32nd Workshop of the EURORDIS Round Table of Companies (ERTC)

Get ready for imminent changes!

The impact of the EU regulatory network strategy 2020-2025 on the development of orphan medicines in a rapidly evolving healthcare environment

Overarching theme: The European Medicines Agencies Network Strategy to 2025 and Regulatory Science Strategy to 2025 are forming a comprehensive approach to plan and prepare for the future. This includes preparing the EU regulatory network for the next generation of regulatory activities. To do this, four EMA Task Forces have been created on: Digital Business Transformation, Data Analytics and Methods, Regulatory Science & Innovation and Clinical Studies and Manufacturing.

The 32nd EURORDIS Round Table of Companies Workshop will place a strong emphasis on exploring and discussing elements of these task forces, and their impact and importance for developers of rare disease treatments.

This is your opportunity to join us to discuss and gain insights into new methods to analyse data, new sources of data and changes in the way new Clinical Trials are conducted.

Workshop objectives:

This workshop will bring together participants spread across pharmaceutical, biotech and clinical research organisations (CRO) together with patient advocates, regulators, HTA agencies, healthcare professionals and academics with the following objectives:

- **Get insights** into the future of the regulatory strategy for data acquisition and analysis
- **Learn** about the new EMA task forces established to prepare the regulatory network for modern methods
- **Understand** how all stakeholders can prepare and plan for the future and benefit from the changes
- **Hear from** regulators, HTA agencies and healthcare corporates about the results/evaluations of adaptive trials along with their impact on HTA, pricing and reimbursement
- **Network** with peers and other rare disease community stakeholders and share your own developments in the field of clinical trials
- **Learn** how clinical trials will be conducted in the next 20 years with the revision of the ICHE6
- **Understand** how patients will benefit from shorter and more innovative clinical trials, more robust HTA assessments, new digital solutions to measure efficacy and how more real-world evidence data will help to reduce risk and make decisions more certain

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EURORDIS Round Table of Companies

PROGRAMME

20 - 21 October 2021

DAY 1: The future of the regulatory strategy for Science & Innovation, Data Analytics & Methods

Wednesday, 20 October 2021

14.00-18.00 CET

Co-Chairs:

Hans-Georg Eichler, Consulting Physician, Association of Austrian Social Security Bodies

Russell Wheeler, Volunteer Patient Advocate at ERN-EYE, EURORDIS and Leber's Hereditary Optic Neuropathy Society

OPENING SESSION

14.00-14.05	**DAY 1 Facilitator**: **Bruno Selun,** Kumquat Consult
14.05-14.10	**Welcome introduction, setting the scene & goals for the workshop** **Yann Le Cam**, Chief Executive Officer, EURORDIS-Rare Diseases Europe
14.10-14.20	**Small group introductions**

SESSION 1: Task Force on Regulatory Science & Innovation

14.20-14.30	**Task force on Regulatory Science & Innovation** Presentation of objectives & main work streams **Ralf Herold,** Senior Scientific Officer, European Medicines Agency (EMA)

14.30-14.55	**Multi-stakeholder discussion** *Moderator:* **Hans-Georg Eichler**, Consulting Physician, Association of Austrian Social Security Bodies *Discussants*: • **Tomasz Grybek**, EURORDIS' Therapeutic Action Group (TAG) & Member of European Medicine Agency's Paediatric Committee • **Martine Zimmermann**, Senior Vice President, Global Regulatory Affairs, Alexion • **Sheela Upadhyaya**, Rare Disease & RAPID C-19 Strategic Advisor, The National Institute for Health and Care Excellence (NICE UK)
14.55-15.10	**Q&A**
15.10-15.25	**Small group discussions**
15.25-15.30	**Highlights from small group discussions**
15.30-15.50	COMFORT BREAK

SESSION 2: Task Force on Data analytics and methods

15.50-16.00	**Task Force on Data analytics and methods** Presentation of objectives & main work streams **Frank Petavy**, Head of Methodology, Data Analytics and Methods Task Force at European Medicines Agency (EMA)
16.00-16.25	**Multi stakeholder discussion** *Moderator:* **Russell Wheeler**, Volunteer Patient Advocate at ERN-EYE, EURORDIS and Leber's Hereditary Optic Neuropathy Society *Discussants:* • **Teresinha Evangelista**, Coordinator, ERN EURO-NMD • **Pablo Botas**, Chief Executive Officer and Chief Scientific Officer, Foundation 29 • **Katharina Tomala**, Regulatory Affairs, Corporate, Boehringer Ingelheim International GmbH
16.25-16.40	**Q&A**

16.40-16.50	**Data Analysis and Real-World Interrogation Network (DARWIN EU)** Introduction to the European Medicines Agency (EMA) coordination centre **Francois Domergue,** Scientific Administrator, European Medicines Agency (EMA)
16.50-17.05	**Small group discussions**
17.05-17.15	**Highlights from small group discussions**
17.15-17.30	**COMFORT BREAK**

CLOSING SESSION

17.30-17.45	**Personal reflection**
17.45-18.00	**Wrap-up, key messages and looking ahead to tomorrow** **Hans-Georg Eichler,** Consulting Physician, Association of Austrian Social Security Bodies // **Russell Wheeler,** Volunteer Patient Advocate at ERN-EYE, EURORDIS and Leber's Hereditary Optic Neuropathy Society

DAY 2: Clinical Studies of the Future

Thursday, 21 October 2021

14.00-18.00 CET

Co-Chairs:

Hans-Georg Eichler, Consulting Physician, Association of Austrian Social Security Bodies

Russell Wheeler, Volunteer Patient Advocate at ERN-EYE, EURORDIS and Leber's Hereditary Optic Neuropathy Society

OPENING SESSION

14.00-14.05	**DAY 2 Facilitator**: **Bruno Selun,** Kumquat Consult

SESSION 1: Task Force on Clinical Studies

14.05-14.15	**Task force on Clinical Studies** Presentation of objectives & main work streams **Pieter Vankeerberghen,** Head, Clinical Trials, European Medicines Agency (EMA)
14.15-14.40	**Multi stakeholder discussion** *Moderator:* **Russell Wheeler,** Volunteer Patient Advocate at ERN-EYE, EURORDIS and Leber's Hereditary Optic Neuropathy Society *Discussants:* • **Jeff Keefer,** Head, Paediatric and Rare Disease Centre of Excellence, IQVIA • **Toni Mathieson,** CEO, Niemann-Pick UK • **Toni Andreu,** Scientific Director, EATRIS
14.40 -14.55	**Q&A**
14.55 – 15.10	**Small group discussions**
15.10 – 15.15	**Highlights from small groups discussions**
15.15-15.35	**COMFORT BREAK**

Session 2: Revision of the ICH E6 & Adaptive Designs in Clinical Trials

15.35-15.55	**Introduction to the revision of the ICH E6** **Fergus Sweeney,** Head of Clinical Studies and Manufacturing, European Medicines Agency
15.55-16.20	**Multi stakeholder discussion** *Moderator:* **Hans-Georg Eichler,** Consulting Physician, Association of Austrian Social Security Bodies *Discussants:* • **Francois Houÿez,** Information & Access to Therapies Director & Health Policy Advisor, EURORDIS

	• **Jeanette Doorduijn**, ErasmusMC, Rotterdam; Member of European Hemotology Association's Clinical Trials Expert Group
16.20-16.30	**COMFORT BREAK**
16.30-16.40	**Outcomes of Adapted Clinical Trials** **Martin Posch**, Centre for Medical Statistics, Informatics, and Intelligent Systems, Medical University of Vienna
16.40-16.50	**Experiences and Reflections from a Healthcare Corporate** **Duncan Nickless,** Director, Global Regulatory Leader, Janssen
16.50-17.00	**Experiences and Reflections from an HTA Agency** **Anja Schiel**, Special Advisor, Lead Methodologist, Norwegian Medicines Agency
17.00-17.10	**Reactions and Reflections from a Patient Advocate** **Russell Wheeler**, Volunteer Patient Advocate at ERN-EYE, EURORDIS and Leber's Hereditary Optic Neuropathy Society
17. 10-17.25	**Q&A**

CLOSING SESSION

17.25-17.35	**Personal reflection**
17.35-17.40	**Key take home messages from a healthcare corporate** **Sandra Paci,** Director of Market Access and Patient Advocacy for Europe, argenx
17.40-18.00	**Wrap-up, key messages and next steps** **Hans-Georg Eichler,** Consulting Physician, Association of Austrian Social Security Bodies // **Russell Wheeler**, Volunteer Patient Advocate at ERN-EYE, EURORDIS and Leber's Hereditary Optic Neuropathy Society