Evaluation of drugs in rare diseases

Eudipharm & EMEA seminar

London, Thursday 6 and Friday 7, April 2006

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Rare diseases belong to an area of great therapeutic needs. However, innovation, development and evaluation of new treatments for rare diseases are impaired by a number of well known hurdles. The aim of this Eudipharm-EMEA two-day seminar is to address barriers to drug evaluation in rare diseases. State of the art lectures and new ways to overcome the current barriers will be presented and discussed in order to help the participant in debugging their projects.

Preliminary Programme

Moderators**: Gérard Pons, Jean-Pierre Boissel, Jean-Marc Husson, Agnès Saint Raymond**

DAY 1 Thursday 6th April 2006

1) 10h00 - 10h50: **Josep TORRENT-FARNELL, Fundacio Dr. Robert, Barcelona, Spain: Orphan drug clinical development: looking for a new paradigm**

- Opening new ways to overcome long standing hurdles

2) 10h50 - 11h40: **Giuseppe REMUZZI, Negri Bergamo Laboratories, Bergamo, Italy: Specificities and specifications of rare disease therapies**

- Treating rare patient presenting a rare disease raises specific issues; inclusion in a trial needs a careful assessment of patient's wishes, history and environment

3) 11h40 - 12h30: **Yann LE CAM, EURORDIS, Paris, France: Role of patients groups**

- Networks for facilitating patient recruitment; funding research; participating in development of agenda and trial design (primary outcome, placebo, trial duration)

12h30 - 13h30 LUNCH

4) 13h30 - 14h20: **Patrice NONY, School of Medicine RTH Laennec, Lyon, France: N of 1 design**

- N of 1 design looks fitting well with therapy evaluation in rare diseases. Is it true? How to pool data from several N of 1 trial?

5) 14h20 - 15h10: **Stuart POCOCK, London School of Hygiene and Tropical Medicine, London, United Kingdom: Relevant unbiased designs**

- Can current principles for bias management in trials specific for rare disease? Apparent barriers for using randomised trials in rare diseases are shortage in patients and ethical issues. Are they real barriers? If yes, could they be overcome? If not, why? Do non randomised designs play a role in rare diseases? Are they less likely to give biased results in such an instance? Are biased results less a problem in rare diseases?

6) 15h10 - 16h00: **Christian GLUUD, Rigshospitalet Copenhagen University Hospital, Copenhagen, Denmark: Control intervention**

- The choice of control treatment is even much more an issue in controlled trials in rare diseases. There are very little established therapies although there are a number of standard – sometimes recommended – treatments.

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16h00 - 16h30 BREAK

7) 16h30 - 17h20: **Jacobus LUBSEN, SOCAR Research SA, Nyon, Switzerland: Muticentre trials in rare diseases**

- Setting and running a trial – most of the time multicentre – in rare diseases does not raise really new issues. However, attention should be paid to some specific problems.

8) 17h20 - 18h10: **Pierre CHATELAIN, Hospital Debrousse, Lyon, France: Short term efficacy – long term efficacy**

- Most of rare diseases impair both life expectancy and daily life. Treatments should be evaluated after either a short time or a long time or both.

9) 18h10 - 19h00: **Marc BUYSE, National Institute of Cancer, Paris, France: Surrogates and biomarkers in rare diseases**

- Because long time effects are sometimes difficult to wait for, surrogacy is considered. However, is assessing the validity of a surrogate outcome easier in rare diseases?

10) 19h00 - 19h30: **Round table: QA on today's topics**

DAY 2 Friday 7th April 2006

1) 9h00 - 9h50: **Rembert ELBERS (to be confirmed): Long term follow-up and risk management for orphan medicines**

- Is traditional pharmacovigilance relevant to drugs in rare diseases? Either because the drug is specific to the disease or because its interactions with the diseases and / or patients is specific, information obtained through the pharmacovigilance system in adult is rarely fully relevant. Also, the size of the cohorts is small. A dedicated approach is needed.

2) 9h50 - 10h40: **Jose GROSSWASSER, Queen Fabiola University Hospital, Brussels, Belgium: Is there any specificity in ethics for rare diseases?**

- Although the question has been raised already at the beginning of the seminar, it's worth coming back in depth on this issue: should the ethical rules and the moral perspective be different in rare diseases?

3) 10h40 - 11h30: **Brendan BUCKLEY, University of Cork, Ireland: EU support for orphan Drugs**

11h30 - 12h30 LUNCH

4) 12h30 - 13h20: **Silvio GARATINI, Istituto Ricerche Farmacologiche Mario Negri, Milan, Italy: Rare disease investigational centres in Europe**

- Are networks of expertise and facilities for evaluating new therapies in rare diseases needed? Do they already exist in Europe?

5) 13h20 – 14h10: **Agnès SAINT RAYMOND, EMEA, London, United Kingdom: Guidelines for clinical drug development in rare diseases**

- Where are we today?

6) 14h10 – 15h00: **Dirk MENTZEL, Paul Erhlich Institute, Germany: Pharmacovigilance in Rare Diseases**

- Do the present tools of the routine Pharmacovigilance System match with the special demand evaluating ADR in rare disease.

- Present situation versus possibilities available.

15h00 - 16h00 BREAK

7) 16h00 - 16h50: **Industry's point of view, Dr José PICO, AMGEN, PARIS, FRANCE**

- What difficulties are pharmaceutical companies facing when developing a new treatment in rare diseases? Do they see way for alleviating the barriers?

8) 16h50 - 17h30: **Round table: QA on today's topics**

9) 17h30 - 17h45: **Closing remarks**

10) 17h45: **End of the workshop**
