Oxymoron no more: nonprofits can deliver medicines for the poor of the world
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The Need...

Overlapping Burden of Neglected Diseases and Malaria

The 6 neglected diseases are leprosy, lymphatic filariasis, onchocerciasis, schistosomiasis, soil-transmitted helminths and trachoma.
Disease Impact

Mortality Associated With Infectious Diseases, 2001

% of Deaths Due to Diseases Accounted By Each Region

- Lower Respiratory Infections
- HIV/AIDS
- Diarrhoeal Diseases
- Tuberculosis
- Malaria
- Measles

Colors represent:
- Africa
- Southeast Asia
- Western Pacific
- Eastern Mediterranean
- Americas
- Europe
Disease Impact

Infectious Disease Deaths as a Proportion of All Deaths, 2001

- Africa: 62%
- Southeast Asia: 31%
- Western Pacific: 11%
- Eastern Mediterranean: 34%
- Americas: 10%
- Europe: 5%
The Status Quo

10/90 GAP – only 10% of global health R&D is devoted to conditions that account for 90% of the global disease burden.

98% of deaths of children occur in developing world.
Global Health Inequities

POVERTY

Differential disease distribution
No new R&D in diseases of the poor
No access to essential medicines
Malnutrition & poor immune function
End of colonialism
## Neglected Diseases: Incidence Rates

<table>
<thead>
<tr>
<th>Disease</th>
<th>Incidence</th>
</tr>
</thead>
<tbody>
<tr>
<td>Intestinal Worms</td>
<td>3 billion</td>
</tr>
<tr>
<td>Diarrheal diseases</td>
<td>&gt;1 billion</td>
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<tr>
<td>Malaria</td>
<td>273 million</td>
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<tr>
<td>Schistosomiasis</td>
<td>200 million</td>
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<tr>
<td>Lymphatic filariasis</td>
<td>90 million</td>
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<tr>
<td>Chagas disease</td>
<td>14 million</td>
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<tr>
<td>River blindness</td>
<td>16 million</td>
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<tr>
<td>Leishmaniasis</td>
<td>14 million</td>
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</tbody>
</table>
Example: Malaria

- Malaria deaths in Africa account for the vast majority of malaria deaths since 1980.
- Mortality due to malaria is compounded by:
  - poor public health infrastructure
  - lack of patient education
  - lack of access to effective medicine
  - social, political, and economic turmoil that drives poverty and limits access to basic care.
Example: Malaria

Worldwide malaria distribution in 2002

[Map showing malaria distribution worldwide, with regions color-coded for risk levels.]
Of the 1393 new drugs approved between 1975 and 1990, just 13 (1%) were for tropical diseases.

Doctors Without Borders, 2001
Global Health PPP Model

- Perceived response to “market failure”
- Usually funded as drug donation programs
- Several product development PPPs for drugs, vaccines, diagnostics
- Logical pathway for academic discoveries
- Engage discovery/research of a few corps
- Model works reasonably well for three big diseases: AIDS, malaria, TB
PPP Challenges

- Unable to attract companies to neglected diseases
- Only viable with secure IP (cannot use old drugs)
- Narrow mission – cannot bring in new technologies and platforms
- Very few development-stage projects
- Dependent on philanthropy
Pharmaceutical Technologies

- Many more discoveries than can be developed
- Technologies primarily intended for developed markets
- No established path to complete new technology R&D for people in the developing world
- Huge untapped opportunities!
Why A New Player Is Needed

• Many infectious diseases lack R&D programs to develop new cures
• Gap between pharma industry and major global health players:
  – high-tech corporate vs. low-tech field sectors
  – different language and terminology
  – different corporate culture
  – two sectors rarely interact except through drug donation programs
The Market...

1.1 billion people live on less than $1 a day

2.7 billion people live on less than $2 a day
The Result…

Committed Scientists

New Drug Leads from Academia and Industry

OneWorld Health

Philanthropic Investment

Partners
A Simple Experiment

- Model the successful pharmaceutical industry
- Build pharmaceutical company that removes profit element from business plan
- First project must be successful: late stage drug with quick path to approval and leverage work of others
- Focus core competencies: R&D of new drugs & regulatory approval (not distribution)
- Strategic selection of drug projects that have distribution mechanisms in place, or soon will
- First project in India, expand programs in Africa
OneWorld Health develops safe, effective, and affordable new medicines for diseases of poverty in the developing world.
An institute of pharmaceutical scientists that:

- IDENTIFIES promising drug candidates in late stage R&D
- COMPLETES animal and human studies
- SECURES quality manufacturing in disease endemic countries
- OBTAINS regulatory approval in disease endemic countries
iOWH – Guiding Principles

• Do not compete with pharma/biotech/PPPs
• Do not duplicate available resources
• Focus on D, versus R
• Be the bridge - industry & public sector
• Do not allow paths to sustainability to influence decision-making
• Create a scenario where everyone wins
iOWH – Niche

- Focus on diseases in new and creative ways
- Parasitic diseases: no vaccines but drugs can cure infections
- Study old, off-patent, safe drugs as new cures
- Adopt high risk-high reward projects
- Provide industry with flexible and innovative partnership opportunities
- Demonstrate that a nonprofit can be (partially) sustainable through sales of products
Core Expertise: Product Development

**Discovery**
- Basic research
- Screening

**Development**
- Formulation Development
- Preclinical Studies
- Clinical Trials
- Technology Transfer
- Regulatory Approvals

**Manufacturing and Distribution**
- Production
- Distribution
- Education
# iOWH - Pipeline

<table>
<thead>
<tr>
<th>Disease</th>
<th>Portfolio Screening</th>
<th>Pre-Clinical Studies</th>
<th>Phase 1</th>
<th>Phase 2</th>
<th>Phase 3</th>
<th>Regulatory Approval</th>
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</thead>
<tbody>
<tr>
<td><strong>Leishmaniasis</strong></td>
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<td>2006</td>
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<td>Portfolio product 1</td>
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</table>

- **Funded project**
- **Funding sought**
- **Not Applicable**
Case Study: Visceral Leishmaniasis (VL)

Facts:
1.5 million currently infected
200,000 deaths annually
500,000 new cases per year
Paromomycin: Our First Drug

- West – discontinued antibiotic
- South – new cure for fatal disease
- Initial approvals expected in India; subsequently in US, Sudan, Ethiopia, Nepal, Bangladesh
- Will be first-line therapy in disease elimination program (S. Asia)
- Cost per cure $10-20 (one-tenth of current therapies)
2006 Pilot Distribution Program

- **Phase 4 demonstration study**: to construct an effective and sustainable delivery strategy (partnership & coordination)

- **Clinical trial in children** under five years of age.

- **Indian and U.S. FDA** approval

- **Monitoring and Evaluation** of delivery system/structure and of drug
Case Study: Visceral Leishmaniasis (VL)
How To Change the World

• Examine the world and select a significant problem that touches your heart
• Identify opportunities
• Focus on the solutions
• Study the work of others
• Acknowledge obstacles & work around them
• Persevere with integrity, passion and vision
• Finish the job

This is Social Entrepreneurship
Your First Project

• Later-stage technology
• Disease of a manageable size
• Geographical region with some infrastructure
• Supportive national and state governments
• Innovative partnerships and creative solutions
• Possible distribution partners exist TODAY
• Measurable outcome/impact
• Keep your head down and finish the project – little media focus early
Innovative Partnerships: Biotech Partnership for Malaria

Partnership with UCB (QB3) and Amyris

• High risk science (synthetic biology).
• Success would have huge economic impact.
• Build metabolic system in *E. coli* to ferment artemisinin antimalarials.

• Royalty-free license from UCB to IOWH.
Innovative Partnerships: Artemisinin Collaboration

UC Berkeley Keasling lab. → Amyris (small biotech) → iWH

- Synthetic Biology
- Bioengineering
- Link to lab
- Scale-up
- Generic substitution
- Neurotoxicology
- Manufacturer

- Alternative source of world’s best antimalarials
- Obviate shortages
- Reduce cost
- 5-year program – to be completed in 2010
- High risk program with enormous benefit to the world
Creative New Solutions

• Many global infectious diseases can be made insignificant
• Most of these problems will not be solved with existing systems, corps and governments
• We must create new solutions and new paths
Conclusions

• A nonprofit pharmaceutical company can exist, produce and thrive

• Industry and academic scientists are anxious to develop and advance new technologies for neglected diseases

• Young scientists play a major role in forcing this new direction

• Leadership and creativity are needed to create unique opportunities for industry participation

• Adequate funding exists if we do our jobs well
Only Achievable With Partners

- Bill and Melinda Gates Foundation
- Keasling lab (UCB)
- Amyris Technologies
- QB3
- WHO/TDR (Geneva)
- Int’l Dispensary Association (Amsterdam)
- Gland Pharmaceuticals (Hyderabad)
- Janani (Patna)
- Walter Reed
- Large pharmaceutical companies
Never doubt that a small group of thoughtful, committed citizens can change the world. Indeed, it is the only thing that ever has.

*Margaret Mead*