



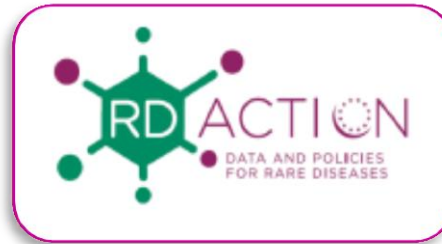
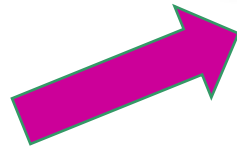
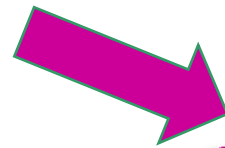
Introduction to the Workshop: Aims and expected outputs

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RD-ACTION : Data & Policies for Rare Diseases



RD-ACTION Key Stats

- 1st June 2015 - 31st July 2018
- 34 beneficiaries (APs)
- 30 collaborating partners
- 40 countries incl. Argentina and Australia
- Total Budget: €8,344,079.80



RD-ACTION Supported the conceptualisation and Implementation of ERNs

- <http://www.rd-action.eu/wp-content/uploads/2018/05/Summary-of-RD-ACTION-Support-for-ERNs-2015-18.pdf>
- As ERNs are established & evolve, dedicated guidance is important to support and also to ensure a baseline compatibility and interoperability (at many levels) between the ERNs
- ERNs are a great opportunity to embed good practices and disseminate outwards
- Focus over 2nd half on spreading good practices to all 24 ERNs and generating new guidance *with* the ERNs, *for* the ERNs
- Focus on policy support – topics of relevance for broad RD and specialised care field

Workshops

- Goal: work with challenges, avoid myriad goals &
- Need to be Flexible

common
ort in meeting



EUROPEAN MEDICINES AGENCY
SCIENCE MEDICINES HEALTH

RD-ACTION
WPs

The ERN 24
via WGs

Member
States

DG SANTE
& RTD

ePAGs

Topic
Experts

Key Outputs of RD-ACTION:

OUTPUT and LINK	DESCRIPTION	DATE PUBLISHED
Workshop Resources 'Creating a Sustainable Environment for Holistic & Innovative Care for Rare Diseases & Complex Conditions'	The dedicated webpage for our latest Workshop (12-13 Apr, 2018, Frambu, Norway) co-organised with INNOVCare, hosting the agenda, presentations and outputs	April 2018
Workshop Resources 'ERNs & Clinical Practice Guidelines'	The dedicated webpage for our Workshop (6-7 Dec 2017, Rome) co-organised with DG SANTE and the ERNs, hosting the agenda, presentations and outputs	December 2017
Summary of RD-ACTION Support for ERNs (2015-17)	Two-page overview summarising the main forms of RD-ACTION support for ERN implementation and cooperation around key policy priorities	September 2017
'Recommended Practices for Data Standardisation in the Context of the operation of ERNs'	Main Output from the Workshop 26-7th April 2017 on 'Using Standards and Embedding Good Practices to Enable Interoperable Data-Sharing in ERNs'	September 2017
Workshop Resources & Outputs on 'Indicators and Outcomes for ERNs'	The dedicated webpage for our Workshop (1-2 June, Newcastle) on Indicators and Outcomes for ERNs hosting the agendas, presentations and outputs.	June 2017
Tool-Kits on key resources, resulting from Workshop on Data & ERNs (April 2017)	'Tool-Kits summarising where to find practical information on Orphanet Nomenclature; HPO ; FAIR Data; PPRL for rare diseases	June 2017

Context for this Workshop

- ERNs are first and foremost dedicated to care. However:
- ERNs are also required to contribute to research in their focal area
- This raises major opportunities for these multistakeholder pan-European networks to act as ‘game changers’: e.g.
 - Permanent
 - Comprehensive disease coverage
 - Patient-centred
 - Build upon research networks, in some cases
 - Opportunity to embed good practices from the beginning
- How do we move from an appreciation of the benefits and opportunities to a tangible impact?

Aims of this Workshop

1. To share the state of the art of tools and resources which exist in 2018 to streamline and optimise each 'point' in the clinical research pipeline
2. To better understand the priorities and needs of the ERN community specific to clinical research, and explore case studies (both of pre-ERN successes on the part of research networks, and early ERN-era achievements/goals) in particular detail
3. To elucidate the services and opportunities offered by the European Medicines Agency which are of relevance to clinical research in rare and highly specialised domains
4. To identify concretely *how* and *where* ERNs could make a positive difference to each 'timepoint' in the clinical trial pathway, including points of engagement specifically with the EMA, to agree a 'roadmap' to a more strategic and streamlined collaboration in future.

Agenda

- Day 1 is very much ‘setting the scene and establishing the status quo’
- Day 2 is focused on ‘where we wish to go, and how we get there’ = Discussion Topics:
 1. **WHAT SORTS OF ACTIVITIES UNDER THE HEADING OF ‘CLINICAL RESEARCH’ WILL ERNS ENGAGE IN, AND WHAT ARE THE ADVANTAGES OF THE ERN STRUCTURE?**
 2. **WHAT OPPORTUNITIES EXIST UNDER CURRENT EMA STRUCTURES AND RESOURCES AND HOW MIGHT ERNS ENGAGE WITH THESE?**
 3. **IDENTIFYING CONCRETE ROLES AND RECOMMENDED PRACTICES TO INVOLVE PATIENTS IN THE VARIOUS TYPES OF ERN-RELATED CLINICAL RESEARCH**
 4. **HOW CAN ERNS GENERATE/LINK/EXCHANGE DATA TO SUPPORT THE PLANNING AND EXECUTION OF CLINICAL TRIALS AND STUDIES?**

Towards an effective Workshop!

- Very broad topic! And a first start
- Key points from today should be noted and raised in the sessions tomorrow
- Lots of discussion time tomorrow: active participation from all is essential for success of the workshop!
- We will sometimes 'refocus Qs' to keep what goes together, together
- Outputs are to be determined: e.g.
 - Workshop Report
 - Consensus 'Recommendations' on Engagement of ERNs with key stakeholder groups for the full spectrum of clinical research (TBC)?
 - 'Toolkit' of existing resources which could support ERNs in clinical trials/studies?
 - Publication of Survey results

This workshop unites 66 participants:

- 20 ERNs
- 10 ePAG representatives
- DG SANTE & DG Research
- EMA experts
- RD-ACTION partners & topic experts

Many thanks to the Workshop Organising Committee:

- **RD-ACTION:** Victoria Hedley, Hannah Murray, Yann le Cam, Valentina Bottarelli, Virginie Hivert, Ines Hernando
- **DG SANTE:** Helene le Borgne & Enrique Terol
- **EMA:** Monica Ensini, Isabelle Moulon, Ivana Silva
- **ERNs:** Luca Sangiorgi, Maurizio Scarpa, Franz Schaefer