European Joint Programme on Rare Diseases

Executive Committee Annual Meeting 2022

EUROPEAN JOINT PROGRAMME
RARE DISEASES

ExCom (annual) Meeting
05/07/2022 _ Online

Sustainability

Roadmap for EJP RD

WP3

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RARE DISEASES

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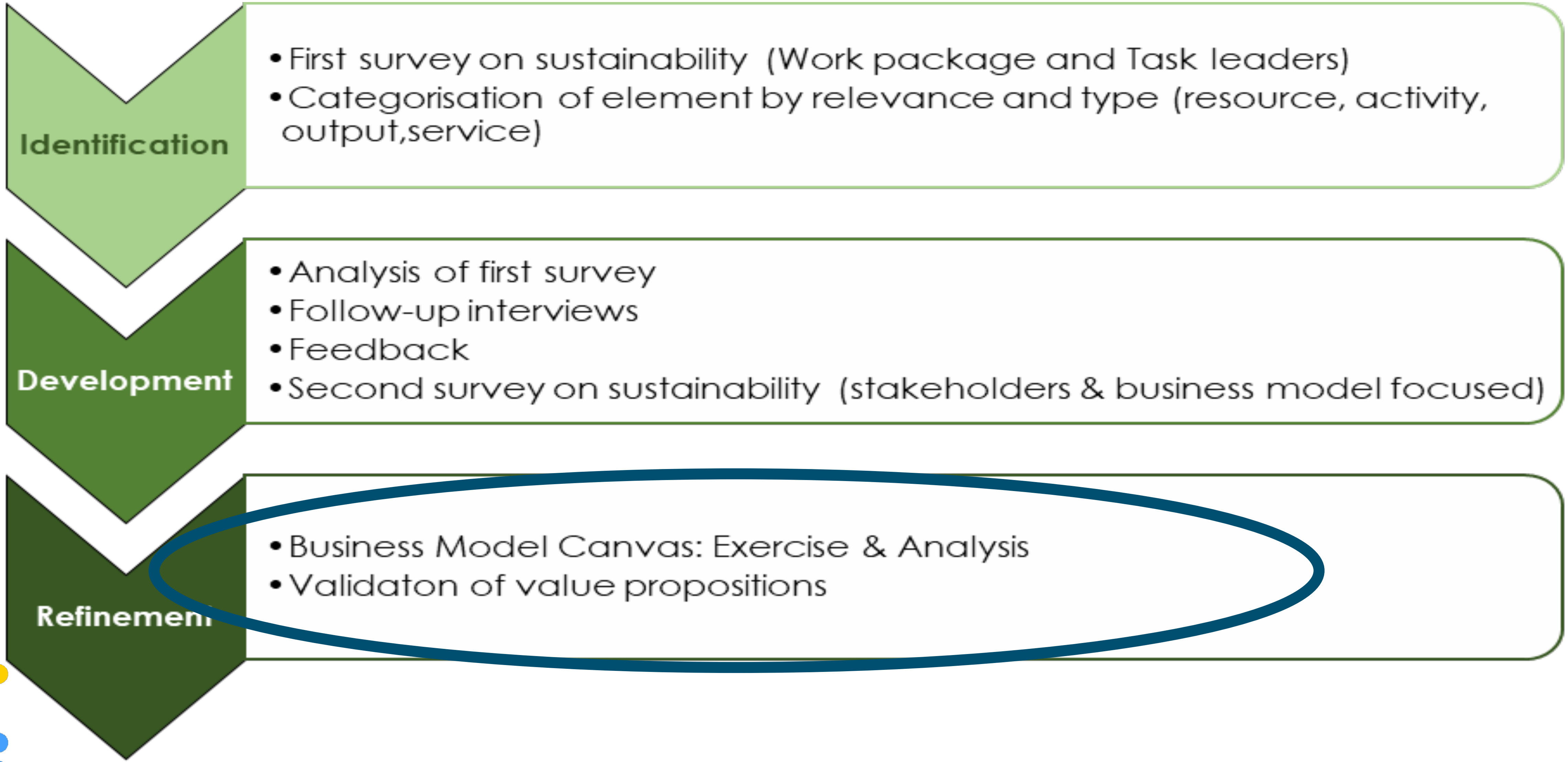
Processe summary



Identification

- First survey on sustainability (Work package and Task leaders)
- Categorisation of element by relevance and type (resource, activity, output,service)

Development

- Analysis of first survey
- Follow-up interviews
- Feedback
- Second survey on sustainability (stakeholders & business model focused)

Refinement

- Business Model Canvas: Exercise & Analysis
- Validaton of value propositions

EJP RD catalogue

PILLAR	ELEMENT/ASSET	TYPE	VALUE-CHARACTERISATION (SUMMARISED)
0	**Coordination services EJP RD & IRDiRC SciSec**	Service, Resource	Proactive, adaptable and efficient planning, execution, monitoring and issue solving to optimise quality results, fostering links with other initiatives for efficiency and synergistic actions.
0	**Central Helpdesk**	Service, Resource	Provision of centralised easy access to RD expertise, with request processes and tailored services for users, outreaching through dissemination strategies to increase visibility of EJP RD services.
0	**Sustainability Handbook**	Output	The general guidance on sustainability key factors helps the RD Community and other areas to plan and operate in a sustainable manner.
0	**Advisory Regulatory Ethics Board (AREB ELSI Services)**	Service, Resource	Reference single contact point, easily identified, for ELSI questions, with the provision of quality and time-efficient ethics review of RD research proposals, accelerating their implementation.
0	**Re(ACT) Congress and IRDiRC Conference**	Activity	Promotion of scientific cooperation and research on rare and orphan diseases in a unique Face-to-Face congress that fosters RD Research and Policy independently on medical domain.

EJP RD catalogue

PILLAR	ELEMENT/ASSET	TYPE	VALUE-CHARACTERISATION (SUMMARISED)
1	**Monitoring**	Service	Tool that permits the centralisation and analysis of monitored funded projects, reinforced by feedback from experts. Annual collection of information from projects and analysis of their progress.
1	**Networking Support Scheme**	Activity	Organisation of networking events that involve RD research's stakeholders, key for RD research to advance. Encouragement of underrepresented stakeholders and countries' involvement.
1	**Research Challenges Scheme**	Activity	Innovative funding scheme involving consortia of applicants (Academia, SME, and PAOs) Advocacy Organizations) and industry sponsors. Public-private partnerships facilitator.

EJP RD catalogue

PILLAR	ELEMENT/ASSET	TYPE	VALUE-CHARACTERISATION (SUMMARISED)
3	**Orphanet training material & module**	Activity	Empowerment of Orphanet Network members at national level, to deliver local training of the nomenclature and ontology. Training for trainers' sessions and trainer's toolkit.
3	**Training quality assurance Next Generation Sequencing (NGS)**	Activity	Large scope on NGS diagnostics, with yearly updates, helping in the translation of research tools to diagnostic applications. Up to date trainings with at international level with expert trainers.
3	**Registries and undiagnosed courses**	Activity, Resource	Opportunity to meet and create networking among professionals involved in RD registries, undiagnosed rare conditions and FAIRification, promoting further interaction & collaborations
3	**Trainings on biobanks and samples**	Activity, Output	Favouring communication and knowledge exchange about RD research biobanking and related operating procedures to deliver training, increasing visibility and sharing of preparatory material.
3	**Program delivery for the 3 schools (EURORDIS Academy)**	Activity	Empowerment of rare disease patient advocates with knowledge and skills to take part in patient engagement roles side-by-side with researchers, regulators, and industry and policy makers.
3	**Workshops/training contents for paediatric patients**	Activity, Output	Facilitator of the involvement of paediatric patients in ethically sound rare diseases research. The workshops aim to provide young rare disease patients with engagement skills in RD research.
3	**Online academic course (MOOC)**	Activity, Output	Cover the lack of transversal training on RD, by delivering free, easily accessed, and ergonomic MOOCs on transversal rare diseases research topics, with scalable content, to a wide audience.
3	**ERN Workshops (training multiplicators)**	Activity, Output	Sharing of knowledge and methodologies applicable for several different RD fields, highly relevant for ERNs, at the European level, with expectative of accreditation.
3	**ERN Workshops material**	Output	ERNs Workshops (Training multiplicators) material. This training material comprehend research concepts and topics that are cutting edge and of cross-ERN benefit.
3	**Secondments (Mobility fellowships)**	Activity, Output	The fellows have the possibility to obtain a highly specific and tailored training, acquiring new research experiences and learning new methodologies, through exchanges between institutions.

EJP RD

EJP RD catalogue

PILLAR	ELEMENT/ASSET	TYPE	VALUE-CHARACTERISATION (SUMMARISED)
4	**Innovation Management Toolbox**	Service, Resource	Centralised repository of resources that provides searchability and answers to specific questions. It may contain outputs as the Sustainability Handbook.
4	**Mentoring Service**	Service	Easy access to full gamut of therapy development expertise that increases the success rates in translational research projects of individual researchers by stimulating interdisciplinarity and building capacities.
4	**Clinical Trials Support Office**	Service	Support/advice for the planning and design of clinical studies for Rare Diseases (RD), with special focus in multinational research. Built up on the expertise of partners with years of experience in their respective fields.

EJP RD catalogue*

PILLAR	ELEMENT/ASSET	TYPE	VALUE-CHARACTERISATION (SUMMARISED)
2	**Virtual Platform assets**	Service, Resource Activity, Output	Federated platform of resources for RD research that facilitates the discovery, access and analysis of data and samples scattered across several resources in Europe. It represents the central value of the research ecosystem.

**Under construction for P2*

VIRTUAL PLATFORM

STEPS

01

MATURITY
CONFIRM ASSETS x
MINIMAL VIABLE PRODUCT

↓

CATALOG OF PRODUCTS
WITH VALUE PROPOSITIONS

02

SET OF QUESTIONS FOR THE ANNUAL RETREAT (besides step 1)

- ☐ Operating model vs excelled model
- ☐ Components that could:
    - Be standalone vs Interdep*
    - Reusable/ Reusable for other purposes
    - Interchangeable with other platforms/ projects (distributed efforts)
    - Stakeholders (bundles)

03

ROUNDS OF
BUSINESS MODEL CANVAS

ALREADY STARTED

EXPECTED BEFORE
SUMMER

04

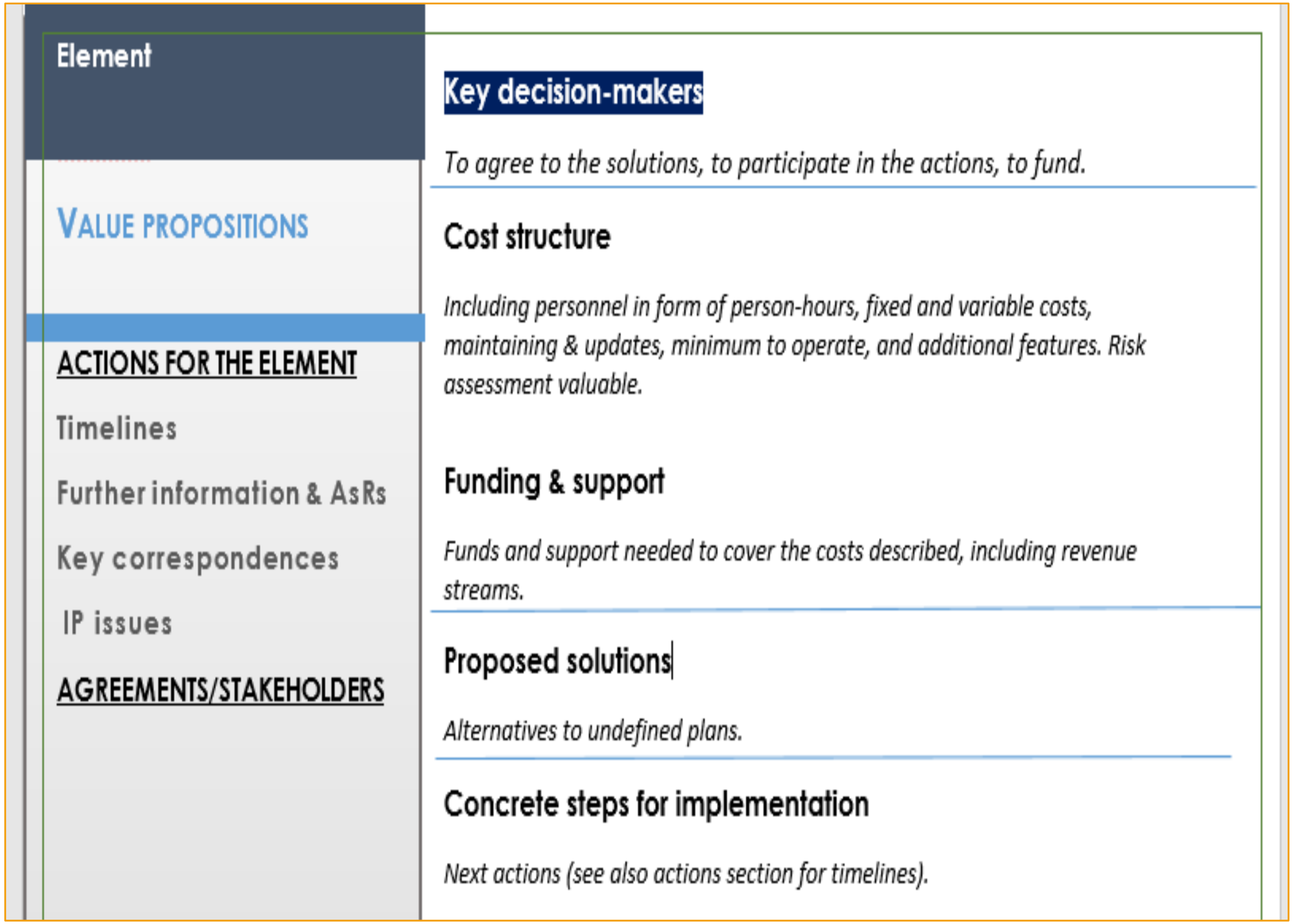
INFORMATION-SERVICE
PROVIDER

SUSTAINABILITY PLANS
PROPOSALS/ALTERNATIVES

STAKEHOLDERS APPROACH

Individualised feedback

- Will start a round of individualised feedback*
  - Revisit value propositions if needed
  - Costs structures when needed
  - Funding alternatives and stakeholders approaches



Element

VALUE PROPOSITIONS

ACTIONS FOR THE ELEMENT

Timelines

Further information & AsRs

Key correspondences

IP issues

AGREEMENTS/STAKEHOLDERS

Key decision-makers

To agree to the solutions, to participate in the actions, to fund.

Cost structure

Including personnel in form of person-hours, fixed and variable costs, maintaining & updates, minimum to operate, and additional features. Risk assessment valuable.

Funding & support

Funds and support needed to cover the costs described, including revenue streams.

Proposed solutions

Alternatives to undefined plans.

Concrete steps for implementation

Next actions (see also actions section for timelines).

This feedback may be done in collaboration with the External Service Provider

Services Roadmap

- Elements that:
  - have considerations as services (Virtual Platform assets excluded for now)
  - May share channels, communication, ítems and resources

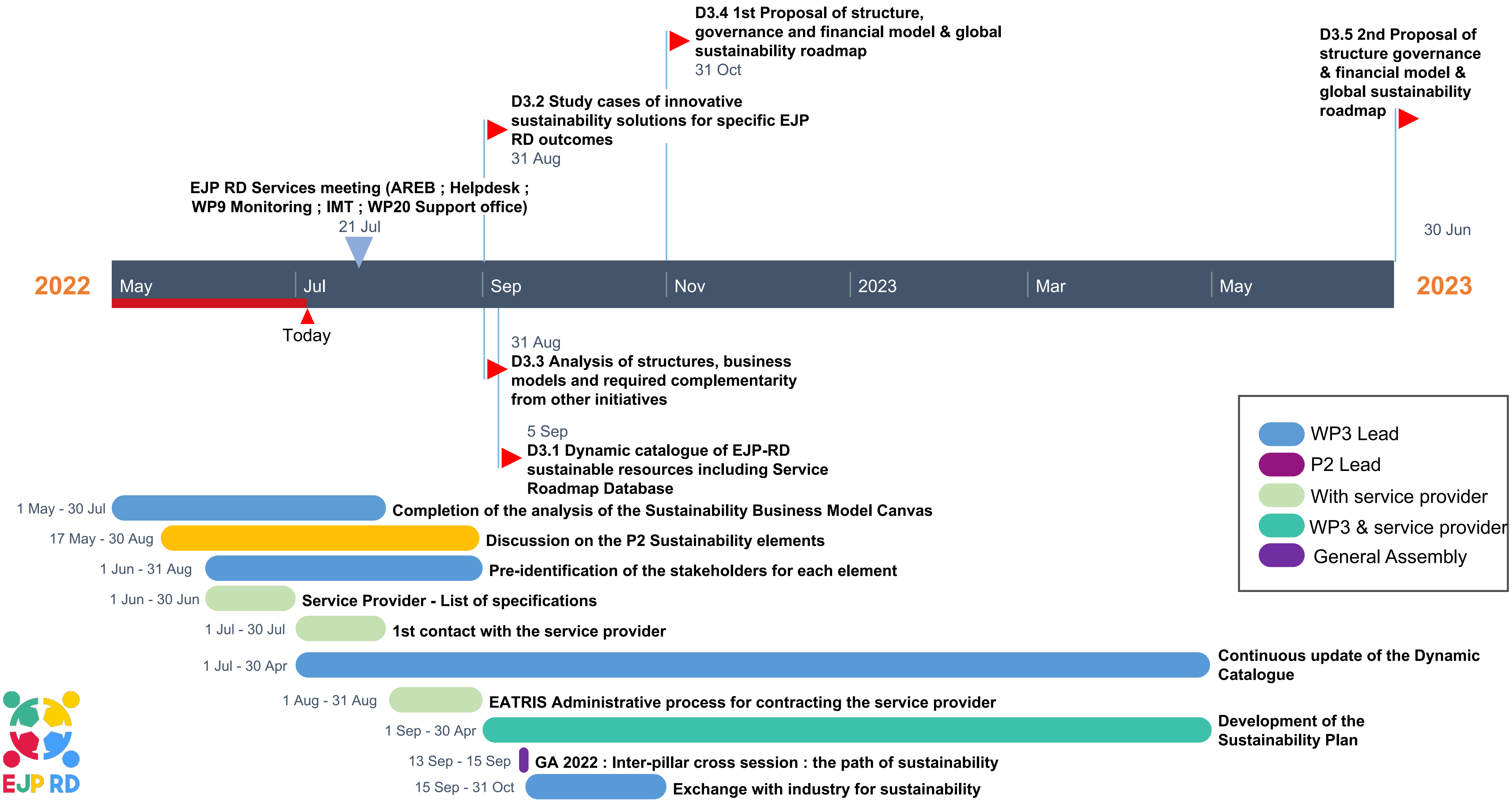
- This roadmap includes:
  - **Coordination services EJP RD & IRDiRC SciSec.**
  - **Central Helpdesk.**
  - **Advisory Regulatory Ethics Board (AREB).**
  - **Monitoring.**
  - **Innovation Management Toolbox.**
  - **Mentoring services.**
  - **Clinical Trials Support Office.**

- An internal meeting is planned

Next actions

- Complete P2 processes (steps presented at P2 retreat)
- Individualised feedback
- Services Roadmap internal meeting
- Stakeholders approach, including industry connections and Policy Board suggestions and questions (last slide).

General Timelines



D3.4 1st Proposal of structure, governance and financial model & global sustainability roadmap
31 Oct

D3.5 2nd Proposal of structure governance & financial model & global sustainability roadmap
30 Jun

D3.2 Study cases of innovative sustainability solutions for specific EJP RD outcomes
31 Aug

EJP RD Services meeting (AREB ; Helpdesk ; WP9 Monitoring ; IMT ; WP20 Support office)
21 Jul

31 Aug
D3.3 Analysis of structures, business models and required complementarity from other initiatives

5 Sep
D3.1 Dynamic catalogue of EJP-RD sustainable resources including Service Roadmap Database

1 May - 30 Jul	**Completion of the analysis of the Sustainability Business Model Canvas**
17 May - 30 Aug	**Discussion on the P2 Sustainability elements**
1 Jun - 31 Aug	**Pre-identification of the stakeholders for each element**
1 Jun - 30 Jun	**Service Provider - List of specifications**
1 Jul - 30 Jul	**1st contact with the service provider**
1 Jul - 30 Apr	**Continuous update of the Dynamic Catalogue**
1 Aug - 31 Aug	**EATRIS Administrative process for contracting the service provider**
1 Sep - 30 Apr	**Development of the Sustainability Plan**
13 Sep - 15 Sep	**GA 2022 : Inter-pillar cross session : the path of sustainability**
15 Sep - 31 Oct	**Exchange with industry for sustainability**

- 🟦 WP3 Lead
- 🟪 P2 Lead
- 🟩 With service provider
- 🟩 WP3 & service provider
- 🟪 General Assembly

Policy Board meeting questions

- What **specific stakeholder(s) in your country** can contribute to the sustainability of the EJP RD elements?

- Does your country have any investment roadmap or support service for RD that might be aligned with the sustainability plan of EJP RD elements (apart from project calls?)

- Do you have any national resource that would connect to the Virtual Platform?
  - In case there is any, how is this national resource supported/sustained at the national level? (This information would be valuable also for the forthcoming Rare Disease Partnership (RDP))

And now … MIRO !!!!

Thanks from WP3

EUROPEAN JOINT PROGRAMME RARE DISEASES

Update on the Rare Diseases Partnership

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National Meetings to Foster National Commitments

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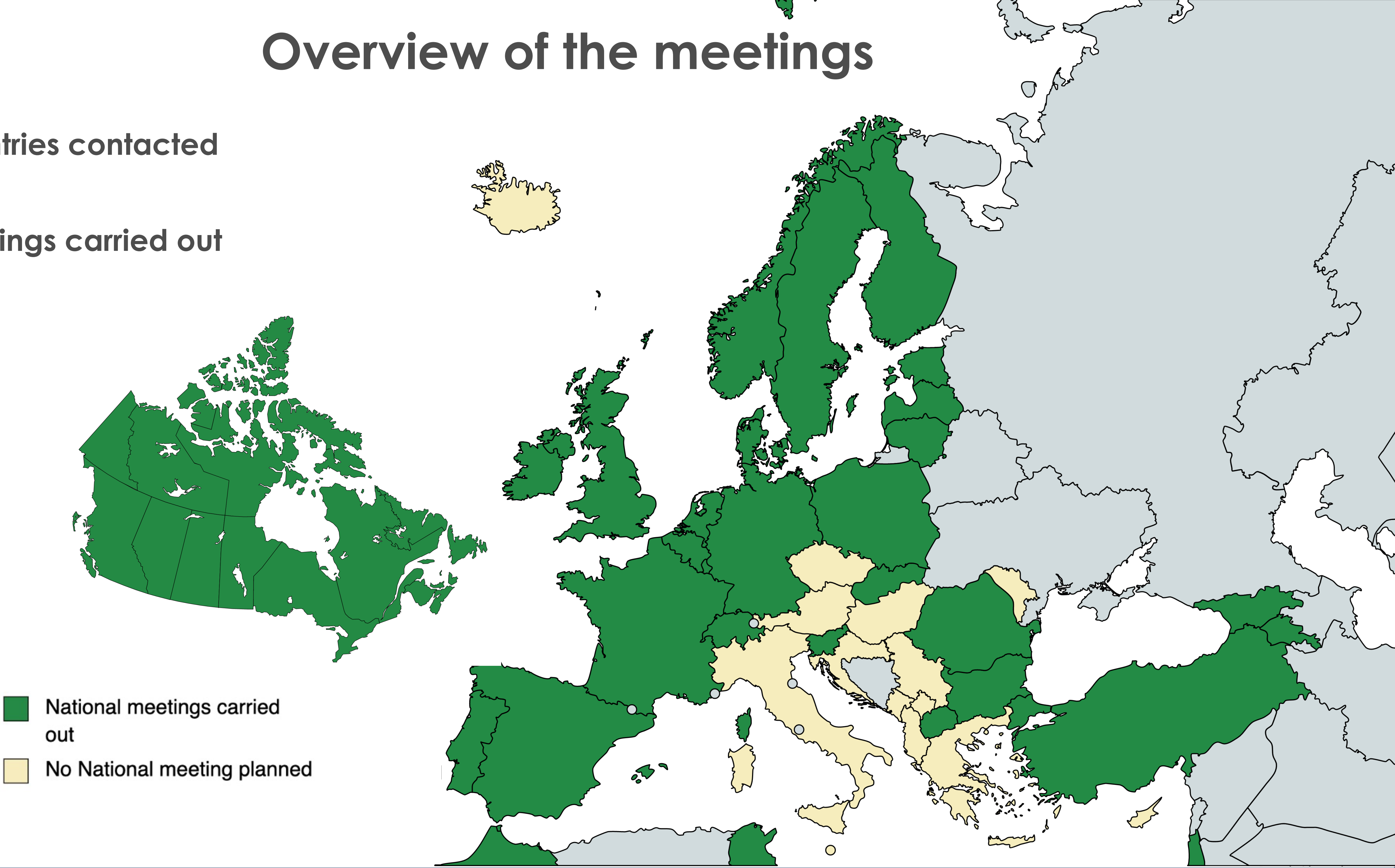
Agenda of the national meetings – Commitment for the RDP

- Rare Disease Partnership – Short Presentation
- In-Kind / In-Cash contributions - information
- Letter of commitment – Presentation of what is expected
- Identification of strengths – [Country] State of Art
- Exchange on the possible contribution, needs, institutions to be involved etc.

Overview of the meetings

- 44 countries contacted
- 30 Meetings carried out



Strategic Research and Innovation Agenda (SRIA)

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Objectives _ SRIA TF

1. Agree on the **structure of the SRIA**
    - (**level of granularity** with which different topics will be addressed);
2. Agree on **the process for SRIA preparation**
    - (type of activities that will be part of this process, e.g., organisation of Working Groups, public consultation, process for inviting experts, etc.)
3. **Help in coordinating the work of the whole process**,
    - and notably of the working groups if created.

EJP RD

Initial members identification _ SRIA TF

Active experts involved in the Concept paper development representing (<u>but not limited to</u>):

- **Various fields of activity**
  - (preclinical, translational and clinical research; drug development and diagnostics innovation; biostatistics; data science; regulatory science; research funding);
- **Different types of stakeholders**
  - (research organisation/institutions; hospitals/university hospitals; EU research infrastructure; patients’ organisations; foundations; funding bodies; regulatory & health technology assessment bodies, Member States representatives, European Commission);
- **Relevant programmes, initiatives and networks**
  - (EJP RD; Solve-RD; ERNs; Innovative Health Initiative; European Health Data space; DARWIN EU; CSA STARS; C-PATH).

Composition of the SRIA Task Force

14 ‘Funders’, including 6 representative of **funding agencies** + **8 experts nominated by Ministries**

2 IRDiRC representatives

2 representatives of **Patients organisations**

3 Experts for **ERNs** (+2 ERNs coordiators in the experts below)

11 Experts representing **relevant EU-funded projects or initiatives**

3 Experts on **Data**

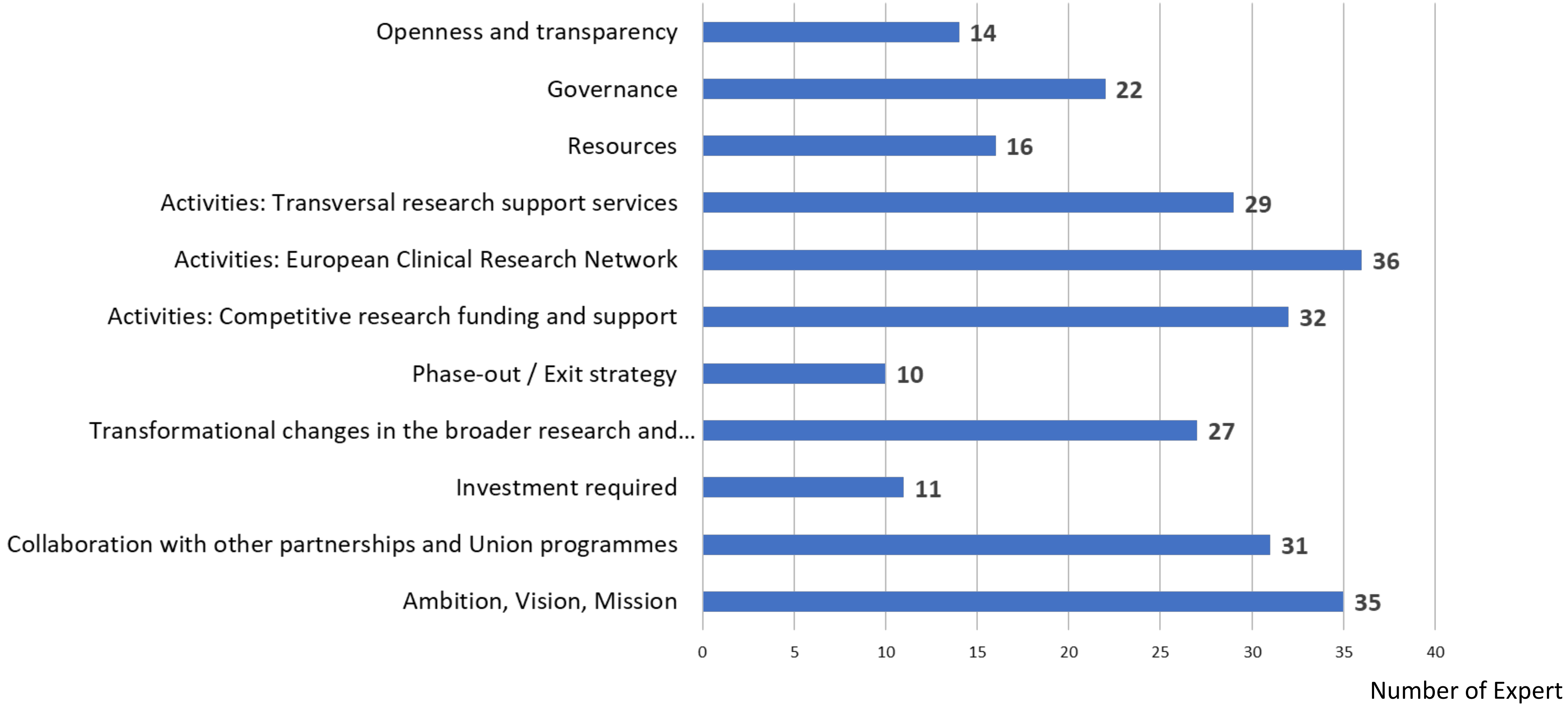
2 Experts for **relevant Research infrastructures**

4 representatives of **Foundations**

2 representatives from **Other organisations** (Industry/Private)

10 representatives of **European Commission and agencies (**

Distribution of the expertise in TF SRIA RDP



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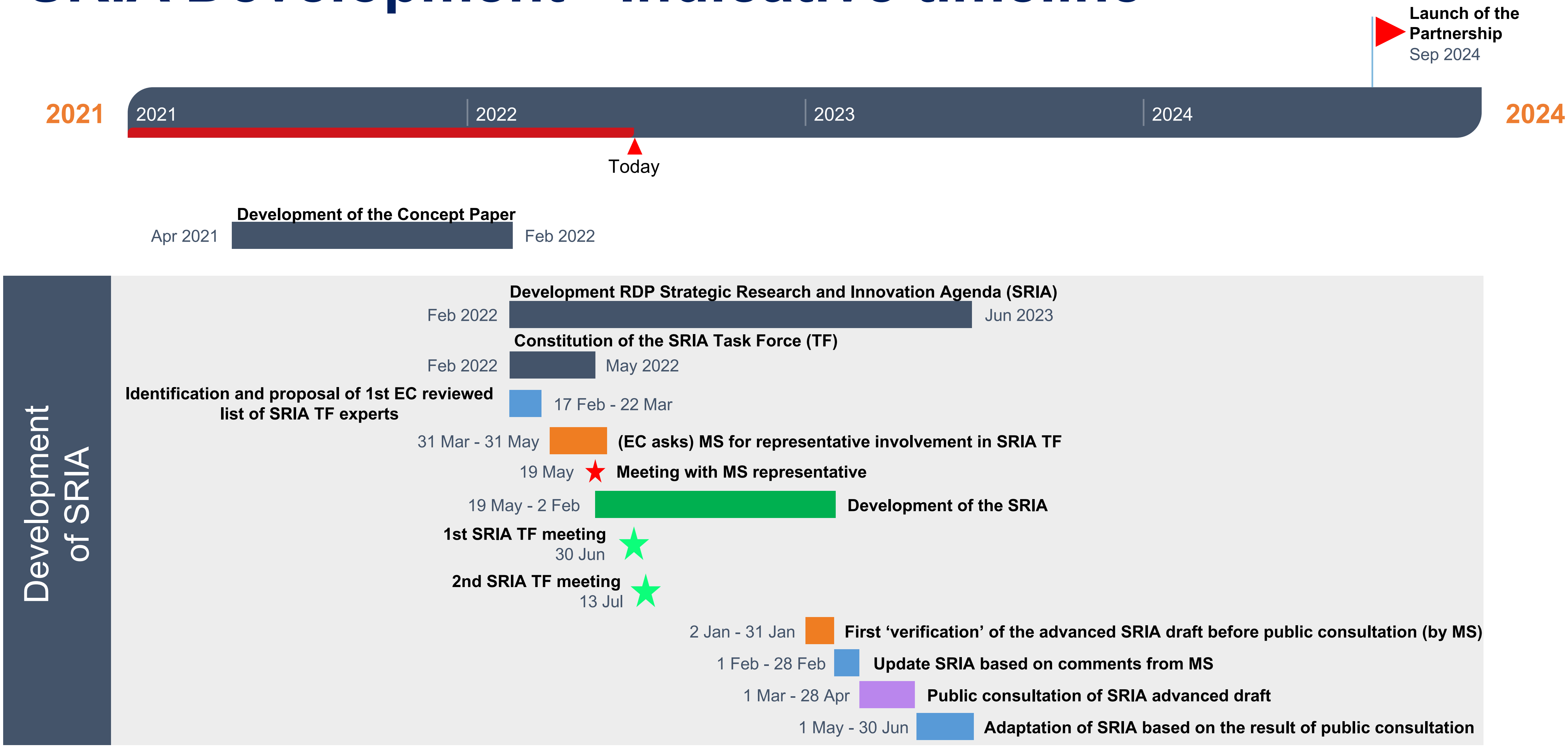
SRIA Development - Indicative timeline



Launch of the Partnership
Sep 2024

2021 2024

Development of the Concept Paper
Apr 2021 – Feb 2022

Development RDP Strategic Research and Innovation Agenda (SRIA)
Feb 2022 – Jun 2023

Constitution of the SRIA Task Force (TF)
Feb 2022 – May 2022

Identification and proposal of 1st EC reviewed list of SRIA TF experts
17 Feb - 22 Mar

31 Mar - 31 May **(EC asks) MS for representative involvement in SRIA TF**

19 May ★ **Meeting with MS representative**

19 May - 2 Feb **Development of the SRIA**

1st SRIA TF meeting
30 Jun ★

2nd SRIA TF meeting
13 Jul ★

2 Jan - 31 Jan **First ‘verification’ of the advanced SRIA draft before public consultation (by MS)**

1 Feb - 28 Feb **Update SRIA based on comments from MS**

1 Mar - 28 Apr **Public consultation of SRIA advanced draft**

1 May - 30 Jun **Adaptation of SRIA based on the result of public consultation**

This timeline is subject to change

Proposal for SRIA development process

(according to the Concept paper)

Preparatory/Initial phase

- Set-up a dedicated expert team to guide and oversee the development and implementation of the SIRA
- Identify the key stakeholders to include in the SRIA framework development process

Writing & Consultation process

- Initial reflection and analysis of gaps and opportunities
- Early ideas about scope, impact, outputs should be consulted by the core partners with several test audience
- Preliminary draft should be open for public consultation
- Above-mentioned results should be used for the SRIA revision
- Draft will continue to evolve as more information about the scope of other EU programmes become available

Validation phase

- Final SRIA will be submitted for approval of the General Assembly and EC (before signature of the Grant Agreement)
- SRIA will be reviewed at the occasion of the Partnership interim evaluation

Proposed Template Structure for the SRIA

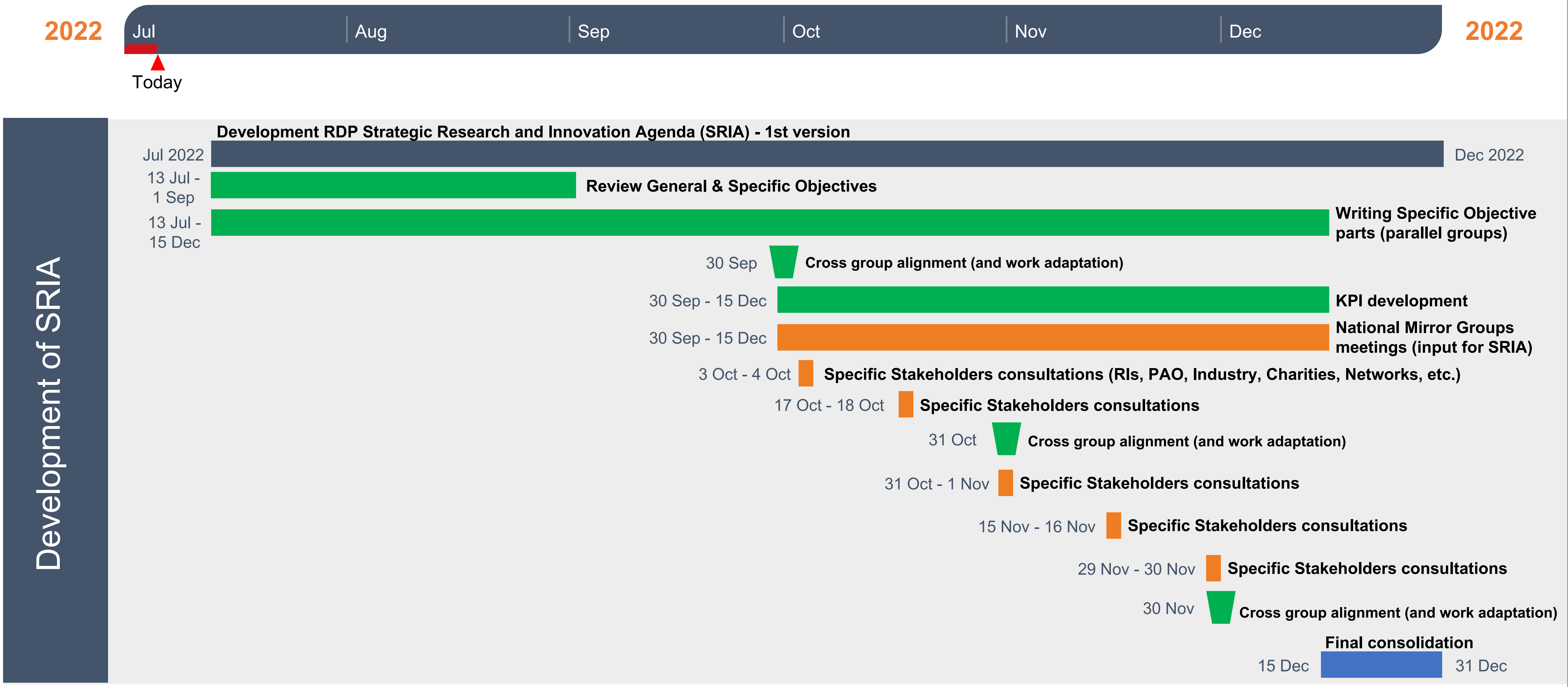
- **Chapter 1: Vision of the Rare Diseases Partnership**
    - Missions
    - Building on Lessons learned in the RD context (general from Conxcept paper
    - General Objectives
    - Intervention Logic
    - Thematic focus ?
    - Synergies with other initiatives

- **Chapter 2: Specific Objectives of the Rare Diseases Partnership**
    - **SO 1:** Patient-need led relevant research enabled by outcome-oriented investments strategically deployed along the R&D value chain
        - **Challenge**
        - **Scope**
        - **Potential Outputs**
        - **Expected Impacts**
    - SO 2; SO3; SO4; etc.

- **Chapter 3: Performance Indicators**

- **Chapter 4: Conclusions**
- **Annexes** (intervention logic, (candidate) Details of synergies including European Partnerships & EU Missions of Potenial relevance, Glossary, etc.)

SRIA Development Methodology Proposal - 1st version focus



Development RDP Strategic Research and Innovation Agenda (SRIA) - 1st version
Jul 2022 – Dec 2022

13 Jul - 1 Sep **Review General & Specific Objectives**

13 Jul - 15 Dec **Writing Specific Objective parts (parallel groups)**

30 Sep **Cross group alignment (and work adaptation)**

30 Sep - 15 Dec **KPI development**

30 Sep - 15 Dec **National Mirror Groups meetings (input for SRIA)**

3 Oct - 4 Oct **Specific Stakeholders consultations (RIs, PAO, Industry, Charities, Networks, etc.)**

17 Oct - 18 Oct **Specific Stakeholders consultations**

31 Oct **Cross group alignment (and work adaptation)**

31 Oct - 1 Nov **Specific Stakeholders consultations**

15 Nov - 16 Nov **Specific Stakeholders consultations**

29 Nov - 30 Nov **Specific Stakeholders consultations**

30 Nov **Cross group alignment (and work adaptation)**

Final consolidation
15 Dec – 31 Dec

This timeline is subject to change

THANK YOU

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