Dr Susan Conroy

Chief Executive

Expectations for the ENPR EMA from an SME perspective

Therakind

- Paediatric healthcare company (SME)
- Expertise in scientific evaluation, drug formulation, clinical development and regulatory approvals
- Specialises in developing licensed children's medicines

The heart of the problem – Paediatric formulations

Age	Inadequate dosing information	Dose information but no paediatric dosage form
< 1 month	80.5%	26.5%
1–3 months	79.1%	25.1%
3 months – 2 years	77.5%	23.3%
2–6 years	73.2%	21.9%
6–12 years	71.6%	24.0%

- 25% of 'licensed' drugs have no appropriate paediatric dosage form

Tan E, Cranswick NE et al (2003). Dosing information for paediatric patients: are they really 'therapeutic orphans'? Med J Aust.;179(4):195-8

Therakind's vision

- Create a portfolio of on-label medicines for children
- Improve availability of paediatric medicine
- Address the significant unmet need for paediatric drugs

Therakind's Concept

- Identify therapeutic and market needs
- Identify partners
- Drug formulation
- Scientific advice and input
- Clinical development programme
- Regulatory requirements, strategy and approvals

Therakind's Concept

Where might EU networks help?

- Identify therapeutic and market needs
- Identify partners
- Drug formulation
- Scientific advice and input
- Clinical development programme
- Regulatory requirements, strategy and approvals

Therakind

- Recent experience of paediatric clinical trials
- UK and EU, both within and outside of any network
- Single and multicenter

Key to success is the best possible (study) TEAM

What does TEAM mean?

- Investigator
- Full study team
  - including nurses, pharmacist, etc.
- Research & Development Office

How could an EU network help? J3

- Medical experts – high scientific quality of the study/ early involvement in study design (also with respect to practical issues)
- Early identification of differences in treatment regimes that might affect trials
- Precise local issues
  - e.g. consent in a particular country
- Ensuring consistent costing using an appropriate template
- Ensuring contracts are processed rapidly
- Establish contacts with study sites

Slide 9

J3 is the red font supposed to indiate those items that network can help with? If so, shoudl they not all be red?
Jackie, 07/03/2011

Advantages of the network for an SME

- Expert therapeutic network
  - Clinical expert identification
    - State-of-the-art treatment of condition nationally and internationally
    - Study design also with respect to feasibility in practice
    - Prevalence of condition/number of potentially available patients
- Paediatric clinical trial network
  - Identify high quality study sites
  - Provide information on specific local requirements

Potential issues

- Is the network adequately resourced?
- Can ‘the network’ have just a limited role?
  - e.g. site selection, but not staff
- Does the network verify the site is adequate for a study?
  - e.g. monitoring of equipment, storage of samples
- Is there duplication of effort?
  - adoption and request for changes
- How effective can the network be at ensuring agreed timelines are met?