RARE 2030

D2.7 Brief on Pilot of EU Health Policy Platform

December 2020

Report Information

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Project Information

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Brief on Pilot of EU Health Policy Platform

Technical Information

Official title: Stakeholder Network on Rare Diseases

Creation Task Force:
- Ana Rath – Orphanet
- Till Voigtlaender – BoMs Austria
- Valentina Bottarelli - EURORDIS
- Victoria Hedley – University of Newcastle
- Anna Kole - EURORDIS

Composition: Panel of Experts, former members of the Committee of Experts on Rare Diseases or their successors with an equivalent role in national administration, and newly interested stakeholders

Projected Launch Date: end of Rare 2030 Foresight Study

Background

The discontinuation of the European Commission Expert Group on Rare Diseases (CEG-RD, 2013-2016), previously EUCERD, and the end of *RD-ACTION,* the second EU Health Programme funded Joint Action for Rare Diseases (2015-2018), has raised concerns about weakened future EU action on rare diseases and a lost line of communication between stakeholders of the rare disease community and the EC in setting recommendations for rare disease policy at the EU level.

The launch and implementation of the European Reference Networks (ERNs) and the creation of the Board of Member States for ERNs or the ERN Coordinator Group, who support the enterprise with the European Commission, are important fora for the rare disease community. Yet, they cannot take over the mandate of the Expert Groups on RDs, as they do not represent the full range of stakeholders in the RD community nor have they a mandate to address the full range of topics and issues under the rare disease 'spectrum', nor should they, at the risk of jeopardising the achievement of their ERN-focused goals.
While the conceptual inclusion of rare diseases into the broader umbrella of the non-communicable diseases (NCDs) is still uncertain at the international level (WHO in particular), at the EU level it has become operational with the inclusion of RD in the scope of the Steering Group on Health Promotion, Disease Prevention and Management of Non-Communicable Diseases (SGPP) set up by the Commission in late 2016.

Currently The SGPP has selected an initial set of policy interventions that will be implemented by multiple EU countries. It is planned to periodically select the next priority areas for the implementation of best practices.

The SGPP is composed of high-ranking representatives of national Health Ministries. There is no permanent representation of other stakeholders. Interested parties in the NCD field are able to participate in discussions via the EU Health Policy Platform (HPP), where a number of discussion groups have already been created.

When RD-ACTION came to an end, a group of partners and members of the RD community undertook the analysis of resources and channels of participation available within the HPP. This has led to a number of face-to-face meetings, letters and email exchanges with representatives of the European Commission's Directorate General for Health and Food Safety, DG SANTE (see Annex 1 and 2), and eventually to the proposal to create a Stakeholder Network for Rare Diseases within the EU Health Policy Platform. Stakeholder Networks are one of the possible channels of participation and created in the HPP in response to a direct request from stakeholders, who are already approved members within the Health Policy Platform, wishing to use the Platform to share knowledge and information. However, opportunities to support the SGPP in its work may be identified in the future within the list of resources available on the Platform.

In parallel, one of the main objectives of the "Rare2030" project is to contribute and add value to EU policies formulated (or in the process of being so) in the field of rare diseases, building on the wealth of knowledge and recommendations brought forward by the RD-ACTION and RD Expert Groups in the last decades. In particular, the Rare2030 project, which will end its work by the end of March 2021, aims to inform EU (as well as national/local) policies in the field of RDs, currently being included in a broader basket of non-communicable diseases.

The EU Health Policy Platform, and specifically the creation of a Stakeholder Network for Rare Diseases, has been explored as an opportunity to encourage sustainability of the policy dialogue initiated in the Rare2030 project, after its end. This specific deliverable (D2.7) of the project presents a "Brief on pilot of EU Health Policy Platform as a dialogue to for rare disease policy". This document outlines the objectives, Terms of Reference, potential challenges and next steps for the Stakeholder Network for Rare Diseases.

Specific outcomes of the work that emerge as evidence-based best practices may be also submitted to the SGPP, in compliance with competences and procedural requirements, with the aim of potentially being taken up and adopted in EU countries.

Objectives

The purpose of the Stakeholder Network on RD is establish a platform for multi-stakeholder dialogue on the rare diseases policy framework, discuss and integrate recommendations and best practices, and eventually outline policy strategies and actions (at EU, national and local level, as appropriate) to improve health outcomes and quality of life of people living with a rare disease. EU Health Policy Platform will be explored as an opportunity to encourage sustainability of the policy dialogue initiated in the Rare 2030 project, after its end.

Main tasks:

- Exchange information on rare disease-related challenges and opportunities;

- Discuss the status quo of rare disease policy both in terms of regional, national, European and global frameworks for rare diseases (e.g. national plans and strategies, regional cross-border collaborations, etc.) and topic-specific activities;
- Identify and share best practices regarding rare disease areas such as diagnostics, treatment, holistic care and research;
- Promote awareness in the field of rare diseases and on rare diseases policy in particular, including relevant EU and national policy and legislation, recommendations and opinions, including those adopted by the Expert Groups on RDs (EUCERD, CEGRD);
- Promote discussion on subjects proposed as priorities;
- Issue opinions, statements, positions or other relevant documents;
- Create new opportunities for collaboration between stakeholders from different backgrounds (sometimes from outside of the field of rare diseases);
- Liaise and ensure synergies with key groups such as the Board of Member States of ERNs, the ERN Coordinators' Group, the SGPP, etc.;
- Encourage sustainability and implementation of recommendations put forward by the Rare 2030 project.

Progress To-Date

1. Familiarise ourselves with the SGPP and EU Health Policy Platform to identify opportunities to provide recommendations and exchange best practices amongst stakeholders, key opinion leaders and policy makers at the EU level on issues around RDs
2. Creation of Stakeholder Network for RD on the EU Health Policy Platform (HPP): below screenshot of the actual platform

STAKEHOLDER NETWORK *for*
Rare Diseases

The Stakeholder Network for Rare Diseases is a multi-stakeholder initiative aimed at facilitating dialogue on rare diseases policy. Its ultimate objective is to instigate the co-design and co-creation of impactful solutions for the challenges faced by people living with rare diseases in Europe by drafting and supporting the implementation of recommendations for policy strategies and actions at the European, national and local levels.

3. Setting up of a Creation taskforce to put together all contents of this Brief

Ana Rath, ORPHANET/INSERM


Valentina Bottarelli, EURORDIS


Anna Kole, EURORDIS


Till Voigtlander, ERN BoMs Austria


Victoria Hedley, Newcastle University

4. Drafting of the Terms of Reference (See Annex 3)
5. Appointment of moderators

Identified Challenges and Opportunities

Challenges

- Unlike the Commission Expert Group on Rare Diseases, the Stakeholder Network is not chaired by the European Commission and does not mandate the member states to work alongside stakeholders. Unlike joint actions on RD (EUCERD, RD-ACTION) Stakeholder Network members are not mandated by MS
- There is no financial support for its activities, including face-to-face exchanges.
- Specifically, on face-to-face exchange, despite the high value, inclusiveness and efficiency that can be offered by moving meetings and on-sight events online, previous projects have shown us that we may experience some disadvantages of not meeting face-to-face. Brainstorming and collaborating online to achieve milestones may be extremely challenging. In addition, stakeholders may feel less accountable online than they would face-to-face.
- Virtual tools to monitor progress in the field of rare disease policy are not available through this platform.
- Access to the EU Health Policy Platform is not user friendly. Login procedure is problematic and does not encourage newcomers to browse opportunities before going through the process of setting an account.

Opportunities

- Free, virtual platform providing publicly available information to all stakeholders and actors interested in the field of rare diseases.
- A key tool in the Sustainability Plan of Rare 2030.
- Opportunity to ensure continuity of all reflection in Rare 2030 including implementation of a number of recommendations coming out of the project particularly in best practices identified in:
    - D4.2 – Methodological paper governing the literature survey for horizon scanning
    - D5.1 – Rare 2030 Scenarios space and stories: a preliminary version of the Scenarios to be validated through the stakeholders workshop
    - D5.2 – Rare 2030 Scenarios – a stakeholders view: an outcome of the workshop(s)
- Opportunity for multi-stakeholder exchange on new policy opportunities identified in D6.2 *Brief describing new policy opportunities*.

Next Steps

1. Official launching of the Network after the Rare2030 Final Conference

2. Inviting Panel of Experts to join as part of the network
3. Defining roles within the Secretariat (divide the Secretariat roles)
4. Updating the Terms of Reference
5. Appointing Chair
6. Planning the 1st meeting of the Network

Annex 1

OPEN LETTER
ON THE FUTURE OF THE EU RARE DISEASE POLICY

Mr. Xavier Prats-Monné, Director General
Mr. Martin Seychell, Deputy Director General for Health
Dr. Andrzej Rys, Director for health systems, medical products and innovation
DG Health and Food Safety, European Commission
Mr. John Ryan, Deputy Director General for Health
c/c
Mr. Stefan Schreck, Head of Unit, Health Programme and Chronic Diseases
Mr. Tapani Piha, Head of Unit, Cross border healthcare, eHealth

Ms. Donata Meroni, Head of Unit, Health and Food safety, CHAFEA

Paris, 28th of June 2018

Subject: Open Letter from RD-ACTION on the future of the EU Rare Diseases policy

Dear Mr Prats-Monné, Mr. Seychell, Dr. Rys, Mr. Ryan,

We, partners of RD ACTION, the Joint Action for Rare Diseases are writing on behalf of the rare disease community to express our collective concerns about the future of EU action on rare diseases, and to set out our proposals for solutions for a renewed impetus for rare disease policy at EU level.

Over the last 20 years, ground-breaking legislation and policy in support of rare diseases and orphan medicines have been driven for the most part by the EU institutions, with a strong leadership of the European Commission, in recognition of the fact that rare diseases are an **area with high European added value for which the most effective strategies are cross-border and EU-wide**.

The EU Regulation on orphan medicinal products, the Council Recommendation on action in the field of rare diseases and the European Reference Networks (ERNs) demonstrate the added-value a European approach can bring to improving the lives of the people living with a rare disease in Europe. The European Commission also supported the creation of consultative, expert bodies like the EUCERD and the EC Expert Group on RD, and financed projects and Joint Actions in the area of rare diseases.

As a result, this is, in many ways, a **triumphant** moment for rare diseases, with the creation of the ERNs, and with the anticipated adoption of a European Joint Programme Co-Fund for Rare Diseases next year. Great strides have been taken towards the realisation of, for instance, the points set out in the 2009 Council Recommendation on an action on a field of Rare Diseases – not least the adoption of a national plan or strategy for rare diseases in 25 EU countries.

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However, important work still remains to be done. For instance, European-level support for the implementation and evaluation of national plans and strategies for rare diseases remains essential, while ***new* priorities emerge**. Moreover, remarkable technological and scientific advances of the past years unlocked the potential of diagnostic tools for rare diseases and present an exponential increase in novel technologies and therapies on the market.

These new opportunities embody hope for patients, but in parallel bring about concerns in terms of access and of sustainability for health budgets. Recent developments in IT and infrastructures are conducive to greater data generation and allow collection, processing and standardisation of large datasets that were not previously available. Strong political guidance and better cross-country cooperation is necessary to tap at this potential while addressing the ethical and legal matters that are generated.

This dynamic environment necessitates adequate policy responses and a solid policy drive. We are delighted that support is continued for Orphanet and coding for rare diseases remains a priority for the European Commission. Nevertheless, there is a feeling that 'rare diseases' as a policy area have reached a plateau and the European Commission is investing less in this area.

Our concerns are two-fold:

1) There is more to rare diseases than ERNs

European Reference Networks are an enormous step forward for the rare disease community, and a lot rides on their success. We are enthusiastic about the potential of ERNs to be truly transformative for European healthcare systems and ultimately for people living with rare diseases. However, although ERNs should be fully integrated to national pathways and networks of care for rare disease patients, a number of central rare disease activities, such as newborn screening portfolios, national population-based registries, coding, integrated and holistic care, access to diagnostic genetic testing and to orphan medicines and other therapies for rare diseases, cannot be solely addressed by ERNs. Other crucial actions, such as the need to generate high quality information on rare diseases and patient pathways, the necessity to better measure the burden of these conditions (both burden for individuals and socio-economic burden for health and social systems), the drive to continue to designate national Centres of Expertise (which are the units that concretely *fulfil* the roles defined for a Centre of Expertise) – these are areas in which the ERNs may play a role but cannot be the primary drivers of progress.

All of these activities that are often framed into a national plan or strategy need to be maintained, strengthened or revamped, with a renewed political stimulus that from the EU level shall reverberate at national level, as it happened one decade ago with the Council Recommendation on action in the field of rare diseases.

2) The end of a community of stakeholders built around joint EU work on rare diseases

At European level, rare disease policies have benefited greatly from the drive of multi-stakeholder expert committees, since the establishment in 2004 of the **Rare Disease Task Force**, which was followed by the **European Union Committee of Experts on Rare Diseases (EUCERD)** and, more recently, the **European Commission Expert Group on Rare Diseases (CEGRD)**. Since the latter was dismantled last year, there is no replacement body to fulfil this central advisory and steering role, nor are there plans to establish any such an entity from the European Commission side.

While this is a trend affecting most expert groups in the field of health policy, in the framework of an overall reconsideration of the European Commission's role in the health sphere, the question remains as to what will be created in their stead.

The Steering Group on Health Promotion, Disease Prevention and Management of Non-Communicable Diseases (or *Steering Group on Prevention and Promotion*, SGPP) does not seem equipped to replace former expert groups. Firstly, while the SGPP plans to involve experts in particular disease areas (such as rare diseases) as and when required, the Group is only composed of representatives of Member States. Unfortunately, the views of different society or stakeholder groups are no longer represented on a permanent basis and the wealth of knowledge and expertise of the experts from academia, industry, civil society and patient organisations- all members of the former expert groups- is now lost. The new tools that have been made available, notably the Health Policy Platform, cannot fill this gap as they cannot replace the face-to-face exchanges, the gruelling consensus-building sessions or the technical in-depth work accomplished by these groups, often times with the support of dedicated Joint Actions. This is especially regrettable in our community as **a multi-stakeholder approach has always been the key to successful European action in the fight for rare diseases**, and should be again experienced in this new era.

Additionally, the chosen approach of exchange of best practices developed in other disease areas that the SGPP will promote and lead is to be commended, as it will foster a better use of existing resources. However, it also generates concerns, especially for the rare disease community. There is a major risk that for rare conditions, a 'one-size-fits-all' approach simply will not work and the specificities of rare diseases will not be catered for.

Along these lines, it is not reasonable to expect either the **Board of Member States for ERNs** nor the ERN Coordinator Group to take over the mandate of the EUCERD / CEGRD. These two bodies are dedicated specifically and solely to ERNs, which do not have a mandate to address the full range of topics and issues under the rare disease 'spectrum', nor should they, at the risk of jeopardising the achievement of their goals. Additionally, the representation of stakeholders is not ensured therein, although individual experts are invited on an ad hoc basis.

It is also of concern that there are **no plans either for any future Joint Action in the field of rare diseases**. As argued in a previous letter from the RD-ACTION Coordinator (July 17 2017), EU Joint Actions are valuable instruments to strengthen this link between stakeholders, EU Member States and the European Commission and to give the necessary support to go from recommendations to their real-life implementation in Member States. The three Joint Actions for rare diseases succeeded in providing support to the Expert Groups and significantly contributed to the implementation of national plans and strategies for rare diseases and to the initiation of the first European Reference Networks.

In particular, the strength of the current joint action RD-ACTION lies on its multi-stakeholder nature and on the pan-European well-structured coverage through, but not limited to, the Orphanet network and EURORDIS. In the current ERN deployment phase, RD-ACTION is fostering their interaction with many entities and issues possessing a particular value and resonance for rare diseases, such as registries, coding, data-sharing, newborn screening, diagnostics, care guidelines, accessibility and availability of orphan medicinal products, and integrated/social care.

With the end of the current joint action in one month, the successful approach implemented with RD-ACTION, with its twin pillars of data collection and policy support, and integration with different stakeholders and initiatives, is sadly going to disappear, while it could have been an excellent model for support to rare disease policy in the ERN era.

The future of rare disease policy

In light of the above considerations, it is natural to wonder whether the collaboration to overcome difficulties relating to rare diseases at the EU level will continue to be driven by the same energy as it was over the last two decades.

It is vital not to 'dilute' the concept of 'rare diseases' too far and to maintain the shared identity engendered by this broad heading 'rare diseases', which gives visibility to all the thousands of conditions under that title, which would otherwise have no voice.

Our proposals

At a time when the achievements of past years of successful rare disease policy are coming to fruition and new challenges and opportunities arise, we **need to accelerate the momentum we have built over the last 20 years to ensure no single person living with a rare disease is left behind.**

We need a **strong drive from the EU Institutions. We call on the European Commission to reaffirm its leadership in an area where it is unanimously recognised that the EU added value is very high**, by means of:

- A **dedicated policy for rare diseases** that, while creating synergies with other policy areas, effectively addresses the specificities of rare diseases;
- An overarching approach that i**ntegrates rare disease policies within a successful, harmonious and consistent framework**;
- A **multi-stakeholder approach and collaborative strategies** that have proved key to the success of the actions of Europe in the fight for rare diseases;
- A rare disease community equipped with the **most appropriate tools for exchange and policy-making** for the years to come.

As **initial, concrete solutions**, we urge the European Commission to:

- Support at least the ***informal* dialogue between national representatives in charge of rare disease policy at national level** and relevant stakeholders representatives, by enabling adjacent meetings to ERN Board of Member States meetings: we, as a community of well-established rare diseases actors in each Member State, are committed to organise content-wise these events and bring around the table the necessary expertise to timely tackle rare disease-related issues that are constantly evolving. This 'RD Think Tank' will be open to invite members of other EC initiatives, and of the SGPP, each time it is needed.
- In the framework of and in compliance with the reorganisation of DG SANTE, maintain a **dedicated policy officer** who supervises all European Commission's initiatives that are relevant and have an impact on rare disease policy, and who attends the informal 'RD Think Tank' meetings we propose.

We would be delighted to meet with you to discuss this proposal further and devise additional concrete solutions. We trust that you will take into careful consideration our appeal and we thank you for your support to the rare disease community.

Yours faithfully,
RD-ACTION partners

(with the abstention of DIMDI and DGS France)

Annex 2

EUROPEAN COMMISSION
DIRECTORATE-GENERAL FOR HEALTH AND FOOD SAFETY

Director General

Brussels,
sante.dg/JFR/CG/Ares(2018)

Dear Ms Rath,

Subject: Open letter on the future of the EU rare disease policy

Thank you for your letter of 28 June 2018, which we have considered carefully. Let me begin by assuring you that rare diseases will continue to be a high priority area for the Commission. It is an issue where the clear added value at EU level is indisputable. In your letter you have already recognize the considerable progress and achievements made at EU and national level on rare diseases with respect to legislation, networking, supportive frameworks and research.

We consider that an essential element to reinforce in the next few years is the interface between the EU level development on rare diseases policies and the implementation of these policies at national level. We agree on the usefulness of a regular dialogue with stakeholders in this area, which could be kept informal and flexible. In this respect, we propose two complementary approaches.

First, to facilitate and structure discussion between EU level, national and regional organizations working on rare diseases, we propose to have a dialogue on how we could best use the Health Policy Platform to support the rare diseases policy. This could happen through a specific thematic group. While the decision on how to take advantage of the full potential of this tool rests in the hands of stakeholders, such a thematic group should always consider supporting the decision-making processes of the Member States and the Commission in pragmatic and operational ways.

Secondly, we propose to hold regular meetings, also with SANTE senior management and other Commission services (such as DG RTD and the Joint Research Centre) as necessary.

The deployment of European Reference Networks (ERNs) is at a crucial stage. We have taken a conscious decision to prioritize this work in the interests of patients, as well as to provide a concrete tool that is essential for taking forward other work in future, such as advancing research, establishing care pathways for all patients in the EU, and cooperation among the Member States.

We acknowledge that ERNs cannot address all aspects of a comprehensive rare disease policy. They are notwithstanding an essential element of the EU's rare diseases policy. The immediate priority is to consolidate them and ensure their sustainability using our limited resources. In our view, it is necessary to link the ERNs with the national rare disease plans and promote access to ERNs for all patients in the EU. The ERN Board of Member States

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will continue to provide a forum where strengthening and improving the implementation of ERNs is discussed. In particular, to ensure the integration of ERNs into national healthcare systems. The Board is for instance already dealing with issues such as the integration of the ERNs in the national rare disease plans as well as with the establishment of pathways for the referral of patients to the ERNs.

We also propose to use the Steering Group on Promotion, Prevention and Management of Non-Communicable Diseases to identify best practices and highest needs in rare diseases policies. The Steering Group consists of high-level officials in charge of the general health policy in Member States. In it, Member States discuss rare disease priorities and select best practices for implementation. It can also identify research needs and increase the coordination of policies and funding mechanisms in the context of the Multiannual Financial Framework.

The Commission will report regularly to the Steering Group on any relevant developments related to the ERN initiative.

In addition, preparatory work for decision-making by the Steering Group could be supported by a thematic group on rare diseases under the Health Policy Platform, as mentioned above. A dedicated call on rare diseases could also be launched under the Best Practice portal.

Our proposed approach uses the potential of existing tools and minimizes the risk of duplicating structures. We would be happy to discuss this approach further with you and how we could address your concerns.

The role of rare diseases research in the Horizon Europe Programme is under discussion by the co-legislators. In view of the latest developments in the multiannual financial framework, this is the appropriate time to have a specific discussion with stakeholders on how we could best utilize DG SANTE's co-chairing role in the Health cluster of Horizon Europe to advance research on rare diseases. We stress the need to advocate in favour of the proposal with Member States and the European Parliament.

At this stage, the Commission will work to ensure that the needs and concerns of the rare diseases community are fully integrated in the frameworks and support structures described above.

Many thanks for your commitment to rare disease policy.

Yours sincerely,

Xavier Prats Monné

Annex 3

[DRAFT] Terms of Reference of the Stakeholder Network on Rare Diseases

1. Title

A Stakeholder Network shall be established within the Health Policy Platform (HPP) of the European Commission with the title of: "*Stakeholder Network for Rare Diseases*", thereafter "Stakeholder Network for RD".

2. Vision of the Stakeholder Network on Rare Diseases

The vision of the Stakeholder Network for RD is to establish a platform for multi-stakeholder dialogue on the rare diseases policy framework, discuss and integrate recommendations and best practices, and eventually outline a policy strategy and actions (at EU, national and local level, as appropriate) to improve health outcomes and quality of life of people living with a rare disease.

3. Strategic Objectives

The Stakeholder Network for RD will aim to:
- be the dedicated forum for all rare disease stakeholders to enable exchanges of information, experiences and good practices;
- steer debate involving different stakeholders in the field of rare disease to collectively outline policy actions within a consistent framework;
- promote synergies and propose integration of national and European action in the field of rare diseases, across all relevant policy areas.

4. Specific Objectives

The Stakeholder Network for RD will:
- Exchange information on rare disease-related challenges and opportunities;
- Discuss the status quo of rare disease policy both in terms of regional, national, European and global frameworks for rare diseases (e.g. national plans and strategies, regional cross-border collaborations, etc.) and topic-specific activities;
- Identify and share best practices regarding rare disease diagnostics, treatment, and holistic care;
- Promote awareness in the field of rare diseases and on rare diseases policy in particular, including relevant EU and national policy and legislation, recommendations and opinions, including those adopted by the Expert Groups on RDs (EUCERD, CEGRD);
- Propose subjects that needs addressing as a priority and promote discussion on those subjects;

- Issue opinions, statements, positions or other relevant documents; integrate and update existing ones where necessary;
- Create new opportunities for collaboration between stakeholders from different backgrounds (sometimes from outside of the field of rare diseases);
- Liaise and ensure synergies with key groups such as the Board of Member States of ERNs, the ERN Coordinators' Group, the SGPP, etc.;
- Encourage sustainability and implementation of recommendations put forward by the Rare 2030 project.

5. Activities of the Stakeholder Network for RD

At the inception of its work, the Steering Committee of the Stakeholder Network for RD (see below, "Governance") will define its action plan for one year. Therein, a list of subjects or policy areas to be addressed by the Network as a priority will be identified. The latter will represent gaps not covered by the current Board of Member States for ERNs or existing Expert Groups

The action plan is proposed for one year by Steering Committee of the Stakeholder Network for RD and adopted by simple majority of the members.

Concretely, the following actions are available for the users of the IT platform, and could be performed by the Networks:

- Post news, updates or opinions on a health issue, with documents, pictures or links;
- Work collaboratively between stakeholders to produce joint statements;
- Start discussions;
- Create events;
- Create opinion polls;
- Upload or download documents to and from the library;
- Promote and share publications, documents or events.

The European Commission (DG SANTE) reports on the conclusions from the different discussions of the IT Platform annually, whilst also monitoring the activity and following-up on the results and outputs. An annual face to face meeting will be organised for all of the networks but participation is limited, therefore not all network participants will be able to attend.

6. Membership & composition

A number of stakeholders, from different groups, will be invited by the Steering Committee of the Stakeholder Network (as defined below, see "Governance") to join the Network. Invitations will be made so to ensure that the representation of interests is as balanced as possible. In particular, a selected number of members of the Panel of Experts of the Rare 2030 project will be invited to join the Network and/or former members of the former Expert Groups for Rare Diseases.

Additionally, spontaneous requests for membership will be accepted. The Network Moderators (see below, "Moderators") will be in charge of accepting or rejecting requests to join the

Network, based on a list of criteria for acceptance into the Network that will be established by the Steering Committee.

To be defined:

- *the specific criteria of selection of members;*
- *the term of office of members (how long? indefinite or renewable? define period)*

Also, what authority would perform the assessment needs to be ascertained.

A maximum size of the Network shall be determined that reflects membership that is active, balanced across stakeholders, and manageable for face to face meetings.

Where possible, representatives of the European Commission and Member State representatives shall be invited to become members of the Stakeholder Network for Rare Disease. However, the purpose of this Network shall not be to recreate an Expert Group, but rather to ensure the continuation of the dialogue amongst all relevant stakeholders within the RD community and guarantee the sustainability of policy measures and recommendations from the rare disease community (including future recommendations from the Rare 2030 project).

Defining meaningful interaction between the Board of Member States for ERNs and the European Commission with this Network will be crucial and shall be concretely defined by the Steering Committee, as one of their first actions once the Network has launched.

7. Commitments

The members of the Stakeholder Group shall commit to:

- Agree to these specific Terms of Reference;
- Attend a minimum of (*to be defined*) online calls each year;
- Attend at a minimum of (*to be defined*) meetings per year;
- Draft and/or contribute to documents relevant for the work of the network.

8. Governance

- A Steering Committee is established within the Stakeholder Network for RD. For the first year of operation, it will be composed of the members of the "Creation Task Force", i.e. the group of stakeholders that submitted the application for the Stakeholder Network for RD to the HPP Secretariat/DG SANTE.

- At the end of the first year, the members of the Network shall appoint the Steering Committee for a duration of two years. The Steering Committee, upon its appointment, will establish the maximum number of its members.

9. Chair

To be defined:

- *Who is chairing? How he/she is appointed? For how long?*
- *Will there be a Vice-chair or alternates?*
- The network shall be chaired by *(to be appointed)* or his/her alternate.

10. Moderators

- A minimum of two Moderators are appointed for the Stakeholder Network for RD, who will be in charge of the acceptance and rejection of access to the Network, based on a list of criteria for acceptance into the Network that will be established by the Steering Committee. In case of doubts, decision is submitted to and taken by the Steering Committee.

- The Moderators will also be responsible for creating the library structure of the Network and shall moderate its content. They shall keep the community informed and encourage members to participate in the discussions by commenting, downloading the material that will be uploaded to the library, and publishing information, if desired. They will also report any misbehavior.

11. Secretariat

- The secretariat shall draw up the agenda, send invitations to the meetings and relevant supporting documents to the members.

- Other tasks: draw up and disseminate minutes

12. Meetings

As for any other entity established within the HPP, the majority of exchanges of the Stakeholder Network for RD will be virtual. Face to face meetings will be possible. However, the Network will be required to fund its own meetings, which will most likely take place back-to-back with other rare disease meetings (e.g. meeting of the ERN Board of Member States) or EC events.

To be defined:

- *How many meetings per year?*

The views expressed during meetings by different stakeholders shall be recorded in the minutes and transmitted for approval to all the members of the Network. Once approved unanimously, they should be adopted and uploaded by moderators to the library.

13. Photograph/logo

The Stakeholder Network for Rare Diseases is a multi-stakeholder initiative aimed at facilitating dialogue on rare diseases policy. Its ultimate objective is to instigate the co-design and co-creation of impactful solutions for the challenges faced by people living with rare diseases in Europe by drafting and supporting the implementation of recommendations for policy strategies and actions at the European, national and local levels.

14. Action preceding the decision to create a Stakeholder Network for Rare Diseases

- End of European Commission Expert Group on RDs
- End of RD-ACTION
- RD-ACTION members' Letter to DG SANTE raising concerns about weakened future EU action on RDs and lost opportunities for setting recommendations for rare disease policy at the EU level.
- Application for Thematic Network
- Participation to the annual meeting of the Health Policy Platform
- Meeting with Deputy Director General Martin Seychell and Director Anne Bucher to explore opportunities for setting up a rare diseases stakeholder network within the framework of the EU Health Policy Platform.
- EURORDIS' meeting with relevant DG SANTE (Stefan Schreck) and CHAFEA (Donata Meroni, Georgios Margetidis) officers and Heads of Unit, as well as DG SANTE Director General Anne Bucher
- Meeting Nicoline Tamsma and Abigail with further guidance and working details of the procedure to set up a Stakeholder Group (Ana Rath and Sylvie Maiella)
- Internal calls and email exchanges of the "initiating task force" for a Stakeholder Network: Ana Rath, Victoria Hedley, Valentina Bottarelli, Till Voigtlaender