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Preparing patients' representatives for Protocol Assistance

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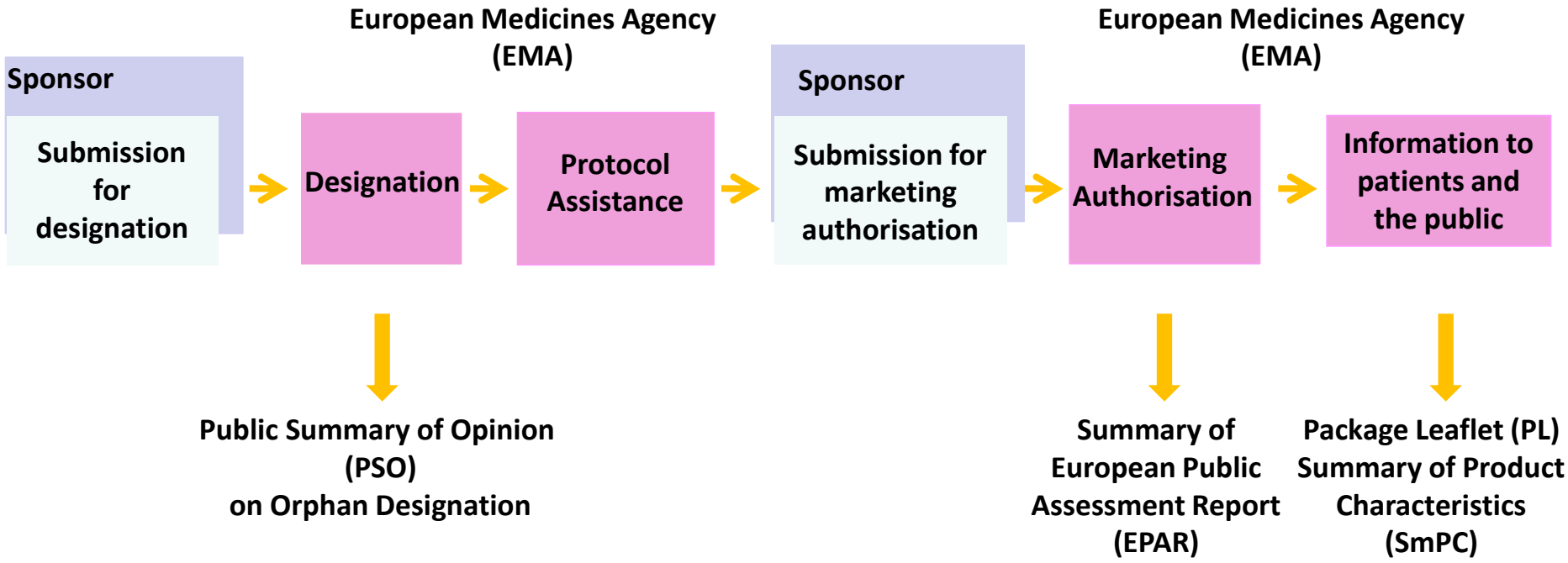
Regulatory Process for Orphans



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What is protocol assistance?

- **Protocol Assistance is a benefit offered by the *European Regulation on Orphan Medicinal Products* to pharmaceutical companies developing orphan medicinal products.**

- **It offers those companies an opportunity to receive advice on the development process of the medicinal product, before they apply for Marketing Authorisation. Advice is provided by the Scientific Advice Working Party (SAWP) at the European Medicines Agency (EMA).**

Role of patients and patient representatives in PA?

- **Patients and patient representatives are sometimes asked to participate in a Protocol Assistance meeting for a drug related to their disease**

- **They are asked to share their experience of the disease with the Scientific Advice Working Party (SAWP) and the pharmaceutical company, therefore providing help for the further development of the drug.**

Some statistics…

Year	Number of patients' representatives in PA	Number of dossiers for PA received by EURORDIS
2008	8	56
2009	13	77
2010	18	68
2011	20/16	74
2012	22/19	60
2013	1	1 (to date)

In approximately 50% of the cases of participation, patients' input influenced the outcome of the protocol assistance procedure

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Patients´ Contributions?

You should think about how your organisation and expert-patient members can give "input" into the orphan clinical development.

- You know your rare disease and its natural history best
- You know the needs of your patients and families best
- You know how your rare disease is clinically managed and who are the "true" clinical experts and where they work.
- You know the lack of treatment and what can be expected from innovative therapies
- You know the feasibility of the clinical investigations best

Take-home message

- **Be ready for action and don't panic when you are invited by authorities**
  - Prepare yourself well in advanced of the meeting
  - Focus on relevant points of the clinical investigation
  - Streamline what your contribution can be
  - Liaise with health professionals (win-win approach)
  - Positive thinking and active listening
  - Be prepared to manage uncertainties and avoid creating false or hyper – expectations
- **Your position needs to be underpinned by appropriate justifications**
  - Remember that emotional-only responses have a transient value.

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Dialogue - communication

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Dialogue - communication

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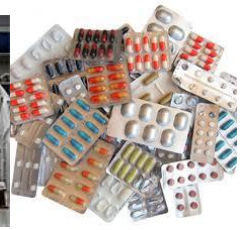

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Preparation and support

When a representative is contacted to participate in Protocol Assistance, they are sent:

- Letter of invitation/explanation
- Regulatory diagramme
- EURORDIS flyer on Protocol Assistance

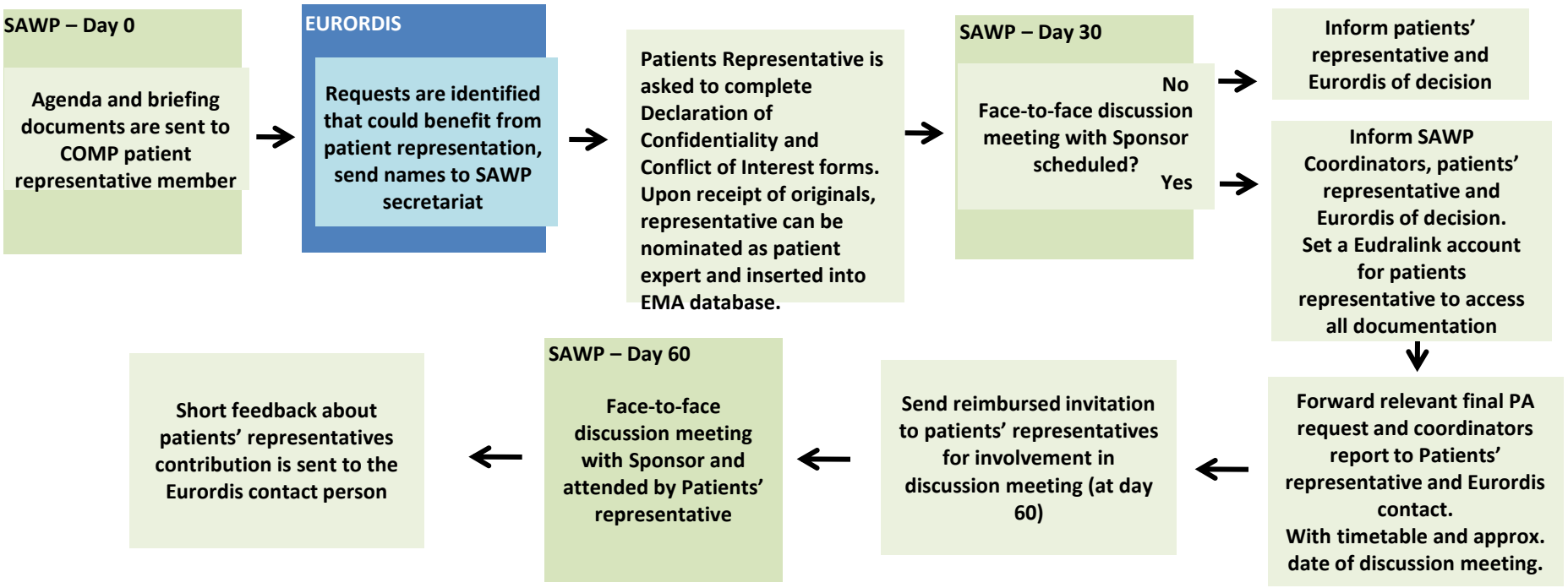
Each of these documents has been tabled

Letter of invitation/explanation

- Introduction to EURORDIS and my role
- Explanation of European Medicines Agency – their role
- Explanation of Orphan designation
- Invitation to be involved in the regulatory process
- No need to be a scientist or a medical expert
- What type of intervention could be envisaged
- Next steps

EURORDIS and Rare Disease Patients' Representatives involvement

Scientific Advice Working Party (SAWP)



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Preparation: F2F training

For patient advocates in clinical trials and drug development

Format: 4-day workshop in Barcelona, Spain

Focus: Role that patient representatives play during the regulatory process

To date, more than 150 patients' representatives representing more than 65 different rare diseases and 31 countries

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EURORDIS Summer School

AIMS:

- **Educate, train and provide an environment for information exchange**
- **Bring together patient advocates for the first time and**
- **Enable patient representatives to interact with regulators, academics and industry partners**
- **Further the understanding of patient representatives of the process of drug development and clinical trials**

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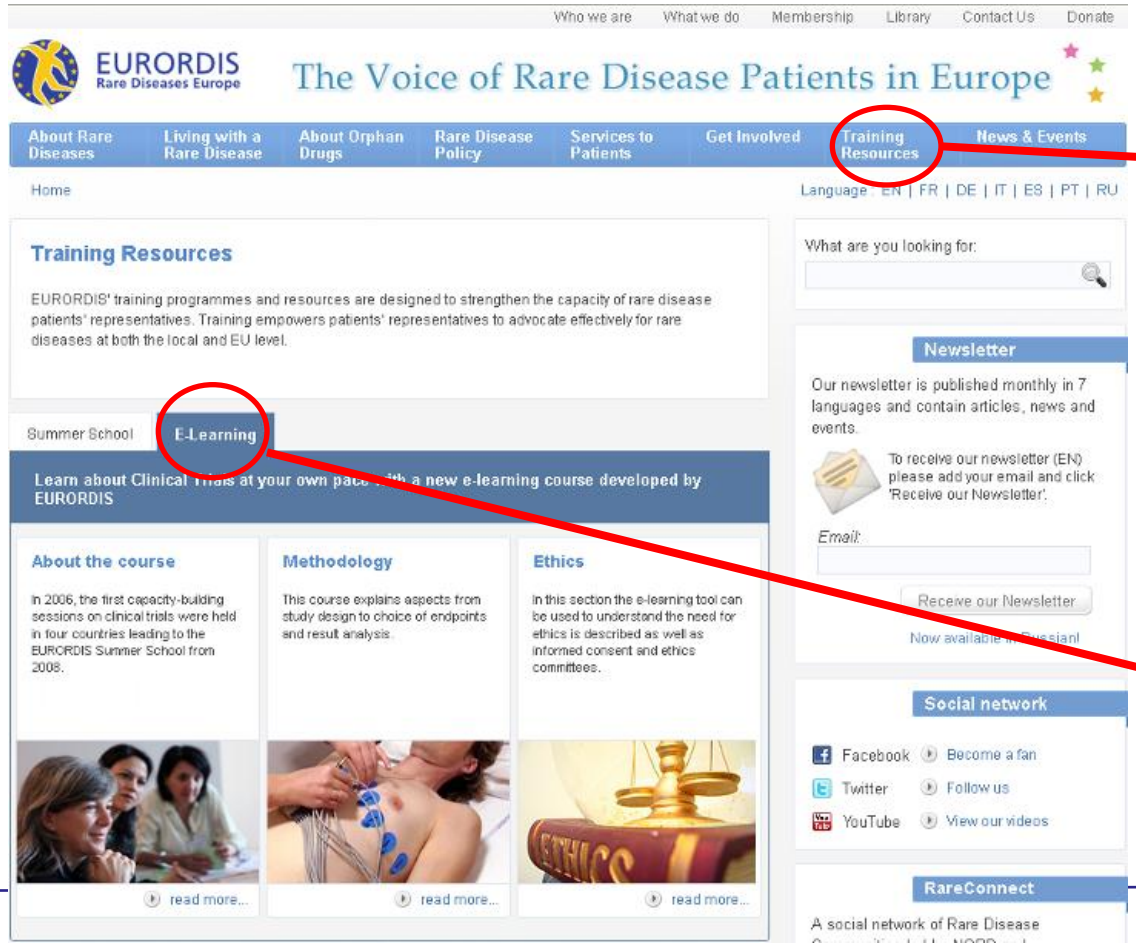
Preparation: on-line training

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 patient organisation representatives.

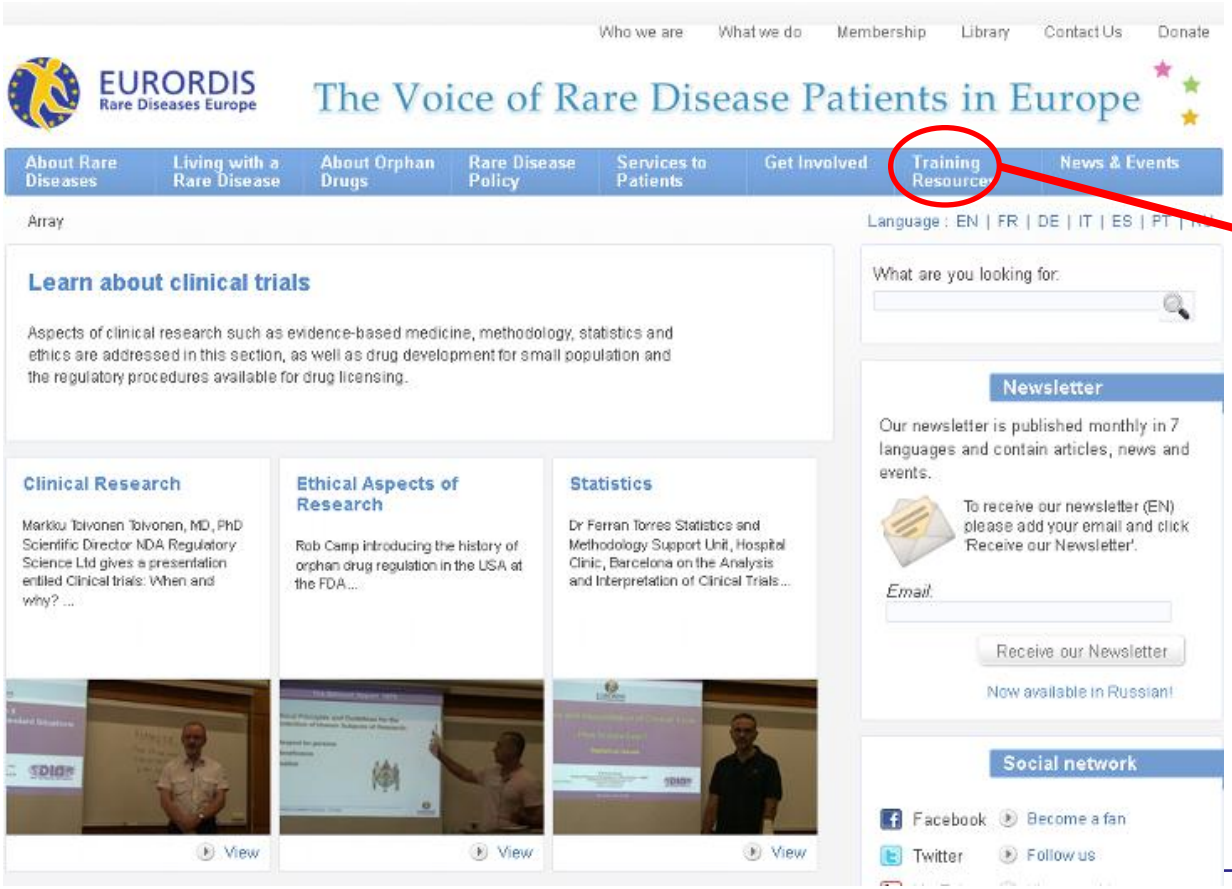
Content adapted to learning styles
Each of the three modules (Methodology, Ethics, and Statistics) includes six topics divided into five sections corresponding to different learning approaches.

- **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**

Current modules: Methodology, Ethics and Statistics.

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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Support for patients' representatives

Follow up after procedures by EMA and EURORDIS – questionnaire for protocol assistance

Continued training via:

- **Webinars**
- **Therapeutic reports – Update on monthly activities related to therapeutic development and participation of patients' representatives in EMA activities**
- **Therapeutic Action Group (TAG) is composed of EURORDIS representatives in the scientific committees and working party at the EMA (PDCO, PCWP, CAT, COMP)**

  These patients' representatives dedicate their time, experience and expertise to the task of evaluating dossiers of medicinal products requesting orphan drug designation, evaluation for use in paediatric patients, evaluation of advanced therapies as well as ensuring accurate, transparent and available information to patients on authorised medicinal products.

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Training initiatives – other patients' organisations

EFNA – HTA School

EPF – European Patients Forum
www.eu-patient.eu/Initatives-Policy

EATG – European AIDS Treatment Group
www.eatg.org/eatg/Capacity-Building/Trainings

EUPATI – European Patients' Academy in Therapeutic Innovation
www.patientsacademy.eu

Summary

Importance of:

- **Involvement of patients' organisations in regulatory activities**
- **Appropriate and adapted information**
- **Communication**
- **Training**
- **Benefit is mutual**

EMA becomes more informed and aware

Patients become more empowered

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