

# **EJP RD**

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### **Del 2.2**

## **Prioritization scheme for EJP RD actions, including decision-making process**

### **– Guidelines for prioritization –**

**Organisation name of lead beneficiary for this deliverable:**

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## 1. Introduction and Objectives

This deliverable D2.2 is a result of the activity developed in Task 2.1 of the Work Package 2 (WP2), within the transversal Pillar 0 of the European Joint Programme on Rare Diseases (EJP RD). WP2 is specifically aimed at the development of EJP RD research and innovation strategy in connection to all related stakeholders. Within WP2, Task 2.1 focuses specifically on the "Prioritization scheme for EJP RD actions". The main outputs of Task 2.1 are:

- **Deliverable 2.1 (D2.1): Final list of prioritization criteria.** According to the EJP RD approved proposal, such prioritization criteria should serve to order mapped needs and actions that contribute to the EJP RD objectives or that stand in need of further exploration. The identified criteria will encompass scientific evidence aspects, demands of the RD community, regulatory and societal concerns, and take into account financial and technical feasibility. D2.1 includes an enumeration and description of the main principles which could be applied for the assessment of any EJP RD activity that should be subject to prioritization.
- **Deliverable 2.2 (D2.2): Prioritization scheme including decision-making process.** Such scheme will provide detail on the different steps for the process of prioritization. Such process will include the application of the prioritization criteria (expressed in D2.1) whenever needed, and its successive validations to progress on the decision-making. Therefore, this document (D2.2) has to be read and applied in conjunction with the content of deliverable D2.1.

In general, "**prioritization**" can be defined as the process of deciding what should be built and when, based on what will bring most value to the user (in wide sense) and what is feasible. The objective of the prioritization in this context is to optimize the use of existing resources to achieve the goals of the EJP RD for the benefit of patients. Such optimal use requires also some **decision-making** that should be transparent and should avoid arbitrariness. Therefore, a double approach should be provided for these two closely linked processes and their interactions: **prioritization and decision-making**. The **objective of this document** is to provide guidelines for the application of both processes to EJP RD activities when needed.

Within a project, for best practice, any action should be assessed taking into account the expected outputs and advantages that such action adds to the project, weighing also the inherent risks of its implementation, the interdependent tasks, as well as constraints, allocation of resources, budget and schedule. For the prioritization process, all this has to be taken into account, together with the stakeholders' needs, and also those needs of the project itself for the achievement of its objectives. Under such premises, any activity or element in the project should be included in one of these four general categories:

- What must be done
- What should be done
- What could be done
- What will not be done

Such qualitative categorization can be rather complex sometimes and will require a careful process that should be approached always in an objective manner, with transparent procedures. Also, especially when considering the choice between different options, apart from the qualitative categorization, it could be needed some scoring or subcategorization, in order to establish the best option among all those available.

Therefore, this document aims at providing some different practical methods that have proven being effective in the reviewed literature and that, made available for all the activities in the EJP RD, will ensure some homogeneity in the methods, but at the same time will enable the needed flexibility with respect to their selection for best fitting to those needs.

## 2. Methods for the preparation of D2.2 (and D2.1)

In order to establish the prioritization scheme and decision-making process, WP2 components have **followed several steps** in a sequential order:

1. Internal discussion among WP2 components.
2. Presentation and discussion of the Work Plan for Year 1 of the project, with the Executive Committee during its kick-off meeting.
3. Discussion of WP2 team with the EJP RD Coordinator and Coordinating Team, during the progress of the work.
4. Further discussions of WP2 co-leaders with the Executive Committee.
5. Discussion with the Policy Board in its first face-to-face meeting.
6. Further work within WP2, taking into account the input obtained from the above mentioned bodies for the preparation of deliverables D2.1 and D2.2, including a literature review focused on existing methods for prioritization and selection of the ones that best fit the EJP RD characteristics.
7. Critical examination of D2.1 and D2.2 by the Coordination and Executive Committee.
8. The Executive Committee endorsed D2.1 and D2.2 and submitted the prioritization scheme for further input and validation of the Governing Board.

9. The prioritization scheme was submitted to the Policy Board for their adoption.

From the work on the above steps, **some important premises were obtained**, to be taken into account as the base for all the developments regarding prioritization and decision-making process. Such premises came from all the internal work and discussions of WP2 components with the different mentioned bodies, and these were the specific points that were identified:

1. All the activities in the EJP RD were specified in much detail in the proposal approved by the European Commission. The description of the planned work for the 5 years of the project was exhaustive and, therefore, all the activities were planned, distributed (among partners and along the time) and prioritized accordingly in advance, from the beginning until the end of the project. This means that only in exceptional circumstances there will be a strong need to apply the prioritization strategy for major reasons.
2. The EJP RD Executive Committee would like to use the prioritization process internally to help the decision-making within a task/subtask of the project.
3. There will not be need to involve the Policy Board in the routine prioritization process and decisions. Thus, only the points of major importance would be discussed with the Policy Board and submitted to their advice, being consulted to collect their feedback. Its role is moreover essential to bring specific national needs in the discussion.
4. The development of a "novel" prioritization strategy or methodology is not an objective itself for the EJP RD, since the EJP RD has a different objective and because the resources, time and budget needed for that purpose would have been different since the inception of the project.
5. Therefore, the prioritization strategy becomes a "**Guideline for prioritization**" that can be rather managed as a guiding tool for the prioritization in everyday activities within the EJP RD, to optimize time and resources. Thus, it can benefit also from the complementarity of other means that can be considered useful by the different leaders who are responsible for the diverse actions, if it is justified.
6. The prioritization strategy/guidelines are not intended as a way of evaluation of the different activities, because other tools were foreseen for that purpose.
7. The prioritization strategy/guidelines should be applied transversally by all parties of the EJP RD to all the actions requiring a decision between different choices.
8. The prioritization strategy/guidelines will be of special relevance and useful for the leaders of the different Tasks (and Subtasks), WPs and Pillars when determining each Annual Work Plan (as part of the planned routine), to establish the most convenient order of their activities for the following year. In fact, the project itself included that flexibility of the annual work plans to make it better through the process of prioritization in a changing scenario.
9. Given the diversity of activities that are included in the EJP RD, the general procedure can be applied with some flexibility, adapting it to some specific needs (if this is justified and reasoned).
10. To make easier the application of the procedure, it includes several methodologies (and tools), to ensure that it can be applied to the diverse

activities in the project, according to their different characteristics. It is relevant to emphasize the importance of being consistent in the choice of the method, therefore using the same methodology for the same kind of choice.

11. **The process of prioritization and decision-making must be always objective and transparent.** This means that whenever a prioritization process is applied (and decision taken), it should be stated in the minutes of meetings, in the corresponding deliverable(s) or any other linked document, as well as the specific method applied, the reasons to do it and the result of prioritization.
12. Due to the complexity and the duration of the EJP RD, as well as the early stage of some activities when this document was prepared, it is difficult to fully predict all future pathways and requirements. This implies that this document should be general enough to cover most eventuality, and the procedure flexible enough to make it applicable along the whole life of the project and to any of its activities.
13. In any case, these guidelines for prioritization could be improved and modified if it is justified and reasoned, for further validation by the corresponding bodies.
14. Prioritisation should be seen as an opportunity for all to increase the impact of EJP RD, and this assumption should be integrated into all its activities.
15. As a recommendation from the Policy Board, in case external opinion is needed in that process, a committee involving different stakeholders would have to be built.
16. For decision-making, the Policy Board emphasized that a consensus should be finally reached.

### 3. Results

From the review of the scientific literature, numerous methods on how to prioritize have been found. Prioritization is a common subject in business, so there are many references in that field. In the field of medical research, however, there are less references devoted to the methods for prioritization. On the other hand, some of them refer to the *prioritization of projects* rather than the *prioritization of specific activities in the frame of a project*. Nevertheless the principles are equally applicable in their essence, because they use the same or comparable criteria.

In a review performed on research priority-setting at the World Health Organization (WHO) [Terry et al, 2018], several methods were identified after examining 115 data captures summarizing publications which contain research priorities. These ranged from meetings or surveys that collect expert opinion, to more systematic methods that combine a review of the literature, Delphi surveys of stakeholders and the use of methods with weighted criteria. The most widely reported method used to identify priorities was expert consultation (in 86%), followed by literature review (52%), with some overlap since in some cases the expert consultation was combined with literature review. Almost 70% of the identified research priorities were developed without using any additional criteria to rank the priorities. Other methods used were Multiple Criteria Decision Analysis (MCDA), Delphi studies, the Child Health and Nutrition Research Initiative (CHNRI) method and the Council for Health Research and Development (COHRED 3D) Method. In short, these methods are based on the discussion of the problem and developing some way to reach a consensus, or

checking and assessing several features for each topic, assigning them the corresponding scores.

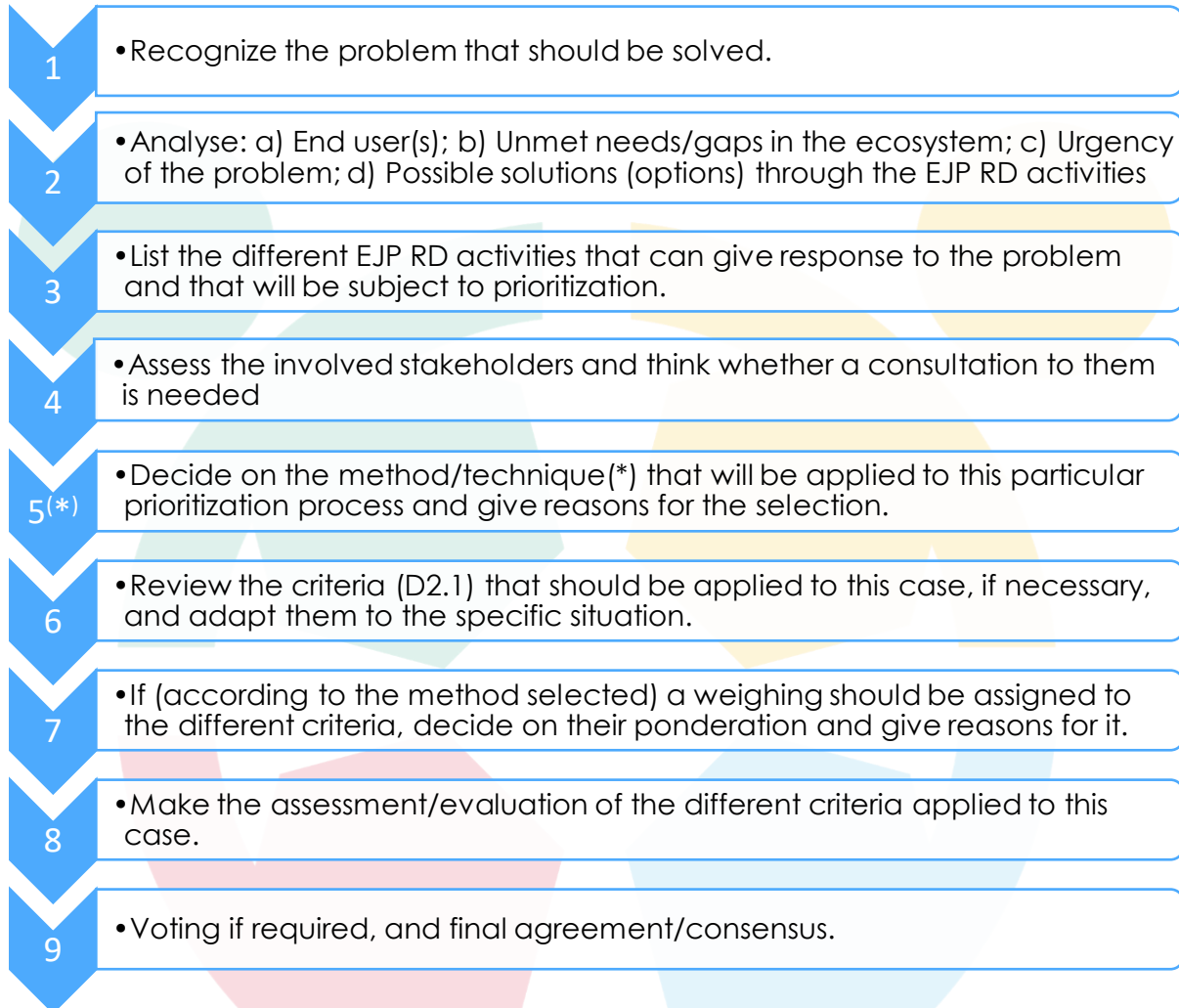
Now, as part of the Results, it will be presented:

- First, the **prioritization** aspects, including the different methods that could be applied.
- Second, the issues related to the systematic for **decision-making**.



### 3.1. Main steps for the process of prioritization in the EJP RD

In **Figure 1** below, the main steps for the **prioritization process** in the EJP RD are summarized:



(\*) See next section of D2.2 for further details on the different methods/techniques that can be applied for the prioritization process.

**Figure 1.** Main steps for the prioritization process.

#### 3.1.1. Main methods proposed for the process of prioritization in the EJP RD

From the review performed, it has been concluded that, while there are a number of published methods describing different approaches for setting priorities for health research, there is no single best practice [Terry et al., 2018]. Therefore, in this deliverable D2.2, several methods that should work best for EJP RD, are summarized. The reason for not offering just one method is that given the huge diversity of activities included in the EJP RD, this is a way to ensure that a method that best fits to the purposes of prioritization in each case, will be available for all, according to the characteristics of the considered activities. This practice of adopting several methods for prioritization is also used by other organizations, like WHO [Viergever, 2010], which recognizes a wide

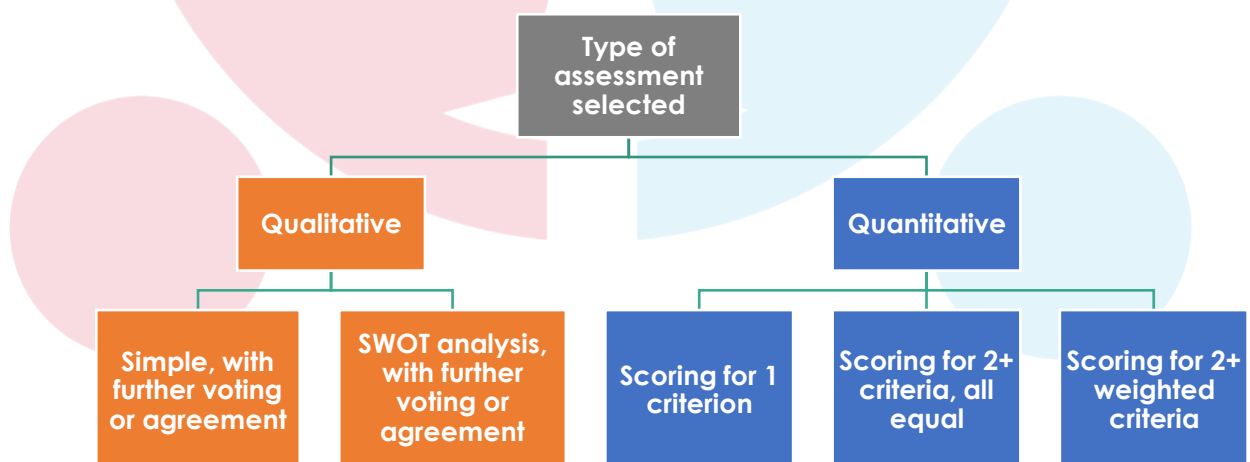
variety of research priority exercises undertaken, and these indicate that there can be no gold standard or best practice in setting those priorities.

As indicated in the first step of Figure 1, prioritization starts recognizing the problem that should be solved, so the greater the knowledge on the problem, the more likely the best solution will be found. Depending on the case, this may require a review of the literature, a characterization of bottle-necks in processes, detailed examination and characterization of the different parts of the problem, diverse involved stakeholders, and a variety of features that should be clearly defined in order to better prioritize and for further decision-making. The ideal situation when several elements should be prioritized is to study and determine the same features for the different elements, in order to compare them.

Once that knowledge has been completed, when trying to prioritize between different options, there are some general solutions:

- Simple qualitative assessment of the different activities, and further voting/agreement.
- Qualitative assessment of the different activities through a SWOT (considering internal Strengths and Weaknesses and external Opportunities and Threats against a specific objective) analysis and further voting/agreement.
- Quantitative assessment (through a scoring) of the different activities, based on just one criterion.
- Quantitative assessment of the different activities, based on 2 or more criteria, with all the criteria equally considered.
- Quantitative assessment of the different activities, based on 2 or more criteria, with different weighing of the criteria.

According to that, Figure 2 represents the most simple decision tree that can be approached in first instance to select a method for the prioritization process:



**Figure 2.** Initial decision tree to select a method for the prioritization process.




As anticipated, here several methods are proposed to be applied for prioritization in the EJP RD, to provide the necessary flexibility to accommodate different contexts for prioritization. It will be explained from the simplest to the more complex ones.

The question is: “Which prioritization technique is best?”

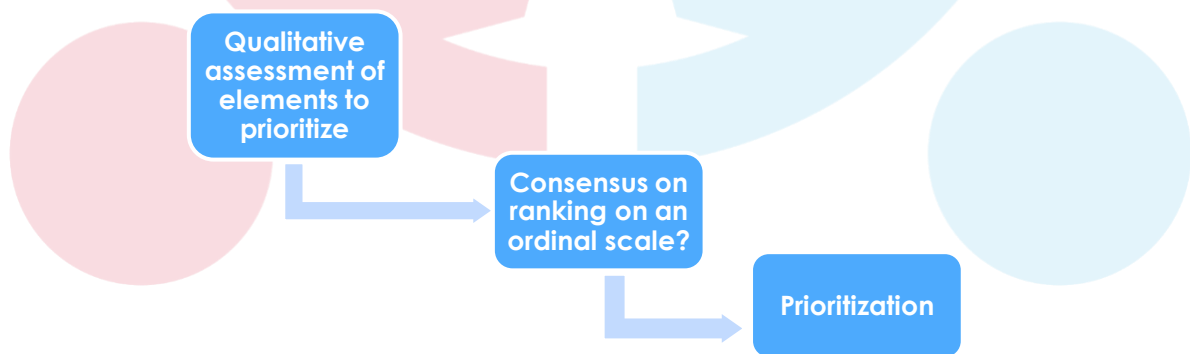
The best technique will be the one that fits your needs the most and accomplishes your goals in the least amount of time and with a minimal amount of resources.

For reporting purposes, this template could be used to describe the prioritization process chosen:

 <b>Template for describing the prioritization process that has been selected</b>	
Date (dd/mm/yyyy)	
Who performs the prioritization	
Elements subject to prioritization	
Qualitative / quantitative assessment	
Method selected for prioritization	
Reason(s) for the selection	
Other:	

### 1. Simple qualitative assessment with further ranking

After a simple qualitative assessment of the different elements to prioritize (Figure 3), these are ranked on an ordinal scale, so each one is assigned a numerical value based on its importance (according to some previously agreed criteria, like the ones indicated in D2.1), where number 1 is the most critical/urgent/imperative/important one, and as the number grows such character decreases. This method works best when there is only one stakeholder. If several stakeholders make their assessment, a consensus is needed on the ranking to establish. Alternatively, an average could solve the problem.



**Figure 3.** Simple qualitative assessment with further ranking.

If there is not a consensus on the ranking, a complementary method should be adopted, either through voting and agreement, through another qualitative more detailed method, or a quantitative method.

## 2. Qualitative assessment with further voting or agreement – MoSCoW method

The MoSCoW method [Clegg & Barker, 1994] was just merely presented in the Introduction of this document. Its name is an acronym of the four categories that will be established for the different elements to be prioritized, assigning them to one of these groups:

- **Must have** – critical elements without which a project could not succeed.
- **Should have** – essential elements that could impact the success of the project.
- **Could have** – interesting elements that are not essential to the project's success.
- **Won't have** – additional elements that can be eliminated.

Therefore, it is based on the establishment of what is necessary for the stakeholders and/or for the achievements of the project. It is a quite simple method, but sometimes it requires some refinement, because otherwise it is not enough to discriminate among different options.

Unless a strong agreement exists, this can be complemented in different ways:

- by surveying the stakeholders
- by voting of experts or in the working group
- combining it with some quantitative method

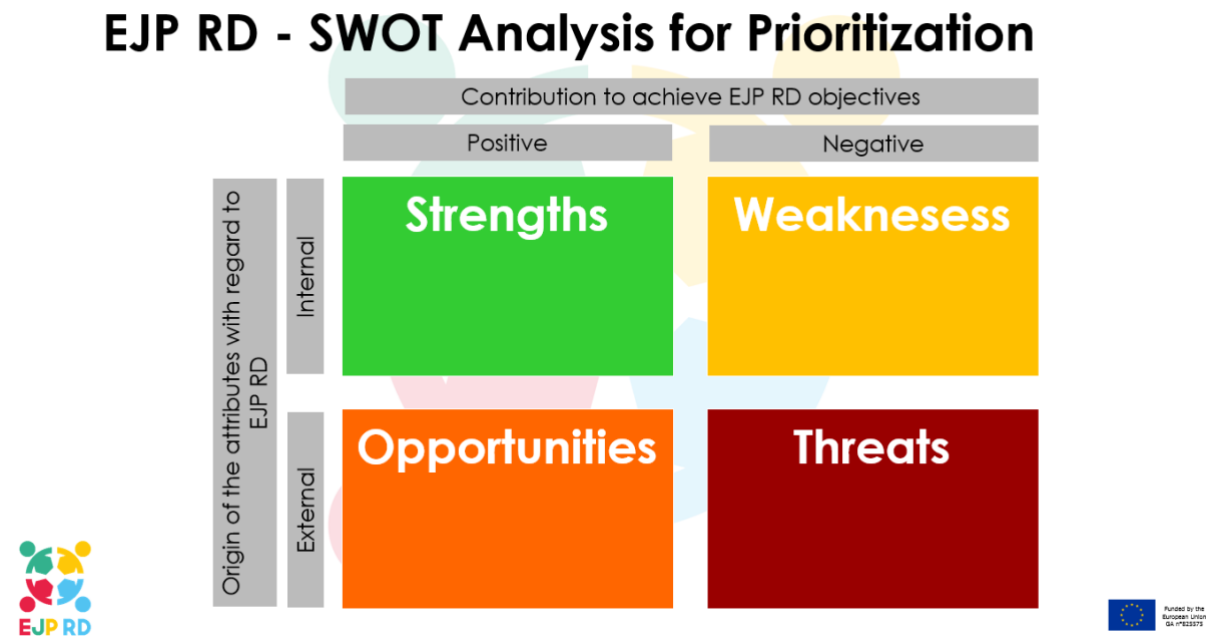
For **voting**, there is also a system if a Step-by-Step mode is adopted. Instructions for **voting on a Step-by-Step mode**:

1. **Round 1 vote** – Once a list of elements/subjects/problems has been established, each participant votes for their highest priority items. In this round, participants can vote for as many health problems as desired or, depending on the number of items on the list, a maximum number of votes per participant can be established.
2. **Update list** - Elements/subjects/problems with a vote count equivalent to half the number of participants voting remain on the list and all other elements/subjects/problems are eliminated (e.g. if 20 participants are voting, only items receiving 10 or more votes remain).
3. **Round 2 vote** – Each participant votes for their highest priority items of this condensed list. In this round, participants can vote a number of times equivalent to half the number of items on the list (e.g. if ten items remain on the list, each participant can cast five votes).
4. **Repeat** – Step 3 should be repeated until the list is narrowed down to the desired number of priorities.

### 3. Qualitative SWOT analysis with further voting or agreement


SWOT is the acronym for: Internal Strengths and Weaknesses, and external Opportunities and Threats. The assessment of these four characteristics for any activity and the comparison of the results for different activities provides the base for prioritization.

This is the essential matrix, represented in Figure 4, for the SWOT analysis:



**Figure 4.** Basic matrix for SWOT Analysis for prioritization purposes.

Such matrix in Figure 4 would be used for the characterization of one activity or element in the EJP RD. However, when comparing several of them, this matrix would be used:

 <b>SWOT Analysis for prioritization</b>				
Activity/element	Strengths	Weaknesses	Opportunities	Threats

In principle, for such SWOT analysis for prioritization, a qualitative assessment would be done. However, a scoring would be possible also, and useful if many options are managed.

#### 4. Quantitative assessment, scoring for one criterion

This method is very similar to the first method described here ("Simple qualitative assessment with further ranking"). For the first one, the qualitative assessment leads to ranking. In this case, the quantitative assessment leads to the assignment of a score for a selected criterion, to each element subject to prioritization, and the scores serve for establishing the ranking.

#### 5. Quantitative assessment, scoring for 2 or more weighted criteria: Analytical Hierarchy Process (AHP)

It is a mathematical tool of problem solving. The AHP method [Saaty, 1980] looks at the problem and decompose it into smaller sub-problems, which can easily be comprehended and analysed (in the form of a hierarchy).

Once the hierarchy is built, the elements are evaluated by comparing pairs to each other.

The total number of comparisons is:  $n \times (n-1)/2$  (where  $n$  is the number of criteria) at each hierarchy level. Numerical values (based on priorities) can then be assigned to each element of the hierarchy.

This method is not suitable for a high number of criteria as the number of requirements determine the number of comparisons that need to be made.

Here a template can be found for prioritization in an AHP: [Template link](#)

#### 6. Quantitative assessment, scoring for 2 or more equally considered criteria

Each criterion is valued on a scale from 1 to 10, with 10 the most positive value, according to a relevant metric scale which will depend on the nature of the activity subject to prioritization. Each criterion will originate a total value between 1 and 10 (All criteria's metric values are summed and divided by the total number of aspects considered for such criterion). If four criteria are selected, each action/choice will be listed summing the values for the four criteria ( $C1+C2+C3+C4$ ).

$$\text{Activity A, total priority} \quad A_{tp} = C1 + C2 + C3 + C4$$

Where the value of each criterion  $C$  is the sum of  $n$  criteria's metric values ( $n1 + n2 + \dots + nx$ ) divided by the total number of aspects ( $nt$ )

$$C1(\text{ the same for } C2, C3, C4) = \left( \frac{n1 + n2 + \dots + nx}{nt} \right)$$

All the alternative actions/choices are then listed by Priority Total value, and equal priority total items ordered by decreasing  $C1$  values, followed by  $C2$ ,  $C3$  and  $C4$  values.

The identification, for each Pillar, of significant aspects related to each of the four main criteria (see **D2.1**) is followed by the application of a metric scale and of a simple algorithm allowing to grade the different options and to make consistent decisions across the pillars and respond to novel requirements in the upcoming years.

Each criterion may have multiple aspects to be assessed as shown in the examples listed below (Examples of the aspects applicable to each activity or element for each criterion).

The aspects number and type are different due to the specific characteristic of each pillar and action to be assessed.

An example is shown in the following table:

Choice	Priority Total	C1	C2	C3	C4
A	34	10	10	7	7
B	33	10	5	8	10
M	33	5	10	10	8
I	33	5	10	9	9
L	30	3	9	9	9
G	28	10	6	5	7
C	27	9	5	4	9
H	25	7	9	2	7
F	24	8	4	5	7
D	23	9	2	4	8
E	23	8	3	4	8

### 3.2. Main steps for the decision-making process in the EJP RD

**Decision making** is the process of making choices by identifying a decision, usually after gathering information and assessing alternative resolutions.

The EJP RD has a clear **management structure** and clearly defines what specific bodies will make a decision in every case, according to their roles. These are the main levels and bodies defined in the **EJP RD governance structure**, on a bottom-up order, and their role in decisions:

- Participants in the different activities
- Subtask (ST) Leaders: making decisions on the activities in their ST, in what is not decided in an upper level.
- Task (T) Leaders making decisions on the activities in their Task, in what is not decided in an upper level.
- Work Package (WP) Leaders: making decisions on the activities in their WP, in what is not decided in an upper level.
- Pillar Leaders: making decisions that affect the WP/T/STs in their Pillar, in what is not decided in an upper level.
- Executive Committee: making decisions on the implementation, progress monitoring and annual programming.
- Operating Group: making decisions for the coordination of activities between Pillars and transversal Work Packages.
- Coordination: making decisions for the day-to-day management and transversal activities. The Coordination Office has its own structure in different levels that are clearly defined in the governance model of the EJP RD.

- Governing Board (GB): It is a mandated decision-making body.
- General Assembly (GA): It is the ultimate decision-making body.

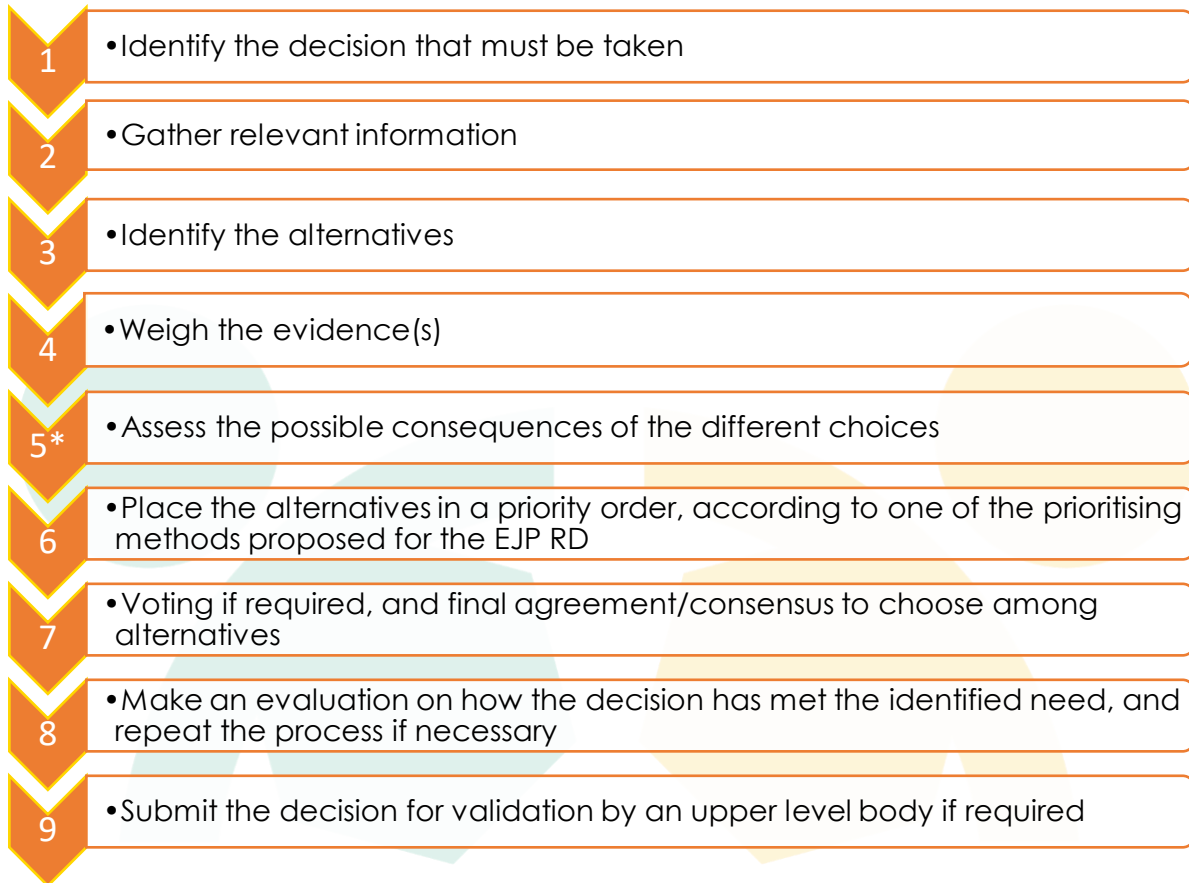
A precision is needed here because some decisions are in fact made by one of the above listed bodies or levels, but they also have to be validated by an upper level body, according to the governance model of the EJP RD, in order to ensure an effective, transparent and fair way to make the decisions, avoiding any arbitrariness.

Apart from the described vertical governance structure, this is completed with the participation of some **Satellite Boards**, which complement the decision-making process for the EJP RD:

- Policy Board (PB): It includes representatives of the national ministries of research and health; representatives of European Commission Directorates (DG RTD, DG Santé, DG Connect); representatives of the pharmaceutical industry and public-private initiatives (e.g. European Federation of Pharmaceutical Industries and Associations, EFPIA; Innovative Medicines Initiative, IMI); representative of regulatory authorities (e.g. European Medicines Agency, EMA, esp. Committee for Orphan Medicinal Products, COMP, EuNetHTA); Chair of the European Strategy Forum on Research Infrastructures (ESFRI); Chair and vice-chair of the International Rare Diseases Research Consortium (IRDiRC).
- National Mirror Groups (NMG): They include, in each country, representatives of the National plan for RD, national nodes of the European Reference Networks, relevant national authorities and research institutions (whether participating to the EJP RD or not), as well as the relevant national partners of the EJP RD and GB member that will report NMG views and positions during GB meetings. The NMG's role is essential to ensure that national activities, strategies and needs are taken into account when taking decisions at the EJP RD level and when designing the annual work plans.
- Board of Funders (BOF): Their role is essential to ensure the independence of joint transnational calls (JTCs) management. Thus, the final decisions on call topics and implementation of calls are autonomously made by the BOF.
- Ad hoc Advisory Board (AB): Constituted by international experts and RD stakeholders, for what refers to the research strategy for JTCs, and for pilot projects on demand of Pillar 2 and Pillar 4 leaders.

For the decision making process, all the above listed bodies will function on a democratic style, being conducted with equality, where it applies, with fairness and in a transparent mode.

Usually, a step-by-step decision-making process can help to make more deliberate, thoughtful decisions by organizing relevant information and defining alternatives, in order to choose the most convenient alternative possible for the purposes of EJP RD and RD Community in general. These will be the ordinarily considered **steps for the decision-making process**:



**Figure 5.** Steps for the decision-making process.

## 4. Conclusion

In this prioritization scheme some procedures and methods that can be applied transversally to any element of the EJP RD are provided as tools to facilitate the planning of future actions of the project.

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