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# Towards Transformative Governance? Responses to mission-oriented innovation policy paradigms

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# Managing urgent innovations
# Exploring restrictive and broad strategic niche management

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Today, society is facing some extraordinary challenges. Environmental pressures and resource scarcity question the sustainability of our current consumption pattern, and longer life expectancy and chronic diseases are putting a strain on the capability of health systems to meet the needs of the (ageing) population. A broader, mission-oriented innovation policy is increasingly seen as being critical for effectively meeting these grand challenges.

Innovation, however, always has a dual character in the sense that innovation might simultaneously spur positive and negative effects. New technologies hold promises for economic growth, improving health or decreasing environmental problems, but also fears for economic decline, health damages or pollution. The duality of potential benefits and negative consequences is poignant when a wide range of actors need the innovation with such urgency that fast market introduction is required. Urgent innovation is challenging since fast introduction may endanger thorough investigation of potential risks.

This paper delves deeper into the challenge of managing such innovations with a high degree of urgency. As case-in-point we focus on innovative pharmaceutical products intended to meet unmet medical need. For these products EU and US government instated specific regulatory pathways that ensure shortening of the clinical testing and regulatory decision phases, but at the same time require stricter commitments about monitoring the drugs while they are on the market. Examples of disease areas to which these regulations are applicable because of their related unmet medical needs are HIV, cancer, pandemic influenza and orphan diseases (Boon et al., 2011).

The introduction of novel pharmaceutical products is heavily regulated and procedures are to a large extent standardised. Accelerated procedures enable fast introduction by bypassing some of these procedures and, therefore, they call for flexible solutions to monitor the effects of these drugs. Uncertainties about these effects, including beneficial impacts (efficacy) and risks (safety issues), might be higher than with normal drug approvals. Accordingly, this paper deals with the challenge of managing innovations with a high degree of urgency while minimising the safety risks of these innovations.

It is assumed that besides new scientific and technological developments, more demand-oriented innovation policies, such as flexibilisation of market approval of drugs, combined with a more thorough post-marketing surveillance of drugs for unmet medical needs, are necessary to achieve transformative changes in dealing with urgent innovations.

Strategic regulatory niche management – defined as creating and managing a space that is protected from the harsh regulatory selection environment – is presented as an approach to this more demand-oriented management challenge. The (temporary) space allows for the marketisation of innovations under the condition of strict monitoring of their effects. Restrictive and broad approaches to the strategic management of regulatory niches are discerned and theoretical advantages and disadvantages of these two approaches are formulated. The advantages and disadvantages of these two approaches to strategic management of regulatory niches are further explored in two case studies: 1) the development of treatments for HIV and 2) the development of a vaccination against the pandemic influenza. Based on strategic niche management, we propose a conceptualisation of the dynamics of creation and maintenance of 'regulatory niches'. The first step in this process is the creation of a shared agenda about the necessity of niche protection through special regulation. This leads to the formation of a regulatory niche in which the innovation can be implemented and monitored. The niche and its demarcating boundaries are then fleshed out by network building and learning. These dynamics inside the niche, but also concerning the actors outside the niche, define and redefine the niche boundaries.

We found that in the HIV case, there was plenty of room for bottom-up, informal user initiatives, and only after a few years some post-marketing studies and monitoring organisations were formalised. A relatively open community network existed, in which users played a major part. The excluded actors were mostly powerless or included strategically along the way. Accordingly, the HIV case answered to a *broad strategic niche management* approach.

In contrast, in the influenza case there was a high degree of top-down steering. A formal, close-knitted network of regular users was in place that was adjusted to the pandemic and augmented by new initiatives set-up by the Netherlands Pharmacovigilance Center. Informal interactions and rules were placed in a more formal context. 'Critical' groups of non-users remained outside this regulatory niche network. The pandemic influenza case answered to a *restrictive strategic niche management* approach.

The two cases focused on post-marketing surveillance in the Netherlands. The politico-economic tradition and governance culture in the Netherlands can be characterised as

democratic, deliberative, and supportive to discourse-based decision-making. This is further expressed in the large variety of intermediary organisations engaging in the political arena in a corporatist/participatory rather than a confrontational/activist way. The two cases show on the one hand a situation that leans more towards corporatism (HIV) as compared to, for example the US in the 1980s. On the other hand a situation leaning towards confrontation (pandemic influenza), which is more or less comparable to other countries.

Furthermore, the cases can be regarded as exceptional, even singular, e.g. because of a high sense of emergency and media attention. However, the context of these cases is not unique. In the pharmaceutical field, the lessons are relevant because the European Medicines Agency Road Map 2015 provide openings for a more flexible approach to drug approval. One consequence can be that several approval and post-marketing practices co-exist inside and protected from the more general drug regulation niche, i.e. the EMA standard drug approval procedure. Furthermore, also scientific and technological developments, such as the advent of personalised medicine and pharmacogenomics, might make the use of drugs more fragmented, leading to a growing number of small disease niches. In this respect it is hypothesised that personalised medicine will increasingly resemble orphan disease fields (Boon and Moors, 2008).

The exploration of the two cases provided support for the theoretical advantages and disadvantages of the proposed two approaches to regulatory niche management, i.e. restrictive and broad strategic niche management. The homogeneous network in the restrictive approach is able to learn faster but offers less second-order learning than the (more) heterogeneous network in the broader approach to regulatory niche management. In line with our expectations, the restrictive approach was more contested.

This paper increases our understanding of the management of urgent innovations in the context of 'regulatory niches'. The focus on these niches provided knowledge about how the specific post-marketing practices in these regulatory niches are maintained, not necessarily through the protection provided by the regulation alone, but also because the protection spurs network and learning. The research highlights the advantages and disadvantages of restrictive and broad approach to strategic management of regulatory niches. The advantage of a restrictive approach is that it enables fast first-order learning and the disadvantage is that this may come at the risk of a decline in legitimacy. The advantage of a broad approach is that it enables more reflection and second-order learning which result in more legitimacy. At the same time, this may result in less ability to move fast and, interestingly, a heterogeneous niche may become too comfortable to break down. We conclude that strategic management of regulatory niches requires a balancing act between fast but superficial learning processes and

slower but more reflective ones. This balance determines the ability to react fast to the need for urgent innovations but also the long-term legitimacy of these innovations. Restrictive and broad strategic niche management could then be deployed as policy instruments to deal with the grand challenge of unmet medical needs and to govern transitions towards more sustainable healthcare.

**References**

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