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Transforming the medical innovation and access ecosystem in pandemic preparedness and response: reform proposals to break the status quo and improve global public health outcomes

(Przekształcenie ekosystemu innowacji medycznych i dostępu w zakresie przygotowania i reagowania na pandemie: propozycje reform w celu zerwania ze status quo i poprawy stanu zdrowia publicznego na świecie)

SUMMARY

Doctoral dissertation prepared in the Chair of International Law and International Relations under the supervision of Prof Joanna Połatyńska in the discipline of legal sciences

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Presented dissertation addresses the problems of the existing medical innovation and access ecosystem, with a particular focus on medical countermeasures.

It is focused on systemic changes in the system, as well as in political thinking about the role the public sector should play in it.

The emphasis is placed on policy reforms in recognition that it is politics and states' ideological and cultural heritage that shape the law. This is also true at the international level, where most regulations and guidelines are *soft law* rather than legally binding instruments. Changing states' approaches to pharmaceutical R&D and access will have a direct impact on the formation of law and practices at national, regional and international levels.

The fundamental reforms discussed here would form the basis for specific regulations which should be then introduced to achieve the desired objectives. This thesis, however, does not aim to provide an in-depth analysis of the current legal framework or to present comprehensive legal proposals. It does, however, touches upon some legal problems, for example in the context of human rights, including the right to health and the responsibility of private companies in this regard, and makes general *de lege ferenda* proposals. These observations refer, among other things, to establishing an appropriate policy and legal environment for companies pursuing public interest so that they can be sustainable and competitive in the pharmaceutical market (while at the same time not being able to game the system), or to revising the international legal framework to improve global pandemic preparedness and response through greater cooperation between countries on R&D of medical countermeasures and ensuring equal access to them worldwide.

While the dissertation makes these selected observations on existing regulations and points to the need for new ones, its objective is to identify flaws in the design of the current medical innovation and access ecosystem and show how its underlying principles and ways of working can be transformed to better serve the public interest. This policy and conceptual shift should be supported by appropriate legal changes in order for the pharmaceutical sector to operate effectively under the new conditions. However, as noted, proposing these detailed legal options is beyond the scope of this dissertation.

The dissertation demonstrates how the current pharmaceutical research and development model is not able to deliver the most relevant medical innovations while ensuring sustainable, affordable, and equitable access to them. It discusses many of the

reasons for this, not least the handing over of responsibility for the development, production and supply of pharmaceutical products to for-profit companies which, due to their statutory form and intrinsic characteristics, are guided by profit-maximising strategies instead of providing the most needed and effective products to as many people as possible.

Importantly, the fact that the public sector has ceded much of its responsibility for pharmaceutical R&D and access to the private sector does not mean that it has ceased to engage in it. The public sector continues to fund the highest-risk research and is most likely to discover medicines that offer significant therapeutic benefits over the existing ones. Therefore, the dissertation analyses the roles of public and private actors in the pharmaceutical system and how these are shaped by states.

A number of technological breakthroughs – both health emergency- and non-emergency-related – were (and still are) funded by government programs and institutes. While the amount of public funding was unprecedented during the COVID-19 pandemic, the patterns it followed are typical of the current model: the public sector provides significant funding, transfers the technology to private companies that further develop it and manufacture end products ultimately purchased by governments at a premium.

Pharmaceutical policies that allow public research and knowledge to be privatised and the resulting products to be supplied and priced based on market forces to maximise profits, rather than becoming the most effective public health tools, result in gross inefficiencies. This model is particularly lucrative for the private sector but has dire consequences for the public.

The dissertation discusses the root causes of the current system's failures and inefficiencies arguing that they lie in its flawed design and misconceptions reflected in various aspects. The way the system is structured ignores the fact that the functioning of pharmaceutical markets differs from the neo-capitalist model. For one, due to limited competition guaranteed by strict intellectual property rights and exclusivities, pharmaceutical companies have considerable power to determine the availability and affordability of medicines. What is more, demand for drugs is inelastic and pharmaceutical prices are opaque and do not reflect the value of products but what the market can bear. The discussion confirms the inadequacy of neo-capitalist markets to drive medical R&D and provide affordable access to its outcomes.

While the divergence between public interest and private considerations driving medical innovation decisions is evident for all types of pharmaceutical products and circumstances, it is particularly pronounced in the context of pandemic preparedness and response. The current system does not prioritise the development of the most appropriate medical countermeasures and the race to get a product to market as quickly as possible and fend off competition is counterproductive for the public.

It is also evident that the system of rewarding medical innovation by monopolies guaranteed by a strict regime of intellectual property and market exclusivities leads to the hiding and fragmentation of knowledge, a lack of collaboration and less access to technologies and products. Consequently, this significantly reduces states' ability to effectively prepare for and respond to health emergencies.

Finally, the dissertation analyses one of the worst consequences of the current system's failure – global inequalities. The imbalance of power between states widens the gap in terms of access to expertise and products between high-income countries with the originator companies they host on the one hand, and developing countries with their generic manufacturers on the other.

Inequalities in access to medicines are the result of inherent conflicts in the global pharmaceutical system. The discussion on the evolution of the global health architecture shows how this directly stems from the ways the existing mechanisms and initiatives have been shaped and evolved, including the ubiquity of public-private partnerships, where the balance is significantly tilted in favour of private interests. The response to the COVID-19 pandemic is an example of this system's inherent flaws and the lack of solidarity that prolongs health crises, causing enormous suffering and preventable deaths.

From the massive public investment and direct involvement in medical innovation that do not provide an adequate return in terms of equitable and affordable access to end products, to the dependence of public health interventions on the willingness of private companies to engage in them, the dissertation provides ample examples of how the current pharmaceutical R&D and access ecosystem is unable to effectively respond to public health needs. It is argued that the failure is neither accidental nor exclusive to health emergencies. The current pharmaceutical system is not fit for purpose.

Building on this analysis, the second part of the dissertation discusses how to change the way medicines and particularly medical countermeasures, are developed and accessed.

The overarching premise of the dissertation is that governments themselves should take greater responsibility for defining the direction of health innovation, ensuring access based on equity and human rights principles and shaping the R&D ecosystem accordingly.

This will require an end-to-end system that, from basic research to clinical trials, production, procurement and delivery of final products, is guided by these principles. It should be publicly and transparently governed as well as substantially funded while ensuring that the overarching goal of enhancing health security is embraced before any economic interests, and that risks and benefits are shared fairly between public and private actors from the outset.

Even before such an overarching system is put in place, states can improve access to health technologies by attaching specific and strict conditions to public funding for pharmaceutical R&D. These should include guarantees that products developed (entirely or partially) with public money are priced fairly so that people can afford the medicines they helped to develop. A key condition in the context of health emergencies should be that, in times of crisis, all forms of intellectual property, data, know-how and biological resources required to develop medical countermeasures are made widely available to scale up their production.

Whereas the first part of the dissertation argues that the current system of incentivising medical innovation through monopolies is grossly inefficient, the second part presents alternative options.

There are various models developed for the purpose of making investment in R&D more cost-effective and responsive to public needs. They are analysed in recognition that different disease areas and different products may require specific ways of funding, incentivising and rewarding R&D activities. A wide range of these models are presented and examined. To illustrate how they can be used, often together, in specific disease areas, the examples of their application in specific contexts are also described.

Different alternatives such as pooling of intellectual property rights, technologies and funds; advance market commitments; patent buyouts or regulatory incentives are discussed in more detail. Of the various mechanisms, options based on decoupling investment in innovation from drug volumes and high prices may most effectively stimulate innovation while ensuring affordability and accessibility.

What is more, in the context of neglected diseases, the strengths and weaknesses of product development partnerships are presented while various types of regulatory incentives are analysed using EU regulations on orphan and paediatric medicines as an example. In the context of attempts to increase innovation of new antibiotics and manage adequate access to them to limit antibiotic resistance, alternative models, such as offering governments to pay a subscription or licence fee for priority access to them at a certain price or an options market model for antibiotics are demonstrated.

Finally, special consideration is given to alternative models for the development and access to medical countermeasures. Innovation in this area would ideally be based on an open knowledge model, which could generate technological advancements free to use, with no legal restrictions. An open approach to innovation, including open source and open access schemes, could maximise research potential, speed up the development process, increase the scale of production and consequently provide broad access to end products. Cobervax can be considered proof of this concept. The dissertation argues that the best approach would be for countries to jointly fund and develop products such as vaccines making them available to all as public goods. For this to happen, the public sector (in partnership with private actors or through direct involvement in R&D and production, known as a *public option*) should invest in and steer their development. Ideally, this should be done through an international mechanism for joint financing and R&D, or at least by pooling resources.

Following this discussion, the dissertation delves into how to reduce global inequalities in access to health technologies and make the pharmaceutical system work for all. It argues that to increase equitable access to medical countermeasures worldwide, it is necessary to expand R&D and production capacity in the Global South. The Global South countries need to develop the expertise, know-how, skilled workforce and infrastructure to absorb existing technologies, be able to adapt them and develop them further. The technologies should be controlled by governments, who should also be in charge of the allocation and pricing of end products. The role of international cooperation, which can be strengthened through a new *pandemic treaty* and revised International Health Regulations, is key, and ways to achieve this were also discussed.

All these models and structural changes demonstrate the breadth of alternative approaches to increasing medical R&D activities and providing more equitable access to health products. Some of them also show the potential to go beyond the current mainstream,

profit- and market-driven commercial system. Their use would allow the public sector to actively shape innovation and the market.

Selecting the most appropriate models and implementing them at the national or regional level according to specific circumstances and identified medical needs pose a significant challenge. Various instruments may in theory fill similar gaps or have comparable effects, but differences between them make their suitability dependent on specific contexts. Local conditions and existing models could result in the same policy choice having different effects when applied elsewhere.

The discussed proposals aim to transform public sector governance and leadership, increase multilateral cooperation, shape the market, and influence the decision-making of private companies. These actions, however critical, do not exhaust the possibilities of changing the pharmaceutical R&D and access ecosystem.

Given that the poor outcomes of the system are a consequence of its ineffective design, far-reaching options must also be considered, such as altering the ways in which private actors operate on the market – or even changing the actors themselves – to promote corporate governance which considers aspects beyond profit and leads to better value creation.[1]

In this context, solutions such as limiting the practice of share buybacks, setting conditionalities of profits' reinvestment, or tying executive compensation not to stock value but to equal access to the produced goods, among other things, are proposed.

To improve the performance of pharmaceutical companies in line with public health needs, changes can also go beyond their governance and operations. While the model of shareholder-owned corporations is currently dominant in the pharmaceutical market, it is not the only possible way to structure economic activity leading to the development and manufacturing of medicines. The dissertation argues that to combine the ability of attracting private capital with delivering on public health needs, public policies should encourage the involvement of corporations with other legal forms, such as non-profit or limited-profit companies and benefit or social purpose corporations in the sector. The statutory form of currently prevailing for-profit companies could be changed to one of the above to *alter their incentives from the inside* and to enable and require them to consider other interests beyond shareholder value.

[1] UCL Institute for Innovation and Public Purpose, *The people's prescription, op. cit.,* p. 42.

Changing the statutory form of pharmaceutical corporations can be a difficult task, but one that may be worth the effort. As with other major reforms, transformational changes to bring greater benefits to the public will require broad policy and regulatory action. Without the right environment, companies that are not solely driven by profit maximisation will be at a significant disadvantage, unable to compete in the current profit-driven capitalist market, unless other mechanisms are put in place to favour them.

On the other hand, it is also argued that for companies whose statutory form would require them to pursue public interest goals, there must be robust enforcement laws put in place. The ability to hold corporations accountable for adhering to their statutory objectives through rigorous oversight mechanisms is critical to effecting meaningful change. Corporate law should be equipped with the right tools and laws to ensure that corporations do not game the system.

Lastly, besides changing the ways for-profit companies operate in the pharmaceutical sector and introducing corporations that have other statutory forms and hence could more efficiently serve the public interest on the market, it is also possible to bring another actor directly into the mix – the public sector.

The *public option* involves the creation of national public pharmaceutical R&D institutes, manufacturing sites as well as wholesale and distribution companies. Public companies would need to be based on the principles that should guide all public sector activity in pharmaceutical innovation, i.e. they should be fully public interest driven, oriented toward public health goals, transparent and include safeguards against undue influence and conflicts of interest. The dissertation also gives consideration to how public companies in each segment should operate, including examples of their successful implementation (as well as failures in this regard) around the world. It is noted that the *public option* can be complementary to other reforms discussed above or tested independently of them.

The dissertation argues that the *public option* for pharmaceutical R&D, production and supply can be of particular importance in the context of health emergencies. Having an independent public capacity that can effectively execute a preparedness strategy and, perhaps even more importantly, respond quickly and efficiently during a crisis can greatly improve the way countries deal with pandemics and similar crises.

The dissertation recognises that the implementation of most of the alternative models, including the *public option* and leading to changes in the statutory form of

companies in the pharmaceutical market requires strong leadership and robust public sector's structures. Introducing them would be a major, costly and long-term commitment. Their full success will depend on resilience to frequent political changes.

However, only through bold and dedicated public policies implemented by visionary political leaders can universal access to medicines and the right to health as such become a reality.