Joint Workshop ERICA – c4c
"Exploring and enhancing the potential for ERN registries to advance research in rare and paediatric conditions"

4th-5th February 2025
Heidelberg, Germany
Marsilius Kolleg – Heidelberg University Hospital

Goals of the workshop:

- To bring together representatives of ERN registries, c4c partners, and additional experts, to better understand the current potentiality of the existing registries managed by the 24 ERNs with respect to paediatric research and regulatory purposes
- To discuss ERNs collaboration and communication in the post-ERICA stage.
- To identify gaps and areas which would need to be addressed to make these registries more appropriate and powerful for these activities.
- To suggest steps or actions which could be taken forward by post c4c projects to maximise the potential

4th February

08:15 - 08:45	**Welcome and Registration**
08:45 - 09:10	**Introduction, goals of the workshop, tour de table** – Franz Schaefer, Maurizio Scarpa, and Victoria Hedley
SESSION 1: Status Quo of ERN Registries	
09:10 - 09:40	**Update on ERN registries** – Jose Ramírez-García • General overview of ERN registries based on previous ERICA surveys
9:40 – 10:20	**Discussion – what are the bottlenecks for ERN registries? What practical challenges are we facing?** Facilitator – Franz Schaefer
10:20 - 11:00	**Role of ERN Registries in Improving Care: Key Performance Indicators** – Franz Schaefer, Jose Ramírez-García • Overview of KPI strategies in ERN registries **ERN Registry testimonies – how is your registry improving care?** • Invitation to share your Registry successes in the care domain
11:00 - 11:30	**Coffee Break**

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SESSION 2: Optimizing the power of ERN Registries – making our data more FAIR and enhancing the data capture features (20 min presentations, 10 min discussions)	
11:30 - 12:00	**Why and How to be FAIR: ERDERA solutions** • Use of ERDERA Virtual Platform by ERN registries (Review of current state of development, onboarding of registries, potential use for inter-ERN collaborations
12:00 - 12:30	**Registry data acquisition by automated EHR extraction: holy grail or fata morgana?** – TBD • EHR diversity (JARDIN survey results); existing tools for data extraction and current state of use; issues around data availability and quality; ERDERA WP9.1 activities • Critical appraisal of barriers, effort/benefit ratio)
12:30 - 13:00	**Pseudonymisation: SPIDER and EUPID** • Feedback from registries currently implementing pseudonymisation tools, open discussion
13:00 - 14:00	**Lunch**
14:00 - 14:30	**The mobile registry: How can we reach out to patients?** – TBD • App solutions: Direct patient access to registry data, PROM data capture
SESSION 3: Introducing conect4children and its data standards work, and key projects working towards the realisation of the EHDS	
14:30 – 15:00	**Overview of the c4c project and its future as c4c-Services**
15:00 -15:20	**Data-related achievements of c4c to-date** – Rebecca Leary, Victoria Hedley, Claudia Pansieri
15:20 - 16:00	**How will the EHDS impact ERN registries?** – Representative of the xPanDH or xShare projects and European Commission team (TBC)
16:00 -16:30	**Coffee break**

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SESSION 4: Leveraging data-related opportunities	
16:30 - 17:20	**Breakout Session:** **The major bottlenecks and how to tackle them** a) Technical challenges (data acquisition, interoperability, etc.) b) Legal frameworks c) Sustainability
17:20 - 18:45	**Feedback in Plenary ('30 total)** **Full Group Discussion: How can we best apply data standards and FAIR 'best practices' to the ERN registries, and leverage data science, to make our registries most powerful?** • What standards should we be embedding in our registries, and how, to make data FAIR and allow it to reflect the needs of rare and paediatric populations? • How might the EHDS and its various tools really apply to ERN registries? • Who needs to do what, where and when?
18:45	**Day 1 ends**

Networking Dinner 20:00 (TBC)

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5th February

08:00 - 08:30	**Welcome and registration**
8:30 - 8:45	**Recap of Day 1**
SESSION 5 - Research applications of the ERN Registries and initial public-private case studies	
8:45 – 9:15	**How have ERN registries been supporting research?** Success stories from ERN registries
9:15 - 9:30	**Eroding barriers to ERNs working with Industry – how Together4RD is fostering collaboration** – Sheela Upadhyaya
9:30 – 10:30	**Use of ERN and other Rare Disease registries to support medicines development** – Case studies, including: • Together4RD Pilots • Use of registry data for PIPs (ERKReg) • Case studies from outside of ERN space (TBC)
10:30 -11:00	**Coffee Break**
11:00 -11:30	***Case studies cont.***
SESSION 6: Considerations for using ERN registries to generate regulatory-grade real-world evidence	
11:30 - 11:55	**Industry perspectives (c4c)**
11:55 – 12:45	**Methodological and practical considerations around the use of patient registries for regulatory purposes**
12:45 – 13:45	**Lunch**
13:45 - 14:00	**Data Access Policies of ERN registries** – Jose Ramírez-García

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14:00 - 16:00	**Group work in break-out sessions – assessing current aspirations and challenges to using ERN registries to support medicines development, and identifying solutions** • Plenary Feedback • Final Discussions on next steps
16:00	**End of the workshop**

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