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1. Introduction and Objectives of the training

In 2008, EURORDIS created the EURORDIS Summer School (now Open Academy School on Medicines Research & Development) in recognition of patients' needs for support and training that includes an overview of clinical research and methodology, medicines development and regulatory procedures. Specific needs were first identified by expert patients, EURORDIS, the European Medicines Agency, academic researchers and industry.

The School consists of a face-to-face 5-day course held in Barcelona annually. It aims to provide patients with the knowledge and skills needed to understand the basics of medicines research and development, regulatory affairs and access to medicinal products and the role patients have in ensuring access to the best treatments available as well as what role they can have in influencing the development of new therapies. A selected group of researchers also attend the training to enable them to understand the needs of patients in early stages of their research as well as to understand the regulatory procedures that are needed for treatments to reach markets.

The programme allows patients and researchers to sharpen their advocacy skills and gain an understanding of the regulatory processes of therapeutic research and development, so that they can advocate at a European level. It was demonstrated that joint training of patients and researchers is of great benefit for both stakeholders, helping to raise awareness and sharpen advocacy skills. Joint training enables both stakeholders to become more active in this

ecosystem. The 15.2 EJP RD task supported the human resources necessary to organize, develop and carry out all activities as well as the operational support needed to ensure participation of researchers.

2. Training programme: definition of training content and methods

The programme has online pretraining (available for free to anyone) and face-to-face training components. The face-to-face portion trains a group of expert patients jointly with researchers annually as part of an intensive 5-day course held in Barcelona, Spain; or 5 half-days online for the exceptional years of 2020, 2021 and 2022. Over 20 trainers provide the training each year. The training of researchers alongside patient representatives allows researchers to truly understand the unmet needs and priorities of patients in terms of new therapies in a unique classroom setting including up to 30-35 patient representatives and 10 researchers. It allows them to position their future research based on clearly identified needs expressed by patients. Researchers are selected based on their interest in working closely with patients to develop new treatments adapted to their needs and priorities.

3. Programme Committee and speakers

An informal Programme Committee supported the development of the programme for the period 2020-2021 and again for the period 2022-2023, via Programme Committee calls and ad-hoc consultations. Members of this Programme Committee included EJP partners as well as other experts in medicine research and development, notably: patient advocates (EURORDIS), researchers (LUMC), representatives of the European Medicines Agency and a representative of the Medicines and Healthcare Products Regulatory Agency.

As there have been 11 previous editions, the faculty of this training programme is mostly recurrent. Some new faculty members were identified specifically for the most recent editions, with the support of the Programme Committee experts and of other faculty members.

4. Participants: applications and selection method

The applications for the School take place in September/October/November. Participation is usually limited to 30-35 patient advocates and 10 researchers, whilst additional applicants are put on a waiting list. 2-3 patient advocates based outside of Europe are also permitted to attend as observers.

The application form is composed of over questions divided into the following main areas:

- Personal information, including disease/country represented and roles as patient advocate/researcher.
- Experience/knowledge in the training areas, previous training experience and motivation to attend the training.

- Information about the applicant's organisation and its relations to national alliances/European federations for rare diseases.
- Agreement to fully attend pre-training and face-to-face session.
- Consent for data processing in the scope of the review of applications.

The applications' review of this EURORDIS Summer School consisted of 3 phases:

- Exclusion of non-eligible applications (e.g., non-EU; no affiliation to patient organizations).
- Scoring of applications (from 1-5 based on the main sections underlined above) by EURORDIS staff working with therapeutic development, research and training; researchers' applications were also reviewed by Professor Aartsma-Rus from Leiden University Medical Center (WP15.1 task partner).
- Final selection: selecting final list and waiting list from the highest scored applicants, considering also disease and country diversity.

5. Participants: profile

	2020	**2021**	**2022**	**2023**
Number of Participations	**34**	**33**	**22**	**38**
Number of patient advocate participations	27	27	18	31
Number of researcher participations	7	7	4	7
Number of Applications	**77**	**58**	**81**	**58**
Young Patient Advocates		**3**	**4**	**4**
Eastern European Countries, Balkan and Caucasus*	**5**	**8**	**5**	**6**
Number of countries	**16**	**19**	**16**	**25**
Nimber of diseases	**19**	**19**	**26**	**26**

6. Fellowships: process for attribution and results

5 fellowships are offered to patient advocates each year to attend the training in-person. This was not offered for the online editions.

The fellowship applications take place following the selection of attendees. The fellowships were attributed in accordance to the EURORDIS fellowship evaluation scale available here: Fellowship application evaluation scale_updated090922.docx

7. Pre-training

The pre-training allows trainees to familiarise themselves with the concepts and terminology that is used during the onsite training in Barcelona, in view of optimizing the onsite training and allowing for more fruitful discussions.

The pre-training takes place from February to May and includes 3 webinars and e-learning courses.

The 3 webinars served to introduce participants to the programme, to the pre-training and to important logistics information. Webinar recordings are shared with all the participants.

The following e-learning courses available online here: https://openacademy.eurordis.org/summer-school/ also make up the pre-training (composed of video presentations, reading materials and case studies). For example:

• Unit 1 Medical Research and Development: Course 1: Introduction; Course 2: Study design; Course 3: Controlled trial; Course 4: Randomization; Course 5: Endpoints; Course 6: Criteria of inclusion; Course 7: Analysis of results;

• Unit 2 Ethics in Medicines Development: Course 1: Introduction; Course 6: Informed Consent; Course 7: Ethics Committee.

• Unit 4: Benefit-risk assessment and pharmacovigilance: Course 1: Patient involvement in benefit-risk at the EMA; Course 3: The Role of Patient Organizations in Pharmacovigilance; Course 4: Resources and reporting tools;

• Unit 5: The European Medicines Agency (EMA): Course 1: Introduction to the EMA; Course 3: Various EMA Sub-Organisations; Course 4: Patient Interaction with the EMA

• Unit 6: Medical Regulatory Framework and Procedures: Course 1: Introduction; Course 2: The Current Regulatory Framework; Course 3: Regulatory Procedures.

• Unit 7: Market access & HTA: Course 1: Introduction to HTA.

8. Training delivery: programme outline

The face-to-face training (or online for the years 2020, 2021 and 2022) takes place in June in Barcelona.

The 5-day programme includes a series of plenary interactive presentations, breakout sessions, work in small groups, and Q&A sessions with the faculty.

An example outline for the training week is as follows:

• Monday: Therapeutic development in RD – A multi stakeholder environment; Clinical Trials: Methodology, Design and Ethics.

• Tuesday: Clinical Trials: Methodology, Design and Ethics.

• Wednesday: European Medicines Agency (EMA) – Regulatory pathways for Orphan drugs and Patient Engagement; Pharmacovigilance.

• Thursday: From Health Technology Assessment to Pricing and Reimbursement.

• Friday: Actions that can be taken by you.

9. Training materials available

The e-learning courses used for the pre-training and additional courses on medicine research and development are available online here: https://openacademy.eurordis.org/summer-school/ . The pre-training webinar recordings and presentations (including recordings in the case of the online editions) were made available to the participants after the training. These are available to EJP partners on demand.

10. Evaluation: methodology and results

The evaluation of the programme is conducted via feedback questionnaires, filled in online and anonymously. At the end of the training day, participants receive a short questionnaire via which they are required to indicate, for each presentation, if it was "informative", "relevant" and "too technical". On the last training day, participants also receive the overall feedback questionnaire, including questions on the impact/value of the training, on the pre-training and on the preparation/logistics. In 2022, we adapted the questionnaire to be sent only after the full training, which increased the response rate and to adapt to changes in the programme.

	2020	**2021**	**2022**	**2023**
% of participants who recommend the OA's schools	**100%**	**100%**	**100%**	**100%**
Rate of statisfaction with the OA's schools: I am satisfied with my experience of the OA Schools				
Strongly disagree	0%	0%	0%	4%
Disagree	0%	0%	0%	0%
Neither Agree nor disagree	0%	0%	0%	0%
Agree	26.09%	22.00%	25%	9%
Strongly agree	73.91%	78.00%	75%	87%
Rate of response to evaluation questionnaires	**68%**	**79%**	**77%**	**84%**

Quotes on participants' key takeaways from the training:

"The motivation to do much more for my organisation and for the European organisation of my disease"

"I have much more idea of how it works overall and though I may not remember it all I will know where I need to go for the information."

"A lot of knowledge about clinical trials and the people involved. This event was again great for networking!"

"Patients are important at every step of medicine development"

"More confidence as a patient advocate"

"The people I have met, with whom I will be able to have synergies and collaborations in the near future. In addition, hearing the voice of patients and their families and the problems they face helps to think about rare disease research from a different perspective."

"This is what patients need to be heard. Skilled patients have great value and are needed for pharma companies and physicians"

11. Dissemination

The applications for the trainings were widely disseminated via EURORDIS partners, social media as well as via the EJP's communication tools and partners.

12. Conclusion and Next Steps

The next edition of the School will take place in June 2024 during the extension period of the EJPRD project in the same in-person format as 2023.

Applications took place September-October 2023 and generated 102 potential candidates. We are in the final stages of finalizing the participant list and the pre-training stage will start in January.

We will introduce several changes as of 2025 with the advent of the ERDERA project, including different levels to the training (basic, intermediate, advanced), different tracks for participants to follow, translation of e-learning courses, a more blended format with more online elements to increase accessibility and more interactive in-person sessions. The advanced level will eventually entail access to a buddy/mentoring programme.