35th Workshop of the
EURORDIS Round Table of Companies (ERTC)
22 February 2023
09.30-17.00 CET
Doubletree by Hilton Brussels City in Brussels and ONLINE

Bringing Clinical Trials into the Future

Overarching objective and scope for the workshop

EURORDIS-Rare Diseases Europe, along with the broader community of patient organisations, have been on a journey to shape the European policy landscape to bring more and better treatments for rare disease patients. From advocating for the EU Regulation on orphan medicinal products (1997) to the EU Directive on Patients' Right to Cross-Border Healthcare (2011), the rare disease community has continued to lead collective efforts towards the adoption of legislations impacting lives.

Another case in point is the evolution of the Clinical Trials Regulation, which will profoundly change the way trials are conducted in Europe and improve further healthcare delivery pathways for rare disease patients. **This workshop is set to evaluate the potential and ambition of this legislation and its implementation, one year after it came into effect in Europe, and to appreciate the benefits and challenges of this new approach to clinical practice for developers, patients and regulators.**

Context for the Clinical Trials Regulation

The Clinical Trials Regulation (EU No 536/2014) was initially published in 2014 to ensure greater harmonisation of the assessment and supervision rules for conducting clinical trials throughout the EU. It introduces an authorisation procedure for sponsors to submit one application to run a clinical trial in multiple countries, and it enables national competent authorities to take a single decision, via the Clinical Trials Information System (CTIS), a clinical trial portal that was launched on 31st January 2022.

Prior to the Regulation, sponsors had to submit individual applications to national competent authorities and ethics committees to obtain regulatory approval to run a clinical trial.

This legislation aims to ensure that the EU offers an attractive and favourable environment for carrying out clinical research on a large scale and provides an easier framework for conducting multinational trials. With the implementation of more robust standards of public transparency and safety for clinical trial participants, it also aims to increase the number of

studies conducted within the EU and represents a turning point for the future of medicines development.

Objectives

This workshop will provide participants with valuable information on the following points:

- **Get insights into the European legislative environment** that is shaping clinical trials;
- **Explore the strategy for Accelerating Clinical Trials in the EU (ACT-EU)** and better appreciate which political actions are taken towards reaching this goal;
- **Learn about key public initiatives and public-private partnerships** that are contributing to the ACT-EU objectives in developing and implementing innovative approaches to clinical trials;
- **Understand which guidelines and recommendations are being developed to inform clinical trial practice** as it evolves (e.g. recommendations for decentralized trials, ICH guidelines for clinical trials, etc);
- **Discuss how rare disease patients will benefit** from advancements in clinical trials;
- **Contribute to building collective knowledge** by sharing your experiences and challenges throughout the day alongside peers.

PROGRAMME

Co-Chairs:
Álmath Máire Spooner, Director of Regulatory Policy and Intelligence, AbbVie
Avril Daly, Chief Executive Officer, Retina International & President, EURORDIS - Rare Diseases Europe

9.00 – 9.30	**REGISTRATION**
9.30 - 9.45	**Welcome & Introduction by the Co-Chairs**
9.45 – 10.00	**Setting the scene** **Yann Le Cam,** Chief Executive Officer, EURORDIS-Rare Diseases Europe
10.00 – 10.30	**1st Anniversary of the Launch of the Clinical Trials Information System (CTIS)** **Peter Arlett,** Head, Data Analytics and Methods Task Force, European Medicines Agency
10.30-11.30	**Feedback from sponsors on their experience with the CTIS** **Stéphanie Kromar,** Head of Regulatory Affairs Department, European Organisation for Research and Treatment of Cancer (EORTC)

	Louise Cassidy, Director, GRA Development Strategy, Clinical Trial Excellence**,** Alexion Pharmaceuticals **Floriane Courbard-Nicolle,** Director, Global Regulatory Strategy, Sarepta Therapeutics **Christopher Bamford,** Director, Regulatory Affairs, IQVIA
11.30-11.45	**Comfort break**
11.45-12.00	**The Regulatory Context: Implementing the Clinical Trials Regulation** **Sylvain Giraud**, Head of Unit DG SANTE D2 Medical Products, European Commission
12.00-13:00	**From political commitment to implementation** **DG – Research: Catherine Berens,** Deputy Head of Unit 'Combatting Diseases', European Commission **Public-Private Partnerships: Magda Chlebus,** Executive Director Science Policy & Regulatory Affairs, EFPIA **RD Partnership: Roseline Favresse**, Director, Research Policy and Initiatives, EURORDIS – Rare Diseases Europe
13:00-14.30	**LUNCH**
14.30-15:15	**Innovative approaches to Clinical Trials in Public-Private Partnerships** **EU-PEARL: Cecile Spiertz,** Senior Director, Clinical Trial Platforms at The Janssen Pharmaceutical Companies of Johnson & Johnson **conect4children (c4c): Heidrun Hildebrand,** Paediatric Development Alliance Manager, Bayer AG, Research & Development, Pharmaceuticals **Trials@Home: Sonia Houston Pichardo,** Product Owner, MyStudyWindow.com, Boehringer Ingelheim
15:15 – 15:30	**Recommendations for Decentralised Clinical Trials** **Ditte Zerlang Christensen,** Project Manager, Danish Medicines Agency
15:30 – 15:50	**Comfort Break**
15:50 – 16:35	**Patient-led moderated discussion/Q&A with audience** **Julián Isla-Gómez,** Founder, Foundation 29 **Michela Onali,** Stakeholder Manager, MetabERN
16:35 – 16:50	**ICH Guidelines for Clinical Trials** **François Houÿez,** Information & Access to Therapies Director & Health Policy Advisor, EURORDIS – Rare Diseases Europe
16:50-16:55	**Key takeaways from a healthcare corporate** **Mireille Muller**, Regulatory Policy Executive Director, Novartis
16:55-17:00	**Wrap-up and next steps by the Co-chairs**


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