



# Rethinking R&D to sustainably address patient needs

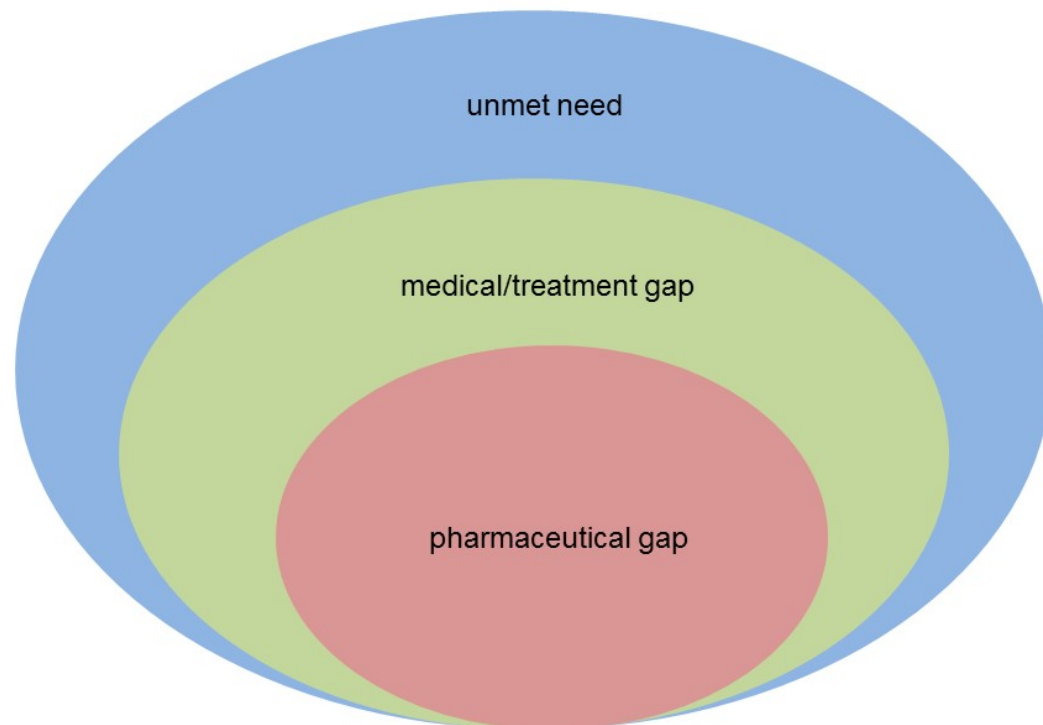
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# Unmet need: what do we mean?



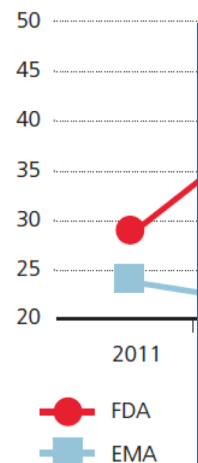
Focus here is on R&D, but also

- (1) Slow or missing launches
- (2) Market withdrawals
- (3) Reimbursement issues
- (4) Medicines shortages
- (5) Prescribing practices, adherence

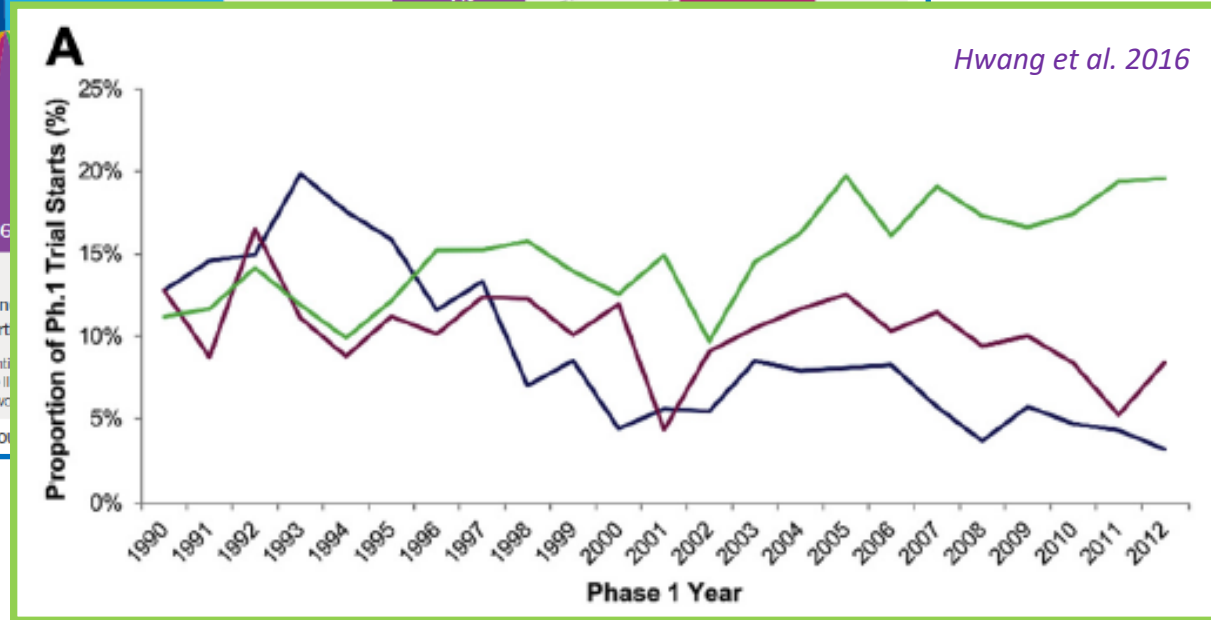
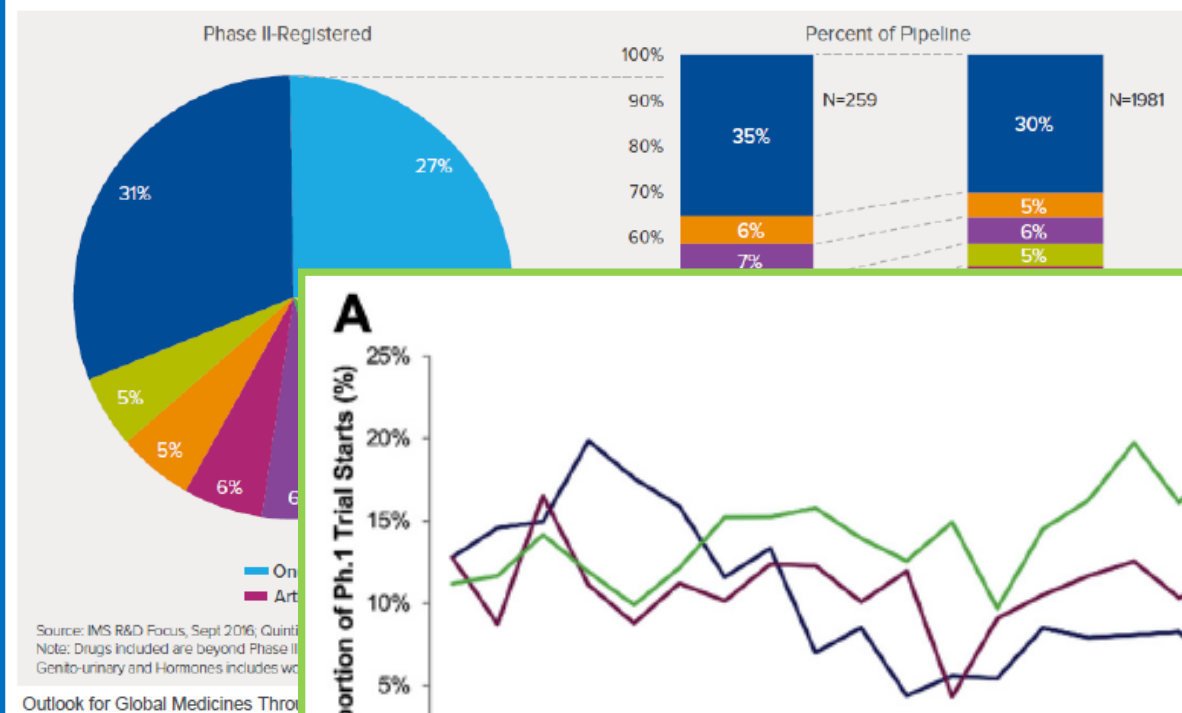
# Pharma gaps: not enough medicines?



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



## Global Medicines in Late Stage Development in 2016



# Pharma gaps: when and why?

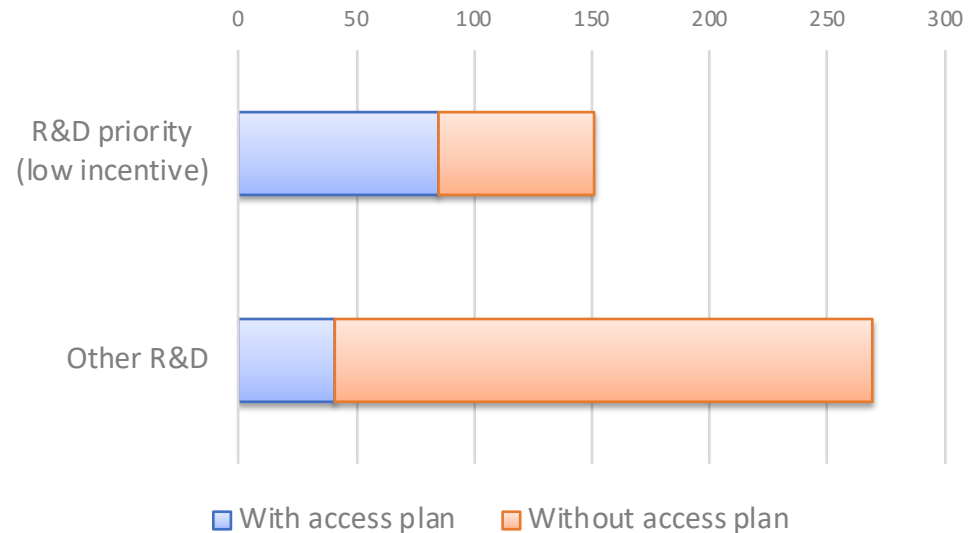


- 1) For certain indications
- 2) For certain patient groups
- 3) Within indications

due to

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

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

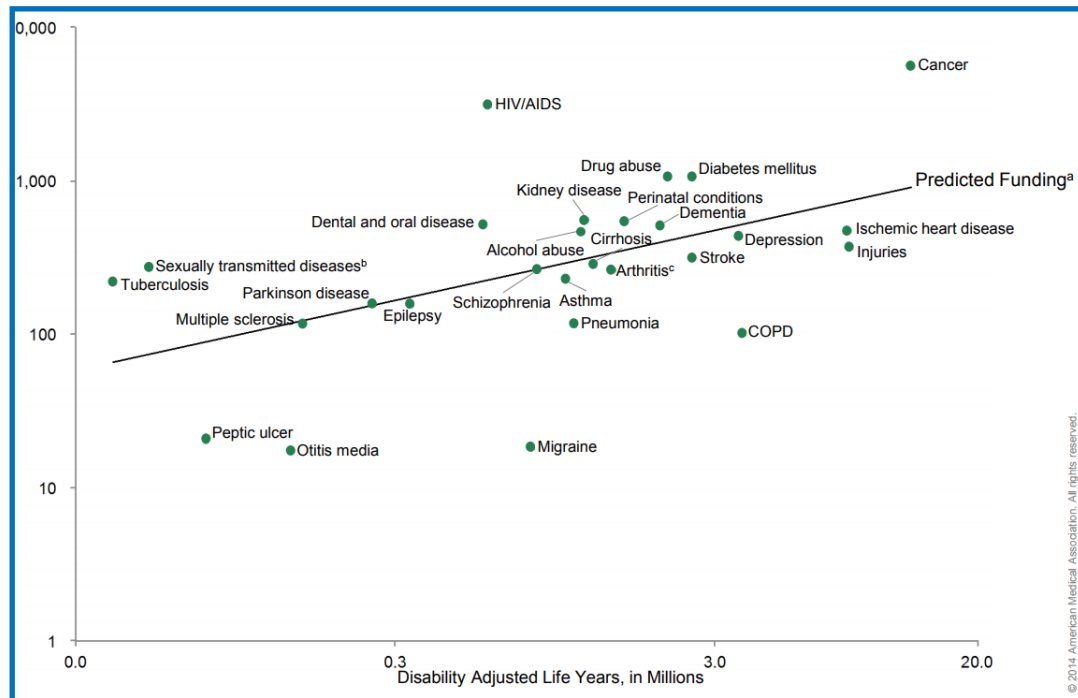


*Cole, Trolle & Edwards 2018*

# Research outputs... and inputs



## Burden of disease and NIH (US) funding for research



Moses et al. 2015

...we see a similar picture for the UK  
in Jones & Wilsdon 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



## 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?

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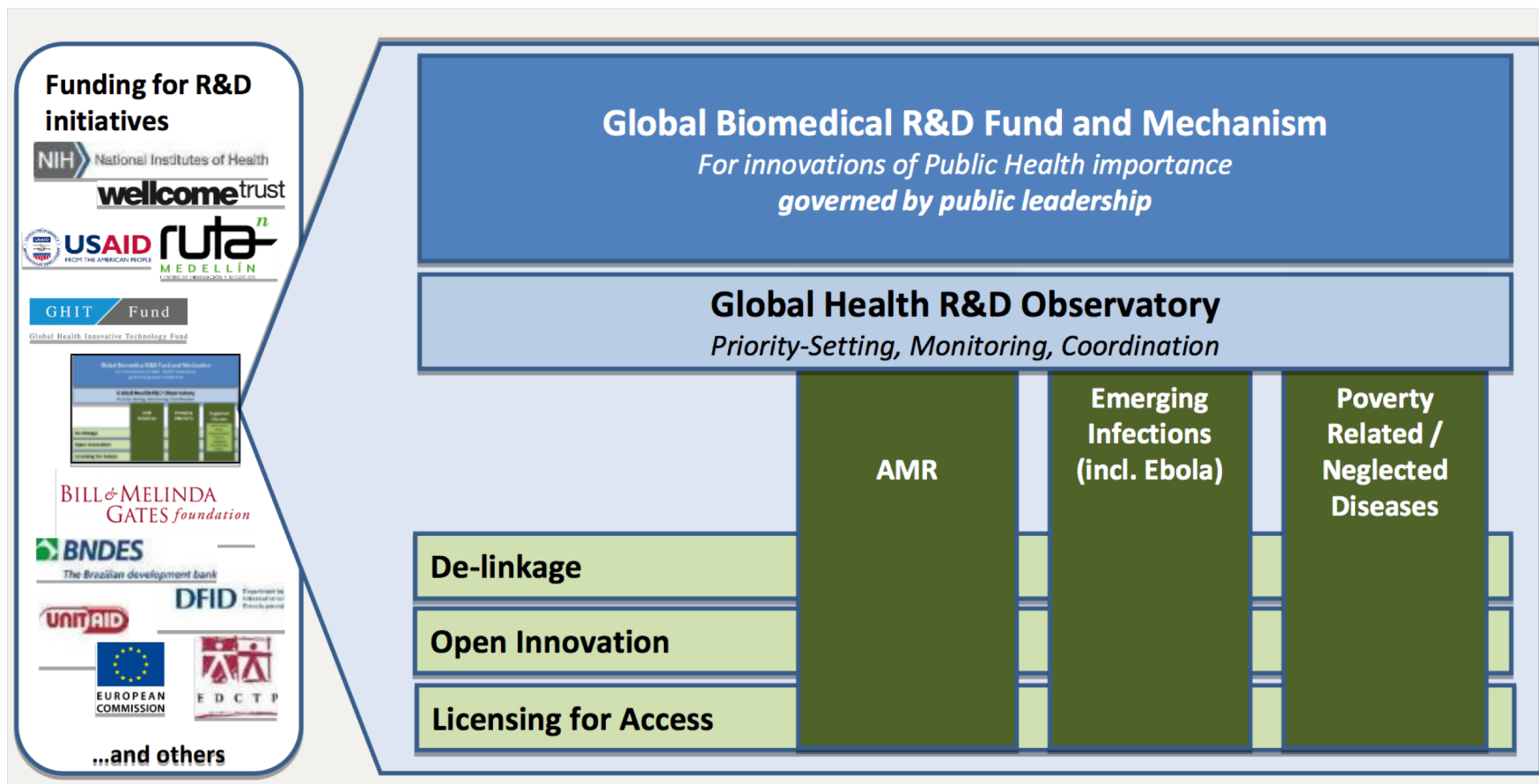
- ❖ **Push funding** tools, such as (conditional) grants, direct funding or tax incentives
  - ❖ **Pull financing** tools, such as (milestone) awards, research tournaments, pay for performance, advance market commitments etc.
  - ❖ **Pooling** of funds or intellectual property
  - ❖ **Collaborative**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





## 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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