How Good Are Our Drugs?

Assessing the Quality of Innovation

Ameet Sarpatwari, J.D., Ph.D.

Program On Regulation, Therapeutics, And Law (PORTAL)
Division of Pharmacoepidemiology and Pharmacoeconomics,
Department of Medicine, Brigham and Women’s Hospital & Harvard Medical School
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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

- 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

<table>
<thead>
<tr>
<th>FDA New Drug Approvals</th>
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<tbody>
<tr>
<td>2006</td>
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<tr>
<td>2017</td>
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<td>2018 (1 Oct.)</td>
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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