Going Beyond the Current Regulatory and Incentives Frameworks

A new proposal to foster greater orphan drug development

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No Disease Is Too Rare to Deserve Treatment
EveryLife Foundation

• Dedicated to accelerating biotechnology innovation for rare disease treatments
• Advocating practical and scientifically sound change in policy and law to increase the efficiency & predictability of the development process through scientific analysis and dialogue, grassroots support & expert-led workshops
• We believe:
  – No disease is too rare to deserve treatment
  – All treatments should be safe & effective
  – We could be doing more with the science we have
The development process

Good Science

And then a miracle happens
Thousands of Rare Diseases Need Treatment
How can this be done with the current process?
Is there really just the valley of death?

IDEA  Model POC  Tox., IND/CTA Ph. 1 Study  Ph2/PH3 NDA  Reimbursement

Lost in Space  Wandering in Wilderness  Valley of Death  Clin-Reg Hell  Reimbursement Purgatory

IDEA  Yr 5  Yr 10  Yr 13  Yr 15
FDASIA – Great start but not enough

• Sec. 901. Enhancement of accelerated patient access to new medical treatments (ULTRA/FAST)
  – Considerations. – In developing the guidance . . . . the Secretary shall consider . . . . for drugs designated for a rare disease or condition under section 526 of the Federal, Food, Drug, and Cosmetic Act; and
  – (2)how to incorporate novel approaches to the review of surrogate endpoints based on pathophysiologic and pharmacologic evidence in such guidance, especially in instances where the low prevalence of a disease renders the existence or collection of other types of data unlikely or impractical.”

• Sec. 902. Breakthrough therapies
• Sec. 903. Consultation with external experts on rare diseases
• Sec. 908. Rare pediatric disease priority review voucher incentive program
• Sec. 1137. Patient Participation in Medical Product Discussions

**95% of rare diseases have no approved treatments**

At the current rate of 10 new drugs approved each year it will take more than 500 years to treat all rare disease patients!
Successes in FDASIA showed there is momentum for more rare diseases legislation

- Patients are motivated & ready to take action
- Rep. Upton is actively seeking proposals to improve FDA, spur drug development & innovation
Small policy changes that will dramatically increase the availability of rare disease treatments in the next 5-10 years

- **Specialize:** Create more specialized FDA New Drug Review Divisions; give reviewers sufficient time and opportunity to stay connected to the scientific and academic community
- **Rationalize:** Allow for a more scientific rationalized application of the ICH guidelines for safety studies
- **Incentivize:** Create an additional market incentive to encourage industry drug sponsors to repurpose major market drugs for rare diseases
Current Market Incentives that Foster Drug Development

- **Orphan Drug (ODE)** - 7 years
  - Extremely successful for incentivizing drug development for orphan products
  - Does not include major market drugs that would be repurposed for rare diseases

- **New Chemical (NCE)** - 5 years

- **Pediatric Exclusivity (PED)** - 6 months added to existing Patents/Exclusivity
  - Requires a study only - not a new label indication

- **Antibiotic Exclusivity** - 5 years
  - GAIN Act passed in FDASIA in 2012
We Can Do More with the Science We Already Have

The Potential of Drug Repurposing for Rare Diseases

• Many patented drugs already developed and approved for common conditions which might effectively treat rare diseases
  • Quality drugs with high potency and selectivity
• A single targeted drug is likely to have multiple therapeutic uses
• But rare disease indications will not be developed for patented drugs: Why not?
Roadblocks for Repurposing Large Market Drugs for Rare Diseases

• The perception of RISK to a billion dollar product is too great to allow any rare disease development
  — RISKS: Fear that potential adverse effects in clinical trials on very sick patients would risk the product’s market
  — NO BENEFIT: Adding a few hundred or few thousand rare diseases patients does not increase market revenue enough to justify the costs of repurposing or the potential risk
Need for an incentive for repurposing patented drugs

- Can be developed for less usual 10 to 15 yrs
- Sponsor already exists
- Much lower investment than traditional drug discovery process
- Drugs have already been proven to be safe
- Pricing for product based on a larger markets, so drug prices should be lower relative to usual orphan pricing
Learning From Policy That Has Worked: Best Pharmaceuticals for Children Act

- BPCA provides 6 months of market exclusivity on top of patent life if studies for pediatric use are conducted
- Prior to BPCA, drugs were infrequently tested in children
- Off-label use in the pediatric population was over 70% which has since dropped to about 50%
- Since 1998, **over 400 labeling changes** have occurred, indicating whether the drug is safe for children
Building on BPCA

An additional incentive can help fulfill the goals to ensure children have access to safe & effective medicines

- BPCA is still not enough incentive for sponsors to develop new pediatric cancer treatments
  - In the past 20 years, only one drug has been expressly developed for any form of pediatric cancer
- BPCA only requires a study & does not require a new labeled indication be achieved
- A new Orphan Product Market Exclusivity Extension is needed to incentivize repurposing drugs for rare and pediatric diseases & cancers
How a New Orphan Product Repurposing Exclusivity Could Work

• Sponsor receives FDA approval for their major market drug or has a current approved drug still under patent protection

• Sponsor could seek rare disease indications to extend its patent life & protect revenue from market competition for 6 months
  – Must be a Rare Disease – under 200,000 patients in the US
  – Must qualify for Fast Track Designation :life-threatening disease
  – Must obtain data to place the new rare disease indication on the label

• Sponsor might begins rare disease indication development: multiple trials for multiple rare diseases

• Company receives new rare disease indication on the label
  • Safety, efficacy and dosing demonstrated

• Obtains 6 months additional market exclusivity at the end of the product’s patent life or NCE exclusivity period
  – Would stack on top of BPCA to provide an additional incentive to develop pediatric cancer & rare pediatric disease treatments
Patents & Market Exclusivity

Original Patent Term - 20 years

- Data Exclusivity, NCE
- Data Exclusivity, Non-NCE
- Orphan Drug Market Exclusivity
- Pediatric Drug Exclusivity, NCE
- Pediatric Drug Exclusivity, Non-NCE
- Orphan Drug Exclusivity Extension

From www.drugregulations.org
Key Benefits of **Rare-purposing*** that would speed development

- Sponsor already exists for the program
- Leverages existing expertise of clinical development staff and scientists
- Manufacturing and toxicology work complete
- Safety is known in humans
- **Reduced time for development** trials & approval
  - Focus on science, and rare disease clinical studies
- **Rare-purposed Orphan Drugs will likely cost less than typical orphan products:** Drug price set by large market indication

* Nickname courtesy of Kay Holcombe, BIO
Finding the right balance

• An estimated 120 drugs go off patent each year
  – Once a drug is off patent there is no sponsor support
  – No financial incentive to study a drug for a rare disease
  – Complete loss of opportunity

• An economic incentive will allow companies to
  – Recoup the clinical trial & FDA regulatory costs of
    multiple repurposing trials
  – Provide sufficient financial benefit that a company might
    be willing to risk their current product market

• Still allows for timely generic competition

• Maximizes the number of drugs in development NOW
  for rare disease patients
Impact of Legislation
Surge in Patented Drug Repurposing
Investment in the next 15 years

Small change in regulation: **Large** effect in innovation

- Immediate surge in research investment
- New high paying biotech Jobs
- Increased tax revenue
- Rare Disease patients access to clinical trials

• 100’s of drugs available for rare disease patients
Benefits to the Economy

• Macro/Economic
• Surge in Biotech Investment from development
• New high paying Biotech Jobs
• Lower cost orphan drugs
  – Potential of hundreds of new rare disease treatments on the market priced at major market drug prices
  – Lower healthcare costs for government, private insurance and out of pocket costs for patients
  – Helps solve the problem more quickly with so many rare diseases and so few treatments
We must ACT now to Accelerate Cures & Treatments

• A new Repurposing Exclusivity Incentive could more than double the current number of rare disease products approved each year in the next 5-10 Years

• More Patients would have access to safe, effective and affordable treatments

• Off Label Usage Would Decline
  – Correct drug choice and dosage would ensure patients would have access to safer & more effective treatments
  – Reimbursable treatment options for patients
We Want Your Feedback!

Join the EveryLife Foundation’s Roundtables Today at 10:40 & 11:30

Roundtable 7 – How can extended patent and market exclusivity provide an alternative to the high price of orphan drugs?
THE OPEN ACT

ORPHAN PRODUCT EXTENSIONS NOW ACCELERATING CURES & TREATMENTS

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