Promoting implementation of Recommendations on Policy, Information and Data for Rare Diseases: RD-ACTION.

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Purpose

Rare diseases (RD) have been identified as a paradigmatic field in which actions conducted at the European level constitute the adequate response to the specific problems encountered by stakeholders in the RD field. RD-ACTION (www.rd-action.eu) is a 3 years Joint Action (JA) co-funded by the EU Health Programme 2014-2020. It aims at ensuring an integrated European approach to the challenges faced by the RD community and at promoting, catalysing and triggering multi-stakeholder debates which are necessary both at European level to build shared strategies and at national level to support the integration of EU policy development across member states .

Methods

To gather specific expertise and to build shared strategies to address the specific issues of RD, the work has been organised into 6 sub-projects and the governance of the action is organised into three levels:

- a General assembly composed of one member per designated authority* which is the decision-making body of the consortium in charge of review and steer the project.
- An Executive committee composed by the 7 sub-project leaders in charge of the supervision , communication and cross-talk promotion
- Sub-project teams in charge of executing, monitoring and cross-talks.
- Workgroups established 'à la carte' according to the need identified

*Beneficiaries and European collaborating stakeholders have been designated by the national authorities as competent authorities for Joint Action participation.

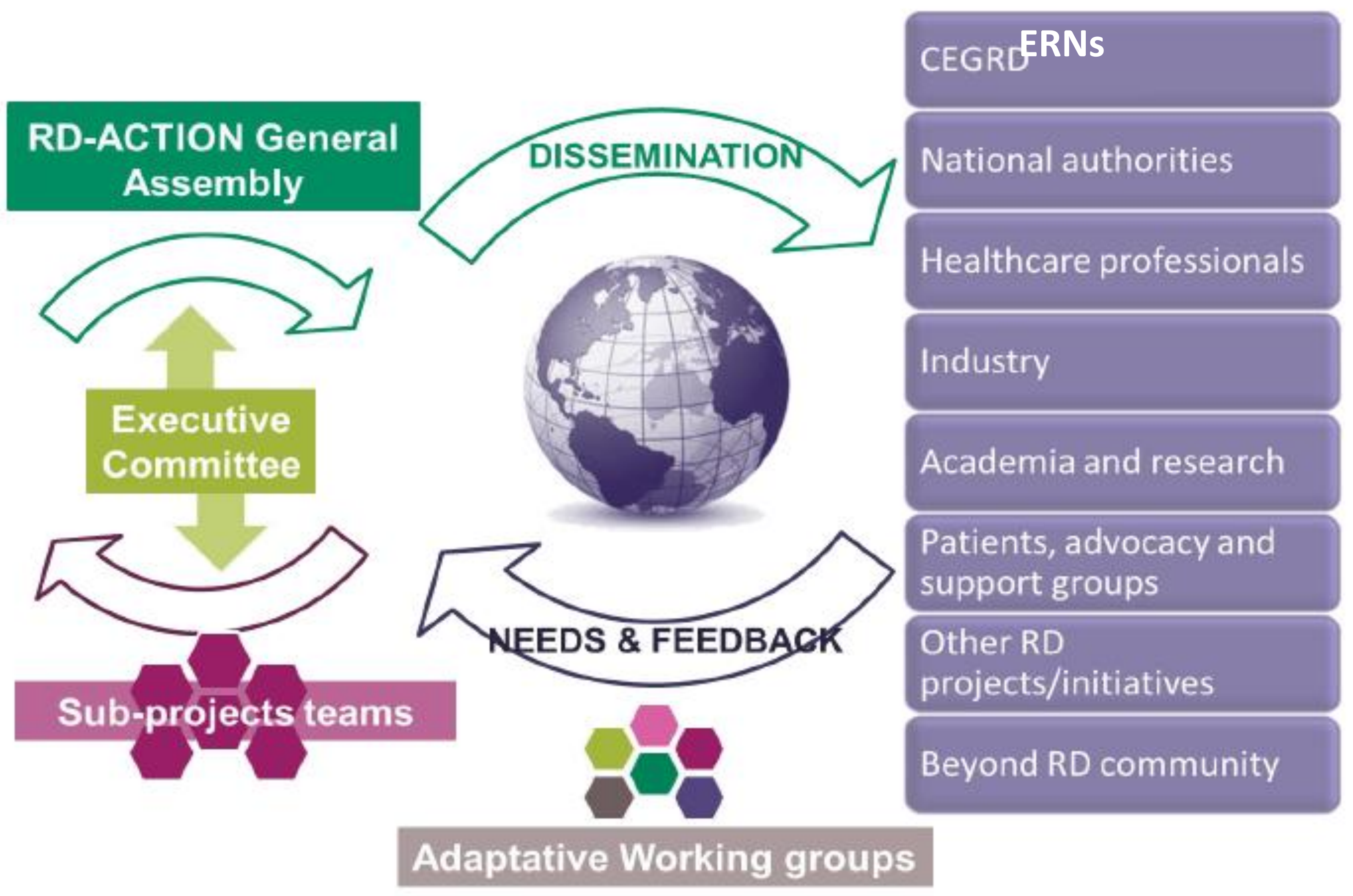
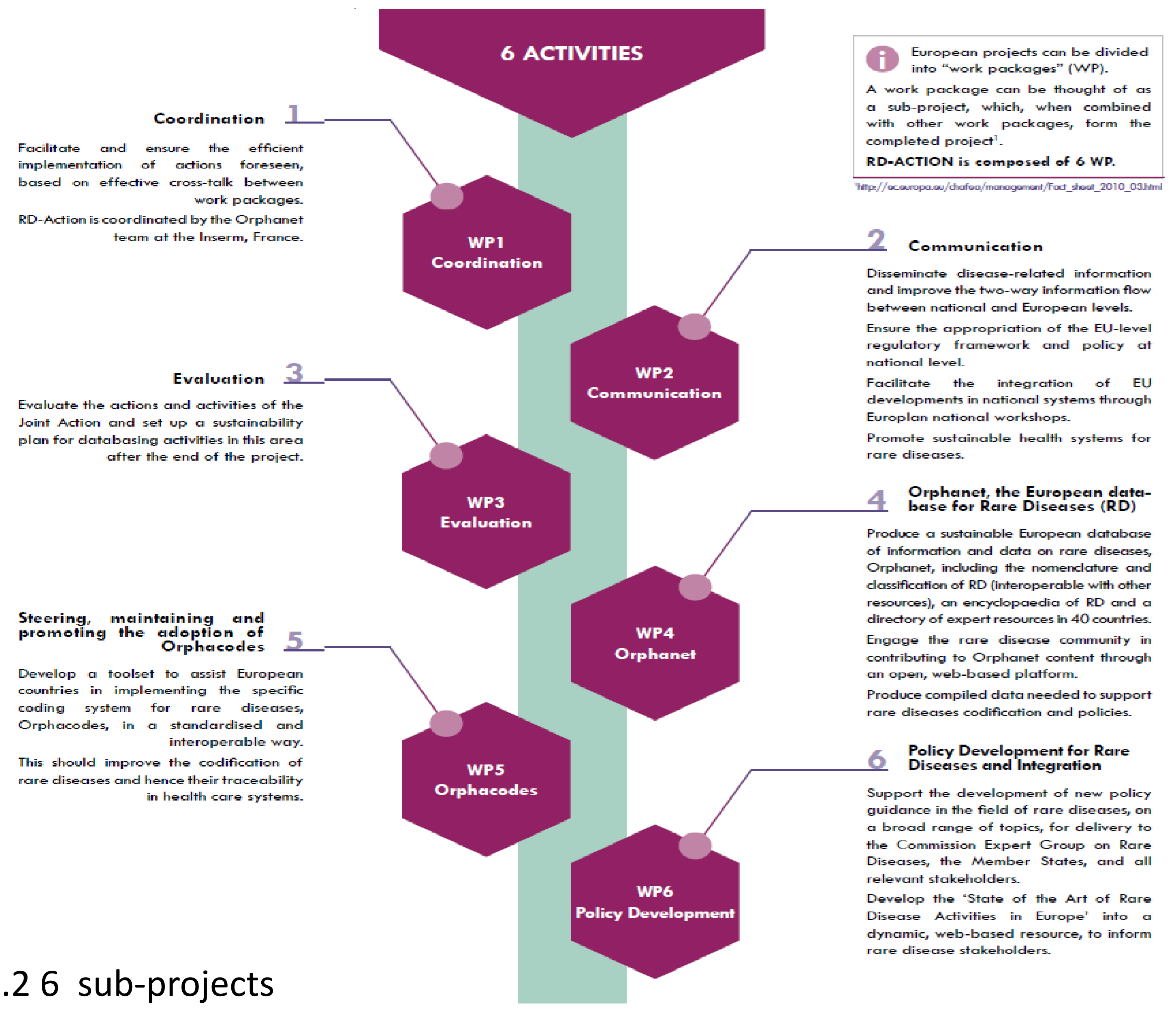


Fig.1 Method



European projects can be divided into "work packages" (WP).

A work package can be thought of as a sub-project, which, when combined with other work packages, form the completed project[1].

RD-ACTION is composed of 6 WP.

[1]http://ec.europa.eu/chafea/management/Fact_sheet_2010_03.html

1 Coordination

Facilitate and ensure the efficient implementation of actions foreseen, based on effective cross-talk between work packages.

RD-Action is coordinated by the Orphanet team at the Inserm, France.

2 Communication

Disseminate disease-related information and improve the two-way information flow between national and European levels.

Ensure the appropriation of the EU-level regulatory framework and policy at national level.

Facilitate the integration of EU developments in national systems through Europlan national workshops.

Promote sustainable health systems for rare diseases.

3 Evaluation

Evaluate the actions and activities of the Joint Action and set up a sustainability plan for databasing activities in this area after the end of the project.

4 Orphanet, the European database for Rare Diseases (RD)

Produce a sustainable European database of information and data on rare diseases, Orphanet, including the nomenclature and classification of RD (interoperable with other resources), an encyclopaedia of RD and a directory of expert resources in 40 countries.

Engage the rare disease community in contributing to Orphanet content through an open, web-based platform.

Produce compiled data needed to support rare diseases codification and policies.

5 Steering, maintaining and promoting the adoption of Orphacodes

Develop a toolset to assist European countries in implementing the specific coding system for rare diseases, Orphacodes, in a standardised and interoperable way.

This should improve the codification of rare diseases and hence their traceability in health care systems.

6 Policy Development for Rare Diseases and Integration

Support the development of new policy guidance in the field of rare diseases, on a broad range of topics, for delivery to the Commission Expert Group on Rare Diseases, the Member States, and all relevant stakeholders.

Develop the 'State of the Art of Rare Disease Activities in Europe' into a dynamic, web-based resource, to inform rare disease stakeholders.

Fig.2 6 sub-projects

MISSIONS

1

Support the development and sustainability of Orphanet

2

Contribute to solution to ensure codification of RD in health information systems across Europe

3

Work on priority issues for people living with RD by implementing the actions identified in the EU Council recommendation on an action in the field of RD

Ensure the sustainability of these actions and support the work of the CEGRD

4

Promote, catalyse and trigger multi-stakeholder debates around RD

Results

1 **Creation of the Orphanet Network**

30 Countries worldwide

86% Coverage of EU MS

de facto monopoly ***in line with Article 190 (1) (c) of Delegated Regulation (EU) No 1268/2012***

2 **Orphacodes implementation**

↑ 11 MS

↑ 3 MS Ongoing pilot testing

Since the beginning of the action

3 4 **Awareness and understanding dissemination**

2000+ Participants

14 EUROPLAN Conferences

4 Multi-stakeholder Workshops

ECRD 2016

4,600+ Users

www.rd-action.eu

16.000+ Subscribers

Orphanews

3.200.000+ Downloads

Orphanet Report Series

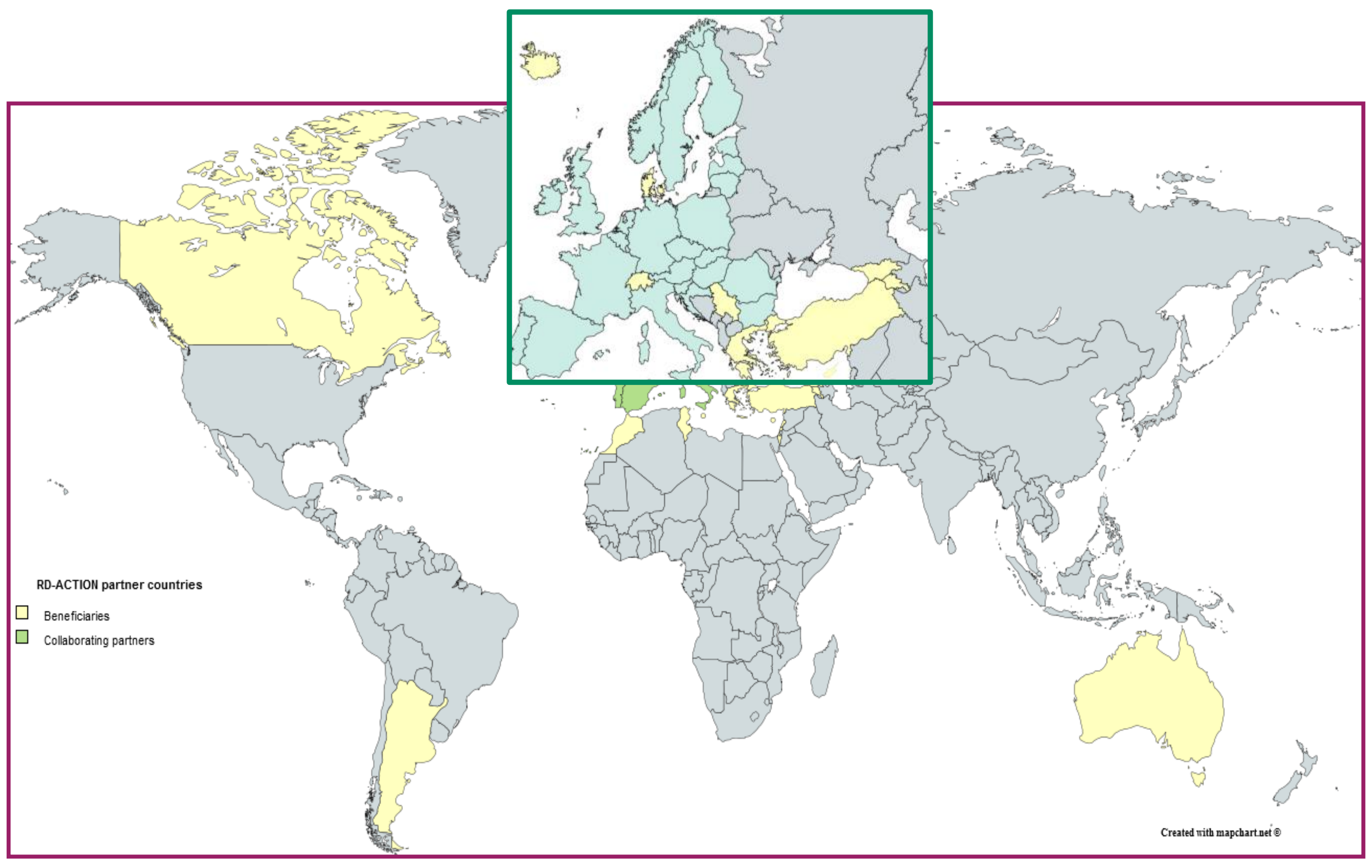


Fig.3 RD-ACTION consortium

Conclusions

RD-ACTION large geographical coverage and the wide array of competences represented gives the project a global, multistakeholder perspective. Moreover the three levels participative governance allows an effective and adaptive response to the RD community needs and allows cross-talks between internal and external projects resulting in a flexible approach which ensures that the products delivered are consistent, non redundant and instrumental to the transfer of European recommendations into national policies and in supporting the newly established European Reference Networks.

RD ACTION DATA AND POLICIES FOR RARE DISEASES

Co-funded by the Health Programme of the European Union

Inserm La science pour la santé From science to health

This poster is part of the project / joint action '677024 / RD-ACTION' which has received funding from the European Union's Health Programme (2014-2020).

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