REiNS Patient Representative Training

Andrea Gross & Claas Röhl

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Response Evaluation In Neurofibromatosis & Schwannomatosis

REiNS

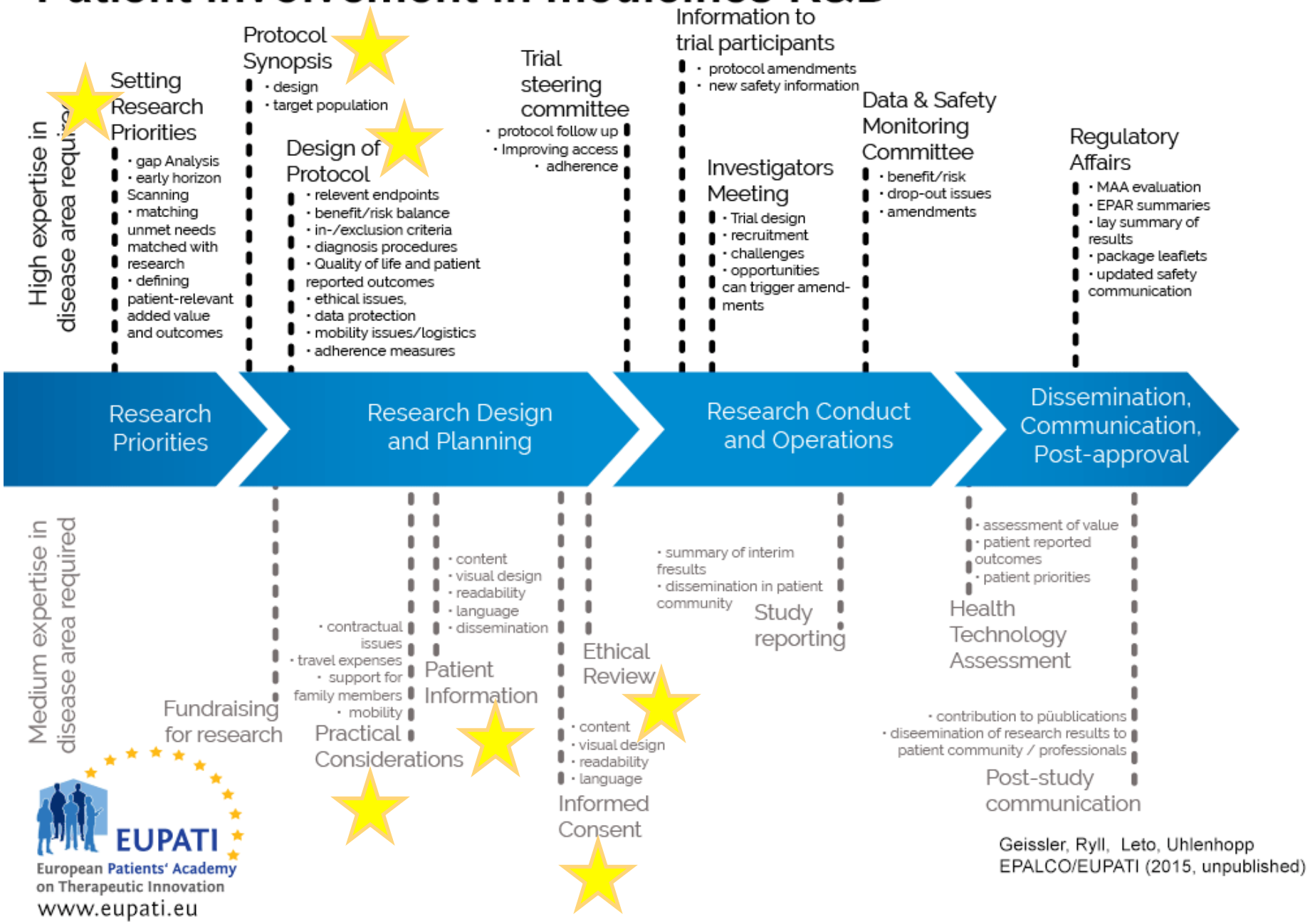
Response Evaluation In Neurofibromatosis Schwannomatosis

INTERNATIONAL COLLABORATION

Patient Engagement in Study Development and Design

- Extremely important!
- Key Benefits:
  - Increased relevance of clinical trial
  - Meaningful trial endpoints
  - Unique perspective on benefits/risks
  - Community engagement/recruitment

Patient involvement in medicines R&D



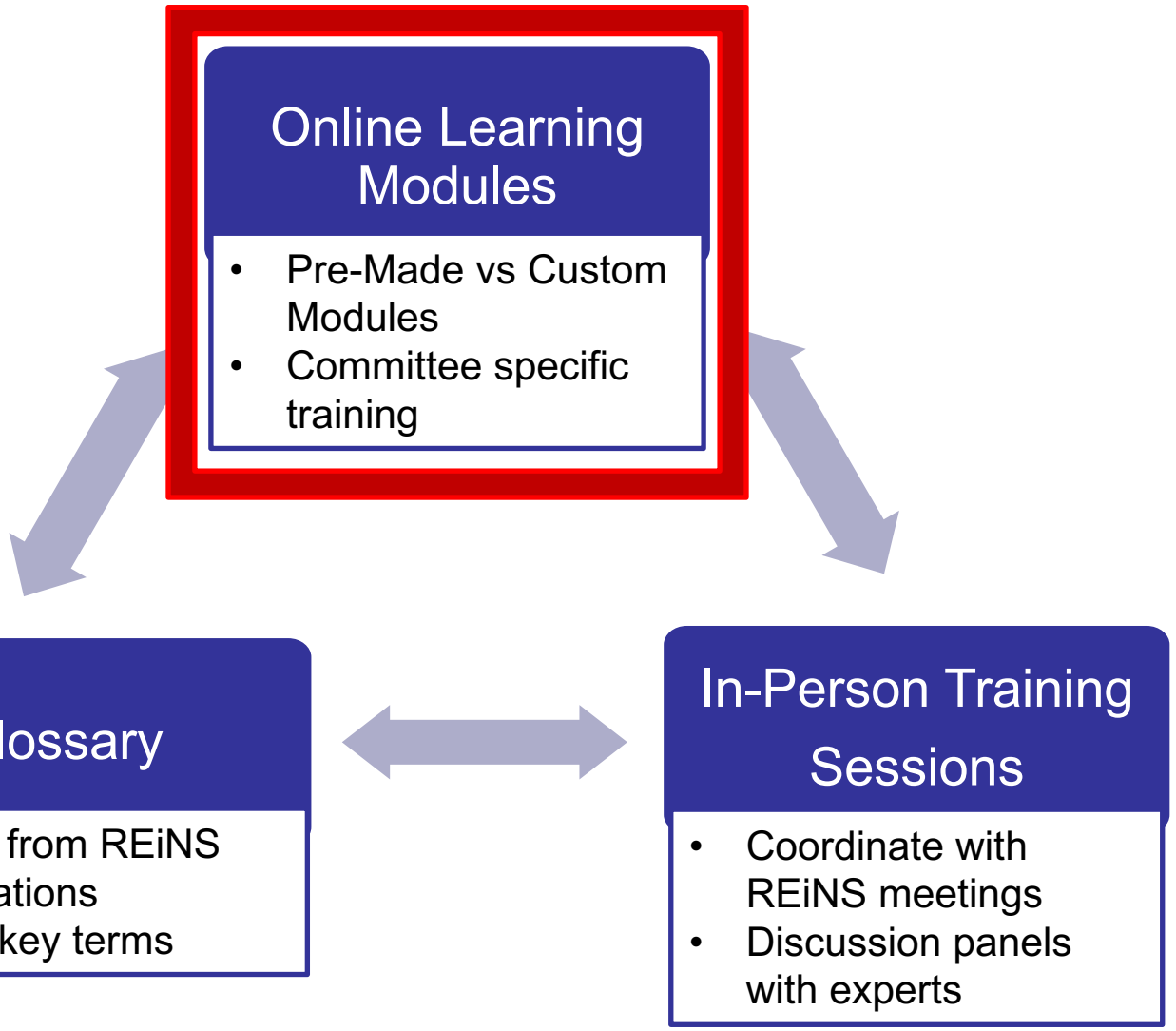
EUPATI
European Patients' Academy on Therapeutic Innovation
www.eupati.eu

Geissler, Ryll, Leto, Uhlenhopp
EPALCO/EUPATI (2015, unpublished)

Potential Barriers to Patient Engagement in Study Design

- ☑ Patients interested in participating in the process
- ☑ Researchers open to working with patient representatives on study design
- ☑ Education for researchers about importance of patient engagement
- ☐ Education for patient representatives about protocol and study design methods

Proposed REiNS Patient Representative Training Paradigm

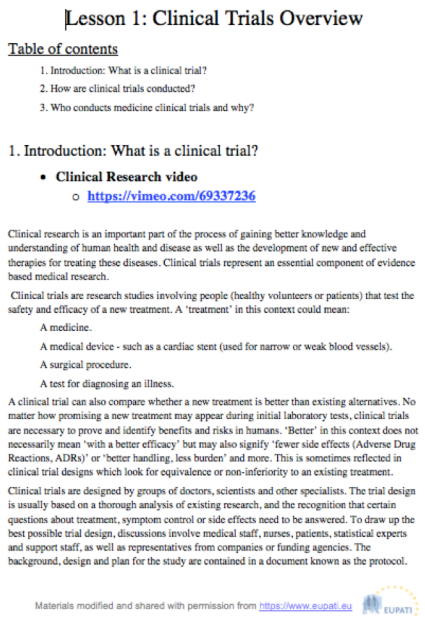


Patient Representative Training: Progress to Date

- Reviewed various available materials for training (e.g. CTF, CITI)
- EUPATI chosen by education subcommittee:
  - Comprehensive
  - Patient-Focused
  - Free access
- Platform for Materials:
  - REiNS Website
  - CTF Website

Educational Materials Online!

- Introduced to the whole Patient Representative committee on Oct 17
- Plan to get feedback at the December REiNS Meeting



Lesson 1: Clinical Trials Overview

Table of contents

1. Introduction: What is a clinical trial?
2. How are clinical trials conducted?
3. Who conducts medicine clinical trials and why?

1. Introduction: What is a clinical trial?

- **Clinical Research video**
  - https://vimeo.com/69337236

Clinical research is an important part of the process of gaining better knowledge and understanding of human health and disease as well as the development of new and effective therapies for treating these diseases. Clinical trials represent an essential component of evidence based medical research.

Clinical trials are research studies involving people (healthy volunteers or patients) that test the safety and efficacy of a new treatment. A 'treatment' in this context could mean:

A medicine.

A medical device - such as a cardiac stent (used for narrow or weak blood vessels).

A surgical procedure.

A test for diagnosing an illness.

A clinical trial can also compare whether a new treatment is better than existing alternatives. No matter how promising a new treatment may appear during initial laboratory tests, clinical trials are necessary to prove and identify benefits and risks in humans. 'Better' in this context does not necessarily mean 'with a better efficacy' but may also signify 'fewer side effects (Adverse Drug Reactions, ADRs)' or 'better handling, less burden' and more. This is sometimes reflected in clinical trial designs which look for equivalence or non-inferiority to an existing treatment.

Clinical trials are designed by groups of doctors, scientists and other specialists. The trial design is usually based on a thorough analysis of existing research, and the recognition that certain questions about treatment, symptom control or side effects need to be answered. To draw up the best possible trial design, discussions involve medical staff, nurses, patients, statistical experts and support staff, as well as representatives from companies or funding agencies. The background, design and plan for the study are contained in a document known as the protocol.

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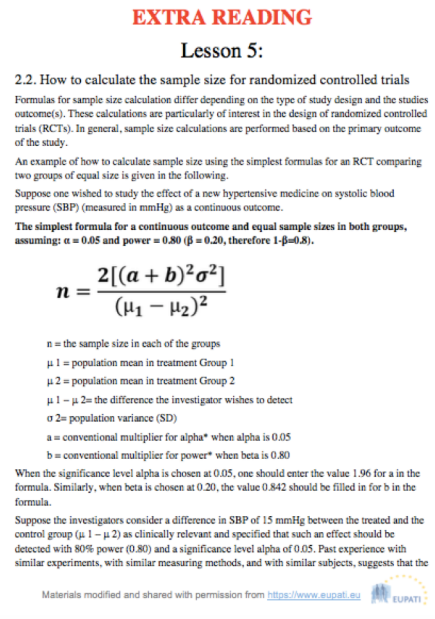

EXTRA READING

Lesson 5:

2.2. How to calculate the sample size for randomized controlled trials

Formulas for sample size calculation differ depending on the type of study design and the studies outcome(s). These calculations are particularly of interest in the design of randomized controlled trials (RCTs). In general, sample size calculations are performed based on the primary outcome of the study.

An example of how to calculate sample size using the simplest formulas for an RCT comparing two groups of equal size is given in the following.

Suppose one wished to study the effect of a new hypertensive medicine on systolic blood pressure (SBP) (measured in mmHg) as a continuous outcome.

The simplest formula for a continuous outcome and equal sample sizes in both groups, assuming: α = 0.05 and power = 0.80 (β = 0.20, therefore 1-β=0.8).

$$n = \frac{2[(a+b)^2\sigma^2]}{(\mu_1 - \mu_2)^2}$$

n = the sample size in each of the groups

μ 1 = population mean in treatment Group 1

μ 2 = population mean in treatment Group 2

μ 1 – μ 2= the difference the investigator wishes to detect

σ 2= population variance (SD)

a = conventional multiplier for alpha* when alpha is 0.05

b = conventional multiplier for power* when beta is 0.80

When the significance level alpha is chosen at 0.05, one should enter the value 1.96 for a in the formula. Similarly, when beta is chosen at 0.20, the value 0.842 should be filled in for b in the formula.

Suppose the investigators consider a difference in SBP of 15 mmHg between the treated and the control group (μ 1 – μ 2) as clinically relevant and specified that such an effect should be detected with 80% power (0.80) and a significance level alpha of 0.05. Past experience with similar experiments, with similar measuring methods, and with similar subjects, suggests that the

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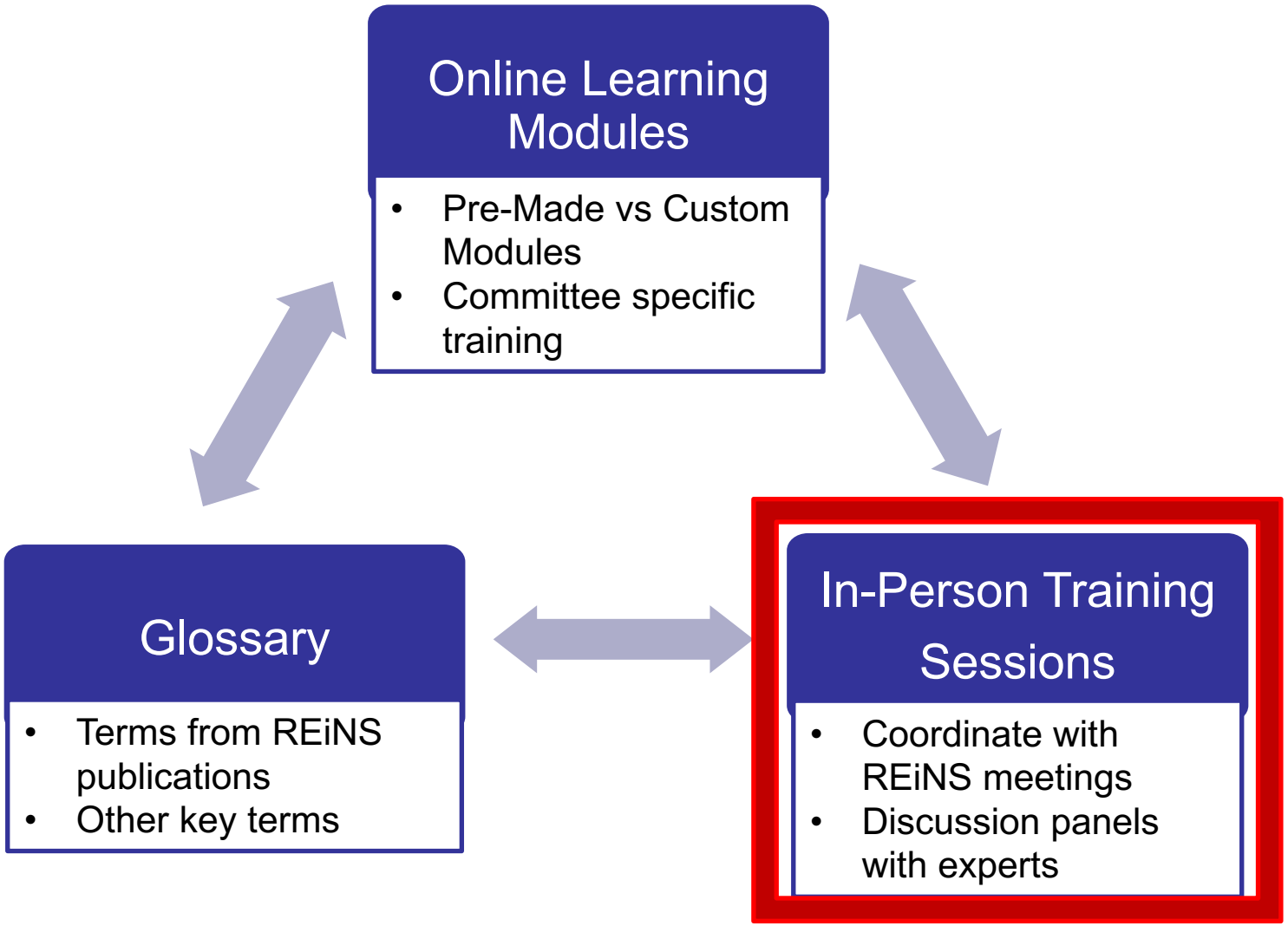
Future Plans for Online Materials

- Incorporate patient representative feedback
- Add additional NF/Schwannomatosis specific materials
- Add committee specific educational materials

Proposed REiNS Patient Representative Training Paradigm



In-Person Training Session

- December 2018:
  - REiNS Meeting focused on cutaneous NF
  - Pre-Meeting with REiNS subject experts and patient representatives to give overview of material before main conference
- Goal:
  - Encourage understanding of background and jargon to help increase patient representative participation in the main conference

Future Directions

- Additional online Modules
  - Webinars
  - Disseminate clinical trial results
- Future In-Person training Sessions
- Glossary of Terms for REiN articles

THANK YOU to the REiNS Patient Representative Education Subcommittee:

- Dale Berg
- Claas Röhl
- Maureen Hussey
- Sarah Adsit
- Herb Sarnoff
- Barbara Franklin
- Scott Plotkin

…and many others who have contributed ideas and have volunteered to help moving forward!

Any Questions?