



The European Organisation for Rare Diseases, EURORDIS, is a patient-driven alliance of patient organisations and individuals active in the field of rare diseases.

Our vision is to enable **better lives and cures for people living with a rare disease.**

Our mission is to **work across borders and diseases to improve the lives** of people living with a rare disease

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738 rare disease patient organisations
in **65** countries covering over **4000**
diseases.

- 38 National Alliances of RD Patients Organisations
- 55 European Federations of specific rare diseases
- Outreach to over 1800 patient groups
- 35 staff, offices in Paris, Brussels and Barcelona with also Geneva, Zagreb, Belgrade
- Over 320 Volunteers: +80 patient advocates and +250 moderators

Webinar and Workshops

EURORDIS Webinars:

EURORDIS webinars take place to update member organisations or the wider rare disease community on a new activity, to engage participants in a consultation or to educate on a new topic that is relevant to rare disease patients, such as genome editing. They are designed to be interactive so that participants can ask questions via a chatbox or their microphone!

WATCH THE RECORDINGS: <http://www.eurordis.org/content/eurordis-webinars>

- **GENOME EDITING:**

- EURORDIS webinar (October 2016) aimed to start informing and engaging rare disease patient representatives on the topic of genome editing.
- Webinar followed by a face to face workshop EURORDIS workshop on gene therapy and genome editing now available to watch on <http://www.eurordis.org/tv>.
- The workshop brought together patient representatives from across Europe with experts to discuss the scientific progress of these technologies and their relevance for rare diseases, as well as associated ethical, legal and social issues. Watch part 1 (What is genome editing? The science and beyond), part 2 (Ethical, legal and social issues) and part 3 (Perspectives of the biotech sector and regulatory aspects).

- **THE NEW GENERAL DATA PROTECTION REGULATION (GDPR)**

- **Social Services in the context of national plans for rare diseases (RD ACTION)**

- **ePAG Webinar on Virtual healthcare and data sharing in an ERN**

- **consultation on position paper on access to rare disease medicines**

- **AND MANY MORE...**

Capacity Training Programmes

EURORDIS Summer School

- Capacity building programme started in 2008
- Multi-stakeholder Programme Committee (EMA, LUMC, Catalan RD platform)
- Therapeutic Development & beyond – Emphasis on Patient engagement all along the life cycle of product development
- On-line pre-training modules
 - 5-day annual on-site training on:
 - Clinical Research – Methodology, Ethics, etc
 - EU Regulatory Processes – EMA & patient engagement
 - HTA & Reimbursement – national opportunities
 - Networking

EURORDIS Summer School

- **First Training Programme with Patients and Researchers being trained together** (new format since 2015) sharing experiences & peer-to-peer training
- **Blended approach** with plenary/formal lectures, small groups sessions, round table discussions and hands-on/practical approach
- **ExPRESS 2016:** 33 patient representative & 11 academic researcher participants



- So far, **300 patient representatives** have been trained, coming from **40 different countries** and representing more than **70 diseases**
- Alumni involved in regulatory processes **at the EMA** and/or **in collaboration with sponsors** and/or as **EURORDIS' Volunteers**

Plan for EURORDIS Winter School (2018)

- Training Programme on Scientific Innovation & Translational Research in partnership with others (New project Solve-RD)
- 5-day annual on-site training (supported by e-learning) on:
- Overview of the Research landscape/environment and opportunities
- Diagnosis (history, state-of-the art, interpretation, ethics)
- Data (data collection, interoperability, data sharing and protection)
- Therapies (animal models & alternatives, data mining/repurposing, success in gene/cell T)
- Care (ERNs)
- Visits to sequencing lab, bioinformatics institute and animal facilities

ePAG Leadership Programme

A capacity-building programme for ePAG representatives to provide them with skills and knowledge to effectively engage in ERN governance

