RD-ACTION & DG Sante Workshop: How can ERNs generate, appraise and utilise clinical practice guidelines, to enhance the impact and deployment of consensus guidelines in national health systems?

Dates: 6th and 7th December 2017

Venue: Istituto Superiore di Sanita

Aula Marotta, National Centre for Rare Diseases
Viale Regina Elena, 299 00161 - Rome, Italy

Day 1: 10.00 am – 5.45pm

Day 2: 09:00 am– 15:00pm

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 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, for which input is sought from RD-ACTION Partners, DG Sante, the ERN Coordinators, and Board of MS of ERNs, and more.

Context for this workshop

Amongst the most powerful tools to generate and disseminate knowledge in the clinical and research settings are up-to-date best practice guidelines, which may be generated for a range of purposes, such as clinical diagnosis, management and treatment. High quality treatment pathways and clinical guidelines, as well as the presence of a core multidisciplinary team, are important prerequisites for improved clinical outcomes and ultimately survival and improved quality of life of patients living with a rare disease or rare cancer. Many of the ERNs report the existence of a high number of clinical guidelines, however the implementation and adherence of these evidence-based clinical guidelines is limited (in some cases, less than 40% of patient care is provided according to existing evidenced-based guidelines). Furthermore, several guidelines have been generated with a certain level of influence from pharmaceutical companies, and therefore cannot be considered fully scientifically independent. ERNs frequently report that their goals in the first years include building consensus, updating and implementing guidelines, and increasing adherence to published guidelines.

Clinical practice guidelines/best practice guidelines serve as a great equaliser in the RD field: they can mean the difference between no care/substandard care and patients living longer, healthier lives with fewer complications. Guidelines, whether designed to support diagnosis or care, can serve as a blueprint of excellence, to advise doctors closer to the patients on how to treat them in a way that reflects the best possible knowledge and will generate the best possible outcomes.

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The legal Acts mandate various activities for ERNs, relative to clinical practice guidelines. Each ERN has by now made its initial plans relative to clinical practice guidelines, which reflect the status quo in each disease domain and also the partners the Networks will engage with.
However, given the centrality of this topic to the operations of ERNs, it would be beneficial **to pool and share knowledge of existing approaches, tools, and resources which could support the ERNs in this mission.**

Aims of the Workshop

1. **To agree a list of terminology pertaining to Guidelines, to be used between Networks**
   It will be important for all ERN stakeholders to use terminology in the same way when speaking of 'guidelines': there is sometimes confusion over the naming of various types of guidelines/statements/standards etc. and it would be beneficial to reach consensus on this, for future use. Starting from an existing Thesaurus relating to Guidelines, the participants will adapt this if necessary and adopt as a guide for the ERN community.

2. **To pool knowledge and clarify existing methodological approaches to guideline generation/implementation, to be pursued under the ERNs**
   The rare disease and specialised healthcare field has strong consensus on robust methodologies to generate guidelines (e.g. GRADE II) and appraise them (AGREE), and indeed these methodological approaches were highlighted in the ERN operational criteria. The workshop will allow the ERN community to affirm the methodological approaches the Networks will use to generate and appraise guidelines, and to highlight briefly what these entail (the workshop will not provide dedicated training on how to use each of these approaches, preferring instead to focus on how the ERNs can add-value in this field)

3. **To raise awareness of existing relevant tools and resources specific to rare diseases/specialised healthcare which might be useful for ERNs.**
   Repositories for various types of guidelines exist, for instance the RARE-Guideline database developed as an output of the RARE-BestPractices (http://www.rarebestpractices.eu) EU funded project (coordinated by the Istituto Superiore di Sanità), and the Orphanet Emergency Guidelines resource. These will be presented to the participants, as resources for use by the wider ERN community. This is particularly important in view of the European Commission's plans in future to somehow pool existing resources from previous initiatives and use these to identify good practices and tools for adaptation and deployment in particular fields

4. **To align and coordinate the actions envisaged and developed by the Joint Action on Rare Diseases and the Joint Action on Rare Cancer, with regards to concepts and methodology concerning clinical guidelines.**
   The JA for Rare Cancer (JARC), coordinated by Paolo Casali has a WP dedicated to Clinical Practices Guidelines. It is important to make synergies with these activities, and identify issues for future collaborative work to support guidelines generation, appraisal, and usage in the rare disease and rare cancer field

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5. **To clarify the roles ERNs could and should play in the generation, appraisal, use and dissemination of guidelines (and with which actors should they partner in such tasks)**
    - What is 'new' under the advent of ERNs – what added-value can they bring to this process?
    - How can ERNs partner effectively with learned societies, patient groups etc. (can we agree any good practices across ERNs here?) It will be very important to explore established good practices in incorporating **patients and patient organisations** to the generation and dissemination of guidelines – the workshop will seek to promote shared good practices in this respect.

6. **To better understand the 'status' ERN guidelines might hold within individual Member States, and explore actions to support the use of such Guidance in local hospitals (i.e. How can Member States USE Guidelines generated within the ERN framework? Which are the sources to sustain independent guidelines?)**
   It is acknowledge that perhaps the major challenge is not in generating or disseminating CPGs, but actually promoting their use in European countries. *Therefore, the workshop will focus primarily on what might –i.e. what should- change under the advent of the ERNs, and how these new structures can positively impact on the generation of Guidelines, involving all relevant actors, being scientifically independent and sustainable, but also on how these outputs might better be used in health systems, to bring the best possible practices in diagnosis, treatment and care closer to patients. This will entail an analysis of examples in which European-level expert consensus in the field of health has been adopted and implemented effectively at the national level.*

Expected Outputs:

This workshop will produce consensus Guidance across several aspects of this broad topic, including the following:

- **A Workshop Report, which will summarise the recommended methodologies and approaches regarding the generation and appraisal of Guidelines**
- **An agreed list of terminology /Thesaurus for use by the ERNs community**
- **Recommended practices for involvement of stakeholders in guideline generation/appraisal/dissemination**
- **Further suggestions? Especially re. the impact at national level**

Participants will include:

- ERN representatives (each ERN is invited to bring one delegate, funded by RD-ACTION)
- RD-ACTION partners
- JARC representatives
- ePAGs (up to 10 ePAG representatives, with expertise in this topic)
- RARE-Bestpractices experts
- DG Sante representatives;

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- Representatives of two or three Learned Societies
- BoMS/National representatives (up to 8)
- Experts from Orphanet Guidelines team, Guidelines International Network (GIN), NICE, etc.

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Agenda

Session 1: Introduction and Aims of the Workshop

10:00 – **Welcome Message from host of this Workshop** (Domenica Taruscio and DG Sante?)

10:10 - **Introduction to this Workshop and Presentation of Aims and Expected Outcomes** (Victoria Hedley, RD-ACTION)

10:30 - **The context for this workshop: added value of clinical practice guidelines in highly specialised domains and rare diseases/cancers** To include a good technical outline: What are the different types of CPGs? Why should CPGs be produced? What problems can they solve (perhaps what problems might they create?) What methodologies have been used traditionally?)

11:00 Discussion and Q&A

Session 2: Clinical Practice Guidelines – Tools and Resources to increase efficiency and harmonisation in future

11:15: **ERNs and Clinical Practice Guidelines: the results of the pan-ERN survey** (a presentation to include: a brief summary of what ERNs are expected to do in this area, based on the legal Acts and Operational Criteria; briefly introduce the WG; then present a summary of information gathered from the 24 ERN leads (see survey document on email of 6.11.17) So this would summarise the plans, priorities and challenges of the ERNs, what methodologies are they using, etc.)

12:00 – 13:00 Lunch (buffet provided)

13:00 - 14:00 **Harmonising Terminology for use in the ERN community** (interactive discussion **(*To fulfil Aim 1)***

14:00 – 15:00: **Tools and Resources relating to the generation and appraisal of Guidelines in rare diseases/rare cancers (*To fulfil Aim 3)***

i. Key Conclusions and Outputs from RareBestPractices (Karen Ritchie? Domenica Taruscio?)
ii. Summary of Orphanet Resources relating to best practice guidelines (Ana Rath)
iii. How is the Rare Cancer community supporting the generation, dissemination and use of Guidelines: strategy under the JARC **(*To fulfil Aim 4*)**

15:00 Coffee Break

Session 3: Optimising the capacity of ERNs to generate, appraise, use and disseminate CPGs

15:20 - 16:20 **ERN-Led Case Studies: Plans and Priorities to address the topic of Clinical Practice Guidelines in the ERN Framework**

i. Case Study 1

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ii. Case Study 2
iii. Case Study 3
iv. Case Study 4

16:30 – 17:45 ERN RoundTable Discussion: Co-Chairs TBC ***(To Fulfil Aims 2 and 5)***

17:45 Day 1 ends

DAY 2

08:45 Welcome Coffee

09:00 **Summary of Day 1 (Victoria Hedley and an ERN Representative)**

Session 4: Good practices and multistakeholder collaboration in Guideline Generation, Revision, and Dissemination (To Fulfil Aim 5)

09:20 **UEMS: Activities and objectives directly related with the knowledge and training generation in the case of highly specialised healthcare and in particular in the area of generation of clinical guideline** (Bela Melagh)

09:45 **Perspective of a Scientific Society: added-value of working with ERNs and how to form a successful partnership** (??)

10:10 – **A Case Study for mutually beneficial, ethical collaboration with Companies/CROs in CPG generation (TBC)**

10:30 - **Group Discussion** (Goal is to identify modes of engagement with these sorts of stakeholders which can be replicated across the Networks, e.g. ways to co-author Guidelines, pitfalls to avoid, (possibly touching upon Nicoline's work on codes, if we refer to Industry??)) Co Chairs perhaps Domenica Taruscio, ERN representative, BoMS representative?)

11:00 Coffee Break

11:20 Benefits and Opportunities for Patient Involvement in Guidelines (ePAG with RBP expertise)

11:40 'Lightning' Interventions: Examples of meaningful patient involvement in Guidelines

12:10 **Group Discussion**: can we identify and agree some good practices for patient participation to guideline development or standards of care under ERNs (Chairs: EURORDIS and an ERN representative?)

12: 30 – 13:15 Lunch

Session 5: Towards Implementation of ERN-associated Guidelines in Member States (To Fulfil Aim 6)

13:15 **Case Study: How can European level expert consensus result in the implementation of Guidelines at the national level?** DG Sante

13:40 – Member State 'Lightning' Perspectives on anticipated use of ERN-led clinical guidance

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14:10: Discussion – Building the case for ERN impact: how do we optimise usage of CPGs 'on the ground'? Chairs Till Voigtlaender and DG Sante?

14:50 Summary of Action Points, Outputs, and Next Steps (Victoria Hedley, Paolo Casali, Enrique Terol)

15:00 Closing of the Worksop (Domenica Taruscio/Primiano Iannone)

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