Limitations of traditional funding in Bio-pharma

Karl-Heinz Krause
Geneva University Hospitals and Faculty of Medicine
research grants

seed funding

- venture capital
- funds from big industry

~0.5 million  ~100 million
Venture Capital and start-up companies

• until recently, most important mean for start-ups to raise substantial amount of money
• reality check: does the start-up concept stand a chance in the real world?
• investment by VC not only brings money, but (often) also competence to the board of directors
• but: .......
VCs as the only substantial source of money for start-ups is a problem

- What the world needs, and what VCs like to fund is not the same thing
Why Funding for Neglected Tropical Diseases Should Be a Global Priority

Sharon L Reed, James H McKerrow


Published: 24 April 2018 Article history ▼

Abstract

Neglected tropical diseases affect >1 billion of the world’s poorest persons. Control programs range from near-elimination (dracunculiasis) to increasing prevalence (dengue and cutaneous leishmaniasis). These are some of the most cost-effective public health interventions and should be a global priority.
1/3 of new drugs: anti-cancer treatment

doi: [10.4103/2229-516X.112228]

Bedaquiline: First FDA-approved tuberculosis drug in 40 years

Rajiv Mahajan
<table>
<thead>
<tr>
<th></th>
<th>tuberculosis</th>
<th>cancer</th>
</tr>
</thead>
<tbody>
<tr>
<td>estimated annual number of death (world wide)</td>
<td>1-2 million</td>
<td>9-10 million</td>
</tr>
<tr>
<td>Pharma industry spending in research and development</td>
<td>&lt;100 million</td>
<td>&gt;50 billion</td>
</tr>
<tr>
<td>new drugs</td>
<td>1 (in 40 years)</td>
<td>72 (2002 to 2014)</td>
</tr>
<tr>
<td>impact of treatment</td>
<td>cure</td>
<td>~3 months increased survival (data from 2014)</td>
</tr>
</tbody>
</table>
Is cure of disease a desirable outcome?
ATMP= Advanced Therapy Medicinal Products

• gene therapy medicines
• somatic-cell therapy medicines
• tissue-engineered medicines

ATMP: parmi les progrès médicaux les plus importants des dernières années
Goldman Sachs Analysts Question Whether Curing Patients Is Good for Business

"The potential to deliver 'one shot cures' is one of the most attractive aspects of gene therapy, genetically-engineered cell therapy and gene editing. However, such treatments offer a very different outlook with regard to recurring revenue versus chronic therapies.... While this proposition carries tremendous value for patients and society, it could represent a challenge for genome medicine developers looking for sustained cash flow."

---

When curing a disease with gene therapy is bad business

Just today, we saw GlaxoSmithKline sell off its pipeline of gene therapies for rare disease to a London startup called Orchard Therapeutics for a 20 percent stake in the young company.

The treatments Glaxo didn’t want were bona fide miracles: one-and-done cures that replace a broken gene and save a life.
Discrepancy between the medical needs and therapy development by biopharma is not limited to diseases of low income countries.
CNS diseases as unmet medical need

• Central nervous system (CNS) diseases presently represent the largest and fastest growing area of unmet medical need.
• It is estimated that 1.5 billion people worldwide, including over 100 million people in the United States, suffer from CNS diseases or disorders.
• According to the National Institutes of Health, the medical costs of treating brain disorders exceed $600 billion a year in the United States alone.
  – more than any other group of illnesses.
• The economic impact due to loss of productivity is estimated to exceed $1 trillion each year.
CNS disease in need of new drug development

- Neurodegenerative diseases
  - Alzheimer, Parkinson, ALS, Huntingon ....

- Neurovascular diseases
  - stroke (ischemic, hemorrhagic)

- Psychiatric diseases
  - schizophrenia, depression, autism ...

- Pain disorders
  - migraine, chronic headaches, .....
Ask a venture capitalist to invest into development of CNS therapeutics
Brain diseases are the graveyard of drug development

Pharma has downsized its drug development efforts

Why?
• Difficult to access the human brain
  → indirect access through symptoms, but different diseases cause similar symptoms
• Limitations of animal models
• Limitations of classical *in vitro* models

Clinical approval success rate

<table>
<thead>
<tr>
<th>Category</th>
<th>Success Rate</th>
</tr>
</thead>
<tbody>
<tr>
<td>Infectious</td>
<td>23.9%</td>
</tr>
<tr>
<td>Musculoskeletal</td>
<td>20.4%</td>
</tr>
<tr>
<td>Oncology / Immunology</td>
<td>19.4%</td>
</tr>
<tr>
<td>Metabolism</td>
<td>9.4%</td>
</tr>
<tr>
<td>Cardiovasc</td>
<td>8.7%</td>
</tr>
<tr>
<td><strong>Brain</strong></td>
<td><strong>8.2%</strong></td>
</tr>
</tbody>
</table>


Miller, 2010
What novel funding mechanism for biotech should allow

• attack the big problems
  • tropical diseases and disease linked to poverty
  • brain diseases

• favor cure over chronic treatment

• allow patients and regular citizens to participate
Where blockchain technology could help

• democratization of financing of novel therapy development
• allow patients and families to have a stronger influence
• search financing world-wide
• others?