WP10: FP7 projects

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Evaluation of off-patent medicines in Europe: The FP7 experience

London, 30 January 2014

Representatives of projects funded through the EC FP7 on off-patent medicines in children were invited to a meeting organised by the Enpr-EMA chair and hosted by the European Medicines Agency.

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- To share experience with other international networks funded by FP7
- To identify best practice for evaluating off-patent medicines in Europe
- To identify key learning points for investigators, sponsors and regulators
- To summarise the progress so far of the FP7 projects
- To develop strategies that optimises the development and evaluation of off-patent medicines in Europe in order to avoid "reinventing the wheel" in future projects
- To write an Enpr-EMA position paper about the development of off-patent medicines for children through private-public partnerships

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There participants from 12 / 20 projects:

- Adriana Ceci (DEEP)
- Carlo Giaquinto (NeoMero, NeoVanc)
- Gilles Vassal (O3K)
- Heike Rabe (NeoCirc)
- Stephanie Laer (LENA)
- Valery Elie (TINN, TINN2)

Representatives from SMEs:

- Martin Whittaker (Diurnal Ltd; TAIN project)
- Vincent Grek (O4CP; LOULLA and PHILLA project; NEMO project)
- Luc-Andre Granier (Advicenne; KIEKIDS project)

Apologies received from Alan Boddy, Evelyne Jacqz-Aigrain, Eugene Dempsey, Hugo Lagercrantz, Irja Lutsar

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"Successful private-public funding of paediatric medicines research: lessons from the EU programme to fund research into off-patent medicines”

Lucia Ruggieri, Viviana Giannuzzi, Paola Baiardi, Fedele Bonifazi,; Elin Haf Davies, Carlo Giaquinto, onato Bonifazi, Master in Business Administration; Mariagrazia Felisi, Catherine Chiron, Ronit Pressler, Heike Rabe, Martin J Whitaker,; Antje Neubert, Evelyne Jacqz-Aigrain, Irmgard Eichler, Mark A Turner, Adriana Ceci,

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Summary of FP7 projects

- Twenty paediatric projects have been funded from a total of 76 proposals, investigating 24 medicines, in 10 therapeutic areas and all paediatric age-groups.
- 246 partners are involved including 51 private companies, including at least 40 SMEs.
- 15 Paediatric Investigation Plans have been approved.
- 32 paediatric clinical trials are planned,
- about 7300 children are planned to be recruited in more than 380 investigational centres.

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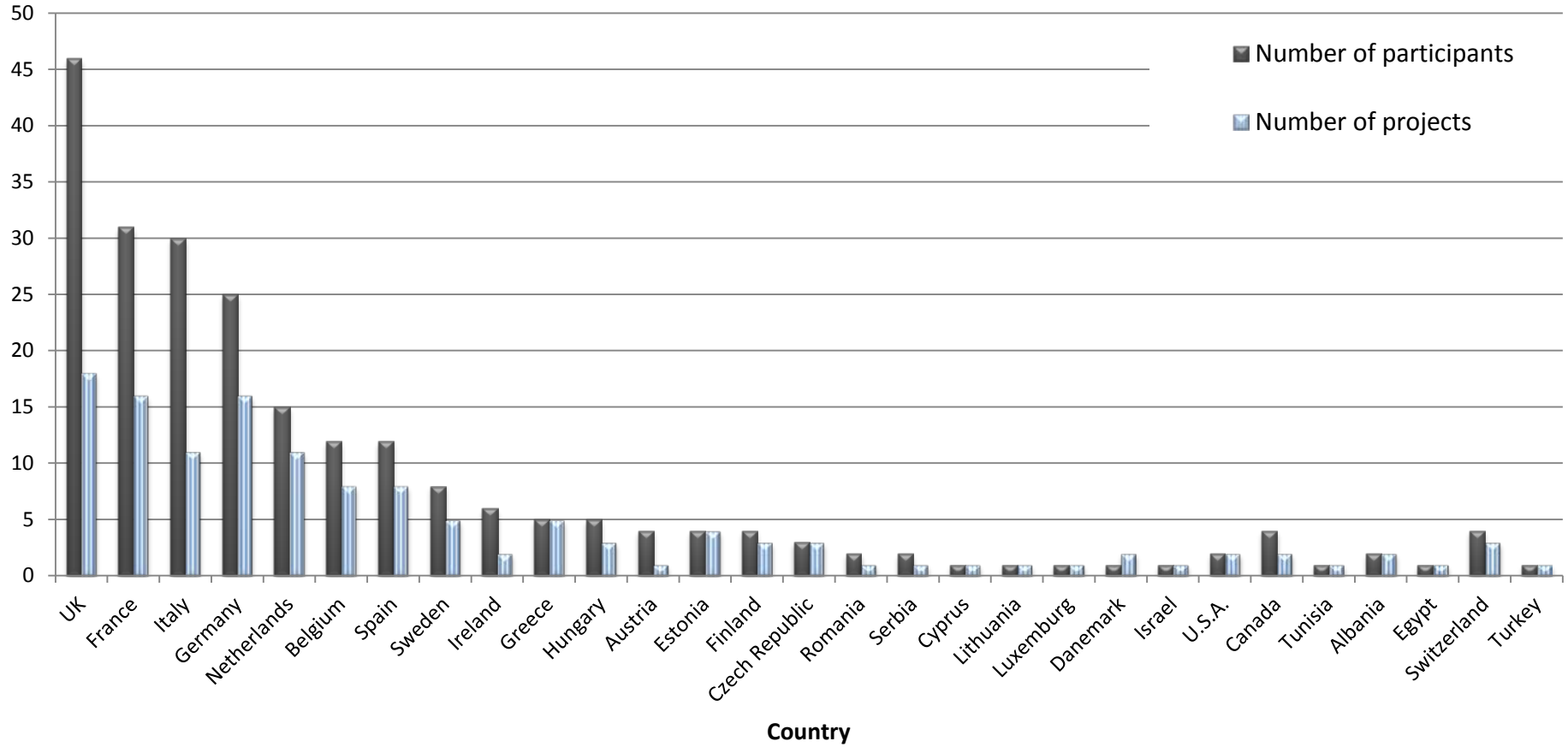

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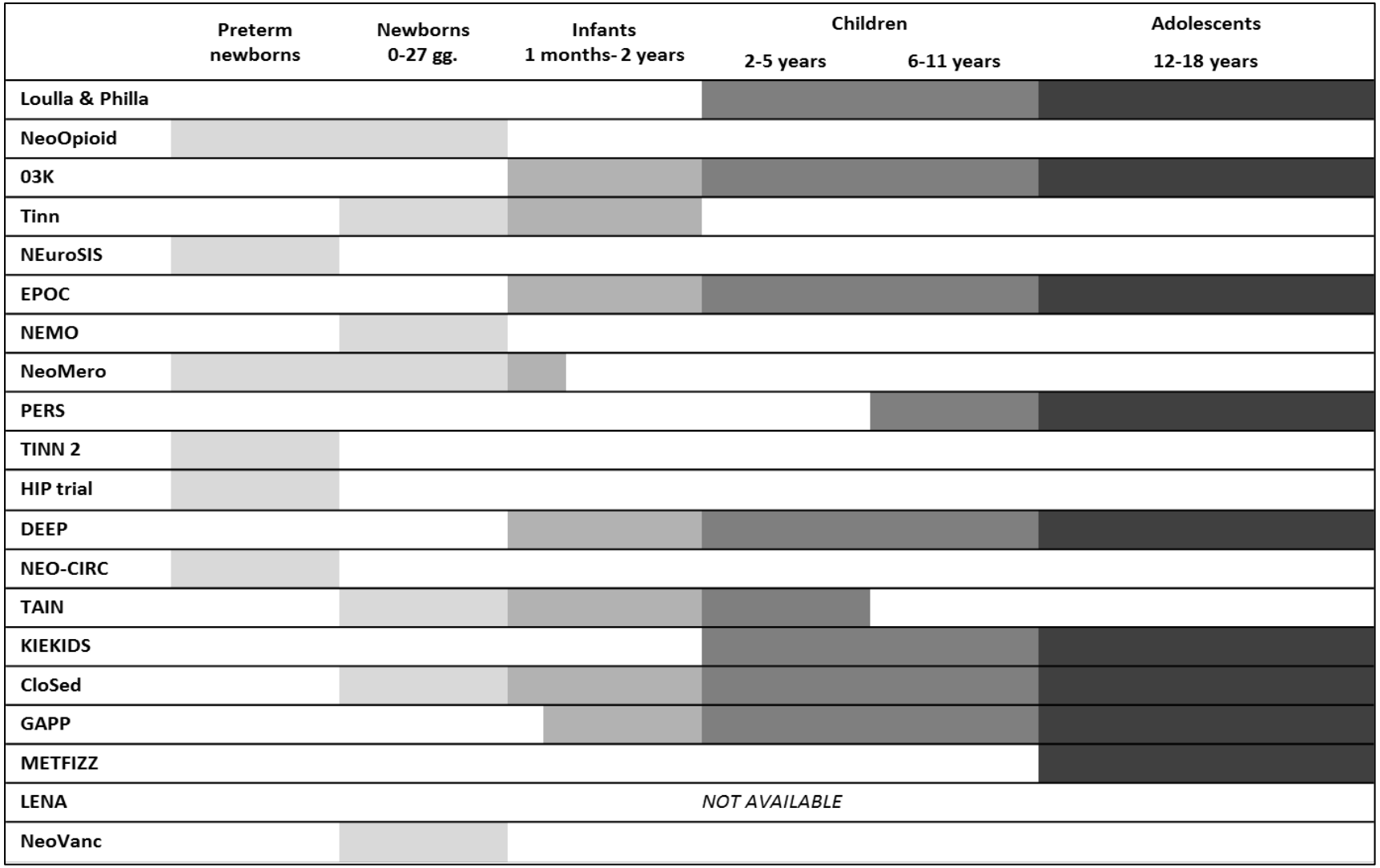

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Consortium perspectives

- Recruitment difficulties, thus necessitating a request for extension of the project
- Unforeseen problems/difficulties while developing new age appropriate formulation, necessitating a request for extension of the project, increasing the costs
- Difficulties for academia consortia to find SMEs willing to become a partner
- Approval time for a submitted PIP was not accounted for in the agreed project duration

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Consortium perspectives

- Preparing a PIP is costly and needs specific funds
- Even if PIP is agreed, no guarantee that CHMP will accept
- One project failed completely because during the agreed project period the SME partner went into bankruptcy, it was difficult to find a new industry partner and the requested extension of project duration was declined by the EC.

Consortium perspectives

- Most successful partnerships either had a pre-existing interest in the field that the call provided resources for, or, had developed a PIP before submitting a bid.
- Consortia need to consider “deep learning” to overcome the issues related to implementation of GxP in multiple organizations
- The consortia do not meet all the costs of drug development. Hospitals and other organizations have to bear significant costs in addition to the FP7 funding.
- Deep learning can include teaching people principles of high quality research rather than just a standard GCP course and building SOPs through meeting and discussing rather than using templates.

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SME perspectives

- while the downstream incentives are good, such as free paediatric scientific advice, the upstream incentives are not adequate: the expected remuneration for off-patent products will not cover the costs for developing age appropriate medicines for children;
- only when developing in orphan conditions, the incentives granted to orphan drugs is attractive enough
- there would be a need for protecting the market, granting exclusivity for not orphan drugs;

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SME perspectives

- the current regulatory/legal systems is unfair as MAH of off-patent products are not required to submit a PIP in contrast to non-MAH, who fall under Art 7 requirements;
- some companies choose a regulatory pathway such as hybrid applications, which are exempt of the requirements of the Paediatric Regulation, to obtain a MA for a novel formulation for use in adults only, expecting that once this formulation which is also appropriate for children is on the market, it would be used off-label in children.

SME perspectives

- Confidentiality about the paediatric development plan would be needed: an agreed PIP publishes the development plans with the risk for SMEs that the original MAH, usually company's with more financial and staff resources than SMEs, could identify the market, develop a much simpler development plan because of their ability to avoid developing a PIP, complete it faster and place it on the market at a lower price than SMEs would need to recover the development costs.

SME perspectives

- Not all academic investigational sites are familiar with regulatory and GCP requirements, thus rendering the conduct of clinical regulatory trials difficult and prone to fail.
- Additional requirements, such as additional studies, development of new formulations by the PDCO which render the overall costs of the development programme substantially higher but are not covered by the EC fund.

Solutions to PUMA issues

- To reduce the costs of off-patent medicines in children by developing and maintaining a small number of manufacturing center(s) of excellence to manufacture age appropriate formulations; usually it's only a small market to supply.
- the EC should accept and anticipate that drug development programmes are prone to many unforeseen difficulties and problems, and should allow more flexibility regarding duration of project and potentially costs

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Solutions to PUMA issues

- EC should consider changing the legal regulatory requirements for PUMA, increase the incentives, protect the market.
- To consider changes to the pricing of off-patent medicines with MA (although this would be difficult because of off-label use of other products, extemporaneous formulations and Specials products)

Learning points

- A lot of enthusiasm for research leading to applications for Marketing Authorisation
- Some good practice
- Some areas for improvement
- Need to disseminate good practice

Future Work

- Lobby EC
- Formalise learning from experience