New and Existing Incentives to Support Drug Repurposing

Dr. Bruce Bloom
Chief Collaboration Officer, Healx
Founder, Cures Within Reach

Innovating with Existing Drugs and Nutraceuticals Conference

Conflicts and Support

- Conflicts
  - None at this time
- Support
  - Half the people in this room!

Exponential Expansion of Interest in Repurposing Research

- Pharma (e.g. Horizon, Pfizer, etc.)
- Biotech/Academic (e.g. Healx, Audacity, Cyclica, Emory, Vanderbilt, Stanford)
- ‘Omics data, EMR data, *in silico* evaluation, HTP drug and patient sample screening and other tools to create homogenous disease groups, find lead candidates;
- Personalized/Precision Medicine use of repurposed drugs and nutraceuticals

Exponential Expansion of Interest in Repurposing Research

- Non-profits (e.g. CWR, ADDF, Noah's Hope)
  - Registries, data collection, advocacy, research management, research funding, transition to for-profit organizations
- Funding Groups (e.g. Patient Impact Capital, 505b2 Fund)

Incentives for Repurposing

- 505(b)(2) Pathway
  - New dosage form that is faster acting or some other patient value
  - Combine two active ingredients in a novel way
  - Provide a route of administration or mechanism of drug delivery that patients and/or doctors prefer
  - Carry out an Rx-to-OTC switch

Incentives for Repurposing

- 505 (b)(2) Pathway
  - Prodrugs of an existing drug
  - DESI drugs
  - Drug-device combinations
  - Possibly old drugs combined with new active ingredients

Incentives for Repurposing

- 505 (b)(2) Pathway
  - Must have active ingredients present in existing, approved drug products (reference drugs)
  - Create a bridge previously approved reference drug and the novel drug product or indication.
  - In Europe, a similar pathway is the hybrid procedure based on Article 10 of Directive 2001/83/EC.

Incentives for Repurposing

- 505 (b)(2) Pathway
  - Lower risk because of previous drug approval
  - Lower cost, faster development due to fewer studies
  - Possible three, five or seven years of market exclusivity

Incentives for Repurposing

- Current incentives that help, but aren't necessarily focused on repurposing research
  - Priority Review Vouchers-repurposing generics from outside the US, or nutraceuticals
  - Qualified Infectious Disease Product Designation-5-year exclusivity extension
  - EMEA/STAMP initiative for repurposing research label changes Purpose and Framework

Incentives for Repurposing

- Possible potential new incentives within FDA
  - New PRV program for generic repurposing
  - Changes to Qualified Repurposed Disease Product designation
  - Adaptive phase 3 programs; i.e. confirmatory efficacy and safety data could be collected from post-marketing vigilance studies, after the drug has been given a provisional marketing authorization-new type of authorization? (thalidomide vigilance example)

Incentives for Repurposing

- Possible potential new incentives outside FDA
    - Social impact bonds-pay for success
    - Donor Advised Funds-can invest in for profit
    - Value Added Medicines Group, part of Medicines for Europe-proposal to change reimbursement above generic cost level for new indication

Incentives for Repurposing

- Possible potential new incentives outside FDA
  - Differential pricing programs
    - Express Scripts example
    - Use of insurance Prior Review System
    - Self-insured employers
    - Might require legislative action

Incentives for Repurposing

- Delinkage-drug development not funded by drug sales (e.g., the Advanced Market Commitment by the Gates Foundation for pneumonia)
- Exclusivity for repurposed indications for generic drugs from other jurisdictions
- Packaging that has compliance or other clinical care benefit and is patentable
- Companion diagnostics and precision medicine evaluations/cocktails as a source of economic incentives for the repurposing of generic drugs

Impediments to Repurposing

- Lack of legislative acknowledgment or priority
- Difficulty accessing data about drugs and research information
- Cost of preclinical and clinical validation
- Especially for rare diseases, and for common diseases of slow progression and diseases with difficult outcome measures

Impediments to Repurposing

- Few economic or other incentives
- IP and other exclusivity challenges
- Generic substitution
- Off-label use vs. SOC vs. market approval
- Public outcry about combinations of generics
- Unknown or poorly known issues about repurposing and FDA regulatory process

The Future is Bright

- 20-25% of all prescriptions in the US written off label
- More than 1000 repurposed therapies with some support waiting to be driven to patients
- AI and other resources are developing more
- Economics and precision medicine will eventually support reliance on repurposed medicines

Contact

- Bruce Bloom | Chief Collaboration Officer

e: bruce.bloom@healx.io
m: +1 847-529-6888
w: healx.io
a: 3500 Church Street, Suite 112, Evanston, IL