Workshop 3: Indicators and Outcome Measures to demonstrate the impact and efficacy of ERNs

Dates: 1st and 2nd June 2017

Venue: International Centre for Life, Newcastle upon Tyne, UK

Day 1: 9-5.45 (Dinner at 7pm)

Day 2: 8.45 – 2 pm

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 1st ERNs are established and evolve, shared consensus guidance is important to support the Networks but also to ensure a baseline compatibility and interoperability (at various levels) between the ERNs.

Context for this workshop

ERNs have a broad and ambitious mandate – they need to be able to demonstrate their impact across a broad range of fields and activities, which requires carefully-selected indicators and at the same time a monitoring system including Key Performance Indicators should be set up to follow up the activity and caseload of the ERNs. In the initial ERN application process, would-be ERNs were asked to summarise how the network will 'implement outcomes measures and quality control',[1] and subsequent Grants have asked Coordinators to define Indicators [2]. Although all ERNs have thus touched upon this issue already, the majority of the newly-formed ERNs are still considering how best to identify, agree and monitor achievements and outcomes. Determining appropriate disease-specific clinical outcomes, to monitor over time, is particularly challenging in the field of rare diseases and in highly specialised healthcare domains. Alongside the efforts of the ERNs themselves to address these issues, there is growing recognition in DG SANTE and in the ERN BoMS of the need for **continuous monitoring and assessment**, at various levels: [3]

1. **Development of a continuous monitoring system of the ERN activities**
2. **Periodical self-assessment and reporting of the ERNs and HCPs (similar to the Assessment performed at the initial stage);**
3. Stronger involvement of Member States in the assessment of their national HCPs wishing to participate in, or participating already in related ERNs

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[1] Question **9: Please provide a summary explaining the approach or plans the Network has adopted or will undertake to produce good practice guidelines and implement outcome measures and quality control**

[2] E.g. the 1 year (SGA) and 5 year (FPA) Coordination funding applications required the ERNs to propose Specific Objectives and accompanying Indicators (in this case Process; Output; and Outcome/Impact.)

[3] These 4 forms of monitoring and assessment have been identified in the DG SANTE Non-Paper disseminated amongst the ERN Coordinators in April 2017.

4. Further assessment by the IAB of HCP not assessed in the initial process

Of these four, the RD-ACTION workshop on the topic of 'Indicators and Outcomes' primarily concerns point 1 (and will touch upon point 2) above, but with a particular emphasis on how ERNs will demonstrate their added value. **Specifically, this workshop will address two broad categories of Indicators, relevant to the activities of the ERNs:**

1. *ERN Common indicators i.e. Indicators which should be shared by all 24 ERNs and which are based upon the common activities and objectives of the Networks.*
    o The collection of these Common Indicators will enable a tangible demonstration of the added value of ERNs, at a high level .
    o Agreed Indicators should be both feasible and realistic. It is essential to balance ambition with reality here: the Indicators should reflect the fact that the Networks are a completely new construct, but also demonstrate that the ERNs are operational and are making an impact from the beginning (the latter is important for the sustainability of the Networks, and for securing ongoing/additional support)
    o The 24 Networks should be active partners in the selection of these common Indicators, through the sharing of past experiences in highly specialised healthcare and via the analysis of indicators proposed by the ERNs to-date

2. *ERN Specific Indicators i.e. based upon disease area or specialised procedures/techniques*
    o ERNs will be aware of the challenges and opportunities inherent in monitoring the impact of the Networks clinically, through the selection and monitoring of clinical outcomes for patients.
    o Establishing a 'culture of learning' at the heart of ERNs will enable the rapid generation of new evidence and innovative approaches to caring for people with a rare disease, and will allow the identification of emerging best practices and innovation whilst evolving philosophies of care.
    o Simple disease-specific clinical outcomes are the key to unlocking clinical dialogue across ERNs, enabling the identification of optimal diagnosis and treatment approaches that lead to improved health and quality of life for patients. Clinical outcomes are seen as a key priority area for all ERN to enable benchmarking on care.
    o These will often be specific to the ERN's area of expertise, but to support the 24 ERNs in refining and revising these, the workshop will share examples of how certain rare disease communities select clinically-focused outcome measures at present.

Key Questions for discussion will include:
- How will ERNs demonstrate their added value? What sort of impact will be demonstrable quickly, and what will only be feasible in the long-term?
- How will the operation of the ERNs be monitored. Which activities/ actions and events other that the clinical performance and outcomes should be collected?
- What can realistically be measured across each Network? How can this information be collected, and by whom, and how often?

- What Indicators have ERNs already proposed to monitor in their Grants? What could be common to all?
- What kind of clinical outcome measures have the ERNs identified, to demonstrate their effectiveness?
- How can ERNs embed a culture of knowledge generation, through the implementation of clinical outcome measures that are centred upon learning through complex case studies?
- How can ERNs optimise use of patient-reported outcomes and promote good practices in this area?
- How might health systems compare outcomes for patients who receive care via the ERN vs. those who do not?

Aims of the Workshop

- To better understand terminology around indicators and become acquainted with the different types of indicators (structure, process, outcome) and their use in health systems

In terms of Common Indicators:

- To agree on an initial set of indicators, common to all ERNs, for the purposes of monitoring impact
- To discuss key issues and challenges on data collection and reporting
- To develop a timeline for finalizing a monitoring system

In terms of disease/process –specific clinical outcomes

- To elucidate the challenges in selecting clinically-oriented indicators in the rare disease/highly specialised healthcare field
- To gain an overview of which indicators are currently being used in some disease areas and explore how such decisions might be made in the ERN context
- To clarify the options for collecting patient-reported outcomes

Expected Outcomes:

- A workshop Report and PwPs
- An advanced draft of Common Indicators for all ERNs (encompassing structure, process and outcome indicators)
- Guidance on how to embed a 'peer learning approach' in ERNs, including case studies on identifying clinical impact in highly specialised healthcare (TBC)

Day 1: Thursday 1st June

SESSION 1: INTRODUCTION TO INDICATORS AND THEIR USE IN HEALTH SYSTEMS (Chairs Matt Bolz-Johnson and Jaroslaw Waligora)

9:00 Welcome, Introductions, and Aims of the Workshop - Victoria Hedley

9:20 Indicators and Outcomes: Relevance to the ERN context - Enrique Terol and Hristina Mileva

Q&A

10:00 Coffee Break

10:20 Indicators '101' – Key definitions and Terminology - David Vizer (DG SANTE and ECHI (European Core Health Indicators))

10:50 Good practices in indicator development - Charlotte Roberts (ICHOM - International Consortium for Health Outcomes Measurement)

11:10 Q&A

11:30 How are Indicators managed in Healthcare systems in the context of highly specialized care?

Country Case Study: Spain (Ines Palanca and Laura Marin)

12:00 – Discussion

12:30 -13:30 Lunch (provided onsite)

SESSION 2: ANALYSING COMMON INDICATORS FOR ERNS (Chairs Victoria Hedley and Enrique Terol)
13:30: Applying common indicators to the ERN framework – presentation of the non-paper - Enrique Terol (DG SANTE)

13:40 View of the Working Group on the monitoring of ERNs (Michelle Battye)

14:00- 15:20 Common indicators discussion, in mixed groups (4 groups) – for 80'

- Reviewing some of the proposed indicators
- Identifying strengths and weaknesses
- Identifying gaps
- Focused discussion on survival/mortality/morbidity indicators

(15:20- 15:40 Coffee Break)

15:40: Presentation of draft conclusions of each group and joint revision/annotation of the Common Indicators table

Outcomes of the working groups and discussion

- Discussion on the first set of proposed indicators in Plenary, working through what might be changed for the next draft, AND:
- **How can we practically collect some of this data?**
  - Frequency and burden of measurements
  - Centralization of the data
  - Tools to be used
  - How to incorporate direct PROMs/PROIs

Day 1 Ends 17:45

Group Dinner at Blackfriars Restaurant, Medieval Banqueting Hall – 7pm -ca. 9.30pm

Day 2: Friday 2nd June

8:45 Summary of Day 1 - Victoria Hedley

SESSION 3: DISEASE-SPECIFIC OUTCOMES – CONTEXT AND METHODOLOGIES
9:00-9:20: Indicators and Clinical Outcomes in Rare Diseases: EU activities to-date – Jaroslaw Waligora (DG SANTE)

9:20-9:40: Measuring Disease-specific Outcomes in Practice - Alethse de la Torre (ICHOM - International Consortium for Health Outcomes Measurement)

09:40-10:00 Challenges of Identifying 'what works' in rare disease clinical care and monitoring clinical outcomes - Matt Bolz-Johnson EURORDIS

10-10:20 Coffee Break

SESSION 4: PATIENT-REPORTED OUTCOMES (Lenja Wieje and Jaroslaw Waligora)
10:20-10:50: Patient Reported Outcomes: Merits and Methodologies - Niek Klazinga (OECD)

10:50-11:10 The state of the art in Patient Reported Outcomes – patient insights and advice summarising the status quo and applicability to the ERN context - Matt Bolz-Johnson

11:10-11:30 Discussion: How can we best elicit patient report outcome data in the ERN framework?

SESSION 5: IDENTIFYING DISEASE/PROCEDURE-SPECIFIC OUTCOMES IN ERNS (Chair Kate Bushby and Till Voigtländer)
11:30-12:20 Brief ERN Case Studies: 'Clinical outcomes – the key to unlocking the potential of ERNs' (10 min presentations)

Michelle Battye – Helen Cross – Ruth Ladenstein

12:20-13:20 ERN-Specific Indicators - discussion (in plenary)
- How can clinical outcomes be identified and how can services /centres review and discuss emerging best practice?
- What specific indicators have ERNs proposed to monitor thus far?
- How can we share these (and adopt each other's, where relevant)?
- What specific indicators are indicative of the added value of the network?
- Can we agree any good practices or means to overcome the inherent challenges?

13:20-13:30 Final conclusions including responsibilities for next steps (Chairs: Victoria Hedley and Enrique Terol)

13:30 Buffet lunch

14:00 Workshop Ends