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1. Introduction and Objective

The EJP-RD WP2 Integrative research and innovation strategy has as specific objectives:

- to map the needs for RD research and innovation
- to define the prioritization model for the EJP RD actions as a part of the annual planning and in connection with WP 3 (Sustainability) and WP4 (Ethical, Regulatory, Legal and IPR framework)
- to prioritize topics for the joint transnational calls (JTCs) to be implemented within Pillar
- to feed the medium and long-term RD research and innovation strategy in collaboration with IRDiRC
- to prepare a scoping paper to be promoted (and implemented) by the national mirror groups and within the current and forthcoming EC Framework Programme.

All this activities are part of Pillar 0 “Coordination and transversal activities”, the central coordination Pillar that assists the actions of the four major Pillars of the EJP RD.

This document (Del.2.8 “First scoping paper”), prepared by Task 2.2 leaders (ISCIII, ISS) in collaboration with CO, is foreseen for M7 by the Grant of the EJP RD as an additional document and tool reformulating the outcomes of the mapping and prioritization actions described in Deliverables:

- Del.2.1 “Final list of prioritization criteria”
- Del.2.2 “Prioritization scheme including decision making process” and the confidential report
- Del.2.3 “Summary document on Mapped Research and Innovation needs”

The present Deliverable 2.8 is a public report that gives an overview of the progressing work done in WP 2, task 2.2 and has to be transmitted to the leaders of Task 2.3, 2.4 and 2.5 for complementary actions, e.g. provide information to link with the ongoing or future Task Forces planned within IRDiRC (Task 2.4).

2. Methodology

The outcomes of the Deliverables 2.1, D2.2 and D2.3 were summarized to form this document.

It has to be mentioned that the present Deliverable is based on the ones that are still ongoing (D2.1, D2.2, D2.3) and that at the time of July 10, 2019 were available. This is to rely to the complexity of the activities and to the recent constitution of EJP RD Policy Board. This means that the Policy Boards' inputs will be effectively given in a further moment, and that the details from its feedback will be implemented to a future version of the above-mentioned documents (enclosed to this First Scoping Paper), to ensure that its advise will feed the coming actions of the EJP RD (as set in the Grant Agreement).

During the meeting held in Brussels on July 3 and 4 (2019), first inputs and suggestions were collected from the ExCom and new constituted Policy Board about the Prioritization Criteria and Research and Innovation Needs.

The process of deliberation and validation of the Research and Innovation started, using the contribution given from D2.1, D2.2 and D2.3 as a first strong basis and guideline for the prioritization of actions and decisions that will support the obtaining of EJP RD main objectives.

Moreover, also the National Mirror groups are under construction for each Country and will give their contribution on Research and Innovation Needs in the coming months. Their inputs will as well be implemented to a further version of D.2.3.

Furthermore, according to the AWP, Task 11.2 of WP 11 is progressing with the yearly analysis of the state of play of funded research and drug development, overlaps and trends at international level, and their input will be obtained and incorporated to the "Summary Document on Research and Innovation needs" (D2.3).

The present Deliverable is therefore based on the three documents available at the date of July 10, 2019:

- Del.2.1 "Final list of prioritization criteria" (v.05, 19-07-10)
- Del.2.2 "Prioritization scheme including decision making process" and the confidential report" (v.04, 19-07-10)
- Del.2.3 "Summary document on Mapped Research and Innovation needs" (VF-2, 19-07-10)

3. Summary Results

D2.1 "Final list of prioritization criteria", identifies a set of wide scope criteria that may apply to all EPJ RD activities and Pillars have been proposed.

The 4 general criteria identified in the approved DoA of the EJP RD Consortium are:

- (i) scientific evidence aspects,
- (ii) demands of the RD community,
- (iii) regulatory and societal concerns,

- (iv) financial and technical feasibility.

For a definition of each criterion and a number of different aspects for each criterion that may be used depending on the Pillar see document D2.1.

Examples that clarify how to apply the criteria exposed in D2.1 are reported in D2.2.

More extensively, the “Prioritization Scheme, including decision-making process” (D2.2) is intended to set the criteria and procedures defining the decision-making process by which to order and map emerging needs and actions that contribute to the EJP RD objectives or for necessities that stand in need of further exploration.

The prioritization Scheme will be therefore used to:

- support and assess the decision-making process by which to prioritize mapped needs and actions that could contribute to the achievement of the EJP RD objectives
- facilitate the planning of future actions within the annual work plan of the programme
- intervene when some deviation from the EJP RD’s plan happen or are envisioned (in such case, the involved WPs should notify the Coordination Team, so the most adequate measures can be adopted)
- further ameliorate the criteria, indicators and methodology used for the process itself, after assessing the impact of the decisions taken.

The criteria identified in Del 2.1 will be applied to the state-of-the-art of existing strategies/priorities/needs that has been performed in the confidential report D2.3. The publication of this document and its contents can be published only after finalization and publishing of the IRDiRC Roadmap.

D2.3 underlines the support from the European Union for research on Rare Diseases and provides a reference on the research and innovation needs that can serve to plan further actions, to assess progress and to contribute to the development of the long-term strategy of the EJP RD.

As considered and agreed, a review of existing elements on the Research and Innovation Needs has been performed in D2.3 to provide a wider and richer approach to better delineate the mapping of the needs, in addition to the inputs foreseen by the Grant. In particular for the version of D2.3 of July 10 that was taken into account to elaborate this first scoping Paper, Del 2.8 the elements reviewed were:

- existing formal documents from previous or existing initiatives in the European Union (such as E-Rare)
- existing reports and formal documents made public through the websites of organizations (such as EURORDIS)
- documents from other international collaborations (such as IRDiRC)
- articles or reports published in the scientific literature

The results of the analysis of the above-mentioned documents on Research and Innovation Needs were divided in different areas of interest:

- Data on the Research and Innovation Needs coming from Patients
- Data on Research and Innovation Needs coming from the European Reference Networks (ERNs)
- Data coming from the document produced in 2017 by the Directorate-General for Research and Innovation of the European Commission
- Data from Joint Transnational Calls (JTC)
- Data from Horizon 2020
- Data regarding existing infrastructures ESFRI (the European Strategy Forum on Research Infrastructures)
- IRDiRC' s contributions to delineation of Research and Innovation needs
- Considerations about ethical, legal and social issues (ELSI).

For the specific sources, documents or group of documents that provided relevant information for the purpose of the Summary see the enclosed D2.3.

4. Next actions:

This document will be transmitted to the leaders of Task 2.3, 2.4 and 2.5 for complementary actions and, as planned in the Grant Agreement, a new report of the Scoping paper will be released yearly with the aim to orient actions to be implement and the ones that require medium and longer-term investigation .