Amsterdam UMC
University Medical Centers

Proposed revision of the EU pharmaceutical legislation: orphans and repurposed drugs

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Knowledge platform

Projects

Research

Improve access to medicines …

… for patients with rare diseases

Medicines for Society

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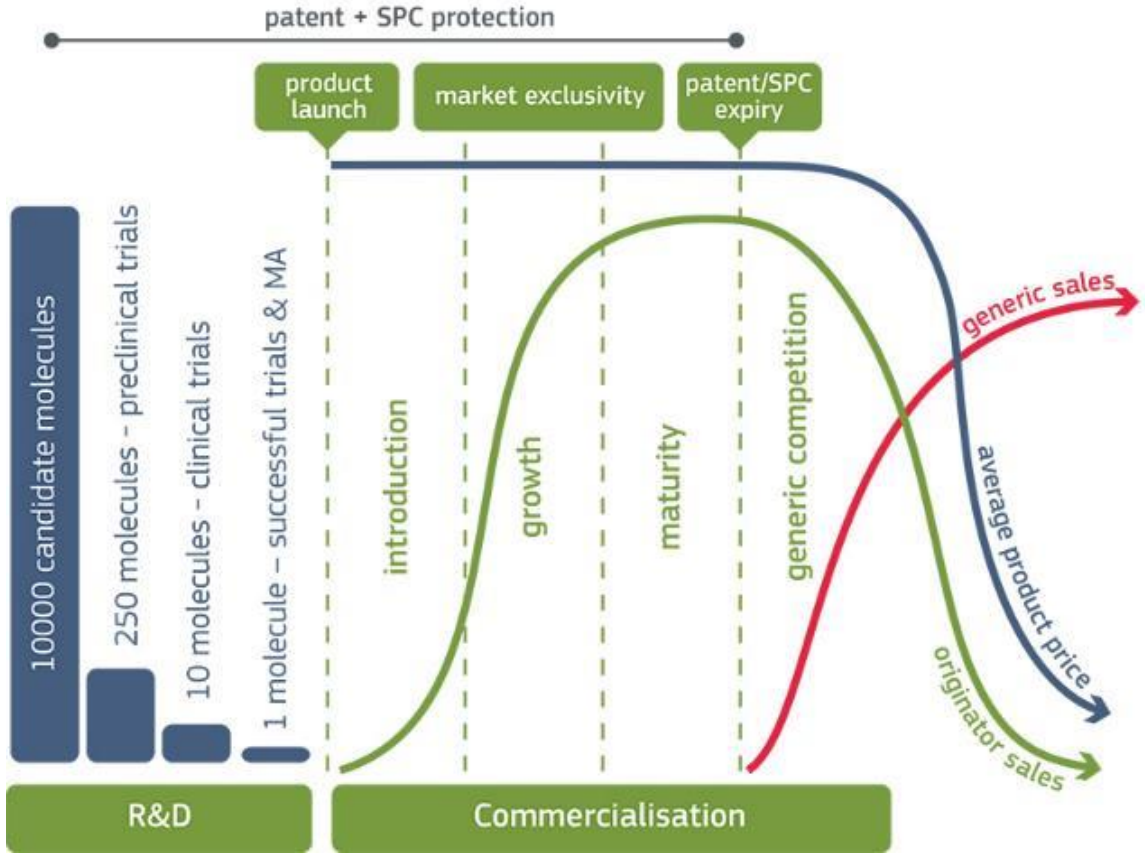
Market exclusivity changes

Preliminary (unpublished work)

- Market exclusivity
  - To stimulate orphan drug development (solidarity) by protecting the market for competitors

    (now: 10y+2y)
- Presumption:
  - Sharp price decrease after patent/ market exclusivity expiration because of competition

- Does this occur in rare diseases?



Picture: European commission (europa.eu)

Market exclusivity changes

Preliminary (unpublished work)

Market exclusivity changes

- Price mechanism after market exclusivity?

    1. Biologicals
    2. Small molecule with small market

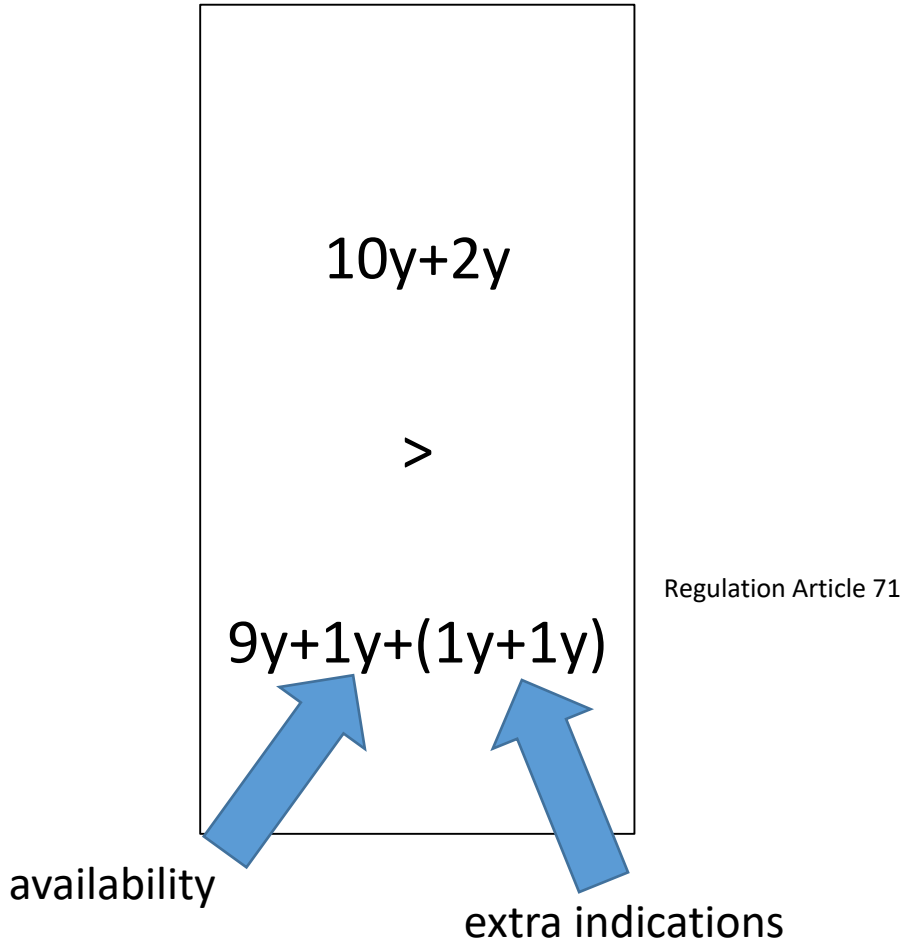
        > no generics/biosimilars or price pressure

    3. Small molecule with large market

        > Some generics and price pressure

- Generic entry long after market exclusivity end

- Will changing the duration of market exclusivity have any effect?



Drug repurposing

- Drug repurposing for rare diseases
  - 1 out of 5 orphans is repurposed
  - Most new indications start in academia but do not get on-label
  - Current routes:
    - Marketing authorization holder
    - New product

- Proposal:
  - Directive article 84: data protection
  - Regulation article 48: not-for-profit

Drug Repurposing for Rare Diseases: A Role for Academia

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Drug repurposing – Directive article 84

- 4 years data protection to existing product for a new indication
  a) Old product (>25y or no earlier data protection)
  b) Significant clinical benefit
  - Once per product

- Addresses non-viable business case 
  - 4 years enough?

- Substitution remains a problem 
  - Enforcable?
  - Who will enforce?

Drug repurposing – Regulation article 48

- ‘Substantial clinical or non-clinical evidence for a new indication’
  - Entities ‘not engaged in economic activity’
  - EMA: scientific opinion
    - **all available evidence**
    - Favourable > MAH**s shall submit** a variation
  - During or after exclusivity

- Long term availability
- Label
- Reimbursement

👍

- What level of evidence?

?

- Withdrawals?

👎

  - Especially when market is small
  - Requires update full dossier
  - Extra PV

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