PS8: New medicines, medical technologies, clinical trials & stakeholders support

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Victoria Hedley

The John Walton Muscular Dystrophy Research Centre
Newcastle University
The Newcastle upon Tyne Hospitals NHS Foundation Trust

Conflict of Interest Disclosure

"I have no actual or potential conflict of interest in relation to this program/presentation"

Victoria Hedley

Rare Disease Policy Manager

Newcastle University

Victoria.Hedley@ncl.ac.uk

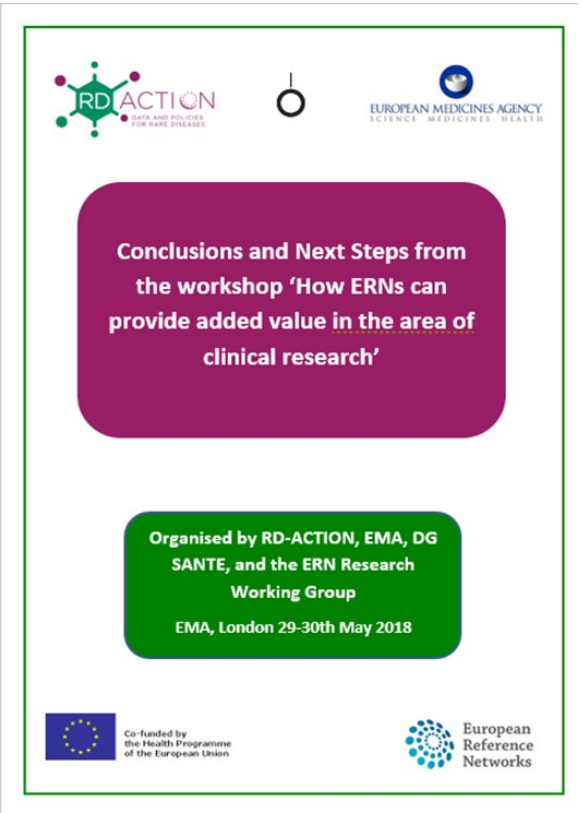
@RareDiseasesEU

Why this topic?

- Despites years of research and investment, only ca 400 of the ca. 8000 rare diseases have an approved therapy.
- Worrying trends in medicines development – development of new therapies is stalling whilst costs are rising
- There are challenges all across the R&D pipeline. Need to ensure the products which do make it are effective (and accessible)
- We see estimates like ‘at the current rate, it would take 500 years for all RD to receive a treatment’

Foundations of this Session

- ERNs are first & foremost dedicated to care. However:
- … ERNs are also required to contribute to research in their focal area
- Workshop May 29-30th @EMA (http://www.rd-action.eu/european-reference-networks-erns/)
- How do we move from an appreciation of the benefits and opportunities to a tangible impact?
- Action Points identified for some key topic. And…



RD ACTION
DATA AND POLICIES FOR RARE DISEASES

Concrete advantages inherent in the ERN model

- ✓ Permanence
- ✓ Proximity of clinical and research spheres
- ✓ Comprehensive disease coverage
- ✓ Cross-fertilisation of expertise
- ✓ Data generation/linkage opportunities
- ✓ Patient involvement
- ✓ Reputational excellence

PS8 Outline

- Very limited time this afternoon and several topics:
- Some have received more attention than others to-date
- Therefore, to guide next activities we need your views and input
- Q&A time but… you can provide your input during/after the Session:
  - Email luca.sangiorgi@ior.it or victoria.Hedley@ncl.ac.uk
  - Write your comments on note-paper and hand them to us
- Feedback will help generate concrete actions to feed into a research roadmap

RD ACTION
DATA AND POLICIES FOR RARE DISEASES

Key questions to stimulate debate

- *3 Sub-sessions with connecting HTA update:*
  - Clinical Trials
  - Medicines' Repurposing
  - Medical Devices & Medical Technologies

- For each, we invite you to consider the following:
  - *How could ERNs approach/add value to each activity?*
  - *What are the challenges and what solutions exist (with reference to case study examples, where relevant)?*
  - *Which stakeholders need to be engaged for each activity and how might this be achieved?*

- Luca Sangiorgi (ERN BOND)
- Flora Giorgio (DG SANTE)
- Helen Lee (DG SANTE - Representative of the Expert Group STAMP)
- Jean-Noël Bouillon (MedTech Europe)
- Isabella Brambilla (ePAG advocate
- Thomas Allvin (EFPIA representative)
- Eileen Treacy (Expert and BoMS Representative for Ireland)
- EC Support: Patrizia Tosetti. Unit B3 DG SANTE