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Capacity building

Training Programmes for Patients' Representatives: EURORDIS Summer School

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EURORDIS Membership Meeting
Brussels, May 23, 2012

Purpose of the EURORDIS Summer School

- The aim of the Summer School is to provide an opportunity for patient representatives, involved in drug development and clinical trials, to meet and interact with regulators, academic partners and each other for in-depth exchanges of information and experiences.
- To provide an opportunity for an overview of the entire drug development process
- To empower more patient representatives in advocacy activities

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Selection of Participants

Participants filled in the application form and were selected by an *ad hoc* committee on the following criteria:

- level of English language skills,
- experience
- willingness to be involved in drug development and regulatory affairs at the European level.

2008

2010

2009

2011

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Alumni (Participants) – 2008-2011

A total of **116** participants have been trained in the basic Summer School to date.

These participants represent:

- **27** countries (Armenia, Belgium, Bulgaria, Canada, Croatia, Czech Republic, Denmark, Finland, France, Georgia, Germany, Greece, Hungary, Iceland, Ireland, Italy, Netherlands, Poland, Portugal, Romania, Russia, Serbia, Slovakia, Spain, Sweden, Ukraine, United Kingdom)

- and more than **45** different rare diseases (including Addison and Cushing's, Ataxia, Behcets syndrome, Congenital heart disease, Cystic fibrosis, Ectodermic dysplasia, Ehlers-Danlos, Epidermolysis bullosa, Fabry disease, Fragile X, Gaucher, Haemophilia, Hungtington's disease, Marfan syndrome, Multiple myeloma, Noonan syndrome, Pemphigus/Pemphigoid, Prader Willi, Mucopolysaccharoidosis (type 3), Spinal muscular atrophy, Syringomyelia, Thalassaemia, Tuberous Sclerosis and Williams syndrome).

Organisation of four-day programme

- **Pre-Summer School preparation** http://summerschool.eurordis.org/online_tutorials/index.html

- **Four-day programme**
  - General organisation
    - Days 1 and 2 – Clinical trials and Drug development
    - Day 3 – EMA overview and committees
    - Day 4 – Eurordis

  - Specific organisation
    - Mix of small group sessions and large group formal p
    - Problem-based learning model
    - Small group sessions of 10 individuals (maximum) to encourage interactions and exchanges
    - Case-based tutorials
      - Diacomit® (for treatment of severe myoclonic epilepsy in infancy),
      - Fabrazyme® (for treatment of Fabry disease) and
      - Pedea® (for treatment of patent ductus arteriosus)

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Content – Days 1 and 2

- **Clinical Trials and Drug Development – Day 1**
  - **Basic principles** of clinical trials:
    - Need for evidence-based medicine (levels of evidence)
    - Principles of drug development (preclinical versus clinical, animal models)
  - **Rationale** of a clinical trial:
    - Introduce Protocol (with objectives, endpoints, trial design, population size)
    - Gold standard clinical trial
  - **Statistical** aspects of clinical trials:
    - Concepts such as ‘significance’, ‘variance’ and ‘confidence’
    - Clinical versus Statistical significance

Content – Days 1 and 2

- **Clinical Trials and Drug Development – Day 2**
  - **Ethical** aspects of clinical trials:
    - Informed consent forms
    - Therapeutic versus experimental situations
  - **Non-standard** situations
    - Small population numbers
    - Insufficient knowledge on disease
  - **Regulatory** procedures
    - Designation
    - Marketing Authorisation
    - Conditional and exceptional circumstances
    - Compassionate use

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Day 3 and 4 – EMA committees and EURORDIS activities

An overview of the European Medicines Agency (EMA) and its activities.
Each of the committees active in the EMA, relevant to the Summer School and emphasising the role of patient representatives in these committees is presented.

COMP – Committee for Orphan Medicinal Products

SAWP – Scientific Advice Working Party

PDCO – Paediatric Committee

CAT – Committee for Advanced Therapies

CHMP – Committee for Human Medicinal Products

PCWP – Patient's and Consumer's Working Party

PhWP - Pharmacovigilance Working Party (soon to become Pharmacovigilance Risk Assessment Committee)

Mini COMP and PDCO sessions on designation and decisions on waivers
Workshops for Review of Product Information (PCWP)

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Continued training

EURORDIS website now has a dedicated **Training Resources** section since 2011

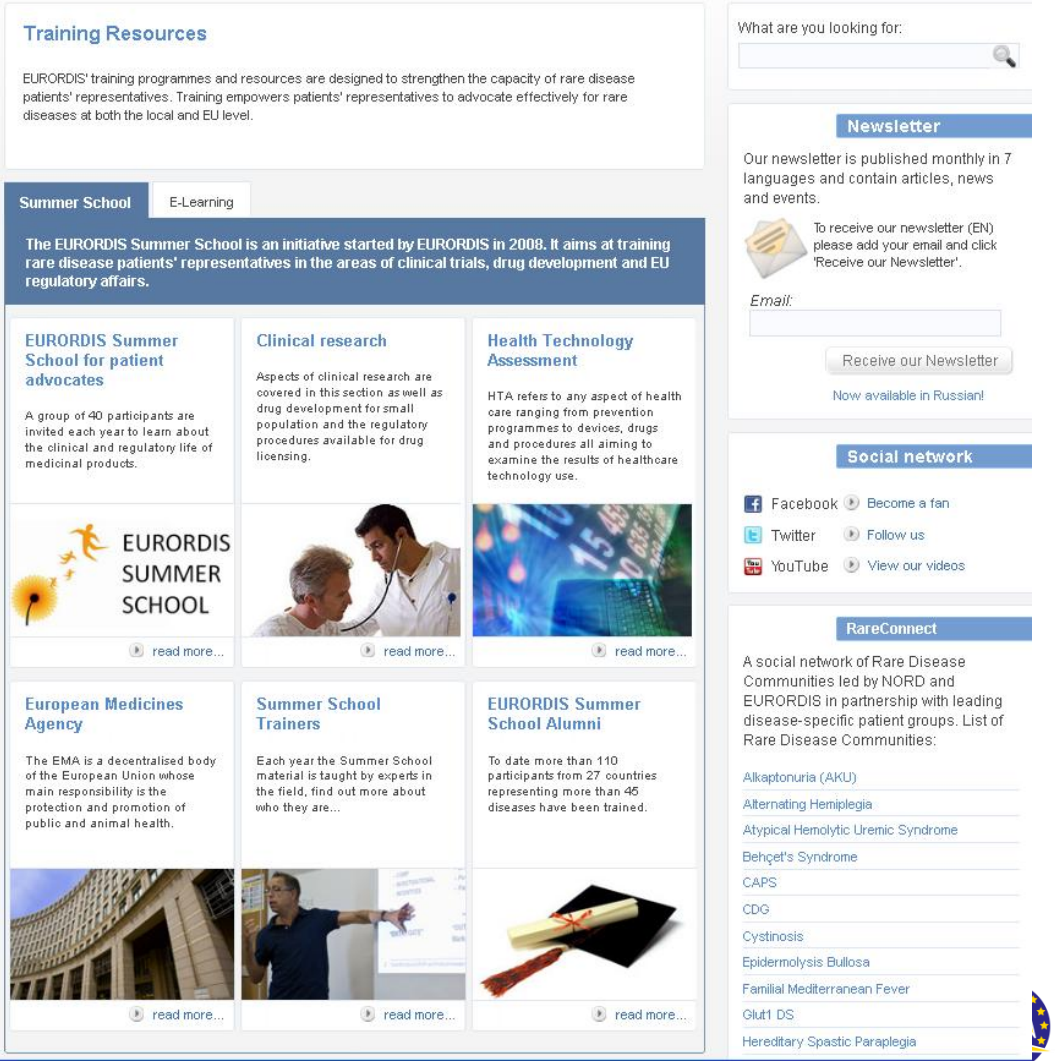
Section includes:
Two tabs:

- Summer School
- e-learning

<u>Webinar</u> tab to be added

Summer School tab:
6 nodes providing access to:

- General Information
- Video presentations of:
  - Clinical Research
  - HTA
  - EMA
- Summer School Trainers
- Summer School Alumni



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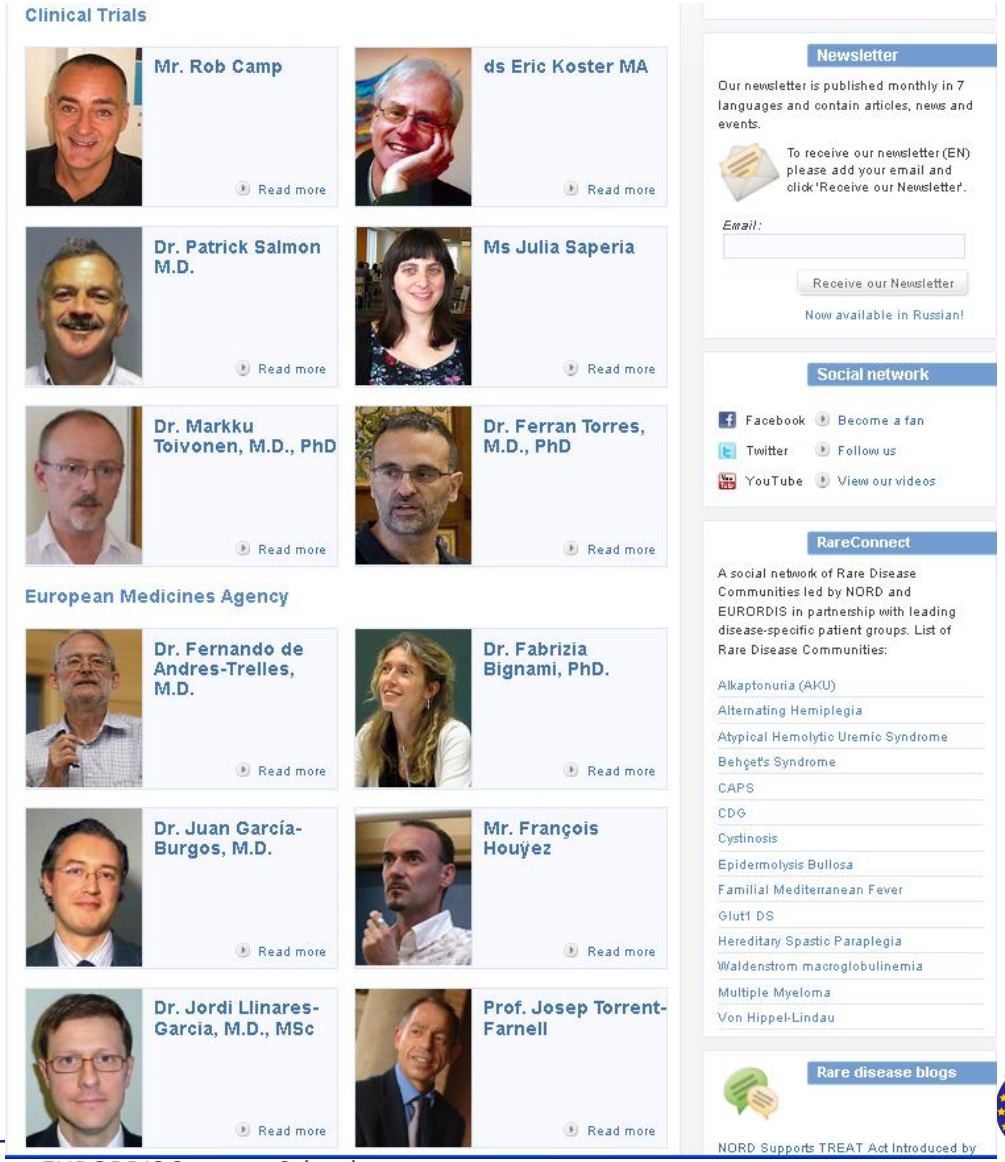
Faculty (Trainers)

A page dedicated to our trainers with:

- Photos
- Short biographies
- Link to their organisations



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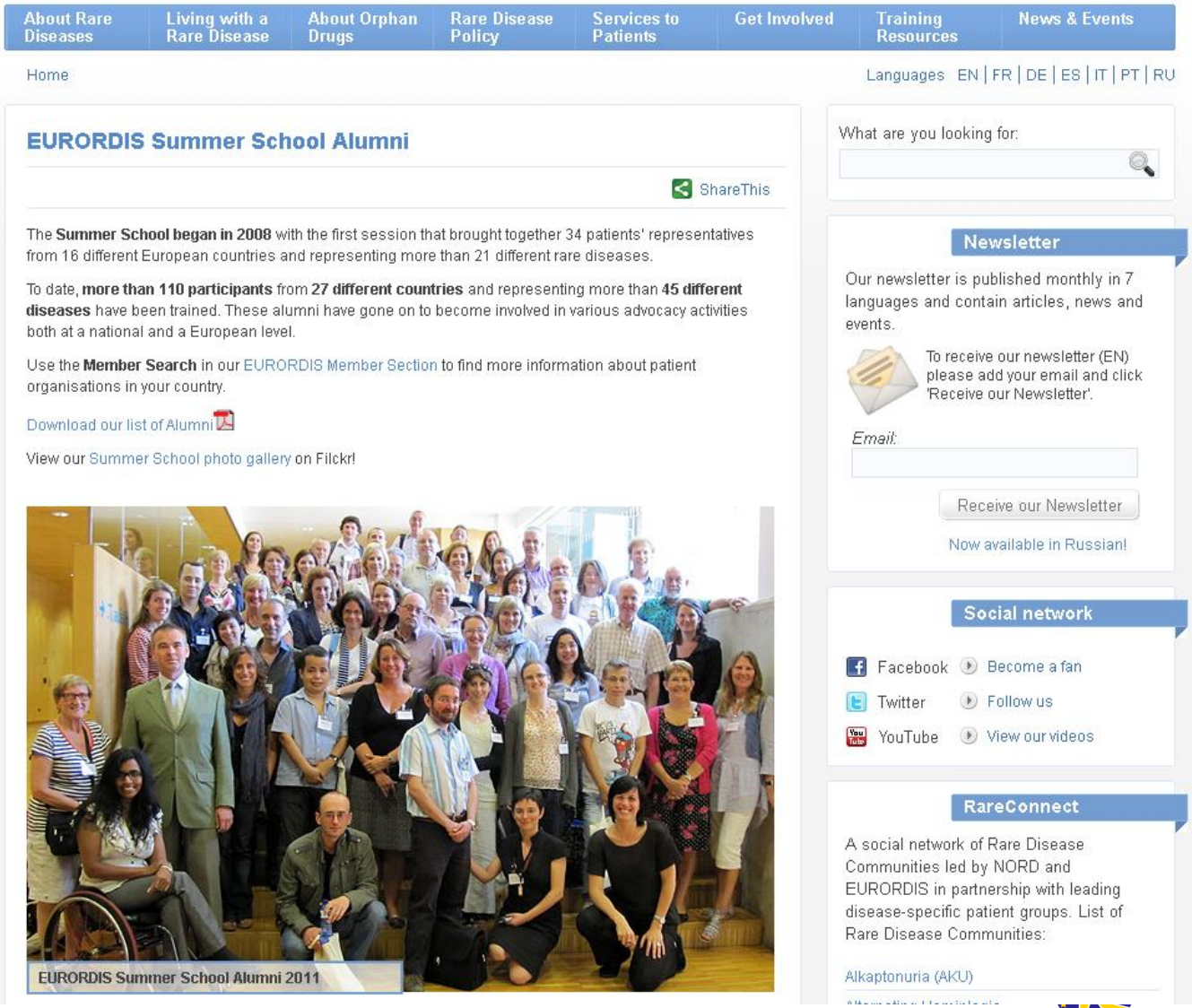
Alumni page

Downloadable list of all participants by country and affiliation.

Link to EURORDIS membership section

- By country
- By disease

Access to photographs from all Summer School events



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Preparation: on-line training

On-line learning section:

http://www.eurordis.org/training-resources

Training Resources

e-learning

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E-learning

The aim of this e-learning course is to provide an accessible tool that can fulfill immediate training requirements for all patients' representatives.

Content adapted to learning styles

Each of the three modules (Methodology, Ethics, and Statistics) includes six topics divided into five sections corresponding to different learning approaches.

- A theoretical approach in the "Lesson" section
- A practical approach in the "Case" section
- A semantic approach in the "Words" section proposing terms relevant to each topic
- A bibliographic approach with the "Documents" providing 3-4 documents from different perspectives (regulatory, historical, controversial,...)
- A challenging approach through 2 "problematic" quizzes

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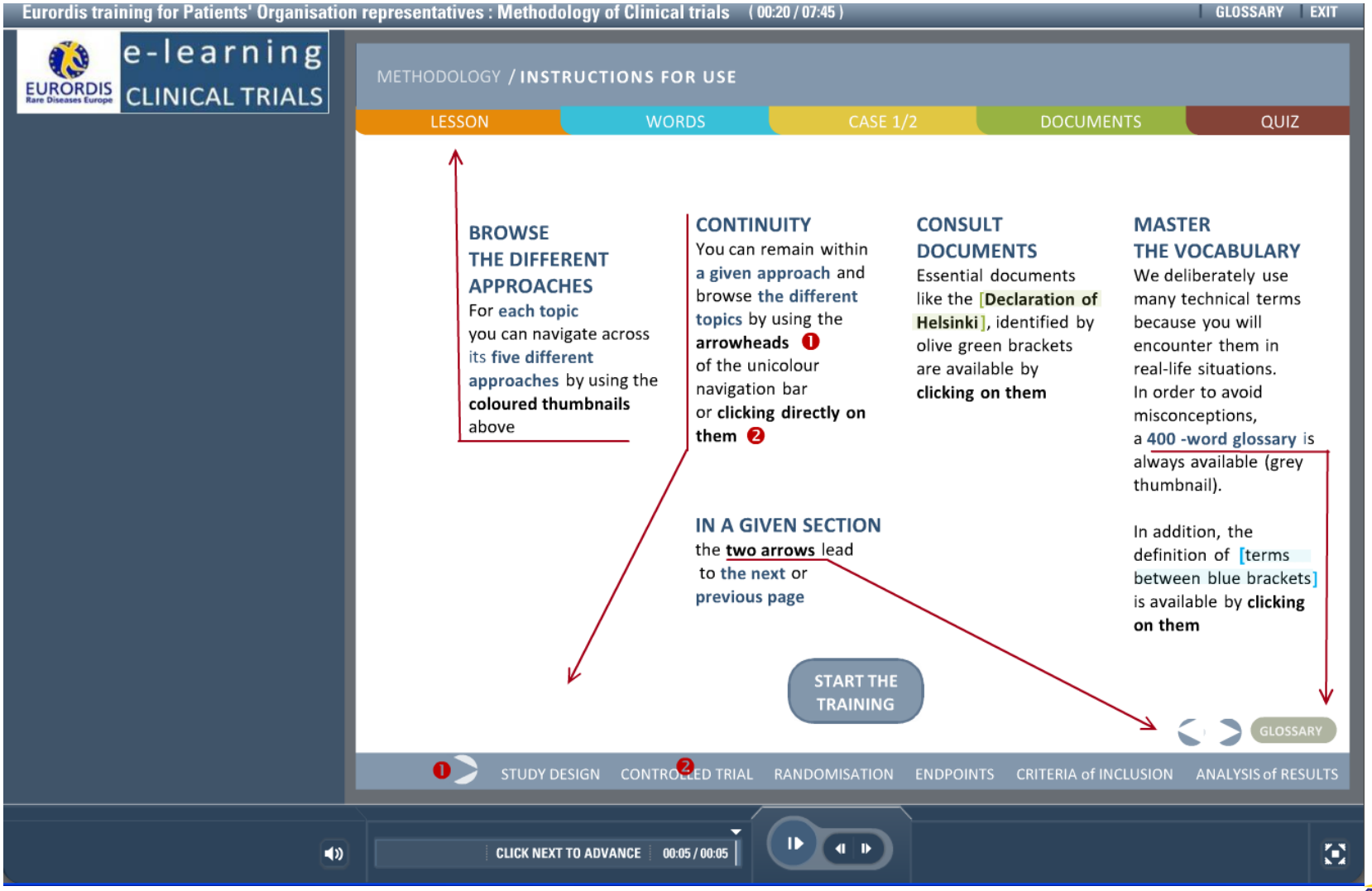
e-learning explanatory slide



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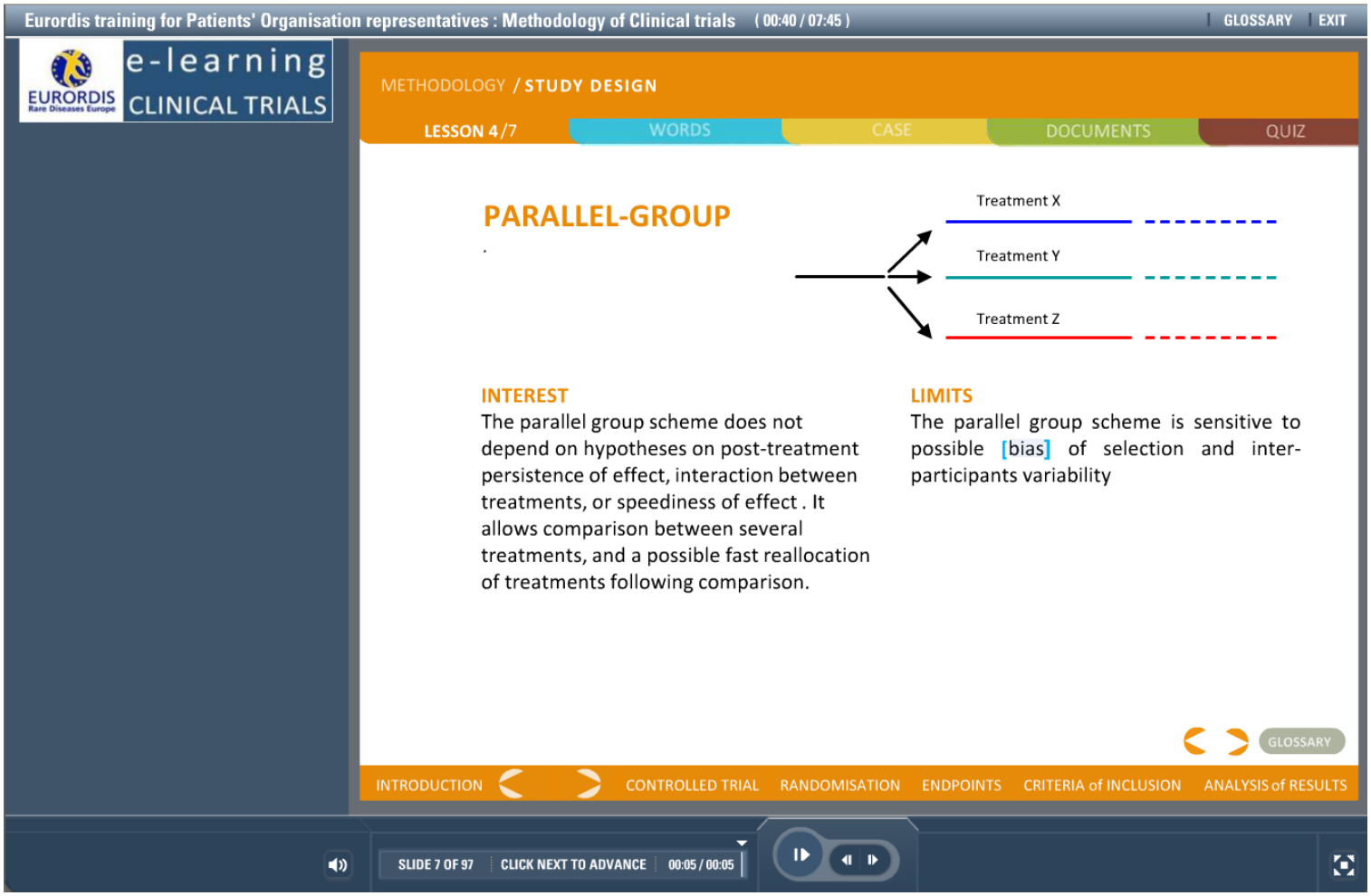
Example of ‘lesson’



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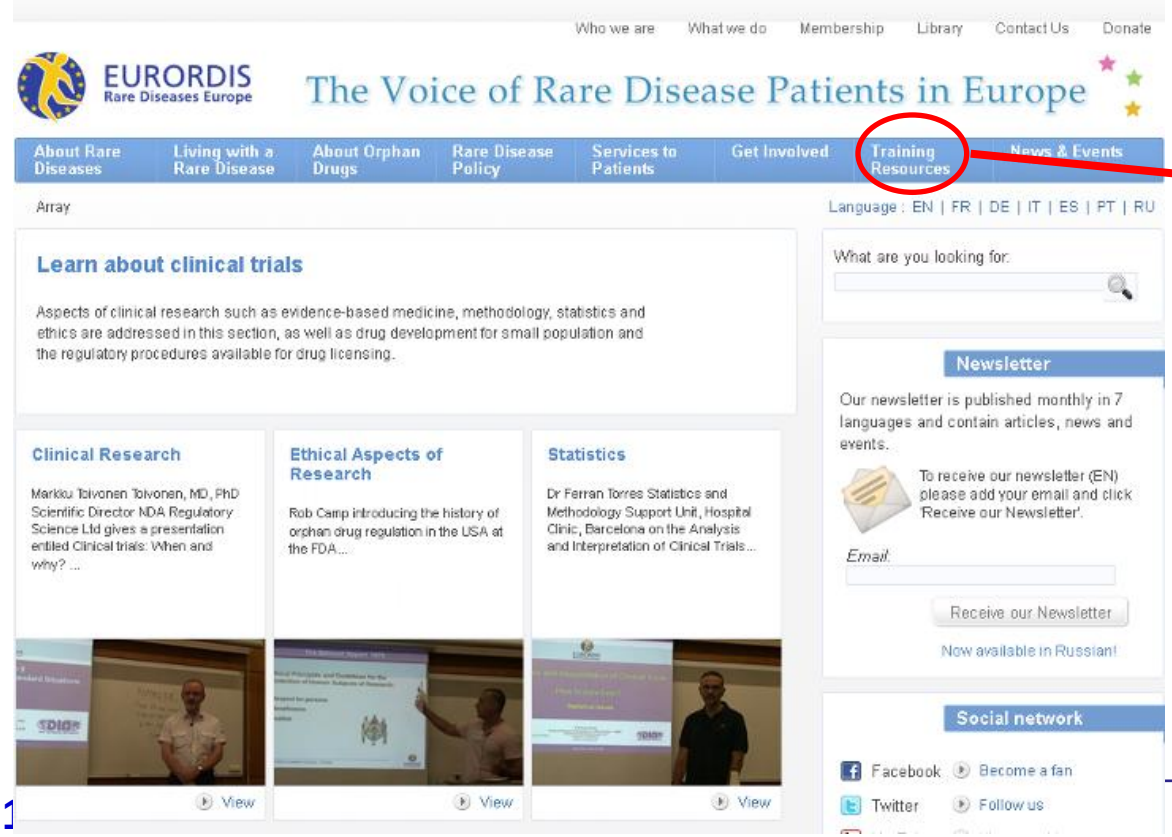
Video presentations

Video presentations on Clinical Trials, European Medicines Agency and Health Technology Assessment are currently available.



Training Resources:

Summer School tab

Clinical Research

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Training initiatives – other patients' organisations

European Federation of Neurological Associations (EFNA) – HTA School

European Patients Forum (EPF)

http://www.eu-patient.eu/Initatives-Policy/

European AIDS Treatment Group (EATG)

http://www.eatg.org/eatg/Capacity-Building/Trainings

And of course… EUPATI….