PROTECT

imi efpia

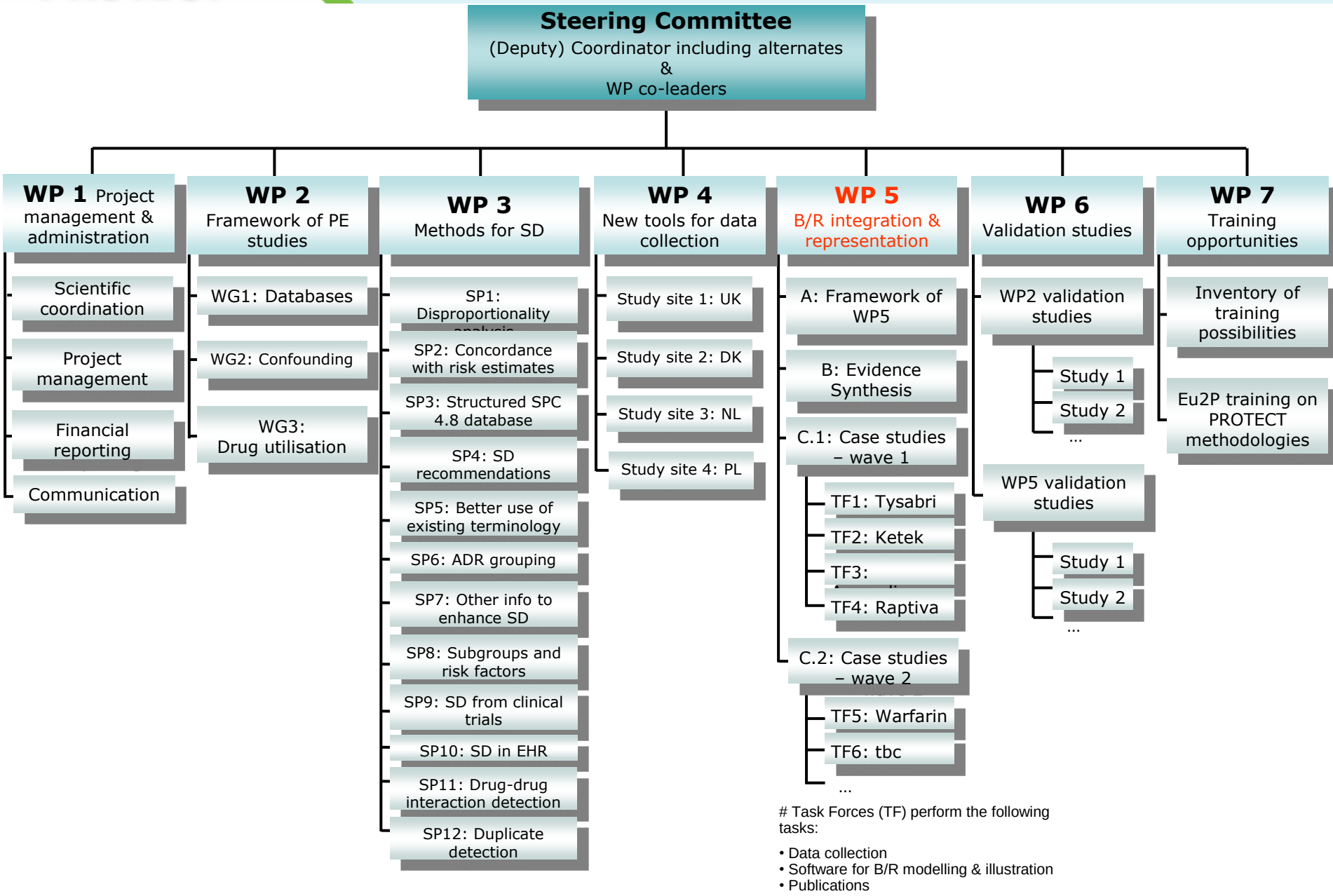
Pharmacoepidemiological Research on Outcomes of Therapeutics by a European Consortium

The PROTECT project

Work Package 5: Benefit-Risk Integration and Representation

PCWP May 2012

Presented by Georgy Genov, EMA



Task Forces (TF) perform the following tasks:

- Data collection
- Software for B/R modelling & illustration
- Publications

PROTECT

Representing the benefit and risk of new medications is a challenge

- Issues include how to weigh adverse effects/side effects against clinical benefits
  - Especially if these occur in different time frames
- Further issues include how to represent changes over time, e.g. benefit/risk accrued from taking a drug for 1 or 5 years
- Perception of the relative importance of risks and benefits may differ depending on experience of the disease/role in the process e.g. regulator vs patient

Specific objectives of WP5

The overall objective of WP5 therefore is to develop methods for use in

- **benefit-risk (BR) assessment, including both the underpinning modelling and the presentation of the results**
- **with a particular emphasis on graphical methods.**

Work Package 5: Benefit-Risk Integration and Representation

1. Review of methodologies used to model effects of medicines, elucidation of patients' preferences and integrating effects and preferences.

   Review of methodologies for graphical representation and visualisation techniques.

2. Case studies (**waves 1 and 2**)
3. Data selection/requirements for case studies
4. Identification/development of software for B/R.
5. Application of methodology, recommendations, finalisation of tools, protocols for validation studies.

WP participants

35 individuals currently participating in case study teams.

Participants are drawn from:

- **Pharmaceutical companies**
- **Regulatory agencies**
- **Other organisations including Universities**

- Experience in statistics, decision analysis, regulatory and safety processes, visual communication, medicine, pharmacology.

So far....

- We have reviewed the existing tools that have been developed for measuring benefit risk and recommended 13 key tools
- We have reviewed the graphics that are traditionally associated with these 13 tools
- We have carried out 4 initial case studies to try out as many of these tools as possible also trying out both traditional and some new graphics
- We are drawing conclusions about this first phase of testing
- The next stage is to test benefit risk methods in more complex case study situations and to further investigate use of visual representation
- We hope to be able to involve patients and/or patient groups at this stage

Patient involvement

- From an ethical and moral perspective, the values and preferences of patients should be included in regulatory decision-making
  - Who can be involved?
  - How can they be involved?
- PROTECT WP5:
  - Discrete choice experiments will be used to evaluate treatment preferences

Discrete Choice Experiments

- Questionnaire sent out by email to members of patient advocacy organizations:
  - Respondents face a series of hypothetical scenarios (approx. 15 to 20), where they are given information on two hypothetical treatments and have to decide between them
  - I.e. they are asked to select the treatment they prefer the most

Example

	Treatment A	Treatment B
Improvement	35 out of 100 patients	65 out of 100 patients
Mild adverse events	5 out of 100 patients	50 out of 100 patients
Method	Topical cream (3x day)	Injection (1x week)

For the treatment of plaque psoriasis, which treatment would you choose?

Treatment A ☐ Treatment B ☐ Neither ☐

Use of Results

- From the results, we can determine how much patients value the attributes used in the questionnaire
  - This information can then be used to inform the model/analyses used in the evaluation of benefit and risk
  - As this approach requires ethical approval which takes time to obtain we will only be able to explore this in selected case studies.

Wider involvement of patient, consumer and healthcare professional representatives

- In addition to the focused involvement of patient groups: *for the elicitation of preferences to help build the Benefit-Risk models for individual case studies.....*
- We should like to ask for your assistance with regard to the visual representation of benefit and risk.
- As discussed, we are developing a 'toolkit' of methodologies for analysis of benefit and risk and linked visual representations of this.
- We are using our case studies to run different methods of analysis and visual representation to explore different ways of solving some of the complexities inherent in this area.
- By autumn 2012 we will have examples of different visual representations
- We would vey much like to present these to you and to ask for your feedback

Wider involvement of patient, consumer and healthcare professional representatives

- We would be delighted to receive any thoughts, feedback.
- Tentatively we could propose that we present our visualisation outcomes to you in winter 2012 or spring 2013. Adapt in accordance with your comments and return in summer or autumn 2013 to present an updated version.
- Following this early evaluation we would plan to link in to EUPATI to explore refining the toolkit for different levels of expert patient/consumer .
- We hope to engage with the healthcare professionals working party in a similar manner.
- The final step would be to carry out a formal and rigorous evaluation of the toolkit with patient populations, for which ethical approval and additional funding would be required.

Today we should like to,

- Ask if you have any comments on the process so far?
- Ask for your thoughts with regard to our plans going forwards....
  - Particularly whether you would be interested in helping to provide comments with regard to the visualisation and if so...
  - Discuss how we proceed from here in terms of setting up future contact

PROTECT

Thank you!