



TOOL 5

Needs and priorities for Industry – and what does Industry need in a collaboration with ERNs?

Forging a successful collaboration entails all parties understanding and appreciating each other's needs, priorities and realities. The needs and priorities of ERNs, on the one hand, and Industry, on the other, are quite different, in many respects.

It is important for ERNs to understand the motivations, requirements and priorities for private sector organisations, especially perhaps for stakeholders (whether researchers, clinicians, patients, or otherwise) from disease communities which have not worked extensively with Industry to-date, and for whom, therefore, public-private collaborations are very new.

Several helpful resources exist to build awareness of the needs and priorities of private sector organisations in the context of public private partnerships or collaborations; and although not always specific to ERNs, the messages therein are useful for ERN stakeholders.

One essential message to convey is that Industry brings more to a partnership or collaboration than simply financial resources. [A dedicated webinar hosted by Together4RD and ERICA](#) (the European Rare Disease Research Coordination and Support Action) in early 2025 highlighted what industry can bring to partnerships and projects beyond funding, and provided attendees with concrete examples of how industry has brought value to research projects conducted with ERNs in recent years:

A useful resource for understanding some of the needs and realities of the private sector, specific to working in rare diseases (if not specific to working ERNs), is a 2021 report prepared for EFPIA (the European Federation of Pharmaceutical Industry Association). This report, entitled [‘Addressing unmet needs in extremely rare and paediatric-onset diseases: how the biopharmaceutical innovation model can help identify current issues and find potential solutions’](#) focuses on medicines' development, but is helpful in conveying the complexity and risk for companies working in this area.

- For instance, it explains how Industry is central to medicines' development, and

illustrates the high costs of drug development, the timescales involved, and the high failure rates – all of which made developing medicines a very risky enterprise, from an investment point of view, not least in areas such a rare disease which carry particular challenges of their own. “When making investment decisions, companies first consider the scientific opportunity, then examine commercial viability within the policy environment”. Key aspects of the decision-making process are explained.

- The report summarises some of the additional challenges of seeking to develop medicines in extremely rare diseases, knowing that 80% of all rare disease patients are affected by one of the approximately 150 diseases with the highest prevalence, but that 84.5% of the conditions classed as rare have a prevalence of lower than 1 in a million and affect only 0.33-0.55% of all people living with a rare disease. It explains why, as a rule of thumb, the rarer the disease, the more significant the scientific and commercial challenges inherent to rare disease therapeutic development.

[The report is available here.](#)

The International Rare Disease Research Consortium, IRDiRC, has recently released a [report](#) which is extremely valuable in helping to convey the benefits which Industry can bring to a collaboration (and, in connection with this, illustrating the needs and priorities of the private sector).

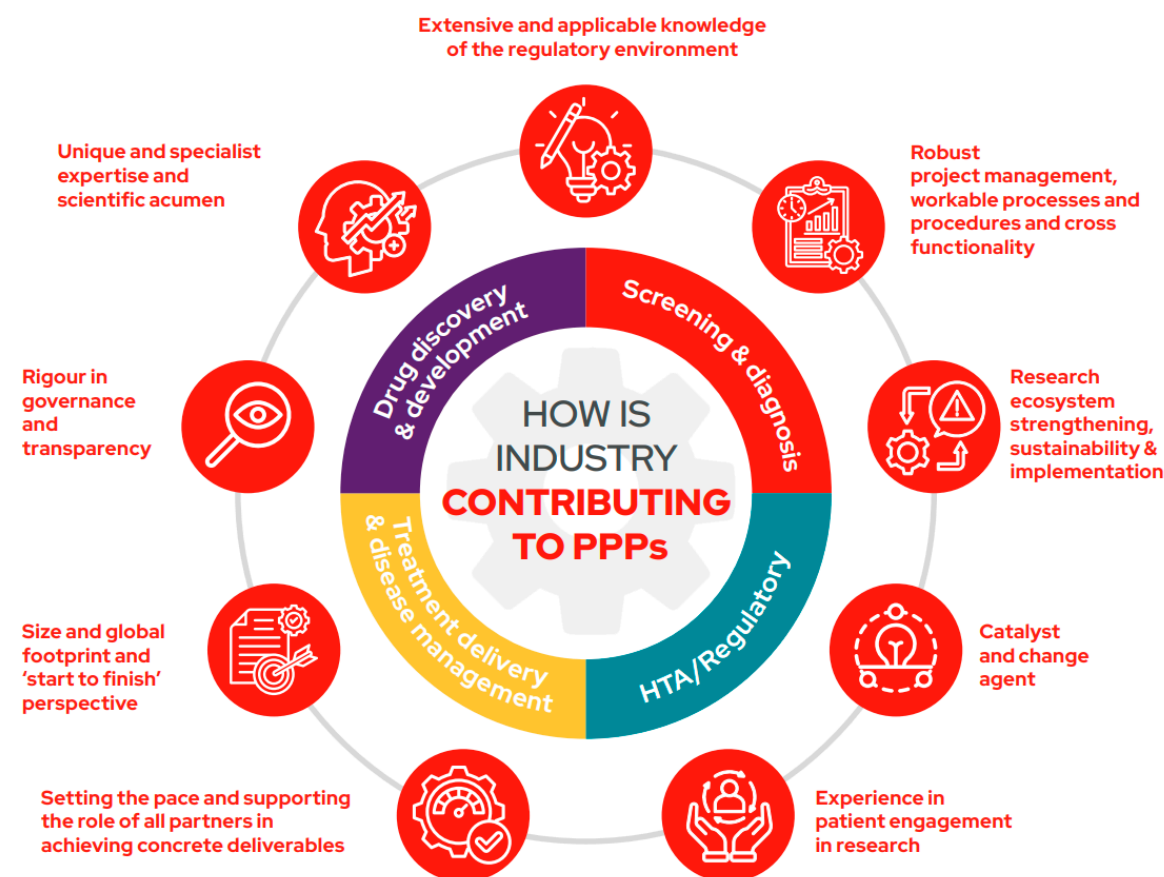
This report [‘The different contributions of the industry in Public-Private Partnerships in Rare Diseases Research continuum’](#) stems from a collaboration between EFPIA, the Rare Disease Moonshot, and the IRDiRC Companies Constituent Committee. It highlights “the unique value pharmaceutical and biotech companies bring to Public-Private Partnerships”, providing four key takeaway messages:

- **Scientific & Regulatory Expertise**
Companies provide cutting-edge infrastructure, data, technical know-how, and a deep understanding of regulatory pathways.
- **Operational Capacity**
With a global footprint and strong project management skills, industry partners help scale innovations, manage risks, and drive efficient implementation.
- **Collaborative Leadership & Patient-Centric Approach**
Industry actors know how to meaningfully engage patient organizations early in the

R&D process, supporting co-creation of impactful solutions.

○ A Strategic Role in Rare Diseases

In a fragmented and resource-scarce field, industry involvement helps connect the dots, enrich efforts with real-world data, and accelerate impact.



Last but not least, **other tools within this Together4RD Toolkit serve to illustrate the needs and priorities of the private sector**, when considering or embarking on public private collaborations or partnerships with ERNs; in particular, Tool 9 '[Report on the Experiences and Learnings from the first ERN-Industry pilots supported by Together4RD](#)' and Tool 10 '[Key recommendations for both ERNs and Industry from the experiences of the first ERN-Industry pilots](#)'. The latter includes recommendations to support an effective partnership, and there are several layers to this; however, some of the points in this document relate specifically to the need for non-Industry partners to understand certain parameters and realities for private sector partners, such as the following:

- Understand that public and private actors tend to place value on different sorts of outputs.
- Consider how research can result in wider impact, beyond publications, in terms of changing patient pathways and diagnostics practice, and outcomes beyond publications.
- Acknowledge that companies in the rare disease space may have goals and vision to improve the wider rare disease ecosystem, beyond simply developing and selling a product.
- Accept that industry partner(s) will wish to have input to the scientific development of a project plan and should be viewed as an equal partner. Indeed, this should be welcomed, as it will bring significant advantages to the research, as it will bring access to the vast in-house scientific expertise but also expertise in medicines development, HTA, data science, and much more.
- Have realistic expectations of both the level of resources industry can contribute to projects, and the way in which it does this. Avoid thinking of companies as purely funders of research. Companies are generally unable to dedicate large sums of money, for the subsequent definition of a detailed project plan – in fact it is the reverse: funding can only be found, internally, based on the contents of a proposed plan. Companies do not award funds as unrestricted grants, without any direct involvement.



Fig 1 – Graphic to accompany '[The different contributions of the industry in Public-Private Partnerships in Rare Diseases Research continuum](#)' report, taken from the launch page for this report.



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