They need new drugs, vaccines and diagnosis now: reality of neglected diseases

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Defining neglected diseases

Global Diseases

Most Neglected Diseases

Neglected Diseases

World pharmaceutical market
> $518 bn in 2004
Sleeping sickness is a most neglected disease

- An estimated 300,000 infected
- 55 million at risk in sub-Saharan Africa
- Difficult to diagnose
- **Fatal** if untreated
- Existing drugs: old - toxic - resistance - difficult to use - expensive
The needs remain huge

Arsenical

Anti-cancer drug
Buruli ulcer

Plate 22. Ulcerative lesion (Photo: Marco Pirovano)

Plate 23. Ulcerative lesion (Photo: Marco Pirovano)

AIDS is a neglected disease for adults and children

- Drugs not adapted to health systems of endemic countries
- No treatment for children
- Limited tools for diagnosis and follow up
- No field-adapted preventive tools
Treatments do not exist or are inadequate and inaccessible

- Toxic
- Expensive
- Painful to deliver
- Difficult to follow up
- Not adapted to patient’s needs
- Not registered in endemic regions
- Restricted by patents
Analysing the problems:
Fatal imbalance
Developing countries have a tiny share of the pharma market

World Pharmaceutical Market, 2004: Total $518 billion

Source: IMS Health
Only 1% of new drugs developed are for neglected diseases

- Approx. 1-2% is spent on R&D for neglected diseases
- 10/90 gap in health research spending
- 1975-1999: 1,393 new chemical entities marketed
- 68.7% registered products presented little or no therapeutic gain

Tropical diseases: 13
Tuberculosis: 3
Spending on health R&D has increased

- World-wide spending on **health R&D** was never so high
- Since 90’s: private sector has become biggest investor

**US-spending on health R&D:**
(>2/3rd total)

Sources:
For government: National Science Foundation 2004,
For Industry: PhRMA 2004, 
Gaps exist in the R&D process for neglected diseases...

New knowledge on drug targets and lead compounds is published but pre-clinical research does not begin mainly industry (in North)

mainly public sector

**GAP1**

**GAP2**

Validated candidate drugs do not enter clinical development because of strategic company choices.

**GAP3**

New or existing drugs do not reach patients: registration problems, lack of production, high prices, or not adapted to the local conditions of use.
...due to failure of the market and public policy

**Market failure**
- Drug development largely confined to the R&D-based pharmaceutical industry operating for profit
- Poorer patients are thus neglected

**Public policy failure**
- Public policy does not redress this imbalance
Governments should tackle this imbalance

• The response should not be purely philanthropic

• Governments should increase public responsibility towards R&D of drugs for neglected diseases
  – More political leadership
  – Sustained financial support
  – New rules to stimulate drug R&D
Increased public responsibility:
1. More public leadership

• Make global health and medicines a strategic priority

• Set R&D agenda according to the needs of patients
Increased public responsibility:
2. Sustained financial support

Governments need to

• Raise current levels of funding for neglected diseases by 3 billion euros per year to correct the 10/90 gap
• Put in place new, sustainable funding mechanisms
2. Sustained financial support

This funding should be focused on:

- A needs-driven R&D agenda for safe, effective, affordable and field-adapted treatments
- Encouraging scientific community to do basic research on neglected diseases
- Translation of basic research to new medical applications, e.g. by supporting PDPs
- Encouraging R&D capacity strengthening in disease-endemic countries
- Securing the market
Increased public responsibility: 3. New rules to stimulate drug R&D

- Regulatory standards
  - Streamline regulatory approval processes to rapidly deliver essential medicines to patients
  - Analyse risks and benefits of each drug or vaccine in relation to the needs of patients, severity of the disease and lack of alternative solutions
  - Regulatory authorities (FDA and EMEA) should provide support and transfer know-how to authorities in developing countries
3: New rules to stimulate drug R&D

Intellectual Property: develop drugs as public goods

• Guarantee that the public sector develops open access to information (recent Wellcome Trust policy) => open source (Human Genome Project) => freedom to operate

• Ensure that industry provides sustainable access to knowledge, chemical compounds and tools

• Make technology transfer happen to disease-endemic countries
The world needs policy action, not promises
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