Innovative Partnership and Financing Models for Rare Disease Drug Repositioning

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Cures Within Reach
Rare Disease Healthcare Outcome and Funding Crisis

• >7000 rare diseases worldwide with no universally effective therapy
• Globally impacts >50M people creating significant healthcare costs
• Pharma generates 20-30 expensive new medical solutions per year
The Repurposing Opportunity

• Clinically testing drugs, devices and nutriceuticals human approved for one disease indication, to create a “new” treatment in a different disease indication

• 66% of researchers and 25% of clinicians have a scientifically based Repurposing Research idea ready for a pilot clinical trial validation
Why Repurposing?

• Faster from idea to patient use
• Affordable and safe research
• Often the only economical solution for rare diseases
• High likelihood of success
• Off-label use or market approval options
• Improve outcomes and reduce healthcare costs for both patients and payers
Repurposing Roadblocks

- Poor/no economic incentives for repurposing
- Strong science but weak IP
- Inexpensive generic drugs widely available
- Hard/expensive to find patients for research
- Global issue, local solution
Solutions

• Innovative Partnerships
  - Public Private Partnerships
  - Hacking Medicine
  - Computational Medicine

• Social Finance Models
  - Rare Disease Social Impact Bond

• Bringing it all together
  - CureAccelerator™
Public Private Partnerships

• The Learning Collaborative
  - University of Kansas
  - Leukemia & Lymphoma Society
  - NCATS TRND
  - Government, Academia, Non-Profit
  - Repurposing Auranofin for Refractory CLL
  - In Clinical Trial
Public Private Partnerships

• Alzheimer’s Disease Initiative
  - Alzheimer's Drug Discovery Foundation
  - Alzheimer's Society (UK)
  - Cures Within Reach and other non-profits
  - Canadian government and hopefully UK government
  - Fund projects up to $1.5 million each focused on drug repurposing
  - Project #1 repurposing Nabilone for agitation in AD
Public Private Partnerships

• DevelopAKUre Nitisinone Trial
  - AKU Society initiation/organization
  - European Commission-£4.8m in funding
  - £3.2m in co-financing
    • European consortium including 13 hospitals
    • Pharmaceutical companies, universities, biotech companies
    • AKU patient groups
Public Private Partnerships

• Repurposing NSAID to reduce breast cancer recurrence
  - Nigerian Government
  - US Researchers Michael Retsky and Romano Demicheli
  - Cures Within Reach/CureAccelerator™
Innovation-Hacking Medicine

Hacking Medicine’s mission is to energize and connect the best minds the health ecosystem to teach, learn, and launch the next generation of healthcare solutions to solve healthcare’s biggest challenges.

http://hackingmedicine.mit.edu
Hacking Medicine successes include
- PillPack, which fills, sorts, and delivers prescriptions in personalized packets to help patients take their medications
- Careport, a startup run by Harvard Medical School students, that uses software to improve the hospital discharge process.
Hacking Medicine Successes

Citizen Hackers Tinker With Medical Devices
Diabetes Patients, Family Members Try to Make Glucose Monitors More Useful

Do-It-Yourself Medical Monitor

Diabetes patients and family members devised a system to monitor blood-sugar levels from afar, essentially hacking an FDA-approved device and uploading data to the Internet.

1. A transmitter on the body gathers glucose-level data every five minutes and...
2. ...transmits them to a receiver for display. This works only if the receiver is within 20 feet of the transmitter.
3. To monitor from afar, a smartphone with NightScout software is connected to the receiver. The software downloads the glucose data and uploads it to a server.
4. The data is gathered by a customized website and displayed. Almost any device with access to the Internet and a Web browser can view the data.

Sources: Dexcom; NightScout

The Wall Street Journal
Innovation-Computational Medicine

Hacking Medicine—many people coming together to create innovative solutions

Computational Medicine—many data points brought together to create opportunities for innovative solutions
Innovation-Computational Medicine

-The systematic electronic collection, classification, storage, and analysis of trillions of individual biochemical and biological data points to generate testable hypotheses for drug discovery and other medical solutions

- Omic data (genomics, exomics, proteomics)
- Drug data (PK, PD, BBB)
- Patient data (EMR, Phase IV trial, side effects)
- Literature (PubMed)
Innovation-Computational Medicine

-Over a trillion data points and growing every day

-Over 3000 articles in PubMed on computational medicine and drug discovery

-Over 120 companies using bioinformatics in drug discovery or drug repurposing
  http://bioinformaticsweb.net/biocompany.html
Innovation-Computational Medicine

-BioVista LLC

-COSS system finds both obvious and non-obvious drug repurposing using proprietary algorithms

-Chronic Fatigue Syndrome (2013)
-Friedreich's Ataxia (2010)
-Novartis (2011); Pfizer (2010); BiogenIdec; Cempra (2010)

-University of Pittsburgh
-Alpha-1 Antitrypsin Deficiency (2013)
Social Finance

Any financial investment method that intentionally delivers both a social dividend and an economic return.
Type of Social Financing

1. Social impact bonds
2. Social enterprise lending
3. Program-related investments
4. Outcome-based philanthropic grant making
5. Venture philanthropy
Why Social Finance?

1. Sometimes industry and/or non-profit organizations are not creating enough social return using the capitalism or philanthropy models, and

2. The government is not able or willing to raise taxes to fund new programs for social impact or has been unable to create the social return through existing programs
“With government budgets increasingly tight, this could be a major innovation,” says Sir Ronald Cohen, a financier who began chairing the global Social Investment Task Force of the G8 in 2010.
Social Impact Bond Benefits

- Attract new forms of capital
- Gov’t only pays for effective services
- Shift $ risks from gov’t to investors
- Opportunity for greater and more vigorous ongoing evaluation
- Independent evaluation creates transparency
- Feasible for small projects and patient populations
Social Finance Opportunities In Repurposing

- Potential Pay for Success Guarantors
  - Single Payer Government Systems (NHS)
  - US
    - Medicare, Medicaid, VA, Exchanges
    - Private Insurance Payers
    - Self-Insured Corporations, Pension plans
    - Hospitals
  - NPO Funders
Rare Disease SIB Diagram

CWR finds and manages rare disease repurposing research

Repurposing Clinical Trials

Cures Within Reach

Investors provide repurposing research funding

Investors receive return

Investors fund more repurposing research

Improved lives
Lower costs

Cost savings meets payer’s goals

Gov’t or other payer

Social Impact
Bond Pays

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ROI from Rare Disease Repurposing

Autoimmune Lymphoproliferative Syndrome
-CWR researcher repurposes the drug sirolimus
-Research cost <$250,000, takes < 3 years
-Treatment alleviates symptoms of fatal disease
-New treatment saves $100K/patient/year
-600 US patients could be using this off-label
-Up to $60M in annual healthcare cost savings

The Children’s Hospital of Philadelphia®
CuresWithinReach
Generic vs. Proprietary Example

- **Generic Drug Repurposing-Nutriceuticals for Familial Dysautonomia**
  - 6 years of research cost $1.2M
  - 5 daily oral nutriceutical treatments costing <$200/month
  - $60,000 average cost SAVINGS/patient/year

- **Proprietary Drug Creation-Enzyme Replacement for Gaucher’s Disease**
  - 10+ years of research, much of it funded by the US government, cost >$100 M
  - IV admin 2 hrs every 2 weeks costing $300,000/year
  - $250,000 average cost INCREASE/patient/year
## Generic Drug Rare Disease Rediscovery
### Research SIB Calculator

<table>
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<tr>
<th>Total Repurposing Research Costs</th>
<th>Average cost per clinical trial</th>
<th># of clinical trials</th>
<th>Potential success rate</th>
<th># of &quot;new&quot; treatments created</th>
<th>Average # of patients who have the disease</th>
<th>Projected annual healthcare £ saved per average patient</th>
<th>Total potential 5 year savings for all patients with these diseases</th>
<th>% patients actually using the &quot;new&quot; treatments</th>
<th>Total actual savings for patients using the &quot;new&quot; treatment</th>
<th>Healthcare £ saved on this sub-population of patients AFTER repaying investors</th>
<th>5 year ROI on £50M investment</th>
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*Image: CuresWithinReach*
Use of ICD Codes in Repurposing Research SIB

• ICD (International Classification of Disease) codes
  • Tracks all patients by same disease codes
  • Tracks treatments by specific codes
• Used around the globe
• Computerized existing system
• Can be used to determine target diseases/patients
• Patients can serve as their own control
Changes Required for Repurposing Research SIB

• **Regulatory**
  - Support for quicker 505b2 approval

• **Legal**
  - Ability to legally promote off label use
  - Standardized agreement templates

• **Reimbursement**
  - Pay for success payers might agree to reimburse even if repurposed therapy is not on formulary
  - Costs might be low enough that reimbursement is a non-issue

• **Dissemination**
  - May need new journal or other ways of publishing findings quickly
  - Partnerships with physician associations and patient advocacy groups
Current Development of Repurposing Research SIB

• Working with British government
• Early discussions with Swedish and Canadian government
• MaRS Centre for Impact Investing (canada) undertaking a feasibility study funded by the Mindset Foundation
• Contacting US Healthcare Payers
Connectivity-put it all together

Drug repurposing
Public-Private Partnerships
Hacking Medicine
Computational Medicine
Social Investing
CureAccelerator-put it all together

Hacking Medicine-
  -Virtual Hacking through Discussion Forums
  -Plan to have live regional CureAccelerator Hacking Medicine Conferences co-sponsored by current Hacking Medicine experts
CureAccelerator-put it all together

Computational Medicine-
  -Participation by many bioinformatics companies (BioVista, NuMedii, Epsilon 3, Stemcycle, twoXAR)
    -NIH NCATS providing a pilot Computational Medicine project
CureAccelerator-put it all together

Drug Repurposing/Social Medicine-
- Singular focus on Drug Repurposing Clinical Trials
- Talking with UK Gov’t and others about using CureAccelerator as a base for rare diseases SIB
CureAccelerator Organizational Hub

Where it starts

What’s being tried

What’s possible
CureAccelerator-put it all together

Beta launch in progress
Public Launch 1 June, 2015
http://cureaccelerator.org
Questions & Answers

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