101057442 – REMEDi4ALL

Building a Sustainable European Innovation Platform to Enhance the Repurposing of Medicines for All

WP3 – Training & Capacity Building

D3.1 Training Landscape Map

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Document History

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V0.2	03/08/2023	Second draft sent to reviewers.
V0.3	16/08/2023	Third draft sent to SC members.
V1.0	30/08/2023	Final version.
V1.1	11/10/2023	Updated version sent to SC members.
V2.0	25/10/2023	Final version updated.

Table of Contents

Definitions

- **Consortium -** The REMEDi4ALL Consortium, comprising the above-mentioned legal entities.
- **Consortium Agreement -** Agreement concluded amongst REMEDi4ALL participants for the implementation of the Grant Agreement. Such an agreement shall not affect the parties' obligations to the Community and/or to one another arising from the Grant Agreement.
- **Curriculum –** The package of training that aims to educate all stakeholders involved in general drug development on the specificities of drug repurposing in all its stages and forms. This training will be coordinated and developed by REMEDi4ALL partners.
- **Curriculum Setting Meeting (CSM) –** A meeting of representatives from all REMEDi4ALL partners to discuss the key themes, topics and methodologies for REMEDi4ALL to develop training. The meeting included representation from all key stages of the drug repurposing pathway.
- **Grant Agreement -** The agreement signed between the beneficiaries and the HADEA for the undertaking of the REMEDi4ALL project, with agreement nº 101057442.
- **Project -** The sum of all activities carried out in the framework of the Grant Agreement and its Annexes.
- **Repurposing Community –** Anyone with an interest or active role in drug repurposing. These are the individuals that the curriculum aims to engage and educate.
- **Target Product Profile (TPP) –** Set of characteristics outlining the desired characteristics of a drug product that is aimed at a particular disease or diseases. TPPs state intended use, target populations and other desired attributes of products, including safety and efficacy related characteristics and are a useful multistakeholder communication tool.
- **Training Landscape Map –** A comprehensive assessment and database comprised of the existing training relevant to drug development and drug repurposing. The Training Landscape Map is a living project that will be updated and amended as the landscape evolves externally to REMEDi4ALL.
- **Work Package 3 (WP3) –** The work package within the REMEDi4ALL consortium committed to training and capacity building for stakeholders across the drug repurposing ecosystem.
- **Work plan -** Schedule of tasks, deliverables, efforts, dates and responsibilities corresponding to the work to be carried out, as specified in the Grant Agreement.

Abbreviations

Acronym / Abbreviation	Meaning
WP	Work Package
EUPATI	European Patients' Academy on Therapeutic Innovation
PIs	Principle Investigator(s)
ROADMAP	Repurposing Of All Drugs, Mapping All Paths
EJPRD	European Joint Programme on Rare Diseases
IMT	Innovation Management Toolbox
IRDiRC	International Rare Diseases Research Consortium
R&D	Research & Development
HTA	Health Technology Assessment
ePAGs	European Patient Advocacy Groups
ERNs	European Reference Networks
TPP	Target Product Profile

Abstract

To understand what role REMEDi4ALL's training will serve, it was crucial that there was an in-depth assessment of pre-existing training both specifically covering drug repurposing and covering different aspects of the drug repurposing/translational research pathway. Work Package 3 (WP3) believes that a strong training platform will not reinvent the wheel but use a collaborative approach and robust signposting to ensure REMEDi4ALL's offering complements and enhances the existing training ecosystem.

A number of methods were applied to build as full a picture of the current training landscape as possible. This included guided, targeted searching, one-to-one interviews, and the formation of a new, user-friendly and focused database of training.

While many resources exist that offer signposting, information and guidance on drug repurposing, there is a significant gap in the formation of a centralized education and training platform that brings all stages of the process and all stakeholders together to offer a fully accessible and collaborative knowledge base. REMEDi4ALL's training and education work package should aim to fill this gap.

1. Introduction

This deliverable provides a detailed summary of **currently available training resources within the fields of drug repurposing and translational research.** Through in-depth mapping, categorisation, and analysis we have selected several key resources relevant to REMEDi4ALL stakeholders and analysed where training gaps exist to begin the planning of our REMEDi4ALL training curriculum. Furthermore, we have produced both an internal and external signposting tool that allows REMEDi4ALL to signpost the most relevant training resources to our repurposing community in the coming years of the project, both through our own website, and our more direct interactions with stakeholders (webinars, in person meetings, conference attendance etc.). This tool has been designed to be sustainable, with regular updates retaining its relevance throughout the project's lifespan. All of this ensures that REMEDi4ALL's training programme can initially be developed to address the major needs in the field, while leaning on the best examples of existing training available in the ecosystem.

We advise that, while reading this document the reader has access to:

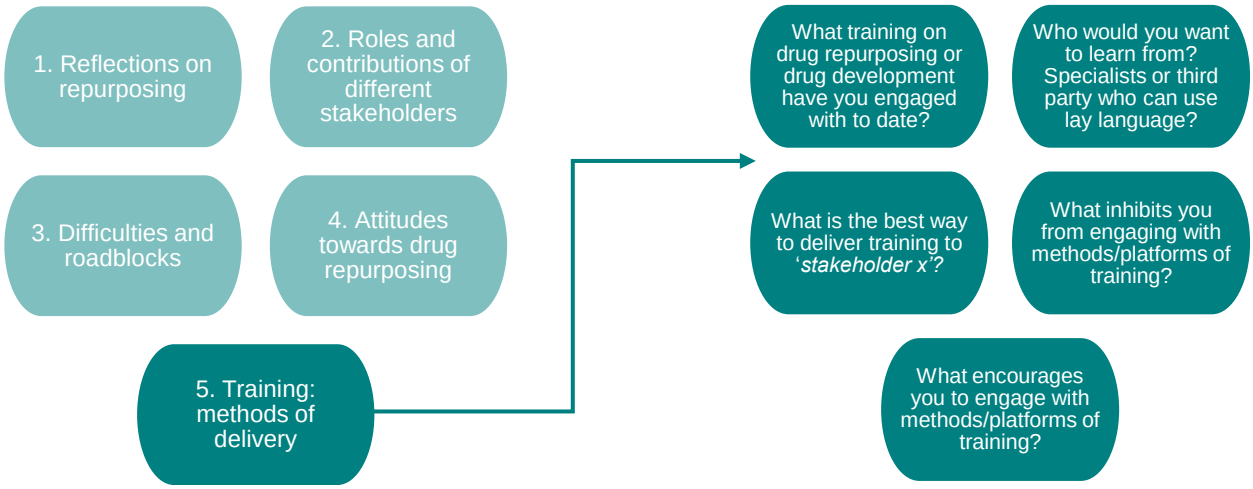
- The Excel file that comprises our Training Database (Existing Training Database)
- The 6 key listed resources in section 4, hyperlinks provided and otherwise accessible via web browsers

2. Methods

2.1 Targeted interviews with external and internal partners

To understand what the current mood of learners is in relation to the available depth, breadth and delivery methodologies of existing training, a number of external interviews and focus groups were undertaken, ensuring the wide range of stakeholders involved in the drug repurposing pathway were represented in this initial 'market research'.

Our interviews were divided into 5 broad categories. Most relevant to the questions this document seeks to address were those asked in category 5. 'Training: methods of delivery'. The aim(s) of the questions asked within this category were to explore what training methods each stakeholder had experience with, which training methods were effective or ineffective and what would incentivise individuals or organisations to participate in future training from known or new providers. The key goal here was for us to understand what training exists, is being accessed, and is needed by different stakeholders. A list of interviewees can be found in *Annex I*.



2.2 Survey to consortium partners

A survey was sent to all internal members of the REMEDi4ALL consortium to secure recommendations of existing relevant training resources. This included training not specific to drug repurposing, but specific to the drug development journey and translation in general. Recommendations thus far span pre-clinical development through to regulatory and market approval.

The lead organisations in WP3 (BEACON & EURORDIS) have a strong bias towards knowledge and awareness of resources in the space of rare disease and patient engagement; by circulating this survey to consortium partners we aimed to alleviate some of the effect of bias on our training landscape research through online searches (outlined in 2.3 and 2.4) by reaching out to experts in other stakeholder areas.

The survey was left intentionally broad – asking for the training provider, how to find the training and a few comments or thoughts on the recommended training, if appropriate. A copy of the survey can be found in Annex II.

2.3 Database of key resources

An excel spreadsheet was compiled to collect all the available training resources in the drug repurposing sphere that could be found from November 2022 – June 2023.Consequently, the information found and reported only provides a snapshot of the current situation. This changing landscape will evolve over time, these changes should be captured throughout the life of the REMEDi4ALL project.

2.3.1 What to look for?

The main search goal was to find relevant existing training for the REMEDi4ALL platform. Hence, we looked for drug repurposing-related resources, without searching specifically for a topic within this field, but with key information related to it. So, data was collected through basic Google searches, typing ‘drug repurposing trainings’ and ‘drug development trainings’.

From there, we refined our search by targeting specific training resource types such as ‘drug development courses’ and ‘drug repurposing webinar’.

The first training resources found were more general, covering different steps of the development pathway. This allowed us to expand our search by looking for resources on more specific topics like ‘training in drug development and regulation’ or ‘research methodologies in drug repurposing’.

2.3.2 Who are these training resources for?

Learning who the training resources were targeting was a priority of our research as we wanted to understand which stakeholder groups were receiving less training and which stakeholders were benefiting the most from the training landscape. In general, we collected the characteristics of the training resources, outlined in sections 2.3.5 and 2.3.6 as well as their target audience from their description, outlined in 2.3.7.

Consequently, we also looked specifically for relevant training that targeted specific groups. Thus, latter searches followed the structure ‘topic of interest and a concrete target group’, for example, ‘drug development training for researchers’ and ‘drug repurposing training for patients’.

2.3.3 Where to search?

Initially, the Google search was broad and not focused on any training platform or organisation. However, when our basic searches only identified paid resources or only those resources from top universities (often including PhD, Masters or college degrees), we expanded our search by looking

specifically on specific platforms that usually offer specific high-level training, or free of cost training such as Coursera or FutureLearn.

This approach allowed us to find relevant resources for the future REMEDi4ALL training that cover different steps of the repurposing pathway. We followed this search by outlining a list of institutions known to offer training (webinars, short courses, residential training etc) within the UK, such as Cambridge University and within the EU such as the European Patients' Academy on Therapeutic Innovation (EUPATI).

Our research was guided by a flexible and adaptable process, with the aim of collecting and characterising the key available training resources that would then help us shape our future curriculum *(see Annex III- Non-exhaustive list of major search terms).* Therefore, crucial elements framing our research included the non-specific topic selection (but with a clear drug repurposing and translational research focus in mind), the audience the training was targeted to (all were considered), and the site or organisation hosting the training (all were considered). The search was expanded and adapted on the way to ensure representativeness and that quality and accessibility aspects of training found were collected.

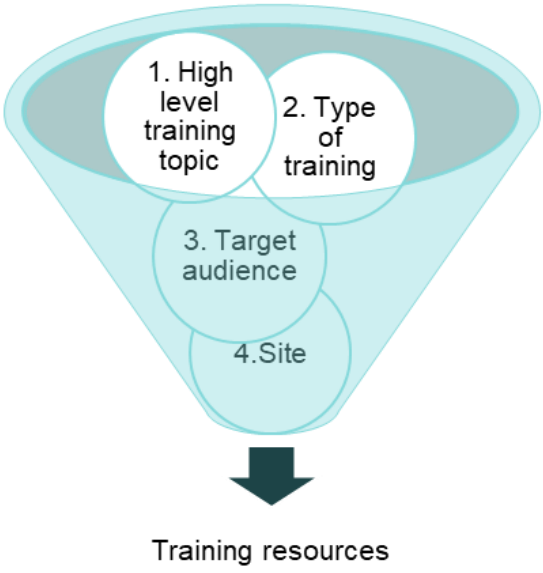


Figure 1. Filtering the search for training resources

2.3.4 Topic alignment

All training resources identified were categorised based on the alignment of their content to the core aims of the REMEDi4ALL training work package. There are two primary categories, with the latter itself subdivided:

1. **Core –** Core training represents training resources that strike to the heart of our purpose in the REMEDi4ALL consortium: providing direct guidance on the pathway to deliver treatments from concept to the patient. As such trainings that focus directly on drug development, translational research, and drug repurposing are defined as **core trainings**. They are likely to be of broad appeal to our community, and to shape the production of REMEDi4ALL's own resource.
2. **Tangential –** Drug repurposing spans a great breadth of skills and disciplines. As such, much of the training identified focusses on a limited portion of the developmental pathway and could apply to research that is outside of the translational pathway. These training resources are all defined as **tangential training**. They are not the core focus of REMEDi4ALL but may be useful to a subset of our community focussed on a specific project stage, or from a specific audience.

As tangential training represents the bulk of the training landscape, it was subcategorised as follows to simplify signposting, and enhance its value to the consortium:

a. **Clinical trials –** Training on clinical trial design and delivery.
b. **Data –** These trainings provide skills and knowledge in handling large datasets, which can be of relevance to drug repurposing projects in the identification of opportunities.
c. **Disease Areas –** Training relevant to specific disease types (note that rare disease is given its own category due to prevalence).
d. **Funding –** Training that can support project leads in securing necessary research funding.
e. **Identifying repurposing opportunities –** These training resources provide specific advice and guidance on *in silico* and *in vitro* screening techniques for drug identification.
f. **Market Access –** Training focussed on business models for drug development, market access, and reimbursement.
g. **Patient engagement –** These training resources provide guidance on the establishment and maintenance of effective patient relationships, and patient-led research.
h. **Population Health –** Training that looks at population health considerations in drug development.
i. **Rare disease –** Training that explores the specific challenges of the rare disease space.
j. **Regulation –** Training covering the regulatory landscape, and routes to market access for drug development, and repurposing.
k. **Research methodologies –** These training resources are specifically targeted on upskilling academic researchers, expanding their knowledge of specific aspects of the drug development landscape or research techniques.

2.3.5 Further categorisation

To better understand the landscape, the following categories were used to characterize the identified training resources:

Name and organisation	Timeline and duration [1]	Type of training [2]	Location of delivery [3]	Target audience [4]	Cost of training [5]	Field of training [6]	Available certification [7]

[1]On demand training or training with a specific time duration.
[2] Webinar, course, toolbox/guidebook, other (e.g., mentoring). See definitions below.
[3]Training is available online, in person, or hybrid.
[4]Training is targeted to students, industry, professionals, researchers, public. See definitions in figure below.
[5]Free or if it has any cost.
[6]Topic or subject of the training.
[7]A certificate of completion is available or not.

2.3.6 Type of training classification

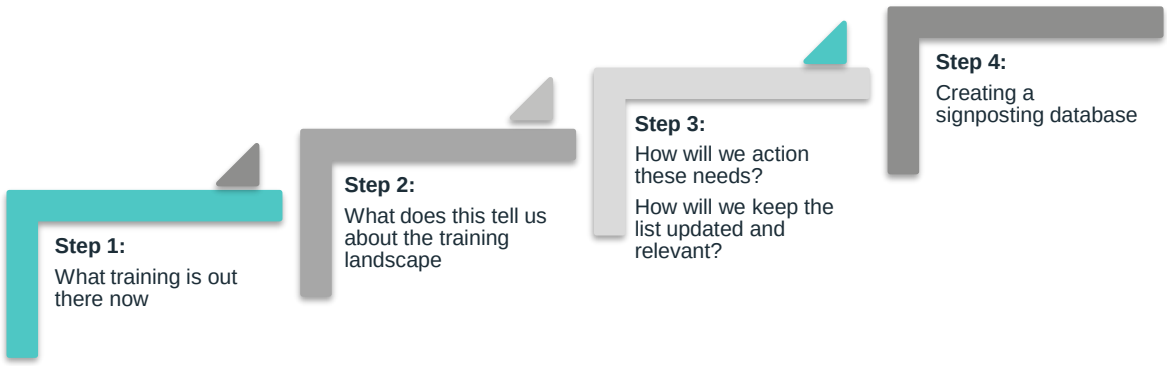
Course	Online or in-person learning laid out in chapters or modules with a finite end date and/or assessment once completed. A participation or attainment certificate is available when completed.
Webinar	Exclusively online learning attended by people via internet. The information is presented by speakers or recorded and available to attend live. Short format, time commitment is less than 4 hours including questions
Platform	Online learning resources that present many functions – text, video, chat, available on demand with no or very few live elements.
Guidebook/Toolkit	Online learning resources containing a wealth of information tailored to its target audience (e.g., LifeArc toolkit). This could be a single webpage with many headings, primarily text-based.
Others	Other formats such a mentoring (which involves direct contact with person/people who are organising the training that have previous knowledge or experience with the topic in question) or interview videos.

2.3.7 Target audience

Students	Have studied the topic to some degree and/or have experience in some relevant fields
Industry	Includes pharmaceutical and biotechnology professionals
Professionals	Support services e.g., specialist nursing staff, genetic counsellors, clinical trial management teams
Researchers	Clinicians and academics - people who are researching or have experience with a relevant or similar topic
General Public	Includes patients and families, people with little to no experience in the field
Patient Groups	Patients and families with more experience/knowledge than the public. Could be considered semi-expert by experience.

2.4 Compiling a list of existing training resources

2.4.1 Schematic showing assessment of existing training



2.4.2 Using the landscape map

Having identified a wide range of available training in, or tangential to, the repurposing space, and identified the relevant gaps and needs that REMEDi4ALL should aim to address, we next wanted to understand the value of the listed training resources to the repurposing community, and how REMEDi4ALL could make use of it.

With a major aim of REMEDi4ALL to improve the knowledge of the repurposing ecosystem, to help more repurposed drugs deliver patient benefit, it is crucial that we identify the most useful, relevant, and complementary training currently available. In doing so, REMEDi4ALL will be able to:

- Develop our own training materials to complement and build on the existing best-in-class training materials.
- Signpost the repurposing community to pre-existing quality training resources, both before REMEDi4ALL's own resources are available in the short term, and alongside our own materials in the mid-term.

To achieve this, we conducted a further review of the training landscape materials to understand their value to the repurposing community and the REMEDi4ALL project (see Signposting categorisation below). In doing so, we identified a range of different approaches to signposting the resources to maximise the benefit to our community, without a perceived recommendation or endorsement of an existing training programme where none was due.

As part of this process, the longevity of each identified training resource was assessed, and a monitoring approach was assigned. We defined the periodicity to check the availability and status of all listed resources, to ensure relevance of our existing database. Finally, we developed a simple protocol to conduct additional searches for the training landscape throughout the project, so that new training could be identified.

2.4.2.1 Signposting categorisation

To assess the value and use of the identified training opportunities to REMEDi4ALL and our community, we defined three core categories for training that would govern how, and with whom, the courses would be shared.

1. **Open –** These training resources have the broadest applicability to REMEDi4ALL and our repurposing community. They are accessible, maintained, and cover either a broad part of the repurposing pathway, or focus on key aspects. They should be openly shared and signposted through the REMEDi4ALL website and training programme. A training – specific page within the REMEDi4ALL website is currently under development.

2. **Targeted –** These training resources are relevant to a subset of our community, either based on the stage of repurposing they are interested in, or their own background. Some have more limited accessibility – either through language or cost – while others may have a more limited perspective on their topic, having been devised by a limited number of individuals. As such, these training resources will not be freely and openly promoted to the community of the REMEDi4ALL website, however they will be sign-posted as useful resources to a targeted audience. This could take the form of:
    a. A training referral to patient champions by the patient engagement team.
    b. A citation of additional training resources within a specific and relevant piece of REMEDi4ALL training.
    c. A recommendation to a researcher in a Repurposing development team meeting.

    As such, all targeted training must be readily available within the REMEDi4ALL consortium and maintained regularly.

3. **Monitored –** Monitored training resources are those that could prove useful to the training work package for training development or could be the source of more relevant training in the future but are not suitable for direct signposting at this stage. These training resources will be monitored regularly to identify new opportunities.

Alongside this, we also identified several resources that were recordings of recent live online training. These types of recordings can be very valuable to the correct audience but are never updated – so their value is reduced over time. We termed such resources **case study webinars**, as an additional subtype of training that is likely to be particularly useful to our community before the REMEDi4ALL training programme has officially launched.

2.4.3 Maintaining the signposting resources

To build a training map of value to both the REMEDi4ALL consortium and our external community, it is essential that the map remains current. Updating the map and monitoring the availability of training resources is therefore crucial to its success.

To ensure this relevance, each training resource was categorised to define our approach to monitoring as follows:

1. **Collaborative updates –** REMEDi4ALL should work collaboratively with the producers of this resource, to ensure alignment between resources, and to allow us to highlight training updates to the REMEDi4ALL community.
2. **On-demand resource, review annually –** This is an on-demand resource, likely to remain widely available and stable online. It should be reviewed at least annually to ensure it remains available, accessible, and supported by the host.
3. **Ongoing monitoring –** These resources should be monitored continuously, at the very least checking in monthly for updates or new training opportunities.
4. **Reoccurring live event, define scheduling window –** This is a live training event, which occurs at a specific time and location. We should define the delivery date for each project, and after completion carry out a process of ongoing monitoring to identify the next edition.
5. **Review at key date –** This resource is likely to either be valuable for a fixed period, or to be superseded by REMEDi4ALL's own training resources. As such we plan to review its value at a specific time point (for example end of year 2), at which point we can decide whether to keep signposting to it. Most **case study webinars** will fall into this category.

To ensure that all monitoring of training resources is completed as planned, the REMEDi4ALL Training Landscape Map will be converted to a Monday.com board which will include monitoring date fields. This will generate automated email alerts to relevant team members to ensure monitoring is completed at an appropriate time, and in the correct manner. The Monday.com board will be maintained by BEACON and EURORDIS consortium members along with some wider WP3 members who have an active role in curriculum development and execution.

2.4.4 Preliminary Signposting Database

To enable content to be uploaded to REMEDi4ALL's training pages, hosted within the main REMEDi4ALL website and publicly available, as soon as possible, the training landscape map has been additionally filtered based on the aforementioned categories.

Those falling into the Open Signposting and Open Case Study Webinar signposting categories will form the preliminary signposting dataset.

2.5 Assessment of existing tools that will run parallel to REMEDi4ALL

To explore, in depth, the scope of current training resources that are either entirely drug repurposing focused or contain an element of drug repurposing content, we assessed 6 resources filtered from the main training database and that form the core of our signposting database. Our assessment focused on the overall usability, content, and accessibility. The full analysis can be found in section 4. The chosen resources are:

- European Joint Programme Rare Disease (EJPRD) – **Innovation Management Toolbox**
- Castleman Disease Collaborative Network (CDCN), Chan Zuckerberg Initiative (CZI), and Every Cure - **Repurposing Of All Drugs, Mapping All Paths (ROADMAP)**
- LifeArc – **Repurposing Medicines Toolkit**
- EURORDIS – **Open Academy**
- Beacon – **Resources Hub**
- IRDiRC – **Drug Repurposing Guidebook (in development)**

2.6 Observing and Documenting the Changing Landscape

As well as ensuring that the training currently in the landscape map remain relevant, we will need to add new training to the map. There will be three main pathways for these updates:

1. **Consortium and collaborator recommendations –** BEACON will maintain a simple web form for consortium members to highlight any new training opportunities they have identified, and to share with collaborators or programme users who have their own recommendations. These suggestions will then be reviewed, categorised, and added to the training map, monthly.
2. **Regular online searches –** We will re-run the online searches used to identify the existing training map every 6 months and identify any significant new training resources for incorporation into the map.
3. **New targeted searches –** Based on user feedback and community need, we will conduct new training searches to expand the database and incorporate these searches into our on-going search maintenance programme. For example – it may become apparent that a set of detailed training on health technology appraisal is required for all REMEDi4ALL project champions, or PIs. We would then work with consortium experts to conduct searches and identify the most relevant training for either **open** or **targeted signposting** within the landscape map.

Like the maintenance of existing resources, the regular updates of the landscape will be monitored through our Monday.com landscape map. Automated email alerts will drive the update cycle.

We should continue to actively seek new collaborations with resources, consortia, etc. that will see significant changes to the training landscape as it stands presently to ensure we are appropriately signposting to these or adapting our curriculum as a response.

3. Results

3.1 What Training is Out There?

A total of 56 training resources were identified during our research and collected into our internal database (*see section 3.2).* The categorisation established allowed for analysing them and highlighting the following findings.

3.1.1 Training field

A little bit less than half of the training resources found (41%) fell under the 'core' category (*see Figure 2)* meaning they are of transversal importance within REMEDi4ALL, and somehow cover the end-to-end journey of drug development, with a possible focus on translational research and/or drug repurposing.

On the other hand, most of the resources identified fall out of the translational pathway and are tackling some specific steps of the developmental pathway (for example, patient engagement, regulation, or market access). Those training resources account for 59% of the resources, categorized within 'tangential' training.

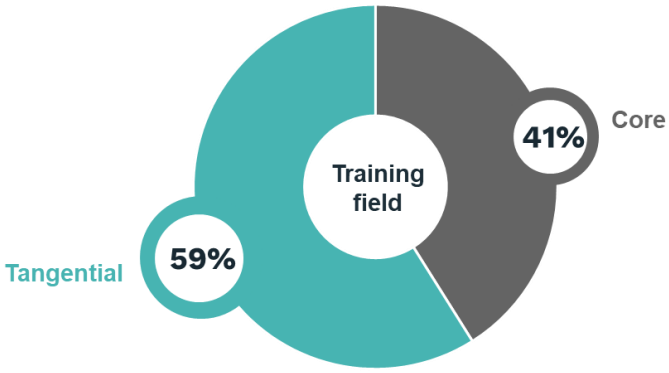


Figure 2. Training field

A great breadth of tangential topics was found (*see Figure 3*). Most of them were focused on the patient engagement field (n=15), followed by rare diseases training (n=4) and training on regulation (n=4). Few resources were found for the subcategory 'identifying repurposing opportunities' (n=2) and 'research methodologies' (n=2). Finally, specific disease areas, data management, market access, population health, clinical trials, and funding, were the subtopics where only a single resource was identified in the landscape.

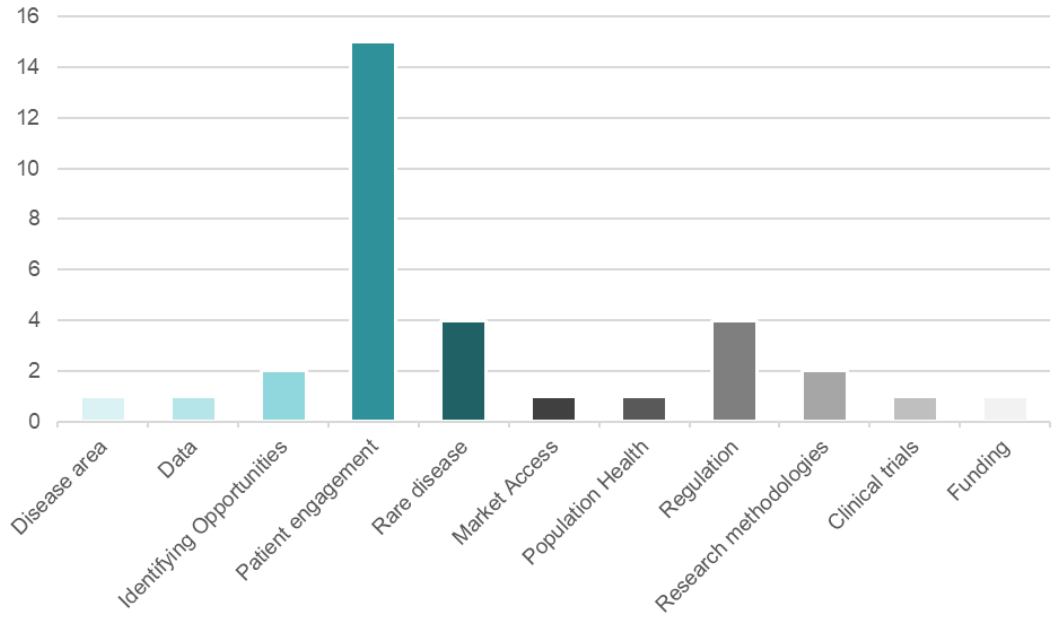


Figure 3. Number of tangential training resources

3.1.2 Target audience

When looking at the target audience of resources *(see section 2.3.7. Target audience for more information on the categorisation of the target audience),* we found that there is a training offer for a wide range of stakeholders (*see Figure 4)* that are involved in different steps of the development journey.

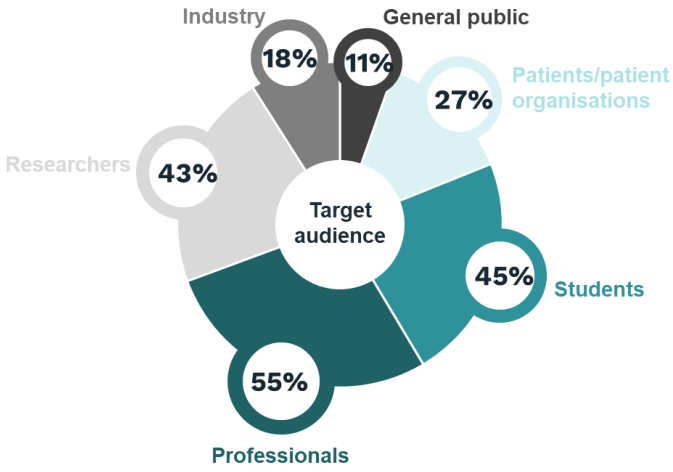


Figure 4. Target audience

We found that most of these resources (55%) were for life science professionals followed by students (45%) and researchers (43%).

Some of the training resources also tackle other audience groups less frequently, such as patients, patient groups, and patient organisations (27%), and even less often to industry (18%) or the general public (11%).

We found that this pattern applied both for core training and tangential training (*see Figure 5)*, where in both cases, professionals were the most targeted group together with researchers and students. For both fields of training, there was little focus on the general public, the industry and patients or patient organisations. However, for this last group a difference could be appreciated; more tangential (n=12) than core training resources (n=3) were targeted to them, meaning that many training resources on specific topics are aimed at reaching patients and patient groups attention.

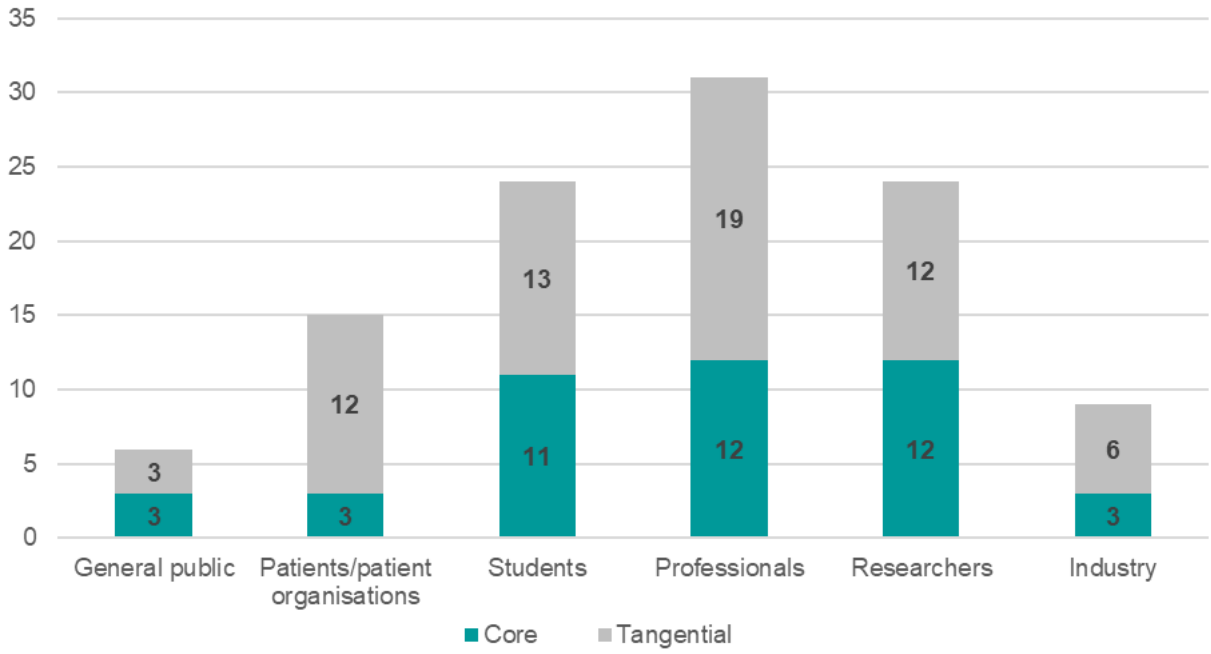


Figure 5. Number of training resources by target audience and field of training

Figure 6 shows the distribution of the training fields per stakeholder group. We found that at least one core training was available for every audience group but not all tangential training was tackling every stakeholder. Patients and patient organisations, researchers, and industry (groups outlined in red) are the groups of audience that through our curriculum setting meeting held in March 2023, and internal discussions with experienced professionals in the training field and the consortium partners, arose as key stakeholders to tackle through our curriculum. For this reason, it was especially interesting to see which main training topics were offered to them and which are still lacking.

We found that patients and patient organisations received mainly patient engagement training (n=8), very little core resources (n=3) and a few training courses on specific topics such as regulation (n=1), funding (n=1) and research methodologies (n=1). No training resources were found specifically tackling this audience group for relevant subjects like clinical trials (n=0), the handling of datasets (n=0) or market access (n=0).

Conversely, researchers seemed to have a wider offer of subject training (almost one training of each field was targeted to them) but not many options to choose from in every field. Core training was very frequently targeted to researchers (n=12), and patient engagement training was also quite often offered to researchers (n=5). However, other key training on specific topics relevant to researchers

like identifying opportunities (n=1), population health (n=0), the handling of datasets (n=1), regulation (n=1) or funding (n=1) were very rarely offered to this group.

Finally, we found that not many resources were tackling the pharmaceutical industry. Core training (n=3) and patient engagement training (n=4) were the fields that industry could benefit from the most. However, just as for patient organisations, few core training courses (n=3) were targeting this stakeholder group. Important topics to this audience like funding or information on the rare disease space were training subjects lacking dedicated for the pharmaceutical industry.

It is important to note that training individuals working in industry may pose a significant challenge as, through our interviews with representatives from larger pharmaceutical companies, we discovered there is access to and encouragement to utilize internal training resources that are developed by the company themselves. In larger companies that have a breadth of staff (spanning scientists, lawyers, and regulatory experts) there is already an internal culture of sharing expertise.

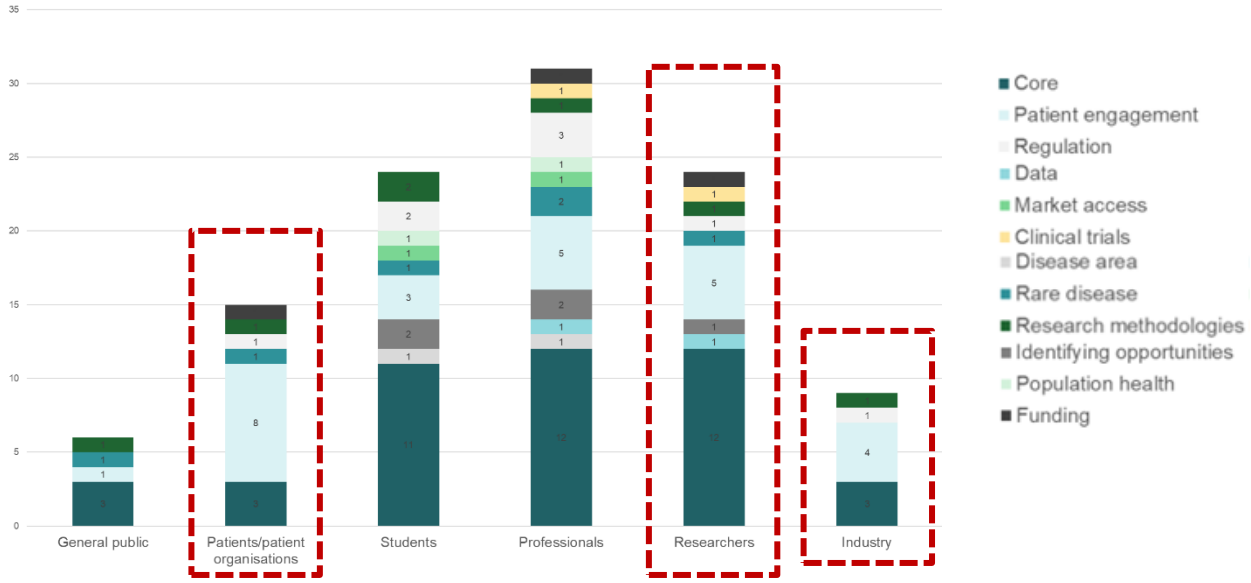


Figure 6. Distribution of training field by audience

3.1.3 Type of training

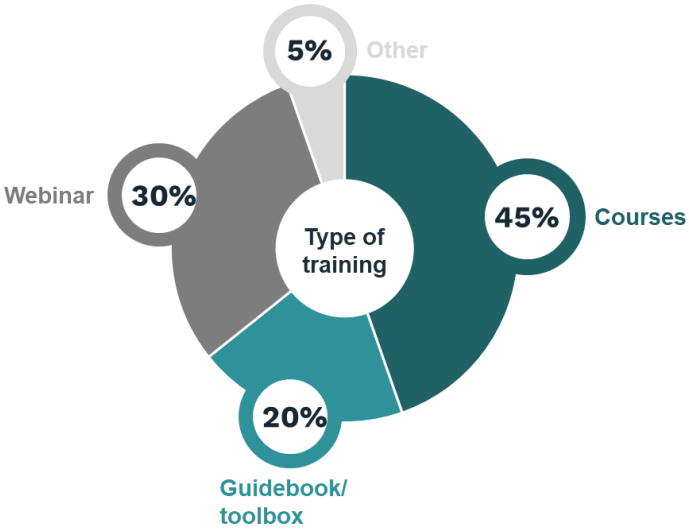


Figure 7. Type of training

Different formats of training were found in our research *(see section 2.3.6. Type of training for more information on the categorisation of the different formats)*

Figure 7 shows that courses were the most common format for delivering training (45%), and this was followed by webinars and/or webinar series (30%), and guidebooks/toolbox (20%), which were the third most used format to deliver training.

Lastly, other types of formats such as mentoring were also present but represented a minority, accounting only for the 5% of training materials being delivered through this format.

3.1.4 Timeline

We saw that most of the training resources found tended to be available on-demand (*see Figure 8)* (84% of them) allowing the trainee to learn in a self-paced mode.

However, 16% of the resources were live, taking place at a specific date and time.

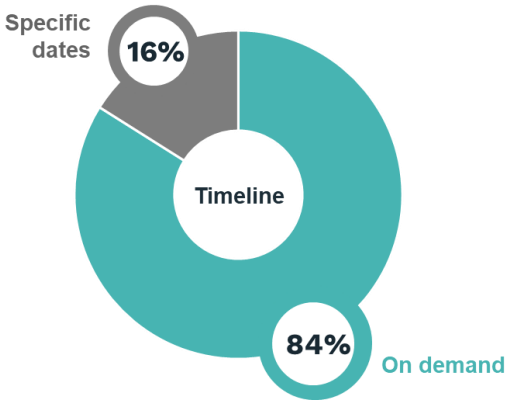


Figure 8. Timeline of training

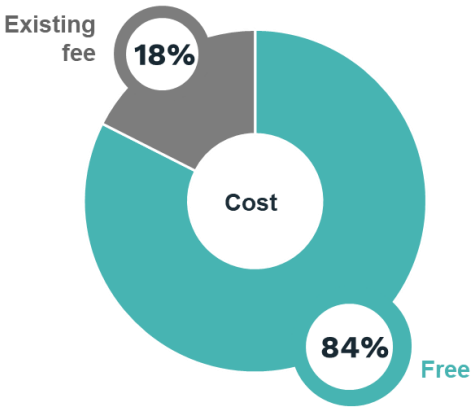


Figure 9. Cost of training

3.1.5 Cost

The training resources found were mostly (84%) free of charge (*see Figure 9)*, so there was no fee for registering nor accessing the content of the training (though note our search strategy did actively pursue such training),

Some of the resources found (18%) did have a registration fee to be enrolled or have access to its content.

3.1.6 Location

The majority of training and resources were available online (89%) (*see Figure 10)* and only a few were offered in person (7%) or in hybrid mode (4%). If we examine courses specifically (*see Figure 11),* as the only format that can be offered in different locations, we also found that most of them (80%) were offered online, a few less in person (16%) and only 4% of them were delivered in hybrid format.

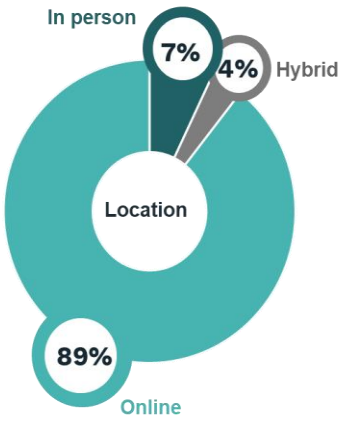


Figure 10. Location of all training

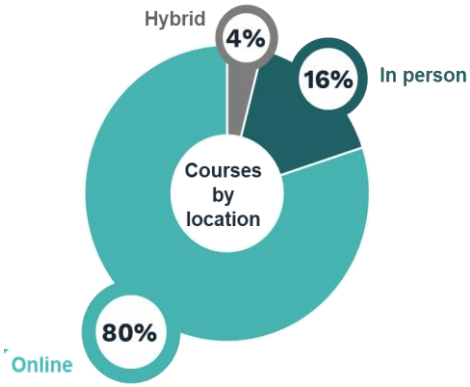


Figure 11. Location of courses only

3.1.7 Certification

Whether a certificate of completion of the training resource was offered or not, was only explored for courses (*see Figure 12)*, as in the current training landscape it is the only resource where a certificate could usually be expected.

We found that most training courses (68%) do offer a certificate after completing the training. For some of them this recognition was not free, and an additional fee for obtaining it was in place. On the other hand, we also found that many of the identified courses (32%) did not offer the possibility to obtain a certificate of completion of the course.

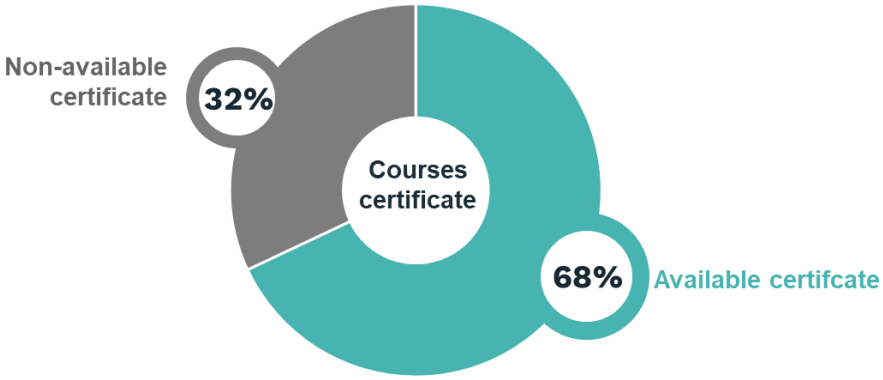


Figure 12. Courses certification

3.2 The Training Landscape Database

The following 7 pages show the training database in full. This is the raw form of the data collected in the methods outlined in section 2.3.

The categories chosen to filter the training database are the elements which we believe would be most important to any individual or organisation when searching for educational materials in any given field, not specifically drug repurposing.

These categories are explained in further detail throughout this document and justification of their selection is provided. The hope is that as a living document, the training landscape map can continue to be refined and updated and provide the basis of a tool that provides a fully searchable, filterable, and user-specific training signpost based on need.

The training landscape database will be available to all consortium partners who are encouraged to use the database to find training relevant to them and to add any training they have undertaken or encountered that they believe is relevant to the community.

Name	Organisation	Topic alignment	Type of training	Location	Timeline	Target audience	Cost	Sign-posting type	Maintenance
Introduction to Translational research for Rare Diseases	FutureLearn	core	Course	Online	5 week course	Researchers and students in medicine and health-related research fields, health professionals, patient advocacy organisation representatives	Free for 'Basic learning', £19.99 subscription fee for completion certificate and results	Open signposting	On demand resource. Review annually.
Clinical Pharmacokinetics: Dosing and Monitoring	FutureLearn	core	Course	Online	6 week course	Students / professionals with baseline knowledge	Free for 'Basic learning', £19.99 subscription fee for completion certificate and results	Open signposting	On demand resource. Review annually.
LIfeArc Toolkit	LifeArc	core	Guidebook / toolbox	Online	Available on demand	Students / professionals with baseline knowledge.	Free	Open signposting	Collaborative updates
Drug Repurposing	Beacon: for rare diseases	core	Course	Online	Available on demand (1,5h total)	General public	Free upon registration	Open signposting	Collaborative updates
TMEX – TRANSLATIONAL MEDICINE EXPLAINED 5 DAY WINTER SCHOOL 2023	EATRIS	core	Course	In person (Barcelona)	Held annually. 5 days	Students / researchers	The full fee is of €650. Special discounted fees of €475 applies for EATRIS members, I2TRON students and iNext-Discovery project members, and of €250 for VHIR students and EUPATI fellows.	Open signposting	Reoccurring live event - check for scheduling
IRDiRC - Orphan Drug Development Guidebook	IRDiRC	core	Guidebook / toolbox	Online	Available on demand	Researchers, industry, professionals	Free	Open signposting	Collaborative updates
Drug repurposing Handbook	IRDiRC	core	Guidebook / toolbox	Online	under development (but will be available on demand)	Industry, researchers, professionals, patients / patient organisations	Free (when finished and published)	Open signposting	Collaborative updates
ROADMAP - Repurposing all Drugs, Mapping all Paths	Every Cure	core	Guidebook / toolbox	Online	Available on demand	General public	Free	Open signposting	Collaborative updates

Name	Organisation	Topic alignment	Type of training	Location	Timeline	Target audience	Cost	Sign-posting type	Maintenance
EURORDIS Open Academy Summer school	EURORDIS	core	Course	Hybrid (online and in person)	Annually. (upcoming 19-23 June 2023 in Barcelona)	Rare disease patient advocates, researchers, professionals	Free. EURORDIS covers patient representative's training costs, accommodation, meals during the training hours, welcome dinner and training materials	Open signposting	Collaborative updates
Innovation Management Toolbox	European Joint Programme on Rare Disease (EJPRD)	core	Toolkit	online	Available on demand	Researchers, Academics	Free	Open signposting	Collaborative updates
Repurposing TIN Seminar: Lessons Learnt in Drug Repurposing	UCL	core	Webinar	Online	Available on demand	Students / professionals with baseline knowledge.	Free	Open case study webinar	review end Y2 - aim to replace with R4A content
EJRPD Eatris	Eatris	core	Webinar (YouTube)	Online	Available on demand (recorded in 2021)	Researchers / professionals / industry	Free	Open case study webinar	review end Y2 - aim to replace with R4A content
Therapy Development Webinar Series	Genetic Alliance and EspeRare	core	Webinar series	Online	Available on demand	Students / professionals / researchers	Free	Open case study webinar	review end Y2 - aim to replace with R4A content
Drug repurposing approaches to fast-track the development of new therapies for COVID-19	TECAN	core	Webinar	Online	Available on demand	Students / professionals	Free upon registration	Open case study webinar	review end Y2 - aim to replace with R4A content
Translational Science and Global Health	University of Oxford	core	Course	Online	24 - 28 April 2023	Students / professionals with baseline knowledge.	Short course in Health science £2165.00	Targeted signposting	Reoccurring live event - check for scheduling
Introduction to Translational Science	Coursera	core	Course	Online	Available on demand (approx. 4 weeks total)	Students / professionals with baseline knowledge. People with some understanding	Free. Certificate available under purchase	Targeted signposting	On demand resource. Review annually.

Name	Organisation	Topic alignment	Type of training	Location	Timeline	Target audience	Cost	Sign-posting type	Maintenance
Translational Science Specialization	Coursera	core	Course	Online	Available on demand (approx. 4 months total)	General public	Free. Certificate available under purchase	Targeted signposting	On demand resource. Review annually.
Fundamentals & Applications of Clinical and Translational Research	The Harvard Catalyst Education Program	core	Course	Online	It takes place 2 times / year, every winter and fall (4 months total)	Students / professionals	Fee: - Free for Harvard-affiliated institutions - Non-CTSA member: $500.00 - CTSA member: $375.00	Targeted signposting	On demand resource. Review annually.
Translational Science Panel: Research Insights and Best Practice for Clinical Relevance	insidescientific, The American physiological society, ADInstruments	core	Webinar	Online	Available on demand (Recorded in 2022). 1h	Students / professionals / researchers	Free. Must register to watch the recording	Targeted signposting	On demand resource. Review annually.
2023 AGM Preclinical Translational Science	NCATS AGM	core	Webinar series	Online (June-Dec 2023)	Available on demand if registered (upcoming)	Researchers	Free upon registration	Targeted signposting	Reoccurring live event - check for scheduling
Mentoring packages	EJPR	core	Other (Mentoring)	Online	Available on-demand, undergoing updates	Researchers	Free	Targeted signposting	Collaborative updates
Project Management: The Basics for Success	Corusera	core	Course	Online	Available on demand	Professionals	Free. Certificate available under purchase	Targeted signposting	review end Y2 - aim to replace with R4A content
Translational Research Application Manager (TRAM) Toolkit	NIHR (National Institute for Health and Care Research)	core	Guidebook / toolbox	Online	Available on demand	Researchers	Free	Monitoring	Collaborative updates
Essentials of Clinical Trials	London School of Hygiene & Tropical Medicine	Tangential - clinical trials	Course	Hybrid: London, UK or online	3 - 7 July 2023	Professionals, researchers	Fees 2023 £1,750	Targeted signposting	On demand resource. Review annually.

Name	Organisation	Topic alignment	Type of training	Location	Timeline	Target audience	Cost	Sign-posting type	Maintenance
Introduction to multiomics data integration and visualisation	EMBL-EBI Training	Tangential - Data	Course	In-person (European Bioinformatics Institute)	06 - 10 March 2023	Professionals / researchers	£825.00 (inclusive of four nights accommodation and catering, including dinner)	Targeted signposting	Reoccurring live event - check for scheduling
Causes of Human Disease: Exploring Cancer and Genetic Disease	FutureLearn	Tangential - Disease area	Course	Online	2 week course	Students / professionals with baseline knowledge	Free for 'Basic learning', £19.99 subscription fee for completion certificate and results	Open signposting	On demand resource. Review annually.
Just start grant writing	Udemy	Tangential - funding	Webinar	Online	Available on demand	Professionals, researchers, patient / patient organisations	Free	Targeted signposting	On demand resource. Review annually.
EU-OPENSCREEN	EU-OPENSCREEN-training courses and webinars	tangential - identifying opportunities	Webinar	Online	Available on demand	Professionals, researchers, Master and PhD students, postdoctoral scientists and independent principal investigators	Free	Targeted signposting	Ongoing monitoring
In silico talk	Swiss Institute of Bioinformatics	Tangential - identifying opportunities	Webinar series	Online	Available on demand (website videos and YouTube channel)	Students / professionals with baseline knowledge. People with some understanding	Free	Monitoring	Ongoing monitoring
An Introduction to Innovation in Healthcare	FutureLearn	Tangential - Market access	Course	Online	4 week course	Students / professionals with baseline knowledge	Free for 'Basic learning', £19.99 subscription fee for completion certificate and results	Targeted signposting	On demand resource. Review annually.
Introduction to Health Literacy	FutureLearn	Tangential - PE	Course	Online	3 week course	Students / professionals with baseline knowledge. People with some understanding	Free for 'Basic learning', £19.99 subscription fee for completion certificate and results	Open signposting	On demand resource. Review annually.

Name	Organisation	Topic alignment	Type of training	Location	Timeline	Target audience	Cost	Sign-posting type	Maintenance
Conflict of interest in patient engagement	Eurordis	Tangential - PE	Course	Online	Available on demand (2h)	Industry, researchers, professionals, patients / patient organisations	Free	Targeted signposting	On demand resource. Review annually.
EUPATI Open Classroom and patient expert training program	EUPATI	Tangential - PE	Course	Online	Available on demand	Patients / families	Free. Fee to earn a certificate	Targeted signposting	On demand resource. Review annually.
European Patient Ambassador Programme (EPAP)	European Lung Foundation	Tangential - PE	Course	Online	Available on demand (total 10 hours)	Patients / families	Free. Need to register to access. Certificate available after completion of each module	Targeted signposting	Reoccurring live event - check for scheduling
Patient Engagement Certificate Program	DIA Global	Tangential - PE	Course	Online	Available on demand	Patients / families	Registration fees specific to each course / module	Targeted signposting	Reoccurring live event - check for scheduling
PFMD (Patient Focused Medicine Development) - Patient Engagement Training	Developed by the PFMD Patient Engagement Meta-framework Co-creation Team	Tangential - PE	Course	Online	Available on demand	Industry, researchers, professionals	Free upon registration	Targeted signposting	On demand resource. Review annually.
PEM Suite	Developed by the PFMD Co-creation Team	Tangential - PE	Guidebook / toolbox	Online	Available on demand	Industry, researchers, professionals, patients / patient organisations	Free	Targeted signposting	On demand resource. Review annually.
Patient engagement and relationship building	University of West London	Tangential - PE	Course	In person	Held annually. Sept- August	Students / professionals with baseline knowledge. People with some understanding	Sept 22 - Aug 23 fees: £850 Sept 23 - Aug 24 fees: £900	Targeted signposting	On demand resource. Review annually.
PARADIGM Patient Engagement Toolbox	PARADIGM, co-led by European Patients Forum and EFPIA	Tangential - PE	Guidebook / toolbox	Online	Available on demand	Industry, researchers, professionals, patients / patient organisations	Free	Targeted signposting	On demand resource. Review annually.

Name	Organisation	Topic alignment	Type of training	Location	Timeline	Target audience	Cost	Sign-posting type	Maintenance
2023 CoRE Webinar Series: Opportunities for capacity building and training for patient groups in patient engagement and involvement	DukeNUS Medical School. Centre of regulatory excellence	Tangential - PE	Webinar	Online	Available on demand (recorded in 2023). 1.5h	Students / professionals	Free	Targeted signposting	On demand resource. Review annually.
Science Webinars: Rare disease diagnosis	Fondation Ipsen	Tangential - PE	Webinar series	Online	Available on demand	General public	Free when registered	Targeted signposting	On demand resource. Review annually.
International PPI Network: Learning Live webinar series	Cochrane Training	Tangential - PE	Webinar series	Online	Available on demand (recordings in 2019-2021)	Researchers	Free	Targeted signposting	Ongoing monitoring
Patient Involvement in Health Technology Assessment- A toolkit for patients and patient organizations	European Cancer Patient Coalition	Tangential - PE	Guidebook / toolbox	Online	Available on demand	Patients / patient organisations	Free	targeted signposting	On demand resource. Review annually.
Training for patient representatives and advocates on leadership and communication	European Joint Programme Rare Diseases	Tangential - PE	Course	Online	26th-27th October 2023	Patient representatives and advocates	Free. At the end of the course a certificate of attendance will be handed to the participants who attended 100% of the course.	Monitoring	On demand resource. Review annually.
How to justify budget for patient engagement initiatives	Twistle	Tangential - PE	Webinar	Online	Available on demand (recorded in 2021). 15 min talk	Professionals	Free	Monitoring	review end Y2 - aim to replace with R4A content
Translating Research to Communities	Coursera	Tangential - Population health	Course	Online	Available on demand (approx. 4 weeks total)	Students / professionals with baseline knowledge. People with some understanding	Free. Certificate available under purchase	Targeted signposting	On demand resource. Review annually.

Name	Organisation	Topic alignment	Type of training	Location	Timeline	Target audience	Cost	Sign-posting type	Maintenance
Genetic Alliance UK and ITN Business to Launch 'Caring together for rare conditions'	Genetic Alliance UK and ITN Business	Tangential - rare disease	Other (not traditional training)	Online	Available on demand (Launching 28 Feb 2023)	General public	Free	Targeted case study webinar	review end Y2 - aim to replace with R4A content
SWAN UK- Online Forum	SWAN UK	Tangential - rare disease	Other (interview videos with caste studies)	Online	Available on demand	Patients / patient groups / individuals with links to patients	Free for families / patient registered	Targeted signposting	Collaborative updates
Rare Disease webinar series	MEDPACE	Tangential - rare disease	Webinar series	online	Available on demand	Students / professionals	Free. Three part webinar series.	Targeted signposting	On demand resource. Review annually.
Duke-Margolis Center for Health Policy		Tangential - rare disease	Webinar	Online (2 day workshop)	Available on demand	Professionals, researchers	Free	Targeted signposting	On demand resource. Review annually.
STARS Core Curriculum	STARS	Tangential - regulation	Course	Online	Available on demand	Students / professionals with baseline knowledge.	Free	Targeted signposting	On demand resource. Review annually.
Pharma Drug Regulatory Affairs course - 2023	Udemy	Tangential - regulation	Course	Online	Available on demand (approx. 10 hours total)	Students / Professionals / Industry	£39.99. Certificate of completion	Targeted signposting	On demand resource. Review annually.
EMA Basics videos	EMA	Tangential - regulation	Webinar (Webinar style videos + presentations)	Online	Available on demand	patients	Free	Targeted signposting	On demand resource. Review annually.
Market Approval	Paul Janssen future Lab / Leiden	Tangential - regulation	Course	Online (10 / 30 / 2023)	5 weeks	Professionals, researchers	1600 euro (1200 euro non-profit)	Targeted signposting	On demand resource. Review annually.
SLAS Applied	Society of Laboratory Automation and Screening	tangential - researcher training	Guidebook / toolbox / webinars / courses / meetings	Online	Available on demand	SLAS members	Some free, some paid, mixed models	Targeted signposting	Ongoing monitoring

3.3 Results: Signposting categorisation

The assignment of training resources to signposting categories is a crucial step in converting the Training Landscape map from a piece of summary research to a useful tool for the repurposing community.

Across the 56 resources currently identified in the map, 35 are identified as suitable for **targeted signposting**. Of the remaining resources, 4 are recommended for monitoring by WP3, 5 identified as useful case study webinars (4 of which are for open dissemination), and 12 resources are assigned for open signposting.

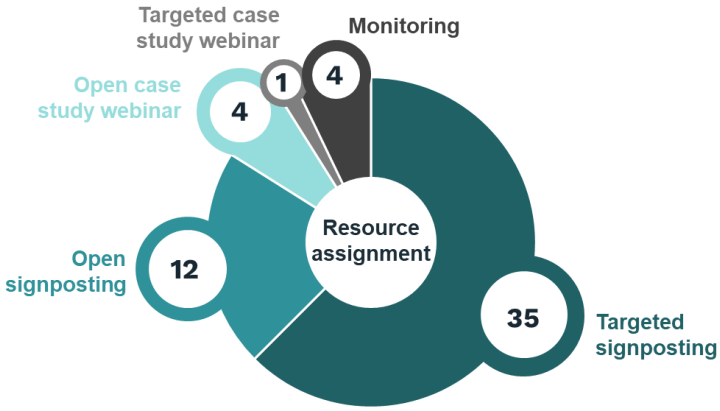


Figure 13. Resource Assignment

Effective updates of the training landscape will be key to maintaining meaningful signposting. The type of updates required for each resource is directly related to their route of delivery. Most resources will require an annual review (28 of 56), primarily due to their nature as an online on-demand resource. Ten resources are produced by organisations with which REMEDi4ALL's WP3 has, or needs, strong collaborative interactions, while seven represent reoccurring live events which are delivered in a specific time window. Of the remaining resources, 4 will require ongoing monitoring, and 7 represent resources that may be superseded by REMEDi4ALL.

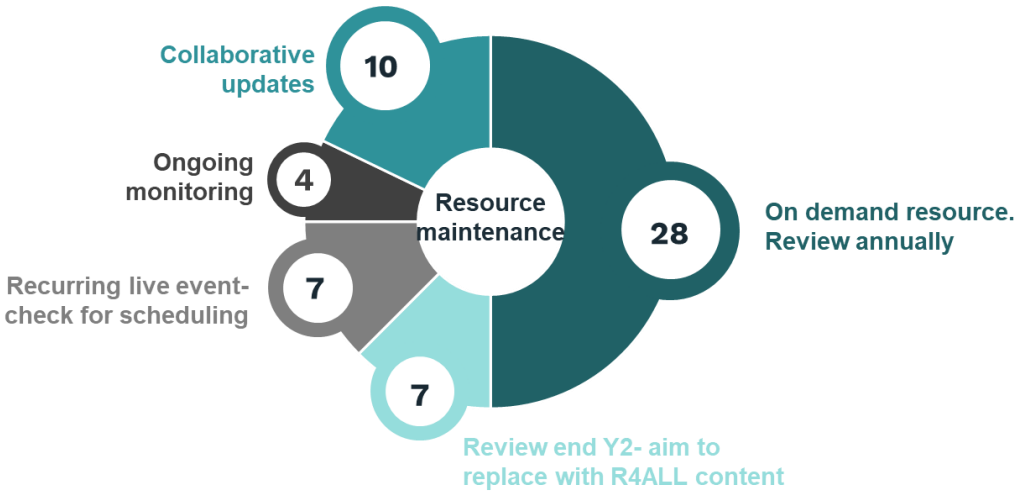


Figure 14. Schedule of resource maintenance

4. Assessment of Existing Tools

These resources were assessed between May – July 2023 and information is relevant as of these dates. We acknowledge that at the time of report submission and review that these resources may have been amended or updated and these updates will not have been captured within our assessments.

The tools we have assessed were chosen based on criteria from the refined version of the Training Landscape Database based on the following filters:

- *Open signposting* – a resource that we should be signposting too, that is accessible in terms of function, cost, and target audience.
- *Topic Alignment: Core* – a resource that covers translation and/or drug repurposing specifically i.e., core content for our stakeholders and eventual curriculum.
- *Maintenance: Collaborative* – a resource that, rather than needing active surveillance, will be frequently engaged with by the consortium partners so there is a constant dialogue relating to updates and new information.

European Joint Programme Rare Disease (EJPRD) – **Innovation Management Toolkit**

Reviewed 26 May 2023.

In June 2022, the European Joint Programme Rare Diseases (EJPRD) launched the Innovation Management Toolbox (IMT), a reference library of resources in rare disease translational medicine. It has been designed to help academic researchers to self-navigate the complexities of translational research processes and give them a clear overview on the resources and services that are available to help them. The overall aim of this innovative platform is to provide academic researchers with all relevant information and resources to counter uncertainty and inefficiency in academic translational research.

User accessibility:

Access to the EJPRD-IMT is free via the following link: https://imt.ejprarediseases.org/

The starting webpage is user-friendly and easy to navigate. A 5-minute video tutorial on how to navigate the website is provided. This video gives a great overview on the structure and layout of the platform and clearly guides the users through the different items and gives a clear overview on how to find and use the provided resources.

The overall outline of the platform itself promotes self-guided learning and searchability.

To promote self-guided need-based searches, different tools are available on the starting page (*see Figure 15*):

- Basic search tool where the user can search for relevant keyword.
- Browser function with three different subsections:

1. Toolbox Resources

Around 320 resources addressed by 5 main categories and various subcategories. The user can take advantage of some suggested popular subjects, including e.g., *Clinical Trials Toolkit, Regulatory Affairs, Preclinical Research* etc. (see Figure 15) and/or filter the resources by geographical scope. Opening the relevant subcategory all resources are listed (PDF or link). Every resource is additionally provided by a short description and can be bookmarked for further easy access.

2. Questions and Answers

Question and Answers are organized by 4 categories that roughly mirror the Toolbox Resources. Selecting a question shows a brief answer and signposting to relevant resources.

3. Use cases

This part is still under development (mentioned also in the video tutorial); currently there are resources (short videos) available, that fall under the categories *Research and Drug Development* (2) and *Regulatory Science* (5).

The Innovation Management Toolkit contains two unique sections that offer high level signposting:

Collections: This refers the user to additional internal and external resources including a detailed overview on the Clinical Trial Toolkit and references to the IRDiRC Orphan Drug Development Guidebook (https://orphandrugguide.org/) as well as to the EJPRD mentoring programme and additional online courses.

Services: catalogue that lists several support services provided by national or European organisations or initiatives. Categorized in 5 main categories. Interestingly, this catalogue of services cross-refers to different initiatives and training resources on patient empowerment and engagement (i.e., EURORDIS, EJPRD, EUPATI).

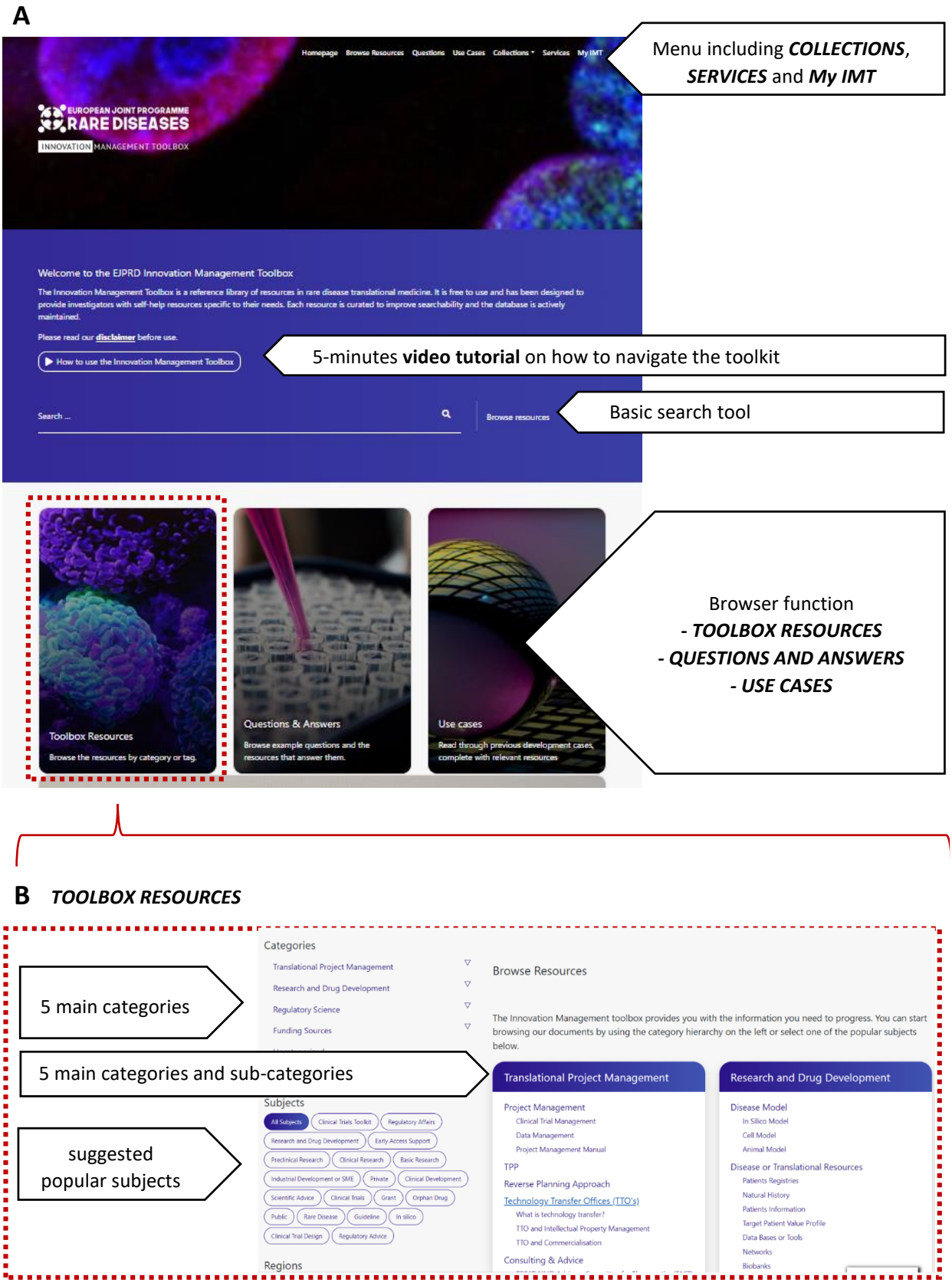


Figure 15. Snapshot of the EJPRD-IMT starting page (A) and Toolbox resources (B).

Practicalities:

The main target audiences of the EJPRD-IMT are academic researchers involved or interested in translational approaches in the rare diseases field. Although the provided information and resources are complete, an easily accessible representation/overview (an interactive graph or image) highlighting the "entire drug developmental process" and important steps is missing. Such an overview would be helpful for "non-, or semi-experienced" researchers approaching this field and might make this toolkit also accessible for other stakeholders, e.g., patient advocates, patient representatives, in the rare disease community.

Moreover, a short one/two-pager describing the main topic might be also useful for this purpose. Opening the relevant subcategories in the toolbox, the user is referred to a list of external resources, mainly PDF of scientific papers or direct links to external websites which give a very detailed and complex information on the topic. To make the toolkit "more accessible" to "non- or semi-experienced" users, providing a draft summary on the related topic, eventually supported by a short video, interactive tools or similar, could be a plus.

However, it is worthwhile to mention that the Clinical Trial Toolbox (accessible through the upper right menu – Collections; developed by ECRIN and recognized IRDiRC resource) is a good starting point to approach clinical trial design and execution and provides developers (main target audience are clinical trialist and R&D managers, but this resources gives a good overview also to additional users) with a practical guide to understand the regulations and requirements for conducting trials, specifically investigator-initiated trials for rare disease.

The information and resources (around 320) signposted in the toolbox are continuously updated.

Breadth and Depth of Information:

As mentioned above the provided information and highlighted resources are very "audience"- tailored. Supplementing these with some "easy-to-read" summaries supported by visuals or videos could make this great toolkit of resources accessible to additional stakeholders.

Signposting & Collaboration:

As EJPRD-IMT itself is a toolkit which refers to external resources we could directly refer to these specific resources in our training. However, as EJPRD-IMT gives a great and complete overview on the available resources there is clear value in directly cross-referring to it. This is especially true of the Clinical Trial Toolbox. The Question and Answer Section as well as the Use Cases could additionally act as inspiration for the REMEDi4ALL training programme and be highlighted when appropriate.

It is important to highlight that the Toolbox category *Research and Drug Development > Drug Discovery* has a specific section on *Drug Repurposing*. This signposts 12 very valid external resources. Some of these are already described in detail in this deliverable, e.g. The Repurposing Medicines Toolkit developed by LifeArc, while others, e.g., reviews and recent publications should be mentioned in the relevant training subtopics within the REMEDi4ALL training platform.

Castleman Disease Collaborative Network (CDCN), Chan Zuckerberg Initiative (CZI), and Every Cure - **Repurposing Of All Drugs, Mapping All Paths (ROADMAP)**

Reviewed 10 July 2023.

> *'…(we) launched the Repurposing Of All Drugs, Mapping All Paths (ROADMAP) project to identify the paths that can be taken to repurpose drugs, highlight the roles of various stakeholders, and centralize information on how to do this most effectively'.*

The ROADMAP project is focused on creating a centralized 'hub' of information that both informs on the various roles of stakeholders within the drug repurposing pathways and offers guidance on how to successfully progress drug repurposing projects.

The project was highly researched in a systematic way, with a paper due to be published on the findings this year (2023). The research had a unique focus on approaching each anticipated stakeholder group with a survey to investigate what and where roadblocks were encountered while trying to further their drug repurposing project. This is somewhat similar to the approach WP3 took to their research into stakeholder training needs and wants.

Figure 16: Schematic diagram of the detailed steps and research leading to the launch of the ROADMAP tool. https://everycure.github.io/about.html, 2023

User Accessibility:

The ROADMAP tool follows the same principles as the LifeArc toolkit (reviewed later in this document) in that it breaks the drug development process into individual steps and then dives further into each of these. The ROADMAP toolkit delivers visually where the LifeArc toolkit falls short, offering

interactive graphics and breaking up text with icons and smart, short sub-headings. Comparatively, LifeArc's Toolkit has a distinct lack of graphics and focus on long, unbroken streams of text.

Despite the clear use of visuals, there are several text heavy sections which are less appealing to the user. The tool does not utilise a 'search' function so the user must have some idea of where within the process they need guidance to begin their education – this can be a barrier as it can be time-consuming to read multiple webpages to find the desired information, time our stakeholders seldom have.

There is an 18-minute video showing how to get the best from the tool, using both the body of the tool itself and the extensive stakeholder research alongside this. This is useful to first-time users however, if a user has a specific query about a specific function, would be lengthy to sit through and pinpoint the correct guidance. WP3 does applaud the use of tutorial videos to ensure users get the most from their training but we must package these in the right way for REMEDi4ALL.

The tool is also downloadable as a PDF for those who struggle with the interactivity elements online and perhaps for those who want a hard copy while they are practicing in clinic, the lab etc.

Practicalities:

Access to the toolkit is free via the following link: https://everycure.github.io/roadmap.html

The key audiences are 'rare disease researchers and organisations' which aligns with our expected stakeholder uptake of the REMEDi4ALL curriculum.

ROADMAP keep their links to external resources up to date and relevant however they have an entirely transparent disclaimer about their use of survey data:

> *'…we did not make updates based on any other status updates that were discovered throughout the research and interview process, and the dataset provides a snapshot of the state of drug repurposing as of survey date completion and some of the data may be or soon will be outdated'.*

Breadth & Depth of Information:

As with all our resources there is a disparity between different topics and the breadth and depth at which the ROADMAP toolkit covers them. There is a large focus on referring to the statistics from their survey-led research which is not usable in terms of training but useful when referring to gap analyses in existing information.

The 'Validating the Drug' section is significantly less populated than other sections however this is in alignment with our expectations from development of our Training Landscape Database and user research in that, a significant amount of information and training already exists in these areas and that these are the most well understood aspects of drug development, particularly within ROADMAP's target audience of 'rare disease researchers and organisations'.

ROADMAP offers an accessible and engaging visual representation of the steps involved in drug repurposing but does not offer a detailed overview nor does it offer significant signposting to alternative or complementary resources. Uniquely it does include suggestions of the role and contribution patients/patient groups can make at every stage.

Signposting & Collaboration:

The ROADMAP is a signposting tool, so, much as with the LifeArc Toolkit reviewed below, we must be conscious not to signpost for the sake of signposting: if the end destination is the same resource, our REMEDi4ALL materials should link directly to these.

Despite this, the ROADMAP offers a perspective into the repurposing landscape in the US and includes some useful statistics. It has a particular focus on the value patient groups can bring to a drug repurposing project. It tells researchers how to utilise this input, and importantly, why this would improve the chances of the repurposed drug getting market authorisation and approvals. This focus on patient group value acts as both a call to action to the researchers and a proof-of-concept case study.

Collaboration with the ROADMAP toolkit and its authors will be crucial for REMEDi4ALL in that it will be a useful tool for any future projects or queries that come in from US-based stakeholders, an area perhaps lesser known by the REMEDi4ALL partners.

LifeArc – **Repurposing Medicines Toolkit**

Reviewed 28 June 2023.

The LifeArc Medicines Repurposing Toolkit is specifically tailored to educate, inform and guide on issues related to drug repurposing. With feed-in from experts in each step of the drug development process, the Toolkit highlights common challenges and knowledge gaps and suggests ways to plan for and mitigate these.

User accessibility:

This is the area in which the Toolkit is weakest.

The Toolkit is lacking in graphical or visual aids which often are preferred by individuals, particularly in the target audience of the Toolkit, to break up or better explain some of the more complex pathways and steps in the drug repurposing pathway. In addition to this, there is no ‘search’ function which means that unless the user has some idea of what broad theme the information they require falls under, it can be hard to locate and access it.

Practicalities:

Access to the Toolkit is free via the following link https://www.repurposingmedicines.org.uk/.

The Toolkit is intended to have a broad audience with a focus on researchers and charities or patient groups. These are stakeholders we would consider to be semi-expert in the drug development process and this target audience is aligned with the research we conducted in determining the stakeholder groups most likely to engage with REMEDi4ALL’s training platform.

Some of the resources that are linked within the Toolkit are outdated or lead to a page redirect suggesting that the links are not routinely checked for relevance or usability.

Breadth & Depth of Information:

As with all of our resrources,the Toolkit is inconsistent in terms of breadth and depth of information. There is a notable lean toward resources and advice focused within the pre-clinical development and clinical development aspects of drug repurposing; however, based on our research and the opinion of the stakeholders within the consortium, these areas are well covered and already well served by training and other resources.

Areas less well understood such as market access and the overarching theme of regulation throughout the process of drug development are not as well covered within the Toolkit. It is difficult to determine if the lack of scientific understanding in these areas is the cause or consequence of limited access to well-developed and specific training.

The Toolkit uses guiding questions alongside suggestions for actions to direct users to appropriate resources to help them determine if they need to answer these questions before entering the next stage of their drug repurposing project and if so – how best to answer them. This allows the user a degree of autonomy over assessing how much depth they need on a particular topic or sub-topic.

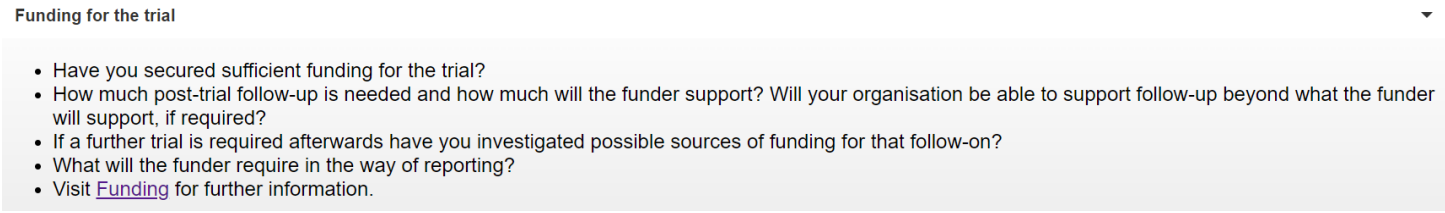


Figure 17: A section from the 'Clinical Development' section of the LifeArc Toolkit. Guiding questions encourage the user to think about process and bigger picture next steps – with a link to an appropriate resource to help answer these questions in more depth.

Signposting & Collaboration:

The Toolkit is itself a signposting tool which perhaps is indicative of why some topics are covered in less depth than others – LifeArc acknowledge that they are not the experts in certain areas and instead forward the user onward to more detailed resources.

For this reason, when utilising LifeArc's toolkit in REMEDi4ALL training, we must be cautious about what could become an unhelpful chain of links onwards which will both decrease user engagement and create a second 'checkpoint' that would need checking for relevance and useability.

There are areas within the Toolkit which REMEDi4ALL could signpost to and add clear value to the resource and information being offered. Signposting to the 'Funding' section for example, with LifeArc's well-established position as a philanthropic funder, would offer a truly expert voice.

We must also consider when signposting to the Toolkit that it is very much tailored to the drug repurposing process within the UK while REMEDi4ALL has a broader EU audience. While the specifics may be less relevant for some EU and global projects, the overarching themes and questions to be asked at each step will be the same regardless of geography.

EURORDIS – **Open Academy**

Reviewed 11 July 2023.

The EURORDIS Open Academy is tailored to patient advocates in particular and aims to empower patients (representatives) and organisations as well as increase their knowledge and skills in the rare disease space.
It offers 4 training programmes composed of a mixture of face-to-face training complemented by preparatory online elements. The online courses have been developed with subject-area specialists, such as researchers and patient experts, and are regularly updated for the best learning experience and the latest information on all topic areas. The online training resources are also suitable for a wider audience, basically anyone interested in learning more about the rare disease landscape.

User accessibility:

Access to the online elements of the open Academy is free via the following link https://openacademy.eurordis.org/
The landing page briefly introduces what the academy is about and what main training are offered. Through the 'all courses tab', users can further explore the offering.
Courses are structured by topic and include either a link to detailed information on the EURORDIS website or include 1 or more self-paced online training. The open academy's 4 training programmes are:

1. **Research & Development training**
   The EURORDIS Open Academy School on Medicines Research & Development aims to provide rare disease patient advocates with the knowledge and skills needed to become experts in medicines research and development. It is made up of e-learning modules, pre- and post-training webinars (estimated ~20 hours) and a yearly in-person 5-day training taking place in Barcelona. The pretraining includes e-learning modules on Pharmacovigilance, Market access & HTA, Ethics in Medicines Development, and more:
   - Medical research and development
   - Ethics in medicines development
   - Statistics in medicines research and development
   - Benefit-risk assessment and pharmacovigilance
   - Medical regulatory framework and procedures
   - Market access & health technology assessment

2. **Scientific Innovation & Translational Research**
   EURORDIS launched this training with the aim of deepening patient representatives' understanding of how pre-clinical research translates into real benefits for rare disease patients. It is made up of e-learning modules, pre- and post-training webinars (estimated ~20 hours) and a yearly in-person 5-day training taking place in Barcelona. The pretraining includes e-learning modules on sharing patient data, genome editing, genetic research to clinical diagnosis of rare diseases, and more:
   - Introduction to the European and international research landscape
   - From research to therapies
   - Setting the landscape

3. **Leadership & Advocacy**
   The training for patient advocates on leadership and communication skills empowers European patient advocacy group (ePAGs) advocates and other rare disease patient advocates to build strong partnerships with, and to be seen as a credible partners in, European Reference Networks (ERNs). The training programme is composed of an intensive 2-day course and pretraining materials including:
   - Establishing an international cooperation
   - International advocacy for rare diseases
   - Introduction to the European Union institutions
   - Ordinary legislative procedure – How are decisions taken by the EU?

4. **Digital & Communication**
   The EURORDIS Digital School aims to empower rare disease patient advocates to use digital communication tools to improve the strategic outreach and community-building capacities of their organisations and consists of prerecorded webinars and the following online course materials:
   a. The power of community
   b. Reaching the right people: celebrities and influencers
   c. Plan & create for social media success
   d. Creating great mobile video

Practicalities:

Access to the training is free via the following link https://openacademy.eurordis.org/
The main target audiences are patient (representatives), patient advocates and patient organisations, but materials are also very useful for non- or semi-experienced researchers.
A search tool is always available in the top navigation bar, allowing users to search for topics of interest. However, a simple overview of all available online courses is missing, and for this purpose a short one/two-pager describing all available courses including learning objectives and investment time might be useful.
The online courses offered are bite size in length (between 1 and 5 hours of investment time) in a variety of themes and seem to be relatively new and up to date. In general, courses are a mixture of video lectures complemented by cases, glossaries, quizzes and are self-paced, giving the learner a great deal of autonomy. There is one course consisting of slide decks only with no narration of accompanying explanatory text, making it hard to understand the presented content.
The course navigation is easy to understand and course progress is always visible on the side. After completing all modules in one course, a participant will be provided with a downloadable certificate of completion.

Breadth & Depth of Information:

The online academy offers a great variety of training topics, all related to the rare disease space, spread over 23 online courses. The strongest component of the online materials are patient empowerment, community engagement and advocacy sections, for which EURORDIS is well known.
The sections on regulatory, market access and HTA are a great starting point for less experienced research wanted to learn more about these topics.
Although many aspects of the rare disease space are applicable to repurposing, there is very limited training material available on repurposing. Also, the preclinical stages are underrepresented.

Signposting & Collaboration:

There are areas within the online academy which REMEDi4ALL could signpost to and add clear value to the resource and information being offered. Signposting to the 'patient engagement', 'community engagement' section for example, with EURORDIS well established position as a patient voice, would offer a truly expert voice.
We must also consider that the academy is mostly targeted to patient (representatives). Although it can be of interest to a much larger audience, e.g. non- or semi-experienced researchers, it might not reach enough depth for (research) experts.
As for the repurposing section of the online academy, it consists of a self-reading slide deck with no accompanying narration or text and is almost an exact copy of the slides provided in the Beacon Resource Hub. In the later, the slides have been transformed into concise and comprehensive lectures, complemented with relevant use case studies. Therefore, for this section, it would be best to directly signpost to the Resource Hub instead of the open academy.

Beacon – **Resources Hub**

Reviewed 11 July 2023.

Beacon's Resources Hub is a 100% free and accessible e-learning platform for rare disease, targeting mainly patients, patient groups and advocates, but the resource hub could be very helpful for a broader audience, basically anyone looking to understand more about the different stages of a rare disease journey. There is a dedicated section on drug repurposing, which includes a short online course on repurposing, exploring the benefits and challenges associated with drug repurposing and defining the different strategies for drug repurposing projects. The course is complemented by a series of conference recordings and webinars and is intended for those looking to design or undertake a drug repurposing project.

These are stakeholders we would consider to be naïve to semi-expert in the drug development process and this target audience is aligned with the research we conducted in determining the stakeholder groups most likely to engage with REMEDi4ALL's training platform. The repurposing course could serve as a preparatory course for the REMEDi4ALL Repurposing academy or for the candidates participating in the REMEDi4ALL hackathon.

User accessibility:

The landing page briefly introduces what the Resource Hub is about, for whom it is and what main training and events are available. The resources are a mixture of online courses, workshops, webinars, and conference recordings. There is a search function as well as an explore function which allows a user to search by subject, course, or event.

For each course, there is a clear description provided including time commitment per course required and when the course was last updated. Courses fall under three skill level categories to ensure

optimal learning: beginner, intermediate and advanced and allow participants to start at beginner's level and slowly work their way up.

The following subjects are covered in the online courses:

- Access and reimbursement (Course)
- Building a rare disease registry (Course)
- Collaborating with medical professionals (Course)
- Drug Repurposing (Course)
- Patient group and charity collaborations (Course)
- Patient Group Research and Trial Design (Course)
- Recruiting and managing trustees (Course)
- Utility values and QALY's for health economic models (Course)
- What are rare diseases? (Course)
- What is health economics? (Course)
- Working from home (Course)
- Working with industry (Course)

Accessing the resources themselves requires a login which is easily created and could help collect important metrics on what kind of users are utilising the tool. Users can only go through the course(s) in a chronological order, on the one hand ensuring that the participants complete all activities, on the other hand restricting the autonomy of the learner to choose what topics they would like to explore or skip.

Practicalities:

Access to the resource hub is free via the following link https://resourceshub.rarebeacon.org/about-us/

A search tool is always available in the top navigation bar, allowing users to search for topics of interest. However, a simple listing of all available online courses in one overview is missing and would be helpful.

Most resources are kept up to date (last performed update is mentioned) however some recordings refer to conferences of 5 years ago and content might no longer be as relevant or even be outdated, but this is only applicable to a few materials.

One potential drawback of the hub is the name of the tool not being self-explanatory ('resource hub') without mentioning the areas and topics covered, which could potentially negatively impact visibility and outreach of the tool. A short additional description on what fields are covered in the hub could improve visibility and usability.

Breadth & Depth of Information:

The Resources Hub offers a great variety of training topics, ranging from drug repurposing, research and drug development, data, fundraising, patient group development, to supporting community, building connections and communication.

The online repurposing course is a strong asset of the resource hub and emphasizes on repurposing strategies with well-explained pros and cons of the different approaches and is complemented by

relevant examples and success stories. The course does not address the whole spectrum of repurposing, i.e., from hypothesis to patient access; topics such as Target Product Profile and scientific approach (pharmacology, quality, toxicology, clinical and regulatory…) are missing. However, there are a few other courses from the hub that could complement the repurposing course, e.g., health economic models, access, and reimbursement.

The hub is mostly targeted to patient (representatives). Although it can be of interest to a much larger audience, e.g., non- or semi-experienced researchers, it might not reach enough depth for (research) experts.

Signposting & Collaboration:

There are areas within the resource hub which REMEDi4ALL could signpost to and add clear value to the resource and information being offered. For example, the repurposing course could provide helpful guidance to naïve and semi-experts interested in undertaking a drug repurposing project or even serve as a preparatory course to the REMEDi4ALL Repurposing Academy or the hackathon.

IRDiRC – **Drug Repurposing Guidebook (in development)**

Reviewed 13 July 2023.

IRDiRC are currently developing a guidebook that will follow a similar format to its Orphan Drug Development Guidebook.

The development of this new, repurposing specific, guidebook is the rationale behind us not assessing the Orphan Drug Development Guidebook in full. Despite this, we acknowledge that the Orphan Drug Development Guidebook is an extremely useful resource that should be signposted to and used in parallel with all others listed – offering a broader overview of drug development and approval for rare disease without the lens of repurposing.

A number of key ‘building blocks’ have been identified that will be included within the Drug Repurposing Guidebook.

Each building block represents an existing component of the orphan drug development pathway - many are existing infrastructures or pathways designed to support a specific stage of the development process or type of developmental challenge. Others are resources available to support the independent work of the developer. All combined create a complete suite of tools and pathways to develop any repurposed drug from concept to patient within the EU. The building blocks cover key aspects of drug development for repurposing within the UK, EU, US and Japan meaning it will be our most broad scoping resource in terms of geography.

Ahead of publication of the full Guidebook and complementary web resource, IRDiRC have released a checklist of questions that follow the format of a TPP and aim to help those starting out with or driving forward a drug repurposing programme.

REMEDi4ALL has been identified as a key building block in the Development & Support sections of the guidebook which means there is open to collaboration and signposting opportunities from the inception of IRDiRC guidebook; with REMEDi4ALL partners EURORDIS and EATRIS being part of the taskforce developing this resource.

5. Discussion

REMEDi4ALL is uniquely placed to deliver training in the field of drug repurposing to a diverse audience spanning the life science, scientific, and patient communities. REMEDi4ALL's ambitious goal in creating a value chain aligned with the entire drug repurposing pathway means we must consider the educational and training needs of all individuals involved at any point in this complex pathway.

To ensure an understanding of the needs of different target audiences and trainers, it is important to map out any relevant training that already exists to ensure that REMEDi4ALL will offer complementary training that fills identified gaps in the training landscape where knowledge, tools, and resources are still needed. It is crucial that our curriculum does not unnecessarily duplicate existing information or deliver training that contradicts the messaging of other trusted voices in the field.

To meet this goal, WP3 developed a training landscape map to provide a detailed overview of the existing training resources available and relevant to REMEDi4ALL's repurposing community. This assessment of the training landscape, together with an analysis of stakeholder training needs and wants, and our internal curriculum setting meeting, will help to develop the core drug repurposing curriculum of REMEDi4ALL, that will ensure our training programme sits effectively in the existing training ecosystem.

5.1 What training is out there and what does this tell us about the training gap REMEDi4ALL needs to fill?

5.1.1 Database of key resources

In our non-exhaustive and descriptive research, we found that there is a great breadth of information of many important subjects for REMEDi4ALL, covering all the journey and development path of a drug. This is great news, meaning that a lot of knowledge is already available, and we will have to focus on successfully signposting it as well as looking for synergies and collaborations. In contrast, we still found some gaps in the landscape that could be filled, and REMEDi4ALL has the potential to do this through its curriculum training.

A relevant resource was found for almost all subjects related to the repurposing field. Core and tangential resources were identified, and training on patient engagement and the rare disease field emerged as the most prevalent topic resources. This might be explained by the fact that the main experience and expertise of the authors of this research is focused on patient advocacy, patient empowerment, and patient engagement. Therefore, a little bias could explain the reason why more patient engagement training was found.

We saw that life science professionals were the most targeted audience group for training *(see 3.1.2-Target Audience section. Figure 3)*. This group of people have a cross-cutting appeal, as they have probably also been students and researchers in the past, and also have probably worked in many health-related sectors during their professional career, such in the pharmaceutical industry. It is not a surprise then, that professionals emerged as a group of people that training resources usually tackle, as they can easily be considered as having an interest in the field. A similar explanation can be applied

to life science students and researchers, who might be interested in a broad range of topics which training developers consider when setting out to put training in place.

Frequently, **patients, patient organisations** and the **industry** are key stakeholders for initiating or undertaking a drug repurposing project. However, we have seen that they are **less commonly targeted compared to the other groups**. This could be explained by the specificity of their area of work and the activities they are involved in, which could make it more difficult to assess the suitability of a training resource for them. In the case of patients and patient organisations we saw that few core training courses were targeted to them, but a fair number of tangential training courses were really focusing on capturing the attention of this group. This finding could be also explained for the reason mentioned above, indicating that the research has been conducted by patient engagement professionals, being for them easier to find patient engagement training, which is a field that usually involves patients and patient representatives.

Many gaps in the training landscape were observed for the main audience groups of interest (*see 3.1.2- Target Audience section. Figure 4 and 5)* that the future repurposing curriculum will aim to target (patients/patient organisations, researchers, and industry):

Patients and patient organisations:

This group needs more core training in understanding the end-to-end drug development and drug repurposing pathway, the whole spectrum of stakeholders involved in the journey, and where they could really contribute and get involved. Also, many key tangential areas like funding (e.g.; it would be beneficial for this group to learn how to find opportunities to collect funds for a research project or to raise awareness of the disease group they represent), market access (e.g.; learning about meaningful patient engagement in Health Technology Assessment (HTA)), or clinical trials (e.g.; how to get involved in different steps of clinical trials beyond the participation as a patient in the trial).

> ***Patient Group Leader response to REMEDi4ALL interview question: 'How would you like to be trained, what works for you and what doesn't?'***
>
> *'Mentorship and handholding is essential because often we [patient group leaders] listen to something/learn what to do and how but when it's time to actually do it there is an element of knowing there is something to do, a question to answer but not actually knowing the steps to answer that'.*

Researchers:

Although patient engagement training targeting researchers were found, we observed that this type of training was usually more focused on patients and patient organisations. It is of utmost importance that **researchers** understand the significance of having patients onboard from the very beginning of every research project and learn how to do meaningful patient engagement. Therefore, this group would clearly benefit from more tailored patient engagement training adapted to the specific activities of researchers.

In addition, specific topics of the development pathway like regulation are not frequently offered to researchers. This gap should be filled to address the lack of knowledge of researchers on how to deal

with authorities and early engage with regulators to improve the success of their research and anticipate possible hurdles.

Like patients, researchers could really benefit from having more training on how to find funding opportunities for their research, how to get connected with the pharmaceutical industry, and how to interact with investors and effectively communicate the relevance of their research project idea.

Finally, more resources focused on the management and handling of large datasets, as well as the use of *in silico* and *in vitro* screening techniques, would be very beneficial for this group to broaden their knowledge on identifying repurposing opportunities.

Experienced Academic's response to REMEDi4ALL interview question: 'What do you wish you knew when you were doing your project?'

Patient led can be some of the best clinical trials – patient groups have experience with day-to-day trial and error and often the founder of the charity or board has disease experience. A strong social media outreach as trial expands is invaluable for recruitment and dissemination. The FDA acknowledge well-informed patient groups who can directly approach regulators with high impact.

Pharmaceutical Industry: Often have internally led training which may or may not cross-over into other aspects of repurposing depending on the size of the company and the resources available to them. Larger companies such as AstraZeneca offer a vast internal curriculum which, while not mandatory, offers individuals within certain departments to learn more about their own specialism and others such as legal, regulatory and funding.

i. It is without doubt that an element of these training resources led internally have some focus on commercial benefit, or Life Cycle Management. It is less certain how much of this internal training covers areas we have identified as tangential and certainly there is scope to develop training to fill the obvious gaps in industries' knowledge, particularly in patient centricity and socioeconomic value of drug repurposing.
ii. It is appropriate to develop training for the pharmaceutical industry but particularly with a focus on communication as a training tool, raising awareness of unmet medical needs (e.g., in rare diseases) and the success of drug repurposing in these areas. Using case studies to highlight market opportunity that has both a viable financial return and a significant patient and healthcare impact is crucial in training industry representatives.
iii. At all levels, the industry has the means to fund and support promising projects that may see a drug reach market, however, there is little awareness of these projects within the industry community. Compounded with the fact there is a historical disjoint in the relationship between industry and other stakeholders (patient groups, academics), there is a perceived disinterest in these projects. The way to overcome this is to communicate at every step and involve industry in discussion in the way it is expected academics and researchers would be. Training needs to be on the incentives – social, economic and reputational.

Big Pharma Representative's response to REMEDi4ALL interview question: 'What do the pharmaceutical industry need training on?'

[Industry wants to know] How to get the maximum from your molecule.

We found that courses were the main **format for delivering** the resources identified *(see section 3.1.3- Type of training. Figure 6).* Out of all the types of training found, courses are the ones with a more diverse location, as we saw that some were offered online, others in person and others in hybrid mode. This great variety of ways of delivering the training allows training developers to produce more courses and offer them through different channels. Webinars, which offer exclusively online learning, were also found to be a quite used training type. Its format allows for great accessibility and probably less organisational and logistics costs, which makes it an appealing type of training both for the developers and for the trainees. Guidebooks and toolboxes are also quite common resources that contain a wealth of information in the same platform or webpage. This resource is usually more interactive for the user and cover more than one specific topic (differently to courses or webinars, which are usually focused in one specific subject). However, this type of resource, as well as other resources like mentoring, are less commonly found as they are usually tailored to a specific target audience and contain broader information, which makes their development slower and take more time to set it up.

Most of the training found allowed for autonomous and self-paced learning, letting the trainee adapt the studies to their availability and needs *(see section 3.1.3- Type of training. Figure 7)*. Also, we have seen that a big part of the training landscape offer is available for free, and only a few could only be accessed through fees *(see section 3.1.3- Type of training. Figure 8)*. In addition, we have observed that the online format tends to be the most prevalent method of training delivery *(see section 3.1.3- Type of training. Figure 9 and 10)*. For webinars and guidebooks/toolboxes, the online format is probably the only available option to deliver training, but interestingly we found that courses are most frequently delivered online too. The COVID-19 pandemic might have played a key role in changing delivery methods through necessity, but it is important to be aware of the advantages that delivering a training online could bring more accessible and easier to enroll from any location, and less costly as travel and accommodation expenses are avoided. However, although the benefits of online training are clear, the importance of face-to-face meetings to build trust, encourage engagement and establish connections in an easier way was also clearly stated by the interviewees and also the participants of the curriculum setting meeting. For this reason, hybrid formats were very supported as they would allow for taking the most of each way of delivering training.

REMEDi4ALL must create a drug repurposing curriculum in the future that is of **high quality**, but also and of equal importance, **accessible** and **affordable** for all the stakeholders interested and involved in the repurposing pathway. In that sense, it is very relevant to see that many of the available training courses that already exist are aligned with this approach and so synergies will surely be found to complement already existing resources and fill the gaps with our curriculum.

A final important aspect observed during our research was that most of the training found offered a certificate of completion *(see section 3.1.3- Type of training. Figure 12)*. Interestingly, we observed that the certificate was usually only obtainable with an additional fee. We believe that a certificate of completion could incentivise individuals to complete training. Accreditation formally supports the resumé of training attendees and encourages feelings of empowerment and motivation.

All this information encompassing the already available knowledge and the gaps identified, gives an idea of which stakeholder groups could benefit the most for a well-oriented and specifically targeted training that is currently missing in the training landscape, and that REMEDi4ALL has the opportunity address and fill.

2.6.1.1 5.1.1.1 Expectation of user engagement with training based on research and existing resources

Most Likely to Engage	Patient Groups/Patients Academics	Distinct willingness & interest to learn with awareness of own gaps in knowledge. 'Build it & they will come'.
Potentially Likely to Engage	Industry (Research Level) Clinicians	Requires **targeted promotion** of specific training materials in arenas where these groups frequently obtain **robust and reputable** information e.g., scientific conferences (industry led and disease focused particularly), scientific papers/journals, society meetings.
Least Likely to Engage	Industry (Business Level) Policy Makers Funders	**Requires active outreach.** These stakeholders offer specific, internal training. **Studies of success crucial** to prove value and obtain interest. Operate in arenas these stakeholders frequent e.g., media, scientific & non-scientific conferences, legislative/governmental meetings, board discussions, engagement meetings.

5.1.2 Survey to Consortium Partners

Responses to the internal survey were limited, with all suggested training being very specific to the technical area in which the contributor specialises.

While this was part of the reason to undertake this survey, to limit our own bias in being focused on specific training in specific areas, it clearly highlights that this is a broader issue that should be addressed in a successful training resource or platform.

Technical experts know where best to find training resources that relate to their field of expertise however have limited knowledge or awareness of what exists outside of their field. This has been identified as one of our key barriers to successful repurposing in that all stakeholders need to be aware of the other's skills, needs and wants to succeed in getting new treatments to patients.

The survey was, unfortunately, not hugely informative when developing our training landscape, offering a few useful tangential training resources. Despite this, it is extremely useful in the reaffirmation of our hypotheses on how to best deliver training and where the gaps fall. Crucially, as is the ethos of REMEDi4ALL, we need to see cross-stakeholder collaboration in training to open the ecosystem to individuals who are otherwise 'boxed off' by their specialism.

5.2 The creation of a signposting tool for repurposing training

Understanding the existing training landscape is of vital importance to the development of REMEDi4ALL's own project. It allows us to identify gaps in the field, and to build our own training resources in concert with the ecosystem. However, to realise the true value of this landscape map, we needed to turn it into an accessible signposting resource, appropriate to the needs of the REMEDi4ALL consortium and our community. The categorisation of topic, signposting type, and maintenance approach were designed to do this, and build a tool valuable for the duration of the project, and beyond.

The full repurposing pathway is long and complex, requiring input from many different types of specialists to realise patient benefit. The training landscape we identified truly reflects this. As discussed, there are a small number of end-to-end training resources that deal with the whole pathway – and many of those that do exist are more recent innovations designed to fill this need gap following a surge in awareness of repurposing post-COVID. These full pathway resources are supplemented by many smaller, more focused training that targets specific components of the repurposing (or drug development) pathway. This structure mirrors the siloed nature of scientific knowledge and skills, with few people having a coherent understanding of the full pathway. Our topic classification system reflects this nicely, making specific training easy to identify within the landscape, and clearly defining those training materials that complement the core aim of REMEDi4ALL – improving knowledge across the entire drug development pathway with the aim of converting promising scientific concepts to patient impact. By combining this topic classification system with both a signposting and maintenance type, we are able to create a simple structured database, with clear actions for each training item. Furthermore, this system is easy to expand based on user need. Our current searches have, for example identified no real training in the space of health economics – if deemed valuable for our community throughout the life of REMEDi4ALL, targeted searches can easily be conducted, adding a new topic classification to the training landscape to accommodate the results.

5.2.1 Signposting type – Open vs Targeted

The distinction between open signposting and targeted signposting is a crucial one for the use of the landscape map. Resources were assigned **open** status if they had a broad applicability to the repurposing community. Such resources should benefit people with very different backgrounds (patients, clinicians, funders, industry etc.), cover a topic that individuals would expect to find when visiting the general REMEDi4ALL website (e.g., a subject fundamental to the repurposing pathway), be highly accessible (low cost to access, English, well explained technical terminology), and be from a reputable source. As our initial open signposting will occur on the REMEDi4ALL website, rather than within a dedicated training portal, we decided to apply a high threshold for inclusion in the open category to minimise the chance of inappropriate REMEDi4ALL endorsement at the early stages of the project's life.

Consequently, **targeted** signposting is more widely used across the database. This accounts for 35 of the identified training resources. Targeted training needs to be easy for the whole REMEDi4ALL consortium to find, so that they can be suggested to projects or individuals who could benefit from them during the project's life. They will not be publicly promoted on the REMEDi4ALL website but may be sign-posted online in the correct context (e.g., a number of patient engagement training resources could be promoted within a REMEDi4ALL webinar on the topic). As such, the full training

landscape map will be shared in a prominent location within the REMEDi4ALL SharePoint files, with a simple guide document to promote its effective use by all consortium partners.

A number of **core** training resources are recommended for targeted signposting rather than open. These tend to fall into the following categories:

- **High cost –** Time bound, high-intensity training courses, often residential, with a high financial cost to attend. These may be exceptional courses, but without experience of the courses we should not be publicly endorsing them as a consortium. As we may not have captured an exhaustive list in our searches it is therefore better to provide examples of these courses to those looking for them in a targeted way, allowing us to communicate this clearly.
- **Clear audience focus or limited context –** Some resources are exceptionally useful to a small subset of the audience. For example, the EJPRD mentoring programme is an excellent resource, but only applicable to very specific projects.
- **Uncertain quality or perspective –** There a number of training resources, particularly those on Coursera, which appear to be affiliated to a single university or professor. Here, we should clarify the perspective of that researcher on repurposing or translation before recommending it more widely, making a targeted signposting approach more appropriate.

Finally, some resources are less applicable for signposting, but could help REMEDi4ALL develop our training or awareness of certain issues. These resources are marked for **monitoring** and include:

- **The TRAM Toolkit -** can guide researchers through a specific type of NIH application. It is very useful in that context, but not ideal for broad signposting; however, understanding the NIH pathway and requirements may help us better understand funding requirements for repurposing, and develop our own general guidance.
- **In silico talks (The Swiss institute of Bioinformatics) -** provides a detailed database of on demand webinars, only some of which will be relevant to the REMEDi4ALL audience. These should be identified and promoted directly.
- **The EJPRD training for patient representatives –** this is not directly relevant to much of the REMEDi4ALL community but will be monitored so that those champions who need support can access the benefits of the training.
- **How to justify budget for patient engagement initiatives (Twistle) -** This training lays outside the core purpose of REMEDi4ALL but will be used to help develop our own internal recommendations for this important issue.

5.2.2 Sustainability

To make the landscape map a truly useful tool, the information in it needs to remain relevant. As such, a system of updates is key, and this has been implemented in a clear and simple manner as already described in 2.4.3 and 3.2. The system implemented will ensure that all training types are reviewed and updated in a manner appropriate to their content type and route of delivery.

While effective signposting and avoiding duplication are central to the aims of REMEDi4ALL, it is also important to recognise that there will be some areas where our plans will create new resources which either supersede existing materials, or package them in a better way for our audience. Furthermore, the relevance of some training resources will decline over time. Identifying such resources is key, as it allows us to signpost to them while relevant. When creating our classification, we identified seven resources that fell into this category, all of which will be reviewed by the end of year 2. Those training resources are:

- **Caring together for rare conditions (Genetic Alliance UK and ITN Business) -** This online news-style programme provides an introduction to rare diseases, summarising some of the active work in the space in 2023. This is a time sensitive resource, so likely to be replaced by another resource in time.
- **Repurposing TIN Seminar: Lessons Learnt in Drug Repurposing (UCL) –** A webinar focussed on drug repurposing and support available to academics, run in 2022. Information likely to be superseded over the next year, particularly with the development of REMEDi4ALL and REPO4EU.
- **EJPRD-Eatris repurposing webinar (Eatris) -** A webinar focussed on drug repurposing and support available to academics, run in 2021. Information likely to be superseded over the next year, particularly with the development of REMEDi4ALL and REPO4EU.
- **How to justify budget for patient engagement initiatives (Twistle) –** REMEDi4ALL is actively working to develop its own recommendations and standards in the area, based on best in-field practice. We are aiming to provide our own guidance to improve on this training.
- **Therapy Development Webinar Series (Genetic Alliance and EspeRare) -** A case study webinar series focussed on drug repurposing and a unique model to bring results to market. The story will move on over the next year, and this should be investigated to provide further context or information on future referrals.
- **Drug repurposing approaches to fast-track the development of new therapies for COVID-19 (TECAN) -** A case study webinar series focussed on drug repurposing in COVID. More appropriate and timely case studies may be available in the coming year.
- **Project Management: The Basics for Success (Coursera) –** Project management for scientists is one of the key themes we wish to explore in REMEDi4ALL. While this course may provide a useful way into the topic, we will aim to identify and produce more relevant materials in the coming year.

All other resources will be monitored as planned, while newly identified training will be integrated into this monitoring scheme for periodic review.

5.2.3 The Open Signposting Database

As noted, the original training database was extensive with a wide-reaching breadth of training. The categorisation of this dataset has allowed the identification of a significant subset of training which would benefit the wider repurposing community. The core of this set are the six training resources reviewed in detail in section four above – open, core, collaborative projects which will form the broader ecosystem in which the REMEDi4ALL training programme exists. Alongside these six keystone resources, we have identified a further six open resources, and four case study webinars for wider dissemination. These are shown on the following page.

Name	Organisation	Topic alignment	Type of training	Location	Timeline	Target audience	Cost	Sign-posting type	Maintenance
Introduction to Translational research for Rare Diseases	FutureLearn	core	Course	Online	5 week course	Researchers and students in medicine and health-related research fields, health professionals, patient advocacy organisation representatives	Free for 'Basic learning', £19.99 subscription fee for completion certificate and results	Open signposting	On demand resource. Review annually.
Clinical Pharmacokinetics: Dosing and Monitoring	FutureLearn	core	Course	Online	6 week course	Students / professionals with baseline knowledge	Free for 'Basic learning', £19.99 subscription fee for completion certificate and results	Open signposting	On demand resource. Review annually.
TMEX – TRANSLATIONAL MEDICINE EXPLAINED 5 DAY WINTER SCHOOL 2023	EATRIS	core	Course	In person (Barcelona)	Held annually. 5 days	Students / researchers	The full fee is of €650 with discouts available	Open signposting	Reoccurring live event - check for scheduling
IRDiRC - Orphan Drug Development Guidebook	IRDiRC	core	Guidebook / toolbox	Online	Available on demand	Researchers, industry, professionals	Free	Open signposting	Collaborative updates
Causes of Human Disease: Exploring Cancer and Genetic Disease	FutureLearn	Tangential - Disease area	Course	Online	2 week course	Students / professionals with baseline knowledge	Free for 'Basic learning', £19.99 subscription fee for completion certificate and results	Open signposting	On demand resource. Review annually.
Introduction to Health Literacy	FutureLearn	Tangential - PE	Course	Online	3 week course	Students / professionals with baseline knowledge. People with some understanding	Free for 'Basic learning', £19.99 subscription fee for completion certificate and results	Open signposting	On demand resource. Review annually.
Repurposing TIN Seminar: Lessons Learnt in Drug Repurposing	UCL	core	Webinar	Online	Available on demand	Students / professionals with baseline knowledge.	Free	Open case study webinar	review end Y2 - aim to replace with R4A content
EJRPD Eatris	Eatris	core	Webinar (YouTube)	Online	Available on demand (recorded in 2021)	Researchers / professionals / industry	Free	Open case study webinar	review end Y2 - aim to replace with R4A content
Therapy Development Webinar Series	Genetic Alliance and EspeRare	core	Webinar series	Online	Available on demand	Students / professionals / researchers	Free	Open case study webinar	review end Y2 - aim to replace with R4A content
Drug repurposing approaches to fast-track the development of new therapies for COVID-19	TECAN	core	Webinar	Online	Available on demand	Students / professionals	Free upon registration	Open case study webinar	review end Y2 - aim to replace with R4A content

It is worth noting that two of these resources fall outside our **core** designation. Both are broad appeal introductory courses, the first introducing students to both genetic disease and cancer, and the second to health literacy. The former course is likely to be a valuable introduction to diseases areas likely to feature with high prevalence in the REMEDi4AL portfolio, so useful to highlight to our general audience. Conversely, health literacy is a crucial topic often poorly considered in the communication of scientific research. As all REMEDi4ALL projects should be aiming for a high standard of patient engagement, we have highlighted this training as a key consideration for all groups.

We aim to embed this final list of resources within the REMEDi4ALL website, using a series of searchable tags (relating to the categories e.g., audience, duration) to create a simple interactive filterable database. This will allow a community member with specific repurposing training requirements to filter through these resources and access the most appropriate resource for their needs. We will encourage users to provide us with feedback on the training we signpost to so that the list can be kept relevant and signpost to only the most effective and engaging training as per REMEDi4ALL's high expectation.

As discussed, our initial recommendation is to create an open signposting database comprised of the resources that are free or low-cost to access across stakeholders, and which support users in understanding the end-to-end repurposing pathway. This approach should ensure maximum applicability of our resources and initiate maximum engagement. We can amend this based on stakeholder resources and uptake as the project progresses and further training is produced both within and outside of REMEDi4ALL.

5.3 Assessing Existing Tools

As is evident through our assessment of the landscape, tools do exist that are signposting to or delivering useful information about drug repurposing or drug development. Our in-depth analysis has weighed up where these both succeed and fail in their function.

There is not, presently, a tool or resource that is designed to tailor to the educational or training needs of all stakeholders who wish to start a drug repurposing project. Nor is there a tool that covers each stage of the drug development process in equal depth.

While REMEDi4ALL's job is not to 'fix' this, it has been a useful assessment to determine what the key gaps are in training and education for drug repurposing. In some cases, it is trickier to determine whether the lack of knowledge comes from a lack of effective training or if the lack of effective training comes from a lack of knowledge. This is something we will continue to research and develop within WP3 with ongoing discussions with internal and external stakeholder representatives.

User Accessibility:

Overall, this category fell short of our expectations. Poor searchability made specific information hard to access without some prior knowledge of where within the drug repurposing pathway this information would sit. It is time-consuming to search through each page of the online resources to find one specific piece of information and often our stakeholders do not have this time to waste. Additionally, many of the resources were text heavy with little intractability or use of graphics making them difficult to access for individuals with less knowledge in the field.

Some resources offered expected timelines for each training module they provided. This is excellent in terms of user accessibility in that users can either set aside time to complete training or factor relevant training into their existing schedule with no unexpected side tracking.

Tutorial videos within some of the resources offered insight into how best to use the tool to achieve the best learning outcomes for the user. While we believe this is useful in helping with initial navigation, particularly when as mentioned there is no 'search' function, some of the videos were as long as 18 minutes, which may be perhaps the only time an individual has to set aside for training. If we were to utilise this idea in REMEDi4ALL's training, we would need to restrict these tutorials to a maximum of 5 minutes.

Practicalities:

All of the resources we reviewed are available publicly and for free. We chose this as a critical criterion for our key signposting resources to ensure:

- We are not seen to be advocating for a particular institution/resource that asks for a fee and are being transparent about this.
- We are ensuring maximum accessibility for all stakeholders; an associated access fee may prevent or deter some stakeholders from interacting with these resources.

All the resources have a signposting element within them which works to differing levels of success. In some cases, onward links are outdated or no longer work while in others there are links to policy and regulation that has since been changed. This is why we have built in a regime to review and update our training landscape periodically, ensuring the resources are still relevant, refreshed and still operational. As mentioned, we should also be cautious when signposting to signposting resources as there is a chance of a drop off in engagement. Most users would prefer one-click access to the information they use rather than a chain of redirects.

The target audience across the resources was patient groups and researchers (academics). While this is the user group we expect to most engage with training, it highlights a clear gap in the landscape. There is little 'easy to find' training that targets industry or health economists who would benefit from a broad overview of the drug repurposing pathway as much as researchers – to enable a clear vision on the value and opportunity as well as the challenges which may arise and how best they can collaborate with other stakeholders to ameliorate this.

Breadth & Depth of Information:

Our discussion overall highlights the fact that there is an inconsistency in training across the board. Many of the resources are created by organisations that are experts in a specific part of the pathway and thus, the information has a skew towards this. Key areas where drug repurposing specific information is lacking include:

- Health economics & HTA assessment
- Regulation
- IP and legal management
- Project management

Within REMEDi4ALL we have the opportunity to call on experts from these fields to both deliver and help develop relevant training in these areas. While we do not expect each individual who plays a part

to be an expert in each technical aspect of the process, we believe it is important to offer a top-level oversight that is accessible to all with the option to delve deeper per the individual's needs.

5.4 Observing and Documenting the Changing Landscape

Both within the training Work Package 3 (WP3) and other Work Packages, particularly Work Package 11 (Communication), within the consortium, REMEDi4ALL has a strong oversight of new and pipeline projects relating to both translation and drug repurposing.

We have already been able to establish strong working relationships within some of these projects including crossover into the new SIMPATHIC programme (exploring drug repurposing for neurodevelopmental diseases) and LifeArc's newly launched Rare Disease Translational Challenge, among others.

The necessity for training in drug repurposing and across translation is increasingly under the spotlight in new projects and consortia. By establishing early connections and partnerships within these up-and-coming ideas and projects we can ensure that firstly, REMEDi4ALL is not duplicating work that is due to be delivered by someone else with a stronger mandate or authority. Secondly it ensures that there is continued opportunity for collaboration and improvement – as the training ecosystem continues to grow, we will learn more about what works and what is less successful and should share this. This ensures that we are working towards the end goal and vision of REMEDi4ALL in creating a patient-centric drug repurposing ecosystem and improving the drive of drugs through development to patient access rather than focusing on building a curriculum that competes with others and dilutes focused learning.

6. Conclusion

Mapping the training landscape has been an invaluable basis on which to guide and develop our training for REMEDi4ALL.

By understanding what is already available or is in development, is successful and accessible, we have been able to determine where gaps in training exist and beyond this, where gaps in different stakeholder's knowledge exist.

It is evident that most current trainings are aimed at one particular audience, with little or no cross-over and collaboration between important players in the drug repurposing pathway. There is also little scope with current training to expand on the breadth and depth of information offered.

REMEDi4ALL has a unique opportunity to create and develop a unique training resource that covers the role of each stakeholder group throughout the drug repurposing journey. We must offer differing 'tiers' of training to ensure that the training resource is truly accessible and informative to anyone who has an interest or an active part in a drug repurposing project.

	Level	Main training type	Delivery route	Audience
1	Why repurpose?	Why is repurposing important? Case studies and success stories. Aim to win people to the cause.	General communications, conference presentations	Industry, General public, regulators, funders, patient organisations
2	How to repurpose with patient impact	The core training curriculum, including the TPP, repo pathway, and project management	Repurposing academy, e-learning resources	Academics, patient organisations, champions, users
3	Repurposing techniques	Specific training on scientific tools and techniques. More technical knowledge.	Webinars, e-learning content, sign posting, and user mentoring	Industry, specific branches of academia or life scientists with a specific interest in repurposing

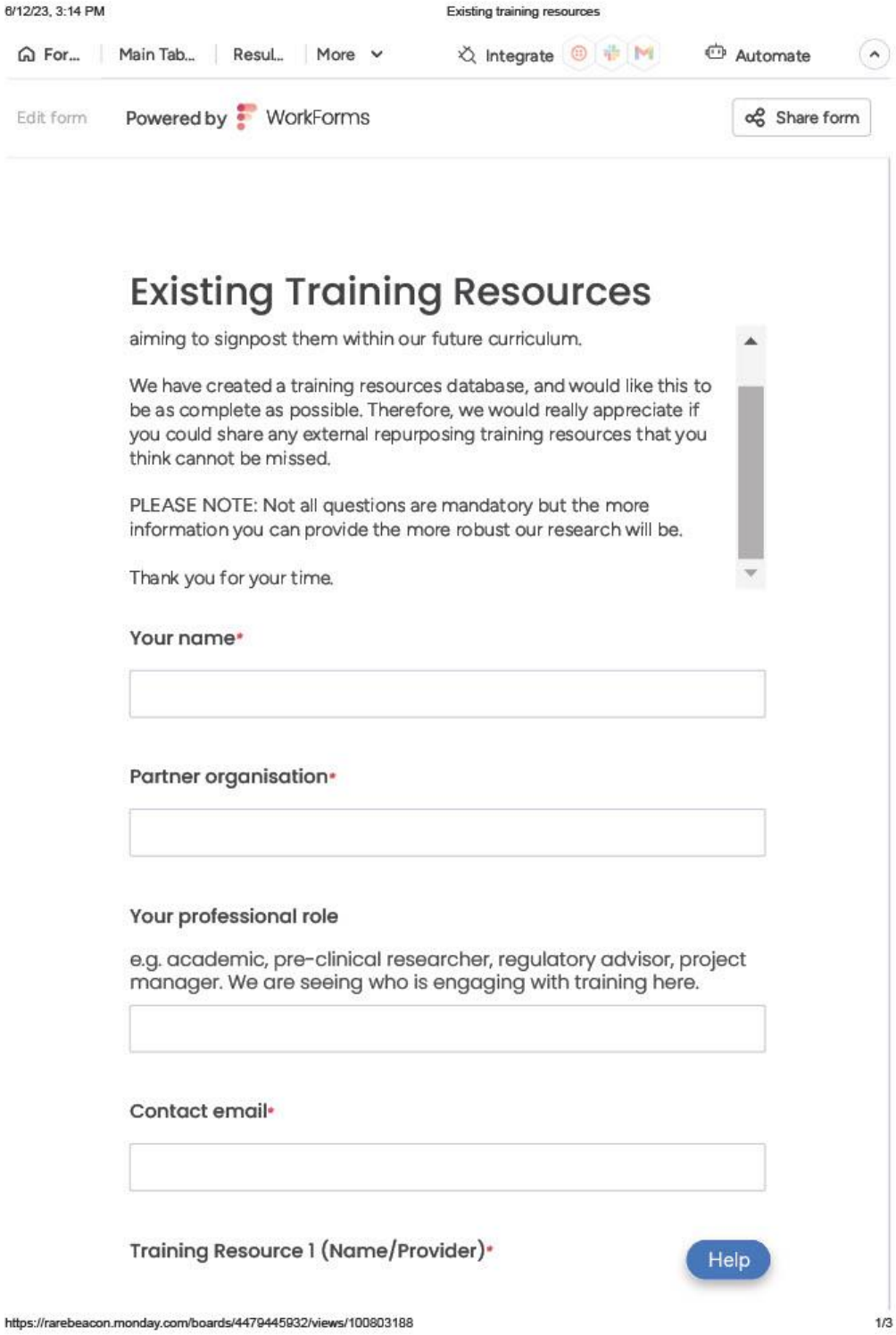
Figure 18: How the current training landscape has informed the structure of our planned curriculum – ensuring consistency of breadth & depth across all subjects while including items targeted at all stakeholders who are relevant to drug repurposing.

ANNEXES

ANNEX I. List of external interviewees for research

Group	Attendee(s)	Affiliated Organisation(s)	Method
Patient Group (Naïve)	Jess Hobart Sophie Muir Amanda Cordell Christine Mutena Isobel Davies	The UK Mastocytosis Support Group Timothy Syndrome Alliance EOS Network Rare Disease Kenya PEM Friends	Focus Group
Patient Group (Experienced)	Will Evans	Niemann Pick UK	Interview
Academics (Experienced)	Timothy Dreyer Professor Timothy Barrett Dr Lakshminarayan Ranganath Dr Melita Irving Dr Michael Wright	UCB/University of Pretoria Wolfram Syndrome UK/University of Birmingham AKU Society/University of Liverpool Guy's and St Thomas' NHS Trust MCDS Therapy/Newcastle University	Focus Group
Industry	Kelly Gray Dave Brown	Astrazeneca (Open Innovation) Healx	Interview
Payer	Sheela Upadhyaya	Former NICE Now advisor/consultant	Interview
Funder UK	Joanna Davidge	LifeArc	Interview
Funder USA	Barbara Goodman & Clare Thibodeaux	Cures Within Reach	Interview
Researcher (larger charity)	Ravindhi Murphy	CRUK	Interview
Training Providers	Rosan Vegter Galliano Zanello Anneliene Jonker Dan O'Connor Marjon Pasmooij	EATRIS/EJPRD EJPRD IRDiRC – Drug Repurposing Guidebook Taskforce IRDiRC – Drug Repurposing Guidebook Taskforce IRDiRC – Drug Repurposing Guidebook Taskforce	Interview

ANNEX II. Survey to consortium partners regarding existing training



6/12/23, 3:14 PM Existing training resources

For... | Main Tab... | Resul... | More | Integrate | Automate

Edit form | Powered by WorkForms | Share form

Existing Training Resources

aiming to signpost them within our future curriculum.

We have created a training resources database, and would like this to be as complete as possible. Therefore, we would really appreciate if you could share any external repurposing training resources that you think cannot be missed.

PLEASE NOTE: Not all questions are mandatory but the more information you can provide the more robust our research will be.

Thank you for your time.

Your name*

Partner organisation*

Your professional role

e.g. academic, pre-clinical researcher, regulatory advisor, project manager. We are seeing who is engaging with training here.

Contact email*

Training Resource 1 (Name/Provider)*

Help

https://rarebeacon.monday.com/boards/4479445932/views/100803188 1/3

Where to find Training Resource 1

Please provide a weblink to any live online training resources, or a URL for any upcoming or past events. If events are in person, please attach schedule or registration link.

Thoughts and comments on Training Resource 1

Please include likes, dislikes, what worked well and what was missing.

Training Resource 2 (Name/Provider)

Where to find Training Resource 2

Please provide a weblink to any live online training resources, or a URL for any upcoming or past events. If events are in person, please attach schedule or registration link.

Thoughts and comments on Training Resource 2

Please include likes, dislikes, what worked well and what was missing.

Help

Training Resource 3 (Name/Provider)

Where to find Training Resource 3

Please provide a weblink to any live online training resources, or a URL for any upcoming or past events. If events are in person, please attach schedule or registration link.

Thoughts and comments on Training Resource 3

Please include likes, dislikes, what worked well and what was missing.

Would you like to add any further Training Resources to our list?*

Select your answer

Submit

Help

ANNEX III. Non-exhaustive list of major search terms

Major search terms (non-exhaustive list)
High level training topics: • Drug repurposing training/ drug repurposing resources • Drug development training/ drug development resources • Translational research training/ translational research resources **Specifying the type of training:** • Courses on drug development • Courses on drug repurposing • Courses on translational research • Webinars on drug development • Webinars on drug repurposing • Webinars on translational research • Drug development workshop • Drug repurposing workshop • Translational research workshop **Specifying the target audience:** • Trainings for researchers on drug repurposing • Trainings for researchers on drug development • Trainings for researchers on translational research • Trainings for patients on drug repurposing • Trainings for patients on drug development • Trainings for healthcare professionals on drug repurposing • Trainings for healthcare professionals on drug development • Trainings for healthcare professionals on translational research **Specific topics arisen from the above search terms:** • Drug repurposing regulation trainings/resources • Drug development regulation training/resources • Pharmaceutical market access training/resources • Patient engagement trainings/resources • Drug repurposing in rare diseases training/resources • Clinical trials trainings/resources • Research in drug repurposing trainings/resources • Training on research methodologies for drug repurposing • *In silico* tools trainings/resources