RD-ACTION, EMA and DG SANTE Workshop: How ERNs can provide added-value in the area of clinical research

Dates: 29th-30th May 2018

Venue: European Medicines Agency, London

Ethos of RD-ACTION workshops

A key objective of the RD-ACTION Policy WP workplan is to continue to provide support to the rare disease and rare cancer community in conceptualising, implementing and evolving robust ERNs capable of meeting the needs and expectations of people living and working with conditions requiring a specific concentration of expertise. As the ERNs are established and evolve, shared consensus guidance and policy documents -generated *with* the ERNs, *for* the ERNs- is important to support the Networks but also to ensure a baseline compatibility and interoperability (at various levels) between the ERNs. These workshops are collaborative events, uniting expertise from RD-ACTION Partners, DG Sante, the ERN Coordinators and member centres, the Board of MS of ERNs, and more. For this final RD-ACTION workshop, the focus of which will be clinical research in the ERN era, the event is being hosted by the European Medicines Agency.

Context for this workshop

ERNs are first and foremost dedicated to optimising access to high quality healthcare for conditions requiring a particular concentration of expertise (with rare diseases being the obvious – although not the *only*- beneficiaries). However, ERNs are also required –through the Legal Acts governing their creation and functioning- to contribute to research in their focal area, which raises major opportunities for these (theoretically permanent) multistakeholder pan-European networks to act as 'game changers'. ERNs are, in many ways, perfectly-positioned to impact positively on the organisation and execution of clinical trials in the domain of rare diseases and highly specialised healthcare: for example, ERNs give an unprecedented level of visibility to European expertise in particular diseases/groups of diseases, in terms of where to find experts/Centres of Expertise; the knowledge concentrated in each ERN will be invaluable in terms of the *planning* of clinical trials, for example in the selection of sites, the selection of endpoints, recruitment of patients, etc.; there are important opportunities to generate high quality data to increase interest in hitherto neglected conditions, facilitate the planning and organisation of the clinical trials, collect Post-Marketing Surveillance data, etc.

This workshop essentially constitutes a merging of previously proposed RD-ACTION workshop topics concerning how ERNs will add-value to the status quo by engaging strategically with stakeholders such as Industry, Regulatory bodies, HTA etc, to facilitate and streamline the full developmental pipeline of therapies for rare diseases. The revised plan is that this workshop will now focus on clinical research in particular, and 'drill-down' to how and where (i.e. at which points along the

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clinical research pathway) ERNs can really add value compared to the previous situation. A particular focus will be to identify how the new Networks can formally engage with the EMA, to streamline activities and facilitate clinical research.

Aims of the Workshop

The aims of this workshop will be addressed through a combination of presentations to establish the state of play (Day 1) and in-depth discussions (Day 2), focused around a series of clinical-research-related topics. The principal aims are as follows**:**

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.

Participants:

This workshop will involve:

- EMA experts (from various units)
- ERN representatives
- RD-ACTION partners
- ePAGs (European Patient Advocacy Groups)
- DG Sante and DG Research representatives
- Board of Member States (BoMS) of ERNs Representatives
- E-RARE / future potential coordinator of European Joint Programme Co-Fund for RD
- Representative of relevant clinical research initiatives, including Conect4Children and IDeAl

Expected Outputs:

- 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