How Good Are Our Drugs?

Assessing the Quality of Innovation

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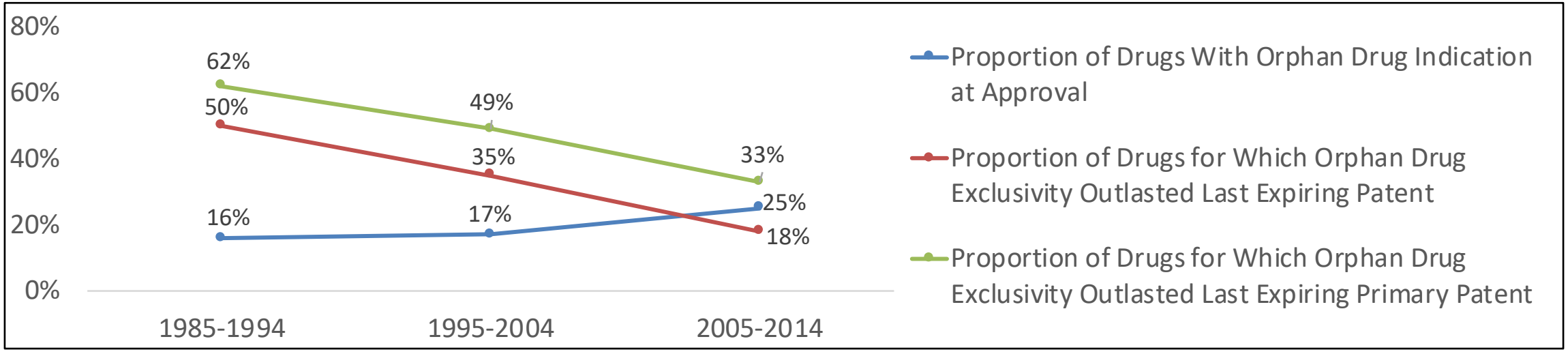
Diagnosis of the Problem from a Public Health Perspective

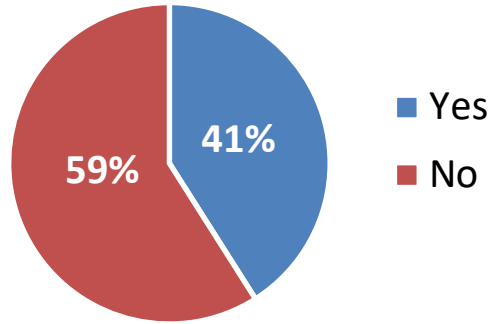
- Despite an increasing number of drug approvals, we are not getting drugs we need
  - Neglected therapeutic areas: infectious and central nervous system diseases
  - Overemphasis on "orphan" diseases but not driven by orphan drug exclusivity

FDA New Drug Approvals	
2006	22
2017	46
2018 (1 Oct.)	40



- Uncertain quality of approved drugs
  - Faster approvals: 61% of 2017 new drugs used an expedited pathway
  - Widespread approval on the basis of limited evidence
  - Problems with post-approval studies: design and enforcement
- Responding to poor quality drugs

Approval Based on Only One Surrogate Outcome Trial: 2005-2012 New Drugs



Possible Solutions

- More assertive and nuanced government steering
  - Mission-orientated public investment: targeted R&D support and phased prizes (over exclusivity)
  - Defining benefit: raising minimum level of expected benefit
  - Defining existing treatments: availability as opposed to approved indications
- Ensuring quality and fairness in return for earlier market access
  - Better alignment of EMA and HTA pre-approval requirements: comparators and outcomes
  - Timely completion of meaningful post-approval commitments
    - Uncertainty concession: e.g., cost plus pricing until commitments met or price reduction for delay
    - Require meaningful outcomes: e.g., overall survival as opposed to progression free survival
    - Require commitments underway at time of approval
  - Routine HTA re-assessment based in part on real-world evidence
- Orphan drug policy
    - Remove or reduce prevalence threshold
    - Reframe as minimum guarantee: claw-back mechanism if combined indication prevalence or revenue exceeds pre-defined thresholds