European Patients Academy on Therapeutic Innovation (EUPATI)

Niels Westergaard, Phd and DSc

www.patientsacademy.eu
A long time ago
Beriberi (B1 or thiamine deficiency)

"White rice can be poisonous!"
This was the conclusion Christiaan Eijkman (1896) after ten years of research in Batavia, Java in the Dutch East Indies (now Jakarta, Indonesia).

- weight loss,
- emotional disturbances,
- impaired sensory perception,
- weakness
- pain in the limbs,
- irregular heart rate

Mentally retarded people 1 year

No symptoms of beriberi and no deaths

34 with beriberi and 18 deaths

Kuala Lumpur 1905
"New winds are blowing across Europe toward a paradigm shift of having patients actively involved in medicines R&D"
Public involvement in health research 1995-2009

- Bibliometric analysis is an effective way to examine how a body of literature evolves within a particular topic area.

**Reviews**

- Non-participatory/action research focus
- Participatory/action research focus

**Empirical studies**

- Non-participatory/action research focus
- Participatory/action research focus

Boote et al, Talking the talk or walking the walk, 2012
Other examples supporting the notion
Patient focused medicines development: Introducing a paradigm shift

Shared goal: Getting the right treatment to the right patients
EUPATI Findings

- What the *public knows and wants to know* about medicines research and development: a survey of general public in six European Countries

- What do the *pharmaceutical industry professionals in Europe believe* about involving patients and the public in research and development of medicines? A qualitative interview study

Parsons et al., BMJ open 2015 and 2016
What the public knows and wants to know

<table>
<thead>
<tr>
<th><em>Baseline (6528)</em></th>
<th>Medicines R&amp;D</th>
</tr>
</thead>
<tbody>
<tr>
<td></td>
<td><strong>Current knowledge (rank)</strong></td>
</tr>
<tr>
<td>Medicines R&amp;D</td>
<td>9</td>
</tr>
<tr>
<td>Drug discovery</td>
<td>2</td>
</tr>
<tr>
<td>Medicines safety</td>
<td>1</td>
</tr>
<tr>
<td>Patients’ role and responsibilities</td>
<td>7</td>
</tr>
<tr>
<td>Personalised medicine</td>
<td>5</td>
</tr>
<tr>
<td>Predictive medicine</td>
<td>4</td>
</tr>
<tr>
<td>Design and objectives of clinical trials</td>
<td>2</td>
</tr>
<tr>
<td>Health technology assessment</td>
<td>5</td>
</tr>
<tr>
<td>Pharmacoeconomics</td>
<td>10</td>
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<tr>
<td>Regulation</td>
<td>7</td>
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* Baseline (GB, France, Spain, Italy, Poland and Germany)

** Ranked according to the percentage of respondents who reported good/very good knowledge of a particular area. (data Parson et al. BMJ Open 2015)
What do the pharmaceutical industry professionals in Europe believe

- Patient and public involvement (PPI) in medicines R&D is an emerging area which is likely to be challenging to implement in the global pharmaceutical industry due to different working practices, regulatory environments and interpretations of codes of practices.

- Varying views regarding patients’ roles in their healthcare across different countries may also present a challenge.

- Increasing PPI in medicines R&D is likely to take some time and resources, particularly in the development of patient information, as well as potential amendments to existing pharma codes of conduct.
Today are largely unaware about:
• Clinical trials
• Translational research
• Personalized medicine
• Pharmacoeconomics
• Their potential supportive roles in those areas

**EUPATI Vision for 2020**

Educate patients to be active in the Drug Development process – from A-Z
• Participation in clinical trials
• Protocol design
• Informed consent
• Ethical review process,
• Marketing authorization,
• Value assessment
• Healthcare policy
• Others
can really make a unique difference to patient empowerment and to medicines R&D.

You can help us to make it a success.

Jan Geissler
jan@patientsacademy.eu
Objectives of EUPATI

- **Primary objectives**
  - **Implementing the European Patients’ Academy,** empowering patients to be involved in all stages of medicines R&D
  - **Building capacity** by training independent, empowered patients (advocates)
  - **Making patient centricity of medicines R&D a reality**

- **Important outcome:**
  - **This is all about achieving better public health and better drugs,** not about individual interests
  - **To implement a true partnership of patients, academia and industry** and regulators
  - **Patients actively and systematically involved** in all stages of medicines R&D, across diseases, stakeholder groups and country borders
The goal for EUPATI

EUPATI will provide scientifically reliable, objective, comprehensive information on medicines development from **A to Z** to be used by patients and patients organizations across Europe to increase their capacity to be effective advocates and advisors.

**Important**

The material will be *neutral* i.e.

- **Not** disease specific
- **Not** medicine specific
- **Not** pharma specific
EUPATI is developing education targeted to different levels

- **EUPATI Patient Experts Training Course** -- for expert patients
  - 100 patient experts

- **EUPATI Educational Toolbox** -- for patient advocates
  - 12,000 patient advocates

- **EUPATI Internet Library** -- for the health-interested public
  - 100,000 individuals

Languages available:
- English
- French
- German
- Spanish
- Polish
- Italian
- Russian
Expert-Level Training Course

Online self-learning combined with 2 face-to-face meetings

Approx. 250 hours of e-learning and 8-10 days for two F2F meetings
Timeline 2015-2016 Course


Module 1
Module 2
Module 3
Module 4 (I)
Module 5 (I)
Module 4 (II)
Module 5 (II)
Module 6 (I)
Module 6 (II)

Face to Face event 1
Face to Face event 2
# Overview of the course modules

<table>
<thead>
<tr>
<th>Module</th>
<th>Description</th>
</tr>
</thead>
<tbody>
<tr>
<td>Module 1</td>
<td>Discovery of medicines and Planning of Medicines Development</td>
</tr>
<tr>
<td>Module 2</td>
<td>Non-Clinical Testing and Pharmaceutical Development</td>
</tr>
<tr>
<td>Module 3</td>
<td>Exploratory and Confirmatory Clinical Development</td>
</tr>
<tr>
<td>Module 4</td>
<td>Clinical Trials</td>
</tr>
<tr>
<td>Module 5</td>
<td>Regulatory Affairs, Medicinal Product safety, Pharmacovigilence and Pharmacoepidemiology</td>
</tr>
<tr>
<td>Module 6</td>
<td>Health Technology Assessment (HTA) principles and practices</td>
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Practical “Roadmap” on patient involvement in R&D:
Patient organizations’ and expert patients’ contribution to the whole R&D development life

**Research design and Planning**

- **Protocol Synopsis:**
  - design, comparators

- **Informed Consent**

- **Study reporting**

- **Trial steering committee**

- **Patient Info Leaflet**

- **Investigators Meeting:**
  - **Level of expertise in the disease area required:**
    - high
    - medium

**Research conduct and operations**

- **Data Monitoring Committee**

- **Information to trial participants**

**Dissemination, communication, post-approval**

- **Regulatory affairs**

- **Health Technology Assessment**

- **Post-study communication**

**Setting research priorities:**

- **Design of Protocol:**
- **Practical considerations**
- **Fundraising**

Source: Geissler, Ryll, EPALCO (2014, unpublished)
Audience 2
Introducing the European Patients’ Academy (EUPATI) Toolbox on Medicines Research & Development
What is the EUPATI Toolbox?

- Over 3000 pieces of content for patients who want to **DISCOVER** more about the medicines research and development process and the role they can play.

- Tools are published under a creative commons license, meaning that you can **ADAPT** them, or add to them, or delete parts of them to suit your learning needs, and the educational and training needs of your patient community.

- Mobile, tablet and desktop accessible, fully downloadable and printable so that you can easily **SHARE** tools with other patients.
The EUPATI Toolbox [www.eupati.eu](http://www.eupati.eu)

By 27th February:
1 month after launch
- 6000+ unique users
- 30,000 page views
- 120 countries
The EUPATI Toolbox – 6 languages

The A to Z of how medicines are developed
Patient education!

Search our library by keyword
For example: drug discovery process

Search our library by category

Basics of Medicine
- Types of Medicine
- Drug Discovery
- Safety of Medicines

Pharmaceutical Development
- Clinical Development and Trials
- Personalised Medicine
- Benefit and Risk Assessment

Regulatory Affairs
- Health Technology Assessment
- Non-Clinical Studies
- Pharmacoeconomics
The EUPATI Toolbox – word search

The A to Z of how medicines are developed

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The EUPATI Toolbox

Search Results for: clinical trials

Statistics in clinical trials: Bias

There are several kinds of bias that may occur in clinical trails, including selection, measurement, and publication bias. Statistics are used in clinical trials in order to avoid or correct these biases.

New approaches to clinical trials: Adaptive designs

Adaptive designs in clinical trials are relatively flexible designs that allow for modifications during the trials.

Enrolling in clinical trials

The process of enrolling in clinical trials is commonly regulated, as is the way in which sponsors advertise clinical trials to prospective participants. Patients are screened and must give their informed consent before they can enrol in a trial.

Compensation in clinical trials

Compensation in clinical trials is not always a standard but may be offered to participants according to different models and in line with the respective legislation and regulations.

Clinical trial designs

There are several types of clinical trial designs: randomised or non-randomised, controlled or uncontrolled, single or double blinded trials, and superiority or non-inferiority trials.

Analysing clinical trial results

Clinical trial results are statistically analysed in terms of demographics, efficacy, and types of clinical trial may warrant different interpretations of trial results.

Attachments

- Principles of New Trial Designs
  Size: 650.547 bytes, Format: .potx
  This presentation evaluates the traditional paradigm of clinical development and discusses alternatives, in particular adaptive design for clinical trials.
EUPATI Toolbox – Content available

- Articles
- Infographics
- PowerPoints
- Fact sheets
- Videos

Fact Sheet: Making a medicine – Safety surveillance and life-cycle management

Marketing and post-marketing safety surveillance

The marketing process involves sharing the information about the new medicine with doctors and other healthcare professionals so that they are aware of the effects of the new medicine, and may prescribe it in cases where they believe patients can benefit.

However, the development process does not stop there. There is still a need to collect and analyse the information about the safety of the medicine when it is used in real life (so-called pharmacovigilance). This is because:

- In clinical trials, patients are designed to give clear answers; patients literally only have the disease being studied and are not taking any other medications.
- In real life, a large number of patients have the new medicine. They may have several other diseases and take a variety of other medicines.

Both the clinical trials and real-life data are necessary to fully understand the real benefit-risk ratio.

Blinding in clinical trials
Glossary

- **AB**
  An antibody (AB), also known as an immunoglobulin, is a protein produced by the body's immune system when it detects harmful substances (called antigens). Antigens can be molecules from microorganisms (bacteria, fungi, parasites, and viruses), or chemicals (insect venom). Antibodies recognise and latch onto antigens in order to neutralise them.

- **Absorption**
  In pharmacology and pharmacokinetics, absorption is the process whereby medicines are transported or taken up from the site of administration (by mouth, inhalation, intravenous or intramuscular injection, etc.) to the blood through capillary, osmotic, solvent, or chemical action in the cells. This could be through the intestinal wall, skin, or mucous membranes. In specific situations, such as intravenous (IV) therapy, absorption is straightforward and there is less variability, because the medicine goes directly into the bloodstream. In the case of IV injection, the bioavailability of the compound is 100%. Absorption is a primary focus in medicines development, as a compound must first be absorbed before any medicinal effects can take place. Moreover, the medicine's pharmacokinetic profile can be significantly changed by factors that affect absorption.

- **Acquired Immunodeficiency Syndrome (AIDS)**
  Acquired Immunodeficiency Syndrome (AIDS) is the group of conditions that develop during the advanced stage of infection with the human immunodeficiency virus (HIV). People at this stage of HIV disease have badly damaged immune systems, which puts them at risk of infection. Patients frequently develop infections and tumours that do not usually affect people who have healthy immune systems.

- **ACS**
EUPATI Toolbox – nationale sider

Kontakt: danmark@eupati.eu
https://www.eupati.eu/denmark/

Sample files to download:

- Analyse af resultater af kliniske forsøg
- Biomarkerer
- Epidemiologi
- Evidensbaseret medicin
- Faser i klinisk udvikling
- Forskning og udvikling af lægemidler
- Forskning og udvikling af lægemidler (pstmt)
- Forsøgsdeltageres rettigheder og forpligtelser og patientforeningers rolle
- Kommunikation om risikoen ved lægemidler
- Markedsføringsstilladelse
- Overview of translated material
- PRO – patient rapporteret outcome – i kliniske studier