

## EJP RD

# European Joint Programme on Rare Diseases

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## Del 15.1

# First Report on ExPRESS

**Organisation name of lead beneficiary for this deliverable:**

Partner 77 – EURORDIS

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**Dissemination level:**

PU

## 1. Introduction and Objectives of the training

*Based on EJP application and updated description for year 2 WP:*

One of the ultimate aims of research is to bring innovative, life-transforming therapeutic options to patients who are also taking on ever increasing roles in advocating for medicines development, equal access to treatments across Europe and ensuring that medical information is clear, accurate and comprehensible.

In 2008, EURORDIS created the EURORDIS Summer School (ExPRESS Expert Patients and Researchers) in recognition of patients' need 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 EURORDIS Summer School is an annual training programme. The face-to-face course trains expert patients and researchers as part of an intensive 4,5-day course held in Barcelona. Over 20 EURORDIS Summer School trainers provide the training each year.

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 are able to 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.

This task will support the human resources necessary to organize, develop and carry out all activities as well as the operational support needed to ensure participation of researchers. The EJP for RD's contribution was requested pro-rata thus leveraging on complementary and diverse sources of funding raised by EURORDIS.

## 2. Training programme: definition of training content and methods

The 2019 edition of the ExPRESS Expert Patients and Researchers training, currently entitled EURORDIS Summer School, built on the programme developed over 11 previous training sessions.

Since 2008, specific training needs and speakers have been identified by expert patients, EURORDIS, the European Medicines Agency, academic researchers and industry in order to shape the training programme.

The programme has been updated and improved along the years, according to the feedback of participants and to the latest developments in the area of medicines research and development.

### 3. Programme Committee and speakers

An informal Programme Committee has supported the development of the programme in 2019, via 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.

Over 20 EURORDIS Summer School trainers provide the training each year. 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 2019 edition, with the support of the Programme Committee experts and of other faculty members.

In October 2019, a formal Programme Committee for the EURORDIS Summer School has been established, to support the upcoming editions (2020-2022). This Programme Committee will take part in two annual Programme Committee calls, in view of reviewing the draft training programme and suggesting speakers or content to help address any identified gaps in the programme/faculty.

### 4. Participants: applications and selection method

The applications for the EURORDIS Summer School 2019 took place in October-November 2018. EURORDIS received a total of 107 applications, including 92 patient advocates and 15 researchers.

30 patient advocates and 10 researchers were initially selected to attend, while additional 5 of each were put on waiting list. 2 patient advocates based outside of Europe were also originally selected to attend as observers.

The application form of the EURORDIS Summer School 2019 was composed of over 30 questions/fields, 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 organisations);
- 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

34 participants attended the EURORDIS Summer School in 2019, including 27 patient advocates and 7 researchers. These represented 16 countries and over 25 rare diseases. In addition, 2 EURORDIS staff also took the course.

The countries represented included: Austria, Belgium, Croatia, Cyprus, France, Germany, Italy, Netherlands, North Macedonia, Norway, Romania, Spain, Turkey, United Kingdom and USA.

Rare diseases represented included e.g.: Acrodysostosis, Aplastic Anemia, Bronchiectasis, Cystic Fibrosis, Dravet Syndrome, Duchenne Muscular Dystrophy, Epidermolysis Bullosa, Familial Adenomatous Polyposis, Gaucher type III, Glycogen Storage Disease Type 2 (Pompe), Limb Girdle Muscular Dystrophy, Lupus, Lynch syndrome, Multiple Myeloma, Myotubular Myopathy, Neurofibromatosis, Norrie Disease, Osteogenesis Imperfecta, Paroxysmal Nocturnal Hemoglobinuria, Prader–Willi Syndrome, Primary Ciliary Dyskinesia, Primary Sclerosing Cholangitis, Rare Autoimmune Inflammatory Diseases, Short Stature, Spinal Muscular Atrophy and Williams Syndrome.

## 6. Fellowships: process for attribution and results

5 patient advocates received a fellowship to attend the EURORDIS Summer School 2019. These come from France, Italy, North Macedonia and the Netherlands.

The fellowship applications took place in April 2019. The fellowships were attributed in accordance to the EURORDIS [fellowship evaluation scale available here: https://openacademy.eurordis.org/wp-content/uploads/2019/07/EURORDIS-Open-Academy\\_Fellowship-application-evaluation-scale.pdf](https://openacademy.eurordis.org/wp-content/uploads/2019/07/EURORDIS-Open-Academy_Fellowship-application-evaluation-scale.pdf).

## 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 optimising the onsite training and allowing for more fruitful discussions.

The pre-training for the EURORDIS Summer School 2019 took place from March to May 2019 and included 3 webinars and 20 e-learning courses.

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

The following 20 pre-training e-learning courses [available online here https://openacademy.eurordis.org/summerschool/](https://openacademy.eurordis.org/summerschool/) (composed of video

presentations, reading materials and case studies) were mandatory for the participants of the Summer School 2019:

- [Unit 1 Medical Research and Development](#): Course 1: Introduction; Course 2: Study design; Course 3: Controlled trial; Course 4: Randomisation; 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 Organisations 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 of the EURORDIS Summer School 2019 took place on the 10th to 14th of June 2019, in Barcelona.

The 4,5 day programme included a series of plenary interactive presentations, breakout sessions, work in small groups, and Q&A sessions with the faculty.

The outline for the Summer School 2019 onsite programme was the following:

- Mon, 10: Therapeutic development in RD – A multi stakeholder environment; Clinical Trials: Methodology, Design and Ethics;
- Tues, 11: Clinical Trials: Methodology, Design and Ethics;
- Wed, 12: European Medicines Agency (EMA) – Regulatory pathways for Orphan drugs and Patient Engagement; Pharmacovigilance;
- Thurs, 13: From Health Technology Assessment to Pricing and Reimbursement;
- Friday, 14: Actions that can be taken by you.

The [full programme for the 4,5 days training is available here](https://openacademy.eurordis.org/wp-content/uploads/2019/05/EURORDIS-Summer-School-2019_Programme-1.pdf):  
[https://openacademy.eurordis.org/wp-content/uploads/2019/05/EURORDIS-Summer-School-2019\\_Programme-1.pdf](https://openacademy.eurordis.org/wp-content/uploads/2019/05/EURORDIS-Summer-School-2019_Programme-1.pdf).

## 9. Training materials available

The e-learning courses used for the pre-training and additional courses on



medicine research and development (total of 25 courses) are [available online here: https://openacademy.eurordis.org/summerschool](https://openacademy.eurordis.org/summerschool). The pre-training webinar recordings were shared with all the participants.

All the presentations of the EURORDIS Summer School were made available to the participants ahead of the training and to all the faculty. These presentations 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 each 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.

The rate of response to the evaluation questionnaires in 2019 was of 88%. The main results include:

- 100% of the respondents stated that they would recommend the Summer School to other patient representatives and researchers;
- 96,2% have found that the EURORDIS Summer School effectively builds the capacity of patient advocates on Medicines Research & Development (85,71% strongly agreed, 10,71% agreed);
- 100% have found the topics relevant for their activity as patient advocate/researcher (50% strongly agreed; 50% agreed);
- 93% considered that the training provided them with essential knowledge and tools to support them in representing/engaging patients in activities related to Medicine Research & Development e.g. clinical trials, regulatory affairs, health technology assessment (60,71% strongly agreed; 32,14% agreed).

The report on the feedback from participants is being used to improve the programme for the 2020 edition.

## **11. Dissemination**

The applications for the EURORDIS Summer School were widely disseminated via EURORDIS media and social media as well as via the EJP's communication tools. A series of social media posts were disseminated during the training, under the hashtag #EURORDISummerSchool.

## **12. Conclusion and Next Steps**

The face-to-face training of the EURORDIS Summer School 2020 will take place on 8 to 12th of June 2020 in Barcelona.

The applications for the 2020 edition took place in October-November 2019. The selection process will be concluded in January 2020 and the first pre-training webinar will take place in February 2020.

The programme for the 2020 edition has been reviewed in October-November 2019, considering the feedback from participants and of the recently established Programme Committee.



**Indicators**

WP	Action/Activity	Result	Indicator	How is the indicator measured?	Target/threshold	Results Year 1, 2019	Indicator type	Who measures the indicator?	Who reviews the Indicator
WP 15.1	Training delivery	Pre-training and training webinars and online courses	All planned webinars and online courses	Number of webinars and courses actually delivered	2 webinars 2 courses	3 webinars 20 courses	KRI	EURORDIS Open Academy Director	Pillar 3 and WP15 leader (EURORDIS Scientific Director)
WP 15.1	Training quality	Programme Committees engagement	All planned consultations with Programme Committees	Number of calls, email consultations, etc. to Programme Committees	2 calls 1 email consultation	<i>To start from 2020</i> 3 ad-hoc calls with PC members in 2019	KRI	EURORDIS Open Academy Director	Pillar 3 and WP15 leader (EURORDIS Scientific Director)
WP 15.1	Training quality	Programme Committees contribution to training programmes	All recommendations implemented in online and/or face to face training courses	Number of reviews of the programmes integrating programme committees' feedback	1 annual review	1 annual review	KPI	EURORDIS Open Academy Director	Pillar 3 and WP15 leader (EURORDIS Scientific Director)
WP 15.1	Training quality	Review of pre-training and online courses according to alumni's feedback	All planned evaluation and annual follow-up with participants	Rate of satisfaction of participants regarding usefulness of the pre-training ( <i>Q: The pre-training was useful to prepare for the Summer School. Rate of satisfaction combines "Strongly Agree"/"Agree"</i> )	60%	76%	KPI	EURORDIS Open Academy Director	Pillar 3 and WP15 leader (EURORDIS Scientific Director)
WP 15.1	Training impact	Review of impact of the training on individual participants	All planned evaluation and annual follow-up with participants	Percentage of participants considering that the training effectively builds their capacity and/or provides essential knowledge and tools for their own activities ( <i>Q: The Summer School effectively builds the capacity of patient advocates on Medicine Research &amp; Development. Rate combines "Strongly Agree"/"Agree"</i> )	70%	96%	KRI	EURORDIS Open Academy Director	Pillar 3 and WP15 leader (EURORDIS Scientific Director)