XV Foresight Training Course Boosting research and innovation in a changing regulatory framework
December 15th, 2022

Session 1 – The new course of clinical studies in Europe

Panel discussion

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EUROPEAN JOINT PROGRAMME
RARE DISEASES

D4. What are the top 3 points of problems you have with conducting clinical trials in your rare disease area?

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Section 6: Needs and Priorities Q10: Obstacles…

Q10: Which of the following would you consider to be major obstacles to YOUR ERN facilitating/streamlining/ delivering clinical research? *

- ☐ Lack of funds
- ☐ Lack of clear opportunities at present to engage with Companies
- ☐ Lack of well-stratified patient cohorts for trials/studies, e.g. through appropriate, robust registries
- ☐ Lack of regulatory know-how to organise trials across borders
- ☐ Uncertainty as to how the ERNs can 'lead' or participate to trials and studies (e.g. how far such activities will be delivered entirely within the Network vs how to engage outside)
- ☐ Uncertainty over the best methodologies to conduct trials in small populations
- ☐ Lack of appropriate clinical outcomes/endpoints, etc.
- ☐ Other

19 – Lack of funds

12 – Uncertainty over methodologies

10 – Lack of well-stratified patient cohorts for trials/studies

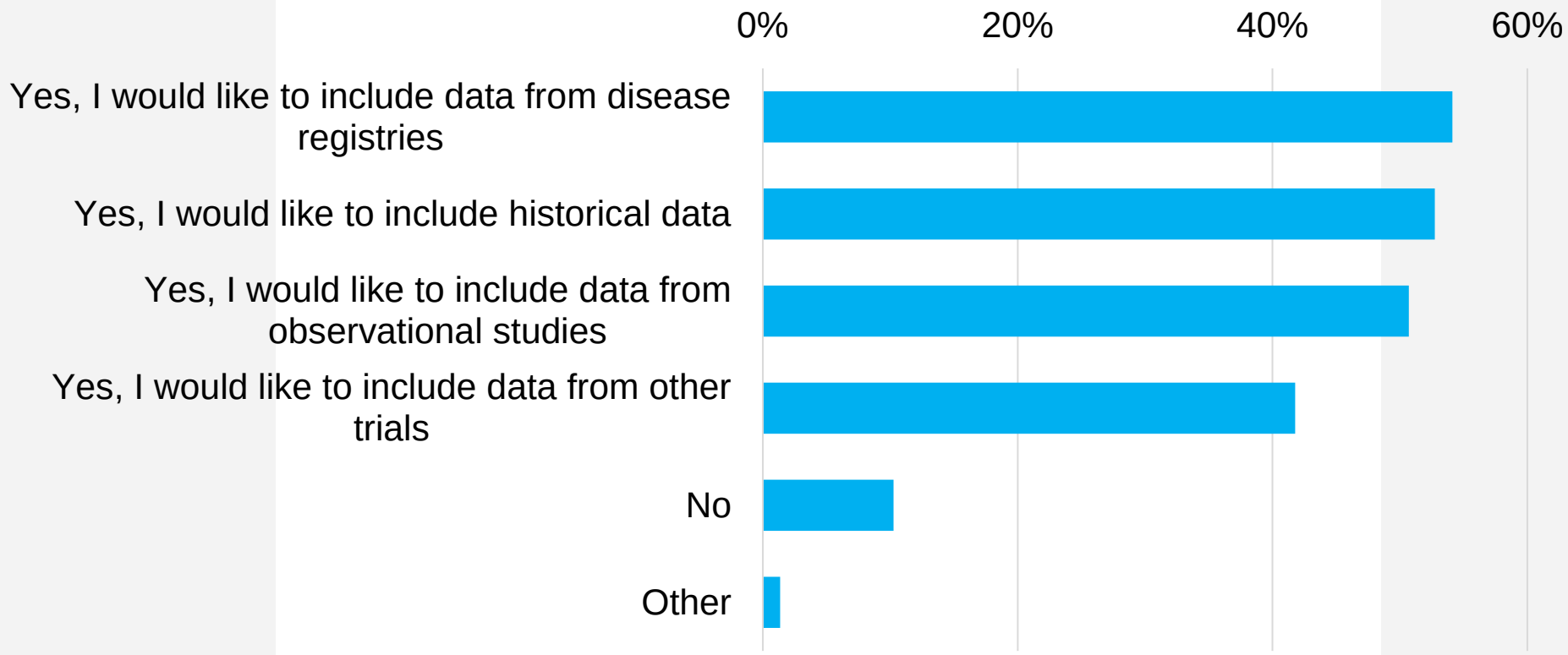
10 – Lack of regulatory know-how

10 – Lack of appropriate clinical outcomes

Other:

- Brexit
- Lack of admin support
- Terrible model of co-funding within the health programme

E11. Do you or would you like to include information from sources external to the data obtained during the trial into your trial design?



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2021-22 Educational programme: Advanced level

	Title	Date	Lecturer	Recording
	**Does Randomization matter in RD clinical trials?**	14 Dec 2021	Ralf-Dieter Hilgers *RWTH Aachen University, Aachen, Germany*	https://www.ejprarediseases.org/event/ejp-rd-training-webinar-does-randomization-matter-in-rd-clinical-trials/
	**Composite endpoints including patient relevant endpoints (Quality of Life)**	6 May 2022	Johan Verbeeck *Data Science Institute & I-Biostat UHasselt, Belgium*	https://www.ejprarediseases.org/event/composite-endpoints-including-patient-relevant-endpoints-quality-of-life/
	**The statistical evaluation of surrogate endpoints in CTs**	18 Nov 2022	Geert Molenberghs *Universiteit Hasselt, Belgium*	

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2023-24 Educational programme: Advanced level

Title	Date	Lecturer
Statistical and operational challenges with master protocols	24 Mar 2023	Franz König *MUW - Medizinische Universität Wien*
Replicated N-of-1 RCTs for Rare Diseases	30 Jun 2023	Patrick Onghena *Katholieke Universiteit Leuven*
Item response models for analysing assessments in rare diseases	29 Sep 2023	Mats Karlsson *Uppsala University*
Modelling natural history in longitudinal data-Challenges and Solutions	29 March 2024	Ralf-Dieter Hilgers *RWTH Aachen University, Aachen, Germany*

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