Rethinking R&D to sustainably address patient needs

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Unmet need: what do we mean?

Focus here is on R&D, but also

1. Slow or missing launches
2. Market withdrawals
3. Reimbursement issues
4. Medicines shortages
5. Prescribing practices, adherence
Pharma gaps: not enough medicines?

Fig. 1: Number of NMEs approved by the FDA and the EMA

Global Medicines in Late Stage Development in 2016

Source: IMS R&D Focus, Sept 2016. Quarterly Note: Drugs Included are beyond Phase II. Gastrointestinal and Hormones includes women's health. Outlook for Global Medicines Through 2020

Hwang et al. 2016
Pharma gaps: when and why?

1) For certain indications
2) For certain patient groups
3) Within indications
due to

- Nascent science
- **Commercial unattractiveness:**
  - Small target populations
  - Short courses of curative treatment
  - Factors complicating study design
  - Limited ability to pay

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**2016 Access to Medicines Index: number of projects for LMIC needs**

- R&D priority (low incentive)
- Other R&D

Cole, Trolle & Edwards 2018
Public funding often comprises a considerable share of R&D expenditure, most associated with basic research. While more than 40% of new medical entities originate with public institutions or SMEs, the vast majority are brought to market by a limited number of large companies. ...we see a similar picture for the UK in Jones & Wilsdon 2018.
What do we know about public R&D funds?

Lack of transparency regarding:

- R&D costs
- Medicines in the pipeline
- Research already commissioned and/or funded by different sources
- Unmet needs in their dynamic nature
- Willingness of public funders to support related work
Reorienting R&D: Pillars of Action

1. **Identifying** (and agreeing on) unmet clinical needs in the population

2. **Communicating** them to the scientific community, developers and other funders (e.g. WHO R&D Observatory)

3. **Securing funding** to address commercial unattractiveness (next slides)

4. Promoting **efficiency** in evidence generation (CTNs, new study designs, full publication of results, streamlined requirements)

5. Ensuring that (only) valuable innovation gets **rewarded** (rethink criteria for marketing approval, priority designation, reimbursement)
Securing funding: which model?

- **Push funding** tools, such as (conditional) grants, direct funding or tax incentives
- **Pull financing** tools, such as (milestone) awards, research tournaments, pay for performance, advance market commitments etc.
- **Pooling** of funds or intellectual property
- **Collaborative** approaches, such as BARDA and the IMI
- **Open** initiatives

→ employ combination of – potentially centralized – push- and pull funding mechanisms to include SMEs and non-entrepreneurial researchers and developers with a reconsideration of current (decentralized) patent-based price signals to guide innovation efforts

→ more and more distinguishing pooled financial resources
New funding models?

An example from DNDi

Medicines funding: the public health view

Food for thought at the end…

• Strategic public buy-in for (some) medicines? Beyond better alignment with unmet need, increased transparency and coordination would facilitate some form of commitment to or obligation for return of investment towards public funders…

• “If we think about unmet need in diabetes, do you really want to fund the next new diabetes drug, which maybe provides a marginal improvement, or do you want to invest in optimizing the long-term management of diabetic patients?” (Quote from a senior expert during last week’s Matchmaking conference in Vienna on alleviating burden of disease)

Thank you for your attention!

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