Membership List and Terms of Reference for Panel of Experts

Milestone 4.1

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WP Participants:
UNEW, ISINNOVA, EURORDIS, INSERM

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Purpose of the Panel of Experts

The breadth of scope of the Rare2030 project is one of its major strengths and the source of significant added value; however, herein lies one of the greatest challenges. To enable a sweeping review of the status quo in European rare disease activities and policies, and to build on this to identify and rank drivers of change and determinants of health, it is necessary to consult as wide a body of stakeholders as possible. The partners therefore agreed to create a Panel of Experts (PoE), to unite up to 250 stakeholders with expertise in various topics of relevance to the diagnosis, treatment, management and care of rare diseases.

WP4 created two outputs to support the creation and functioning of this PoE:

1. A Membership List
2. A Terms of Reference Document, to elucidate the role expected of this PoE

Membership List for Panel of Experts

An online membership list has been created. In M3, this should more accurately be considered an invitation list. The full invitation list is not submitted here; instead, a membership list will be made available as part of the future reports under this WP, once invites have accepted the ginvitation.

The Membership/Invitation list comprises key stakeholders from a very broad background. A relatively small number of individuals have been selected in their own individual expert capacity: this is especially the case with thus representing fields outside of the traditional rare disease community, e.g./ nanotechnology experts, eHealth experts, cancer experts, etc.). However, the vast majority of individuals have been included to this invitation list because of the broader bodies/groups/committees they represent. The following table illustrates the main categories of stakeholders included to this Invitation List.

Types of stakeholders	Organisation
Advocacy and support groups/volunteers representing people living with a rare disease and their families	• EURORDIS Council of National Alliances (including members of Data Contributing Committees for the 'State of the Art of RD Activities in Europe' Resource) • EURORDIS Council of European Federations • other disease-focused organisations at national level
EU policy makers	• Members of the European Commission (e.g. DG SANTE and DG RTD amongst others) • Members of the European Parliament (Network of Parliamentary Advocates for Rare Diseases) • Former members of the Commission Expert Group on Rare Diseases • Members of the Steering Group on Promotion and Prevention (SGPP)
Health Care Providers	• Notably from ERNs and constituent Centres of Expertise; • other relevant integrated care experts e.g. International Foundation for Integrated Care
National/regional competent authorities	• Members of the European Reference Network Board of Member States • Members of Data Contributing Committees for the 'State of the Art of RD Activities in Europe' Resource
Hospital managers	Notably from ERNs and constituent Centres of Expertise
Social care and social innovation experts	• European Network of Resource Centres for Rare Diseases • International Federation of Social Workers Europe • Centre for Social Innovation (ZSI, Austria)
Academia, learned societies	• e.g. European Federation of Internal Medicine (EFIM); European Hospital & Healthcare Federation (HOPE); European Society of Human Genetics (ESHG); International Society for Pharmaco-economics and Outcomes Research (ISPOR)
Basic Researchers	Individual basic researchers in the rare disease field and beyond
European networks and research infrastructures	Such as: • E-Rare and the European Joint Programme for Rare Diseases • EuroBioBank; • ECRIN, BBMRI, ELIXIR • RD-Connect; • SCOPE Joint Action; • Solve-RD on undiagnosed diseases; • EUPATI (IMI); • ADAPT-SMART (IMI), • conect4children (c4c) (IMI); • PARADIGM for good practices on the engagement of patient in product life cycle with all stakeholders (IMI)
Pharmaceutical and Devices Industry and other health-related industries	Pharma, biopharma, Medical Devices and Medical Technology Industry, investors
Regulators	Representatives of EMA Committees and Working Groups
HTA bodies/reimbursement authorities	• HTA Network and its Stakeholder Forum • EUnetHTA Joint Action for Scientific Advice and Relative Effectiveness Assessments;
International initiatives	• International Rare Disease Research Consortium (IRDiRC); • Rare Diseases International (RDI); NGO Committee for Rare Diseases (United Nations, New York); • International Conference on Rare Diseases and Orphan Drugs (ICORD); • International Alliance of Patients' Organisations (IAPO)

Other relevant European Not-for-Profit Organisations	• European Patients' Forum (EPF); • European Cancer Patient Coalition (ECPC); • Rare Cancer Europe; • Social Platform; • European Disability Forum (EDF); • Drug Information Association (DIA).

The table of stakeholder groups was circulated amongst the Rare2030 partners, who amended it in places to give as broad a representation of expertise as possible. individual names were added to the Invitation List based upon the categories above. For instance, win the case of competent national authority representation, the partners ensured at least one formal representative per EU Member State. For key projects, coordinators or leaders of pillars/WPs were selected. Where there could potentially be many hundreds of representatives (e.g. Industry bodies such as the EURORDIS Roundtable of Companies), individuals were based upon track record of playing a key role in strategic meetings or workshops, chairing taskforces etc.

In addition to the 'external' experts, the list contains representatives from the Rare2030 partners plus the invitees of the Research Advisory Board.

The full list of potential invitees involves over 450 stakeholders. Therefore, it was necessary to distinguish these people to two categories: one category for the 'first-line' invitees, and a second as a reserve list in the event that first-line invitees decline to participate in the PoE. Once the deadline has passed for the initial invitees to accept or decline the invitation, the WP4 leadership team will examine the number of positive replies. If this dos not exceed 250, the team will consider extending the invitation to potential members in category 2 (the most persuasive selection factor here will likely be the need to ensure as broad a geographical coverage as possible).

Selection of invitees for two categories relating to the European Reference Networks perhaps warrants particular attention here: To select ERN representatives and ePAG patient representatives.

It was agreed that the project would send an invitation to each ERN coordinator (along with their coordination team i.e. project managers) and ask them to either nominate a colleague from the ERN or else disseminate the invitation amongst their HCP representatives and ask for a volunteer. Thus, the official representative could be the coordinator or a person in the Network to whom they wish to delegate that direct representation. To select a patient representative of each ERN: where an ERN already had an ePAG formally included on the Panel of Experts invitation list, it was decided not to seek a second representative. However, for the remaining Networks (16) the team will send an invitation to the Coordinator, inviting them to select a representative from amongst patient representatives (ePAGs or otherwise) and inform the Rare 2030 partners of this person's contact details.

Upon invitation PoE candidates will be asked to self-nominate themselves as expert or interested in one of 8 subtopics. This will allow for smaller working groups with in this groups to move through the Horizon Scanning stage of the foresight study. The subgroups are listed here and elaborated in **M4.2 Methodological Paper Governing the Literature Review for Horizon Scanning.**

- Political & strategic frameworks relevant to RD
- Data collection and utilisation
- Availability and accessibility of Orphan Medical Products (OMPs) and medical devices
- Basic, clinical and translational research
- Diagnostics
- Social integration of RD and holistic care
- Patient engagement, patient empowerment and patient centred approaches to RD issues
- Accessing healthcare

The Terms of Referemce document is now finalized, as below:

Rare2030 Panel of Experts

Terms of Reference

Overview of the project

In the last decade, we have seen tremendous progress in policies that improve the lives of people living with a rare disease in Europe. The European Union Committee of Experts on Rare Diseases (EUCERD) (2010-2013) and the Commission Expert Group on Rare Diseases (2014-2016) have assisted the European Commission in preparing and implementing community activities in the rare disease field through a number of recommendations (see Annex 1).

In 2018, as a result of a Pilot Project adopted by the European Parliament, the European Commission (DG SANTE) issued a call for a 'Foresight Study' dedicated to planning the next ten years of rare disease policy. A consortium was established, led by EURORDIS, and commenced operations on 1st January 2019. The project will run until 31st December 2020, involves 8 partners, and has a total budget of over €2 million. The goal of the project is to employ innovative research-based methods –'foresight studies'- to guide and support future policy decisions in the field of rare diseases. Drivers of change and determinants of health for people with rare diseases -both 'traditional' i.e. well-known determinants and 'wild card' factors- will be identified and ranked, to develop policy scenarios for the years leading up to 2030. The partners will then use these scenarios to prepare recommendations to guide future policy by using the participatory foresight approach and additional innovative consensus-building methods, encouraging broad and sustainable uptake by patients, all relevant stakeholders, (in particular policy makers) and society at large.

The need for a Panel of Experts

The breadth of scope of this project is one of its major strengths and the source of significant added value; however, herein lies one of the greatest challenges. To enable a sweeping review of the status quo in European rare disease activities and policies, and to build on this to identify and rank drivers of change and determinants of health, it is necessary to consult as wide a body of stakeholders as possible. The partners proposed to create a Panel of Experts (PoE), to unite up to 250 stakeholders with expertise in various topics of relevance to the diagnosis, treatment, management and care of rare diseases.

Selection of members for the Panel of Experts

To achieve the ambitious aims of Rare2030, experts are invited to join this PoE and provide guidance and input to a range of activities across the two years of the project. In appointing members to this PoE, it is acknowledged that, given the breath of scope of the issues to be taken into consideration when identifying drivers of change and determinants of health for rare diseases, the number of individuals capable of adding value to the PoE far exceeds the maximum capacity of this body. Thus, it has been necessary to be selective in inviting members. Individuals have, on the whole, been approached in their capacity as representatives of broader bodies or 'categories'. **Annex II outlines these categories of stakeholders.**

Activities of the Panel of Experts

The role of the PoE members will move through four steps of the foresight process over the two years of the project:

- **Step 1 – Clarifying the Knowledge Base** - From its (expected) inception in M4 (April 2019), the PoE will be divided into a number of broad sub-groups, each organised around an area of expertise (e.g. Political & strategic frameworks relevant to RD; Basic, clinical and translational research; etc.). This will enable the Partners to organise more effective reviews of compiled documentation illustrating the status quo (as below), and to deliver more effective teleconferences.
- Between inception and approximately M9 (September 2019) the focus of these subgroups will be to review the status quo of rare disease-related activities in Europe: this status quo will be summarised for PoE members via a series of **knowledge base factsheets**.
- These knowledge base factsheets will be written in English and will primarily be based upon a) the outputs of a thorough literature review and b) the country-specific and cross-cutting (i.e. topic-oriented) data emerging from the updated resource on the 'State of the Art of rare disease activities in Europe'.

- **Step 2 – Horizon Scanning** - PoE members will meet several times via teleconference in each sub-group, and all PoE members will be invited to share their perspectives on possible drivers of change and determinants of health, based upon the aforementioned knowledge base factsheets and personal insights. (The knowledge base factsheets will contain links to wider repositories of publications and key grey-literature, for any PoE members wishing to delve deeper into each topic.)
    - It is important to stress that determinants of health and wellbeing and drivers of change will not be developed for *specific* conditions – the goal is to address rare and specialised conditions broadly, based upon the common challenges posed by rarity, in order to yield largely 'disease-agnostic' scenarios and recommendations at the end of the project. However, as the project advances it may be desirable to tailor recommendations at a slightly more granular level, to apply to (still very broad) *categories* of diseases, such as developmental vs functional vs degenerative disorders diseases.
    - Particular emphasis will be placed on 'blue-sky thinking', that is, on identifying 'new' drivers or determinants of health beyond the obvious scope of the particular subgroups
- In approximately M9 (September 2019), all PoE members will be invited to complete a survey designed to (preliminarily) rank a broad range of drivers of change and determinants of health and wellbeing: all PoE members will be able to provide their views on key trends identified by all sub-groups, including those in which they did not directly participate.
- In M11 (November 2019) all PoE members will assemble at a face-to-face event: the 'Rare 2030 Panel of Experts Conference'. Here, the members will participate to various activities designed to validate the most

important drivers of change and determinants of health, and begin to discuss how these might manifest into policy scenarios.

- **Stage 3 – Scenario Building** - Between M13 and M15 (January and March 2020), the PoE will be asked to refine a number of policy scenarios (developed based upon the drivers of change and determinants of health agreed in the first year of the project) through webinars, workshops or sessions at key rare disease events such as the European Conference on Rare Diseases.

- **Stage 4 – Back-casting and Policy Recommendations** - Coordinators and healthcare professionals of European Reference Networks and National level stakeholders will take part in a series of workshops between M16-22 (April-October 2020) to propose policies options at the EU and national levels that lead to the preferred futures identified in the project.

Roles and responsibilities of the Rare2030 Panel of Experts

Members of the PoE are expected to contribute to the work of the project in the following ways:

- Complete a short form to select up to 3 sub-groups in which they feel they have particular expertise
- Complete doodle polls when requested, to enable the organisation of teleconferences
- Read all correspondence disseminated to the PoE, either to the full group or (more often) to sub-groups
- Participate to teleconferences, either involving the full PoE or (as will more often be the case) teleconferences for the more thematically-organised sub-groups
- In preparation for teleconferences and face-to-face meetings, members should review all documents and preparatory materials circulated – fundamentally, this means the knowledge base factsheet documents for the subgroups to which any given member belongs (any additional resources and reports will be optional reading). This will support an understanding of the status quo across Europe, from which determinants of health and drivers of change may be identified
- Members should be prepared to contribute their perspectives and suggestions on all sub-group teleconferences
- In conveying their opinions, all PoE members will be respectful of the viewpoints of others and ensure criticism is constructive, to ensure an open and collaborative environment for debate in which each individual can feel respected and valued
- When requested, timely written feedback to documents is appreciated
- Members will be encouraged to respond to two key surveys of the Rare2030 project in a timely manner
- Members are expected to join the face-to-face "Rare 2030 Panel of Experts Conference' in M11 (November 2019). This will be held in a major European capital city. Travel and subsistence expenses will be covered by the Rare2030 project.
- In most cases, members are invited to join the PoE as a representative of a broader body (advisory group, council, board, etc.). Therefore, members are encouraged to strive to incorporate the views and perspectives of these broader entities when acting as their representatives (whether formally or informally). This should be a bi-directional communication i.e. the PoE members should bring the wider views of his/her stakeholder group (e.g. the ERN they are representation) but should equally strive to disseminate news of the Rare2030 project and its activities within that broader stakeholder group. For instance, this could involve organising internal discussions and debate on the most prominent issues.

Annex 1: Summary of EU-level Recommendations on rare diseases

(For more details, see the 2018 *'Overview Report on the State of the Art of Rare Disease Activities in Europe'*)

Consensus European Recommendations on Rare Disease Issues

Recommendation Title	Date Adopted	Link to Document
Quality Criteria for Centres of Expertise for Rare Diseases	October 2011	http://www.eucerd.eu/?post_type=document&p=1224
Improving Decisions based on the Clinical Added Value of Orphan Medicinal Products (CAVOMP) Information Flow	September 2012	http://www.eucerd.eu/?post_type=document&p=1446
European Reference Networks for Rare Diseases **Addendum to EUCERD Recommendations on Rare Disease ERNs**	January 2013 June 2015	http://www.eucerd.eu/?post_type=document&p=2207 https://ec.europa.eu/health/sites/health/files/rare_diseases/docs/20150610_erns_eucerdaddendum_en.pdf
Rare Disease Patient Registration and Data Collection	June 2013	http://www.eucerd.eu/wp-content/uploads/2013/06/EUCERD_Recommendations_RDRegistryDataCollection_adopted.pdf
Core Indicators for Rare Disease National Plans/Strategies	June 2013	http://www.eucerd.eu/wp-content/uploads/2013/06/EUCERD_Recommendations_Indicators_adopted.pdf
Potential Areas of European Collaboration in the field of New Born Screening (Opinion)	July 2013	http://www.eucerd.eu/wp-content/uploads/2013/07/EUCERD_NBS_Opinion_Adopted.pdf
Ways to Improve Codification of Rare Diseases	November 2014	http://ec.europa.eu/health/rare_diseases/docs/recommendation_coding_cegrd_en.pdf
Cross-Border Genetic Testing of Rare Diseases in the EU	November 2015	http://ec.europa.eu/health/rare_diseases/docs/2015_recommendation_crossbordergenetictesting_en.pdf
Support the Incorporation of Rare Diseases into Social Services and Policies	April 2016	http://ec.europa.eu/health/rare_diseases/docs/recommendations_socialservices_policies_en.pdf