The different contributions of the industry in public-private partnerships in Rare Diseases Research continuum

Executive Summary

Objective, scope and approach

A research project to identify and articulate the impact of pharmaceutical and biotech companies in Public Private Partnerships (PPPs) in rare diseases research, and beyond, took place during the first half of 2024.

The objective
The objective of this project was to shed light on different models of PPPs and the contributions industry can bring to the table. In the research we explored PPPs that are composed of stakeholders who work together collaboratively on a question or issue, rather than 'outsource' or 'buy in' expertise, and in such partnerships, companies, through their in-kind contributions, bring an intrinsic added value and help unlock or accelerate the search for solution. We were not analysing the added value or limitations of PPPs as these topics are explored in many other publications. Rather, the project focused on the specific attributes that industry brings to PPPs, without undermining the critical inputs from other partners such as academia, patient groups or public agencies in such collaborations.

The methodology
The project was commissioned by IRDiRC's Companies Constituent Committee (CCC)[1], funded by EFPIA, and led by Montsouris Consilium[2] and Millwater Partners[3], who were supported by several advisors and reviewers from IRDiRC's CCC and Rare Disease Moonshot[4]. A literature review and in-depth semi structured interviews with fifteen leaders involved in PPPs in the rare diseases area and other relevant PPPs (Annexes 1 and 2) identified key attributes and impact that the pharmaceutical industry brings to PPPs generally, and in rare diseases research specifically, which contribute to their success.

Main insights
PPPs are an ecosystem. In that ecosystem or '*community of brains'*, every partner plays a role. In addition to financial resources, **industry brings complementary skills sets, knowledge and competence** outlined in this report. Central to success is a mutual understanding of objectives and the 'end goal', and the importance of building and sustaining trust throughout the partnership.

> *"With PPPs, the members of the value chain come together at different stages and agree on a common language, on objectives and on the KPIs. If all works out, it means, ultimately an accelerated and enhanced value chain."* An interviewee from a patients' organisation

Collaboration can happen at various points along the entire R&D and healthcare continuum, both in the competitive and pre-competitive space. The partnerships explored in this research differ vastly in scope and size and represent **different forms of collaboration for different purposes**. *Pre-competitive collaborations* support structural change in a sector where

[1] https://irdirc.org/ccc/
[2] https://www.linkedin.com/in/mathieuboudes/
[3] https://www.linkedin.com/in/nicola-bedlington-23638218/
[4] https://www.rarediseasemoonshot.eu

companies can work together with their direct competitors, typically in consortia, creating new knowledge and approaches and the outputs are open. *Competitive PPPs* support process improvement for one player, to create proprietary assets, where companies would not work together with their direct competitors. These are most typically one to one collaborations.

Regardless of the nature of the PPPs, a number of common **key attributes are brought by companies** which were identified and validated in the research.

Snapshot of attributes

Scientific and regulatory expertise, access to infrastructure and assets

Companies contribute with their **scientific and technological expertise**. They can also provide access to **state-of-the-art research infrastructure and technology**, enabling high-quality, advanced research that might otherwise be unfeasible because of a lack of such resources among other partners. Some companies practise open science, **sharing data, assets and compounds** with individual researchers, which accelerates discovery and innovation. Their **understanding of the regulatory and policy landscape** in which partnerships operate allows for a 'whole picture', multisectoral and value chain approach, understanding and managing complexity effectively. Companies' **end-to end perspective** (from bench to market and patient), helps anticipate the type of data and evidence that is needed to move through different stages of the bench to beside relay run and help bridging certain "valleys of death" along the research continuum.

Operational capabilities and capacity

Companies' **global footprint** means they are able to facilitate **wider impact and scalability** by enabling easier access to relevant international and regional partners. As they operate in a highly regulated environment, companies have **stringent governance, transparency, and compliance measures** and contribute towards implementing these in the partnerships. Their **project management** skills, **risk management strategies**, and experience in **deployment in real life settings** support cost-effective implementation of co-developed solutions. This operational capacity helps foresee and mitigate risks, supporting smooth operations, and increasing the likelihood of successful outcomes.

Collaborative leadership and innovation drive

Because of the **diversity of profiles in their work forces**, who all need to work together, companies can demonstrate strong collaborative leadership that helps drive innovation. Due to a significant **internal focus on KPIs and timely delivery**, they are able to demonstrate a sense of urgency.

Patient-centred approach and research ecosystem strengthening

There is a long-standing alignment between the industry and regulators about the importance of patient engagement, patient evidence and patient voice in medicines development and evaluation process. Through various initiatives, companies have built experience and processes to move beyond tokenistic involvement to integrate **patient organisations as essential partners in R&D processes.** These approaches support patient organisations in contributing their unique expertise, particularly when other stakeholders may be less experienced in collaborating with patient organisations on systemic issues.

The impact of industry involvement in PPPs specifically in the area of rare diseases

In the rare disease space, because of the acknowledged unmet needs and the scarcity of resources, collaboration across stakeholders is even more important. When several global companies work together in rare disease research PPPs with academia and patient- centred research foundations or patient organisations, because of their footprint and reach, they can contribute to defragmenting the rare diseases research landscape. Industry involvement also helps to enrich PPPs with real-world data and case studies. Understanding and tackling practical challenges in healthcare delivery is essential, specifically in rare diseases.