presidency event (March '17)
- [Tool-Kits on resources for standardising data](#) (Summer '17)
- [Recommended Practices for Data Standardisation in the Context of the operation of ERNs](#) (Sept '17)

The home page for all these pages/document is- <http://www.rd-action.eu/european-reference-networks-erns/>