Medicines & Healthcare products Regulatory Agency

MHRA
Regulating Medicines and Medical Devices

Safe and Timely Access to Medicines for Patients (STAMP)

Background note on re-purposing of established medicines

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Repurposing medicines

- Drug repurposing is the process of identifying a new use for an existing drug in an indication outside the scope of the original indication
  - ➢ could lead to faster development times, reduced costs and risks for pharma

- Repurposing includes:
  - ❖ New therapeutic uses for already known drugs
  - ❖ Developing different formulations for the same drug
  - ❖ Creating new combinations of drugs previously used as separate products
  - ❖ Creating new combinations of drugs with medical devices

  - Identifying re-purposing opportunities comes from a variety of processes including knowledge mining of existing scientific databases, in silico approaches, in vitro and in vivo experiments, clinical observations, epidemiology and post-hoc analysis

Regulatory incentives to support re-purposing

New therapeutic indication for a well-established substance

- Paragraph 5 of Article 10 of Directive 2001/83/EC states that where an application is made for a new indication for a well-established substance, a non-cumulative period of one year of data exclusivity shall be granted

Paediatric-use marketing authorisations (PUMA)

- PUMA is a type of marketing authorisation covering indication(s) and appropriate formulation(s) for the paediatric population
- A PUMA benefits from the 8+2 year period of data and market protection

Orphan drug designation

- The EU offers a range of incentives to encourage the development of medicines intended for small numbers of patients and this includes a 10-year period of market exclusivity for orphan designated products
- For other diseases, there is the provision of ‘unlikely that marketing of the medicine would generate sufficient returns to justify the investment needed for its development’

Discussion points

1. Is there a need to establish specific legal or regulatory terminology and definitions?

2. What are the barriers to re-purposing established drugs for industry and non-profit organisations in terms of:

(a) the development programme

(b) adding new indications to existing marketing authorisations

3. Could existing regulatory routes be used better and in what way e.g. PUMA, orphan designation, including the returns to justify the investment criteria?

4. Is there a need for new regulatory incentives and/ or pathways (non-legislative) to support industry and non-profit organisations

5. Are there particular disease areas that need specific support e.g. neurodegenerative diseases, anti-microbial resistance, rare conditions?

Thank You

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