ERA-LEARN

Translating research into innovation:

Lessons from 3 case studies in health partnerships

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Executive Summary

The aim of this policy brief is to establish a methodological approach to understand translation of research into innovative outcomes. We selected this theme as a result of our previous research into impact assessment (Deliverable 3.2, 2020) which revealed a noticeable disconnect between the research effort of the partnerships and their projects and innovation outcomes.

Studies on knowledge transfer and circulation within a system of relations are often considered as a stage-gate process: knowledge is generated, new technologies are devised and tested and then applied in a business or social setting. From the stage-gate perspective, we often come across the idea of "the valley of death" (Frank et al, 1996; Hudson & Khazragui, 2013; Seyhan, 2019) that is the critical phase where one-step of the innovation process ends and a new one begins. However, health research and innovation is a process of cumulative and distributed learning spanning different communities – biomolecular scientists, clinical and medical researchers, technologists and entrepreneurs, large corporations, regulators and patients (Metcalfe et al, 2005; Consoli & Mina, 2009; Calza et al, 2020, Xu & Gagliardi, 2023).

In this perspective, one of the roles of partnerships is to bridge this 'valley of death' by linking excellent research with technology and solution-minded business undertakings, hence generating virtuous cycles of research and innovation. The role of the partnerships goes beyond the resources and incentives approach and focuses on opportunities for knowledge co-creation and diffusion as well as on entrepreneurial venture.

We decided to focus our study on health-related partnerships, as translational research has been a main concern of medical research and innovation since at least the 1970s and by the 1990s, translational medicine had become an established discipline[1]. Translational medicine is a branch of science looking at how knowledge generated in the lab (either a university, a government or a company laboratory) makes it to the bedside for the benefit of patients.

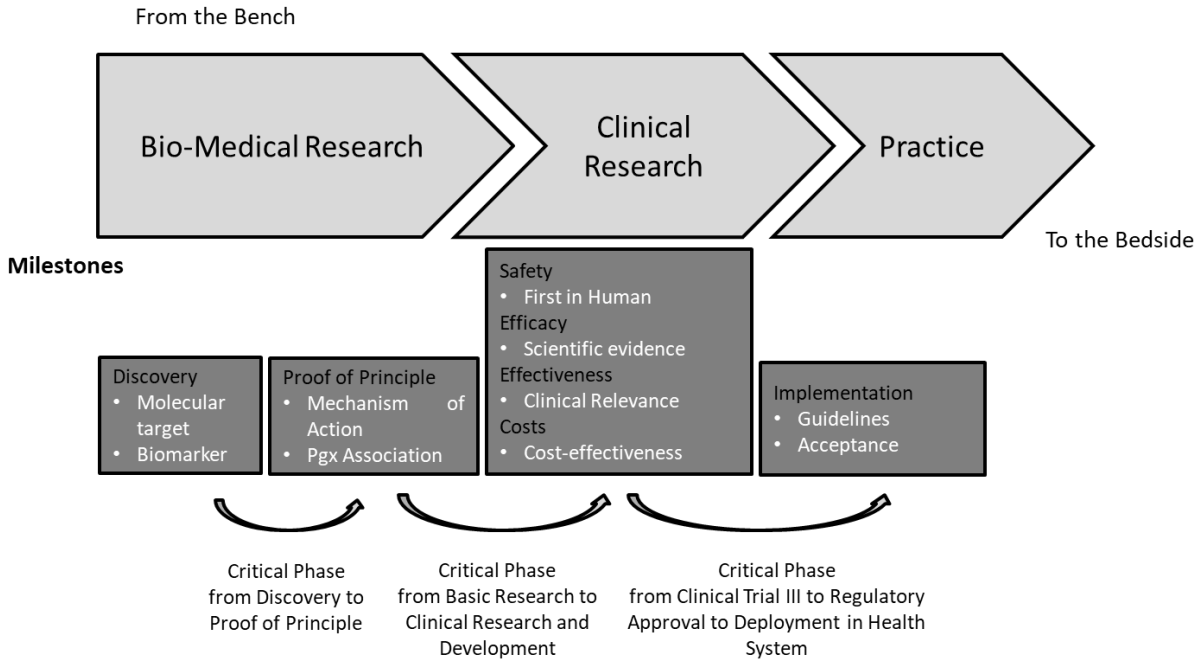
Approaches to translating research into innovation show that different types of networking and network structures link to innovation outcomes. The mechanisms at the basis of successful translation rely on the production of knowledge, experimentation and applications by multiple parties working collaboratively. Moreover, how such cooperation is organised to bridge the divide

[1] The idea of applying the principles of science translation into sectors other than medicine and biology is not satisfactorily developed. A secondary scope of this report is to develop the methodology for investigating the "problem of translating research results into impactful innovation". The methodology will be validated and further research undertaken in future ERA-LEARN activities and in ERA-LEARN next steps.

amongst the various collaborators has a significant effect on the innovation outcome (Molas-Gallart et al, 2016). Translational studies point out that many different competences and capabilities activate throughout the journey. These rely on scientific, clinical and other technological infrastructures to navigate such journeys.

We have adopted the framework described in *Figure i* in order to identify activities carried out within partnerships and their projects and to highlight critical junctures where collaborations, infrastructures, specific competences and skills become crucial to progress from the bench to the bedside.

Figure i. The translational research continuum with milestones and critical phases



Source: adapted from Gagliardi et al, 2018; p. 224; Seyhan, 2019; p. 3

We selected three partnership representatives of ERA-NET (TRANSCAN), European Joint Programme (EJP on Rare Diseases) and a joint undertaking (the Innovative Health Initiative), in order to investigate how partnerships navigate the process of translating research activities into medical and health innovations for the benefit of patients, the European health systems and society. Thirty-four semi-structured interviews were conducted in total and the research team spoke with over forty informants. We proceeded in our analysis based on the eight elements we investigated during our fieldwork. These include: 1) objectives and rationales, 2) the types of collaboration agreements they have with other stakeholders of the European health system, 3) their relations with the network of infrastructure available to research and innovation projects, 4) specialist and technical knowledge necessary for their projects' operations, 5) training activities and capacity building initiatives, 6) their approach to bringing the research results and milestones of projects to the market or their final users, 7) how the partnerships and their projects define and evaluate success and finally, 8) the critical factors in achieving such success.

Summary description of the three partnerships

TRANSCAN is an ERA-NET Co-funded partnership operating in its third round of ERA-NET funding. Each funding cycle of the partnership is of 5 years and in the current embodiment, it is set to operate between March 2021 and February 2026. The current overall budget of the partnership is of €34m. It includes 34 partners comprising of European Member States' ministries, national and regional research funding agencies, charitable organisations and partners from associated EU countries and other countries (Norway, Canada, Israel, Turkey and Taiwan). Its objectives are to pool strategic cancer research and innovation resources from regional and national - public and private institutions in order to align and sustain collaborative cancer research programmes. It does so through the launch and implementation of joint transnational research calls (JTCs) in areas of cancer research and innovation where critical/funding gaps are identified. Projects funded through this instrument are also involved in important training and capacity building activities.

The **European Joint Programme on Rare Diseases** is a Co-funded partnership, which, in its present form, is in operation since January 2019 with an end date expected of December 2023. Following three rounds of funding under ERA-NET as e-Rare, e-Rare 2 and 3, EJP RD now comprises 130 partners including research funders, scientific institutes and universities, European infrastructure, hospital and patient organisations spanning across 35 countries. The main aim of the partnership is to create a research and innovation ecosystem active in the rare diseases areas. Given the heterogeneous nature of rare diseases - characterised by the large number of diseases each affecting few patients -, the objectives of the partnership centres around the criticalities of the research and innovation process: to improve on the integration and effectiveness of research and development in the rare diseases areas by promoting multinational collaborations and to implement more efficient financial support methods for research and innovation activities and for the exploitation of research results.

The **Innovative Health Initiative** (IHI) is a joint undertaking under Horizon Europe - an Institutionalised European Partnership that began operations in 2021. It follows its predecessors, IMI and IMI2, which have been in operation since 2008. IMI/IHI is a public-private partnership whose partners are the European Union (Represented by the EC) and the European health industry embodied by its trade associations: the European Trade Association representing the medical imaging, radiotherapy, health ICT and electromedical industries (COCIR); the European Federation of Pharmaceutical Industries and Associations (EFPIA) – including Vaccines Europe; the European Association for Bioindustries (EuropaBio) and the European trade association for the medical technology industry including diagnostics, medical devices and digital health (MedTech Europe). The partnership operates in the pre-competitive health research and innovation area involving all healthcare stakeholders (patients, academia, healthcare professionals, healthcare delivery organisations, regulators, and pharmaceutical, medical technology and digital health companies). It is involved in the entire continuum of care from prevention, diagnostic, to treatment and disease management. IMI/IHI has three strategic and interconnected objectives: 1) to create a European-wide health ecosystem for the translation of research knowledge into health innovation; 2) to foster the development of medical and health innovations to respond to the strategic and unmet needs of the European health system; 3) to

drive the health ecosystem across the sectors involved for a European health industry which is competitive at the global level.

Pathways to impact

The three partnerships each have a very different approach to translating research into innovation.

TRANSCAN operate as a traditional research funder and positions itself at the frontier between biomedical/molecular research and clinical research in oncology. Its objective is to bridge the gap between proof of concepts, which usually happens in basic research laboratories and universities, led by basic scientists, and early clinical applications, which usually occurs in university and research hospitals, led by clinical scientists. These two domains have traditionally been unconnected, their work happens in independent silos and research and applications progress under different institutional logics and according to different norms. In this space, TRANSCAN provides a bridge through the valley of death connecting basic biomedical research and clinical research. It does so by sponsoring research and innovation projects spanning the two domains. This approach, whilst justified also by the limited budget of the partnership, revealed very effective in connecting the two worlds of basic and clinical research and, at the same time, has consistently provided resources and support for 'first in human' trials. During the course of the partnership, there have been several highly valued innovations that have made their way to patients. These are mostly clinical and therapeutic applications including diagnostics currently used within the health system to assist physicians in the care of children (in paediatric oncology) and that are now deployed at the bedside throughout Europe, in Israel and in North America.

The aim of EJP RD is to coalesce rare diseases stakeholders towards collaborating within a European-wide rare diseases ecosystem. This is progressing by reaching out and fostering collaborative undertakings across research centres, clinical research hospitals, patients' advocacy groups and charities, national and European regulators and, to a lighter extent, industrial partners engaged in rare diseases. The partnership, in its endeavour to extend and consolidate such European-wide ecosystem, provides a wide range of services channelled through infrastructures associated with the European Strategy Forum of Research Infrastructures and developed in house through the wealth of expertise available through its partners. This strategy is coherent with the nature of the problem that the partnership is facing: rare diseases are heterogeneous, there are over 6,000 rare conditions affecting a small number individuals distributed across regions and countries. For these reasons, the approach to rare diseases research and innovation has to be necessarily one of public health that transcends national borders and national health systems of innovation. By including over 130 institutions across 35 countries, the partnership is making serious inroads towards 1) aligning the European and international response to rare diseases, 2) coalescing the expertise, capabilities and competences of the R&I communities working on rare diseases and 3) provide a public health policy response by improving systems' capacity to deal with rare diseases. The EJP RD has become a European and international reference point for the rare diseases communities acting as hub for knowledge generation and exchange, research and innovation support services and,

more generally, providing an international platform for research and innovation in this medical and health area.

IMI/IHI has the ambitious objective of promoting a comprehensive approach to healthcare in Europe. It does so by operating in the pre-competitive space bringing together public institutions, industry, third sector actors working in health, universities, research hospitals, SMEs, patients and their advocacy groups, regulators and other stakeholders. With an overall budget to match its ambition[2], the partnership operates on several fronts: 1) from basic and applied research in critical medical and health areas 2) to supporting and driving the formation of a robust network of infrastructure and 3) leveraging synergies of its partners to accelerate the European health system's response to medical emergencies and longstanding strategic aims in public health policy. Such an approach is carried out through its governance and its projects that are the result of a multi-stakeholder partnership including public institutions and private partners. For example, EFPIA and other partners' companies, which are part of the IMI/IHI partnership, provide in kind and/or cash contributions to projects. This assures to a greater extent that excellent basic and clinical research, evidenced by the quantity and quality of the scientific output, is matched by the drive to bring innovation to market and at the bedside. The partnership's reach also extends to those system-making and enhancing connections established amongst the stakeholders (public institutions, industry, third sector etc.) and functional resources (i.e., infrastructure, clinical trials, regulatory and business capabilities) enabling excellence within the European health innovation system. The stakeholder base is engaged in almost all aspects of the health system and through its activities the partnership can capitalise on its position within the ecosystem and operate 1) as research and innovation catalyst and 2) as a key health policy partner to EU institutions.

Conclusions

The variety of partnerships in the European medical and health system is a valuable asset for policy and for the health sector research and innovation. From a policy perspective, these partnerships provide invaluable input in the policy process as they are directly involved by providing a rich policy learning experience and an extended network of relations amongst the stakeholders, including public institutions, the business community, the third sector and the medical and health communities who have reach across the EU Member States. They are also the players enacting EU and national health policy strategies at the most granular level spanning from actively responding to high-level health policy objectives and research and innovation objectives of the health system. Partnerships boast excellent research competences and increasingly, they have demonstrated capabilities to enabling functional channels to market and to patients for the benefits of patients and the health systems.

Unpacking the diversity of the partnerships, the depth and breadth of activities are such that transnational research and innovation in key areas is reaching high standards of excellence in

[2] The overall budget of the partnership amounts to €2.4B. A half of its funding comes from the European Union and the other half from the private partners including large companies, charities and foundations operating in the medical and health sectors.

tackling specific critical phases of the translational continuum. These partnerships, for example, have also the remit to build active and sustainable ecosystems within their areas of expertise facilitating wider uptake of scientific knowledge as well as providing an invaluable platform for the development of new experimental methodologies and evidence-based advancement of transnational health policy. Finally, they are well positioned to foster their ambitions of integrating and enhancing capacity and capabilities of a European truly transnational health system. They can do so through their system-building operations by bringing to the table stakeholders from various backgrounds, moved by specific incentives and working towards different agenda by focusing their activities and investments on shared objectives responding to health policy targets and research and innovation goals established in their Strategic Research and Innovation Agendas. The partnerships contribute significantly to the sustainability of the European health innovation system through training and capacity building programmes investing in the next generation of researchers, clinicians and health entrepreneurs, by funding PhD and Post-doctoral positions and promoting cross-organisation exchanges.

A policy challenge is that of capturing the potential synergies generated within the complex system of health-related partnerships. In fact, we have seen that relatively small projects, even when extremely successful, may find difficulties in accessing competences and resources to further advance their innovations towards the bedside. Often, for these beneficiaries, the way over such hurdles consists in bootstrapping, repeat applications for funding and in a minor capacity, in searching a way to market through patenting and licencing, spin-offs and collaborations with other established firms. From our interviews it emerged that a small number of very successful research groups had ongoing complementary projects funded by different partnerships (and other sources) and that this opportunity helped them enormously in progressing in their basic and clinical research. Through capitalising on these synergies, they gained better access to knowledge and resources to bring their findings and milestones to clinical trials enabling further pathways to impact involving commercial partners. On the other hand, partnerships focusing on ecosystem-building activities may have capacity and capabilities to foster larger projects with diverse sets of beneficiaries who contribute to research activities as well as advancing potential innovation to the bedside more effectively.

The emerging policy challenge is to encourage the development of connections and links between partnerships in order to exploit the potential synergies that are being established within the European research and innovation health system. The objective should be that of creating a nurturing environment for potentially innovative projects to thrive and develop; whilst such an objective may transcend the boundaries of a single partnership, it is necessary that the health sector act collaboratively.

1. Background and methodology

The aim of this policy brief consists in setting up a methodological approach to understanding translation of research into innovative outcomes. This theme was chosen on the back of our previous research into impact assessment (Deliverable 3.2, 2020) whereby the researchers reported a noticeable disconnect between the research effort of the partnerships and their projects and innovation outcomes. This mirrored the appeal of COM (2020) 628 Final (30/09/2020), which reads: "*Europe is also lagging behind in translating R&I results into the economy. Although Europe is a world leader in some high tech sectors ..., efforts need to be channelled towards strengthening industrial innovation, technology transfer and fostering the uptake of R&I solutions and the diffusion of innovation through knowledge transfer and public-private cooperation*" (page 3).

Studies on knowledge transfer and circulation within a system of relations are often considered as a stage-gate process: knowledge is generated, new technologies are devised and tested and then applied in a business or social setting. From the stage-gate perspective, we often come across the idea of "the valley of death" (Frank et al, 1996; Hudson & Khazragui, 2013; Seyhan, 2019) that is, the critical phase where one step of the innovation process ends and a new one begins. This phase is critical since progressing from one stage to the next requires relevant investments, different capabilities and re-direction of objectives. Whilst from a mere linear perspective this process is somewhat logical - the impact from knowledge generation and transfer is treated as a problem of resources and incentives - health research and innovation may be viewed as a process of cumulative and distributed learning spanning different communities – biomolecular scientists, clinical and medical researchers, technologists and entrepreneurs, and large corporations (Metcalfe et al, 2005; Consoli & Mina, 2009; Calza et al, 2020, Xu & Gagliardi, 2023).

In this perspective, one of the roles of partnerships is to bridge this valley by linking excellent research with technology and solution-minded business undertakings, hence generating virtuous cycles of research and innovation. At the same time, even a cursory reading of the strategic research and innovation agendas (SRIAs) of partnerships conveys the idea that their missions and activities go beyond the resources and incentives rationale of a stage-gate approach and focus on opportunities for knowledge co-creation and diffusion as well as on entrepreneurial venture.

We selected to focus our study on health-related partnership since translational research has been a main concern of medical research and innovation since at least the 1970s and by the 1990s, translational medicine has become an established discipline[3]. Translational medicine is a

[3] The idea of applying the principles of science translation into sectors other than medicine and biology is not satisfactorily developed. A secondary scope of this report is to develop the methodology for investigating the "problem of translating

branch of science looking at how knowledge generated in the lab (either a university, a government or a company laboratory) makes it to the bedside for the benefit of patients.

Translational Research refers to the "*effective translation of the new knowledge, mechanisms, and techniques generated by advances in basic science research into new approaches for prevention, diagnosis, and treatment of disease […] essential for improving health*." (Fontanarosa & De Angelis, 2002, p. 1728).

Translational research may be seen as the "*bench-to-bedside enterprise of harnessing knowledge from basic sciences to produce new drugs, devices, and treatment options for patients*" (Woolf, 2008, p211). This particular view aims at producing promising new treatments that can be used in the clinic or "brought to market". From the care logic perspective of health service researchers, public health managers and policy makers, translational research means assuring that new treatment options for patients are actually implemented in the practice and used for the benefit of patients, population and the health system (Woolf, 2008).

Approaches to translating research into innovation show that different types of networking and network structures are linked to innovation outcomes. The mechanisms at the basis of successful translation rely on the production of knowledge, experimentation and applications by multiple parties working collaboratively. Moreover, how such cooperation is organised to bridge the divide amongst the various collaborators has a significant effect on the outcome (Molas-Gallart et al, 2016). Translational studies point out that many different competences and capabilities are activated throughout the journey. These rely on scientific, clinical and other technological infrastructures to navigate such journeys. The message that clinical and translational researchers need to work together, collaborating across nations, industries and community partners in interdisciplinary teams to move discoveries to practice (Zerhouni, 2007) is not new and it constantly re-emerges in the literature (Gohar et al. 2019).

We recognise that the translation journey is fraught with uncertainty and cannot be easily deconstructed in practice. However, we use the translational continuum (a stage-gate representation of the innovation process) as a 'tool' or 'shorthand' to abridge the journey from basic research - including discovery and proof of principle - to clinical research including validation, drug discovery and clinical trials (CT 1, 2 & 3) and approval (i.e., FDA, EMA). We also include other activities facilitating the use of products and services in health care (guidelines, payment structure, physicians' training and patients' acceptance). A recent publication by Seyhan (2019) applies the concept of a translational continuum by highlighting where, in such a continuum, critical phases occur as challenges of translational research.

We are aware that this represents a broad simplification of the complex relationship in place within a translational journey. Nonetheless, we have adopted this framework, see Figure 1 below, in order to provide our interlocutors with a familiar map of the discussion. This way it is easy to

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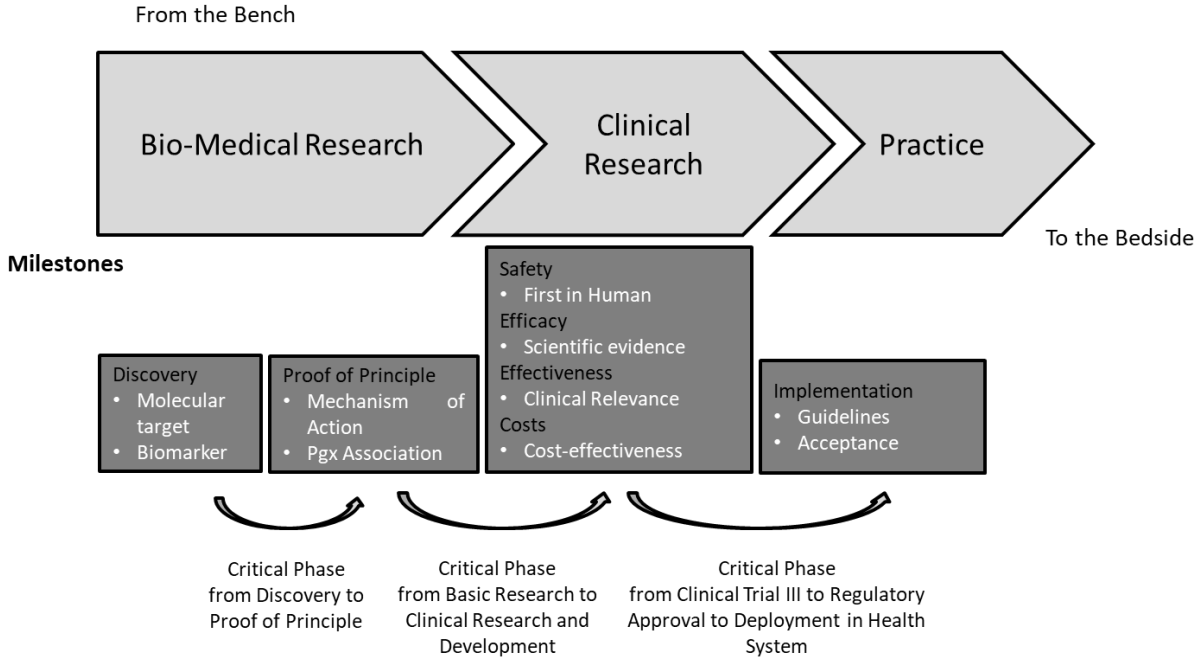
research results into impactful innovation". The methodology will be validated and further research undertaken in future ERA-LEARN activities.

identify activities carried out within partnerships and their projects and highlight critical junctures where collaborations, infrastructures, specific competences and skills become crucial to progress from the bench to the bedside. At the same time, reporting from their experience, our informants could articulate the complexities of their tasks and activities.

Figure 1. The translational research continuum with milestones and critical phases



Source: adapted from Gagliardi et al, 2018; p. 224; Seyhan, 2019; p. 3

Methodology

In this study we focused on the translational activities of three health-related partnerships: 1) ERA-NET Sustained collaboration of national and regional programmes in cancer research (TRANSCAN), The European Joint Programme on Rare Diseases (EJP RD) and the Joint Undertaking, the Innovative Medicines Initiative (IMI) now Innovative Health Initiative (IHI).

The phases of this explorative study consist in the design, validation and roll out of qualitative research on how health-related partnerships manage their translational research. This research was organised in four stages:

Stage 1): collect and collate information on the health-related partnerships, their organisation and governance and their involvement in translational activities in order to inform the selection of three case studies. This phase included desk research through the ERA-LEARN database of Partnerships[4], informal discussions within the ERA-LEARN consortium and with the EC and interviews with partnerships in order to set the parameters of the study. The objective of this stage was to identify what specific factors to investigate, the suitable partnerships to include in our investigation (i.e., capture the translational research rationales of health-related partnerships with

[4] https://www.era-learn.eu/network-information/networks

different backgrounds, stakeholders and governance systems) and set out the level of analysis (i.e., partnership level and project level study).

Stage 2) involved discussions with experts within the partnerships' organisations in order to validate our study plan and understand the institutional factors underpinning their translational activities. These discussions served to confirm the appropriateness of our choice, set out the interview protocol and secure collaboration from the partnerships.

In **Stage 3)** we held three interviews with the partnerships selected, each partnership was represented by with two or more people with specific knowledge on the various aspects of their translational activities.

Stage 4) consisted in holding interviews with projects stakeholders. These included projects coordinators, principal investigators (PIs) and researchers. The interviewees were representative of basic research organisations including research centres and universities, clinical scientists from clinical research centres and research hospitals, company R&D managers and performers from large pharmaceutical and biopharmaceutical corporations, and small and medium sized biopharmaceuticals, digital health companies and med-tech companies.

Thirty four semi-structured interviews were held in total and the research team spoke with over forty informants.

This report is based on the findings of the responses to our interviews held in 2021/2022 and completed by the end of August 2022.

Table 1. Case studies and interviews

IMI/IHI (14 interviews)		**EJP RD (11 interviews)**	**TRANSCAN (9 interviews)**
Partnership level investigation		Partnership level investigation including the RD Research Challenges	Partnership level investigation
Private partners: Large companies SMEs	Project PIs, Research and Clinical Partners	Project PIs, Research and Clinical Partners	Project PIs, Research and Clinical Partners

Interviews covered the main aspects of the translational journey and for each interview we have identified: 1) the stage of translational continuum in which the partnership/project operates; 2) with whom and to what extent they collaborate with other stakeholders within the partnerships/projects and externals; 3) the provision of translational research infrastructures by partnerships and the modes of accessing infrastructure's services by projects; 4) the access to specialist and technical knowledge by partnerships and their projects; 5) the training and capability/capacity building activities; 6) way(s) to market/end user of the research, development

and innovation outcomes of partnerships and their projects. Here we elicited their views, observations and considerations of their strategic approaches to long-term impact.

The interviews concluded with questions upon 7) interviewees' definition of success (of the partnerships and/or the project) and 8) on the factors which may drive such success. We have not used the classical approach of "drivers and barriers" but designed the study in order to understand the translational effort of the partnerships and their projects through an inductive approach connecting partnerships and projects' aspirations to their research, development and innovation outcomes.

Finally, we left a safe space for the interviewees to comment on the interview process, the themes treated and the approach of the study.

In the annex (Annex I), we include the interview protocol.

2. Case studies

2.1 ERA-NET – TRANSCAN (https://transcan.eu/)

Aims and Background

TRANSCAN began its operation as ERA-NET on Translational Cancer Research funded by the EC under the 7th Framework Programme in 2011. The original network comprised 25 founding partners from 19 countries (including 3 associated countries: Israel, Norway and Turkey) joined by three foundations (28 partners in total). Partners are national ministries, health agencies, research funding agencies, including research councils and cancer research not-for-profit foundations actively engaged in funding and supporting cancer research. The main aim of the early partnership was "*the integration of basic, clinical and epidemiological cancer research and facilitation of coordinated, transnational cancer funding in Europe with the ultimate aim to streamline EU-wide cancer screening, early diagnosis, prognosis, treatment and care*"[5].

TRANSCAN launched three Joint Transnational Calls:

1. JTC 2011 on: "Validation of biomarkers for personalised cancer medicine", 10 projects funded
2. JTC 2012 on: "Translational research on primary and secondary prevention of cancer", 10 projects funded
3. JTC 2013 on: "Translational research on tertiary prevention in cancer patients", 10 projects funded

The Research Budget of TRANSCAN (2011-2013) was €33 mil[6].

ERA-NET TRANSCAN-2 ALIGNING NATIONAL/REGIONAL TRANSLATIONAL CANCER RESEARCH PROGRAMMES AND ACTIVITIES followed on the first ERA-NET from 2015 to 2019. TRANSCAN-2 aimed at "*deepening and extending the cooperation among partners through exchange of information, harmonisation of funding mechanisms and assessment of results of the funded research projects, facilitating the transnational cancer funding in Europe and thus contributing to the building of the European Research Area*"[7]. The network extended to comprise 31 partners from 15 Member States, 3 Associate Countries and 1 third Country (Taiwan).

[5] https://www.transcanfp7.eu/index.php/pages/transcan-objectives.html

[6] Data from transcan.eu/output-results/fact-and-figures/

[7] https://www.transcanfp7.eu/index.php/partners/transcan-2-partners.html

The partnership launched in total about 50 projects in 4 Joint Transnational Calls:

1. JTC 2014 (co-funded by the European Commission) on: "Translational research on human tumour heterogeneity to overcome recurrence and resistance to therapy", 16 projects funded
2. JTC 2015 on: “Immunology and immunotherapy of cancer: strengthening the translational aspects”, 7 projects funded
3. JTC 2016 on: "Minimally and non-invasive methods for early detection and/or progression of cancer", 14 projects funded
4. JTC 2017 on "Translational research on rare cancers". 12 projects funded

The research Budget of TRANSCAN-2 was of €52.2mil[8].

The current partnership, ERA-NET: SUSTAINED COLLABORATION OF NATIONAL AND REGIONAL PROGRAMMES IN CANCER RESEARCH TRANSCAN-3 conserves the structure of the previous embodiment; it was launched in 2021 and is due to conclude in 2026. Its aim is to “*provide influential contributions as well as a sustainable model of funding for ground-breaking translational cancer research in Europe and beyond*”[9]. The partnership has foreseen the launch of at least 4 JTC.

JTC 2021 on "Next generation cancer immunotherapy: targeting the tumour microenvironment" closed in June 2021

JTC 2022 on “Novel translational approaches to tackle the challenges of hard-to-treat cancers from early diagnosis to therapy” opened in May 2022

Objectives and rationale

Based on our interviews, the objective of TRANSCAN is to foster links between basic biomolecular research and the clinic. This translates into the practical steps of funding translational research in cancer research. Therefore, research funded by the ERA-NET partnership merges basic research and pre-clinical research and extends to early clinical research. The minimum requirement of a TRANSCAN funded project is that proposals should integrate the work of basic researchers and clinicians and that the objective of the funded undertaking consists in translational research from basic research to clinical applications. Whether translation can be fully achieved through this simple structure is to be seen; however, by including these two categories of researchers within a structured process provides robust foundations for translation. In fact, whilst focusing on basic biomolecular research and pre-clinical studies, TRANSCAN moves the research focus towards including animal models with ‘first in human’ objectives positioning these within reach of the scientists and clinicians.

In other words, these eligibility criteria or requirements are thought out to foster the process of building relationships between scientists and clinicians and ultimately involve the latter directly in

[8] Data from transcan.eu/output-results/fact-and-figures/

[9] https://transcan.eu/the-project/at-a-glance/at-glance.kl

the research process, therefore, bridging the 'valley of death' between basic research and applied research. This is considered particularly important, as clinicians constitute the end users of basic research. Clinicians are committed to clinical practice since they provide care to patients. This last aspect is particularly important in paediatric oncology where children are usually taken into care through phase III clinical trials.

Collaborations

From our interviews, it emerges that TRANSCAN sees as its main end user the clinical research community. However, there is a clear awareness within the partnership and amongst the projects' Principal Investigators (PI) and researchers (both biologists and clinical researchers) that industry, regulators and patients (including charities upholding patients' interests and research funders) are part of the landscape and contribute important resources to the partnership, to the projects and the translation process in general. In fact, since TRANSCAN's inception, cancer organisations such as Cancer Research UK and, from 2014 onward, other charitable organisations such as the French Foundation for Cancer Research, the Norwegian Cancer Society and the Dutch Cancer Society, have joined the list of partners. At the same time, important stakeholders such as industry are not included as they are seen as outside the scope of the partnership, which is to bridge basic research with clinical research rather than fostering the whole process of research and innovation. Interestingly, the partnership recognises the role of the regulator, which stands at the further end of the translational continuum in which TRANSCAN operates and that is of particular importance in the field of paediatric oncology and rare cancers. Whilst there is no direct involvement of regulators in the governance and management of the partnership, TRANSCAN regularly hosts regulator representatives to the network-level symposia as invited speakers. The reason for this level of involvement can be found mainly in the objective of the partnership to promote early clinical trials and 'first in human' but also in the overall size of the partnership, which does not allow research investments large enough to justify the participation of regulators so early in the translation process.

Infrastructure

The services of research infrastructure[10] are of fundamental importance in modern medicine and it is no different for the activities promoted by TRANSCAN. We learned that the partnership's approach to infrastructure is consistent with the overall structure of the partnership and its remit. Specifically, from a funder's perspective, TRANSCAN does not provide infrastructure to its projects; however, it seeks to facilitate access, when possible, for example, to translational and clinical research resources; essentially, TRANSCAN directs applicants and projects towards making use of available research infrastructure coordinated through the European Strategy Forum on Research Infrastructure (ESFRI). These include:

[10] Infrastructure is intended as a longer-term capital-intensive and high-cost investment essential for Research and Innovation. These may be physical infrastructure such as machinery (i.e. next generation sequencers, digital and communication networks), databases including genomic resources, chemical compounds, biobanks and digital patient data. They may also include clinical trial facilities residing in hospitals and in private drug development organisations.

1) the Biobanking and BioMolecular Resources Research Infrastructure (BBMRI),
2) European Advanced Translational Research Infrastructure in Medicine (EATRIS),
3) the European Clinical Research Infrastructure (ECRIN),
4) distributed infrastructure for life-science information (ELIXIR),
5) European Research Infrastructure for the generation, phenotyping, archiving and distribution of mouse disease models (INFRAFRONTIER),
6) Integrated Structural Biology Infrastructure (INSTRUCT).

TRANSCAN also recommends and directs its projects towards the services offered by the newly established European infrastructure comprising the European Infrastructure of Open Screening Platforms for Chemical Biology (EU-OPENSCREEN); the European Research Infrastructure for Imaging Technologies in Biological and Biomedical Sciences (EURO-BIOIMAGING) amongst others.

In its remit as a funder, TRANSCAN established that applicants and project consortia should themselves secure access to the infrastructure that are necessary for their research. Applicants' proposals are also evaluated on the basis of whether projects can carry out the research proposed and this includes access to those services such as sequencing, omics and access to omics platforms, clinical trial facilities etc. These are seen as indicators that projects can perform research activities successfully. Access to basic infrastructure services should be well specified in the proposals submitted, as they are object of the feasibility evaluation. Through this process, TRANSCAN establishes a clear division of labour between funders and projects which is functional to the ERA-NET organisation and governance of multiple R&I funders distributed across different countries.

Training and capacity building

Our interviewees inform us that training and capacity-building activities in the area of translational cancer research are at the core of the TRANSCAN remit. The partnership, in fact, positions itself as a platform in cancer translational research and fostering capacity building in this area is an important aspect of nurturing translation from basic research to clinical research. Training and capacity building activities are directed mainly at early-career researchers whilst clinicians may engage in short training rather than longer terms residencies. This is due to two main reasons: 1) the nature of clinical work usually ties clinicians to their parent organisations and 2) transnational exchange of clinicians is usually cumbersome because of different rules and regulations in different countries and Member States. The partnership makes available to its projects additional resources for capacity building and training activities, including 1) exchange and mobility of investigators, especially young researchers; 2) short term training visits; 3) training through technical workshops and 4) short training run by external experts.

These activities are complemented by the ERA-NET TRANSCAN symposia and research prizes awarded to project researchers.

The rationale is that of training the next generation of translational researcher in cancer and it is generally considered a valuable investment, especially for PhDs and Post-Doctoral researchers. These specific activities by the partnership are considered part of the evolution process of

translational cancer research. The experience of a partnership's interviewee is that such investments show that a significant number of early researchers then go on and progress by conducting well-run projects, securing several rounds of funding either through TRANSCAN but importantly, accessing other sources of funding promoting longer term sustainable research. This type of longer-term project approach to sustainability is validated by several interviews with PIs and researchers of TRANSCAN projects. It is important to note that, usually, those projects that have been successful in several rounds of funding and/or that have sourced their research support from different sources are also very successful in translational activities. It is the case, that several interviewees mentioned how TRANSCAN's support through two or more rounds of funding was key to the development of their early translational work and that subsequent funding helped them bringing innovative products or procedures closer to patients.

Specific capacity building activities in the early clinical trial domain fall usually within the remit of TRANSCAN though, activities beyond the level of Clinical Trial Phase 2 may be seen outside its scope. The reason for this is that Clinical Trial Phase 2 activities, whilst deemed of outmost importance, are too onerous for the ERA-NET budget. In reality, TRANSCAN projects are funded at an average of €1mil each and generally last for 3 years. Clinical trials in phase 2 and 3 usually require well over that amount of resources and are bound to last longer than the average duration of a project. Moreover, the remit of TRANSCAN is not to drive drug discovery to its end, which would require the involvement of commercial entities/organisations but to bridge the first and most challenging 'valley of death'. This is the link between basic research and early drug development phases through a robust process, which may provide solid result upon which further phases can be built. This aspect links to the next step: opening up to market/final users.

Opening up to market/final users

TRANSCAN's objective is to move basic research into early clinical trials and this means that marketable products are/will be still 5 to 10 years down the line, sometimes longer still, after the completion of projects. Therefore, the partnership does not have resources in place to bring project outcomes to market. Taking outcomes to market or to users is at the discretion of the project partners and their own resources and networks if they want to take the project results further. Nonetheless, whilst the partnership can claim numerous success stories within and beyond its remit, with activities reaching more advanced clinical trial phases, some of these success stories have reached the final users.

A success story case that goes beyond the remit of the partnership consists of the discovery and validation of **a pattern of molecular biomarkers used to evaluate paediatric cancer patients affected by Acute Lymphoblastic Leukaemia (ALL).** Studying such patterns helps predict the level of risk of relapse. This diagnostic tool is now generally in use in paediatric trials and allows physicians to target and regulate dosages of potentially harmful chemotherapy drugs. In other words, it helps to provide a more targeted therapy to children affected by ALL .

Success stories of projects funded by the partnership show that research, both basic and clinical, can move forward through early clinical trials and produce promising results. These, our interviewees confirm, can further progress to complete clinical trial phase II or initiate phase III with the involvement of biotech companies, small entrepreneurial pharmaceutical firms or spin off companies. It is in fact on the back of early clinical success that commercial organisations are more willing to take a risk.

At these stages (clinical trials phases II and III) projects may be approached and proposed to licence their discoveries or buying in the idea. Such occurrences, albeit rare, have happened in the lifespan of the partnership even though the antecedents were quite unique. In one case, 10 years of R&D carried out by researchers and clinicians spanned several rounds of funding (not necessarily provided solely by the partnership) and both researchers and clinicians already had strong professional relationships with industry.

It is the experience of the managers of TRANSCAN that **large biotechnology and pharmaceutical firms** monitor, wait and see what a project funded through public monies can achieve. They become involved only if the research is solid and early results are promising. However, this aspect requires a certain degree on entrepreneurial capacity from the project partners coupled with the willingness to bring their research findings to practice.

As mentioned, it is the responsibility of the projects to assure sustainability of the venture and going to market is one way to achieve it. Progressing robust research (basic and clinical) through to the next clinical trial phase is a question of funding and time and as reminded above, the projects funded by the partnership have a short duration (3 years) and a limited budget averaging at €1 mil.

The partnership is somewhat keen to provide funding to those projects that, at the end of their cycle, demonstrate scope for further fruitful research but the terms are those of new submissions to the open calls. Even in successful projects, transnational trials, especially at the phase II and III, usually encounter many delays and difficulties at the administrative level since Member States have different authorisations procedures and branches of the trial need to be authorised in each country in which it takes place. Given the complexities and the sheer volume of work involved in phase II and III of clinical trials, the whole process is usually longer than the life span of the project.

Although in the experience of TRANSCAN, it has happened that projects have entered later stage clinical trials and projects have been allowed to proceed for over 8 - 9 years[11].

Indicators and Drivers of success

The definition of success in TRANSCAN is multidimensional. The partnership has a monitoring system in place used for assessing projects on an annual basis against planned deliverables and more generally in terms of their ultimate objectives. As a baseline, the partnership adopts standardised indicators such as scientific outputs (research publications, conference presentations and posters), patents submitted and assigned and licence agreements. Although the management structure of TRANSCAN is aware that 'count' indicators such as number of publications, patents and licencing agreements do not sufficiently represent projects and the partnership's success. Part of the assessment of activities focused on 'research flows' looking at items and research findings moving through to clinical trials.

The success of the partnership is driven by the success of its projects. The aim of TRANSCAN is to promote collaboration between basic biomedical researchers and clinicians in order to push research findings towards the motions of pre-clinical trials. This implies that knowledge and expertise of the two categories of researchers are shared and understood by both types of scientists: those involved in basic research and those in clinical research. Science, research and innovation can rarely progress to 'first in human' if the expertise, knowledge and capabilities of biomolecular scientists and clinical researchers is not linked and combined.

Critical for success is that the partnership works with renowned experts in the field with a proven track record of achievements in the areas of interest. In fact, sometimes "*the success of a project can be "predicted" by looking at who is working on them*" (quote from TC/PM). Important elements that have been flagged by the TRANSCAN management as indicative of the good standing of projects are the facts that partnering PIs and their research centres work at the scientific, technological and clinical frontiers, their reliability in performing complex tasks and their personality and attitude to collaborating throughout the various stages of the projects. Of course, this approach presents also some systemic weaknesses. Scientists and clinicians operating at the scientific, technological and clinical frontiers may find it more convenient to look for alternative sources of funding, for example, with higher payouts than TRANSCAN can provide. Also concerning the experiential aspects, "*giving the grants to those we know can deliver*" may certainly result in more successful projects but their lower risk profile may come at the expense of "*excellent ideas*" (quote from TC/PM).

Certainly, given the guidelines in place, the selection process depends on the evaluation panels for proposals whose members are appointed by TRANSCAN's national partners/funders rather than by the partnership itself. The focus is on achieving a "*balanced approach between low risk*

[11] The reason for this unusual extension of the project has been clearly enunciated by our interviewee, "*it would have been unethical to interrupt trials involving patients"* (quote from TC/PM).

– *good projects and high-risk excellent projects*". Ultimately, the types of projects that are funded depend on the funding agencies and ministries and not the management committee.

At the project level, indicators of success refer mainly to meeting the milestones stated in the proposals. From the many interviews, there is great pride in meeting and surpassing such milestones even if, largely, the majority of our interviews focus on their findings, results and outcomes as a mean to progress in their research for a solution to the medical problem. In fact, success is driven by the willingness of the PIs and the beneficiaries to progress in their endeavours; the driver to attain expected results is "*not only with the funders, but within the community. This is a great incentive in working together with clinicians and clinical researchers as this is the only way to test the hypotheses and gain new knowledge*" (quote by a PI, molecular scientist). For clinicians, on the other hand, the drivers of success can be summarised by the following quote: "*I'm interested in getting closer and closer to a solution that can help my patients or at least help patients' parents and cares understand what there are going through*" (quote by a clinical scientist).

2.2 European Joint Programme – Rare Diseases (https://www.ejprarediseases.org/)

Aims and Background

The European Joint Programme on Rare Diseases began operations in 2019 following three rounds of ERA-NET funding (e-Rare, e-Rare-2 and e-Rare-3). However, governance, scope and reach of EJP RD have greatly changed since its ERA-NET days. The partnership now comprises some 130 partner institutions distributed across over 35 countries. The 24 European Reference Networks[12] formed by healthcare professionals with expertise on rare diseases across Europe are also amongst the partners.

The aim of EJP RD is "*to create a comprehensive, sustainable ecosystem allowing a virtuous circle between research, care and medical innovation*"[13] in rare diseases. This aim is undertaken in a particular area of health where disease prevalence is rather scarce (a disease is defined rare when it affects less than 1 in 2000) but there are more than 6000 rare diseases, most of them of complex genetic origins. This means that the disease area is very heterogeneous and sparse making regional or national approaches to research, development and innovation fruitless. The rare disease undertaking is, therefore, best suited in an international or global setting. In fact, the objectives of the partnership are[14]:

1) *To improve the integration, the efficacy, the production and the social impact of research on R[are]D[iseases] through the development, demonstration and promotion of Europe/world-wide sharing of research and clinical data, materials, processes, knowledge and know-how*
2) *To implement and further develop an efficient model of financial support for all types of research on RD (fundamental, clinical, epidemiological, social, economic, health service) coupled with accelerated exploitation of research results for benefit of patients.*

Activities of EJP RD are organised under 5 pillars including 1) coordination and management (transversal activities and communication useful for the smooth running of the partnership and its projects); 2) research and innovation funding (done through transnational calls and other funding opportunities such as fellowships and network schemes); 3) virtual platform to standardise, curate and connect data sources that are currently scattered across research and clinical centres working on rare diseases and link them through a federated information infrastructure that respects data ownership and reflect the open data principles of the EU; 4) the training and empowerment programme aimed at enhancing the existent capacity building activities in rare

[12] https://health.ec.europa.eu/european-reference-networks/overview_en

[13] https://www.ejprarediseases.org/what-is-ejprd/project-structure/

[14] https://www.ejprarediseases.org/what-is-ejprd/project-structure/

diseases around Europe and further develop it; and 5) support to innovation pathways and clinical trials in rare diseases across Europe.

The main governance institutions of the partnership consist of a General Assembly formed by all beneficiaries of the EJP RD, which is the ultimate decision-making structure. It works in strict contact with the Governing Body which is the main decision-making body on matters such as organisation, work programme, the annual work plan etc. At INSERM, France, sits the coordination office of the partnership dealing with project management activities, communication and financial and project funding aspects[15].

The partnership uses the services of an Independent Ethics Advisor assuring that its activities are in line with ethical and legal principles of the EU.

The partnership, although in operation since 2019 has a substantial number of beneficiaries. It is in fact estimated that about 85% of the European research communities engaged in rare diseases research and development are directly or indirectly linked to EJP RD. Direct beneficiaries from EJP RD funding for R&I activities include hospitals, research institutes, funding bodies and ministries, university research centres and research hospitals, European infrastructure and charity/foundations.

Objectives and rationale

From our interviews, it emerges that EJP RD work spans several high impact objectives across the research and development domain for rare diseases in Europe and beyond. Its objectives include funding of "*all types of research on rare diseases*" (Quote from EJP RD management) from basic and preclinical research to early clinical, up to late-stage clinical trials. It does so by taking up the role of traditional research and development funder, fostering translational activities and facilitating the inception of clinical trials, providing training and contributing to data resources and tools. Whilst bringing new products - drugs and diagnostics - and new procedures to practices informs the majority of the activities of the partnership (and several important steps have been made through EJP RD work with the 24 European Reference Networks), there is awareness that the involvement of firms is ultimately necessary to bring products to market. To this end, "*Industry is involved but to a light extent*" (Quote from EJP RD management). However, the partnership recently implemented the Rare Diseases Research Challenges. An initiative set out in the form of a public-private partnership focusing on technical challenges. The challenges are based on insights and experience of the industry partners who contribute in the definition of challenges-related calls, participate in research projects and provide funding.

It has been reported that it is difficult to identify the final users as a distinct category of beneficiary since the partnership is working towards creating a transnational ecosystem for tackling the vast and heterogeneous problem of rare diseases. For this reason, the calls issued by the partnership

[15] The governance structure and the roles of each governance bodies are explained at: https://www.ejprarediseases.org/what-is-ejprd/governance/

are rather broad including projects dealing with basic research issues, pre and early clinical research in a number of domains that, together, provide inroads to a better understanding of rare diseases and open new pathways to therapies. The partnership tends to accompany its projects through by providing support and resources to de-risk research and development activities and make sure that results are taken up and can be further used to develop diagnostics and therapeutics. It does so by involving stakeholders such as clinicians and other organisations (including hospital and clinical research centres and companies, usually smaller firms).

To foster this pathway, the partnership's policy on data is particularly relevant. In fact, the open access culture underpins the partnership and its project's policy, which is actively pursued through standardisation activities, participation in data infrastructure initiatives and by providing support for data management and quality assurance. The data repositories are federated across countries, meaning that data and information are kept at the owners' locations but are standardised and pooled in order to allow access and remote consultation from a common access point. The rationale for this commitment is to be found in the partnership's remit to foster a transnational rare diseases ecosystem involving researchers, clinical researchers, clinicians, private organisations and patients' communities.

Collaborations

EJP RD is "*users and recipients oriented*" (Quote from EJP RD management) meaning that activities are geared towards the needs of the potential final users be they patients, clinicians and, to a lower extent, industry. This objective is enacted through a host of initiatives embedded in the various work packages of the partnership and the system of relationships with partners including the European Reference Networks and third parties. In particular, the partnership has strong ties with patient's advocacy groups, patients' associations, and patients' research funding charities across Europe and beyond.

Users and recipients are directly involved in the activities of the partnership's steering board and operating groups. Through these channels, the organisations are able to steer the partnership's activities towards themes and challenges that are relevant to the progress of R&D in the rare diseases and contribute to define calls that meet the broad and specific need of the patient population. In addition, the partnership runs activities organised in "Mirror Groups" whereby these organisations are involved directly in the dissemination of best practices, of the findings and knowledge generated of the partnership's projects. The National Mirror Groups consist of national representatives of the partnership and other relevant stakeholders. The composition varies from country to country but the common objectives are to coordinate the effort on rare diseases between the various member countries and the work of the partnership by involving stakeholders active within the area.

As mentioned, EJP RD also launched the Rare Disease Research Challenges - an initiative directed at setting up and funding collaborations between large companies, academia, SMEs, and patient organisations involved in specific research challenges in rare diseases. This undertaking, foreseen in the workplan of the partnership (WP8) intends to include directly

companies, large companies and SMEs, in the workflow of activities of the partnership. According to our interviewee, it took some organising since the challenges come directly from the experience of the industrial partners and research and clinical organisations have been invited to propose R&I projects to solve such challenges. As the projects are now underway, we were made aware that the organisation and deployment of the projects encountered delays due to the cumbersome technical nature of the network/consortium agreements. In fact, the documents, necessary to initiate projects' work, took several months more than expected because of the time necessary for reaching an agreement by all parties. This experience, however complex, showed two main issues: 1) that there are clear differences in approaches to research and innovation amongst the various parties: researchers and clinicians on the one sides and industry on the other; and 2) that when such different stakeholders are brought together in R&I collaborations, researchers and clinicians, on the one hand, and industry, on the other, should work together from the onset in order to i) establish the project proposals and shared approaches and ii) decide on the governance of the bidding consortia. At the same time, such initiative established a direct pathway to collaborations between researchers, clinicians and industry that otherwise would be virtually impossible in the area of rare diseases.

Infrastructure

The partnership places particular importance in the mobilisation of infrastructure both as services provided to its projects and as developmental activities (promoted by the partnership and its projects). To reach these objectives, representatives of European research infrastructures sit within the EJP RD General Assembly. As a consequence of this strategy, the partnership does not fund directly infrastructure but "*has unfettered access to the services and the facilities*" offered (Quote from EJP RD management). These include access to the Advances Translational Research Infrastructure (EATRIS), biobanking through the Biobanking and BioMolecular Resources Research Infrastructure (BBMRI), the Clinical Research Infrastructure through ECRIN, access to mouse models, phenotyping and other clinical research infrastructure through INFRA.

EJP RD projects' may gain access to the services offered by these infrastructures through their application process. This means that, when projects are set out to work using particular infrastructure they do so through the partnership. These may include support to basic and clinical research processes but also support to long-term sustainability beyond the life cycle of single projects. In addition, in the data domain, the partnership set out the open data federated system with the Distributed Infrastructure for Life-Science Information (ELIXIR).

Another important aspect concerns EJP RD involvement in 'soft-knowledge' infrastructures. From our interviews, it emerged that the partnership has also several links with national and international organisations engaged in the rare diseases areas, these are foundations, patient associations, hospitals and healthcare institutions and industry within the EU. The interviewees highlighted how these constitute important sources of knowledge for their projects and allow co-creation activities, further training capacity and access to research supporting activities able to blend partnership's services with established external infrastructure in the rare diseases area.

Specialist/Technical Knowledge

There is certainly awareness, both at the partnership's and projects' levels, of the need to access specialist technical knowledge which, though related to research and development, are not necessarily included in the traditional understanding of 'core' activities. These are, nonetheless, important complementary activities that are fundamental for the smooth operations and include intellectual property protection and ethical assessment, regulatory submissions processes and collaborative contracts to name but a few. Also important is knowledge of logistics and transport procedures of cell samples across countries, which even though operating within the single market, still have different clearance processes in place.

At the partnership level, there are two main mechanisms in place to provide such necessary complementary services: 1) the Intellectual Property and Ethical Committee and 2) support mechanisms from the Sustainability Teams (WP3). These services are provided by teams of experts coordinated by EATRIS[16] and organised as a Mentoring Programme provided by EATRIS, the French National Institute of Health and medical Research (INSERM) and the Spanish Carlos III Health Institute (ISCIII). The programme provides services to projects covering aspects such as regulatory, commercialisation, drug development IP and patenting, industrial collaborations etc. whilst the sustainability team is engaged in providing support during development pathways and access to services provided by the partnership.

In addition, EJP RD set out an innovation management toolbox, which is intended to support results and findings to increase effectiveness in design and implementation of sustainability actions.

Other specialist and technical services that are necessary for the smooth operations of research and development activities are part of the remit of the funded projects. These include both the decision making regarding strategic aspects of projects' organisational tasks (i.e. patenting and IP protection) and other ancillary services. The rationales for this approach are that 1) projects' strategic decision should be left to the beneficiaries who choose how to protect their research findings and that 2) specific service needs should be part of the problem-solving activities of the projects and, however possible, should be included in the proposals. In any case, projects can rely on the host of competences and services available at the partnership level.

Training and capacity building

The third pillar of activities scheduled by the EJP RD partnership focuses on training and capacity building. Whilst technical development remains the core activity of the projects, the partnership provides many training opportunities in the form of educational activities and translational research support. Training and capacity building activities include IP management, knowledge/technology transfer and commercialisation. The rationale upon which capacity

[16] EATRIS is the European Advanced Translational Research Infrastructure in Medicine with own network and members addressing specific challenges that may be encountered during translational activities.

building and training activities are organised within the partnership is that basic and clinical researchers running the funded projects should be, of course, skilled and specialised in their core activities whilst, at the same time, should have a general and practical understanding of the translational mechanisms and R&D dynamics. Throughout these initiatives, researchers and clinicians supported by EJP RD can gain a general understanding of the translational process and learn about key reference points within the partnership and their own organisation (technology transfer office) to whom they can address their sustainability concerns.

According to our interviewees, the partnership's approach is largely 'reactive' and targeted at enabling and sustaining emerging research and innovation pathways. This is also in line with the objective of the partnership to provide such mentoring and support services which allows projects to reach their milestones and progress independently, whilst promoting the formation of a transnational rare disease ecosystem - "*building the field*", support the creation of networks - "*bring it together*" and capitalise on the emerging complementarities and synergies - "*let it grow*" (Quotes from EJP RD management).

Moreover, the partnership is involved in long-term planning and setting future training and capacity building opportunities with other transnational networks such as EIT Health where some dedicated training on development and entrepreneurship may be provided to the EJP RD beneficiaries. Further, future opportunities of training and capacity building comprise collaboration with the European Medicines Agency and other national regulatory authorities to develop a dedicated programme involving scientists and build capacity in regulatory issues.

Opening up to market/end-users

We should start this section with a proviso: the rare diseases field, whilst rather important, is very heterogeneous and operates across a great number of small/niche markets. Therefore, companies' and other large commercial investments are rather limited.

For this reason, the partnership is 'proactively' engaged in two main types of activities that are directed at the longer-term sustainability of its investments in beneficiaries' projects. The first set of activities consists in monitoring the projects whilst they are running hence being able to provide necessary expertise and ad hoc mentorship, for example by involving directly representatives from industry to provide advice, link the projects to particular firms, or external experts that may incentivise entrepreneurial undertakings.

The second set of activities is to stimulate a forward-looking attitude by "*working at what's next*?" (Quote from EJP RD management)[17]. The objective is to progress through the projects with a sustainability plan in mind. The partnership provides support on project sustainability through 'using' the network to allow projects to select their next steps either in the direction of further

[17] Similar statements have been made also by several PIs and other beneficiaries.

follow-on funding (via the EJP RD, Framework programmes, no-profit calls etc.), reach out to specific partners including companies or venture capitalists.

> Some examples of this strategy at work concerns help and support to projects into **progressing along an innovation pathway**. A project approaching the end of a proof-of-concept work or completing a mechanism of action study may be on a particular innovation pathway where either further research and development funding may be necessary, or it is maturing for venture capital. In these cases, the partnership makes available resources, services and expertise to facilitate such follow-on activities or provide resources and guidance to transition to market-type arrangements.

From the partnership's perspective: no specific innovation pathways can be indicated as preferred because the nature of any project is somewhat unique, nonetheless it is important to show alternatives routes to longer term sustainability via access to resources, services and expertise.

Indicators and Drivers of success

The definition of success by the EJP RD is somewhat complex and multifaceted. It takes into account the success of the partnership and that of its projects on interconnected levels explained below.

As a first, more practical approach to defining success, the partnership uses standardised metrics of outcomes and key research indicators through which all work packages of the partnership are evaluated on a yearly basis. In this area, evaluation and definition of success is somehow standardised and in line with European Commission requirements and abiding to H2020/HE indications. The partnership, however, is elaborating indicators that are more refined and putting in place more forward thinking monitoring activities to be able to provide a more holistic view of the progress made and the impact the partnership is having in creating a transnational rare disease ecosystem. Success for the partnership, in fact, is measured against how the ecosystem is being created, how it is increasingly integrating across the various rare disease areas and across the EU and, equally important, the impact it has on the community through facilitating research and innovation.

The partnership operates two interconnected monitoring systems, one that allows keeping in check the operations within the partnership and one specific to the projects it funds. The systems are deriving from subsequent iteration from when EJP RD was operating as an ERA-NET. In fact, EJP RD is still monitoring projects initiated under the E-RARE partnerships. Overall, the partnership is employing traditional metrics such as publications, patents and licencing agreements, number of genes identified for particular diseases and their validation stages. Together with indicators such as number of animal models and other drug development indicators, these metrics are functional to the monitoring of projects. These activities are carried out yearly and provide useful information through which a longitudinal perspective can be gained. Simultaneously, in order to appreciate the performance of the partnership and its projects, it has been necessary to develop more qualitative indicators that can provide a narrative on the

significance of the partnership within the rare diseases' domain and signal to a wider audience the type of reach and impact the partnership is having.

Drug repurposing is an increasingly common occurrence, and even if drugs and processes are still far away from being implemented in practice with different indications, it gives a sense of the progress made towards building and integrating the rare diseases ecosystem. Being able to be a reference point, provide expertise and indicate possible pathways to patients and for the wider community, this is a clear indication of longer-term impact.

> The case of **drug repurposing** is particularly telling. In this area, researchers and the clinical community is progressing with significant applied research which is being brought to implementation in clinic. Achievements in these areas are cutting down lead time, that currently stands at about 15 years, and costs (which, at present stand at about €30 mil). In some cases, they manage to move to the clinic with significant impact on the lives of people suffering from rase diseases.
>
> Stories like this provide also a very important signal to externals. Organisations engaged in drug repurposing, for example, when incurring in hurdles and bottlenecks into their development and clinical trial processes can now have a reference point in the EJP RD which is able to provide support and open up innovation pathways.

Further indications of success are projects that receive subsequent funding, not necessarily by the same partnership. Here, evidence of progress can be evaluated in relation to the target towards implementation. For example, projects that worked towards achieving proof of concepts then progressed to pre-clinical studies, clinical trials etc. to obtain "*entries in the registry*" becoming new therapeutics and new diagnostics that are then helpfully used in the clinical community (Quote from EJP RD management). Another indicator is the number of companies that have spun off from specific projects and that are now becoming economically viable. As mentioned, success can be appreciated in the longer term: "*now we are seeing benefits from previous rounds of funding*" (Quote from EJP RD management). The timeline to attaining impact extends beyond the single cycle of a project and, often, also the timeline of the partnership. This is particularly evident for EJP RD considering the experience gathered since 2007 (the launch of the first ERA-NET e-Rare). Through continuous monitoring of the activities undertaken since inception, the partnership can now better appreciate its wider societal impact.

Drivers of such success can be ascribed to the logic in place within the partnership, which brings together a vast set of stakeholders and capitalises on their individual strengths. For example, scientists are encouraged to carry out their research and though it is understood that they might not be able to initiate a clinical trial, clinical researchers are brought in with their experience and competence to complement those of scientists. Of course, mentoring, support activities and innovation management backing are provided by the partnership. This is especially important in the rare diseases areas where clinical studies cannot be undertaken nationally because of the low number of cases. To overcome this hurdle, the partnership activates, together with the European Clinical Research Infrastructure (ECRIN), a "*dedicated multinational clinical trial*

support office" (Quote from EJP RD management). Overall, the partnership also facilitates this process through providing regulatory, IP protection and other types of consulting. Eventually it is envisaged that also entrepreneurial skills will be necessary and to that extent, whilst there are provisions in place for training and support, it is foreseen that external companies may be involved.

A final word on the drivers of success should be given to the system of incentives operating across the translational journey affecting actors differently at different periods in the life cycle of projects. Researchers have, of course, different incentives than those of clinicians; researchers tend to use their discoveries for publications and dissemination activities whilst clinicians work in direct contact with patients, hence their research activities are more focused on patient outcomes. The contributions of both categories are fundamental for the progress of projects especially operating at the early phases of discovery, pre-clinical and early clinical research. According to our interviewees, issues such as alignment of incentives are very difficult to deal with and, whilst in the whole of the sector a solution to this puzzle has not been found, championing the development of knowledge and taking it towards the patient is very important. The same observation can be made also progressing in the translational journey when involving industrial partners becomes necessary for bringing new therapies, diagnostics or other milestones to markets. The partnership operates with public funds therefore has a different take than that of industries operating with their investors' monies. Again, in these occurrences different institutional logics and value generating perspective may come into conflict.

The solution pursued by the EJP RD is that of ecosystem formation, that is internalise the incentives of all its stakeholders within a system of relations focusing on the specific issues of the rare diseases areas. This means that stakeholders, whilst supported by the partnership, are invested in the same objectives driving the partnership and this acts as a catalyst for many of the collaborations we have interviewed in the course of this research.

2.3 Joint Undertaking Innovative Medicines Initiative (IMI) / Innovative Heath Initiative (IHI) (https://www.ihi.europa.eu/)

Aims and Background

IMI/IHI has been operating within the EU as a public-private partnership since 2005[18]. Since its origins, the partnership underwent three successive embodiments as Innovative Medicines Initiative (IMI and IMI2) and then as Innovative Health Initiative. The joint undertaking Innovative Health Initiative was launched in November 2021 and, in continuity with its IMI predecessors, transferred ongoing projects under the IHI banner.

This, however, was not only a change in name but also a broadening of the partner-base of the undertaking and its objectives. In fact, whilst IMI partnerships included the EU and the pharmaceutical industry, IHI public-private partnership extends across a wider industrial base including the pharmaceuticals, medical technology, biotechnology, digital health and vaccine industries extending its remit across the health domain.

The aim of the IHI partnership is to foster a comprehensive approach to healthcare leveraging the excellent research and innovation sector within Europe and provide/promote an enhanced ecosystem within which new medical discoveries and population health gains may be obtained by integrating the contributions of all medical and health stakeholders. IHI does so by "*supporting projects that bring together these industries as well as universities, small and medium-sized enterprises (SMEs), patients, regulators and others, we aim to pioneer a new, more integrated approach to health research. Our approach moves the focus from disease care to health care, starting with disease prevention, and covering diagnostics, (personalised) treatments, and disease management. At every step, products and services from different sectors would be seamlessly integrated, making it easier than ever for patients and their clinicians to monitor and manage their health*"[19].

This general aim is spelled out in operative objectives that can be found in the Strategic Research and Innovation Agenda published in January 2022[20]. Moreover, the partnership is also engaged in the EU-wide policy area concerning health. Specifically, the partnership contributes to Horizon Europe (of which it is part), the European Cancer Plan, the Industrial and the Pharmaceutical Strategies for Europe amongst other notable EU policies.

Partners of IHI are the European Union, represented by the European Commission, and representatives of the European life-science industries including the Pharmaceuticals Association

[18] In 2005 the partnership began operation as European Technology Platform on Innovative Medicine. https://www.ihi.europa.eu/about-ihi/history

[19] https://www.ihi.europa.eu/about-ihi/mission-and-objectives

[20] https://www.ihi.europa.eu/about-ihi/research-and-innovation-agenda

(EFPIA) and Vaccine Europe, COCIR, the European trade association representing the medical imaging, radiotherapy, health-ICT and electro-medical industries, representatives of the bioindustry (EuropaBio), and the association of medical technology firms (MedTech Europe).

The partnership's decision-making body is the Governing Board, formed by four representatives of the EU and four representatives of the different industrial trade associations. The governance structure includes a Science and Innovation Panel that provides the Governing Board with scientific and evidence-based advice, the Group of States Representatives, consulted by IHI on different matters regarding national and regional issues and providing the linkages between the European-wide strategy of the partnership and the science and innovation resources located in the various Member States. The partnership has an Executive Director chairing the Programme Office that is in charge of the day-to-day running of IHI activities and projects monitoring.

The ongoing partnership has a significant budget consisting of €2.4bn, of which, €1.2bn is provided by the European Union and €1bn by the industry partners and life-science industries associations and €200m by other life science organisations who joined IHI as contributing partners through either in kind (researchers' time, facilities, materials and data) or cash contributions. Eligibility criteria for beneficiaries are similar to those of the Framework Programmes i.e., universities, research organisations, patient organisations, small and medium-sized enterprises (SMEs), established and operating within the EU and associated countries. In each project, at least 45% of the budget should come from industry and/or contributing partners.

Objectives and rationale

Based on our interviews, the main objective of the Innovative Medicines Initiative/Innovative Health Initiative (IMI/IHI)[21] is to facilitate and accelerate early development of new medicines, new diagnostics and new medical procedures by filling the gap between research and innovation operating in those areas where critical roadblocks (the valley of death) are more important. The partnership works in the pre-competitive space including basic and clinical research, business/market entry of products and their applications into clinical practice with the objective of building a medical/health innovation ecosystem able to operate along the translational continuum by facilitating the translational journey both within and across specific disease areas.

[21] In the reminder we shall use 'IMI/IHI' to indicate the partnership because the projects PIs and other beneficiaries have initiated their project within IMI2 and are carried on in IHI.

The partnership has an extended portfolio of over 150 projects covering the different elements of the IMI/IHI strategic agenda. These include 1) tackling different disease areas focusing on World Health Organisation priority for Europe; 2) integrate effort to move from one stage to the next of the research and innovation domains; 3) use specific projects from various disease areas to develop and enable translation, connect and prioritise research and innovation tools that can be used more broadly; 4) include from the beginning those partners that usually come in late during the research and innovation process (i.e. industry, regulators etc.) and include also end users working side-by-side with other stakeholders to ensure that their health and medical needs are being met; 5) include in projects also partners that, at first, may seem "non-typical" of the medical R&D process but that may be functional to specific development pathways.

An example of this modus operandi is the partnership's effort in developing the **Ebola vaccine** together with Janssen within an IMI's co-funded project. Authorisation for the Ebola vaccine was obtained from both EMA and the US FDA in 2020. The work for the vaccine was then translated and used for early inroads towards the development of the Covid 19 vaccine which obtained authorisation from EMA and the US FDA in early 2021. Further development of this process is now allowing a platform for rapid response to new infectious diseases.

These strategic aspects are being pursued to reflect the general aim of the partnership to create a sustainable European-wide health innovation ecosystem. The idea is that through individual projects and coordinated activities (both at the partnership and the project levels) such an ecosystem forms, grows and becomes sustainable through building operational connections amongst all stakeholders involved.

For example, **networks of hospitals** may be mobilised in order to speed up the trial processes to the benefit of clinical researchers and companies involved in projects. Likewise, **charitable organisations, and patient advocacy organisations** are involved in most projects and these constitute both an effective channel for dissemination of findings and events but also effective collaborators in the innovation process.

Collaborations

As highlighted above, the collaborative nature of the venture is central to the overall objectives of the partnership and can be seen both at the partnership level - constituted as a joint undertaking including a great variety of industrial partners - and through the composition of the project consortia. In fact, the collaborative effort in consortia is achieved through a wide range of organisations contributing their R&I capabilities to each of the funded projects. Even though the idea is that projects do not replicate a common approach to the problem they are tackling, their general structure is somehow reflective of the organisational nature of the partnership. In particular, projects consortia include partners from all (or most) of the stakeholders' group including universities, research organisations, public bodies and not-for-profit groups, biopharmaceutical and MedTech companies (large companies and SMEs) and may include

partners such as hospitals, non-EU research centres etc.. The resulting 'mix' of collaborators is rather varied and includes basic and clinical scientists from universities, public and private research organisations and industry, biopharma and tech experts from healthcare and medical organisations, "*boots on the ground*" clinicians working daily in direct contact with patients and on research and patient advocates. They often also include partners with expertise in the regulatory, intellectual property and businesses.

The partnership has an outward looking approach to collaborations. In fact, IMI/IHI set out a mechanism to reach out to various stakeholders also outside of the European Union or countries associated to the Framework Programme who share the mission and vision of IMI/IHI and want to participate to its activities by bringing in additional resources. These are important international organisations that are active in specific disease areas such as the American Autism Association, the Safari Foundation or in the field of juvenile diabetes, the Juvenile Diabetes Research Foundation from the UK who are Associate Partners of IMI/IHI projects collaborating through providing in kind contributions.

Infrastructure

From our interviews, it emerges that IMI/IHI has a dual approach to infrastructure. On the one hand, the partnership is connected to the major European infrastructure established/coordinated through the European Strategy Forum on Research Infrastructures; on the other hand, the partnership plays a major role in connecting and integrating its activities (and those of its projects) to those of existing European and pan European infrastructure in order to enhance the services provided, their reach and depth.

The first instance is enacted through establishing collaborations with ESFRI organisations. In fact, European research infrastructure are eligible to join IMI/IHI projects and are increasingly called upon to join newly formed consortia. This is a way for the partnership to secure access to infrastructure services for their projects and provide an input into projects' longer-term sustainability.

One example concerns the involvement of **ELIXIR**, the European distributed infrastructure for life-science information, in taking part in large projects so that the data and information produced may have a longer time span than the projects.

In the second instance, the partnership contributes through several projects to enhance the European and pan-European network of existing infrastructure and capitalise on the existing structures to contribute and further its vision of creating a European ecosystem for health innovation. This strategy allows the partnership and its projects to carry out high quality clinical trials for registration purposes.

An example of enactment of this strategy is the link established between projects in the area of **Microbial Resistance.** A host of organisations operating at national and international levels has been brought together to foster precompetitive research and innovation activities leveraging projects' microbial resistance research and clinical findings. This way, the activities of investigators can benefit from and navigate formal registration with the regulators because the process to bringing drugs, diagnostics and methodologies to approval is rather complex and needs to follow rigorous administrative practices. The partnership promoted infrastructural-heavy co-creation activities, undertaken also with private partners, working with existing infrastructure to build a clinical trial network – which now has pan European reach – formed by over 1,000 hospitals with clinical study facilities and capacity (LABnet, a network promoted under COMBACTE).

For example, by leveraging the **networking of regional resources** for early trials in Alzheimer's disease across the Nordic regions and BENELUX, the partnership and its projects extended their trials network beyond the initial stage. By the end of the project, the activities had expanded throughout Europe with over 30 trial delivery centres located in Belgium, Netherlands, Sweden, France, Greece, Ireland, Italy, Switzerland and the UK.

This strategic approach to enhancing European medical and health research infrastructure has been employed in other areas such as paediatric clinical trials and the target reach has been extended beyond European boundaries with collaborative links established with the US.

The strategy revealed also very important during the pandemic as it served as a cross-European infrastructure for the trials of various Covid19 vaccines, monoclonal antibodies and other therapeutics. As stated during our interview: […] *we really see now that there is really this leverage of what existed but putting it on network level […] it was more focused on IMI, now the network is involved in many studies outside IMI*" (Quote from IMI/IHI management).

This concept has also been extended and refined for translation of research in early clinical research and proof-of concepts trials. It has been argued that this perspective was brought in the work of IMI/IHI by the private partners whose institutional logic is that of a global reach: "*because we are a public private partnership, obviously all the companies see the drug development as a global issue*" (Quote from IMI/IHI management). This point is also mirrored by the experience of many of the projects PIs and beneficiaries interviewed during the course of this study. In fact, the rigour of the administrative processes and the capacity of leverage capabilities, competences and resources from a vast network facilitate the work of the project partners. This outward looking approach encompasses other aspects of the partnership. In fact, as see in the section above, Associated/Contributing Partners also contribute to the extending of the infrastructure network.

Specialist/Technical Knowledge – training and capacity building

In terms of specialist and technical knowledge, it was mentioned by both the management and the beneficiaries that IMI/IHI is a very large partnership with a significant budget. As such, the partnership facilitates the involvement of a broad spectrum of specialists and expertise, from industry and other stakeholder organisations that are necessary for research and innovation projects.

Our interviewees highlighted that specialist/technical knowledge necessary for the smooth course of operation is somehow embedded within the network and projects are invited "*to think ahead about their needs for such services and plan accordingly*" (Quote from IMI/IHI management). Of course, a variety of tools and processes are already in place within the partnership in order to provide support, training and respond promptly when needs arise. Established pathways to access to such services may be found in the general activities of the partnership.

> For example, the importance of regulatory aspects should be taken into account from inception of projects. In many cases, projects consider to integrate expert regulatory knowledge within their development processes and validation phases.

To foster projects' success, the partnership encourages that relevant investments focusing on business sustainability and commercialisation or awareness of the importance of regulatory pathways should be integrated from the design phase of R&I projects. To this extent, the employment of business experts may be necessary together with the involvement of the regulator from the early stage of development activities. Whilst the partnership provides training activities through webinars involving, for example, experts from the European Medicines Agency or the US FDA, it now encourages such forward-looking activities by suggesting that these are embedded within the project at the application stage "*This makes sure that projects can think from the start of having a strategy […] embedded*" (Quote from IMI/IHI management).

Another example of partnership approach to securing its projects with specialist/technical knowledge concerns several aspects of projects' sustainability. Expertise in business and long-term project planning are available to beneficiaries who are invited to think about these issues from inception and budget for them accordingly. At the same time, the partnership understands that not all provisions may be predicted and budgeted for before the start of projects and therefore it foresees the involvement of business analysts and legal professionals to help projects design and implement their sustainability plans after the projects have started (when necessary).

When justified, the partnership approach to **access to technical and specialist knowledge** may be summarised in two main 'tools' which become particularly important when specialist and technical knowledge were not fully estimated and/or budgeted for during the drafting of the project proposals. These consist of the capacity to inject flexibility and adapt activities by allowing 1) changes in the technical annex to allow for such expenditure without affecting the scientific priorities of projects. A further opportunity may be given by 2) injecting flexibility in the budget. With the proviso that the European Commission's contribution is unaffected, projects can embed additional expertise from inside or outside the consortia. For example, additional expertise may be provided by industrial partners which i) are not receiving funding from the EU and ii) can change, add resources also through subcontracting (Quote from IMI/IHI management).

Opening up to market/final users

The interviewees confirmed that the partnership operates in the precompetitive space and does not have a standardised legal procedure in place to bring products to the market; but has set in place tools that may help the different stakeholders of IMI/IHI to translate valuable outcomes of research projects into new drugs, diagnostics or therapeutics for the benefit of patients. When considering a biomarker or a database, which have been developed within a project, several tools can be used to leverage them as assets in a more business-oriented venture. Ownership of intellectual property deriving from collaborative work, for example, can be transferred rather smoothly to either an existing company or a new legal entity at certain conditions and in the respect of the intellectual property rights of the other partners.

Like in EU funded projects, partners have access to results generated within a consortium. IMI/IHI has also implemented the option that a third party can gain access and licence relative rights so that medical and healthcare insights generated within projects can be further developed and brought to the 'bedside'. Such access is negotiated by the parties involved "*on fair and reasonable conditions for research use purposes*" under IMI (Quote from IMI/IHI management) as facilitating such transfer of asset results in more commercialisation opportunities.

A further element, specific to IMI/IHI, follows the consideration that, whilst the partnership is not directly engaged in producing new drugs, diagnostics or medical and healthcare products, it certainly is involved in speeding up and integrating their development processes. IMI/IHI does so by capitalising on the basic and clinical research assets produced by the projects. Therefore,

project consortia, in the first instance, may set out a budget to secure or facilitate the establishment of appropriate structures such as infrastructure or spin out legal entities (including companies) when these are not foreseen within the initial request by the beneficiaries.

Of course, these options are open for the decision-making process of the consortia, and not the partnership, since beneficiaries are the original owners of the Intellectual Property generated. Nonetheless, the partnership is proficient in facilitating this process as scientific teams and legal experts work very closely with the beneficiaries to reach fair agreements on sustainability options, which will be implemented from the start of the project and not necessarily at the end of the funding cycle.

The **Open PHACTS project**, one of the early projects launched by IMI in 2008 and now terminated, proved quite successful in developing a drug discovery platform (Open PHACTS Discovery Platform) linking several complementary components of drug discovery databases. The platform was very innovative in providing industry, academic and clinical researchers rapid access to relevant drug discovery data. From the beginning of Open PHACTS operations, it was foreseen that the assets created within the consortium would eventually be acquired by the Open PHACTS Foundation which was formally established in 2016 and is now considered a leader in linking drug discovery data and has been partnering in several research and innovation consortia funded by the H2020 Framework Programme.

A similar, yet more recent example of a pathway to sustainability is given by the case of the **GetReal project** which ran for two funding cycles and developed tools for incorporating real/live data in some aspects of pharmaceutical R&D and healthcare decision making processes including regulatory and health technology assessment. The data and tools are now managed by the Get Real Institute, a newly formed not-for profit organisation operating from the Netherlands.

Indicators and Drivers of success

Defining success for such a large partnership with the ambitious aim of creating and extending a medical and healthcare translational ecosystem in Europe is not an easy task. Significant effort went into setting the parameters within which a comprehensive evaluation could be carried out. Nonetheless, the success of the partnership may be explored through the success obtained by its projects.

During their course, projects success is assessed against key performance indicators, which at the end of each reporting period are collected through standardised forms and published on the IMI/IHI website[22]. At the same time, project deliverables are also assessed against the original aims and targets articulated in the project plan. Overall, this assessment process is comparable

[22] https://www.ihi.europa.eu/about-ihi/impact

to that implemented in the Framework Programmes and the results are published yearly in the IMI/IHI Annual Activity report.

At the partnership level, the aggregation of indicators reveals that IMI/IHI researchers have been very prolific; by 2021 they published over 8,000 peer-reviewed papers. Looking at bibliometrics, such publications are also high-quality with a citation standardised Impact Factor of over 2, which is twice the global average and 80% higher than European average (IMI_Bibliometrics_Report_2022.pdf).

Nonetheless, to understand IMI/IHI contribution and impact, one should take a look at the various achievements that are not directly quantifiable such as, for example, the types of mechanisms of action and biomarkers identified, new methodologies, tools and processes that the partnership nursed to approval and brought to the bedside, their impact on public health in certain disease areas, etc.

However, quantifying and fully articulating the impact of a partnership is a challenge that is very much at the centre of the debate on evaluation of translational research. To this end, IMI/IHI is leveraging expertise to define a methodology for estimating the long-term impact of the partnership, which, for the particular sector within which it operates, has a time line of over 10 years. This aspect is particularly important since understanding trajectories, directions and "*red threads*" could provide evidence for improving and making the activities of such a large undertaking more efficient and effective (Quote from IMI/IHI management).

At the same time, the partnership has been in operation for many years and has gained a certain experience in understanding their way to success and that of its projects. If such wisdom can be synthesised in one sentence that will be "*the willingness to work together*" (Quote from IMI/IHI management). This quote makes particular sense since the overall objective of the partnership is that of creating a medical and health innovation ecosystem, which necessarily entails a certain degree of networking and collaboration.

In this ecosystem, IMI/IHI sees itself as a neutral platform, key to the ecosystem configuration since its role as external coordinator can be fulfilled only by gaining the trust of all the stakeholders involved. By this virtue, the partnership may assume the role of mediators between the various stakeholders involved within the ecosystem, stakeholders who carry with them different agendas and interests. By the same token, directed by clear objectives, the partnership relies on its neutrality to build trust between stakeholders and foster dialogue.

At the same time, the directionality of the partnership's effort and of those of the partners needs to converge towards those objectives set out in the strategic research and innovation agenda. The importance of such approach stands particularly when we consider that 1) the organisational tasks of the partnership require to mobilise substantial resources and competences from a variety of partners and that 2) the outcomes of such effort impacts at the institutional/organisational and at the project levels. The results of this reasoning are evident in the stride made by the partnership during its existence. One interviewee highlighted how, "*twelve years ago, having five or six industry partners around the same table was not so obvious*" (Quote from IMI/IHI management),

whilst now the partnership has at the same table, basic and clinical scientists from different disciplines, large companies and SMEs from different sectors such as biopharmaceuticals, medical technologies, digital and information technologies, etc., medical and health organisations such as hospitals and clinics, patients organisations and advocacy groups, regulators etc. all working towards shared challenges and organised in projects consortia.

3. Discussion and conclusions

This study delved into the translational work of health-related partnerships. We selected three partnerships representatives of ERA-NET (TRANSCAN), European Joint Programme (EJP on Rare Diseases) and a joint undertaking (the Innovative Health Initiative) in order to investigate how partnerships navigate the process of translating research activities into medical and health innovations for the benefit of patients, the European health systems and society.

The three partnerships are very different and mirror, to a greater extent, the great variety of transnational partnerships operating within the EU and beyond. In table 1, we compare the partnerships based on their funding cycles and resources made available for research and innovation activities through their projects. We proceeded in our comparative analysis based on the eight elements we investigated during our fieldwork. These include: 1) objectives and rationales, 2) the types of collaboration agreements they have with other stakeholders of the European health system, 3) their relations with the network of infrastructure available to research and innovation projects, 4) specialist and technical knowledge necessary for their projects' operations, 5) training activities and capacity building initiatives, 6) their approach to bringing the research results and milestones of projects to the market or their final users, 7) how the partnerships and their projects define and evaluate success and, finally, 8) the critical factors in achieving such success. We completed the comparative table highlighting the challenges encountered by the partnerships and their projects in setting up and sustaining their remit.

Table 1. Navigating the translational journey, a comparative analysis of the three partnerships.

	TRANSCAN	***EJP RD***	***IMI/IHI***
Type or partnership Funding cycle Resources	• ERA-NET Cofund. • 5 years funding cycle and projects usually running for 3 years. • €52 mil (TRANSCAN-2 and €34 mil (TRANSCAN-3).	• European Joint Programme. • 5 years funding cycle and projects running for 3 years. • Budget is agreed by the partners for each transnational call: JTC 2021, €12mil and JTC 2022, €17mil	• Joint Undertaking between the EU and private partners. • 7 years funding cycles and projects may overrun the duration of the partnership. • €5.3bn (IMI and IMI2, 2008-2020) and €2.4 bn (IHI, 2021-2027).
Objectives and rationale	• Overcome the critical translational phase between basic and clinical research in cancer. • Structured links between biomolecular research and preclinical studies to provide robust foundation to translational research.	• Create and sustain the rare diseases research and innovation ecosystem in Europe by providing support to research and development (from basic research to clinical trials). • Provide better understanding and open new pathways to therapies in rare diseases.	• Public-Private partnership supporting pre-competitive research. • Enhance and leverage the European health research and innovation ecosystem in the precompetitive area. • Comprehensive approach to healthcare in Europe by bringing together industry, universities, hospitals, SMEs, patients, regulators and other stakeholders.
Collaborations	• Biomolecular scientists and medical/clinical	• Activities are geared toward the needs of the final	• The partnership has a broad base of stakeholders

	researchers. Extended to hospital and other research centres, patients' advocacy and charitable organisations. • Collaborations with other stakeholders is sought (i.e., through symposia) but not actively pursued.	users (patients, clinicians and the health system). These are directly involved in the activities of the partnership and its projects. • Stakeholders of the health system collaborate both at the partnership level and at the projects level. • The Rare Diseases Research Challenges established a pathway to collaboration between researchers, clinicians and industry.	involved in its activities at the programme and project level. • This is reflected in the composition of consortia of beneficiaries.
Access to infrastructure	• Partnership is research funder and does not provide direct access to infrastructure though it facilitates access to translational and clinical research centres and ESFRI. • Projects and beneficiaries should arrange access to infrastructure services necessary for their research.	• The aim to build and sustain a rare diseases ecosystem places particular importance access to and development of infrastructure. Representative of ESFRI sit within EJP RD. Beneficiaries have unfettered access. • EJP RD projects gain access to infrastructure services through their application process.	• The partnership is connected to the major European research infrastructure (ESFRI). • Through its activities and its projects (many projects target the creation of new and/or the enhancement or extension of existing infrastructure), IMI/IHI contributes to the integration and accretion of the European infrastructure capabilities.
Access to Specialist/Technical Knowledge	• As research funder, TRANSCAN does not provide specialist services or project-specific technical knowledge.	• EJP RD provides its projects with access to specialist/technical knowledge through two channels: 1) the Intellectual Property and Ethical Committee and 2) Sustainability Teams. • Several toolboxes are available to beneficiaries in areas of innovation management, clinical trials, and mentoring and support for translational activities and follow-on funding for project sustainability.	• Specialist and technical knowledge are embedded within the partnership given the great number and variety of partners. • A number of tools and processes are in place to assure that such skills and knowledge can be accessed by beneficiaries. • Increasingly, consortia include expertise in project sustainability and long-term project planning. • Consortia own experts or externals can be embedded at the proposal stage or when need arises (without changing the EU contribution).
Training and capacity building	• This is a core activity of TRANSCAN and its remit to training the next generation of cancers researchers and clinicians. Extensive training and capacity building directed at early career researchers. Clinicians can access short residential training. • The partnership makes available extra resources for exchange and mobility of researchers, workshops, short training sessions (also run by external experts) and activities such as the annual symposia and research prizes.	• Training and capacity building is one of the main pillars of EJP RD. The partnership provides training and capacity in several areas: IP management, knowledge and technology transfer and commercialisation to complement the expertise of basic and clinical researchers in translational processes. • Mentoring and support services may also be accessed on ad hoc basis. • In a longer-term perspective, EJP RD is setting up training and capacity building with other partnerships/organisation such	• Training and capacity building are embedded in the projects and beneficiaries and third parties can use resources for educational and capacity building purposes e.g., with patients' organisations. Universities, and research centres fund PhDs and post-docs. • Many events have the objective of providing training and increase skills and expertise; these include workshops, webinars and meetings with EMA and FDA to raise awareness of regulatory pathways and provide early

		as EIT Health (training and entrepreneurship) and regulatory agencies to build capacity in regulatory issues.	interaction/networking with regulators. • Industrial partners involved in projects provide scope and opportunities for entrepreneurship and business training.
Opening up to market/final users	• TRANSCAN objective is to move research to the clinic and early/pre-clinical trials (first in human). Market is still 5 to 10 years away. • Researchers and clinician can bring their findings/milestones to market at their discretion; the partnership is supportive of such activities (and can claim several success stories).	• There is very little market for rare diseases health products and services (small/niche market opportunities). • Involve industry and external experts into project workflows to provide advice and incentivise entrepreneurship. • Stimulate a forward-looking attitude through the identification of project-specific innovation pathways involving further funding opportunities (either within the partnership or external sources) and/or getting ready for venture capital or other market options.	• Activities supported by IMI/IHI are at the pre-competitive stage. SMEs and Industrial partners, being part of projects, provide several assets such as tools and methodologies for projects to explore entrepreneurial avenues. • Findings and milestones belong to beneficiaries but external third parties may be brought in through licencing. • A number of IMI projects led to the creation of spin-off/out are also supported with specific budget lines.
Measures and factors of success	• Success is defined as fruitful collaborations between basic and clinical researchers. Scientific output, patents submitted and assigned and licence agreements are monitored. • Research flow at the clinical frontier is important indicator of success and projects progressing through multiple funding cycles (either in TRANSCAN and other funders/market).	• Traditional metrics are employed for monitoring and reporting (H2020 and HE guidelines). • Metrics include publications, patents and licencing agreements, genes identified and validated in clinic, animal models and other drugs and diagnostic development indicators. • Holistic view of success and impact of the partnership in creating a rare diseases ecosystem in Europe are indicators (and story lines) of integration of activities within the rare diseases' communities.	• In addition to the traditional metrics described in the H2020 and HE guidelines for monitoring and reporting, IMI/IHI developed specific KPI based on the programme's objectives such as number of biomarkers identified and validated, new methodologies, processes and tools that the partnership nursed to approval and brought to the bedside. • It is difficult to assess the overall impact of the partnership during it implementation because of its early positioning on the R&I process. • Evaluating KPI against projects milestones, IMI projects have systematically exceeded expectations.
Challenges	• Projects that meet and exceed targets and expectations may find it difficult to progress further in their translation activities as the partnership has no resources for such occurrences. • Rolling out more advanced clinical trials and links with the industry.	• The partnership operates with public funds and principles and these differ from industry norms. • The number and variety of stakeholders involved in the partnership reflect their respective agendas, differences in incentives and professional norms.	• IMI/IHI is a very large undertaking including a variety of partners from the public, private and social sectors and patients working together on pre-competitive issues. • Its objective is to operate as a neutral platform across these stakeholders but it may operate as mediator, which is to be expected given that the European health ecosystem is rather dynamic.

Source: Own elaboration

As mentioned in section 1, our objective was that of understanding how these partnerships and their projects are undertaking their translational activities, how they relate to the critical phases

identified in the literature and how they build and sustain their network connections in order to progress in their research and innovation objectives.

3.1 Pathways to impact

The three partnerships have a very different approach to translating research into innovation.

In our first case study, TRANSCAN operates as a traditional research funder and positioned itself at the intersection between biomolecular research and clinical research[23]. Its objective is to bridge the gap between proof of concept, which usually happens in basic research laboratories and universities led by biomedical scientists, and early clinical applications, which usually occurs in university and research hospitals led by clinical researchers. These two domains have traditionally been unconnected. The work of basic scientists and clinical scientist usually happens in independent silos and research and applications progress under different institutional logics and according to different norms.

TRANSCAN provides a bridge through the valley of death connecting basic biomedical research and clinical research by promoting research engaged in the two dimensions collaboratively. It does so by sponsoring research and innovation projects spanning the two domains. This approach, whilst justified also by the limited budget of the partnership, is revealed as very effective in connecting the two worlds of basic and clinical research and at the same time, has consistently provided resources and support for 'first in human' trials. During the course of the partnership, which ran for three funding cycles (since 2011), there have been several highly valued innovations that have made their way to patients. These are mostly clinical and therapeutic applications, including diagnostics that are currently used within the health system to assist physicians in the care of children (in paediatric oncology) [24] and diagnostics that, after validation in multicentre studies, are now deployed at the bedside throughout Europe and beyond (i.e., in Israel and North America).

In our second case, EJP RD, the aim is to coalesce rare diseases stakeholders towards collaborating within a European-wide rare diseases ecosystem[25]. This approach has been reached (and is progressing) by reaching out to those research centres, clinical research hospitals, patients' advocacy groups and charities, national and European regulators and to a lighter extent, industrial partners. Its objective is to work collaboratively across Europe in the discovery, validation and development of clinical applications in the area of rare diseases. The partnership, in its endeavour to extend and consolidate such European-wide ecosystem, provides a wide range of services channelled through infrastructure associated with the European Strategy

[23] https://transcan.eu/the-project/objectives/project-objectives.kl

[24] Paediatric oncology suffers from a chronic lack of therapeutic options because clinical applications, trials and requests for approval are mostly run for the adult population. Oncologists in charge of children have little therapeutic options, usually consisting in a scaled down version of the options available for adult patients. At the same time, they may enrol children in advanced clinical trials whereby they can use evidence-based medicine to treat them more effectively.

[25] https://www.ejprarediseases.org/what-is-ejprd/project-structure/

Forum of Research Infrastructures and developed in house through the wealth of expertise available through its partners.

EJP RD has become a European and international reference point for the rare diseases communities acting as hub for knowledge generation and exchange, research and innovation support services and more generally, providing an international platform for research and innovation in this medical and health area. This strategy is coherent with the nature of the problem that the partnership is facing: rare diseases are heterogeneous, there are over 6,000 rare conditions affecting a small number individuals[26] distributed across regions and countries. Whilst the majority of rare diseases have genetic origins, these can be as diverse as rare cancers, autoimmune disorders, hereditary malformations, neurological and developmental disorders but also rare infections. For these reasons, the approach to rare diseases research and innovation has to be necessarily one of public health that transcends national borders and national health systems of innovation. By including over 130 institutions across 35 countries, the partnership is making serious inroads towards 1) aligning the European and international response to rare diseases, 2) coalescing the expertise, capabilities and competences of the R&I communities working on rare diseases and 3) provide a public health policy response by improving systems' capacity to deal with rare diseases. The partnership's approach to rare diseases activates and exploits synergies within the ecosystem to improve research and innovation capacity overall.

Whilst our first case study, TRANSCAN, deployed a focused approach in bridging the gap between basic and clinical research in cancer, and the EJP RD is working towards the formation and the establishment of a European (and international) ecosystem on rare diseases, our third case, IMI/IHI, has a more ambitious objective, that of promoting a comprehensive approach to healthcare in Europe[27]. It does so by operating in the pre-competitive space bringing together public institutions, industry, third sector actors working in health, universities, research hospitals, SMEs, patients and their advocacy groups, regulators and other stakeholders. With an overall budget to match its ambition, the partnership is operating on several fronts: 1) from basic and applied research in critical medical and health areas 2) to supporting and driving the formation of a robust network of infrastructure and 3) leveraging synergies amongst its partners to accelerate the European health system's response to medical emergencies and longstanding strategic aims in public health policy.

In addition, in this case, the approach is justified by the amount and the mix of resources available to the partnership – for half of its funding coming from the European Union and half from the private partners including large companies, charities and foundations operating in the medical and health sectors. It is important to notice that such an approach is carried out through its projects: EFPIA companies do not receive any EU funding but provided in kind and/or cash contributions to IMI project. In IHI, at least 45% of project costs should come from private partners through in kind and/or cash contributions. This consistency assures to a greater extent, that excellent basic and clinical research, evidenced by the quantity and quality of the scientific output, is matched by the drive to bring innovation to market and at the bedside. The partnership's reach also extends to those system-making and enhancing connections - established amongst the stakeholders (public institutions, industry, third sector etc.) - and functional resources (i.e.,

[26] In Europe a condition is defined 'rare' when it affects 1 person in 2,000.

[27] https://www.ihi.europa.eu/about-ihi/mission-and-objectives

infrastructure, clinical trials, regulatory and business capabilities) enabling excellence within the European health innovation system. This strategy is appropriate because of the large stakeholder base of the partnership engaged in almost all aspects of the health system and therefore, through its activities, the partnership can capitalise on its position within the ecosystem and operate as catalyst amongst the stakeholders. This enables the partnership to operate as a key health policy partner to EU institutions and its projects to make important contributions within the European health system by influencing strategic medical and healthcare areas.

3.2 Conclusions

The variety of partnerships in the European medical and health system is a valuable asset for policy and for the health sector. From a policy perspective, we can see how these partnerships provide invaluable input in the policy process as large partnerships and Joint Undertakings are directly involved in the policy process at the EU and national levels. They provide important policy learning experience and an extended network of relations amongst the stakeholders, including public institutions, the business community, the third sector and the medical and health communities who have reach across the EU Member States. They are also the players enacting such policy strategies at the most granular level, spanning from actively responding to high-level health policy objectives through their system-making processes and research and innovation activities by translating basic and clinical research into new therapies and approaches to innovation in the health system. Both small and large partnerships boast excellent research competences and, increasingly, they have demonstrated capabilities to enabling functional channels to market and to patients for the benefits of patients and health systems nationwide.

The partnerships contribute significantly to the sustainability of the European health innovation system through their capillary training and capacity building programmes. The partnerships and their projects invest heavily in the next generation of researchers, clinicians and health entrepreneurs by funding PhD and Post-doctoral positions and promoting cross-organisation exchanges, training and dissemination activities involving new methodologies, symposia and other knowledge exchanges, cross-sectoral connections and collaborations.

Unpacking the diversity of partnerships, we can see that there is a lively and competitive cluster of ERA-NET-type partnerships focusing on specific medical and health areas. Their remits span from specific disease areas such as cancer, cardiovascular diseases, nutrition and health, neuroscience etc. to paradigmatic scientific areas with medical and health applications such as personalised medicine and nano-medicine. The depth and breadth of these partnerships is such that transnational research and innovation in key areas is reaching high standards of excellence in tackling specific critical phases of the translational continuum.

At the same time, the joint partnerships are focusing on health research and innovation activities with a large remit and longer-term impact such as rare diseases and antimicrobial resistance. These partnerships, for example, also have the remit to build active and sustainable ecosystems within their areas of expertise facilitating wider uptake of scientific knowledge as well as providing an invaluable platform for the development of new experimental methodologies and evidence-based advancement of transnational health policy.

Finally, large joint undertakings are well positioned to foster their ambitions of integrating and enhancing capacity and capabilities of a European truly transnational health system. They can do so through their system-building operations by bringing to the table stakeholders from various backgrounds, moved by specific incentives and working towards different agenda by focusing their activities and investments on shared objectives responding to health policy targets and research and innovation goals established in the Strategic Research and Innovation Agendas.

A policy challenge, which emerges from our study, is that of capturing the potential synergies generated within the complex system of health-related partnerships. In fact, we have seen that relatively small projects, even when extremely successful, may have difficulties in accessing competences and resources to further advance their innovations towards the bedside. Often, for these beneficiaries, the way over such hurdles consists in bootstrapping, repeat applications for funding and in a minor capacity, in searching a way to market through patenting and licencing, spin-offs and collaborations with established firms. From our interviews it emerged that a small number of very successful research groups had ongoing complementary projects funded by different partnerships (and other sources) and that this opportunity helped them enormously in progressing in their basic and clinical research. Through capitalising on these synergies, they gained better access to knowledge and resources to bring their findings and milestones to clinical trials hence enabling further pathways to impact involving commercial partners.

On the other hand, we have seen that other partnerships, especially those focusing on ecosystem-building activities may have capacity and capabilities to foster larger projects with diverse sets of beneficiaries who contribute to research activities as well as advancing potential innovation to the bedside more effectively.

The emerging policy challenge is to encourage the development of connections and links between partnerships, forming and leveraging higher-level synergies, in order to exploit the potential interactions that are being established within the European research and innovation health system. The objective is to create a nurturing environment for potentially innovative projects to thrive and develop; whilst such an objective may transcend the boundaries of a single partnership, it is necessary that the health sector act collaboratively.

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5. Annex

1.2. Annex I: Questionnaire script

On behalf of the European Commission, ERA-LEARN supports research funding organisations, policy makers and researchers with general information and services on research and innovation partnership initiatives.

www.era-learn.eu

Background information

Interviewee:
Name and organisation: _____
Type of organisation (Ministry agency, research funding organisation, university/research organisation, private-non-profit organisation, private company – Large company 250+ employees, SMEs
If company: please specify sector of activity
Role in the partnership (Funding/Sponsor organisation, Advisory/steering committee member, project coordinator, project partner, consultant, …)

Interviewer:

Date of interview:

GDPR compliance authorisation for recording the interview and use (anonymised) for research purposes (reports, presentations, academic publications etc).

Q1 - In which 'segments' of the translation continuum does the partnership/project operate? What types of relationships does the partnership/project have with actors external to the translation segments within which it operates?

Very brief description of activities using the translation continuum: _____

In the first part of the question, we would like to know the types of activities undertaken by the partnership/project – this information is cross-checked with the vision/mission of the partnership/information on the project (so kept brief). In the second part of the question we should like to know what type of activities, relevant for the partnership/project are carried out, whether there are milestones at the partnership/project level and what types of interactions they have with the direct users of their results (being they patients, physicians, clinicians and clinical researchers etc..) – ease in next question.

Q2 - Do you have representatives of direct and/or final users of your results steering and participating the activities of the partnership/project? How does this involvement work?

_____

Here we want to understand to what extent the focus on the end users affects activities overall, even if the partnership/project is not directly focused on putting products at the bedside. Do they have representative of clinicians/physicians, patients' advocacy groups in their AB? If the end result of the partnership/project is not a therapeutic or a diagnostic product (i.e. a compound to put through clinical trials, or through regulatory approval) do they have representatives from clinical research or regulatory experts in the AB?

Q3 What types of infrastructure* (i.e. transnational/national public/private, technological/institutional etc.) the partnership/project uses in the activities?

**Infrastructures are capital-intensive / high cost investments that are essential for R&I. These may be physical artefacts such as hospitals and drug development/clinical trial facilities, networks and communication networks. They can be high-cost machines such as sequencers or non-physical databases such as the genome, chemical compound databases and other data repositories.*

_____

Here we would like to know what are the links with structures and institutions outside of the partnership/projects? If they use existing infrastructure or have links with NGOs operating in the field, Universities or Industry facilities…)? At what level they interact (i.e. set joint objectives,

collaborations, support, contractual uses, etc. …)? It would be great to single out one example and explain how they use the infrastructure or work with other institutions outside of the partnership/project/participant.

Q4 – Advancing through the translation continuum requires specialist technical knowledge and skills that often reside outside of the area of activity of the partnership/project (these may include regulatory submissions, patenting, logistics, etc). Is such knowledge residing within the partnership/project or is it actively sought? How is professional guidance/knowledge residing outside the boundaries of the partnership/project accessed?

_____

We would like to know whether ancillary competences are residing within the partnership/project or, if not, whether there are procedures in place to ease access to such competences. Whether this access is facilitated within the partnership/project and whether there are provisions to access them. Whether there are knowledge and technology transfers and resources for capacity & competences building to help moving along the continuum. If yes, what is the rationale for developing and sharing these competences? If not, how they access them when they need to?

Q5 – What types of training, capacity or competences building activities are promoted within the partnership/project or sought from external providers (collaborators, consultants, etc.)? How and to what extent their fit with the core activities of the partnership/project is evaluated against their contribution to achieve successful outcomes?

_____

Here we would like to understand whether the partnership/project seeks specialist or professional knowledge internally or procures it from external providers. Focus on what types of specialist and professional knowledge the partnership/project needs (R&D capacity and competences such as testing capabilities or scale up capacity, knowledge and technology transfer, procedural or legal support for patenting or regulatory submission etc.) and how it develops these capacities and competences.

Q6 – Are there provisions to opening / bringing to market, milestone technologies such as diagnostics, compounds, etc.? Are there provisions to involve VC or promote spin outs / start-ups to carry on project results?

_____

Here we would like to understand whether there is space (or indeed a push) for entrepreneurship within and outside of the projects. i.e. to know whether there are provisions in place for start-ups/spin outs, to what extent outbound activities are encouraged and whether there is a mechanism in place providing for this eventuality.

Q7 - How the partnership/project/participant defines success?

In the translation journey (i.e. moving from a phase to the next in the continuum) are there any indicators of success at the end of the translation segment (i.e. academic output, n. of patents, n. of compounds pushed through CT 1, 2 & 3, activities in the field etc.)._____

Here we want to encourage the interviewee to provide 1) a representative success story and 2) a story that was not successful as expected (highlight the end result, stages to get there, hurdles/bottlenecks to overcome, and how these were dealt with, who was involved in driving the activities from inside the partnership/project or externals)?

Q8 – What are the most important (critical) factors to move forward across the translational continuum for the partnership/project to succeed?

_____

Here, we want to steer the responder away from referring to general competences, excellence, etc. We need to focus on what mix of capacity/competences are relevant in the various phases, how they are obtained within and/or procured from outside the partnership/project or developed inside. How these factors relate to the typical activities of the partnership/project? How the factors in the competence mix are supported by the partnership/project in moving along the translation continuum? Finally, how the partnership/project relates to the users of the outcomes of their activities. We would encourage the interviewee to provide one or two examples of how these factors are linked to successful outcomes.

Q9 – Is there any aspect of the partnership's / project's translation journey which you believe is relevant for our study that we have not taken into consideration in this interview and you wish to point out?

_____

Before we close the interview, can you please indicate suitable candidates for interviews?
Name and organisation: _____

Type of organisation: _____

If company: please specify sector of activity: _____

Role in the partnership: _____

Thank you very much for your time!

1.3. GDPR – informed consent form

On behalf of the European Commission, ERA-LEARN supports research funding organisations, policy makers and researchers with general information and services on research and innovation partnership initiatives.

www.era-learn.eu

CONSENT FORM

We invite you to participate in the ERA LEARN Impact Assessment exercise by being interviewed. The interview will take about one hour. Information collected will be analysed and used for research purposes (reports, policy briefs, publications)

Informed Consent to Participate in ERA LEARN

I have read and understood the information on the ERA LEARN Impact Assessment Study and received answers to any questions I asked.

I agree to take part in ERA LEARN with a recorded interview. My taking part is voluntary; I can withdraw from the study at any time and I do not have to give any reasons for why I no longer want to take part. The interview, recording and collection of any personal details are for scientific purposes only, within the scope of the project.

My personal details will be processed and handled in accordance with European legislation including the General Data Protection Regulation (EU) 2016/679). My words from this interview may be quoted, preserving anonymity, in research outputs (academic publications, reports, etc.).

Name of interviewee:	**Name of interviewer:**
Signature	**Signature**
Place / Date	**Place / Date**

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