Repurposing a medicine

Sub-session 2 of Parallel Session 8 -
New medicines and medical technologies, clinical trials and stakeholders support

4th Conference on
European Reference Networks
21 – 22 November 2018

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Health and Food Safety

Conflict of interest declaration

I have no actual or potential conflict of interest in relation to this programme or presentation

Repurposing and its opportunities

- a new use for authorised medicinal products/ known active substances

✓ already **tested in humans**

✓ **available info** on pharmacology, formulation, dose, toxicity

- **common molecular pathways**
- potential to reduce **cost, risk** and development **time**

➢ **early access** – an accelerated pathway

Current regulatory framework and incentives

- **New therapeutic indication for a well-established substance**, Article 10 (5) of Directive 2001/83/EC, 1 year data exclusivity

- **Orphan drug designation**, a range of incentives includes a 10-year period of market exclusivity, free scientific advice – must be granted before marketing authorisation application submission

- **Paediatric-use marketing authorisation** (PUMA), 8+2 year period of data and market protection

Examples – orphan designation

- **Ibuprofen** solution for patent ductus arteriosus
- **Ciclosporin** (inhaled) for graft rejection after lung transplant.
- **Heparin** for treatment of idiopathic pulmonary fibrosis
- **Zoledronic acid** for the treatment of glioma
- **Propranolol** for treatment of soft tissue sarcoma
- **Dantrolene** for treatment of Wolfram syndrome
- **Ciprofloxacin** (inhaled) for treatment of cystic fibrosis
- **Melatonin** for treatment of Smith-Magenis syndrome
- **Methotrexate** for treatment of alkaptonuria
- **Insulin** for the treatment of short bowel syndrome

Guidance

- **European Medicines Agency**
  https://www.ema.europa.eu/en/human-regulatory/marketing-authorisation

- **National Competent Authorities**
  http://www.hma.eu/

- **European Commission** (EudraLex)
  https://ec.europa.eu/health/documents/eudralex_en

Expert Group on Safe and Timely Access to Medicines for Patients

- STAMP repurposing **scope**
  - multisource medicinal products
  - evidence generated by a third party
  - MAHs have not updated product information
  - no incentives

X withdrawn products reintroduced with a new indication

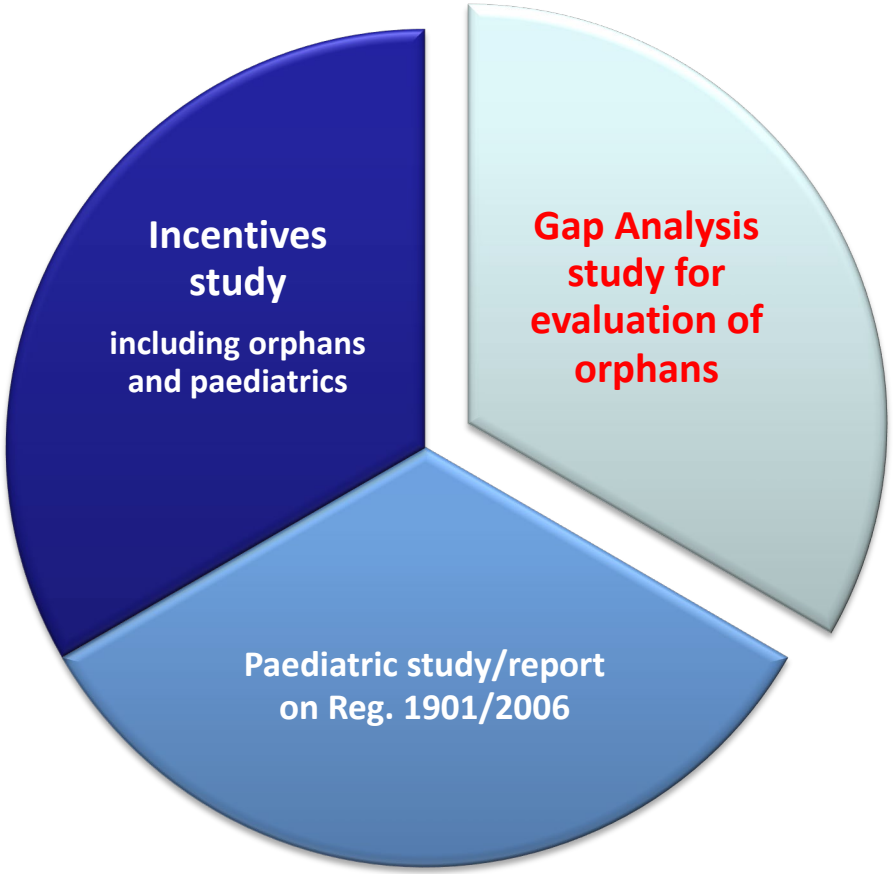
X not for an update of product information

STAMP cont.

Repurposing case studies: barriers / challenges and potential solutions:

- **Engagement with the pharmaceutical industry is desirable**
- **Non-industry developers** raise the issue of **how to proceed to authorisation**, highlighting the lack of regulatory **experience and resource** needed for filing an application (EU will fund support project)
- **Lack of accessible information** / data in the public domain

Evaluation of orphans and paediatrics



Study on Orphans

- The general objective of the study is to gather information and assess, on the basis of the acquired experience, the functioning of the Orphan Regulation

- It will focus on its: relevance, effectiveness, efficiency, coherence and EU added value

- Targetted consultation including ERNs (ended)
- Public consultation until 4 January, see: https://ec.europa.eu/info/law/better-regulation/initiatives/ares-2017-6059807_en

Study on Orphans

TIMELINE

December 2017

Roadmap

4-week public consultation

2018/2019

Study on orphans

Public consultation: 12 October – 4 January 2018
Targeted consultations (incl. ERNs): October – 21 Nov 2018

2019

Evaluation

Staff Working Document

Thank you for your attention

For more information

On STAMP see

http://ec.europa.eu/health/documents/pharmaceutical-committee/stamp/index_en.htm

European Medicines Agency website see

https://www.ema.europa.eu