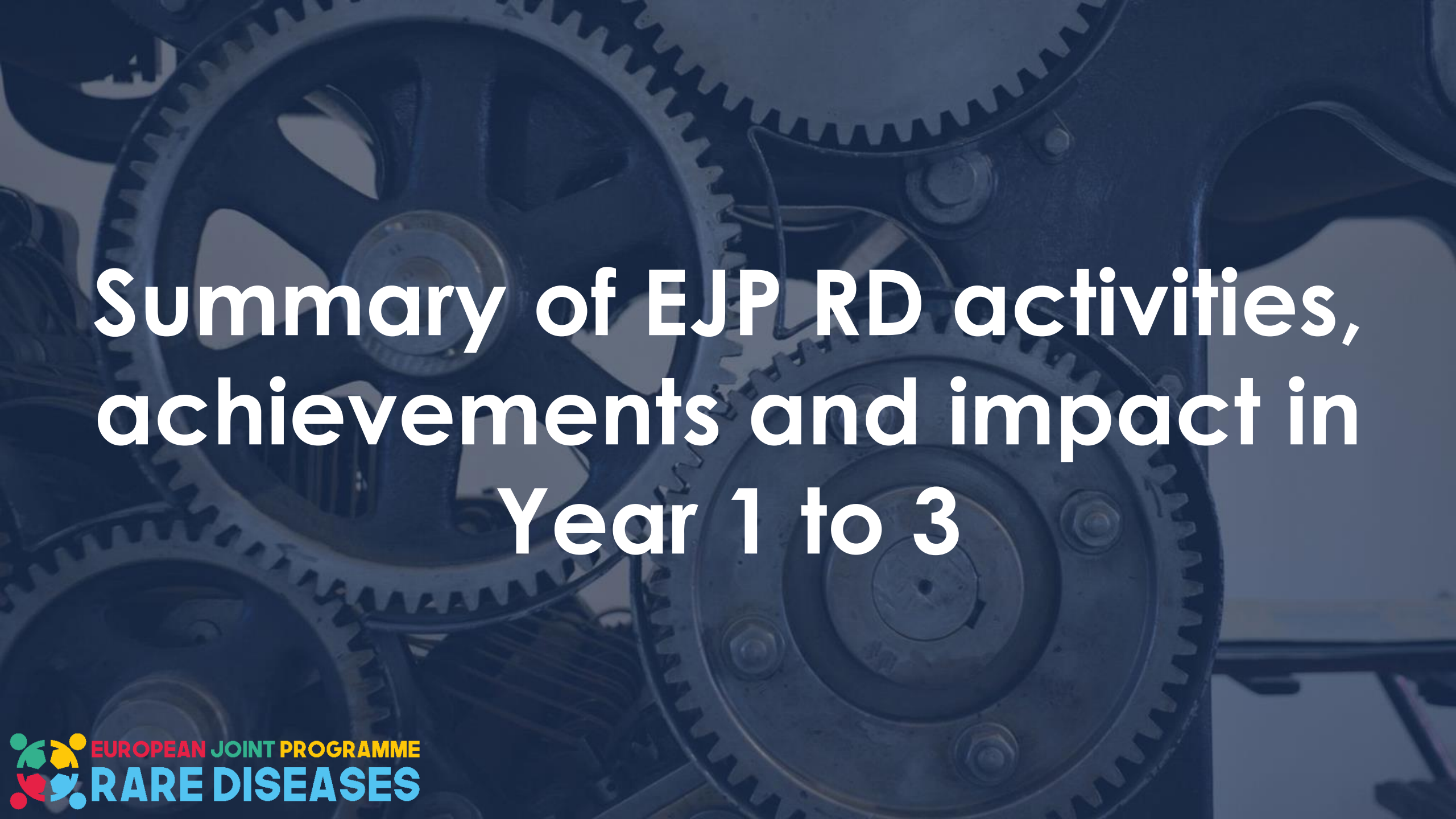
Annex 2 – Slides presented during the EJP RD Policy Board and Governing Board meeting

07/07/2021

EUROPEAN JOINT PROGRAMME ON RARE DISEASES (EJP RD)

EUROPEAN JOINT PROGRAMME
RARE DISEASES



Summary of EJP RD activities, achievements and impact in Year 1 to 3

EUROPEAN JOINT PROGRAMME
RARE DISEASES

Coordinated by Inserm
La science pour la santé / From science to health

EJP RD in numbers

1339 people

35 participating countries

26 EU MS, 7 associated (AM, CH, GE, IL, NO, RS, TK), UK and CA

ALL 24 ERNs

101 M€ Budget

Union contribution: 55 M€ (70% reimbursement rate)

85% of

European RD research community (directly or indirectly) involved in EJP RD

91 beneficiaries

10 hospitals
12 research institutes
31 research funding bodies/ministries
27 universities/hospital universities
5 EU infrastructures
5 charities/foundations
EURORDIS

+ 52 linked third parties
+100% associated networks

EURORDIS RARE DISEASES EUROPE
eatris
elixir
INFRAFRONTIER mouse disease models
European Reference Networks
ECRIN EUROPEAN CLINICAL RESEARCH INFRASTRUCTURE NETWORK
BBMRI-ERIC gateway for health

EJP RD – single entry point & solutions for all

RESEARCHERS

Funding

Research support services

Training at every stage

Access to resources & tools

Access to extensive network & expertise

CLINICIANS

Clinical studies support services

Support for registries

Access to resources & tools to accelerate diagnosis

Access to extensive network & expertise

Funding

PATIENTS

Access to RD specific expertise

Networking

Training at every stage

Access to resources & tools

Access to extensive network & expertise

Funding

POLICY MAKERS & FUNDERS

Joint funding & strategy

Optimisation of investment in research

Access to support for national RD community

Access to extensive network & expertise

Holistic impact evaluation

INTERNATIONAL PARTNERS

Access to extensive RD network & expertise

Multiple collaboration opportunities

Possibility of alignment

Access to resources & tools

EJP RD – A glimpse on 30 months work

Coordination & transversal activities

Qualified coordination team & support

Agile governance & strategy

Sustainability planning from 1st day

Extensive ethics & regulatory support

Perfomant communication & dissemination

Accelerating of research translation & clinical studies

Innovation Management Toolbox created

19 projects mentored

DB of funding opportunities

3 demonstration projects + 2 Innovation projects

Collaboration with EMA established

Capacity building & empowerment

7 F2F + 9 online courses

500 participants trained

15 ERN workshop financed

33 ERN fellowships attributed

1st Online education MOOC created

RD research funding

2 JTCs – 55 M€ - 40 projects

18 Networking events – 487 K€

3 RD Research public-private challenges

78% funded projects involve patient organisations

Access to data, tools & services

VP building blocks developed & upgraded (incld. Metadata model)

First set of resources linked

Pilot tools to query resources & data discovery in test phase

70 biological pathways created

EJP RD mid-term evaluation

- 2 days evaluation (16 & 19 of April 2021), 5 experts from EU & US, in presence of all ExCom
- Final report not yet available

- Some recommendations provided by experts during the online review meeting:
  - IMPACT: the impact measurement is very important! It should be presented in more visible way. Stories, specific examples are important. Graphical representation of participation of patients in projects (geographical coverage). The statistics from trainings can be expanded.
  - Use the network of Horizon EU delegates in different countries! To spread the information about RDs (research, training, etc)
  - Work on the connection between P2 and P4
  - Transmit at EU level the best practices from training activities – train the trainers; show them to NMG
  - Public-private partnerships are key!
  - Better "advertise" the RDs also as starting point to understand other (more common) diseases
  - Disseminate the standards at all levels, to make sure they become "gold standards" at all levels

EJP RD EXPECTED IMPACTS

EUROPEAN JOINT PROGRAMME
RARE DISEASES

EJP RD monitoring obligations & system

- In Grant Agreement Article 23: EVALUATION OF THE IMPACT OF THE ACTION → The Commission may carry out interim and final evaluations of the impact of the action measured against the objective of the EU programme.
- In Annex 1: Definition of expected impacts and measures to maximise impact (dissemination & communication)

INTERNAL PERFORMANCE

- 32 KPIs & 73 KRIs defined for 20 WPs
- In line with the objective of each WP
- Re-evaluated each year
- Allow on measurement of overall operational performance but not always linked to specific impacts

- Quantitative indicators collected via EC reporting system + qualitative annual report

FUNDED PROJECTS

- Yearly reporting including specific quantitative (e.g. N° of publications, patents, students trained, genes discovered, etc.) and qualitative (narrative report)
- Depending on the funding body additional national) report may be required
- Not linked to the EC monitoring system & specific EJP RD impacts

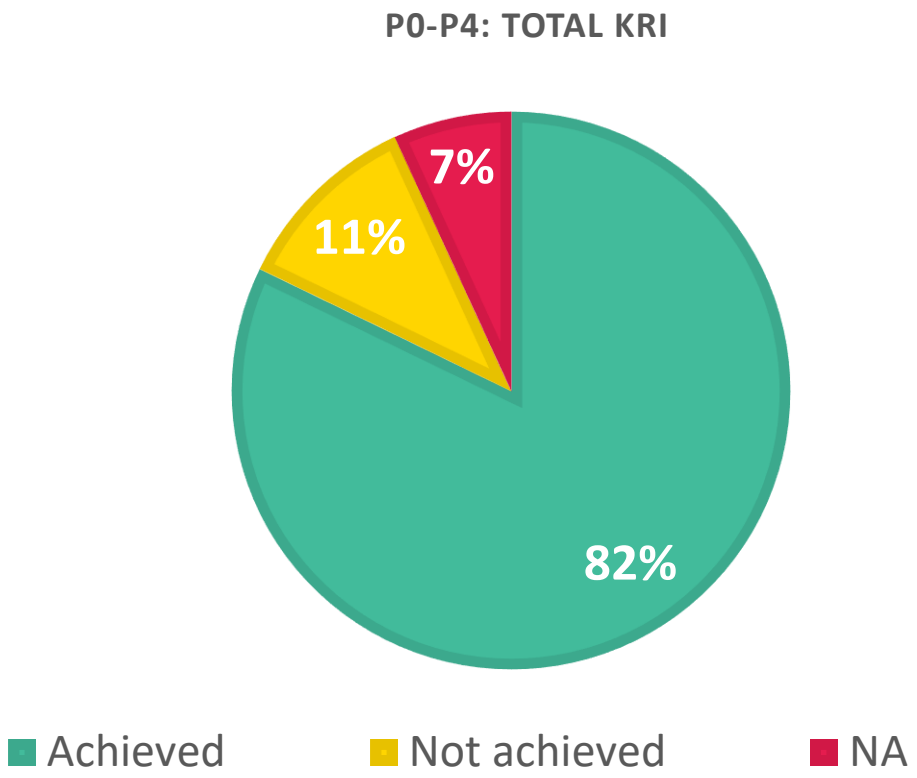
NATIONAL ALIGNEMENT

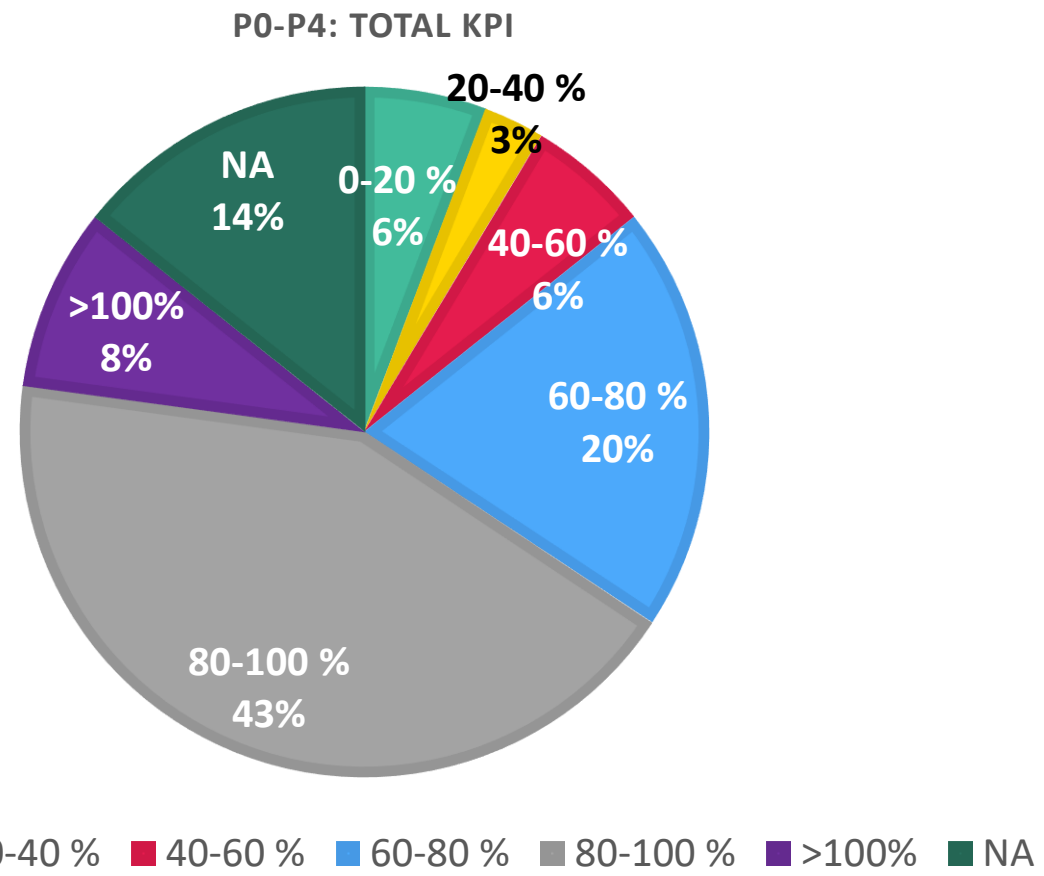
- Yearly survey to evaluate the reported alignment of national activities/actions with EJP RD

SUCCESS STORIES

- Reported for each Pillar towards EJP RD set impacts

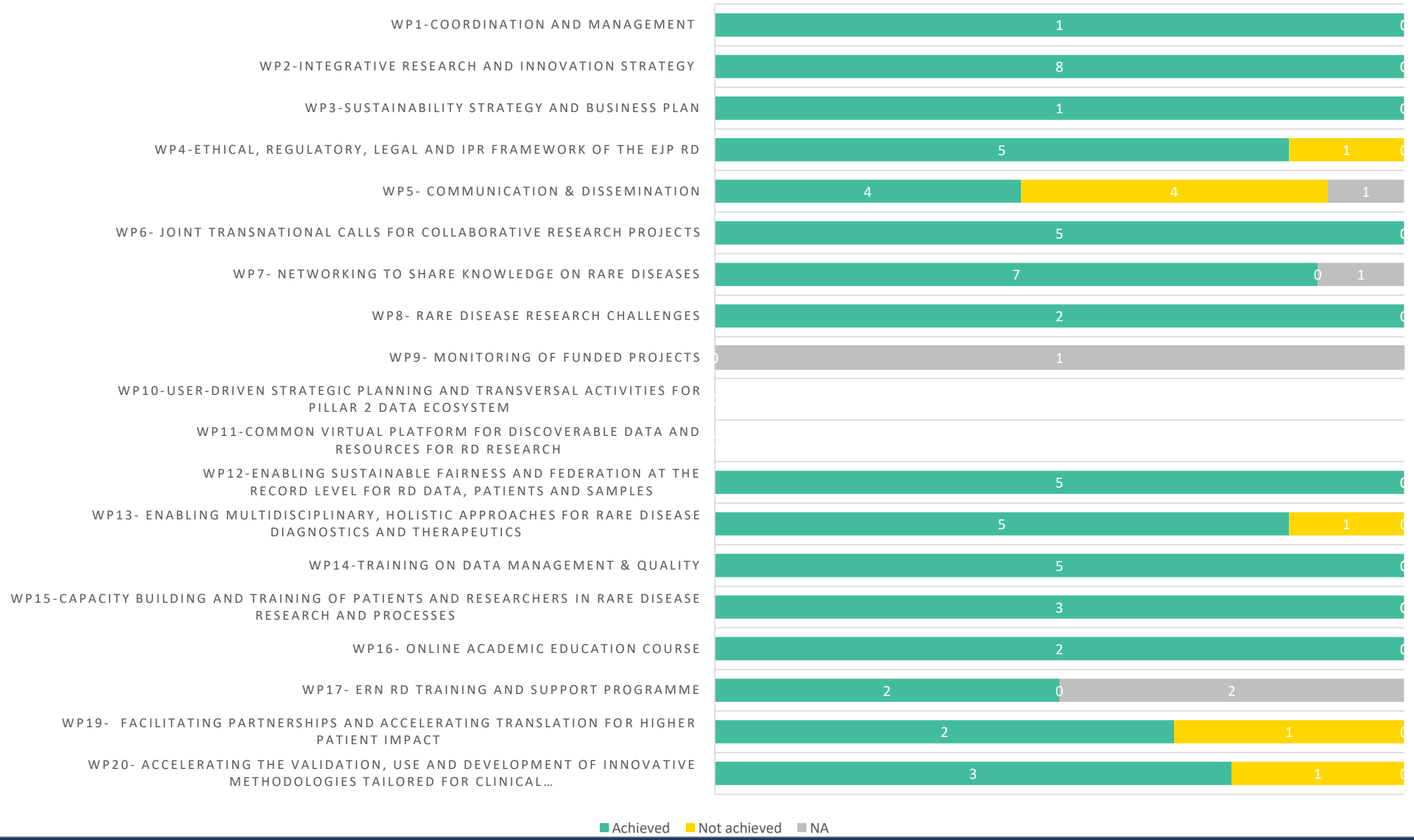
EJP RD INTERNAL PERFORMANCE (2nd monitoring report)





Not all KPIs were set at the start for 5 years objectives

EJP RD INTERNAL PERFORMANCE (2nd monitoring report)



EJP RD INTERNAL PERFORMANCE (2nd monitoring report)

- The indicators were marked as N/A either because its measurement is related to the activities that should take place within following years or the activity has been postponed to later due to the COVID-19

- The 80% of not achieved activities require revision of the indicators – need for requalification of KRIs into KPIs → e.g. the proposed targets N° of new followers on twitter or subscriptions to NL should be considered as KPI for which 100% is to be achieved by end of year 5 and not KRI to be achieved every year

- Some of the activities (e.g. N° of datasets or N° of pathways) perfomed beyond the set target → should be targets for subsequent years be revised?

Overall impact: Improved alignment of national/regional activities and policies in RD

- **Increased awareness of the rare diseases research ecosystem** – EJP RD is featured on websites of national and regional funding bodies, research institutions, all ERNs and patients organisations (e.g. 9710 results on google)

- **Initiation and/or empowerment of National Mirror Groups** bringing all RD stakeholders (e.g. creation of NMG in the Netherlands, Poland and Portugal, full alignment of actions between National Plan for Rare Diseases and EJP RD in France).

- **Alignment with national strategies is now visible**: e.g., in France the EJP RD work, notably in relation to implementation of federated Virtual Platform, standards, ontologies and methods used, is indicated as mandatory for the alignment of national resources (newly created or to be updated rare diseases registries and/or databases), cohorts and health data hub that will host RD data.

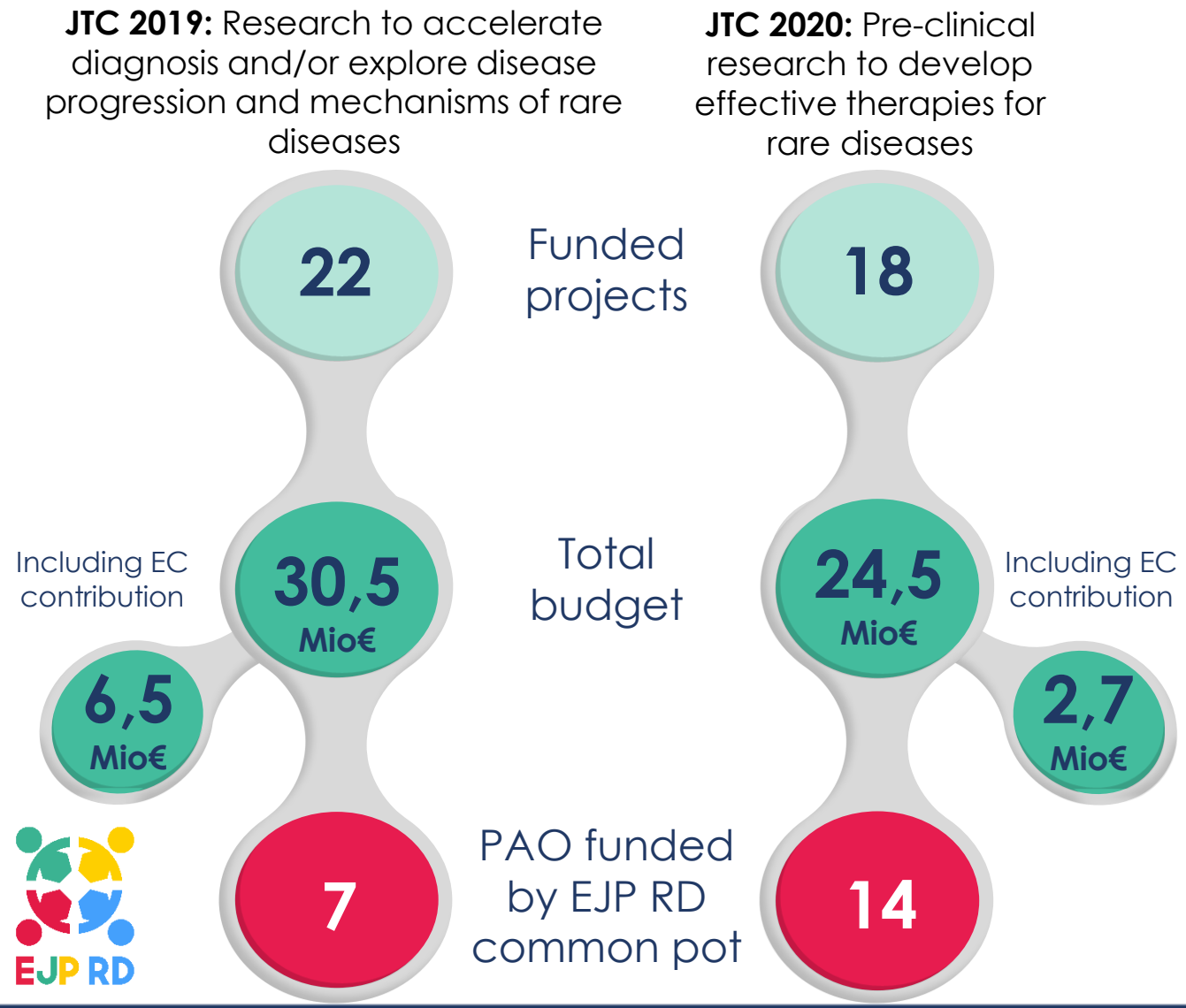
- **The EJP RD standardization work is featured in the calls for projects** of the European Commission/Innovative Medicines Initiative and national calls as reference/recommendation that needs to be taken into account by applicants.

- **The work between ERNs and EJP RD on the registries and related Informed Consent Form** resulted in adaptation of the original ICF template (provided by the European Commission) to include national specificities and facilitate the validation of ERN registries by national ethics committees (out of 24 ERNs 11 use the new ICF, 5 working on adaptation, others already submitted but revise the current ICF).

- **Between 23 and 86,6% of national activities are aligned or complementary to EJP RD** actions (23% for P4 innovative methodologies in CTs and 86% for support of data repositories and tools)

Specific impact 1: Improve lives of rare disease patients by providing new and optimised treatment options and diagnostic tools for these diseases

55 M€ INVESTED IN RESEARCH



BEYOND-OMICS APPROACHES

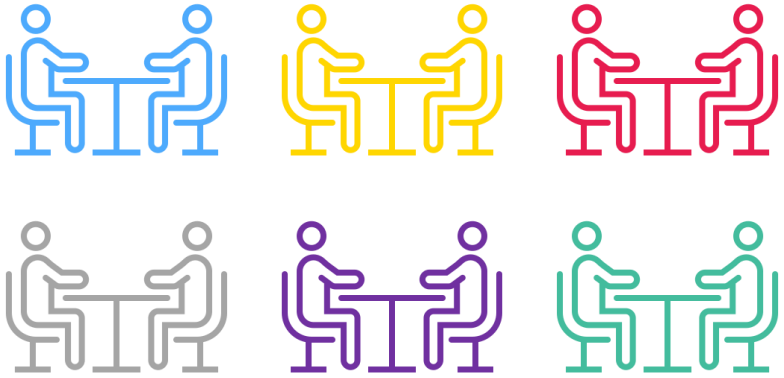
- Rare disease portal on WikiPathways: **70 RD pathways created** to date http://raredisease.wikipathways.org
- **Inborn errors of metabolism**: Pathways and portal included in Blau et al. textbook
- **Network analysis methods** of the **Huntington's Disease** Use case: **guiding the creation of RD networks**
- **Use case: Congenital Anomalies of Kidney and Urinary Tract:** curated CAKUT pathways – identification of implication of vitamin A & D in the genesis of CAKUT

Specific impact 1: Improve lives of rare disease patients by providing new and optimised treatment options and diagnostic tools for these diseases

ACCELERATE RESEARCH BY MENTORING

70+ experts recruited to provide mentoring for the research planning, funding and execution process

In **2019**, 3 applications received. 1 received full mentoring.



In **2020**, 16 requests were made, 15 from JTC2020 applicants.

11 projects were mentored of which 8 received funding.

2021 Currently 13 total mentoring requests from European Commission webinar, JTC 2020 funded projects, Follow-on JTC 2020 mentoring, Telethon Project

Specific impact 2: Decrease fragmentation of rare diseases expertise and research resources

EJP RD Helpdesk

over **300** experts in the current database
Expansion to other resources (paediatric, regulatory expertise from other networks)

In under-represented countries

Widening in JTC2020: 14 new partners included in full proposals

Among different types of stakeholders:

- 138 patient advocates and 14 researchers trained in 2019-20
  - trained in **medicine research and development**: 54 RD patient advocates and 14 RD researchers
  - trained in **translational research and scientific innovation**: 28 RD patient advocates
  - trained in **ERNs, healthcare and leadership** topics : 56 ePAG advocates
- 15 research-focused trainings delivered, 389 participants in total (around 25-30 participants per training)
- 1767 persons enrolled in the MOOC training on RD diagnosis

Specific impact 2: Decrease fragmentation of rare diseases expertise and research resources

16 Resources for Research enhanced

Working locally to make the whole ecosystem sustainable

- Adapted to connect to ELIXIR/LifeScience AAI: **unique login**
- Phenostore: **improved management of phenotypic data**
- Improvements for RD data archive, discovery and access: **adapted for RD**
- Increased number of data collected for RD researchers
- Increased awareness (resource webinars)

BBMRI-ERIC

DECIPHER GRCh37

Cellosaurus

ERDRI.dor

EUROPEAN GENOME-PHENOME ARCHIVE

hPSCreg

INFRAFRONTIER mouse disease models

MetaboLights

PRIDE PRoteomics IDEntifications Datab

RaDiCo Rare Disease Cohorts

RD-Connect GENOME-PHENOME ANALYSIS PLATFORM

RD-Connect SAMPLE CATALOGUE

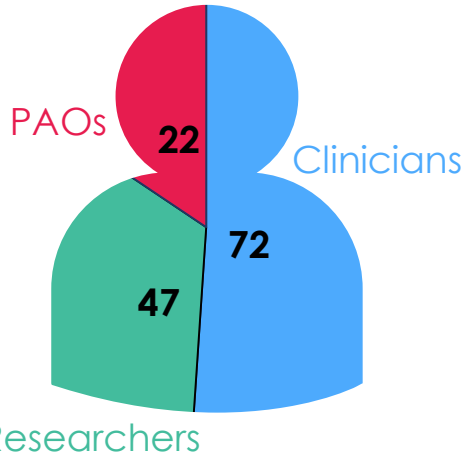
EJP RD

Specific impact 4: Improve healthcare systems' capacity to take up research results

The **joint transnational call 2021** focused on funding of research related to **socio-economic, health care/health services, e-health and studies addressing the impact/burden of the delay in diagnosis and of the lack of therapeutic interventions**. This is the first time such type of multinational research is financed, with the aim of up taking the results to provide direct guidance/recommendations and impact healthcare systems and practices.

Accelerated share of knowledge & increased uptake of research results

Partners in the 18 NSS selected projects (Round 1 to 4)



Making ERN registries FAIR at the source to improve data sharing and re-use aiming to increase health data secondary use for research purposes

	Note: If an ERN has a tool or standard that is not in the list, please add a new row and the tool name to the list		ERN PaedCan	ERKNet	MetabERN
Phase	Function	Tool/ Standard name	PARTNER	ERK-F	
1 Modelling	Data Model	CDE Semantic Model			
1 Modelling	Set of data elements	Common Data Elements JRC	Implemented	Imple	
1 Modelling	Genes ontology	HGNC		Imple	
1 Modelling	Variant Ontology	HGVS		Imple	
1 Modelling	Phenotype ontology	HPO			
1 Modelling	Catalogue of somatic mutations i	COSMIC			
1 Modelling	Mendelian Inheritance Ontology	OMIM			
1 Modelling	Genes ontology	HUGO			
1 Modelling	Systematized Nomenclature of M	SNOMED CT			Non applicable
1 Modelling	International Classification of Dis	ICD-10			Non applicable
1 Modelling	International Classification of Dis	ICD-11			Non applicable
1 Modelling	eagle-i resource ontology	ERO			
1 Modelling	Minimum Information About Biol	MIABIS			
1 Modelling	National Cancer Institute Theasau	NCIT			
1 Modelling	Anatomical Therapeutic Chemica	ATC			Implemented
1 Modelling	Genotype ontology	GENO (for allelic state)			
1 Modelling	International Classification of Dis	**ICDO (oncology)**	Non applicable		
1 Modelling	Code system for medicaments	ATC			Implemented
1 Modelling	NMD ontology	**<getting the name>**			
1 Modelling	Disease concept ontology	ORDO		Implemented	Implemented
1 Modelling	International Classification of Chi	ICCC 3	Implemented		
1 Modelling	International Classification of Ped	ICPED			
1 Modelling	Logical Observation Identifiers N	LOINC			

The FAIRification Stewardship Programme

77 FAIR standards and Tools mapped with 30 ERN registries

Specific impact 5: Reinforce the EU's role as a global leader for rare diseases

- **EJP RD is recognized as major player** in the field of RDs by EU and international stakeholders

Global Alliance for Genomics & Health
Collaborate. Innovate. Accelerate.

EJPRD is actively contributing in the development and expansion of global standards for genomic data sharing

B1MG 1+MG

EJPRD is contributing & providing PoC elements federated model, standards, ontologies building blocks for genome-phenome data federation for clinical research & healthcare

conect 4children
COLLABORATIVE NETWORK FOR EUROPEAN CLINICAL TRIALS FOR CHILDREN

EJPRD collaborates with C4C to mutualise expertise for paediatric clinical trials, share guidelines & knowledge (e.g. training, clinical trials SOPs)

CRITICAL PATH INSTITUTE

The expertise of EJP RD in data modelling and standardization led to a joint proof-of-concept testing the query of data provided by both parties through EJP RD metadata models, ontologies and standards paving the way to interoperability between EJP RD and RDCA-DAP resources.

EJPRD already engaged in the interaction with stakeholders involved in building the EHDS to contribute with its developments (VP) & support RD community

Specific impact 6: Follow the policies and contribute to the objectives of the International Rare Diseases Research Consortium (IRDiRC)

Consortium Assembly

10 FCC members
1 PACC member

Scientific Committees

6 EJP RD representatives involved in IRDiRC Scientific Committees

Task Forces

10 EJP RD members serving in IRDiRC Task Forces

Joint Action

Machine Readable and Computable Consent

Resource Integration

ODDG into WP19 Innovation Management Toolbox

Topic Identification

ELSI and WG3 feeding the JTC call on SHS

IRDiRC experts advising on possible topics in all EJPRD calls

IRDiRC
INTERNATIONAL RARE DISEASES RESEARCH CONSORTIUM

Other substantial impact(s): Contribution to the European Open Science Cloud

- The whole EJP RD platform and resources are EOSC “ready” (FAIRified, using same data models)
- EJP RD aims at being rare diseases specific resource within EOSC

IMPACT MONITORING UNDER HORIZON EUROPE

EUROPEAN JOINT PROGRAMME
RARE DISEASES

New approach to European Partnerships: common elements

- All European Partnerships are designed in line with the new policy approach for more **objective-driven** and impactful partnerships (draft proposals on Europa website)
- Are based on a **Strategic R&I Agenda** agreed among partners and with EC
- For each of them the **objectives, key performance and impact indicators, and results to be delivered**, as well as the related **commitments** for contributions of the partners will be **defined ex-ante**.
- Common approach to **monitoring and reporting** is to track progress towards objectives and improve the understanding of the added value of partnerships (what would not have happened?)

NB! Lesson-learned: Several interim evaluations expert groups call to re-visit and re-define the whole set of KPIs on partnerships, and to make sure that partnerships are assessed also in their proper policy context.

European Commission

European Partnerships: monitoring criteria

a) *A monitoring system **in line with the requirements set out in Article 45** to **track progress towards specific policy objectives**, deliverables and key performance indicators allowing for an assessment over time of achievements, impacts and potential needs for corrective measures;*

b) ***Periodic dedicated reporting** on quantitative and qualitative leverage effects, including on committed and actually provided financial and in-kind contributions, visibility and positioning in the international context, impact on research and innovation related risks of private sector investments;*

c) *Detailed information on the evaluation process and results from all calls for proposals within partnerships, **to be made available timely and accessible in a common e-database**.*

Source: https://data.consilium.europa.eu/doc/document/ST-7942-2019-INIT/en/pdf

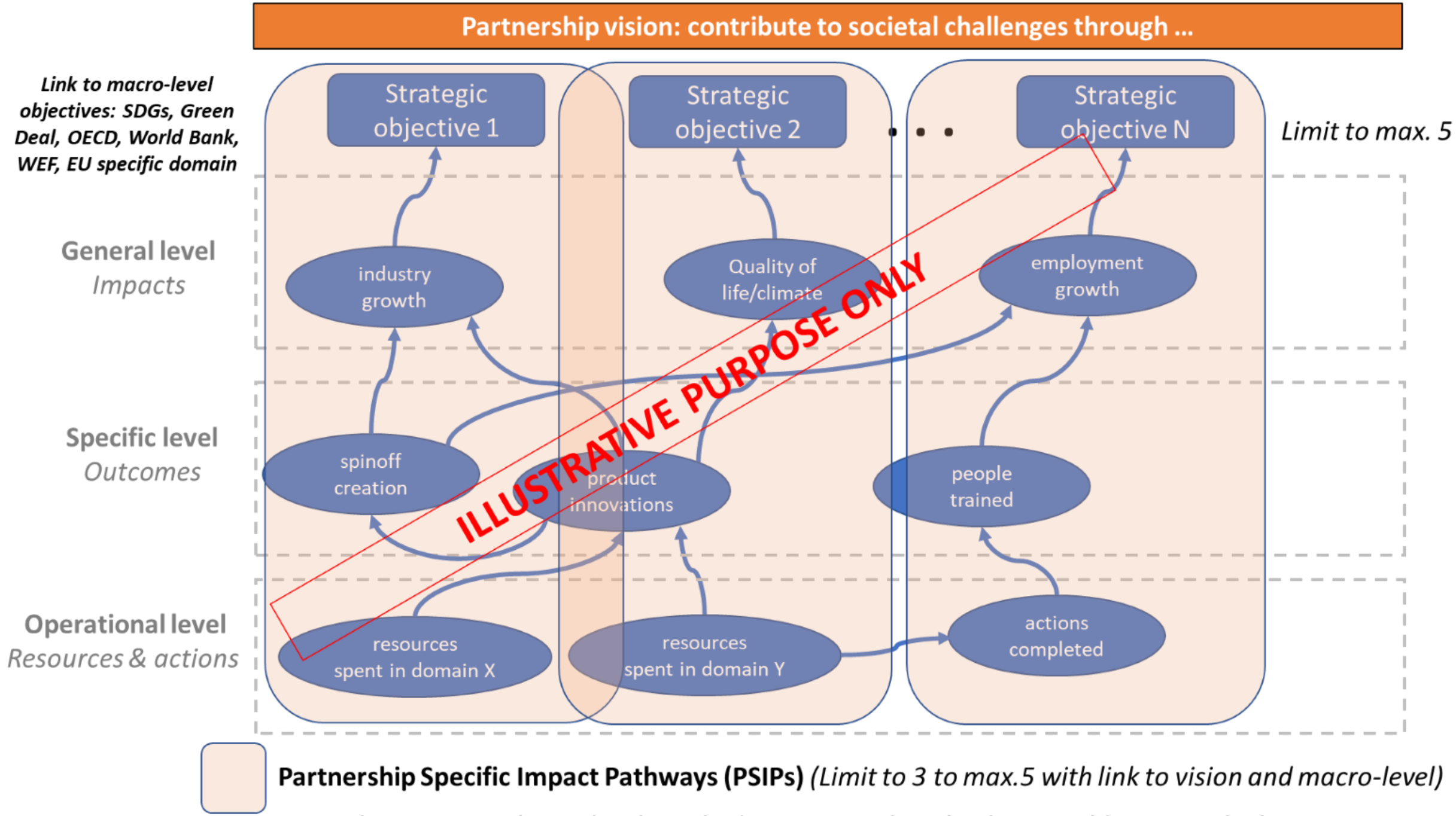
European Commission

HORIZON EUROPE PARTNERSHIPS - INDICATORS

	1. HE Key Impact Pathways (project level)	2. European Partnership (programme level)	3. Functioning of the partnership. (incl. criteria)
INSTITUTIONAL (Art 187)	Art187 Automatic, COM IT tools Art 185 Metrology	To be developed by partnerships (COM in collaboration with partners). Supported by the expert group.	To be developed by the expert group (common to all)
CO-FUNDED & INSTITUTIONAL (Art 185)	Own reporting (national IT systems) but with data import into EC systems		
CO-PROGRAMMED	Automatic, COM IT tools	Important to avoid unnecessary overlaps with 1 & 3	

Biennial monitoring report of EU R&I Partnerships and Horizon Europe evaluations

European Commission


Partnership vision: contribute to societal challenges through …
Link to macro-level objectives: SDGs, Green Deal, OECD, World Bank, WEF, EU specific domain
Strategic objective 1
Strategic objective 2
Strategic objective N
Limit to max. 5
General level
Impacts
Specific level
Outcomes
Operational level
Resources & actions
industry growth
Quality of life/climate
employment growth
spinoff creation
product innovations
people trained
resources spent in domain X
resources spent in domain Y
actions completed
ILLUSTRATIVE PURPOSE ONLY
Partnership Specific Impact Pathways (PSIPs) (Limit to 3 to max.5 with link to vision and macro-level)
Note: this concept relates closely to the 'intervention logic' – these could serve as the basis

Revision of the EJP RD monitoring framework & preparation for Horizon Europe

- **Major points:**
  - **Redefine/reformulate the objectives** to clearly identify three levels (limit the number to 3 objectives per level and 5 indicators per level):
    - General (impacts), connected to macro-level HE goals, SDGs
    - Specific (objectives), focused on EJP RD outcomes
    - Operational, related to internal monitoring
  - **Re-connect the existing indicators** to objectives and impacts
  - **Re-connect different parts of the monitoring system** (internal, funded projects, alignment) between them and to impacts & objectives
  - Explore the possibilities of **adaptation of current project submission-evaluation-monitoring system** to fulfil the criteria (mandatory dataset & API) of linkage with EC monitoring system under HE

THANK YOU

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https://www.youtube.com/channel/UCdZPkPpGydUV7cBqrQogmmQ

Follow us on social media

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The EJP RD initiative has received funding from the European Union's Horizon 2020 research and innovation programme under grant agreement N°825575

EUROPEAN JOINT PROGRAMME RARE DISEASES

EJP RD update on the Annual Work Plan for year 4

EUROPEAN JOINT PROGRAMME
RARE DISEASES

What's new in Pilar 0 in AWP Y4

WP1: Coordination & Management

- Focus on support & featuring of EJP RD outputs & outcomes
- Redefinition of the monitoring framework to HE standards
- Preparation of RD Partnership (in close connection with activities of WP2, national bodies)
- Implementation of IRDiRC Roadmap 2022

WP2: Strategy

- Overall support for EJP RD strategy:
  - To define topic of JTC2023
  - To align with national strategies
  - To facilitate prioritisation

What's new in Pilar 0 in AWP Y4

WP3: Sustainability

- Development of the catalogue of EJP RD "services" *(continued in Y4: dynamic catalogue associated with D3.1)*
- Development of the business plan for each of the EJP RD outputs & for EJP RD as a whole (in close collaboration with WP1, WP2 & WP4)

WP4: Ethical, regulatory, legal and IPR support

- Continue to support all EJP RD partners (ethics monitoring or evaluation of funded projects, support on demand from WPs/pillars, continuous information on ethics/regulatory/legal updates)
- Work in connection with WP3 on identified IP needs

What's new in Pilar 0 in AWP Y4

- **WP5: Communication & Dissemination**
  - Boost of the EJPRD communication strategy with new tools:
    - Instagram, "Takeovers", influencers
  - New videos
  - Impact of EJPRD NL
  - Expansion of EJPRD partners communication managers network & connected actions
  - Revision of the IRDiRC website
  - Expansion of IRDiRC communication strategy to disseminate and publicise the work of IRDiRC members amongst the RD community

PILLAR 1
Funding of research

4th ANNUAL WORKPLAN

New activities

EUROPEAN JOINT PROGRAMME
RARE DISEASES

What's new in Pillar 1 in AWP Y4

- **WP6 - Joint Transnational Calls (DLR)**
  - Co-leader of WP6: ANR
  - Addition of JTC 2023
    - Topic to be decided in collaboration with WP2
  - Follow-up Workshop on Guide for Patient Partnership in Rare Disease Research projects

- **WP7 - Networking Support Scheme (ZonMw)**
  - Face-to-face, online and hybrid events possible (from mid 2021)
    - Results of surveys in Early assessment (Year 3)

- **WP8 - Rare Diseases Research Challenges (FFRD)**
  - No new activities/major changes

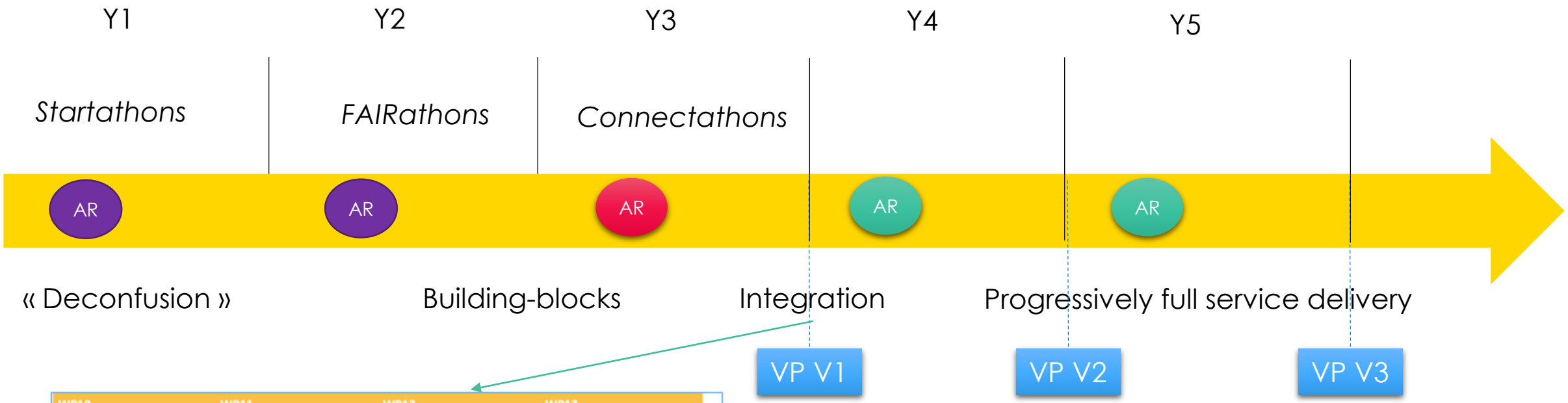
- **WP9 - Monitoring funded projects Pillar 1 (CSO, MOH)**
  - Instalment of a monitoring working group (starting in Year 3)

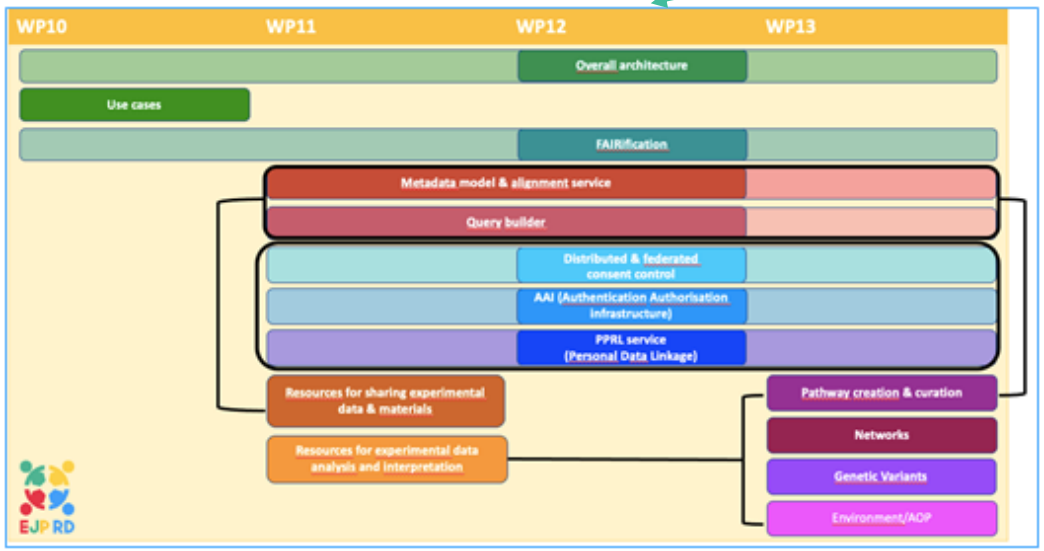
PILLAR 2 4th ANNUAL WORKPLAN

in a nutshell

EUROPEAN JOINT PROGRAMME
RARE DISEASES

Towards subsequent version of the VP





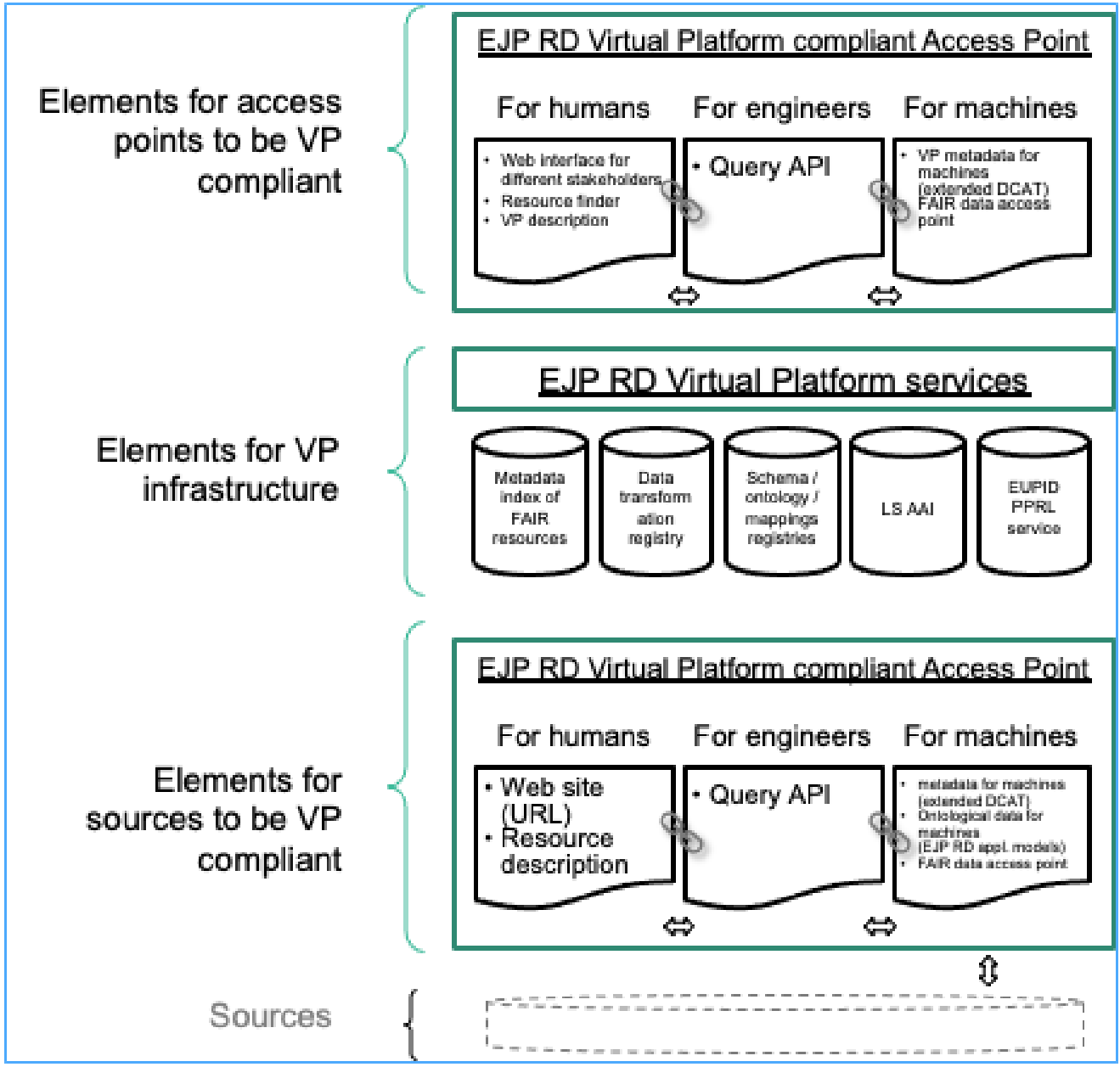
ViPS v2

GDPR
Quality
FAIRness
Sustainability

EJP RD

WP11 & WP12

- Scale-up methodology for resources joining the VP
- Sustain & scale FAIR stewardship with stakeholders, beyond registries
- Going wider:
  - Expand the number of resources in the VP (Knowledge bases)
  - Expand the items by which a resource can be queried
- Going deeper:
  - Bridge resource-level MetaData Model (MDM) & record-level MDM
  - Develop resource-level +record-level QB pilots
  - Continue resources enhancements
    - Inter-connections
    - AAI
    - PPRL



WP13 (+WP11, +WP12): System biology approaches

- Integration of tools, workflows and data with the VP
- Collaborate with WP11 for that
- Solve issues with cloud data storage and cloud computing
- New WP13 use cases to be analysed in EJP-RD Sandbox/cloud, further developed along the lines of GA4GH standards
- Collaborate with WP12 for FAIRification of metadata
- Collaborate with Pillar 3 for training purpose

Additional deliverables

- Case study specific (proof-of-concept) and generic multi-omics analysis workflows as part of subtask 13.1.9 and in alignment with the deliverable D11.19 and subtask 11.4.2 (M48)
- Report for training purposes for Pillar 3 based on the workshops, analyses and VP deployment from pilot case studies (M48)

EJP RD

PILLAR 3
Training and Empowerment

4th ANNUAL WORKPLAN

New activities

EUROPEAN JOINT PROGRAMME
RARE DISEASES

What's new in Pillar 3 AWP Y4

WP14: Research training

- Data management and quality: pretty similar as Y3 in terms of content
- At least 2 additional national trainings on orphanet ontologies
- Hopefully back to residential trainings in year 4 instead of online (Years 2 and 3)

WP15: Patients training & empowerment

- **For 15.1 and 15.2**: similar to Year 3 (hopefully onsite instead of online but still TBC)
- Content adapted based on participants feedbacks and programme committees' input
- **For leadership training** (15.3): cancelled in 2021 (planned in Istanbul), hopefully in Rome in Y4. In Y5 double number of participants with reallocation of some unspent budget and increase in the number of fellowships (from 10 to 35%)
- **Pediatric training**: will be developed in Y3 for the first time
  - Several online workshops/bitesize webinars instead of the 3 days on site in Y3, in Y4 hopefully onsite

EJP RD

What's new in Pillar 3 AWP Y4

WP16: Online academic education course

- MOOC 1: Diagnosis - delivered
- MOOC 2: Innovative personalised therapies (first run Q3)
- MOOC 3: Translational research (first run Q1)
- MOOC 4: Methodologies in CTs to be delivered in Q4
- Will start to develop content of last MOOC 5 on ethics & regulatory processes (kick off call in June 2021)
- Strategy on accreditation of the MOOCs to be tackled
- Start impact assessment of MOOC 1

WP17: ERN workshops and fellowships

- Y4 will be very active to implement workshops and fellowships selected in previous calls
- Evaluation of the budget spent and needs to be done to adjust the scheme in last years

WP18: Additional training needs

- First programme of new training draft to be finalised in Year 3
- New training to be planned in Y4
- For some new trainings, awareness not sufficient: work on the adaptation and increase of awareness of trainings

EJP RD

What's new in Pillar 4 in AWP Y4?

EUROPEAN JOINT PROGRAMME
RARE DISEASES

WP19:Facilitating partnerships and accelerating translation for higher patient impact

19.1 Accelerating translation

- **Innovation Management Toolbox**
  Expansion and maintenance of the IMT and its integration within the Pillar 2 virtual Platform
- **Mentoring**
  - Analysis of newly onboarded mentoring project needs
  - Creating awareness among the RD community of the services provided by WP19 to facilitating partnerships and accelerating research translation, in collaboration with the Communication WP5.
  - Outreach to recruit new projects - advertise the RD community through joint conferences and newsletters.
  - Publication of the White paper

19.2 Support in exploitation and follow on funding

- **Support in exploitation**
  - Application writing support for high potential projects
- **Follow-on funding**
  - Support community through deployment of PoC funding radar

EJP RD

WP20: Accelerating the validation, use and development of innovative methodologies for clinical trials

20.2: Clinical Studies Support Office:

- Process increased demands due to the Horizon Europe call for funding on the topic "Development of new effective therapies for rare diseases"
- Networking Support Scheme (NSS) will have its second meeting

20.3 Demonstration projects:

- EBStatMAX, Improve-PSP, Epistop-IDEAL forecast to complete end of 2022.
- Identification of new CT methodologists' partners
- Disseminate Demonstration projects at mini-symposia that will be organized with the methodologists and projects' teams

20.4 Innovation in methodologies in CTs for RD:

- Expected to start at the end of Y3 (results of the calls in July 2021).
- New Partners will be added.
- Disseminate Innovation projects at mini-symposia (2 mini-symposia per year inviting stakeholders and regulatory).

20.5 Educational Program to disseminate Advanced Statistical Trial Methodologies in RDs

- **MOOC** on CT methodologies for rare diseases
- **Advanced Courses** will be organized in a form of Webinars with EJP-RD partners and collaborators(2 webinars /year)
- Additional recruitment from IOR (Lorena Casareto –L Sangiorgi): reinforce the publications activity and the webinar organization

EJP RD

Feedback on the AWP Y4

- Taking into account your overall knowledge of EJP RD and AWP Y4 : what is missing in AWP Y4?
- How can we still better integrate EU-13 countries ?
- How do you present and get back to your national stakeholders with the key points of the EJP RD AWP Y4 ?
- Are there additional training needs that need to be set and how to ensure better translation of training needs?
- How to make the research resources and data sources more visible for researchers in your country ?
- Taking into account EJP RD developments in previous years and year 4, how would you take them to promote better data structuring and standardisation in your countries (apart from the connection to the VP)?

Opening Remarks: Industry Perspective on Collaboration with Academia

Policy & Governing Board meeting

July 7th, 2021

EUROPEAN JOINT PROGRAMME RARE DISEASES

Simon Bennett
Biogen

Opening Remarks: Industry Perspective on Collaboration with Academia

Introduction

- Brief company background

Industry-academia collaborations across the medicine lifecycle

- Discovery and translational medicine
  - Psychiatry consortium
- Clinical Research
  - Pre-marketing authorisation
    - Large clinical studies with multiple investigator sites
  - Post-marketing authorisation
    - Industry-academia collaboration on a rare disease registry.
  - Foundational Research

Concluding comments

- Addressing the challenge of rare diseases cannot be met by individual stakeholders acting in isolation.
  - Need to create the right framework to facilitate end-to-end interactions

Industry – academia collaboration

Policy & Governing Board meeting

July 7th, 2021

EUROPEAN JOINT PROGRAMME RARE DISEASES

Anton Ussi
EATRIS ERIC

From Bench to bedside and back

It takes many sectors, actors and organisations to

- Understand disease
- Develop new medicines
- Improve healthcare processes

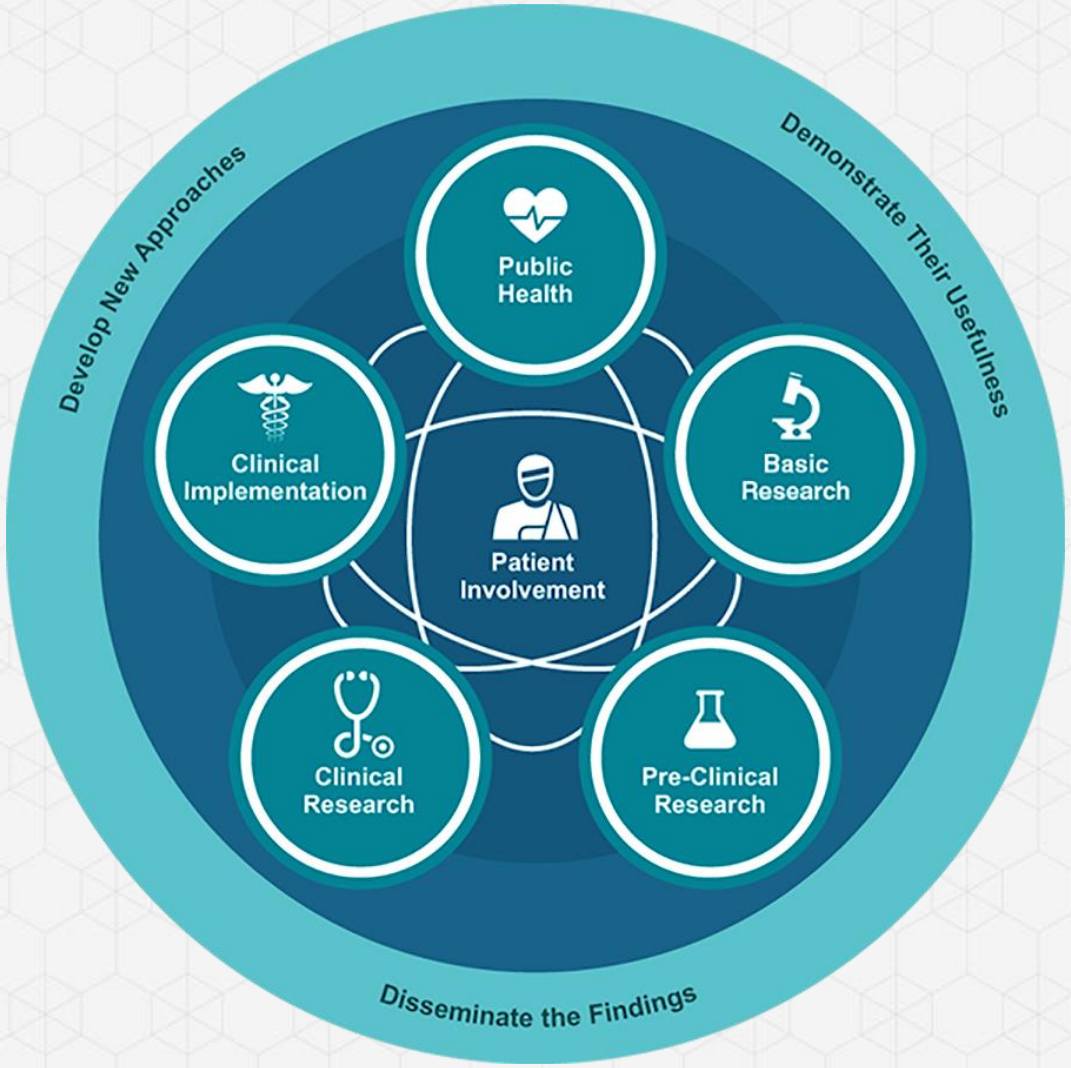
Patient need is the driver

Why collaborate?

Developing new medicines is complex, costly and time-consuming

- Ca. €2.5 billion
- 12 – 15 years

Different phases require different capacities



https://icts.uiowa.edu/about-us/translational-science

Translational Medicine

Patients and clinicians

- Patient need and clinical course
- Understanding of (patho)physiology
- Natural history of disease
- Design of endpoints

Academia

- Generates new knowledge about (disease) biology, biochemistry
- Source of new targets

Industry

- Also new knowledge
- Develops products based on new knowledge

Capacities

Patients and clinicians

- Daily experience
- Clinician scientists
- Healthcare processes
- Link phenotype to biology

Academia

- Exploratory research – creative bly sky approach
- Latest analytical technologies to
- “Knowledge for knowledge’s sake”

Industry

- Applying knowledge into practice
- Rigorous confirmatory research
- Huge financial resources needed
- Risk appetite

Areas of collaboration

1 **Patient registries**
Natural history, endpoints, find patients

2 **Develop new research tools**
Validate in context of use

3 **Biomarkers and clinical endpoints**
Identification, validation

4 **New products**
All along development path

Some do’s and don’ts

1. **Ensure transparency**
   Clear agreement on access rights
2. **Allow data access for product development**
   But with clear terms and limits
3. **Don’t be overly-reliant on industry resources**
   Need to maintain operational independence
4. **Close collaboration academia-clinic-industry**
   Is essential to enable advancement

WP8 Rare Diseases Research Challenges

Challenges & Opportunities

Policy Board meeting – 7 July 2021

Christine FETRO, French Foundation for Rare Diseases

WP8: Team/people & institutions involved

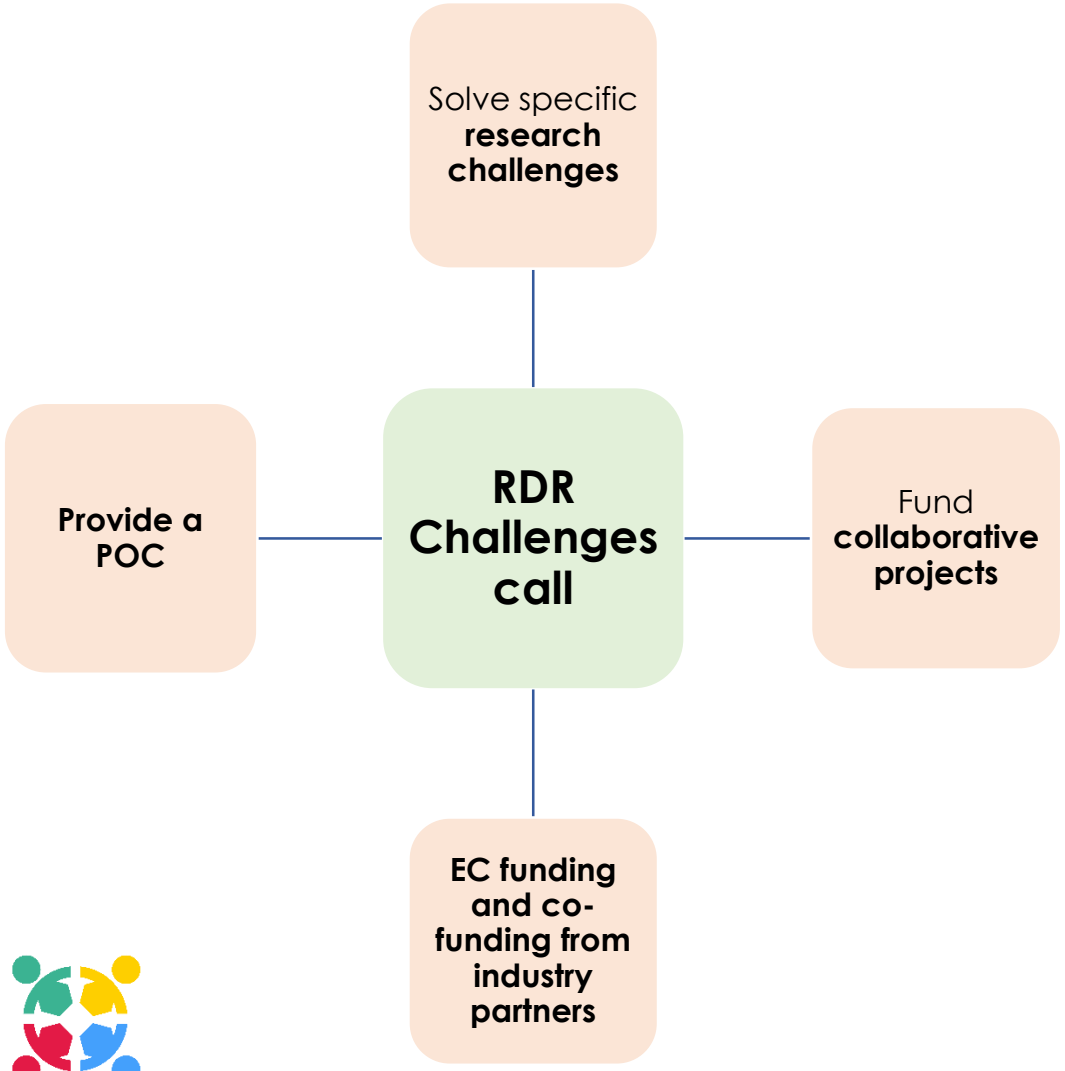
WP leader: French Foundation for Rare Diseases (FFRD)

- Alexandre Mejat – **AFM Téléthon**, France (participation in kind)
- Ralph Schuster – **DLR**, Germany
- Anton Ussi – **EATRIS**
- Virginie Bros-Facer – **EURORDIS** (Task Leader M1-M8)
- Christine Fetro – **FFRD**, France
- Diana Desir-Parseille – **FFRD**, France
- Sonja van Weely – **ZonMw**, The Netherlands

Agenda

1. *Call overview & milestones achieved*
2. *Challenges*
3. *Opportunities*

Call objectives



The **Rare Diseases Research (RDR) Challenges call** is an innovative call and a new funding scheme in the rare diseases environment. Its main objectives are to:

- ❑ Solve specific **research challenges**
- ❑ Facilitate and fund **collaborative projects** between industry, academia, Small and Medium-sized Enterprises (SMEs), Patients Advocacy Organisations (PAOs)
- ❑ Foster **public-private partnerships** combining **EC funding (1.5 Mio€) and co-funding from industry partners (0.5 Mio €)**
- ❑ **Provide a POC** for a **funding activity** that accelerates **translation**, involves **private** stakeholders and is **complementary to other** existing funding instruments like IMI

Call overview and achievement of milestones

1 **4 Challenges identified** as topics of the call *June 2019*

2 **Networking event** to initiate formation of collaborative consortia of applicants *3 March 2020*

3 **Opening of the call** *2 April 2020*
Projects solvable in 30 months = First phase of 18 months and second phase of 12 months

4 **Evaluation Committee meeting**
10 eligible projects
3 projects selected
No project selected for Challenge #3
October 2020

5 **Notification for funding** to applicants and industry sponsors *Feb 2021*

6 **Establishment of contracts and start of the projects** *Ongoing*

2019 2020 2021

EJP RD

3 projects selected for funding

Challenge	**Project title**	**Lead applicant**	N° of partners involved	N° of countries	Industry sponsors	**Total requested Budget**
#1Develop--ment of a non-invasive tool for measuring rare disease patient mobility in daily living	**Digital tools 4 Rare Diseases**	**SME; Netherlands**	5 (2 SME + 2 Academia + 1 PAO)	3 (Netherlands; France; UK)	Chiesi and CSL Behring	**551 446 €**
#2 Delivery system for intranasal administration of biological drugs to neonates	**Intranasal device for neonates**	**SME ; France**	3 (1 SME + 2 Academia)	2 (France; Belgium)	Chiesi	**485 166 €**
#4 Pre-clinical assay to detect instability of microsatellite repeat expansions	**Development and validation of a novel pre-clinical assay to detect triplet repeat expansions**	**Academia; Ireland**	3 (Academia)	2 (Ireland; UK)	Pfizer and Cydan	**486 719 €**

EJP RD

Call overview and achievement of milestones

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2019

2020

2021

EJP RD

Legal and contractual requirements

2 separate agreements. Project starts once funding agreement is signed

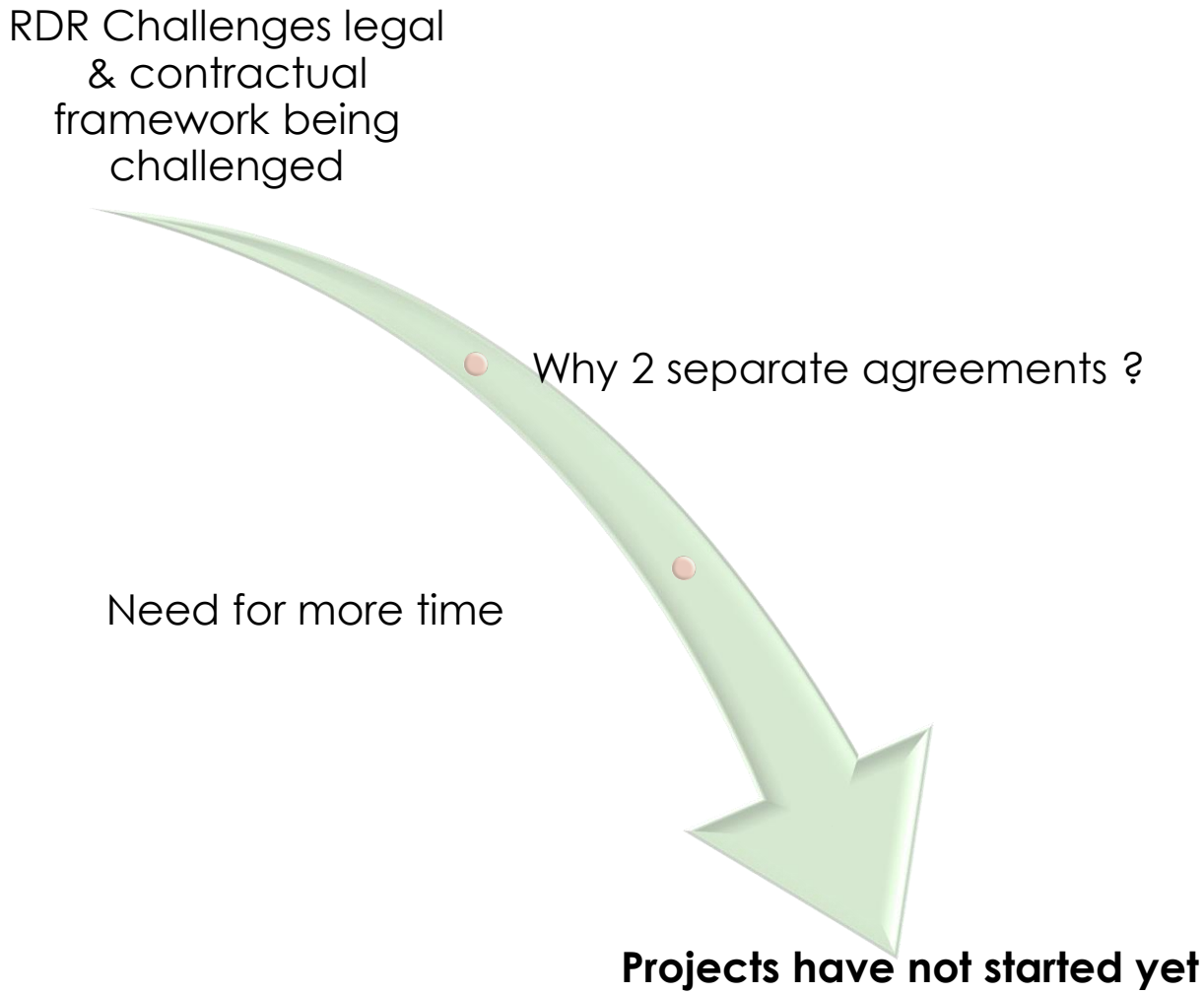
Funding agreement

- ✓Amount/calendar of the **funding**
- **First** to be signed before project starts
- 25% of first instalment
- **FFRD** involved

Consortium agreement

- **IP** issues
- Can be signed in the first 6 months
- 75% of first instalment
- FFRD not involved

As of today


RDR Challenges legal & contractual framework being challenged
Why 2 separate agreements ?
Need for more time
Projects have not started yet

General challenges encountered

From industry perspective

- Don't want to sign the **Funding Agreement** (FA) before consortium agreement(CA) since FA creates a financial commitment without knowing what the terms of CA will be
  - If FA first signed, need for an IP section in the FA « to be reassured »

- "The spirit of the consortium is to grant a **privileged access** - but not exclusive - to the results and IP use to the industry sponsors"

From consortium of applicants'perspective

- Funding Agreement is **not the right place** to discuss IP issues

- Lack of confidence in industry sponsors **accused of « wanting all IP »**
- Questioning from lead applicant and beneficiaries about the **possibility of carrying out the project without the support from industry** and about the possibility of approaching other pharma companies

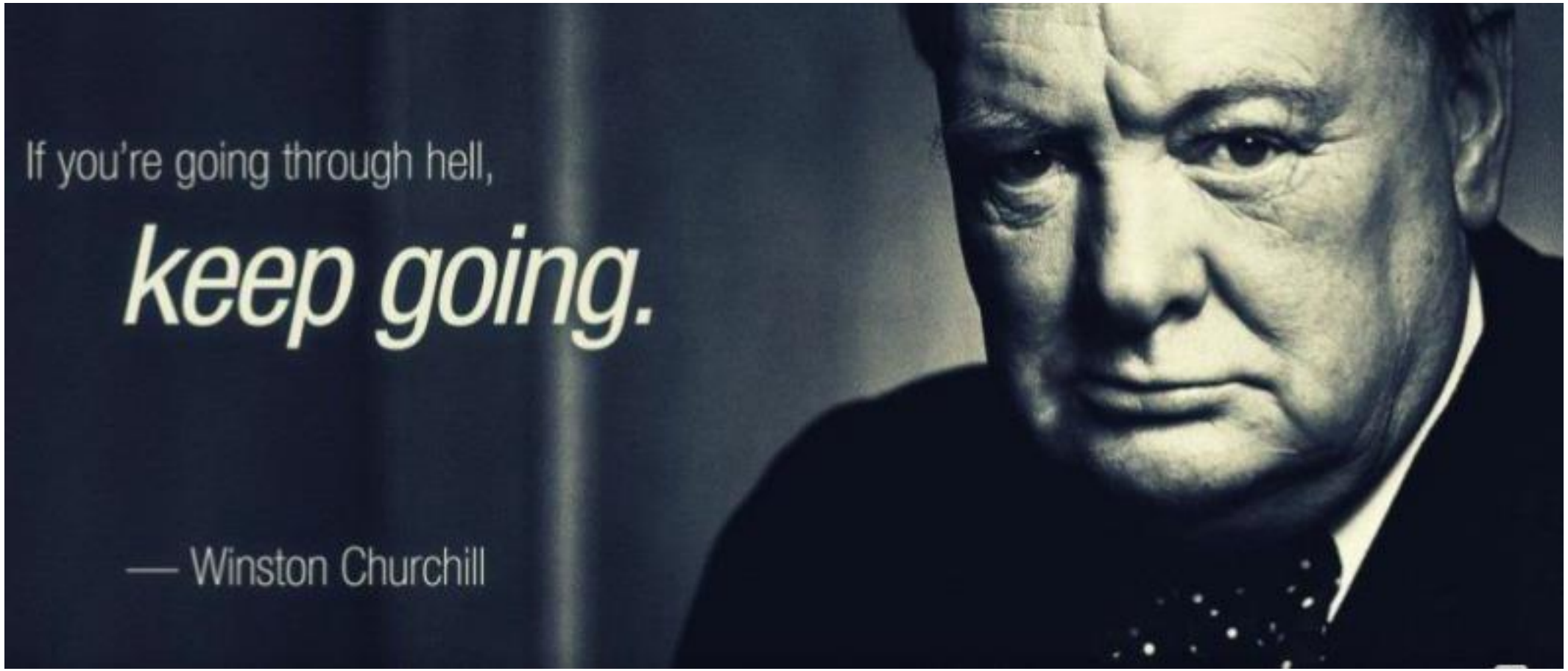
From ALL

- Role of industry sponsors not clear enough
- Legal review is time-consuming without any practical considerations

Main challenges per Challenge

Challenge #1	**- 1 SME with patent issue** / Ongoing IPR (IP mainly protected by proprietary knowledge/trade secret) **-** 20 people involved from 5 countries (2 industry sponsors; 2 SMEs; 1 PAO; 2 academics) with **fragmented availability & project knowledge**
Challenge #2	- First instalment postponed by industry sponsor at M6 → leading to a **non legitimate advance of 32 000 € that should be paid by the SME** (lead applicant)
Challenge #4	**-** 2 industry sponsors with 1 lead and 1 absent - Several changes in industry representatives **- Industry sponsors' withdrawal** from the project

What to do



Opportunities: Strengths & Points for improvement

- **Strengths**

- **Innovative** funding scheme in Rare Diseases field
- **Key milestone** in improving public-private partnerships in the pre-competitive space of therapy development
- **Proof of concept** for a sustainable model
- **Lessons to be learned**

- **Points for improvement**

- Clarification of **industry sponsors'role**
- Compromise found regarding the 2 **agreements** with a short IP section in the Funding Agreement to reassure
- Need for all stakeholders to be **accompanied during the negotiation phase**
- Need for **more time** to build a trusting relationship

EJP RD

Where do we stand today in the implementation of the projects ?

Challenge #1	- A recent meeting organised by FFRD has **succeeded in bringing together the 20 people** involved in the project and establishing a **trusting relationship.** - Contributions and expectations from each partner have been clearly explained enabling a better understanding of the project and of its interactions
Challenge #2	- **Progress** has been made - There is **growing consensus about IP issues**
Challenge #4	- Despite initial industry sponsors' withdrawal and all efforts of FFRD to reopen the door, academics are still so excited about the project that they are currently **approaching 2 other potential sponsors**

PROPOSALS ON HOW TO IMPROVE THE R&D ECOSYSTEM FOR BASIC RESEARCH AND COMPANY TAKE-UP OF DEVELOPMENT

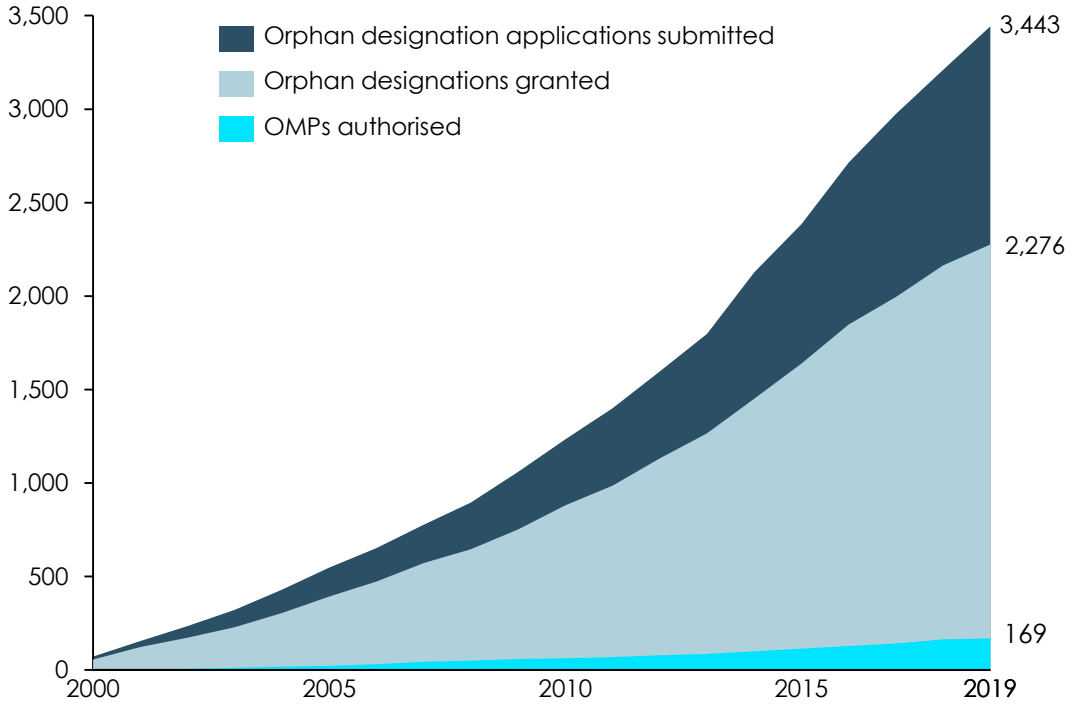
by the European Expert Group on Orphan Drug Incentives

Vittoria Carraro, Eucope and Maciej Gajewski, Alexion

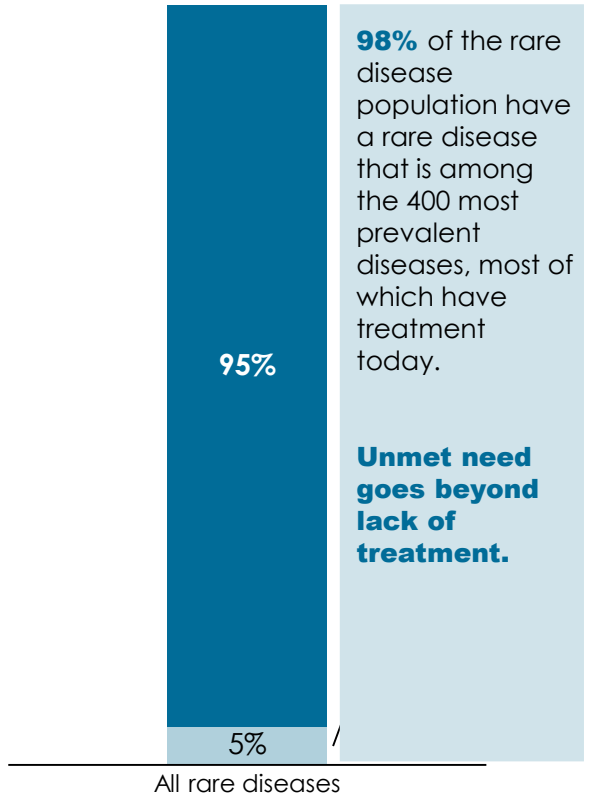
7 July 2021

A look back: the OMP Regulation has been a success but there is still unmet need

Applications submitted, designations granted and authorised OMPs since 2000
Cumulative



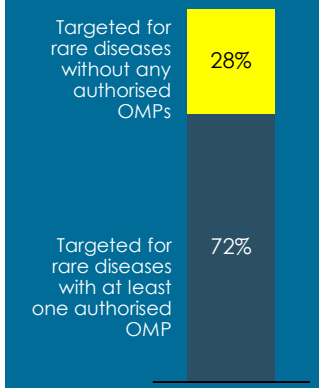
OMPs available for rare diseases
Share of all rare diseases



Source: European Commission (2020), European Medicines Agency (2020), Wakap et al. (2020)

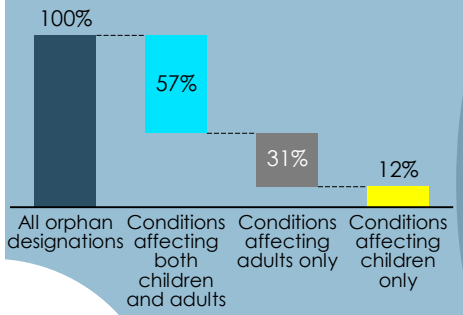
Which areas are underserved today?

1. Most OMP development focuses on disease areas where treatments already exist[1]



72% of authorised treatments between 2000-2017 targeted diseases that already had **at least one authorised treatment available**.

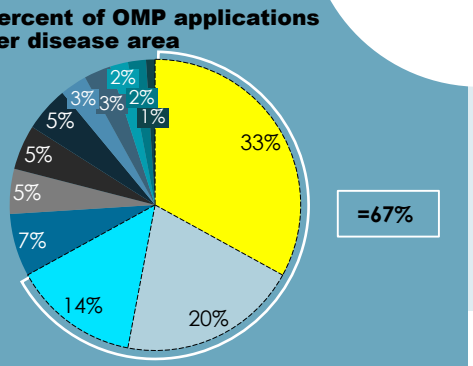
2. OMP development is not equally focused on adults and children[2]



Only **12%** of orphan designations between 2000-2019 related to conditions that **only affect children**, while 31% related to conditions that affect only adults.

Limitations in the current OMP development landscape

Between 2000-2019, **67%** of OMP designation applications targeted the same **three disease areas.**

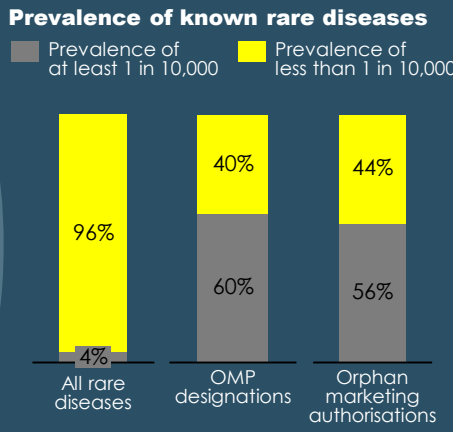


=67%

3. OMP development benefits only a limited number of diseases[3]

60% of orphan designations and **56%** of authorised OMPs were targeted at rare diseases with a **prevalence greater than 1 in 10,000.**

96% of rare diseases have a point prevalence of **less than 1 in 10,000.**



4. OMP development concentrates on the "least rare" diseases[4]

Notes: 1) European Commission (2020), p. 40; based on authorisations between 2000 and 2017 // 2) European Medicines Agency (2019), p. 6; based on orphan designations between 2000 and 2019 // 3) European Medicines Agency (2019), p. 5 // 4) European Medicines Agency (2019), p. 13 and 14, and Wakap et al. (2019).

Four guiding principles for the revision of the OMP policy framework

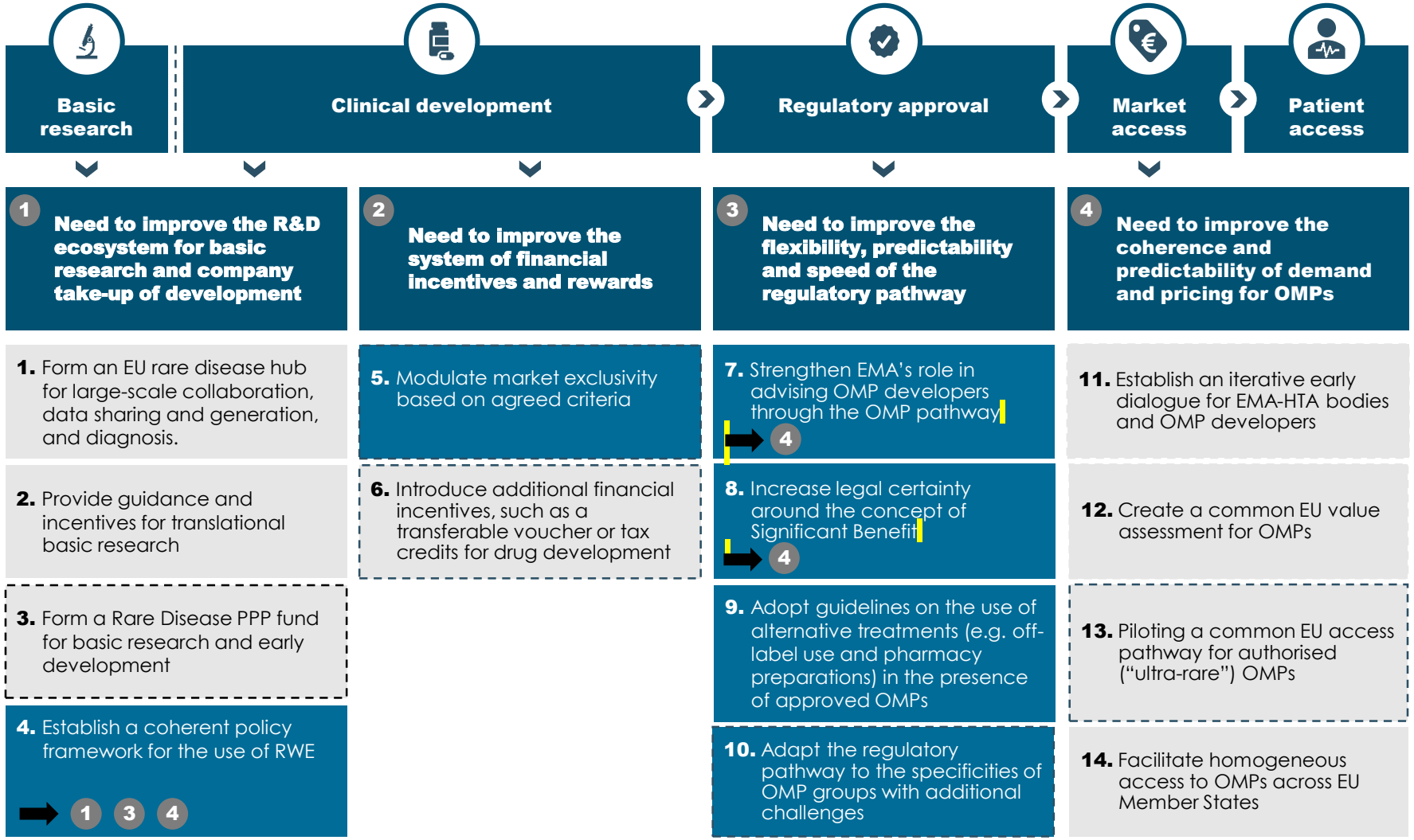
a **Conceive a holistic policy framework for the OMP development path**

b **Lead the revision from a multi-stakeholder perspective**

c **Think about policy changes from an investment perspective**

d **Ensure a competitive EU policy framework**

4 needs and 14 policy proposals



These proposals can be addressed in the revision of the OMP Regulation

Need 1: Improving the R&D ecosystem for basic research and company take-up of development

1. Form an EU rare disease hub for large scale collaboration, sharing and generation of data and diagnosis

Bring together all actors involved in and data on rare disease onto one common platform.

EJP RD, ERNs, RD Connect, EJP Virtual platform, EU RD platform

2. Provide guidance and incentives for translation of basic research

Establish guidelines for development-ready research and appropriate incentives for basic researchers.

Orphan Drug Development Guide of the IRDiRC

Need 1: Improving the R&D ecosystem for basic research and company take-up of development

3. Basic research PPP fund for rare diseases

A singular financial entity, generating (i) more funding and (ii) more conditional funding towards rare disease research.

4. Coherent policy framework for RWE

Standardisation and better access to RWE, and better use of it at different stages.

RWE4DECISIONS
RARE-IMPACT

Full report available here

NCATS

COLLABORATE. INNOVATE. ACCELERATE.

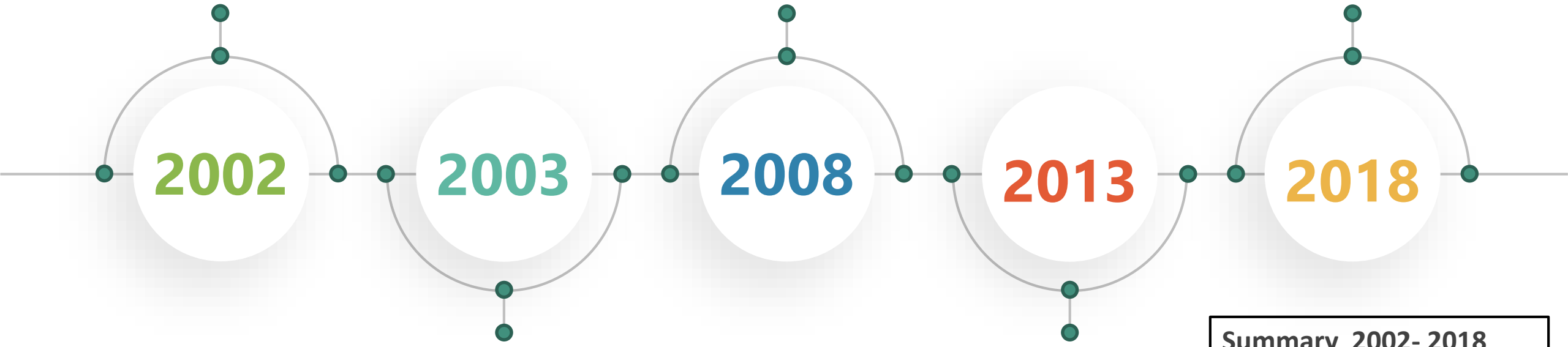
RARE DISEASES CLINICAL RESEARCH NETWORK

Program Director, NIH,
NCATS, ORDR

Tiina K. Urv, Ph.D.

National Center for Advancing Translational Sciences

Rare Diseases Clinical Research Network Timeline



Rare Diseases Act of 2002 Establishes RDCRN

First 7 Consortia Funded.

Program Expanded
19 consortia funded.

Continued Growth
22 consortia funded

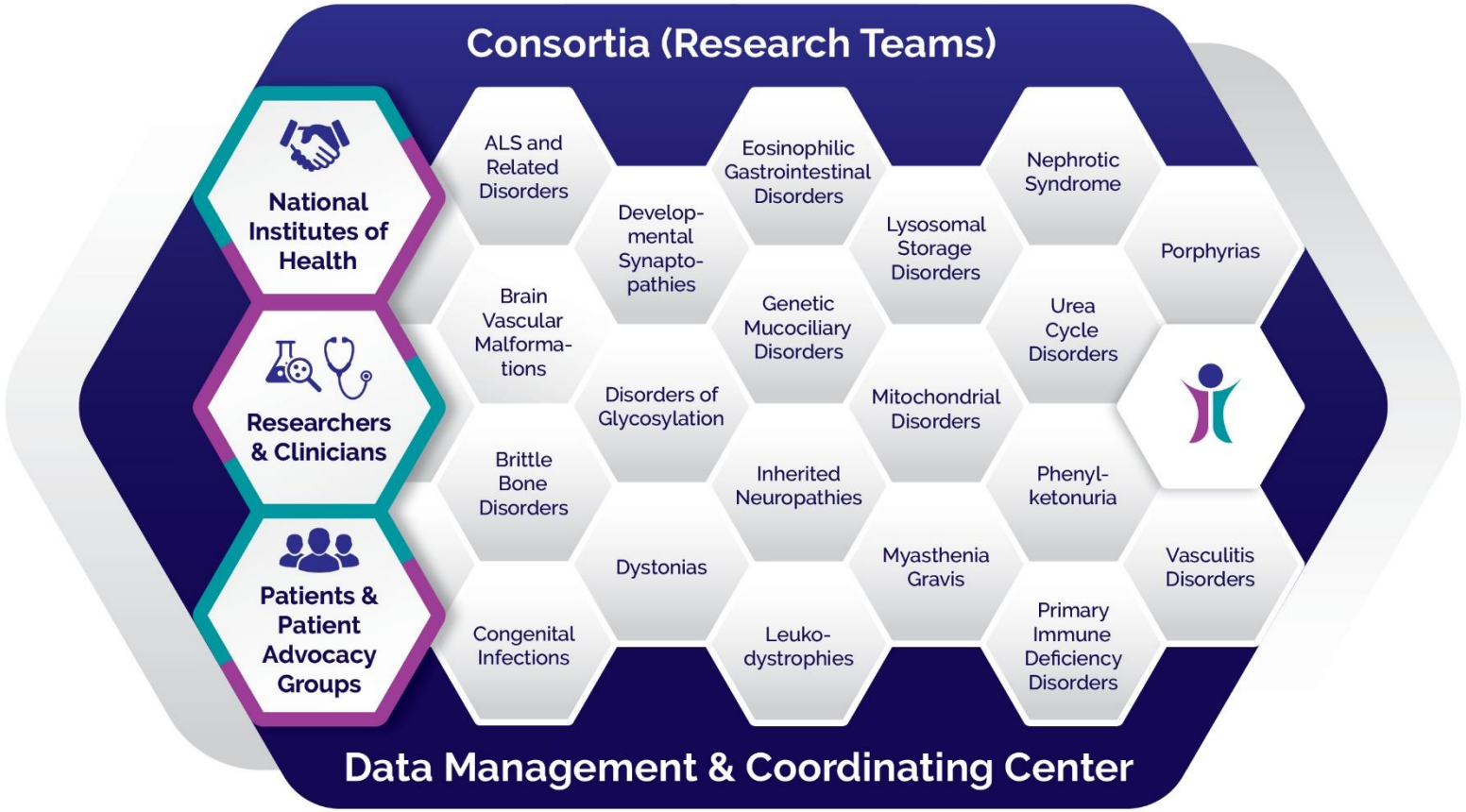
Program Refresh

Summary 2002- 2018

- **31 individual consortia**
- **238 disorders**
- **>40,000 participants**

RARE DISEASES
CLINICAL RESEARCH NETWORK

A network of 20 research teams collaborating to achieve faster diagnosis and better treatments for patients with rare diseases



- 20 Consortia
- 200+ Rare Diseases & **140+ Patient Advocacy Groups**

National Center for Advancing Translational Sciences | National Institute of Neurological Disorders and Stroke | National Institute of Allergy and Infectious Diseases | National Institute of Diabetes and Digestive and Kidney Diseases | *Eunice Kennedy Shriver* National Institute of Child Health and Human Development | National Institute of Arthritis and Musculoskeletal and Skin Diseases | National Heart, Lung, and Blood Institute | National Institute of Dental and Craniofacial Research | National Institute of Mental Health | Office of Dietary Supplements

One current estimate - 10-15 years to get drug to market

Current approach not sustainable

TREATMENTS FOR PATIENTS

It can cost > $2.6 billion to develop a drug from initial discovery to completion

<12% Approval Rate for drugs entering development

Sources:

- Pharmaceutical Research and Manufacturers of America, *Drug Discovery and Development: Understanding the R&D Process*, www.innovation.org
- DiMasi, JA and Grabowski, HG (2007), The Cost of Biopharmaceutical R&D: Is Biotech Different?, *Managerial and Decision Economics 28* : 469-479

- Sullivan T. March 21, 2019. https://www.policymed.com/2014/12/a-tough-road-cost-to-develop-one-new-drug-is-26-billion-approval-rate-for-drugs-entering-clinical-de.html and *Arrowsmith and Miller, Nat Rev Drug Disc 12: 569 (2013)*


FASTER
SUSTAINABLE
TREATMENTS FOR PATIENTS
CHEAPER
HIGH QUALITY

FASTER

Strategies

Networks Established

- Clinical Research
- Patient Advocacy

Natural History Studies

Tools Established

- Outcome Measures
- Biomarkers
- Common Data Elements

RISK

Clinical Trial Readiness

WHY

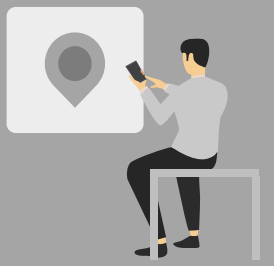
Why is this the best way to go forward?
Show me the data!

WHAT

What is the best potential treatment?

What is the desired outcome

WHERE



Where are the experts?
Where are the patients?

HOW

How should the trial be conducted?

WHO

Who do you treat?

Who will conduct the trial?

WHEN

When is the best time to treat a condition?

Do we have everything in place?

Strategies

Economies of Scale

- Shared work environment
- Shared tools

Innovative Models for Trials

- Basket trials
- Umbrella trials

CHEAPER

The RDCRN Tool Garden – hosted by DMCC

	usage
• REDCap (Vanderbilt)	290+
• Biospecimen shipment tracking system (custom)	9 protocols
• SAS Studio (licensed)	<10 (DMCC)
• Pedigree Drawing Tool (open source)	39
• Public facing web sites for RDCRN and consortia (Drupal and DNN)	
• Moodle classroom training system (open source)	560+
• Grants management software (Northwestern CTSA – coming soon)	
• NIH Toolbox support (coming soon)	
• JupyterHub with RStudio, python etc. (coming soon)	

RARE DISEASES CLINICAL RESEARCH NETWORK

Data Management and Coordinating Center
at Cincinnati Children's Hospital Medical Center

The RDCRN Tool Garden – 3rd party

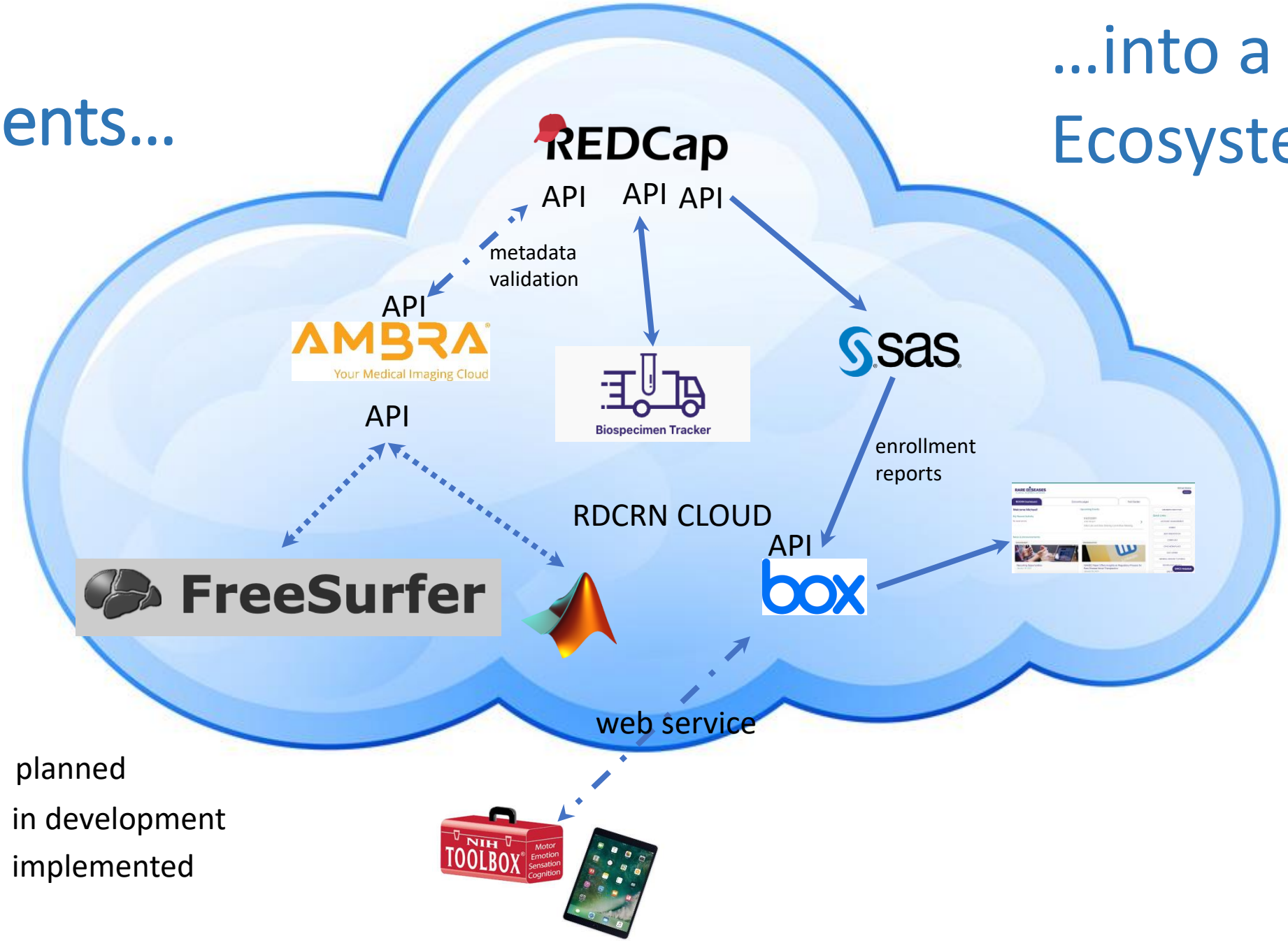
	usage
• Box (secure document management and data sharing)	590+
• Ambra (DICOM image management)	(new)
• Complion (e-regulatory binder system)	770+
• JIRA / Confluence (service desk / bug tracking and documentation system)	2600 tickets
• Slack (communication app)	
• Facebook Workplace (for RDCRN-affiliated patient advocacy groups)	240+
• Twilio Text Messaging (integrated with REDCap)	
• Coming soon: cloud-based genomics data management and processing platform.	

RARE DISEASES CLINICAL RESEARCH NETWORK

Data Management and Coordinating Center
at Cincinnati Children's Hospital Medical Center

Turning Components…

…into a Data Ecosystem



Strategies

Data Standards

- FAIR Principles
- Good data practices

Research

- Scientific Rigor
- Reproducibility
- Transparency

RDCRN Data Standards

Mission: To share rare-disease data across the research community, we will define data standards to improve data quality, usability, and interoperability within and across consortia using FAIR principles (findable, accessible, interoperable, reusable).

Data types:

- Procedures: imaging, genomics, activity monitoring, pharmacokinetic, etc.
- Patient Reported Outcomes & Clinical Outcomes Assessments: Neurodevelopmental testing outcomes, etc.
- Demographics, clinical labs, medical history, adverse events, etc.

RDCRN Data Standards (cont)

Implementing CDISC/CDASH standards where available
Using RedCap Modules for PROs
Identifying standards that facilitates integration of EHR data

Anticipating where the puck is going relative to integrating EHR data into clinical research data bases

Thank you

urvtiin@mail.nih.gov

C-Path: Advancing Innovation in Regulatory Science through Public-Private Partnerships

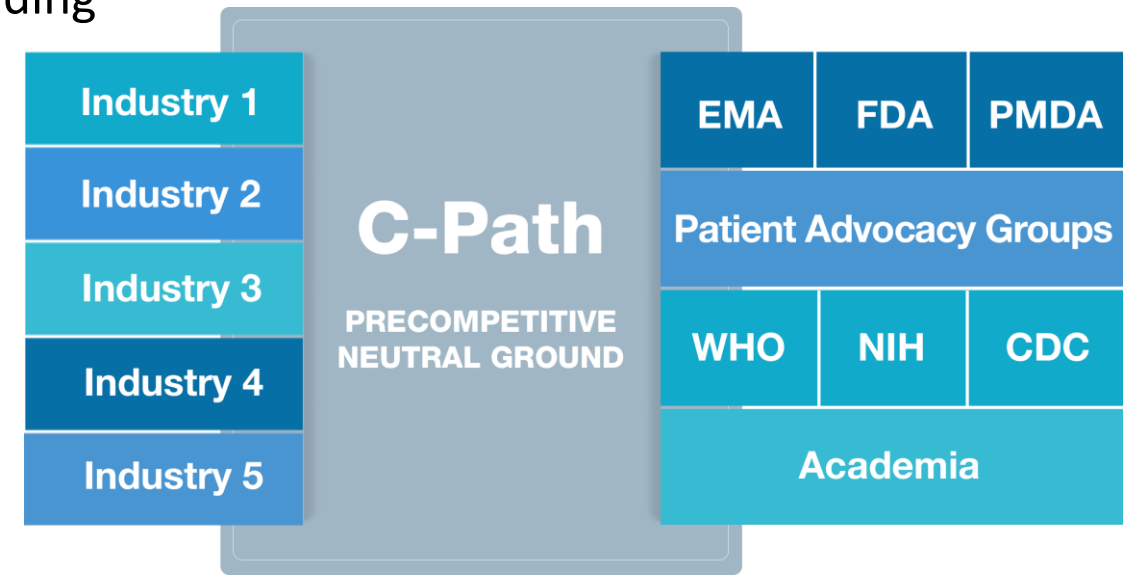
EJPRD 07-07-2021

CRITICAL PATH INSTITUTE

How C-Path Works

CRITICAL PATH INSTITUTE

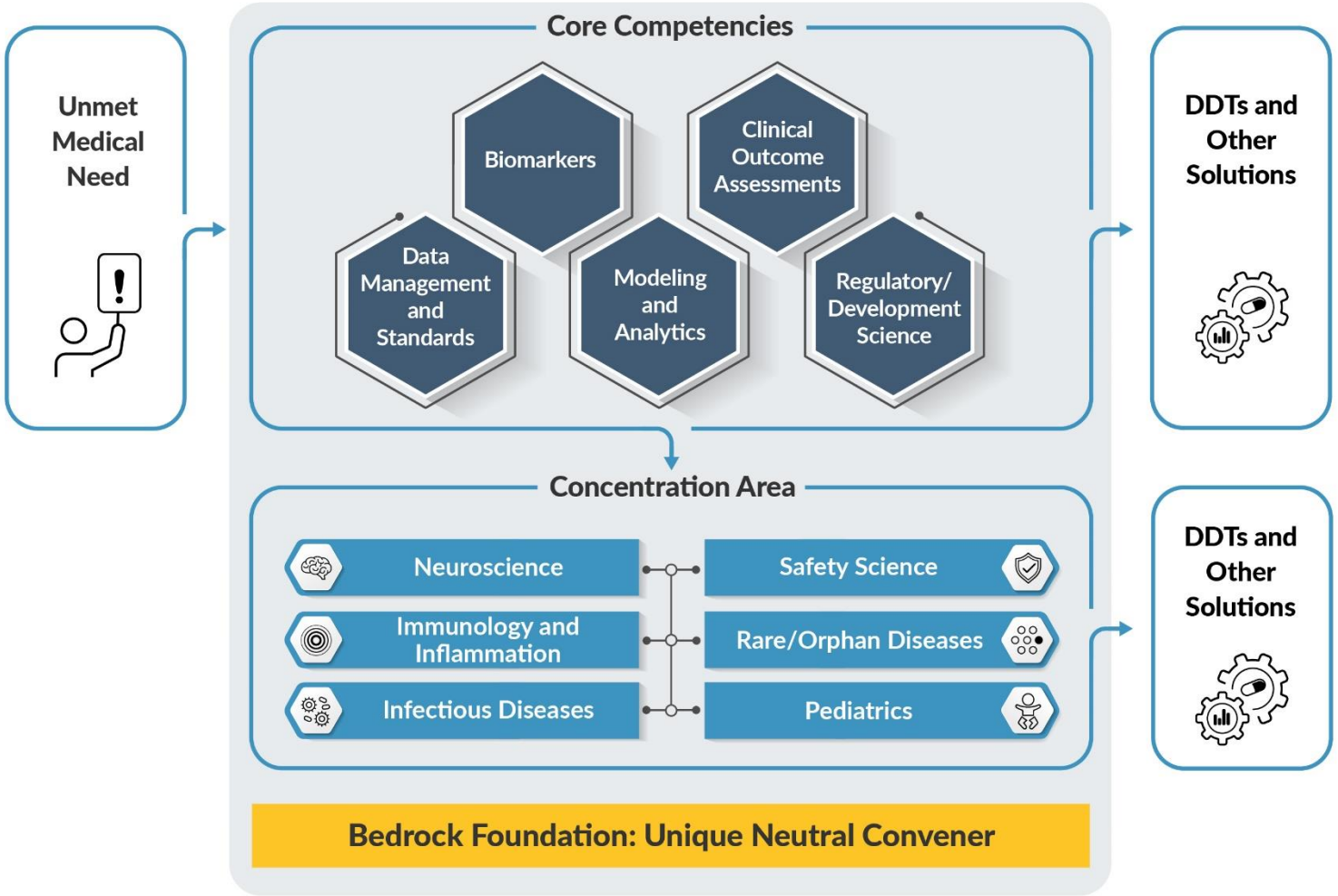
- Acts as a trusted, neutral third party
- Public-Private Partnerships
- Convenes scientific consortia of industry, academia and government for sharing of data and expertise
  - ✓ The best science
  - ✓ The broadest experience
  - ✓ Active consensus building
  - ✓ Shared risk and costs
- Enable iterative FDA/EMA/PMDA participation in developing new methods to assess the safety and efficacy of medical products



Official regulatory endorsement of novel methodologies and drug development tools

C-Path has built scale and expertise along key concentration areas and core competencies

CRITICAL PATH INSTITUTE



A Success Story – Regulatory Firsts

CRITICAL PATH INSTITUTE

C-PATH REGULATORY SUCCESSES

ALZHEIMER'S DISEASE
- FDA & EMA endorsed AD clinical trial simulation tool
- EMA qualified model-based AD biomarker
- FDA & EMA letters of support
  - Model-based AD biomarkers and pre-dementia clinical trial simulator

MULTIPLE SCLEROSIS
- EMA qualified PerfO measure
  - Test battery for all forms of MS

POLYCYSTIC KIDNEY DISEASE
- EMA & FDA model-based qualified Total Kidney Volume (TKV) imaging biomarker
- FDA letter of support
  - TKV imaging biomarker
- FDA designated reasonably likely surrogate marker for PKD trials (TKV)

PREDICTIVE SAFETY TESTING
- EMA, FDA & PMDA qualified non-clinical kidney safety biomarkers
- FDA qualified clinical kidney safety markers
- Six FDA & EMA letters of support

PARKINSON'S DISEASE
- FDA letter of support
  - PD imaging biomarker
- EMA qualified model-based PD imaging biomarker

TUBERCULOSIS
- EMA qualified translational drug development platform

PATIENT-REPORTED OUTCOME MEASURES
- FDA COA qualification
- Symptoms of Major Depressive Disorder Scale
- Non-Small Cell Lung Cancer Symptom Assessment Questionnaire
- Asthma daytime and nighttime symptom diaries

TYPE 1 DIABETES
- EMA letter of support for model-based islet autoantibodies biomarker for trial enrichment

FDA
- 6 Qualification Decisions
- 1 Fit-for-Purpose Endorsement
- 7 Letters of Support

EMA
- 7 Qualification Decisions
- 7 Letters of Support

PMDA
- 1 Qualification Decision

C-Path Current Consortia and Programs

Active Consortia/Programs

BMDR	Biomarker Data Repository	HD-RSC	Huntington's Disease Regulatory Science Consortium	T1D	Type 1 Diabetes Consortium
CDRC	Cure Drug Repurposing Collaboratory	INC	International Neonatal Consortium	TB-PACTS	TB-Platform for Aggregation of Clinical TB Studies
CPAD	Critical Path for Alzheimer's Disease	MSOAC	Multiple Sclerosis Outcome Assessment Consortium	TOMI-T1D	Trial Outcome Markers Initiative in T1D Consortium
CPP	Critical Path for Parkinson's Dis	PKDOC	Polycystic Kidney Disease Outcomes Consortium	TRxA	Translational Therapeutics Accelerator
CPTA	Critical Path to Therapeutics for the Ataxias	PREDICTOX KE	PredicTox Knowledge Environment	TTC	Transplant Therapeutics Consortium
CPTR	Critical Path to TB Drug Regimens	PRO CONSORTIUM	Patient-Reported Outcome Consortium		
CP-SCD	Critical Path for Sickle Cell Disease	PSTC	Predictive Safety Testing Consortium		
DCC	Data Collaboration Center	QUANTMED	Quantitative Medicine		
D-RSC	Duchenne Regulatory Science Consortium	RDCA-DAP	Rare Disease Cures Accelerator- Data and Analytics Platform		
EPRO CONSORTIUM	Electronic Patient-Reported Outcome Consortium	RD-COAC	Rare Disease Clinical Outcome Assessment Consortium		

Data acquisition strategy

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Patient-report registries

- IAMRARE
- Genetic Alliance
- Pulse Infoframe
- Invitae
- TREAT-NMD
- RARE-X

Clinician-report registries

- RD-CRNs
- European reference networks
- MDA-MOVR
- Neurobank
- TREAT-NMD

Clinical Trials

- Vivli
- Individual companies
- Referred by NORD corporate council
- Internal inventory from consortia or FDA priority list
- Outreach through individual patient groups/ companies

EHRs

RDCA-DAP

Intersection of Rare Disease Consortia, Workgroups and RDCA-DAP

NORD/FDA/C-Path recommended issues

RDCA-DAP run discussions on common regulatory issues in rare disease drug development; identification of routes to solutions

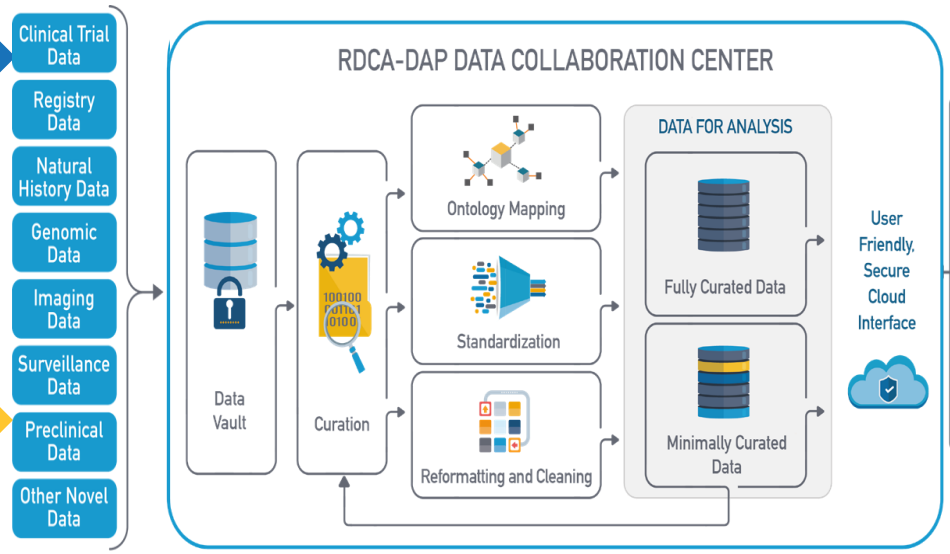
RDCA-DAP workgroups or external users to develop specific tools

C-Path consortia to develop solutions to more complex issues /multiple issues

RDCA-DAP: Develop data inventories, outreach, DCA

Consortia: Frame research questions, define solutions

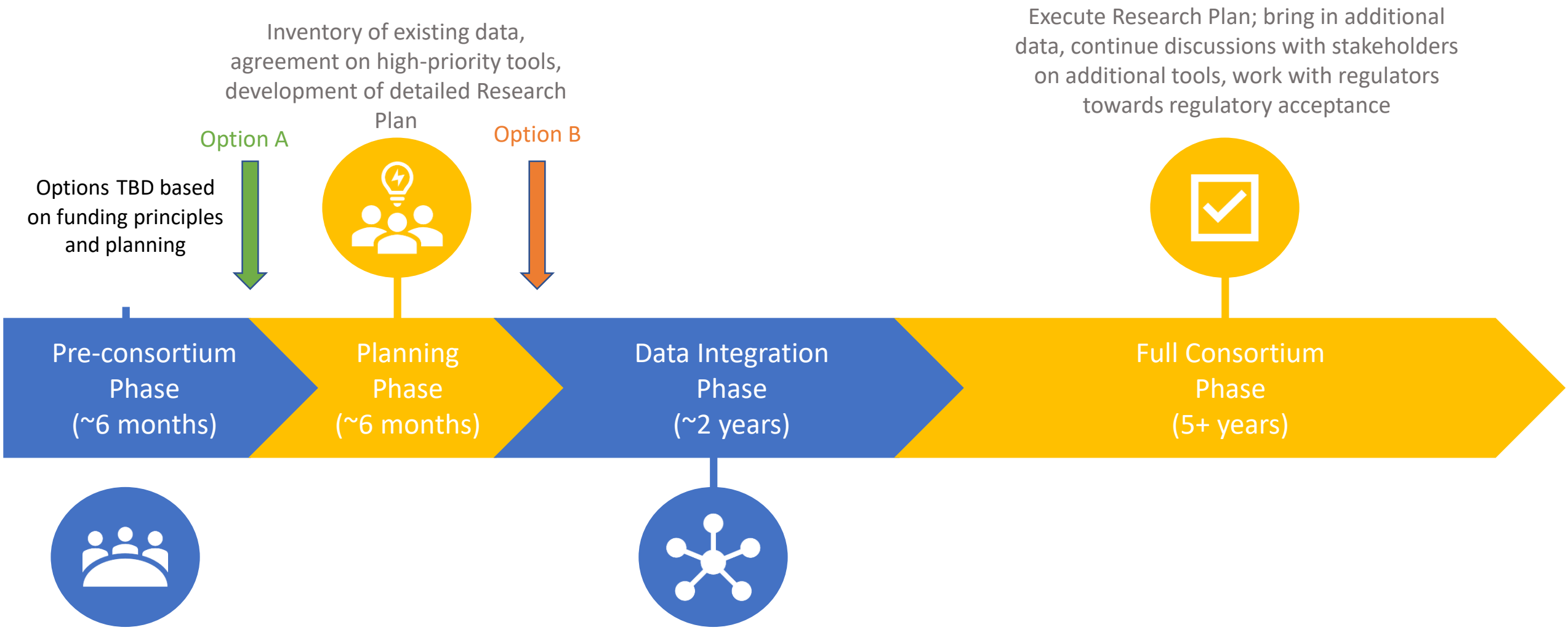
Develop data inventories, outreach, DCA



Internal and external use of data to support regulatory acceptance and other uses

Access data, analyze, steps to regulatory qualification/ endorsement or other acceptance

PPP – One size doesn't fit all



*Phases can be overlapping, *timeline will be very dependent on what tools are desired, the state of the data, the number of work products and the budget available*

Thank You

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