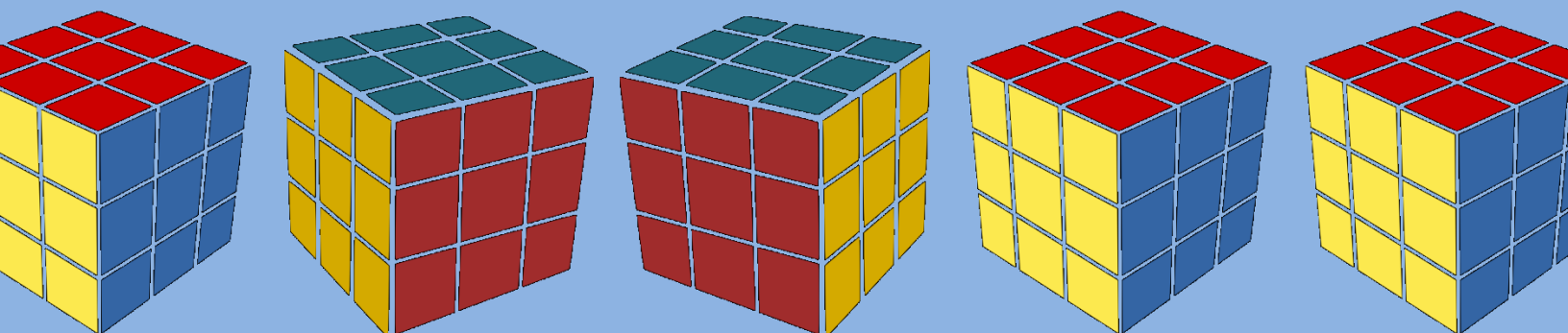


Targeted R&D Policy

Promoting Biotechnology:
A Generalized Toolkit for Policymakers



Annexes

© TARGET. All rights reserved. TARGET – Targeted R&D Policy - is a project within the Seventh Programme and is co-funded by the European Union (Grant Agreement 234522). The project duration is July 1, 2009 – September 30, 2011.

This document has been produced with the financial assistance of the European Union. The contents of this document are the sole responsibility of the TARGET Consortium and can under no circumstances be regarded as reflecting the position of the European Union.

Consortium Members:



Contents

Annex 1: Guiding Questions for Realist Sector Assessment	6
Precondition for a Targeted Policy	6
The Science Driver	7
The Training Driver	8
The Commercial Driver	9
The Financial Driver	10
The Human resources Driver	11
The 'Other Institution' Driver	12
Annex 2: Scientific Annex	13
Benefits of the TARGET Approach	13
Policy challenges facing a Targeted approach in general	14
Specific challenges in biotechnology	14
Annex 3: Stakeholder Cooperation.....	18

Annex 1: Guiding Questions for Realist Sector Assessment

While these questions are posed as Yes/No questions, their answers are based on a thorough investigation which is composed of multiple questions detailed below. It is to the benefit of policymakers to be objective in their decision of whether or not they meet minimum criteria. While answering "No" to the questions below may not necessarily rule out a policy process aiming to support a biotechnology sector, it would mean that missing Sector Drivers will have to be accounted for in the strategy.

Precondition for a Targeted Policy

Is there a political commitment? (YES/NO)

Guiding questions:

- Is the political commitment shared by multiple stakeholders?
- What is the size of the group willing to pursue a targeted policy?
- If it is a small group, how is it able to operate successfully without broader support?

If the answer is "NO":

- If there is no political commitment, is there sufficient private sector support to move a **TARGET** strategy forward any way?

If the answer is "YES":

- Since political commitment is usually tentative and may not be long-term, how would this affect the resources needed for a long-term strategy, and is it realistic to assume that a **TARGET** approach will be adhered to beyond a few years?

The Science Driver

Does a strong science base exist? (YES/NO)

Guiding questions:

- Is the science base strong in a particular niche or overall?
- Is the strength based on large size or just good performance from a small but qualified group?
- What criteria are being used to judge this (e.g. internationally competitive for funding and in high impact journals; patent applications)?
- How is R&D divided between basic research and applied research and is there high quality for each category?

If the answer is "NO":

- Is the particular sector worth pursuing through a targeted approach or are there better candidate industries?

If the answer is "YES":

- What are the possibilities of strengthening the science base in the short-term to achieve acceptable pre-conditions for the industry?

The Training Driver

Is a training personnel program already exist or required? (YES/NO)

Guiding questions:

- Are there sufficient knowledgeable investors, researchers and managers for the range of activity needed for a functioning innovation system?
- What skills are missing?
- Which kind of programs or training can be implemented? (Keeping in mind a time lag between the start of the programs and the first qualified individuals emerging).
- Are there key individuals who can take on mentorship roles or be examples to others?

If the answer is "NO":

- Is the particular sector worth pursuing through a targeted approach or are there better candidate industries?

If the answer is "YES":

- What are the possibilities of creating/strengthening a training program in the short-term to achieve acceptable pre-conditions for the industry?

The Commercial Driver

Is there a strong commercial basis for a targeted policy? (YES/NO)

Guiding questions:

- What is the measure of business activity in the sector?
- What is the number of companies?
- What indicators exist for judging commercial excellence and success in industry?
- Are technically skilled people in the science base willing to work commercially or interact with commercial interests?
- Are commercial skills set in other local industries are transferable?
- What networks and existing contacts already exist?
- Does the economy have any internationally recognized firms? Any local multinationals?
 - Is there foreign MNEs acting in the industry? What activities do they pursue locally? Are the activities high value-added or are they routine manufacturing/back-office work?

If the answer is "NO":

- Is the particular sector worth pursuing through a targeted approach or are there better candidate industries?

If the answer is "YES":

- What are the possibilities of strengthening the commercial base in the short-term to achieve acceptable pre-conditions for the industry?

The Financial Driver

Is there capacity for long run, significant financial support? (YES/NO)

Guiding questions:

- Is there any public financial policy to support life science/biomed activities?
- How much funds are dedicated to life science/biomed projects as percentage of the public R&D budget?
- Is there public investment on life science/biomed research centers?
- Are there public investments on life science/biomed incubators?
- Are there any public Venture Capital funds committed to life science/biomed?
- Do public Venture Capital funds have a special focus on any particular life science/biomed area?
- Are there any Public-Private Equity funds committed to life science/biomed?
- Are there tax incentives for Venture Capital firms with special focus on life science/biomed sector?
- Is there any specific incentive for the creation of life science/biomed focused Venture Capital firms?
- How many companies specialized in Finance Consulting exist?

If the answer is "NO":

- Is the particular sector worth pursuing through a targeted approach or are there better candidate industries?

If the answer is "YES":

- What are the possibilities of strengthening the financial base in the short-term to achieve acceptable pre-conditions for the industry?

The Human resources Driver

Is there human resources availability? (YES/NO)

Guiding questions:

- Are there sufficient knowledgeable investors, researchers and managers for the range of activity needed for a functioning innovation system?
- Is there are at least a small number of outstanding individuals in the economy?
- Do these individuals work as “Knowledge Brokers” / “Deal Makers”?
- Do they network internationally or only locally?
- Are they active in managing firms, or also active working with or in other bodies?
- Are they ideologically committed to developing the industry or sector?
- Does the regulation enable the job mobility for Human Resources in Life science/biomed?

If the answer is "NO":

- Is the particular sector worth pursuing through a targeted approach or are there better candidate industries?

If the answer is "YES":

- What are the possibilities of strengthening the human resource of the sector in the short-term to achieve acceptable pre-conditions for the industry?

The 'Other Institution' Driver

**Do other institutions exist in the economy for facilitating a biotechnology sector?
 (YES/NO)**

Guiding questions:

- Is there a clear IP protection regime?
- Is there clear legislation concerning clinical testing and product regulation?
- Have actions been implemented to increase patent applications in Life science/biomed?
- Have actions been implemented to increase university patent applications in Life science/biomed?
- Are there workers at the Technology Transfer Offices (TTO) with specific knowledge for life science/biomed?
- What is the health system budget as a percentage of the GDP?
- What is the number of medical doctors in Clinical Hospitals?
- How many people are working on clinical trial activities?
- What is the annual number of clinical trials?
- What is the annual number of clinical trials in starting phases?
- Is there any specific action to promote public procurement on personalized medicine?
- Are there actions to encourage the social acceptance regarding to life science/biomed innovations?

If the answer is "NO":

- Is the particular sector worth pursuing through a targeted approach or are there better candidate industries?

If the answer is "YES":

- What are the possible measures in the short-term to achieve acceptable pre-conditions for the industry?

Annex 2: Scientific Annex

Benefits of the TARGET Approach

Innovative activity will occur naturally to varying degrees in an economy. The degree of this activity will be directly influenced by the commercial capacity to exploit new knowledge; which in turn is affected by investments, commitments and knowledge made previously in the economy which may either fruitfully coincide with an emerging technology or conflict with it; and by external (global) market and non-market forces which affect the industry or sector. Moreover, because technologies like biotechnology encompass a fairly wide range of applications, the variety of the possible endpoints for the development of a biotechnology sector is equally wide.

The variety of possibilities means that, while we can present different examples of cases which have pursued a biotechnology sector with some success, there is no one path to that success which can be determined from these cases. The TARGET approach will offer to the policy maker a way to work through this complexity.

- The TARGET approach will help to **understand how the different components of the system are linked and work together.**
- Understanding how components are linked will help to **reduce the radical uncertainty of action**, as well as **present further opportunities for action which may move the biotechnological sector to a desired goal.**
- Understanding linkages in the context of the whole system means policymakers can try to **fill in structural or institutional gaps**, along with correcting market failures.
- By understanding linkages between system components, and how actors work, **supply and demand side policies can be applied.**
- The TARGET approach allows the policymaker to **perceive how things evolve simultaneously.** While a policymaker may be looking to cause a particular driver to evolve (e.g. science capacity), that driver will also be evolving because of the influence of other factors in the system which would have been acting upon it regardless of whether policy was directed towards it or not (e.g. past educational policy, presence of private sector R&D, R&D policy) – the question is how much change will be produced by the different influences.
- The policymaker will be able to **predict how their interventions may impact on other areas of the system**, besides their intended target. Because these drivers will evolve based on their context, an emphasis on regular and consistent system assessment will insure the necessary data flow for informed policy decision-making. Assessment will show how far the biotechnology sector has moved along its evolutionary path according to expectations derived

from other cases, and help policymakers understand the importance of timing regarding interventions; and identify unique characteristics which emerge in their own case.

Policy challenges facing a Targeted approach in general

Five general challenges to effective innovation policy encountered in the case studies conducted by the research team should be kept in mind:

- **Coordination** – ensuring that stakeholders network and build on each others’ efforts to facilitate the functioning of the innovation system, filling any gaps and correcting any bottlenecks.
- **Flexibility and long-term commitment** – sectors or industries that are targeted may be so new that the business plans and technology have not yet been completely determined. Therefore, flexibility in how these are addressed is essential. At the same time, many of the target interventions are systemic interventions, and institutional or system change requires a long-term view – so while flexibility is important, commitment to the overall strategy is also important. In other words, actors would ideally have the ability to change operational course if required, while maintaining long-term commitment to strategic goals.
- **Clarity, understanding and transparency** – clarity in the objectives defined and set out by policymakers and stakeholders is essential. To achieve clarity, and to properly set out a roadmap, an understanding of the national and regional context as well as the requirements of the industries in question is essential. Transparency and communication of the objectives and path is important to help coordination, and to avoid conflict amongst stakeholders which may cause long-term commitment to be more difficult.
- **The ability to create an arm’s length lead organization(s)** able to separate operational from strategic concerns, and able to maintain arm’s-length influence in a risk environment bound to produce a certain degree of failure.
- **Creating an environment accepting of risk and failure** and allowing cycles of failure to be formed.

Specific challenges in biotechnology

Moving an industry or sector through phases of development and facilitating innovation require very specific attention to the needs of the individual sector, as mentioned above. While a general description of a TARGET approach is useful, the development of a biotechnology industry presents

challenges which are unique to it, differing from sectors such as ICT. In fact, it would be inaccurate to refer to a single biotechnology sector, as the activities which are vying for commercial space range from stem cell therapeutics to IT heavy medical devices to environmental and agricultural applications. However, because this group of activities depend on an interrelated body of scientific knowledge and skills, it is addressed by policymakers and private sector investors as a single category.

As discussed above, a Targeted approach must be flexible, market focused, whilst still giving clear indications as to how policy and industrial coordination should proceed; due to the complexity of the industry, success and failure must be expected as specific policies or solutions which are attempted, and these may need to be readjusted. The main challenges encountered in the biotechnology sector are the following:

- **Regulatory system** - The regulatory system of any potential markets will affect the risk structure and cost of developing and delivering innovation to markets, requiring any strategy to take this into consideration. Particularly for products that fall under therapeutic or drug discovery, however, the cost of pre-clinical and clinical trials drives up the cost of development and risk of failure.
- **Different risk profiles for different products** - this creates a challenge since the incentive structure for different sectors within biotechnology will exhibit different risk profiles and therefore will affect how investors behave. Products such as medical devices or diagnostics generally exhibit a lower risk profile; however, they will also generally be low on the value-added scale and long-term growth impacts. On the other hand, the risky profiles of therapeutics, and the extensive difficulty and cost of having potential products make it through all regulatory phases, means that products face a greater risk of failure during a longer period of time than other products, and investors, public and private, may simply wish to discard the risk. If particularly high risk sectors are to be pursued, then differences in incentive structures must be addressed.
- **Time-lines** - Considering costs and the process of phased trials, the timelines for new biotechnology products, particularly in drug discovery in therapeutics are much longer than other technologies, and require constant commitment and monitoring.
- **A still developing business plan** - Mentioned as one of the main challenges above for any knowledge based sector, this is perhaps one of the greater concerns in the biotechnology sectors. Much of the science is new and a large number of potential products must still pass through regulatory measures, therefore what is possible to deliver to market is constantly shifting. Furthermore, the value breakdown of projects, and the inputs and participants into the value chain are constantly being rearranged as a result of the growing complexity of science and increasing cost of research and development. The classic chemical-based large pharmaceutical firm approach can no longer be counted on as the best approach, and even the

image of small firms acting as external laboratories for large pharma may need to be reconsidered. This is the main reason that, while a targeted approach may define starting points and conditions, and subsequent actions, a degree of flexibility is needed for the end goal of the industry's form. The societal goals, however, those which should be met by having a biotechnology sector, may still be defined.

- **A shifting landscape of firms** - As implied above, the multinational pharmaceutical sectors, which may form part of or anchor a national biotechnology sector, has been undergoing extensive changes. Many firms have been acquired, moved or are changing their market focus in terms of the kinds of drugs they seek to produce and research they focus on. The R&D sites that have normally been associated with these firms are constantly pressured to remain attractive, and new potential sites are competing both for R&D and high value manufacturing. While the exact impact of this on other elements of the biotechnology sector is unclear, it creates a sense of instability that will undoubtedly impact investment decisions.
- **Public vs. private systems of health** - The market dynamics for a set of products will be affected by whether there are multiple clients, a single client, competing clients or a single standard of care in a given market. Each of those has pros and cons that must be weighed, and will impact whether a product may even have a chance. For example, according to one comparison of the UK and US regenerative medicine markets, private healthcare systems may be a better environment for the development of new bio-based therapeutics because private hospitals and care providers are competing for patients and are more willing to use and advertise new technologies to attract patient/clients. In contrast, public health care is more reluctant to purchase new therapies if there is not an obvious and overwhelming difference with previous care. Another example compares the Canadian provinces of Ontario and Quebec and the public sector purchasing habits – Ontario's system preference for generic drugs used in the local hospitals and clinics has meant that generic R&D and manufacturing industry arose in the province in contrast to Quebec which uses brand-name drugs and have managed to attract more brand-name pharmacy manufacturers.
- **Niche markets vs. Blockbusters** - One of the main issues that therapeutics developers are wrestling with is whether to aim for a major blockbuster product that will be applicable to either a common disease or range of diseases and conditions, or whether they should focus on small niches in the health care system, or what may be considered orphan diseases. The risk and pay-off incentives of each differ, as well as the resources required for a company to carry something from research to the market.
- **Internal and external agglomerations of knowledge** - For the biotechnology sector, many times agglomerations of knowledge, and knowledge exchange, will occur outside of a regional or national boundary. Researchers will many times exchange ideas with colleagues in other parts of the world where there is expertise, and the setting may not be enough to sustain the

knowledge requirements of an industry. As a result, scales of analysis and interaction are important to keep in mind when building and following a strategy for biotechnology.

- **Demand building** - While many policies focus on supplying the necessary inputs for an industry, it should be considered whether there are any policies that can build demand and uptake for capital, skills or the final products that may create incentives for actors to become more involved. See above point regarding public versus private systems of health and policies that may have to work around institutional limitations.
- **Judging good science** - Different cases claim to have “good science”, but differ widely in comparison with each other in terms of scale and the available skills. What actually constitutes good science? And what is enough of a base for a strong biotechnology sector – whether in one niche or spanning several? This is stated in the assessment section above, but it is worth reiterating due to its importance.

Annex 3: Stakeholder Cooperation

As discussed above, while the kick-off decision does not necessarily have to be made by the party that will eventually manage the process, it is important that as wide an agreement as possible amongst stakeholders to pursue the strategy is obtained. For policymakers involved, this will involve different steps. The first is the identification of potential partners and/or opponents to such an approach. This is not to be confused with the later, more detailed identification of direct and indirect stakeholders who will be participating in the system which occurs simultaneously to the system assessment. Rather, this can be described as an identification of the triangle of public sector organizations that may be involved or whose interests overlap (different ministries or agencies), private sector organizations or firms (such as industry bodies, manufacturing concerns, key entrepreneurs or chambers of commerce) and universities. This may also include labour organizations if there is a large industrial presence in the context of the targeted sector in case proposed policies overlap or conflict with their interests.

Examples of cases where agreement was relatively wide spread occurred in Ireland, Sweden and North Carolina. The agreement in these three cases to pursue an innovation strategy which included biotechnology as a key sector was held relatively widely because there was a perception of economic crisis in each case. This perception of crisis meant that, despite possible different interest and motivation sets amongst stakeholders, there was a common denominator to their interest set (overlapping interest) which meant that they collectively prioritized dealing with the economic crisis by supporting measures to foster innovation. Understanding this phenomenon goes beyond standard economic rationality assumptions for actors, and being able to do so would help policymakers and innovation policy leaders to create consensus outside of a crisis scenario.

Briefly, according to political science theories of rationality, as well as organizational theory, different organizations have bounded rationalities which are determined by their own organizations history, learning capacity and environment. Individuals within one organization, while still rational, will not share the exact same concerns and values as individuals within another – these differences occur between firms in a field, and would be even more different between organizations such as a government ministry and a private firm. To individuals within these organizations, variables such as power, prestige, budget for activity, and profits will weigh, differently. For example, a business may consider a cut in operational budget as perfectly acceptable so long as an increase in profit follows; to a direct in a government department, a loss in budget will likely motivate them to oppose particular measures since profit does not enter into their personal gain, and in fact may be tied in to a loss in personal and departmental prestige. Operationally, therefore, a simple understanding of the different motivations of stakeholders is necessary to understand how different interests may be co-opted, or if that is not possible, overcome. Furthermore, the canvassing of potential partners for kicking off a targeted approach should be done as early as possible.

Besides these individual interests of different organizations, the different stages of a sector's development will have different effects on the ability to obtain agreement for kick-off at the beginning of the process. While it is more risky or entrepreneurial to kick off a targeted approach at an earlier stage of evolution, there will likely be more parties and interests at later stages with potentially more entrenched interests, which may lead to greater complications. With the stage of development in mind, it should be noted that in the cases discussed, it was mostly support for innovative sectors, not just biotechnology – in some cases biotechnology was not really a strength but a desire – perhaps not ideal to pursue. If so, how much willing is there to invest?

Also, in terms of operationalizing the kick-off agreement, what is the minimal achievement? What is the bare minimum ideal for coordination? It may be suggested that if a sufficient majority in any area can agree then this is enough agreement for at least three years initial budget commitment.

The TARGET approach presented here will help policymakers form a strategic roadmap and determine feasible interventions that lead to a functioning biotechnology system of innovation within a country or region. The goal of the toolkit is not to present a single recipe of specific policies for success; as will be noted later on, the variety of cases and their development mean that no single path to a functionally biotechnology sector can be realistically described.



© TARGET. All rights reserved.
TARGET – Targeted R&D Policy - is a project within the Seventh Programme and is co-funded by the European Union (Grant Agreement 234522). The project duration is July 1, 2009 – September 30, 2011.

This document has been produced with the financial assistance of the European Union. The contents of this document are the sole responsibility of the TARGET Consortium and can under no circumstances be regarded as reflecting the position of the European Union.