UNITED NATIONS SECRETARY-GENERAL'S HIGH-LEVEL PANEL ON ACCESS TO MEDICINES

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ABSTRACT

Our current system for medical innovation, though heavily financed by public money, has evolved into a business model designed to maximize return on private investment. It is not effectively responding to critical public health needs and incapable of upholding the right to health. Most new medical products offer little added therapeutic benefit, and the few that do are priced too high for most people and health systems. A radically new approach, "Health

Innovation as a Public Good", is needed to reinstate medical innovation for the public interest. This approach is designed to promote innovation that improves public health outcomes globally, harnessing society's scientific and technological progress to deliver needed health technologies that are accessible and affordable.

This new approach combines elements from the first-generation non-profit Product Development Partnerships with the government responsibility, financing and leadership present in other sectors of strategic importance such as aerospace and defense. Focused on delivering therapeutic advances, it pursues a mission-oriented R&D pipeline informed by global public health needs. Publicly financed and under direct public leadership, it identifies priorities, deploys public and private sector implementing partners, allocates resources, manages portfolios, and makes critical decisions in a transparent and accountable way. Committed to open science and the commons, results are made publicly available to promote data sharing, crowdsourcing, and follow-on innovation. The resulting therapeutic interventions are public goods that can benefit all.

Financed by public money and not aimed at generating financial return, this new approach is cost-effective and sustainable. As it generates cheaper medicines for all public health needs, the Health budget's savings made when buying these medicines are reinvested for further innovation. Taken together with less wasteful R&D processes, a broader spectrum of health needs can be addressed in a more efficient way.

SUBMISSION

1. Introduction

For a sustainable solution to the chronic crisis in access to medicines, the way in which medical innovation is defined, conducted, regulated and financed must be redesigned. It should be redirected to improve health outcomes as a matter of social justice and rights; not to generate profit. Medical innovation must be designed to generate improved health interventions, available and accessible to all, and contribute to the progressive realization of the right to health. For the purpose of this submission, "Public Health Innovation" will be used to designate medical innovation that addresses unmet health needs globally and delivers therapeutic advances that are affordable and accessible to all .

Despite the widespread belief that biomedical innovation has led to an era of unprecedented medical progress, there is mounting acknowledgement of the deficiency and inefficiency of our current medical innovation model . This model fails because it lacks public health directionality, leaves pharmaceutical innovation in the hands of a profit-driven system that provides few levers for the public to control the way priorities and prices are set, misdirects incentives for research which results in wasteful and duplicative processes, relies on high rent-seeking private finance, and has become unaffordable even for the wealthiest health systems.

In the current model, governments reward private investors who develop new health technologies with patents and monopoly pricing in a non-transparent "market." This is so even when significant public investment has supported research and irrespective of a new

product's added therapeutic value. Economic and regulatory incentives have resulted in a highly financialized pharmaceutical business sector that fails to deliver the public health innovation we need. The majority of new medicines brought to market offer no therapeutic benefit over existing medicines. The few new drugs that do are unaffordable for most, save the wealthiest and well insured. Critical health needs remain unmet, such as treatment for poverty-related diseases, multidrug-resistant bacterial infections, and emerging infections like Ebola and Zika viruses . Though the challenge of access to medicines has long been a concern for low- and middle-income countries, high prices now threaten equitable access to treatment in the world's wealthiest countries.

The UN High-Level Panel has the unprecedented opportunity to call for a radical change. It should recommend a new approach to medical innovation that transforms the way the world treats medicines and other health technologies—not as luxury commodities but as public goods. Public Health Innovation must be driven by public health needs and social justice; be transparent and accountable to the population; and deliver products and interventions that improve health outcomes, and that are accessible and affordable to all. This new approach should harness science and technology for the public interest, not profit. It should not rely on private capital that needs return on investment to finance research and development.

This submission lays out the principles of such an approach, but the details—particularly around implementation and governance—will require further thinking and discussion among a broader group of stakeholders, which the panel could initiate.

2. A New Approach: "Health Innovation as a Public Good"

"Health Innovation as a Public Good" (the "New Approach") combines elements from the first-generation Product Development Partnerships (PDPs), that manage not-for-profit needs-driven health technology development, with the government responsibility that is assumed in other sectors of strategic importance such as aerospace and defense.

In this New Approach, a decentralized network of public and private partners is implementing a mission-oriented R&D pipeline, tightly managed according to target product profiles informed by patient and public health needs. Financed by public money, the network functions under direct public leadership, has a participatory governance structure, and is ultimately accountable to citizens. Following R&D priorities defined by WHO and including all unmet global health needs, it identifies Public Health Innovation opportunities, allocates resources, manages portfolios, and makes critical stop/go decisions in a transparent and accountable way. Results are made publicly available to promote transparent data sharing, crowdsourcing, and follow-on innovation. Resulting therapies are public goods and available at a small margin over cost in what is essentially a generics market (under government license or contract).

In order to be financially sustainable, and in contrast to current approaches that focus on neglected diseases, the New Approach is applied across all global public health needs including therapeutic areas that are targeted by the commercial system and consume a large part of governments' pharmaceuticals budget (e.g. cancer, hepatitis C, cardiovascular

disease). The substantial savings generated from less costly medicines in these areas can be pooled and allocated to Public Health Innovation responding to all health needs, for instance through a global biomedical R&D fund as proposed by others .

Given that it focuses on what the commercial system does not deliver, the New Approach is expected to develop in parallel, building upon ongoing public-interest initiatives that could come together into a crosscutting approach to advance the right to health and address public health needs, utilizing public resources in a cost-effective way.

"Health Innovation as a Public Good" differs from other existing and proposed approaches and initiatives, including PDPs or the WHO/TDR proposed pooled fund for diseases of poverty. Its key defining principles are:
- It is a new systemic approach to Public Health Innovation, as opposed to a focus on only a specific neglected therapeutic niche or neglected population;
- It relies on public leadership, and is accountable to government(s) and citizens through a transparent, participatory governance structure. (These details need to be defined, and would include a critical role for WHO);
- Like PDPs, it actively manages R&D projects and portfolios, from discovery research, to implementation of the health interventions. This differs from the "funding investigator-driven research projects" approach that is customary in the public health sector;
- It considers Public Health Innovation a public good, which does not need to generate return on financial investment. It will be financed by public funds and be driven by public leadership, though the private sector would play an important role as a partner in the innovation process.
- It presents a structural, global, transformative and rights-based solution to the chronic problem of unequal access to medicines and to misdirected medical innovation that results from a market-based approach to health; it is not simply a remedy for the worse symptoms of that approach.

3. Core elements for the design of a "Health Innovation as a Public Good" approach

The current R&D system delivers what it was designed for: sales, profits, and a prosperous pharmaceutical industrial sector. These objectives shape R&D priorities, processes, and financing, in ways that often conflict with Public Health Innovation that is accessible and affordable to all.

In the New Approach, the R&D system is fundamentally redesigned to meet global health needs and improve health outcomes. It consists of the following four elements:

3.1. R&D priorities that are determined by global health needs

Goal: Health-needs are prioritized, available scientific knowledge is utilized, and the sole aim is to improve health outcomes.

What we have now:

The commercialization of medical research creates multiple perverse incentives that steer R&D priorities and decision making away from Public Health Innovation. The profit motive dis-incentivizes the prioritization of cost-effective responses to health needs. Instead, investments aim at maximizing sales and tend to prioritize "me-too" drugs and "evergreening" strategies that provide no therapeutic advance over existing medicines. Without a regulatory requirement to demonstrate added therapeutic value, it is no surprise that more than 70% of new medicines are no better than what existed.6

The lack of Public Health Innovation has been well documented for so-called type II and III diseases, which predominantly affect people living in developing countries. Yet, other health needs remain sidelined, including new antibiotics to combat growing multi-drug resistant infections. Only when infectious disease outbreaks become a global security concern is there financial incentive to fund needed R&D efforts—as with the Ebola and Zika virus outbreaks. Disease prevention and cure is neglected, as chronic or life-long treatments offer better prospects for medicines sales.

Profit motives also create wasteful distortions in the drug R&D processes. Most critically, research skews toward what can be patented and commercialized, rather than what brings most medical benefit. When those two objectives occasionally overlap it is by chance; not design. In its most extreme manifestation, commercial disease mongering has artificially created markets, as in the cases of restless leg syndrome, pre-menstrual dysphoric disorder, or low testosterone. In 2014, 41% of new drugs approved by the FDA targeted rare diseases. While such treatments are needed, Orphan Drug legislations that guarantee high prices and generous profit margins may further skew R&D priorities away from public health needs. A recent study found that in cancer research, many more trials test treatments that could prologue survival of terminal patients by a few months than that would improve long-term survival for early stage cancer patients. The latter studies take longer and are more complex, clashing with financial markets' demand for immediate gains.

What the New Approach offers:
The efficiency of the R&D process will dramatically increase by focusing squarely on improving health outcomes, without needing to take into consideration patentability, financial markets, or commercial prospects. It will target resources to address public health needs, determined by a WHO-driven process that can build on the recently created Global R&D Observatory. It will mobilize the world's scientific knowledge and technological capacity to provide cost-effective solutions for those needs. This may involve acquiring government access to patented technologies to further the public interest, as allowed under international law . Freed from the need to only pursue patentable technologies, a wealth of scientific opportunities that remain unexplored—or have been abandoned—will become viable.

Regulatory reform that demands evidence of improved effectiveness over existing options is a straightforward but crucial change that could be enacted today, improve the efficiency of the current system significantly, and be catalytic towards the aspired "Health Innovation as a Public Good" approach described here .

3.2. Sustainable and transparent public financing

Goal: Public financing for that is cost-effective, sustainable, transparent and accountable to the tax payer.

What we have now:
There is significant public investment in R&D throughout the process, including basic research on which much biomedical and medical R&D relies. Product development is often financed, in part, by private capital. The pharmaceutical industry justifies high drug prices by citing the need to yield returns on such investments and to fund future innovation. Despite the lack of transparency on the actual cost of commercial R&D , it is clear that financing medical innovation with private capital is needlessly costly, and that the public ends up paying twice—once for the research, and again for the pricey medicines.

In recent years, the pharmaceutical sector has become exceedingly "financialized." It is interdependent with speculative financial markets and relies on equity investors and venture capital seeking to maximize returns in the short term. This is made possible by high drug pricing in a third-party payer "market," and driven by aggressive marketing. Focused narrowly on maximizing shareholder value, pharmaceutical companies spend more on repurchasing their own corporate stock and boost their stock prices than on R&D, while stifling innovation.

In addition, policy makers often assume that public research funding should be used to promote knowledge-based economic growth—by de-risking and bankrolling private sector efforts—rather than to further public health goals. As a result, public investments are typically undervalued when risk and rewards for medical innovation are assigned, and decisions on R&D priorities and prices are left to the private sector despite that their primary objective is to maximize shareholder value.

What the New Approach offers:
Because it relies on public financing (possibly supplemented by philanthropic funding), this approach does not require private capital, which is expensive and demands high and quick returns. Instead, by generating affordable medicines in place of expensive products, the savings produced within health budgets by buying cheaper medicines will be invested in R&D for the public interest, which itself would be less costly and wasteful than the current commercial model (see 3.3).

3.3. Effective and efficient management of R&D processes for improved health outcomes

Goal: Public Health Innovation that is cost-effective, uses available scientific knowledge to improve health outcomes, and is mission-oriented

What we have now:
Though the risk of failure exists at each step along the R&D pipeline, the current system has institutionalized and accepted failure as inevitable attrition, without disciplining it, while transferring its cost to the payer through ever-higher prices.

Many discovery and development projects in the pharmaceuticals industry "fail" not because

the candidates are not effective, but because of business considerations. These include, "patent position not strong enough," "competition too far ahead," or "expected market size less than another project." The industry's many mergers and acquisitions are typically accompanied by closing down R&D centers or portfolios. The resulting loss of knowledge and potentially valuable research is logged as "failure." And its cost internalized into the overall cost of R&D. 28

A related inefficiency is that the pharma industry discards many potential medical breakthroughs simply because they are non-patentable (compounds that are already known, previously patented, or not inventive enough). Similarly, research efforts are wasted by trying to "invent around" competitors' patents and by trying to find a patentable compound within a compound class of proven benefit. Other inefficiencies result from research secrecy, causing duplication—if not distortion (see 3.4)—of results, as well as from the lack of mission-oriented research (i.e. with the goal of improving a specific health outcome) that characterizes much of the curiosity-driven or technology-driven innovation.

If the need for patent protection is removed, medical innovation can benefit from existing knowledge, as shown by DNDi's development of fexinidazole, a resurrected drug candidate that is currently in phase III clinical development as a breakthrough therapy for sleeping sickness, a neglected disease.

In contrast to basic research, biomedical innovation to address health needs and improve health outcomes must be guided by the end result, and closely managed to achieve to desired outcomes (not just scientific publications). An inspiring example from another sector is DARPA, the U.S. Defense Advanced Research Projects Agency. In contrast to NIH and other public health research funding schemes, where funding decisions are based on scientific merit as judged by peer-review, DARPA commissions mission-oriented project research to tackle a well-defined innovation challenge. Funding recipients are judged based on whether they have met specific milestones, and the development process is actively managed.

An interesting step into that direction was the creation of the US National Center for Advancing Translational Sciences (NCATS). Established to fund and accelerate translational R&D, it focuses on mid to late stage development of new health technologies. By transferring these to the private sector for end-stage development and commercialization, it essentially subsidizes and de-risk the private sector within the current commercial model. As a consequence, and critically distinct from a Public Health Innovation approach, there is no commitment to affordable pricing that would reflect the public contribution, nor is there a development pathway for technologies that could address unmet health needs that are not commercially attractive.

Another interesting example to learn from is BARDA, the Biomedical Advanced Research and Development Authority. It was created by the US government to develop, manufacture and procure medical countermeasures to respond to health security threats like chemical and biological incidents or attacks and public health emergencies, showing that it is possible to harness government responsibility towards needed health innovation, if considered a strategic priority.

What the New Approach offers:
Much wasteful R&D could be eliminated and promising avenues could be freely and fully explored by actively managing mission-oriented R&D projects and portfolios in ways that are analogous to DARPA, BARDA and PDPs. Available science and technology can be harnessed as needed by obtaining government licenses with sole goal of addressing health needs and improving outcomes. The New Approach relies on a combination of grants, milestone prizes, and contracts to enlist relevant public and private partners. Guided by public health needs, independent of patentability or commercial prospect, and focused on efficiency and cost-effectiveness, the economics of Public Health Innovation are more amenable to public financing.

3.4. Transparent and evidence-based innovation, based on independent research

Goal: Reliable and publicly available evidence on safety and efficacy, in particular comparative effectiveness, obtained through independent studies, with open and transparent methods and shared data.

What we have now:
Safety and efficacy research and documentation are held by researchers and companies with vested financial interests in positive outcomes. Under the guise of commercial confidentiality, a systemic lack of transparency in the underlying research data and methods precludes adequate oversight and exposes people to potentially harmful treatments.

Companies tend to highlight products' potential benefits while downplaying their harms, including hiding known risks. The cases of Vioxx and Avandia are just the tip of this iceberg. In addition, and as highlighted under 3.1, regulators do not require drug makers to demonstrate improved effectiveness over existing products. This has incentivized a flood of "me-too" drugs for which the benefits are not established, but are promoted through heavy marketing30. The public health perils of this situation are increasingly highlighted by public health and human rights campaigners worldwide.

Beyond these harms, there is massive waste and duplication in both public and private clinical research as a result of non-published and non-shared research data.

What the New Approach offers:
An independent global network of existing clinical trial centers committed to Public Health Innovation, will conduct open source public interest clinical research under public leadership. It will work in close collaboration with regulatory and health authorities to design and conduct the clinical development of new candidate treatments with potential public health impact to demonstrate improved effectiveness over available interventions in a cost-effective way. Clinical trial designs, methods, results, and data will be in the public domain, providing full transparency, including on costs, and allowing follow-on research.

Like the regulatory reform proposed under 3.1, building such an independent clinical trial

network and initiating public-interest trials is a crucial, and potentially catalytic step towards "Health Innovation as a Public Good" that can be implemented today.

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Newer / Older

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