A framework for considering the role of the public sector in the Research & Development of a health technology

HPR75

David Epstein[1], Juan Carlos Rejon-Parrilla[*2], Jorge Mestre-Ferrandiz[3], Jaime Espin[4]

[1] University of Granada, Spain;
[2] Health Technology Assessment Area (AETSA) & Andalusian Public Foundation Progress and Health (FPS), Spain;
[3] Universidad Carlos III, Spain; [4] Andalusian School of Health (EASP), Spain
[*]Presenting author: juancarlos.rejon@juntadeandalucia.es

INTRODUCTION

- There are several estimates about the importance of the different sectors in the funding of R&D for new health technologies. Estimates of the amount of **public investment** in pharmaceutical R&D range between one and two thirds of the total investment. An argument often raised is that **the public pays twice**, once when governments invest in R&D and again when they purchase the new technology.
- In this study we aimed to produce a **theoretical framework to guide researchers and policy makers** on the potential **impact** (expected or unexpected) **of public sector policies**, decisions and incentives at distinct points on a product's life cycle **on future R&D**.
- The framework will be a **guide classifying the kinds of public investments that can be relevant to the design of public policies**, including P&R negotiations for health technologies.

METHODS

We have structured our framework in the 2 building blocks you can see here → (including sub-divisions A and B within block 2)

Building blocks informed with **ad-hoc literature searches (including peer reviewed and grey literature)**. We relied on the **knowledge of the co-authors** to identify the key sources to consider, together with the knowledge of a group of nine thought leaders that joined a **Scientific Steering Committee**.

1. Research, development and evidence generation over the product life cycle (see figure 1 below)
2. The multiple ways that the public sector influences health technology R&D. Mainly through the role of public sector institutions as Payers, Investors in R&D and Clinical & Market Regulators (see figure 2 below). But also via:
   - A. Research governance in public R&D and public-private collaboration
   - B. Fair pricing models

RESULTS

Figure 1. Research, development and evidence generation over the lifecycle of a medical device (blue) or medicine (green), alongside the potential roles of the public sector as payer, regulator or investor at distinct stages

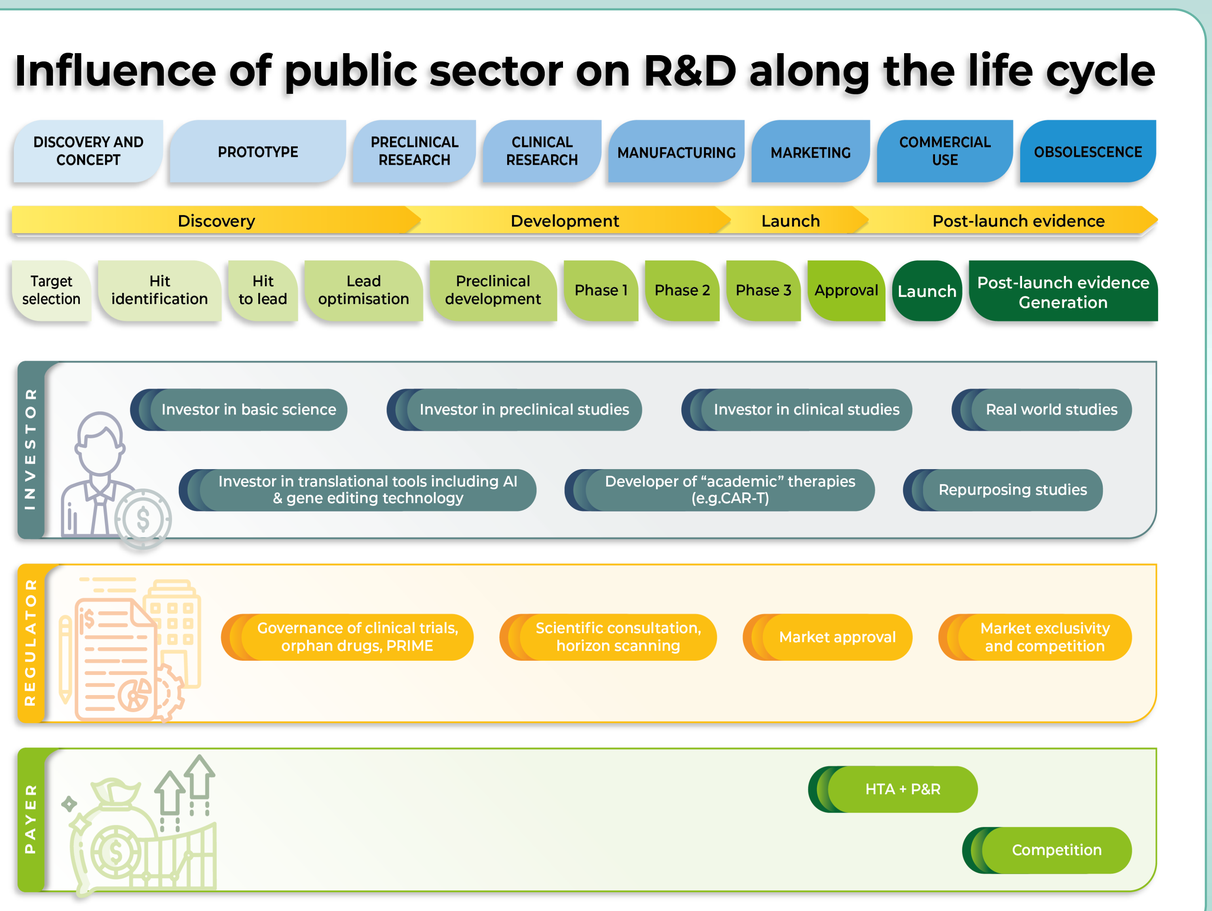
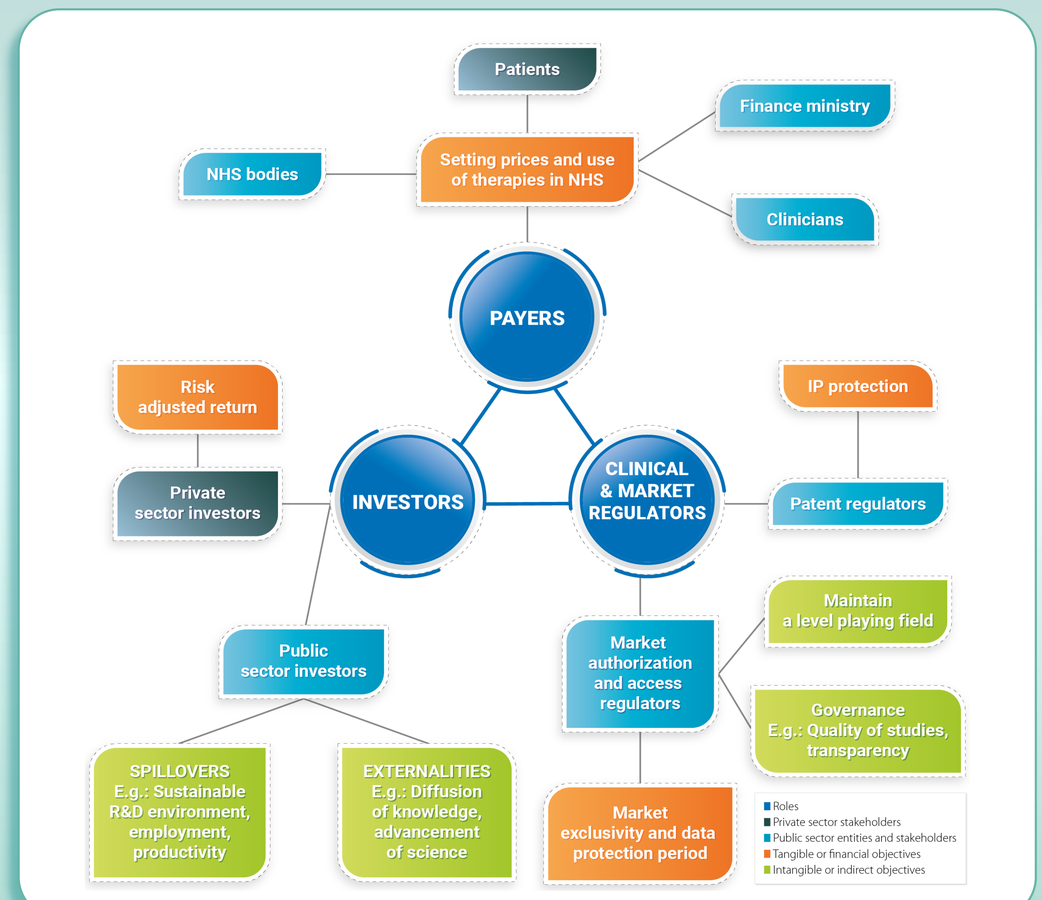


Figure 2. The three principal roles of public sector actors in the healthcare R&D environment: Payer, Investor and Regulator



Box 1. A case study to examines the economic arguments used to justify direct public sector support for development of multifunctional R&D tools (the Case of CRISP-R)

- Developed in 2012 by MIT researchers, CRISPR is a powerful gene-editing technology with applications in gene therapies, diagnostics, agriculture, and bioenergy.
- MIT granted exclusive therapeutic rights to a startup founded by the researchers, raising concerns about balancing universities' public-interest missions with commercial objectives.
- Limited access for smaller companies, due to exclusivity and complex patent disputes, highlights challenges in ensuring equitable distribution of groundbreaking technologies.
- The CRISPR case underscores the need for technology transfer models, such as public-private partnerships, that combine academic innovation with private investment to foster accessibility, scalability, and public benefit.

Box 2. An imaginary case study to showcase the potential distortions on market competition arising if actual prices deviate from Value Based Prices

- In "Innovia," a maximum price of €20,000 per patient is standard for any product generating 1 QALY. For "Helpmerecover," developed with €5,000 per patient in public funding, Innovia's health system proposes a reduced payment of €15,000 to reflect the public R&D contribution.
- This discount on Helpmerecover could set a lower price benchmark for future treatments in the same area. For instance, if "Cureme," offering 2 QALYs, enters the market, it would be capped at €35,000 instead of €40,000, diminishing incentives for innovation.
- A royalty model could avoid price distortions. If Helpmerecover were reimbursed at €20,000 with a €5,000 royalty to Innovia's public sector, Cureme could still receive full value-based pricing, sustaining incentives for future R&D.

Tentative conclusions

- Health R&D is not always linear; it can be iterative, influenced by innovations from other STEM fields and exploratory processes.
- Public institutions−acting as Payers, R&D investors, and Regulators−influence health R&D through varied and sometimes indirect roles.
- Coordinated public R&D strategies can better allocate resources, balancing incentives across areas to maximize public health benefits.
- Value-based pricing models can promote predictability and guide developers, avoiding inefficiencies associated with cost-plus or external reference pricing.
- Market deficiencies exist in areas like basic, translational, and real-world research, justifying strong public investment in high-impact technologies.
- Where the public sector conducts in-house development, such as in gene therapies, this investment should eventually result in a commercial activity if publicly owned (or co-owned) therapies might eventually be in competition with the private sector. The public sector has a duty as a market regulator to maintain a level playing field.

This project has received funding from the European Union's Horizon Europe research and innovation programme under **Grant Agreement nr 101095593**