Pre-Training Programme
ExPRESS 2017
Expert Patients and Researchers
EURORDIS Summer School

Pre-Training Programme:

Unit 1: Medical research & development. **Webinar 17:00 (CET) February 08, 2017**
Unit 2: Ethics in medical research & development **Webinar 17:00 (CET) February 22, 2017**
Unit 3: Statistics in medical research **Webinar 17:00 (CET) March 08, 2017**
Unit 4: Regulatory Procedures. **Webinar 17:00 (CET) March 22, 2017**
Unit 5: European Medicines Agency. **Webinar 17:00 (CEST) April 05, 2017**
Unit 6: Pharmacovigilance and Benefit Risk. **Webinar 17:00 (CEST) April 19, 2017**
Unit 7: Market Access and Health Technology Assessment. **Webinar 17:00 (CEST) May 03, 2017**

INTRODUCTION

The EURORDIS Summer School pre-training programme is to allow trainees to familiarise themselves with the concepts and terminology that will be used during the onsite course in Barcelona. This is expected to optimise the onsite training and provide more time for discussion

On the following pages, you will find the programmes for each of the pre-training units with links to the content and the questions for the end of unit quizzes.

Each Unit will be launched with a webinar and end with an "End of Unit" quiz. Trainees can view the webcast, slide presentations and interactive modules at their own pace. You will receive a link to connect to the webinar the day before it is programmed. During the webinar you will be able to ask question (on an online forum). Students who are not available for the webinar launches will be able to watch recorded versions.

There is no pass or fail evaluation for the end of unit quizzes. They are designed to allow self-evaluation and to help trainees to assess their own level of competence. Trainees can review the pre-training Units and Quizzes as many times as they wish. However, all trainees must follow the pre-training and complete all 7 Units before they arrive in Barcelona.

The time and topics covered in the pre-training will be included on the "ExPRESS 2017 Certificate of Attendance" it is therefore very important that all trainees complete both the online and live sections of the course.

The helpdesk for the pre-training is nancy.hamilton@eurordis.org

The EURORDIS Summer School is a capacity-building programme for patient representatives & researchers on information and access to orphan, paediatric, advanced therapies and health technology assessment.

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RARE DISEASES INTERNATIONAL
A EURORDIS INITIATIVE

Unit 1: Medical Research and Development.

February 08, 2017

Webcast Link: http://www.eurordis.org/training-medical-research-webcasts 1 hour and 10 minutes of training	**How medical research develops into clinical drugs.** Markku Toivonen **Concepts covered:** **What is Medical Research? Learning and confirming. Basic research. Clinical research, Translational Research. Selection criteria, Inclusion Criteria. Observational, Interventional, Retrospective and Prospective Studies. Randomisation, blinding and other methodological issues.**
Webcast Link: http://www.eurordis.org/training-medical-research-webcasts 1 hour of training	**When and why to do Clinical Research.** Markku Toivonen **Concepts covered:** **Planning and designing clinical trials, blinding, placebo, endpoints, efficacy and safety. Cross-over trial, Study protocol, primary and secondary objectives. Outcome measures. Studies in small populations.**
Interactive Training Module Link: http://eurordis-elearning.s3.amazonaws.com/clinicaltrials/methodology/eurordis_e-learning_patients_representatives_clinical_trials/player.html 2.5 hours of training	**Module 1 – Methodology**
	End of Unit Quiz a) What does randomisation mean in the context of a clinical trial and what are some of the advantages? b) What is the difference between an adverse event and an adverse drug reaction? c) What are the different types of “blinding” in a clinical trial? d) What is a surrogate endpoint as opposed to a hard clinical endpoint?

Unit 2: Ethics in Medical Research

February 22, 2017

Reading 30 minutes of training	**Tuskigee experiment**
Webcast Link: **http://www.eurordis.org/training-ethics-webcasts** 1 hour and 10 minutes of training	**Ethical Aspects of Clinical Trials.** Eric Koster **Concepts covered:** **Informed consent, data safety monitoring committee, Helsinki declaration, Nuremberg code, Belmont report, Directive Council of Europe June 2014 on the treatment of subjects in clinical trials. Ethical review boards. Guiding principles: Autonomy, Beneficence, Non-malificence, Justice**
Interactive Training Module Link: http://eurordis-elearning.s3.amazonaws.com/clinicaltrials/ethics/eurordis_e-learning_for_patients_organisation_representatives_ethics_in_clinical_trials/player.html 3 hours of training	**Module 2 Ethics**
	End of Unit Quiz a) What are some of the ethical issue surrounding the Walter Reid trial on yellow fever? b) What principles were violated in the Tuskigee experiment? c) According to the Helsinki declaration, what is the primary reason for carrying out medical research on human subjects? d) Why is it important not to use humans as a means to an end? e) Who is responsible for the well-being of patients in a clinical trial? f) What is the Belmont Report? g) How can the rights of subjects in clinical trials be protected?

Unit 3: Statistics in Medical Research & Development

March 08, 2017

Webcast Link: http://www.eurordis.org/training-medical-research-webcasts 1 hour 30 minutes of training	**Introductory statistics for medical research.** Julia Saperia **Concepts covered:** **Population, sample, variability, mean, data, distribution, standard deviation, variables, confidence intervals, standard error, null hypothesis, blinding, statistically significant difference, p value, probability, power, http://www.consort-statement.org/** **http://openwetware.org/wiki/BMJ_Statistics_Notes_series**
Interactive Training Module Link: **http://eurordis-elearning.s3.amazonaws.com/clinicaltrials/statistics/eurordis-e-learning-patients-representatives-statistics-clinical-trials/statistics-clinical-trials/player.html** 3 hours of training	**Module 3 – Statistics**
	End of Unit Quiz a) True or false: if study results are clinically insignificant, the statistically significant results are of little interest to patients. b) What is a biased sample? c) How could you select a random sample from a population? d) What it variability? e) What is inference? f) What is a standard deviation? g) What is a variable? h) What is the difference between clinical significance and statistical significance?

Unit 4 Regulatory Procedures.

March 22, 2017

Webcast Link: **http://www.eurordis.org/training-regulatory-framework-webcasts** 1 hour 15 minutes of training	**Regulatory Framework's new legislation in the EU.** Solange Rohou **Concepts covered:** **HTA Bodies, Payers, Adaptive Pathway Pilot, Principles of Good Practice: GLP, GCP, GMP, ICH, Dossier, Drug Master File, Common Technical Document, NDA, Clinical Trial Application, CTD Triangle, Risk Management Plan, Ensuring Safety, Ensuring Quality, Ensuring Effectiveness, First-in-man, Use of registries as a means to monitor an approved product, cold chain, cross contamination and traceability.**
Webcast Link: **http://www.eurordis.org/training-regulatory-framework-webcasts** 1 hour of training	**Regulatory Procedures.** Patrick Salmon **Concepts covered:** **Centralised procedure, National competent authorities, Decentralised procedure, Reference member state, Marketing authorization, post authorisation commitments and safety studies.**
	End of Unit Quiz a) What is a risk management plan? b) Why is traceability important in manufacturing of medicinal products? c) Why are registries important even after a medicinal product is on the market? d) What is a SmPC as opposed a package leaflet? e) What type of medical products is evaluated centrally in Europe? f) What is mutual recognition? g) What is a bioequivalent study and what is it for? h) What is an accelerated assessment? i) What is conditional approval? j) What are exceptional circumstances?

Unit 5: European Medicines Agency.

April 05, 2017

Webcast Link: http://www.eurordis.org/training-european-medicines-agency-webcasts 50 minutes of training	**A General Introduction to the European Medicines Agency (EMA):** Nathalie Bere **Concepts Covered:** **Evaluation of marketing authorization, pharmacovigilance, orphan designation, paediatric investigation plans, exceptional circumstances, Centralised procedure for marketing authorisation, scientific advice, arbitration and referral. Risk management plans.**
Webcast Link: http://www.eurordis.org/training-european-medicines-agency-webcasts 2 hours of training	**A Round Table Discussion of Various EMA Sub-Organisations.** Nathalie Bere, Kristina Larsson, Josep Torrent, Fernando de Andres Trelles, Michele Lipucci di Paolo **Concepts Covered: Committee for orphan medicinal products; Scientific Advice, Paediatric investigation plan, Protocol assistance, Committee for Advanced Therapy**
Webcast Link: http://www.eurordis.org/training-european-medicines-agency-webcasts 1 hour of training	**The Committee for Medicinal Products for Human Use (CHMP).** Patrick Salmon **Concepts covered:** **Preparing EMAs opinion on all questions concerning medical products for human us.**
Webcast Link: http://www.eurordis.org/training-european-medicines-agency-webcasts 10 minutes of training	**Patient Interaction with the EMA.** Nathalie Bere **Concepts covered:** **Product information, package leaflet, SmPC, EPAR**
	End of Unit Quiz a) Which of the EMA scientific committees is responsible for licencing medicines in the EU? b) What is the maximum time limit for assessing a new marketing authorisation application? c) How are medicines approved at the European level? d) Is the EMA an EU regulatory body? e) Which EMA committees have patients as full members? f) How can an organization apply to become involved in EMA activities? g) How can patients contribute to EMA activities? h) What is the purpose of a PIP? i) What is the difference between these three types of approval: Normal, exceptional circumstances, and conditional approval? j) What is a five year renewal? k) How can patients have a real impact on the benefit-risk analysis for marketing authorization?

Unit 6: Pharmacovigilance and Benefit Risk.

April 19, 2017

Webcast Link: http://www.eurordis.org/training-european-medicines-agency-webcasts 35 minutes of training	**Patient involvement in benefit-risk at the EMA:** Nathalie Bere and Maria Mavris Concepts covered: **Four fold model of benefit risk, framework of interactions with patients/consumers. Eliciting patient preferences.**
Webcast Link: http://www.eurordis.org/training-european-medicines-agency-webcasts 45 minutes of training	**Pharmacovigilance Risk Assessment Committee:** Albert van der Zeijden Concepts covered: **Referrals and the role of the PRAC, The need for pharmacovigilance rules. The legislation in place for pharmacovigilance.**
Webcast Link: http://www.eurordis.org/training-benefit-risk-assessment-pharmacovigilance-webcasts 50 minutes of training	**The Role of Patient Organisations in Pharmacovigilance:** François Houÿez Concepts covered: **Black triangle, PSUR, ADRs, Risk management plans (RMP)** **Reporting tools** **http://www.eurordis.org/pharmacovigilance** **http://www.adrreports.eu/en/index.html** **http://eudravigilance.ema.europa.eu/human/index.asp** **Web-RADR: two-way reporting. http://web-radr.eu/**
	End of Unit Quiz a) What are the components of the four-fold model of benefit risk for the EMA? b) What is the relationship between the PRAC, the CHMP and the CMDh? c) What is a signal in adverse event reports? d) How do RMPs affect patients? e) What is the signification of the black triangle? f) What can you do if a drug reaction occurs months after you take a medication? g) Can you report adverse reactions for a medicine that you are taking off label? h) Explain why safety and pharmacovigilance post marketing action are so important to ensure a safe use of new therapies?

Unit 7: Market Access and Health Technology Assessment.
May 03, 2017

Webcast Link: http://www.eurordis.org/training-market-access-webcasts **1 hour 30 minutes of training**	**Health Technology Assessment.** Edmund Jessop Before you watch the webcast, spend a few minutes calculating how much it would cost to travel from Paris to Barcelona and back by car. **Concepts covered:** **Assessing Cost: Marginal costs, Payer, Direct costs and Indirect costs, intangible costs: pain and suffering, Unit cost and marginal costs, access schemes, incremental cost, opportunity costs.** **Assessing Benefit: Quality of life, Quality adjusted life year (Qaly)** **Modelling disease state:** **What is special about rarity? Orphan legislation applies to severely disabling multisystem disorders.**
Webcast Link: http://www.eurordis.org/training-market-access-webcasts 1 hour of training	**Market Access Approaches:** Driss Berdaï **Concepts covered:** **Quality of Care, equity, benefit-risk assessment, HTA appraisal, price negotiation, reimbursement, Efficacy, Effectiveness and Efficiency**
	End of Unit Quiz a) What is an example of a marginal cost? b) What is an incremental cost? c) How many HTA organisations are there in the EU? d) What are the main differences between an EMA and an HTA assessment? e) What is a payer? f) If you put a value on avoiding hassle, what type of cost would that be? Tangible or intangible and why? g) What are the two basic types of benefit in an HTA evaluation? h) What is EQ5D? i) Does the orphan legislation include only rare diseases? j) In which settings would you use data from efficacy, efficiency, and/or effectiveness