New Medicines for Neglected Tropical Diseases: The Lessons from Biodefence

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Summary

The U.S. biodefence program may seem an unlikely source to turn to for lessons on how to improve access to medicines and vaccines in poor countries. Yet both the biodefence and the international development communities actually face a similar problem: the diseases they seek new treatments for have been neglected by commercial pharmaceutical companies because financial returns on investments are too small. In the case of biodefense, they are small because a biosecurity threat (like a biological weapons attack) is highly unpredictable, and there are only a small number of government buyers. For neglected tropical diseases (NTDs), they are small because people in low-income countries cannot afford to pay much for medicines. In order to attract more investment from the pharmaceutical industry into these areas, both the biodefence and the international development communities have thus begun to experiment with various incentive mechanisms. Chief among them are public-private partnerships (PPPs). Such partnerships can help to pool financial resources, especially from the public and the philanthropic sectors, coordinate research and development, and create new markets. The U.S. biodefence program has initiated a successful reform program that may offer valuable insights also for PPPs working on NTDs in terms of how to share development risks, how to work with small companies, and how to minimize the administrative burden of contracts. Crucially, these reforms are not based on increased funding but rather on the willingness to take on more risk, thereby increasing the flexibility of existing funds.

The Challenge

New medicines and vaccines are urgently required to fight diseases that mostly affect poor people in low-income countries. More than one billion people suffer from these so-called 'neglected tropical diseases' (NTDs), which also lead to billions of dollars in economic losses every year. While the social demand for new medicines and vaccines is enormous, this does not translate into an effective market demand because people in low-income countries often cannot afford to pay for medical treatment and vaccination. Yet, market demand and prospective returns on investment are the predominant drivers for pharmaceutical development because it is conducted mainly by commercial companies. Since the issue has appeared on the political agenda, much thinking has gone into how to incentivize pharmaceutical companies to invest. Public-private partnerships have emerged as a key mechanism to pool financial resources, coordinate research and development (R&D), and create procurement mechanisms to reduce market risk. While these efforts have yielded notable successes both in terms of the number of new products launched and doses distributed, progress is slow and industry investment in NTDs continues to be low. The challenge therefore remains: how can we strengthen industry involvement in R&D for pharmaceuticals with limited market demand?

The Research

The problem is not confined to NTDs. An unfulfilled social demand for new medicines and vaccines also exists in several other areas. Infectious diseases that pose potential pandemic threats, pathogens that may be used as biological weapons, and infections that are resistant to existing drugs are some of the other health issues where new medicines and vaccines are urgently needed but where industry investment is similarly small. The gap between social and market demand for new medicines and vaccines has thus become a systemic problem in global health. Yet, efforts to address this problem have focused largely on specific health issues. For instance, people working on NTDs have not engaged much with communities involved in strengthening pharma R&D for new antibiotics or biodefence. This research project set out to take a bird's eye perspective on the problem of pharmaceutical development in the absence of commercial drivers across the entire field of global health. The goal has been to identify not only common challenges and but also successful approaches that have emerged in specific areas and could be applied in others. Here, we suggest that industry investment in NTDs may be strengthened by applying lessons from the U.S. biodefense program, and specifically from Project BioShield whose aim it is to develop 'Medical Countermeasures' (MCMs) for bioterrorist and pandemic threats.

Key Messages

- There is an urgent need to develop new medicines for neglected tropical diseases
- Public-private partnerships (PPPs) are important vehicles for developing such medicines
- Lessons can be learned from biodefense in terms of engaging pharmaceutical companies in PPPs
- Sharing development risk – and not just market risk – is a critical factor
- Non-financial incentives and flexible contracting arrangements are also key components

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The Findings

Project BioShield was designed as a public-private partnership and initially sought to attract pharmaceutical companies mainly by providing access to research funding and creating a market for MCMs in form of a national stockpile. Yet, industry engagement was lower than expected. Subsequent reforms have addressed a number of issues that may be relevant also for NTD drug development.

Overlooked development risks - The initial incentive structure of Project BioShield addressed mainly risks that companies face in the early and late stages of the R&D process – during discovery research and marketing. It had largely overlooked the risks that companies face during the process of product development, where many candidate drugs and vaccines fail safety and efficacy tests. In subsequent reforms, the U.S. government has adopted a greater share of the development risk by offering more flexibility in its financial support. More funding has been made available for milestone-payments, when certain points in the development process are reached. Funding has also been made available to reimburse development activities in real-time.

Limited capacities of small companies - Project BioShield initially underestimated the limited financial and technical capacities of smaller companies, who constitute the majority of industry partners. In addition to flexible funding, access to non-financial incentives, such as scientific, regulatory and industry expertise, is particularly important for these companies. The newly established Biomedical Advanced Research and Development Authority (BARDA) responds to this need by providing scientific and technical support services that are otherwise found in large pharmaceutical companies, including around non-clinical and clinical studies, licensure and regulation, and manufacturing.

Burdensome federal contracting - The review of Project BioShield found that the administrative burden imposed on companies by the standard federal contracting process was partly responsible for limited industry engagement. BARDA's Broad Spectrum Antimicrobials program has since offered a more flexible contracting mechanism in the form of the Other Transaction Authority. This approach allows for product candidates to be brought into and out of development without a new agreement having to be negotiated each time a candidate drug or vaccine fails, as would be the case in a conventional federal contract.

Policy Implications

Adopt a comprehensive approach to risk-sharing that includes not only financial support for early research and for market creation, but also flexible and real-time payments for development activities.

Provide non-financial incentives, such as access to scientific, technical and regulatory expertise and guidance as well as industry expertise. Access to such knowledge, in addition to funding, can serve as a powerful incentive to engage smaller companies and companies from low- and middle-income countries.

Offer flexible contracting mechanisms that allow companies to shift candidates in and out of a project without renegotiating the partnership agreement.

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