



Master Document



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Introduction

Why a Toolkit?

The principal goal of Together4RD is to stimulate scientific collaborations between European

Reference Networks (ERNs) and Industry. Achieving this goal entails overcoming a variety of historical barriers hampering interaction in this space, by adopting a multi-pronged approach centred upon pilots (the learnings from which should foster more -and more varied forms of- collaboration in future). [The Together4RD Position Statement](#), published in Orphanet Journal of Rare Disease in 2023, explored the multifaceted reasons for a general lack of collaboration in this space (whilst also highlighting case studies of fruitful collaborations in the public-private rare disease arena and models for collaboration, before introducing how Together4RD is seeking to move the needle here.). As the Position Statement explains, alongside more policy-oriented ‘barriers’ (real or perceived) such as the Board of Member States [Statement of 2019](#), a range of practical obstacles or uncertainties have limited ERN engagement with Industry:

- for some ERNs, coming from fields with a traditional lack of R&D in the rare disease space, there is a lack of practical knowledge on how to work with Industry
- there is sometimes a lack of awareness around the kinds of projects ERNs and Industry could collaborate on
- ERNs are not well known or understood by many companies, and not all actors within ERNs (centred on academics, clinicians and patients) understand the needs and realities of the private sector
- even where a community is relatively mature, research-wise, challenges arise when it comes to entering into discussions for public-private-collaborations, with often quite different needs and expectations creating bottlenecks and delays

Whilst other strands of work within Together4RD seek to address the more policy-related challenges hampering ERN and Industry collaboration (for instance, exploring whether a revision of the 2019 BoMS Statement would make sense, and advocating for more opportunities to support Industry-ERN interaction), it was agreed that the initiative should create a Toolkit, to provide practical support for future ERN-Industry collaborations. This was deemed by the Steering Group, and wider community, to be a very valuable activity, particularly in the light of the experiences around launching the first three pilots.

What kind of activity is this Toolkit intended to support?

The Together4RD Toolkit is intended to support a broad range of collaborative activities in which ERNs and Industry might partner (see section X). The 2023 Position Statement outlined a range of activities which working groups felt would be appropriate for ERNs and Industry collaboration, and would yield added-value, which have been elaborated further across the course of 2024 into the tool above. This toolkit is mainly concerned with these kinds of activities, more so than traditional clinical trials and partnering in initiatives funded by programmes like the Innovative Healthcare Initiative (IHI) – see further section X

Core principles underlying the creation of this Toolkit

In setting out to produce this Toolkit, Together4RD centred the activities on 3 core principles:

It is important to avoid ‘reinventing the wheel’:

Where existing resources (such as webpages, reports, publications, guidelines, webinar recordings, templates, etc) serve an important purpose in line with our central mission, this Toolkit links directly to these, rather than seeking to develop very similar resources anew. Where the community has identified gaps and proposed resources which would be beneficial but did not exist, entirely new resources have been created. It is particularly important to consider what is unique about ERN and Industry interactions, vs any and all research activity an academic, say, might enter into.

This Toolkit would best be viewed as an iterative document

This is the first iteration of a **Together4RD Toolkit to support and streamline ERN and Industry collaboration**. Changing needs and realities in future may necessitate additions to the toolkit. Furthermore, some of the types of tool proposed for inclusion in the course of the 2024 consultations, would require further work and wider stakeholder engagement – for instance, in agreeing model contract clauses to facilitate collaborations between ERNs and

Industry. Ideally, therefore, this should be viewed as a dynamic resource which will evolve over time, developing in a stepwise approach, by whichever organisation or initiative is in a position to do so.

Tools should benefit both ERNs and Industry

The ToolKit is the result of significant outreach and consultative processes across 2024, and is intended to reflect the needs of both ERNs and of Industry, having engaged key groups of stakeholders in a co-creation process. ERNs and Industry may have different needs, and see value in different components of this resource, but the idea is that it should provide added-value for both sets of stakeholders.

Determining the structure and contents of the ToolKit

The process of scoping potential content for this Toolkit began in earnest in Q1 of 2024, via a survey directed to both ERNs and Industry, intended to:

- a) capture examples of any collaborative activity to-date; and
- a) attempt to prioritise the importance and usefulness of a range of categories of resources, for fostering ERN-Industry collaboration.

Simultaneously, Together4RD ran a similar poll via social media, to try to assess how valuable different types of resources might be. Also in Q1 of 2024, interviews were conducted with both the ERN and Industry representatives involved in the three Together4RD pilot projects and all relevant insights from those interviews, concerning practical tools and resources that could realistically be incorporated to a toolkit, were noted (see also Tool 9: [Experiences and learnings from the first ERN-Industry pilots supported by Together4RD](#)).



The survey and social media polls, in particular, were based around a range of tool categories, which form a starting point for the selection of toolkit **domains**:

- ii. *Background knowledge – to support conceptualisation of a collaborative idea or research question*
- iii. *Practical knowledge transfer – to progress from an idea to a well-developed research collaboration*
- iv. *Legal framework*
- v. *Testimonials (this was later merged with the ‘Background Knowledge’ domain)*
- vi. *Existence/creation of third-party brokers to facilitate the collaboration*

Targeted consultations - workshops for the academic/clinical research community and Industry communities

Building on the survey and social media polls, Together4RD organised a broad consultation across May and June, in the form of two workshops.

Firstly, a half-day workshop was organised Bari on 29th May, aimed primarily at the research/clinical academic community. The workshop ultimately involved 25 experts, largely from academic/researcher backgrounds, some formally engaged with ERNs, others not. After initial presentations contextualising this activity, the group engaged in a brainstorming session to do the following:

- Consider the relevance/appropriateness of the 5 domain headings above, and brainstorm on the types of items which might be included as useful and important examples under each domain
- Consider what might be missing, which would help support more -and more effective- ERN-Industry collaborations in future
- Suggest concrete existing examples of the kind of tools the group identifies/agrees together, to avoid reinventing wheels.

Then, as ‘phase 2’ of this initial toolkit design workshop, 10 representatives of 7 Companies and 2 people from 2 trade associations joined a 2-hour virtual meeting on 23rd June. Here, the participants provided feedback on the overall added value of a toolkit and how best to orientate it, whilst commenting on the usefulness and feasibility of tools in the same domains explored in the Bari event.

An interactive Miro board was created, to incorporate all specific comments collected over

Legal Framework Tools		
Suggested item for inclusion under this domain	Comments - is this useful?	Do we have something here we can use/repurpose, or does it need to be created anew?
Contracting template which could be used for ERN-Industry collaboration	<p>Very useful</p> <p>Some comments on the template: it's a good starting point, but it needs to be adapted to the specific needs of the ERN-Industry collaboration. It should be clear that the template is not a one-size-fits-all solution, but a starting point for discussion.</p>	<p>Do we have something here we can use/repurpose, or does it need to be created anew?</p> <p>Comments on the template: it's a good starting point, but it needs to be adapted to the specific needs of the ERN-Industry collaboration. It should be clear that the template is not a one-size-fits-all solution, but a starting point for discussion.</p>
Examples templates which have supported past forms of ERN-Industry collaboration	<p>Some comments on the template: it's a good starting point, but it needs to be adapted to the specific needs of the ERN-Industry collaboration. It should be clear that the template is not a one-size-fits-all solution, but a starting point for discussion.</p>	<p>Do we have something here we can use/repurpose, or does it need to be created anew?</p> <p>Comments on the template: it's a good starting point, but it needs to be adapted to the specific needs of the ERN-Industry collaboration. It should be clear that the template is not a one-size-fits-all solution, but a starting point for discussion.</p>
Data Sharing Principles	<p>Some comments on the template: it's a good starting point, but it needs to be adapted to the specific needs of the ERN-Industry collaboration. It should be clear that the template is not a one-size-fits-all solution, but a starting point for discussion.</p>	<p>Do we have something here we can use/repurpose, or does it need to be created anew?</p> <p>Comments on the template: it's a good starting point, but it needs to be adapted to the specific needs of the ERN-Industry collaboration. It should be clear that the template is not a one-size-fits-all solution, but a starting point for discussion.</p>

both workshops. Specific remarks and perspectives were grouped against each proposed domain (e.g. *Legal Framework Tools*) for this toolkit, along with potential tools (e.g. *data sharing principles*) suggested to sit within each domain.

It also captured examples of existing assets or resources which participants wished to highlight to Together4RD, as possible tools to include in order to maximise use of what is already available and avoid reinventing wheels.

This Miro board formed the basis for structuring this Toolkit – both envisaging what a future, mature Toolkit might look like, and what this current version should encompass.

Both workshops raised a number of fundamental, cross-cutting points concerning the nature and added value of this toolkit, and realistic steps needed to create and then evolve the resource. The workshop participants also identified additional strategic issues of relevance here, in terms of enhancing ERN-Industry collaborations *beyond* a toolkit.

Overarching Comments to guide the Toolkit development

The Bari workshop, in particular, highlighted the fact that the importance of the **BoMS Statement** -and therefore, by extension, the necessity to try to change it – remains a controversial topic, with people sometimes holding polar opposite views here. To those who

would remark that the two Statements really only reflect the views of a minority of MS who advocated for particular caution in public-private partnerships, the group was reminded that Statements have to be adopted by consensus. We should look closely at the 2019 Statement and focus on what it does allow, regardless of any decisions as to whether to seek for a more supportive wording.

We need to think about whether we are creating tools to fill gaps and needs quite unique to the ERN ecosystem, OR if instead we are simply signposting to a community that does not know much about PPP and may lack experience. Again, this was particularly a message from the Bari workshop. This is important, as if we go solely for the former, it will be a relatively contained toolkit. If the latter, we risk straying into a situation where we are trying to ‘teach’ researchers (and it would largely *be* researchers, as opposed to Industry, probably) about how to do research well, how to work with Industry, how to think about regulatory issues, etc. All agreed it is important we do not try to take on that larger task and **seek to focus as far as possible on what is unique for the ERN situation** and *balance* it with key materials to support public-private partnerships or PPPs (NB **the guiding question is ‘how to do PPPs’, not anything and everything to do with ‘research’**)

Both workshops entered into some discussion on **who the toolkit should really be aimed at** – will it be more useful for Industry or for ERNs? Both workshops saw a benefit for both sets of stakeholders, in the end, albeit *different* benefits for each. For instance, the Industry workshop on the whole suggested that perhaps ERNs could benefit from a more concrete setting-out of expected roles and responsibilities, timelines, and steps to collaboration, whilst acknowledging that the general Industry awareness of ERNs and what they could offer is relatively poor, and they need to better understand mechanisms for working with the Networks and what the added value could be compared to ‘business as usual’. It is important to note that even representatives of the more research-mature ERNs felt they would benefit significantly from a toolkit. Both workshops also highlighted the fact that **ERNs really are very heterogenous and have different needs** – this is often mentioned, but should not be underestimated.

This would ideally be an iterative toolkit. In the Industry workshop, creating the toolkit was likened to ‘paving a way to a road we want to walk’ – some participants cautioned that we should not seek to be too premature here, as building capacity to do these types of collaborations well will take time. It is sensible to start with the ‘low-hanging fruit’ e.g. if a legal template or model contract is deemed beneficial, it’s unlikely this can be created very quickly. And unsurprisingly, **both workshops strongly supported our mantra of not reinventing wheels** with this exercise. Again, it is important to maintain balance between building capacity for ERN-Industry interactions, on the one hand, and generally upskilling academics/clinicians to do research here. It is good that we have some resources which can

support with the latter, like the EJP [Innovation Management Toolbox](#). The T4RD Toolbox should also try to reflect, in this **signposting**, the types of activity Together4RD is particularly concerned with, avoiding duplication: for example, when it comes to actually planning and performing clinical trials, c4c-s has developed tools such as Service Level Agreements and Master Agreements which (although not publicly accessible) but could be highlighted in connection with those specific sorts of activity.

Participants in the Industry workshop, in particular, emphasised the fundamental importance of **agreeing an appropriate and specific research question for ERN-Industry collaboration**. There was a general feeling there that idea generation, at least focusing down to a really specific activity, could benefit from more ERN direction – “right now, it seems that although there is huge potential there, all the key people in each ERN have in mind different research questions for different purposes.” This arguably reinforces the need for tools supporting the kind of collaboration that could be entered into. Nonetheless, it is important to emphasise that the pilot experiences of generating research questions have, by their very nature, not been typical examples of the way in which projects might usually – or might in future- be proposed. Here, stakeholders in both the ERN and Industry communities were keen to work together on *something*, to test the notion of ERN-Industry collaboration, and one could argue that that spirit of collaboration was the key driver, ahead of the generation of a really concrete research question – here, the partners were chosen before the research question, in a way, and that wouldn’t necessarily be the case moving forwards.

Both workshops emphasised that when considering tools relating to the pilots, e.g. the ‘lessons learned’ report, that we recognise that these ARE pilots, and they may not work how we want them to work. Related to this, it may be important to convey the message that in these early forays, choosing a small project that can achieve a specific goal, is preferable to a vast project. It is imperative that we keep expectations realistic, at least at first. Furthermore, these ‘scoping’ and ‘developing research questions’ domains need to acknowledge that just because a pilot learnings report explains how something worked or did not work for a given ERN, it is important to remember that ERNs are not one size fits all. Again, they are very heterogenous, thus learning lessons may not be applicable across the board.

There were some questions, especially in the Industry workshop, around the possibility of **structuring some of these tools according to different types of collaboration that could be envisaged**. For instance, this toolkit could somehow distinguish between two broad types of collaborations – those with a defined beginning and end and a very specific research question and accompanying protocol; and then, on the other hand, those collaborations seeking to fundamentally impact how care is delivered to patients, such as reducing time to diagnosis by implementing algorithms, which will likely require

ongoing care and maintenance. These are fundamentally different kinds of partnerships and will require different kinds of legal structures, for instance. Building on this, it was further proposed that rather than tying this work too closely to the first pilots, **perhaps this toolkit (or future versions) could return to the beginning a bit and think instead of all the different types of collaboration we could envisage. Both workshops proposed revisiting the table of possible activities for collaboration, included with our Position Statement**. Participants to the Industry workshop suggested three obvious *categories* for activities could be research, healthcare delivery, and education. Each of these will likely involve different structures and different roles and responsibilities, and different steps, e.g. in terms of how you contract, which could allow future versions of this toolkit to be more practical. If a collaboration is really in the ‘research’ space, how can research be done – is it an ERN/ERNs working with Industry to explore unanswered research questions and see what can be answered using the data currently available, or creating something new, to address new questions? If the collaboration is more about education, what areas would be in focus: would it be about organising webinars, and if so, to whom would they be targeted (patients, healthcare professionals, etc?).

Notwithstanding future directions for the toolkit, **it was acknowledged that there will be core functions and core practical steps that need to be taken** in all activities; therefore, the toolkit as it stands was created with some more general recommendations on roles and responsibilities and timelines, with the possibility for others to build from there.

The eventual content of this Toolkit

In view of the comments received across all consultation activities in 2024, the tools included below fall under three headings:

- **Background knowledge - ERNs, Industry, and the Opportunity**
- **Conceptualising and firming-up a collaborative idea or research question or project**
- **Practical knowledge transfer – initiating and delivering a well-developed research collaboration**

The current resource does not include tools pertaining to ‘the legal framework’, as it was felt that identifying or creating resources here would require much more discussion. Furthermore, this was quite a divisive point, with participants in both workshops adopting very different stances on the feasibility of developing templates for contracts or agreeing common contracting clauses. In both workshops, some supported the concept of trying to develop more *standardised* contracts (to deal with the fact that different centres have very different contracting styles). Or if a standardised contract is not doable, perhaps clauses that all need to use could be identified. The concept of a ‘playbook’ was also raised, with some

standardisation in contracting templates for each category of activity we could foresee. However, all of this would require further work.

It is important to emphasise once more the complementarity of this toolkit to the broader Innovation Management Toolbox or IMT (originating under the EJP RD and maintained under ERDERA, the European Rare Disease Research Alliance). This contains myriad tools which can support translational research per se, which, although not created specifically for ERNs, nonetheless obviously covers many key topics and can provide support to ERN research. Tools in the IMT focus on key areas such as [robust project management](#) (e.g. the EATRIS Research Management Manual), understanding regulatory requirements, ethical and legal issues, etc.

And of course, as this Together4RD Toolkit is not intended to be exhaustive, and does not address every topic emerging from the 2024 consultations, it should be used in connection with other key resources, beyond the IMT, which address these priorities. For instance, the need for robust data management was unsurprisingly highlighted during the Together4RD consultations, and although there is no dedicated 'data' section in this toolkit, resources like the 2024 [Data Sharing Playbook](#) are available. The importance of engaging and involving patients and other people with lived experience in rare disease research cannot be underestimated, and again, a number of tools exist to support this, such as the 'Short guide on patient partnerships in rare disease research projects' generated through the EJP RD in 2020, and the Rare 2030 Recommendations (especially [chapter 5 on 'Patient Partnerships'](#))

SECTION A: Background knowledge - ERNs, Industry, and the Opportunity

Tool 1: [The Importance of Public Private Partnerships in Rare Disease](#)

[This resource](#) explains the importance of public-private partnerships or collaborations for the rare disease community, in the light of the needs of the rare disease field and the current climate around research and innovation.

Tool 2: [Examples of Initiatives which Foster Public-Private Partnerships in Rare Disease and Complementary Areas](#)

[This resource](#) presents examples of programmes and structures which facilitate public-private partnerships in rare disease or a complimentary area.

Tool 3: [What are ERNs?](#)

This Tool:

- Explains the origins of ERNs
- Highlights key resources concerning the conceptualisation, set-up and operations of ERNs, including the legal documents on which they are based
- Points to useful reports and recommendations concerned with analysing the achievements and added-value of ERNs to-date, which suggest where there might be room for improvement
- Points to key resources showcasing the achievements of the ERNs, as a whole, as well as where to learn more about the achievements of specific ERNs

Tool 4: The advantages of ERNs as partners for research

This Tool illustrates how and why ERNs hold so much potential for research. It is based upon content prepared for the Together4RD Position Statement of 2023, and has been updated to include the latest statistics and development connected with ERNs' research potential

Tool 5: Needs and priorities for Industry – and what does Industry need in a collaboration with ERNs?

This is a summary of important considerations for stakeholders less used to working with Industry, coupled with a selection of resources (webinars, reports, publications) to help convey some fundamental principles and realities for the private sector in contemplating collaborations with ERNs.

SECTION B: Conceptualising and firming-up a collaborative idea or research question or project

One barrier to ERN-Industry collaborations, especially for ERNs in fields with limited R&D and Industry engagement to date, is a difficulty in envisaging:

- a) The type of activities that ERNs and Industry might engage in, beyond the major programmes like IHI which support public-private partnerships broadly
- a) The mechanics of enabling collaborations – how can ERNs work with Industry when they are not legal entities?

This section of the toolkit has been designed to support the initiation of projects between ERNs and Industry, by firstly

- summarizing what the first 3 official Together4RD pilots, initiated in 2023, planned to do

- highlighting specific examples of public-private collaborations in the rare and highly specialised conditions space, outside of these pilots (largely pre-dating the ERNs)
- propose a range of activities, beyond the traditional clinical trial or more standard involvement in IHI projects, for instance, on which ERNs and industry could work together to generate added value

Tool 6: [Brief Summaries of the first Together4RD Pilots](#)

The first pilots showcased by Together4RD came about from an open call launched by the T4RD secretariat in the summer of 2022. All ERNs were invited to submit an expression of interest through a survey, which asked Networks to outline the nature of the proposed collaboration with Industry, the added-value of the activity suggested, readiness to begin, how many ERN centres might need to be involved, any existing assets, data, or infrastructure the pilot might build upon (and who owns these), etc.

This survey has been further developed to form Tool 11, a [List of Questions people could ask themselves when considering a collaboration between ERNs and Industry](#)

The Industry sponsors of Together4RD were also encouraged to submit the same survey, to outline their proposals for a pilot. The Together4RD Secretariat initiated discussions with all parties submitting a proposal, wherever possible matchmaking ERN ideas with an appropriate Industry partner, along with other actors deemed essential to the project proposals. These discussions led to the identification of three pilots deemed feasible for launch in 2023 – [Tool 6 – Brief Summaries of the first Together4RD Pilots](#)

The negotiations to evolve the initial ideas for a collaborative project have been, in all three cases, quite lengthy, unsurprisingly as these were the first pilots in this space. Important learning lessons have been captured, to streamline this process in future (see Tool X).

Tool 7: [Case Studies – examples of previous or ongoing public-private collaborations in the Rare Disease space](#)

Case studies for public-private collaborations in the Rare Disease space – beyond the T4RD Pilots

In addition to these initial 3 pilots, Together4RD has identified multiple case studies in the rare disease arena which may also serve as food for thought for other stakeholders wishing to follow suite and engage in projects.

A frequently-heard criticism of the two Statements on ERN-Industry interaction issued by the Board of Member States of ERNs, was that not only are collaborations between ERNs and companies essential to enable ERNs to fulfil their research potential, but that seeking

to avoid any interaction here is illogical and frankly a retrograde step for the field. For many years, experts argued, networks or other types of consortia or groups of non-industry actors, had been delivering successful projects and collaborations with industry, covering a wide range of activities. One of the first activities for Together4RD was to explore some of these prior examples of engagement and collaboration, and a set of case studies were included to the 2023 [Position Statement on collaboration between European Reference Networks and industry](#), largely in the form of supplementary material (supplementary file 1). These [case studies](#) have been revised and updated and are included here as a tool to support ERNs and industry in considering what kind of projects might be beneficial for their communities.

Tool 8: [Summary of areas or activities for potential ERN and Industry collaboration](#)

Stakeholders sometimes struggle to consider the range of activities on which ERNs and Industry could collaborate, besides the more traditional engagement around clinical trials, or more recently, partnering with multiple companies through the European Federation of Pharmaceutical Industries and Associations (EFPIA), in a large project via the Innovative Medicines Initiative, for instance. [This resource](#) builds upon material prepared for the comprehensive Together4RD Position Statement, to present a table listing a broad range of potential projects and activities which might be well-suited to ERN and Industry collaboration.

SECTION C: Practical knowledge transfer – initiating and delivering a well-developed research collaboration

Tool 9: [Report on the Experiences and Learnings from the first ERN-Industry pilots supported by Together4RD](#)

In 2024, the Together4RD Secretariat interviewed key individuals from both Industry and ERNs, about their experiences in launching the first 3 ERN-Industry pilot projects. These interviews were intended to better understand the respective experiences of conceptualizing and initiating these pilots – from who came up with the original idea, to how the project proposals have taken shape, covering activities up to the launch phase (approximately). The main insights are summarized in [this Report](#).

Tool 10: [Key recommendations for both ERNs and industry from the experiences of the first ERN-Industry pilots](#)

Based on the extensive interviews with stakeholders involved pilot project, a list of

recommendations has been distilled, to guide future ERN and industry projects and collaborations: [Key Recommendations for both ERNs and Industry from the experiences of the first ERN-Industry pilots](#)

Tool 11: [Questions people should consider when approaching a new collaboration between ERNs and Industry](#)

<https://docs.google.com/document/d/1ZfdGOjdARCZma7uWJzi0sOiCzL0pO9Q-/edit?usp=sharing&ouid=102195495958509421713&rtpof=true&sd=true>

Strategically-important issues beyond this Toolkit which emerged during expert consultations

The consultations and outreach activities undertaken by Together4RD over the course of 2024 highlighted certain issues or topics which, whilst ultimately not exactly fitting the brief for this Toolkit (in the sense of there being existing tools, or tools Together4RD could prepare alone, for a 2024/5 launch) they nonetheless deserve a mention here as they are very much connected to the overall mission of Together4RD.

Defining ERN-Industry Interaction

The Together4RD Position Statement highlights the challenges of *defining* ERN Research as one of the factors which, historically, has limited ERN research. The Statement explains that “how to distinguish the achievements of a given ERN, collectively, from the day-to-day achievements of its component centres (and, at a still more granular level, of the individuals involved in that ERN)’ has been something of a challenge when generating performance indicators to showcase many different activities of an ERN”.

If we look at the latest document concerning the 19 core indicators issued by the ERN Continuous Monitoring and Quality Improvement System (ERN CMQS), an example Indicator each ERN must report on is ‘Number of accepted peer-reviewed publications in scientific journals regarding disease groups within the ERN and which acknowledge the ERN’. The Definition for this is as follows: *“The total number of unique peer-review publications that have been accepted in scientific journals regarding disease-groups within the ERN and within the specified time period. Publications should be PubMed accredited scientific journals and involve as major contributors at least two Health Care Providers from two different Member States within the ERN, and which include an explicit acknowledgement of the ERN such as “This work is generated within the European Reference Network for ...” or “This work is supported by the European Reference Network for....”*”

Such specificity is clearly necessary to distinguish what individual experts would do even if the ERN did *not* exist from what may be deemed a collective ERN effort. It may be, therefore, that confusion over what constitutes research activity of an ERN has actually hampered research, or certainly hampered the reporting of this. **And just as research-related indicators have been somewhat overlooked to-date, there is rather a lack of criteria for measuring and capturing ERN-Industry interaction.** This issue is raised very often, when Together4RD events speak of past, present and future ERN-Industry engagement – how, precisely, does one define ERN-Industry engagement? As the Networks are not legal entities, a contract from ERN X with Company Y could not be a criterion. As more ERN-Industry projects and collaborations are launched, and the added-value of such activities becomes increasingly recognised by all quarters, some form of agreement on the basic parameters for counting or measuring this activity will be necessary. For instance:

- is the perception of the ERN coordinator that an activity is an ERN-Industry collaboration sufficient to make it ‘count’ and distinguish it from activity of a single investigator?
- should there be some stipulation that an interaction needs to *involve* more than one HCP of a given ERN, even if contracting is focused on one HCP with that one company?
- should there be a requirement that the project is discussed and approved in a working group of the ERN or that investigators from other ERN centres besides the HCP taking on the contracting are named in the project proposal or any MoU or similar?
- should there be a requirement for the project to be highlighted and agreed as an ERN activity in the Exec Board meetings or General Assembly?

[Together4RD believes that although such requirements may be raised in the course of its activities, a formal definition and agreement should probably be the task of the official ERN Monitoring activity highlighted above, with the eventual criteria agreed by all 24 ERNs](#)

It is important, however, that any criteria agreed in future should not be so strict as to actually hamper ERN-Industry collaboration.

Lack of opportunities for ERN and Industry Engagement to be initiated

One of the major points of consensus across the consultation activities which supported creation of this Toolkit, was the acknowledgment that **there is a lack of opportunity for ERNs and Industry to really understand what each other is doing – for this reason, Together4RD initiated very important discussions around ‘a forum for information**

exchange' (or perhaps a forum for collaboration).

It will be extremely important for such a forum, or fora, to be supported, in order to initiate more projects and collaborations between ERNs and Industry, beyond the kinds of large consortia projects funded via IHI, for instance. Although representatives of Companies occupying strategic positions, e.g. in EFPIA or EUCOPE, tend to be quite familiar with ERNs and their potential, most Industry representatives have generally been unable to participate in ERN meetings or workshops. And smaller biotech companies, lacking a strong European footprint, are likely completely unaware of what ERNs can offer. Together4RD proposed a range of options for a forum or for a to help foster research collaborations, ranging from more general events enabling people from all ERNs and Companies to connect, to much more specific and bespoke activities.

What might a Forum for Exchange or Collaboration look like?

Pan-ERN research Conferences

In the early years of ERNs, the European Commission organised 4 large in-person ERN conferences. Should these resume, it should be possible to invite Industry representatives to join the general event and thus hear about the latest successes across ERNs, collectively. Alternatively, some level of awareness-raising could be achieved by involving Industry in other key research conferences dedicated to showcasing ERNs and their achievements/interests.

Pan ERN-Industry strategy forum

Moving a step beyond simply inviting Industry to conferences as observers, a dedicated space could be created for representatives of all ERNs and Companies to come together via an 'ERN-Industry strategy forum'. ERN Coordinators and/or their research leads could meet once or twice a year with Industry representatives and patient representatives, to strategically discuss a subject of mutual interest, from a general (i.e. cross-disease) perspective, of interest to many or all Networks. The model of the EURORDIS RoundTable of Companies could be viewed as a starting point.

ERN-Specific Industry research forum

A more dedicated forum could be envisaged, to more specific and involved discussions between Industry and individual ERNs. (NB this would not necessarily need to be mutually exclusive with the previous ideas - one could envisage a shared event at first, which then focuses down and splinters into ERN-specific sessions, each involving representatives of the Companies most interested in/active in the area with which that ERN is concerned.) Such a forum could take place back-to-back with other meetings, such as the twice-annual ERN Coordinators' meetings, in which all ERNs participate; however, only coordinators and project

managers attend, which could be a drawback. Alternatively, such meetings could take place back-to-back with the annual General Assembly each ERN organises. This would be convenient, from the ERN experts' perspective, and all the key HCPs and researchers would, in theory, be present, which is an advantage; naturally, there would be parts of the GA itself which would need to be closed off.

If scheduling back-to-back does not make good sense, and people were willing to make space for additional meetings in their calendars, one could envisage standalone meetings convened purely for this purpose.

One low-hanging fruit, potentially, is to arrange meetings as part of major disease-oriented conferences (e.g. those run by scientific societies), at which ERNs might routinely bring a booth and a contingent. The feasibility and usefulness here depends on how many of the ERN researchers would actually be present at these conferences. A formal meeting should perhaps be scheduled, as opposed to merely informal drop-ins at the ERN booth. But even the latter would be a step in the right direction.

Wherever they take place, these kind of strategic meetings could perhaps take some inspiration from the events organised by the [ACCELERATE initiative](#) in the paediatric cancer field, and the [Multi-Stakeholder meetings run by the IMI project c4c](#). (Different types of agenda could be envisaged for communities in which there is less research activity and therefore less of a need to prioritise compounds or approaches.)

ERN-specific research matchmaking events

An even more hands-on version of a forum to foster ERN-Industry dialogue would be to envisage cross ERN or ERN-specific research matchmaking events, along the lines of the French 'Proof of Concept' club for rare diseases. Here, a collective of researchers from an ERN could actually pitch their research proposals to Industry colleagues, and a trusted third party would facilitate appraisals and support the development of ERN-based research emerging from the POC meetings.

Together4RD has already advocated for a forum for exchange of information, along the lines of some of the options outlined above. However, it will be important for ERNs and Industry to take the initiative -either under the aegis of an initiative like Together4RD or otherwise- to realise some of these opportunities, to help initiate future proposals and maintain the momentum created by Together4RD.

Thinking to the future - what role could/should a trusted third party play in supporting ERN-Industry collaborations?

During the consultations of 2024, and indeed beyond, the question of whether or not a trusted 3rd party (which would be a legal entity) would be useful in supporting ERN-Industry collaborations in future was quite divisive. Some view this as very useful, in fact essential, in order to scale-up the volume and scope of future ERN and Industry engagements.

“The third party role in a PPP is critical, both conceptually and in practice”

“A third party could provide experts in negotiation, PPPs, contracting, IP etc. This would be very helpful”

Others however see potential to slow things down and overcomplicate collaborations.

“No - this could block or slow down effective collaboration”

“Better to have a lean coordination without ‘heavy’ infrastructures”

The consultations highlighted a range of existing organisations that could potentially take on such a role, ranging from translational research infrastructures used to working in the rare disease space, to paediatric infrastructures and services, to small consultancies, foundations and CROs. Some suggested it would be wise to look to a *single* dedicated third party, or cluster thereof, to specialise in supporting ERN and Industry collaborations in future, in order to build critical mass of knowledge and experience. And indeed, a further suggestion was to create or appoint something quite new, such as a team established at European level for this very purpose, which also included legal specialists, clinicians, and patient representatives. Several experts also noted that it would be useful to explore more precisely how ERN-Industry collaborations might be delivered through the intermediary of Medical/Scientific Societies – especially where an educational activity is envisaged. To this end, it would be useful to know which ERNs consider themselves to be working with Industry through Medical Societies (or feel this is a good structure for the future).

In summary, however, for the purposes of this Toolkit, it would be premature to attempt to provide any strong stance on the desirability and feasibility of this, nor propose suitable third parties to play an intermediary role. **Together4RD recommends that such discussions are prioritised in future by the research structures most closely connected to the ERNs themselves, utilising the resources in this Toolkit (and especially, the ‘lessons learned’ from the Together4RD pilots).**



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