European Patients' Academy (EUPATI) Update

EMA meeting with patient/consumer organisations
11 Dec 2013

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For patient-centric medicines R&D and to contribute to committees, we need more trained patient advocates

EUPATI
European Patients' Academy on Therapeutic Innovation

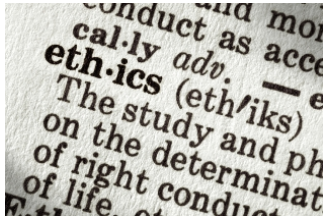
EUROPEAN MEDICINES AGENCY
SCIENCE MEDICINES HEALTH
BfArM
MHRA
swissmedic

Competent authorities

Public

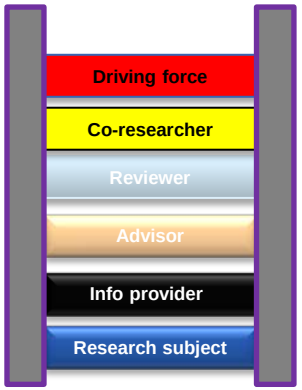
Policy makers

Trial protocol design, informed consent, ethical review, marketing authorization, value assessment, health policy



Research Ethics Committees

HTA agencies & committees



Clinical Research

EUPATI: Training patients as partners in medicines R&D

- Launched Feb 2012, runs for 5 years, 30 consortium members,
- PPP of EU Commission and EFPIA
- will **develop and provide, objective, credible, correct, up-to-date knowledge about medicines R&D**
- will **build competencies & expert capacity** among patients & public
- will **facilitate patient involvement in R&D** to support academia, authorities, industry, ethics committees

Multi-stakeholder consortium, transparently governed

- Led by patient organisations
- Strong impetus from academia and NGOs
- Industry expertise in medicines R&D
- **Advisory bodies** help ensuring independence, transparency, good governance
  - EMA, Swissmedic, MHRA, BfArM
  - Key experts in bioethics, genetics, HTA, economics, evidence based med, patient advocacy, e.g. NICE, Cochrane, EUnetHTA
  - Ethics Panel

EUPATI Audiences: advocacy leaders and the lay public

EUPATI Certificate Training Programme

100 patient advocates

EUPATI Educational Toolbox

12.000 patient advocates

EUPATI Internet Library

100.000 individuals

English
French
German
Spanish
Polish
Italian
Russian

Topics of EUPATI Training Courses
(which will also be covered in web library)

Topic areas of the Training Course Syllabus

1. **Discovery of Medicines & Planning of Medicine Development**
   (→ 16 sub-topics)
2. **Non-Clinical Testing and Pharmaceutical Development**
   (→ 6 sub-topics)
3. **Exploratory and Confirmatory Clinical Development**
   (→ 14 sub-topics)
4. **Clinical Trials**
   (→ 35 sub-topics)
5. **Regulatory Affairs, Medicinal Product Safety, Pharmacovigilance and Pharmaco-epidemiology**
   (→ 38 sub-topics)
6. **HTA principles and practices**
   (→ 10 sub-topics)

= 119 sub-topics of the EUPATI Syllabus

…and *NOT:* develop indication- or therapy-specific information!

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  - Similarly applied by e.g. WikiPedia, Google, Whitehouse.gov and many others

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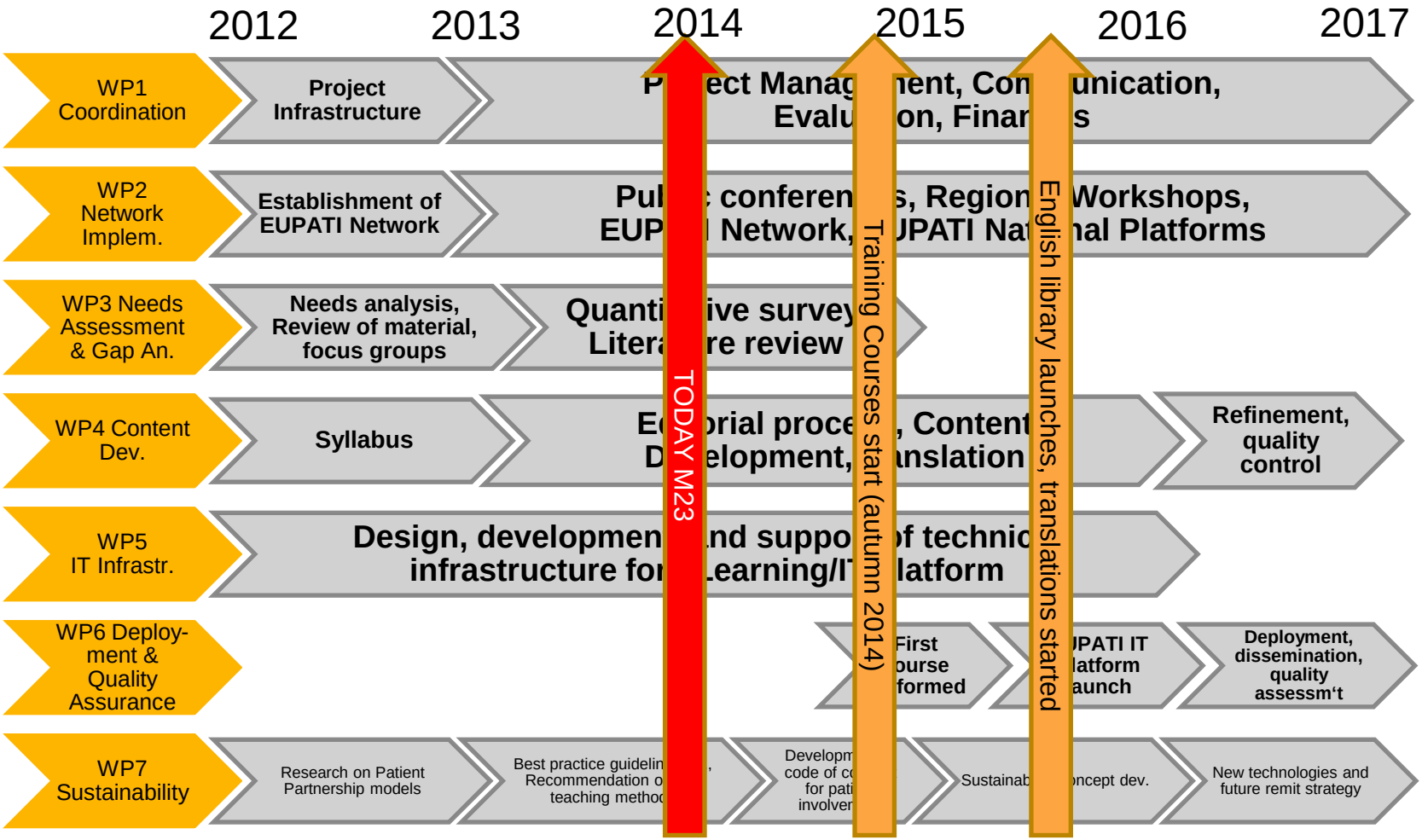
Progress update at Month 23: Production of material has started



Possible largest ever social research on public & private attitudes in medicines R&D

Peer-reviewed publication currently in preparation

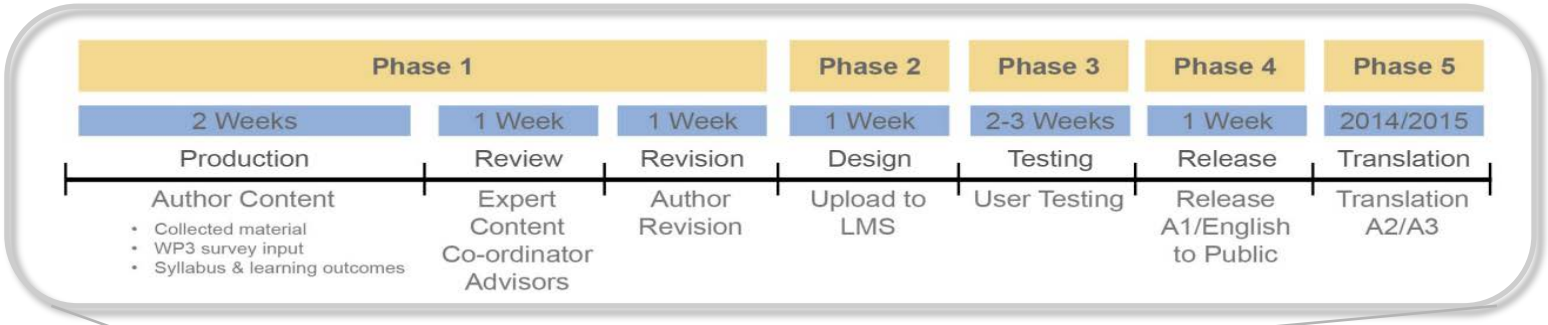
Review Work		Online Surveys		Qualitative Studies
Review of existing information resources on medicines development aimed at patients and the public	**Review of research literature** on patients' and the public's knowledge, attitudes and beliefs regarding medicines development	**General public across 6 European countries** (GB, Spain, Poland, Italy, France and Germany)	**Patient advocates and expert patients** across Europe	**Focus groups and interviews** of patients, public, patient advocates, industry, clinical research professionals and policy makers in UK, ES, PL, pan-EU
Findings				
306 resources submitted. 230 included in review. Highest number of resources covering drug safety. Lowest number covering personalised and predictive medicine.	12600 titles and abstracts reviewed 134 included in review. Medicines development (1 study personalised and predictive medicine 52 studies Medicines safety 28 studies HTA 10 studies Clinical trials 40 studies	7003 members of the public (audience 3) surveyed 6931 responses recorded Interest in learning more about medicines developed areas had a similar ranking in all countries 1. Medicines safety 2. Pers'd. & predictive medicine 3. Drug discovery 4. HTA 5. Clinical trials 6. Patients roles & responsib. 7. Regulation 8. Pharmacoeconomics	470 responses from patient advocates and expert patients across Europe 148 reported current research involvement and 98 previous involvement 125 commented on PILs 70 involved in identifying research priorities 90 member of project advisory group	Across all sites 91 patient advocates 34 members of the public 13 policy makers 20 pharmaceutical industry representatives 23 Clinical research professionals / Health care academics 181 participants I total
Reports				
Information review executive summary and full report, Interim report in (Nov 2012)	Evidence summary doc. Methods and data doc. Abstracts and interim report Final report (Oct 2013)	Methods and data document (Nov 2013) Interim report	Final report (Nov 2013)	Final report (Nov 2013) Country reports (early 2014)

+ Peer-reviewed publication of all results (to be submitted Q1/2014)

EUPATI Content Production has started…

- Syllabus review & feedback loops on syllabus and learning outcomes in Consortium, Executive Committee, 3 Advisory Boards
  First version of syllabus published on website in July 2013

- First cycle of first content production kicked off on 9 Sept 2013, ongoing
- Most difficult issue: Recruitment of authors and expert reviewers. Patient advocates and regulatory experts welcome!
- This will be a system that learns and improves…



Sept 2013 Oct 2013 Dec 2013 …

We are seeking authors and expert reviewers – also patient advocates!

While most of the training will be e-learning, it will include two four-day face2face courses

Day	Time	Syllabus topic/content
EUPATI Course - Face2Face training meeting #1		
Day 1		
	09:00-10:30	Welcome to EUPATI – purpose of training. Introduction of each participant (name, association plus their purpose for attending)
	COFFEE BREAK	
	10:45-11:45	Introductory overview of medicines development process and description of major disease areas
	11:45-13:00	Small group sessions on protocol design and first steps in ethics and regulatory approval.
	LUNCH	
	14:00-15:00	Designing a clinical trial – Ethics and Regulatory review
	COFFEE BREAK	Includes – revision of some concepts (trial design, randomisation, blinding, comparators, endpoints, objective, trial sites) and practical work in small groups to design a trial.
	15:30-18:00	Focus on where patients can be involved in these processes
Day 2		
	09:00-10:30	Revision of concepts of trial design - patient recruitment, sample size, informed consent)
	COFFEE BREAK	
	10:45-11:45	Use of statistics in clinical trials – sample size, type I and II errors, significance, null hypothesis, analysis, p value, variation etc..
	11:45-13:00	Exercises in statistics – analysis of published articles on clinical results; evaluation of statistical significance versus clinical significance, etc..
	LUNCH	
	14:00-15:00	Ethics – review of principles
	COFFEE BREAK	
	15:30-17:30	Simulate an ethics review board and importance of patient involvement in informed consent and patient information.
	17:30-18:30	Approval of protocol, process of data collection during clinical trials, statistical analysis and summary report
Day 3		
3	09:00-10:00	Regulatory aspects of clinical trial approval
	9:30-10:30	Regulatory agency – EMA and committees – patient involvement
3	COFFEE BREAK	
	10:45-11:45	
	11:45-13:00	Practical session on paediatric investigation plan evaluation
	LUNCH	
	14:00-15:00	Practical session on orphan designation of medicines
	COFFEE BREAK	
	15:30-17:00	Training of patients in review of documents destined for public
Day 4		
	09:30-10:30	Role of patients' and consumers' working party
	COFFEE BREAK	
	10:45-11:45	Patients' involvement in Scientific advisory boards and scientific advice in medicines agencies (EMA example)
	11:45-13:00	Types of medicines (e.g. chemical, biological, advanced therapies, vaccines, devices)
	LUNCH	
	14:00-15:00	Clinical trial [illegible]
	COFFEE BREAK	
	15:30-16:30	Generics and biosimilars
	16:30-17:30	Conclusion of EVENT 1 – feedback from participants and general discussion

EUPATI Course - Face2Face training meeting #2		
Day 1		
	09:00-11:00	Continue gaining knowledge after authorisation (Matthew to complete)
	COFFEE BREAK	
	11:15-13:00	Continue gaining knowledge after authorisation (Matthew to complete)
	LUNCH	
	14:00-15:30	INTRODUCTION: Reforming Pharmacovigilance in Europe. Case study: Illustration: the case of [name_of_product] and how it was managed (e.g. Gardasil®)
	COFFEE BREAK	
	16:00-18:00	Basic Principles of Risk Communication Practical hands-on: example of an ADR case-report published in a scientific journal Communicating to the public from competent authorities (5.27) Spontaneous reporting Evaluation of patient reporting to the Yellow Card System European form for self-reporting Eudravigilance database of suspected ADRs
Day 2		
	09:00-11:00	Safety Communication Link to adverse event reporting (5.28) Practical hands-on: participants explain safety issues related to a medicine that they heard about from the media, colleagues, patients etc. The group discusses how the problem was handled, and the outcomes. Focus on how patients can become involved.
	COFFEE BREAK	
	11:15-13:00	Continued monitoring. Controlled Medicinal Products 5.14 Understand the situations where a medicine can be included in the list of controlled medicinal products. Explain how controlled medicinal products are regulated and dispensed (the principles).
	LUNCH	
	14:00-15:30	Case Study I on Implications of product defects/recall and shortage; product withdrawal procedures: the involvement of patient organisations (5.16)
	COFFEE BREAK	
	16:00-18:00	Case study 2 on Principles of risk management, incl. safety specification: Risk Management Plans (RMPs) in the EU and involvement of patient organisations
Day 3		
	09:00-11:00	The role and regulatory responsibilities of sponsors, investigators and patients in medicinal product safety and pharmacovigilance pre- and post- marketing (5.20) including Roles and responsibilities of the National Competent Authorities and EMA and Roles and responsibilities of the marketing authorisation holders ☐ Qualified person of a pharmaceutical company
	COFFEE BREAK	
	11:15-13:00	Basic principles of health economics, health technology assessment (HTA), and evidence-based medicine 60′ • Differences between the concepts of health technology assessment (HTA) and economic evaluation (EE) and evidence-based medicine (EBM) • Key principles, elements, methods of HTA, EE, and EBM
	LUNCH	
	14:00-15:30	Overview of sub-disciplines of HTA including clinical effectiveness assessment, economic evaluation, as well as ethical, legal and social implications (ELSI) analysis • Definitions of the various subdisciplines • Conventional frameworks of analysis for each • Examination of the use of each type of analysis and reasons by international jurisdictions 30′
	COFFEE BREAK	
	16:00-18:00	Free Afternoon/Exercise
Day 4		
	09:00-11:00	Understanding structure of an HTA report and how to develop it • The practical steps involved in developing and using HTA reports • Difference between HTA-specific approaches internationally and reasons for differences 15′ Exploring Clinical Effectiveness Assessment: Essentials of Evidence-Based Medicine, incl. role and concepts for value evaluation of innovative medicines by national bodies • Epidemiologic reasoning • Efficacy versus effectiveness • The concept of value of medicines for the different stakeholders (e.g patients, national competency authorities, HTA, regulators, academia) • Individual clinical decisions versus public policy 45′
	COFFEE BREAK	
	11:15-13:00	Concept of outcomes research and measurement instruments for health-related quality of life, patient-relevant outcomes, patient-reported outcomes • Difference between concepts • Common approaches and deficiencies • Patient-centered care: current thinking and future approaches • How patients can get involved in the HTA process (if applicable to their country) 60′
	LUNCH	
	14:00-15:30	Overview of patient reported outcomes (PRO) assessment and its role in product development • Principles of patient-reported outcomes (PRO assessment • Involving the patient in the development of PRO instruments and what is important to them 30′
	COFFEE BREAK	
	[illegible]	The evolution of [illegible] in HTA • [illegible] patient –centered study end-points • [illegible] • Future paradigms for HTA and the patient 30′ 30 Discussion and Q&A
	17:00-17:30	Conclusion of EVENT 2 – feedback from participants and general discussion

Draft programme - to be finalised by end of 2013

Platform for e-Learning and Internet Library currently being developed

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Home Library Toolbox Course Glossary

You are here: Home / Library / Discovery of Medicines & Planning of Medicines Development / Basic Principals of medicine discovery and development

Search

Search ...

Index

Discovery of Medicines & Planning of Medicines Development (5)

- Basic Principals of medicine discovery and development
- Natural product drug discovery
- Nature as source of drugs
- Drug targets
- Screening and design

Clinical Trials (2)

Basic Principals of medicine discovery and development

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Contents [hide]

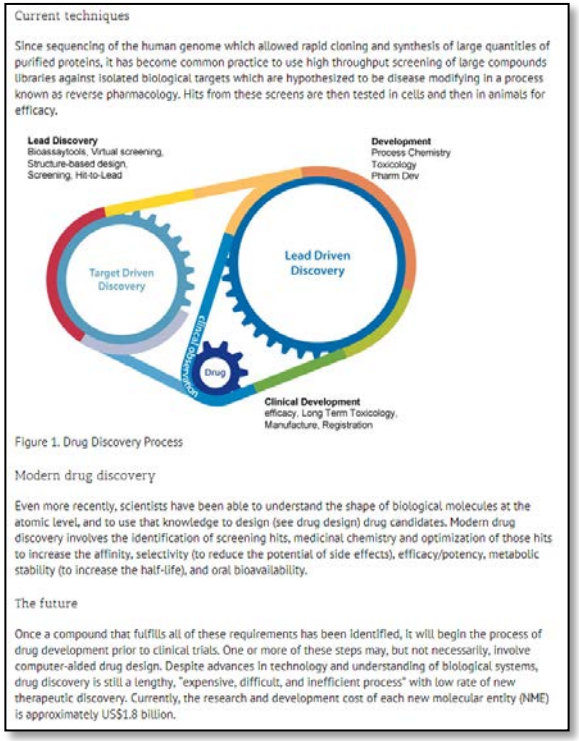
1 Drug discovery
- 1.1 History
- 1.2 Current techniques
- 1.3 Modern drug discovery
- 1.4 The future

Drug discovery

In the fields of medicine, biotechnology and pharmacology, drug discovery is the process by which new candidate medications are discovered.

History

Historically, drugs were discovered through identifying the active ingredient from traditional remedies or by serendipitous discovery. Later chemical libraries of synthetic small molecules, natural products or extracts were screened in intact cells or whole organisms to identify substances that have a desirable therapeutic effect in a process known as classical pharmacology, *Natural product drug discovery*.



Current techniques

Since sequencing of the human genome which allowed rapid cloning and synthesis of large quantities of purified proteins, it has become common practice to use high throughput screening of large compounds libraries against isolated biological targets which are hypothesized to be disease modifying in a process known as reverse pharmacology. Hits from these screens are then tested in cells and then in animals for efficacy.

Figure 1. Drug Discovery Process

Modern drug discovery

Even more recently, scientists have been able to understand the shape of biological molecules at the atomic level, and to use that knowledge to design (see drug design) drug candidates. Modern drug discovery involves the identification of screening hits, medicinal chemistry and optimization of those hits to increase the affinity, selectivity (to reduce the potential of side effects), efficacy/potency, metabolic stability (to increase the half-life), and oral bioavailability.

The future

Once a compound that fulfills all of these requirements has been identified, it will begin the process of drug development prior to clinical trials. One or more of these steps may, but not necessarily, involve computer-aided drug design. Despite advances in technology and understanding of biological systems, drug discovery is still a lengthy, "expensive, difficult, and inefficient process" with low rate of new therapeutic discovery. Currently, the research and development cost of each new molecular entity (NME) is approximately US$1.8 billion.

Currently being initiated: National EUPATI Platforms

- **Initiated by "trio"**
  of patient orgs, academia, industry
  - **make sure EUPATI understands educational needs** in R&D on national level when developing content
  - **disseminate EUPATI's existing training material and information** on the national level
  - **To raise public awareness & interest about EUPATI** in 12 countries
  - **To identify training faculty, logistics and financial support** on the national level

AT
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Please engage with EUPATI!

- authors/reviewers
- members of national platforms

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