EUROPEAN COMMISSION

DIRECTORATE-GENERAL FOR RESEARCH & INNOVATION

SP1-Cooperation

Coordination and support action

Coordination (or networking) actions

FP7-HEALTH-2012-INNOVATION-1

Grant Agreement Number 305690

RARE-Bestpractices

Platform for sharing best practices for management of rare diseases

HEALTH-F5-2012-305690

SEVENTH FRAMEWORK PROGRAMME

GRANT AGREEMENT No 305690

PROJECT TITLE RARE-Bestpractices

Coordination and support action

Coordination (or networking) actions

The **European Union** ("*the Union*"), represented by the **European Commission** (the "*Commission*"),

of the **one part**,

and ISTITUTO SUPERIORE DI SANITA, established in Viale Regina Elena 299, ROMA, 00161, Italy represented by ENRICO GARACI, PRESIDENT or his authorised representative, the *beneficiary* acting as "*coordinator*" of the *consortium* (the "*coordinator*"), ("*beneficiary no. 1*"),

of the **other part**

HAVE AGREED to the following terms and conditions including those in the following annexes, which form an integral part of this *grant agreement* (the "*grant agreement*").

Annex I - Description of Work
Annex II - General conditions
Annex III - Non applicable
Annex IV - Form A - Accession of *beneficiaries* to the *grant agreement*
Annex V - Form B - Request for accession of a new *beneficiary* to the *grant agreement*
Annex VI - Form C - Financial statement per funding scheme
Annex VII - Form D - Terms of reference for the certificate on the financial statements and Form E - Terms of reference for the certificate on the methodology

Article 1 - Accession to the *grant agreement* of the other *beneficiaries*

1. The *coordinator* shall endeavour to ensure that each legal entity identified below accedes to this *grant agreement* as a *beneficiary*, assuming the rights and obligations established by the *grant agreement* with effect from the date on which the *grant agreement* enters into force, by signing Form A in three originals, countersigned by the *coordinator*.

• **JAMARAU,** established in ST CHARLES SQUARE NORTH KENSINGTON 20 D, LONDON, W10 6EE, United Kingdom represented by Joanne Auld, Director or her authorised representative ("*beneficiary no. 2*"),

• **KAROLINSKA INSTITUTET,** established in Nobels Vag 5, STOCKHOLM, 17177, Sweden represented by Miles Davies, Head of Unit and/or Erik Forsse, Head of Office or their authorised representative ("*beneficiary no. 3*"),

• **HEALTHCARE IMPROVEMENT SCOTLAND,** established in HILLSIDE CRESCENT 8-10, EDINBURGH, EH7 5EA, United Kingdom represented by Sara Twaddle, Director of SIGN or her authorised representative ("*beneficiary no. 4*"),

• **LONDON SCHOOL OF ECONOMICS AND POLITICAL SCIENCE,** established in Houghton Street 1, LONDON, WC2A 2AE, United Kingdom represented by David Coombe, Director, Research Division and/or Jonathan Deer, Deputy Director, Research Division or their authorised representative ("*beneficiary no. 5*"),

• **CONSIGLIO NAZIONALE DELLE RICERCHE,** established in PIAZZALE ALDO MORO 7, ROMA, 00185, Italy represented by Sveva Avveduto, Director or her authorised representative ("*beneficiary no. 6*"),

• **EURORDIS - EUROPEAN ORGANISATION FOR RARE DISEASES ASSOCIATION,** established in RUE DIDOT 96, Paris, 75014, France represented by Yann Le Cam, Chief Executive Officer or his authorised representative ("*beneficiary no. 7*"),

• **Associazione per la Ricerca sulla Efficacia della Assistenza Sanitaria Centro Cochrane Italiano,** established in Via Fra Cristoforo 14/D, Milano, 20142, Italy represented by Vanna Pistotti, Vice-President and/or Graziella Filippini, or their authorised representative ("*beneficiary no. 8*"),

• **UNIVERSITAETSKLINIKUM FREIBURG,** established in HUGSTETTER STRASSE 55, FREIBURG, 79106, Germany represented by Annette Seitz-Fix, Head of Financial Department or her authorised representative ("*beneficiary no. 9*"),

• **BULGARIAN ASSOCIATION FOR PROMOTION OF EDUCATION AND SCIENCE,** established in BRATYA SVESHTAROVI STR 4, PLOVDID, 4017, Bulgaria represented by Rumen Stefanov, President or his authorised representative ("*beneficiary no. 10*"),

• **FUNDACION CANARIA DE INVESTIGACION Y SALUD,** established in BARRANCO DE LA BALLENA s/n , LAS PALMAS DE GRAN CANARIA, 35010, Spain represented by Maria Gomez, Legal Representative or her authorised representative ("*beneficiary no. 11*"),

• **UNIVERSITEIT MAASTRICHT,** established in Minderbroedersberg 4-6, MAASTRICHT, 6200 MD, Netherlands represented by Frans Ramaekers, Director GROW or his authorised representative ("*beneficiary no. 12*"),

• **UNIVERSITY OF NEWCASTLE UPON TYNE,** established in Kensington Terrace 6, NEWCASTLE UPON TYNE, NE1 7RU, United Kingdom represented by Amanda Tortice, Head, Joint Research Office and/or Douglas Robertson, Director, Research & Enterprise Services or their authorised representative ("*beneficiary no. 13*"),

• **EUROPEAN ACADEMY OF PAEDIATRICS AISBL,** established in AVENUE DE LA COURONNE 20, BRUXELLES, 1050, Belgium represented by Jose Ramet, Secretary-general and/or Elizabeth Siderius, Coordinator Working Group Rare Diseases or their authorised representative ("*beneficiary no. 14*"),

• **INSTITUTO DE SALUD CARLOS III,** established in CALLE SINESIO DELGADO 4-6, MADRID, 28029, Spain represented by JOAQUIN R ARENAS, Director or his authorised representative ("*beneficiary no. 15*"),

All the *beneficiaries* together form the *consortium* (the "*consortium*").

2. The *coordinator* shall send to the *Commission* one duly completed and signed Form A per *beneficiary* at the latest 45 calendar days after the entry into force of the *grant agreement*. The two remaining signed originals shall be kept, one by the *coordinator* to be made available for consultation at the request of any *beneficiary*, and the other by the *beneficiary* concerned.

3. Should any legal entity identified above, fail or refuse to accede to the *grant agreement* within the deadline established in the previous paragraph, the *Commission* is no longer bound by its offer to the said legal entity(ies). The *consortium* may propose to the *Commission*, within the time-limit to be fixed by the latter, appropriate solutions to ensure the implementation of the *project*. The procedure established in Annex II for amendments to this *grant agreement* will apply.

Article 2 - Scope

The Union has decided to grant a financial contribution for the implementation of the *project* as specified in Annex I, called *Platform for sharing best practices for management of rare diseases (RARE-Bestpractices)* (the "*project*") within the framework of the *SP1-Cooperation* and under the conditions laid down in this *grant agreement*.

Article 3 - Duration and *start date* of the *project*

The duration of the *project* shall be 48 months from 1st January 2013 (hereinafter referred to as the "*start date*").

Article 4 - Reporting periods and language of reports

The *project* is divided into reporting periods of the following duration:

- P1: from month 1 to month 18
- P2: from month 19 to month 36
- P3: from month 37 to the last month of the *project*.

Any report and deliverable, when appropriate, required by this *grant agreement* shall be in *English*.

Article 5 - Maximum financial contribution of *the Union*

1. The maximum financial contribution of *the Union* to the *project* shall be EUR 2,000,000.00 (*two million EURO*). The actual financial contribution of *the Union* shall be calculated in accordance with the provisions of this *grant agreement*.

2. Details of the financial contribution of *the Union* are contained in Annex I to this *grant agreement* which includes:

- a table of the estimated breakdown of budget and financial contribution of *the Union* per activity to be carried out by each of the *beneficiaries* under the *project*. *Beneficiaries* are allowed to transfer budget between different activities and between themselves in so far as the work is carried out as foreseen in Annex I.

3. The bank account of the *coordinator* to which all payments of the financial contribution of *the Union* shall be made is:

Name of account holder: ISTITUTO SUPERIORE DI SANITÀ
Name of bank: BANCA D'ITALIA - TESORIA CENTRALE DELLO STATO
Account reference: IT65U0100003245350200022349

Article 6 - Pre-financing

A *pre-financing* of EUR 866,666.00 (*eight hundred and sixty six thousand six hundred and sixty six EURO*) shall be paid to the *coordinator* within 45 days following the date of entry into force of this *grant agreement*. The *coordinator* shall distribute the *pre-financing* only to the *beneficiaries* who have acceded to the *grant agreement* and after the minimum number of *beneficiaries* required by the *Rules for Participation* as detailed in the call for proposals to which the *project* is related, have acceded to the *grant agreement*.

Beneficiaries hereby agree that the amount of EUR *100,000.00* (*one hundred thousand EURO*), corresponding to the *beneficiaries'* contribution to the Guarantee Fund referred to in Article II.20 and representing 5% of the maximum financial contribution of *the Union* referred to in Article 5.1, is transferred in their name by the *Commission* from the *pre-financing* into the Guarantee Fund. However, *beneficiaries* are deemed to have received the full *pre-financing* referred to in the first indent and will have to justify it in accordance with the *grant agreement*.

Article 7 - Special clauses

The following special clauses apply to this *grant agreement*:

Special clause 6

Notwithstanding the provisions of Article 6 the *pre-financing* shall be paid not earlier than 45 days before the *start date* of the *project*.

Special clause 15

1. The *beneficiary(ies)* shall provide the *Commission* with a written confirmation that it has received (a) favourable opinion(s) of the relevant ethics committee(s) and, if applicable, the regulatory approval(s) of the competent national or local authority(ies) in the country in which the research is to be carried out before beginning any *Commission* approved research requiring such opinions or approvals. The copy of the official approval from the relevant national or local ethics committees must also be provided to the *Commission*.

Special clause 39

In addition to Article II.30.4, *beneficiaries* shall deposit an electronic copy of the published version or the final manuscript accepted for publication of a scientific publication relating to *foreground* published before or after the final report in an institutional or subject-based repository at the moment of publication.

Beneficiaries are required to make their best efforts to ensure that this electronic copy becomes freely and electronically available to anyone through this repository:

- immediately if the scientific publication is published "open access", i.e. if an electronic version is also available free of charge via the publisher, or
- within 6 months of publication.

Article 8 - Communication

1. Any communication or request concerning the *grant agreement* shall identify the *grant agreement* number, the nature and details of the request or communication and be submitted to the following addresses:

For the *Commission*: European Commission
Directorate-General for Research & Innovation
F5
B-1049 Brussels, Belgium

For the *coordinator*: Dr. Rosa Maria Martoccia
ISTITUTO SUPERIORE DI SANITA
Viale Regina Elena 299
ROMA 00161
ITALY

2. For information or documents to be transferred by electronic means, the following addresses shall be used:

For the *Commission*: RTD-FP7-HEALTH-PERSONALISED-MEDICINE@ec.europa.eu

For the *coordinator*: dirgensa@iss.it

3. In case of refusal of the notification or absence of the recipient, the *beneficiary* or the *consortium*, as the case may be, is deemed to have been notified on the date of the latest delivery, if notification to the *coordinator* has been sent to one of the addresses mentioned in paragraphs 1 and 2 and to their legal representative. Other *beneficiaries* are deemed to have been notified if notification has been sent to the address mentioned in Article 1.1.

4. Any communication or request relating to the processing of personal data (Article II.13) shall be submitted, using the address(es) for the *Commission* identified in paragraphs 1 and 2, to the Controller responsible for the processing: Head of Unit of F5.

Article 9 - Applicable law and competent court

The financial contribution of *the Union* is a contribution from *the Union* research budget with the aim to implement the 7th Research Framework Programme (FP7) and it is incumbent on the Commission to execute FP7. Accordingly, this *grant agreement* shall be governed by the terms of this *grant agreement*, the European Community and European Union acts related to FP7, the Financial Regulation applicable to the general budget and its implementing rules and other European Community and European Union law and, on a subsidiary basis, by the law of Belgium.

Furthermore the *beneficiary* is aware and agrees that the Commission may take a decision to impose pecuniary obligations, which shall be enforceable in accordance with Article 299 of the Treaty on the Functioning of the European Union and Articles 164 and 192 of the Treaty establishing the *European Atomic Energy Community*.

Notwithstanding the *Commission's* right to directly adopt the recovery decisions referred to in the previous paragraph, the General Court, or on appeal, the Court of Justice of the European Union, shall have sole jurisdiction to hear any dispute between *the Union* and any *beneficiary* concerning the interpretation, application or validity of this *grant agreement* and the validity of the decision mentioned in the second paragraph.

Article 10 - Application of the *grant agreement* provisions

Any provision of this part of the *grant agreement*, shall take precedence over the provisions of any of the Annexes. The provisions of Annex III shall take precedence over the provisions of Annex II, and both shall take precedence over the provisions of Annex I.

The special clauses set out in Article 7 shall take precedence over any other provisions of this *grant agreement*.

Article 11 - Entry into force of the *grant agreement*

This *grant agreement* shall enter into force after its signature by the coordinator and the *Commission*, on the day of the last signature.

Done in two originals in English.

For the *coordinator* done at ROMA

ISTITUTO SUPERIORE DI SANITA'

Name of the legal entity

PROF. ENRICO GARACI

Name of the legal representative

ISTITUTO SUPERIORE DI SANITÀ

Stamp of the organisation (if applicable)

Signature of legal representative

9.10.2012

Date

For the *Commission* done at Brussels

Ruxandra DRAGHIA-AKLI
Director

Name of the legal representative

Signature of legal representative

Date 23 OCT. 2012

EUROPEAN COMMISSION
Research and Innovation Directorate-General

FP7 GRANT AGREEMENT

ANNEX IV – FORM A – ACCESSION OF BENEFICIARIES TO THE GRANT AGREEMENT

JAMARAU, represented for the purpose hereof by Joanne Auld, Director, or her authorised representative, established in WILLOW WREN WHARF HAYES ROAD SOUTHALL 'RUMIAN', MIDDLESEX, UB2 5HB, United Kingdom acting as its legal authorised representative, hereby consents to become a *beneficiary* (*"beneficiary no. 2"*) *to grant agreement* N° 305690 (relating to *project "Platform for sharing best practices for management of rare diseases"*) concluded between the European Commission and ISTITUTO SUPERIORE DI SANITA established in Viale Regina Elena 299, ROMA, 00161, Italy and accepts in accordance with the provisions of the aforementioned *grant agreement* all the rights and obligations of a *beneficiary*.

Done in 3 copies, of which one shall be kept by the *coordinator* and one by **JAMARAU**, the third being sent to the *Commission* by the coordinator in accordance with Articles 1.1 and 1.2 and Article 8 of the grant agreement.

JAMARAU	ISTITUTO SUPERIORE DI SANITA
Joanne Auld	PROF. ENRICO GARACI
Name of legal representative(s)	Name of legal representative(s)
Signature of legal representative(s)	Signature of legal representative(s)
24/11/2012	13/12/2012
Date	Date
Stamp of the organisation	Stamp of the organisation

jamarau
Administration Unification

FP7 GRANT AGREEMENT

ANNEX IV - FORM A - ACCESSION OF BENEFICIARIES TO THE GRANT AGREEMENT

KAROLINSKA INSTITUTET, represented for the purpose hereof by Miles Davies, Head of Unit, and/or Erik Forsse, Head of Office, or her/his/their authorised representative, established in Nobels Vag 5, STOCKHOLM, 17177, Sweden acting as its legal authorised representative, hereby consents to become a *beneficiary* ("*beneficiary no. 3*") to *grant agreement* N° 305690 (relating to *project* "*Platform for sharing best practices for management of rare diseases*") concluded between the European Commission and ISTITUTO SUPERIORE DI SANITA established in Viale Regina Elena 299, ROMA, 00161, Italy and accepts in accordance with the provisions of the aforementioned *grant agreement* all the rights and obligations of a *beneficiary*.

Done in 3 copies, of which one shall be kept by the *coordinator* and one by **KAROLINSKA INSTITUTET**, the third being sent to the *Commission* by the coordinator in accordance with Articles 1.1 and 1.2 and Article 8 of the grant agreement.

KAROLINSKA INSTITUTET

Erik Forsse

Name of legal representative(s)

Erik Forsse

Signature of legal representative(s)

13/4/2012

Date

KAROLINSKA INSTITUTET ANNO 1810 Karolinska Institutet

Stamp of the organisation

ISTITUTO SUPERIORE DI SANITA

PROF. ENRICO GARACI

Name of legal representative(s)

Signature of legal representative(s)

06/12/2012

Date

ISTITUTO SUPERIORE DI SANITA

Stamp of the organisation

FP7 GRANT AGREEMENT

ANNEX IV - FORM A - ACCESSION OF BENEFICIARIES TO THE GRANT AGREEMENT

HEALTHCARE IMPROVEMENT SCOTLAND, represented for the purpose hereof by Sara Twaddle, Director of SIGN, or her authorised representative, established in HILLSIDE CRESCENT 8-10, EDINBURGH, EH7 5EA, United Kingdom acting as its legal authorised representative, hereby consents to become a *beneficiary* (*"beneficiary no. 4"*) to *grant agreement* N° 305690 (relating to *project "Platform for sharing best practices for management of rare diseases"*) concluded between the European Commission and ISTITUTO SUPERIORE DI SANITA established in Viale Regina Elena 299, ROMA, 00161, Italy and accepts in accordance with the provisions of the aforementioned *grant agreement* all the rights and obligations of a *beneficiary*.

Done in 3 copies, of which one shall be kept by the *coordinator* and one by **HEALTHCARE IMPROVEMENT SCOTLAND**, the third being sent to the *Commission* by the coordinator in accordance with Articles 1.1 and 1.2 and Article 8 of the grant agreement.

HEALTHCARE IMPROVEMENT SCOTLAND	ISTITUTO SUPERIORE DI SANITA
SARA TWADDLE	PROF. ENRICO GARACI
Name of legal representative(s)	Name of legal representative(s)
Signature of legal representative(s)	Signature of legal representative(s)
8 November 2012	06/12/2012
Date	Date
Stamp of the organisation	Stamp of the organisation

ISTITUTO SUPERIORE DI SANITA

Received
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08 NOV 2012

FP7 GRANT AGREEMENT

ANNEX IV - FORM A - ACCESSION OF BENEFICIARIES TO THE GRANT AGREEMENT

LONDON SCHOOL OF ECONOMICS AND POLITICAL SCIENCE, represented for the purpose hereof by David Coombe, Director, Research Division, and or Jonathan Deer, Deputy Director, Research Division, or her/his/their authorised representative, established in Houghton Street 1, LONDON, WC2A 2AE, United Kingdom acting as its legal authorised representative, hereby consents to become a *beneficiary* ("*beneficiary no. 5*") to *grant agreement* N° 305690 (relating to *project* "*Platform for sharing best practices for management of rare diseases*") concluded between the European Commission and ISTITUTO SUPERIORE DI SANITA established in Viale Regina Elena 299, ROMA, 00161, Italy and accepts in accordance with the provisions of the aforementioned *grant agreement* all the rights and obligations of a *beneficiary*.

Done in 3 copies, of which one shall be kept by the *coordinator* and one by **LONDON SCHOOL OF ECONOMICS AND POLITICAL SCIENCE**, the third being sent to the *Commission* by the coordinator in accordance with Articles 1.1 and 1.2 and Article 8 of the grant agreement.

LONDON SCHOOL OF ECONOMICS AND POLITICAL SCIENCE

DAVID COOMBE
Name of legal representative(s)

Signature of legal representative(s)

23/X/2012
Date

The London School of Economics
and Political Science
Houghton St, Aldwych, London WC2A 2AE
Stamp of the organisation

ISTITUTO SUPERIORE DI SANITA

PROF. ENRICO GARACI
Name of legal representative(s)

Signature of legal representative(s)

06/12/2012
Date

ISTITUTO SUPERIORE DI SANITA

Stamp of the organisation

FP7 GRANT AGREEMENT

ANNEX IV - FORM A - ACCESSION OF BENEFICIARIES TO THE GRANT AGREEMENT

CONSIGLIO NAZIONALE DELLE RICERCHE, represented for the purpose hereof by Sveva Avveduto, Director, or her authorised representative, established in PIAZZALE ALDO MORO 7, ROMA, 00185, Italy acting as its legal authorised representative, hereby consents to become a *beneficiary* ("*beneficiary no. 6*") to *grant agreement* N° 305690 (relating to *project* "*Platform for sharing best practices for management of rare diseases*") concluded between the European Commission and ISTITUTO SUPERIORE DI SANITA established in Viale Regina Elena 299, ROMA, 00161, Italy and accepts in accordance with the provisions of the aforementioned *grant agreement* all the rights and obligations of a *beneficiary*.

Done in 3 copies, of which one shall be kept by the *coordinator* and one by **CONSIGLIO NAZIONALE DELLE RICERCHE**, the third being sent to the *Commission* by the coordinator in accordance with Articles 1.1 and 1.2 and Article 8 of the grant agreement.

CONSIGLIO NAZIONALE DELLE RICERCHE

IL DIRETTORE
(Dott.ssa Sveva Avveduto)

Name of legal representative(s)

Signature of legal representative(s)

16/11/2012

Date

Stamp of the organisation

ISTITUTO SUPERIORE DI SANITA

PROF. ENRICO GARACI

Name of legal representative(s)

Signature of legal representative(s)

06/12/2012

Date

ISTITUTO SUPERIORE DI SANITA

Stamp of the organisation

FP7 GRANT AGREEMENT

ANNEX IV - FORM A - ACCESSION OF BENEFICIARIES TO THE GRANT AGREEMENT

EURORDIS - EUROPEAN ORGANISATION FOR RARE DISEASES ASSOCIATION, represented for the purpose hereof by Yann Le Cam, Chief Executive Officer, or his authorised representative, established in RUE DIDOT 96, Paris, 75014, France acting as its legal authorised representative, hereby consents to become a *beneficiary* ("*beneficiary no. 7*") to *grant agreement* N° 305690 (relating to *project "Platform for sharing best practices for management of rare diseases"*) concluded between the European Commission and ISTITUTO SUPERIORE DI SANITA established in Viale Regina Elena 299, ROMA, 00161, Italy and accepts in accordance with the provisions of the aforementioned *grant agreement* all the rights and obligations of a *beneficiary*.

Done in 3 copies, of which one shall be kept by the *coordinator* and one by **EURORDIS - EUROPEAN ORGANISATION FOR RARE DISEASES ASSOCIATION**, the third being sent to the *Commission* by the coordinator in accordance with Articles 1.1 and 1.2 and Article 8 of the grant agreement.

EURORDIS - EUROPEAN ORGANISATION FOR RARE DISEASES ASSOCIATION

Mr. Yann LE CAM
Name of legal representative(s)

Signature of legal representative(s)

09 November 2012
Date

Stamp of the organisation

ISTITUTO SUPERIORE DI SANITA

PROF. ENRICO GARACI
Name of legal representative(s)

Signature of legal representative(s)

06/12/2012
Date

ISTITUTO SUPERIORE DI SANITA

Stamp of the organisation

FP7 GRANT AGREEMENT

ANNEX IV - FORM A - ACCESSION OF BENEFICIARIES TO THE GRANT AGREEMENT

Associazione per la Ricerca sulla Efficacia della Assistenza Sanitaria Centro Cochrane Italiano, represented for the purpose hereof by Vanna Pistotti, Vice-President, and/or Graziella Filippini, or her/his/their authorised representative, established in Via Fra Cristoforo 14/D, Milano, 20142, Italy acting as its legal authorised representative, hereby consents to become a *beneficiary* (*"beneficiary no. 8"*) to *grant agreement* N° 305690 (relating to *project "Platform for sharing best practices for management of rare diseases"*) concluded between the European Commission and ISTITUTO SUPERIORE DI SANITA established in Viale Regina Elena 299, ROMA, 00161, Italy and accepts in accordance with the provisions of the aforementioned *grant agreement* all the rights and obligations of a *beneficiary*.

Done in 3 copies, of which one shall be kept by the *coordinator* and one by **Associazione per la Ricerca sulla Efficacia della Assistenza Sanitaria Centro Cochrane Italiano**, the third being sent to the *Commission* by the coordinator in accordance with Articles 1.1 and 1.2 and Article 8 of the grant agreement.

Associazione per la Ricerca sulla Efficacia della Assistenza Sanitaria Centro Cochrane Italiano	ISTITUTO SUPERIORE DI SANITA
GRAZIELLA FILIPPINI Name of legal representative(s)	PROF. ENRICO GARACI Name of legal representative(s)
Graziella Filippini Signature of legal representative(s)	 Signature of legal representative(s)
15 November 2012 Date	06/12/2012 Date
Stamp of the organisation	Stamp of the organisation

ISTITUTO SUPERIORE DI SANITA

FP7 GRANT AGREEMENT

ANNEX IV - FORM A - ACCESSION OF BENEFICIARIES TO THE GRANT AGREEMENT

UNIVERSITAETSKLINIKUM FREIBURG, represented for the purpose hereof by Annette Seitz-Fix, Head of Financial Department, or her authorised representative, established in HUGSTETTER STRASSE 55, FREIBURG, 79106, Germany acting as its legal authorised representative, hereby consents to become a *beneficiary* ("*beneficiary no. 9*") to *grant agreement* N° 305690 (relating to *project* "*Platform for sharing best practices for management of rare diseases*") concluded between the European Commission and ISTITUTO SUPERIORE DI SANITA established in Viale Regina Elena 299, ROMA, 00161, Italy and accepts in accordance with the provisions of the aforementioned *grant agreement* all the rights and obligations of a *beneficiary*.

Done in 3 copies, of which one shall be kept by the *coordinator* and one by **UNIVERSITAETSKLINIKUM FREIBURG**, the third being sent to the *Commission* by the coordinator in accordance with Articles 1.1 and 1.2 and Article 8 of the grant agreement.

UNIVERSITAETSKLINIKUM FREIBURG

A. Seitz

Name of legal representative(s)

Signature of legal representative(s)

13. Nov. 2012

Date

Stamp of the organisation

K★27

ISTITUTO SUPERIORE DI SANITA

PROF. ENRICO GARACI

Name of legal representative(s)

Signature of legal representative(s)

06/12/2012

Date

Stamp of the organisation

FP7 GRANT AGREEMENT

ANNEX IV - FORM A - ACCESSION OF BENEFICIARIES TO THE GRANT AGREEMENT

BULGARIAN ASSOCIATION FOR PROMOTION OF EDUCATION AND SCIENCE, represented for the purpose hereof by Rumen Stefanov, President, or his authorised representative, established in BRATYA SVESHTAROVI STR 4, PLOVDID, 4017, Bulgaria acting as its legal authorised representative, hereby consents to become a *beneficiary* (*"beneficiary no. 10"*) to *grant agreement* N° 305690 (relating to *project "Platform for sharing best practices for management of rare diseases"*) concluded between the European Commission and ISTITUTO SUPERIORE DI SANITA established in Viale Regina Elena 299, ROMA, 00161, Italy and accepts in accordance with the provisions of the aforementioned *grant agreement* all the rights and obligations of a *beneficiary*.

Done in 3 copies, of which one shall be kept by the *coordinator* and one by **BULGARIAN ASSOCIATION FOR PROMOTION OF EDUCATION AND SCIENCE**, the third being sent to the *Commission* by the coordinator in accordance with Articles 1.1 and 1.2 and Article 8 of the grant agreement.

BULGARIAN ASSOCIATION FOR PROMOTION OF EDUCATION AND SCIENCE

PROF RUMEN STEFANOV

Name of legal representative(s)

Signature of legal representative(s)

08 / 11 / 2012

Date

Stamp of the organisation

ISTITUTO SUPERIORE DI SANITA

PROF. ENRICO GARACI

Name of legal representative(s)

Signature of legal representative(s)

06/12/2012

Date

ISTITUTO SUPERIORE DI SANITA

Stamp of the organisation

FP7 GRANT AGREEMENT

ANNEX IV - FORM A - ACCESSION OF BENEFICIARIES TO THE GRANT AGREEMENT

FUNDACION CANARIA DE INVESTIGACION Y SALUD, represented for the purpose hereof by Maria Gomez, Legal Representative, or her authorised representative, established in BARRANCO DE LA BALLENA s n , LAS PALMAS DE GRAN CANARIA, 35010, Spain acting as its legal authorised representative, hereby consents to become a *beneficiary* ("*beneficiary no 11*") to *grant agreement* N 305690 (relating to *project* "*Platform for sharing best practices for management of rare diseases*") concluded between the European Commission and ISTITUTO SUPERIORE DI SANITA established in Viale Regina Elena 299, ROMA, 00161, Italy, and accepts in accordance with the provisions of the aforementioned *grant agreement* all the rights and obligations of a *beneficiary*.

Done in 3 copies, of which one shall be kept by the *coordinator* and one by **FUNDACION CANARIA DE INVESTIGACION Y SALUD**, the third being sent to the *Commission* by the coordinator in accordance with Articles I 1 and I 2 and Article 8 of the grant agreement.

FUNDACION CANARIA DE INVESTIGACION Y SALUD	ISTITUTO SUPERIORE DI SANITA
MARÍA GÓMEZ PEÑATE	PROF. ENRICO GARACI
Name of legal representative(s)	Name of legal representative(s)
Signature of legal representative(s)	Signature of legal representative(s)
15/11/2012	06/12/2012
Date	Date
Stamp of the organisation	Stamp of the organisation

FP7 GRANT AGREEMENT

ANNEX IV - FORM A - ACCESSION OF BENEFICIARIES TO THE GRANT AGREEMENT

UNIVERSITEIT MAASTRICHT, represented for the purpose hereof by Frans Ramaekers, Director GROW, or his authorised representative, established in Minderbroedersberg 4-6, MAASTRICHT, 6200 MD, Netherlands acting as its legal authorised representative, hereby consents to become a *beneficiary* (*"beneficiary no. 12"*) to *grant agreement* N° 305690 (relating to *project "Platform for sharing best practices for management of rare diseases"*) concluded between the European Commission and ISTITUTO SUPERIORE DI SANITA established in Viale Regina Elena 299, ROMA, 00161, Italy and accepts in accordance with the provisions of the aforementioned *grant agreement* all the rights and obligations of a *beneficiary*.

Done in 3 copies, of which one shall be kept by the *coordinator* and one by **UNIVERSITEIT MAASTRICHT**, the third being sent to the *Commission* by the coordinator in accordance with Articles 1.1 and 1.2 and Article 8 of the grant agreement.

UNIVERSITEIT MAASTRICHT

Frans Ramaekers

Name of legal representative(s)

Signature of legal representative(s)

November 9, 2012.

Date

Stamp of the organisation

Maastricht University, FHML
Institute for Public Health Genomics
P.O. Box 616
6200 MD Maastricht, The Netherlands

ISTITUTO SUPERIORE DI SANITA

PROF. ENRICO GARACI

Name of legal representative(s)

Signature of legal representative(s)

06/12/2012

Date

Stamp of the organisation

ISTITUTO SUPERIORE DI SANITA

FP7 GRANT AGREEMENT

ANNEX IV - FORM A - ACCESSION OF BENEFICIARIES TO THE GRANT AGREEMENT

UNIVERSITY OF NEWCASTLE UPON TYNE, represented for the purpose hereof by Amanda Tortice, Head, Joint Research Office, and/or Douglas Robertson, Director, Research & Enterprise Services, or her/his/their authorised representative, established in Kensington Terrace 6, NEWCASTLE UPON TYNE, NE1 7RU, United Kingdom acting as its legal authorised representative, hereby consents to become a *beneficiary* (*"beneficiary no. 13"*) to *grant agreement* N° 305690 (relating to *project* *"Platform for sharing best practices for management of rare diseases"*) concluded between the European Commission and ISTITUTO SUPERIORE DI SANITA established in Viale Regina Elena 299, ROMA, 00161, Italy and accepts in accordance with the provisions of the aforementioned *grant agreement* all the rights and obligations of a *beneficiary*.

Done in 3 copies, of which one shall be kept by the *coordinator* and one by **UNIVERSITY OF NEWCASTLE UPON TYNE**, the third being sent to the *Commission* by the coordinator in accordance with Articles 1.1 and 1.2 and Article 8 of the grant agreement.

UNIVERSITY OF NEWCASTLE UPON TYNE	ISTITUTO SUPERIORE DI SANITA
Amanda Tortice	Prof Enrico Garaci
Name of legal representative(s)	Name of legal representative(s)
Signature of legal representative(s)	Signature of legal representative(s)
14 November 2012	06/12/2012
Date	Date
Stamp of the organisation	Stamp of the organisation

FP7 GRANT AGREEMENT

ANNEX IV - FORM A - ACCESSION OF BENEFICIARIES TO THE GRANT AGREEMENT

EUROPEAN ACADEMY OF PAEDIATRICS AISBL, represented for the purpose hereof by Jose Ramet, Secretary-general, and/or Elizabeth Siderius, Coordinator Working Group Rare Diseases, or her/his/their authorised representative, established in AVENUE DE LA COURONNE 20, BRUXELLES, 1050, Belgium acting as its legal authorised representative, hereby consents to become a *beneficiary* (*"beneficiary no. 14"*) to *grant agreement* N° 305690 (relating to *project "Platform for sharing best practices for management of rare diseases"*) concluded between the European Commission and ISTITUTO SUPERIORE DI SANITA established in Viale Regina Elena 299, ROMA, 00161, Italy and accepts in accordance with the provisions of the aforementioned *grant agreement* all the rights and obligations of a *beneficiary*.

Done in 3 copies, of which one shall be kept by the *coordinator* and one by **EUROPEAN ACADEMY OF PAEDIATRICS AISBL**, the third being sent to the *Commission* by the coordinator in accordance with Articles 1.1 and 1.2 and Article 8 of the grant agreement.

EUROPEAN ACADEMY OF PAEDIATRICS AISBL

JOSE RAMET

Name of legal representative(s)

Signature of legal representative(s)

3/12/2012

Date

E.A.P EUROPEAN ACADEMY OF PAEDIATRICS

Stamp of the organisation

ISTITUTO SUPERIORE DI SANITA

PROF. ENRICO GARACI

Name of legal representative(s)

Signature of legal representative(s)

13/12/2012

Date

ISTITUTO SUPERIORE DI SANITA

Stamp of the organisation

FP7 GRANT AGREEMENT
ANNEX IV - FORM A - ACCESSION OF BENEFICIARIES TO THE GRANT AGREEMENT

INSTITUTO DE SALUD CARLOS III, represented for the purpose hereof by JOAQUIN R ARENAS, Director, or his authorised representative, established in CALLE SINESIO DELGADO 4-6, MADRID, 28029, Spain acting as its legal authorised representative, hereby consents to become a *beneficiary* (*"beneficiary no. 15"*) to *grant agreement* N° 305690 (relating to *project "Platform for sharing best practices for management of rare diseases"*) concluded between the European Commission and ISTITUTO SUPERIORE DI SANITA established in Viale Regina Elena 299, ROMA, 00161, Italy and accepts in accordance with the provisions of the aforementioned *grant agreement* all the rights and obligations of a *beneficiary*.

Done in 3 copies, of which one shall be kept by the *coordinator* and one by **INSTITUTO DE SALUD CARLOS III**, the third being sent to the *Commission* by the coordinator in accordance with Articles 1.1 and 1.2 and Article 8 of the grant agreement.

INSTITUTO DE SALUD CARLOS III

JOAQUIN R. ARENAS
Name of legal representative(s)

Signature of legal representative(s)

28th NOVEMBER 2012
Date

MINISTERIO DE ECONOMIA Y COMPETITIVIDAD · DIRECCION · INSTITUTO DE SALUD CARLOS III
Stamp of the organisation

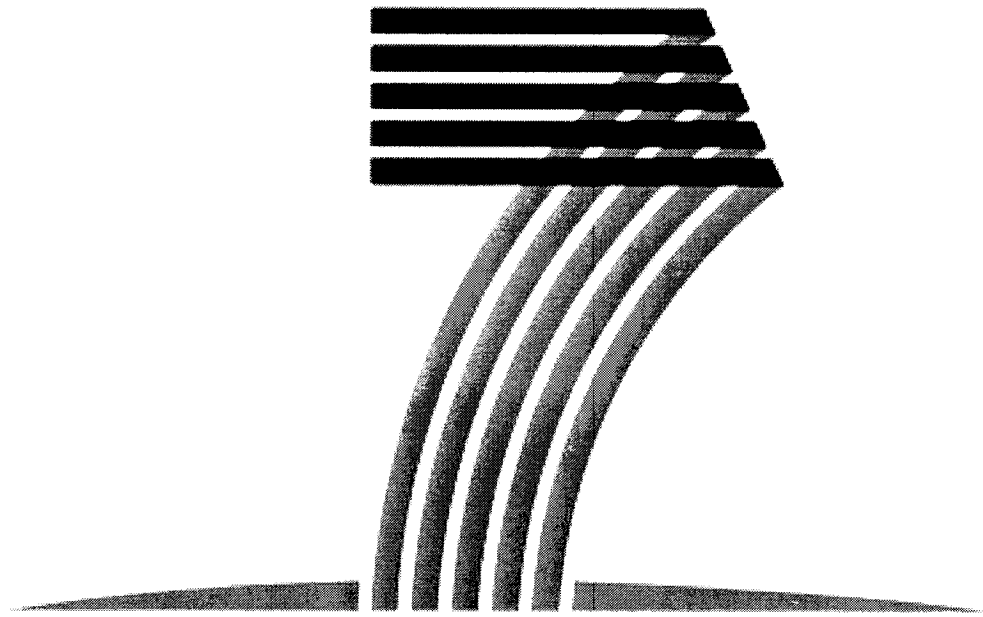
ISTITUTO SUPERIORE DI SANITA

PROF. ENRICO GARACI
Name of legal representative(s)

Signature of legal representative(s)

06/12/2012
Date

ISTITUTO SUPERIORE DI SANITA
Stamp of the organisation



SEVENTH FRAMEWORK
PROGRAMME

THEME [HEALTH.2012.2.4.4-3]
[Best practice and knowledge sharing in the clinical management of rare diseases]

Grant agreement for: Coordination and support action

Annex I - "Description of Work"

Project acronym: RARE-Bestpractices

Project full title: " Platform for sharing best practices for management of rare diseases "

Grant agreement no: 305690

Version date: 2012-06-26

Table of Contents

Part A

Workplan Tables

A1:
Project summary

Project Number [1]	305690	Project Acronym [2]	RARE-Bestpractices

One form per project

General information			
Project title [3]	Platform for sharing best practices for management of rare diseases		
Starting date [4]	01/01/2013		
Duration in months [5]	48		
Call (part) identifier [6]	FP7-HEALTH-2012-INNOVATION-1		
Activity code(s) most relevant to your topic [7]	HEALTH.2012.2.4.4-3: Best practice and knowledge sharing in the clinical management of rare diseases		
Free keywords [8]		Rare diseases, Community Network, Practice Guidelines, Information Systems, Healthcare Quality Assurance, Clinical Management, Business Process Modeling	

Abstract [9]

RARE-Bestpractices will develop a sustainable networking platform, supporting the collection of standardized and validated data and efficient exchange of knowledge and reliable information on rare diseases (RD).
RD are characterized by low prevalence (EU – 5:10000 persons). There are more than 5000, overall affecting about 30 million citizens of all ages in the EU. RD are often life-threatening and chronically debilitating, and healthcare is impaired by limited knowledge.
Collaborative efforts are needed to tackle RD to prevent significant morbidity, perinatal or early mortality, to reduce socio-economic burdens and to improve an individual's quality of life.
RARE-Bestpractices aims to improve clinical management of RD patients, narrowing the existing gap among EU MS and other countries, also considering the application of patients' rights in cross-border healthcare (EU Directive 2011/24).
The platform deals with RD as a global health issue, exploiting and integrating contributions from all EU MS and other world areas (Caucasus, Europe, America, Oceania, PAHO/WHO) and will identify additional research needs to further improve clinical practice.
Fostering synergistic collaboration among experts, patients representatives, policy makers, institutions, agencies, and other organizations experienced in systematic reviews and guidelines production, RARE-Bestpractices will focus on:
a) collection, evaluation and dissemination of existing best practices;
b) an agreed methodology suitable to develop and update best practice guidelines;
c) training activities targeted at key stakeholders to spread expertise and knowledge; and
d) a forum for exchanging information, sharing lessons learnt, and facilitating collaborations.
The platform is conceived for health care providers, experts, patients, policy makers and best practice guideline developers with outcomes that support closure of healthcare gaps among countries and improved clinical management of RD patients globally.

A2:
List of Beneficiaries

Project Number [1]	305690	Project Acronym [2]	RARE-Bestpractices

List of Beneficiaries

No	Name	Short name	Country	Project entry month[10]	Project exit month
1	ISTITUTO SUPERIORE DI SANITA	ISS	Italy	1	48
2	JAMARAU	JAMARAU	United Kingdom	1	48
3	KAROLINSKA INSTITUTET	KI	Sweden	1	48
4	HEALTHCARE IMPROVEMENT SCOTLAND	HIS	United Kingdom	1	48
5	LONDON SCHOOL OF ECONOMICS AND POLITICAL SCIENCE	LSE	United Kingdom	1	48
6	CONSIGLIO NAZIONALE DELLE RICERCHE	CNR	Italy	1	48
7	EURORDIS - EUROPEAN ORGANISATION FOR RARE DISEASES ASSOCIATION	EURORDIS	France	1	48
8	Associazione per la Ricerca sulla Efficacia della Assistenza Sanitaria Centro Cochrane Italiano	AREAS-CCI	Italy	1	48
9	UNIVERSITAETSKLINIKUM FREIBURG	UKLFR	Germany	1	48
10	BULGARIAN ASSOCIATION FOR PROMOTION OF EDUCATION AND SCIENCE	BAPES	Bulgaria	1	48
11	FUNDACION CANARIA DE INVESTIGACION Y SALUD	FUNCIS	Spain	1	48
12	UNIVERSITEIT MAASTRICHT	UM	Netherlands	1	48
13	UNIVERSITY OF NEWCASTLE UPON TYNE	UNEW	United Kingdom	1	48
14	EUROPEAN ACADEMY OF PAEDIATRICS AISBL	EAP/UEMS-SP	Belgium	1	48
15	INSTITUTO DE SALUD CARLOS III	ISCIII	Spain	1	48

A3:
Budget Breakdown

Project Number [1]	305690	Project Acronym [2]	RARE-Bestpractices

One Form per Project

Participant number in this project [11]	Participant short name	Ind. costs [13]	Estimated eligible costs (whole duration of the project)				Total receipts	Requested EU contribution
			Coordination / Support (A)	Management (B)	Other (C)	Total A+B+C		
1	ISS	T	568,529.60	34,290.00	151,414.80	754,234.40	0.00	675,016.00
2	JAMARAU	T	232,634.40	0.00	2,880.00	235,514.40	0.00	210,000.00
3	KI	T	165,344.40	0.00	2,880.00	168,224.40	0.00	150,000.00
4	HIS	T	131,700.00	0.00	2,880.00	134,580.00	0.00	120,000.00
5	LSE	T	131,700.00	0.00	2,880.00	134,580.00	0.00	120,000.00
6	CNR	S	95,004.00	40,009.00	8,667.00	143,680.00	0.00	95,000.00
7	EURORDIS	F	182,041.20	0.00	6,370.80	188,412.00	0.00	168,000.00
8	AREAS-CCI	T	87,045.60	0.00	25,105.20	112,150.80	0.00	100,000.00
9	UKLFR	T	63,930.00	0.00	3,360.00	67,290.00	0.00	60,000.00
10	BAPES	F	50,551.20	0.00	30,180.00	80,731.20	0.00	71,984.00
11	FUNCIS	F	64,411.20	0.00	2,880.00	67,291.20	0.00	60,000.00
12	UM	A	62,621.00	0.00	4,296.00	66,917.00	0.00	40,000.00
13	UNEW	T	41,980.80	0.00	2,880.00	44,860.80	0.00	40,000.00
14	EAP/UEMS-SP	F	24,350.40	0.00	9,295.20	33,645.60	0.00	30,000.00
15	ISCIII	T	64,410.00	0.00	2,880.00	67,290.00	0.00	60,000.00
Total			1,966,253.80	74,299.00	258,849.00	2,299,401.80	0.00	2,000,000.00

Note that the budget mentioned in this table is the total budget requested by the Beneficiary and associated Third Parties.

*** The following funding schemes are distinguished**

Collaborative Project (if a distinction is made in the call please state which type of Collaborative project is referred to: (i) Small of medium-scale focused research project, (ii) Large-scale integrating project, (iii) Project targeted to special groups such as SMEs and other smaller actors), Network of Excellence, Coordination Action, Support Action.

1. Project number

The project number has been assigned by the Commission as the unique identifier for your project, and it cannot be changed. The project number **should appear on each page of the grant agreement preparation documents** to prevent errors during its handling.

2. Project acronym

Use the project acronym as indicated in the submitted proposal. It cannot be changed, unless agreed during the negotiations. The same acronym **should appear on each page of the grant agreement preparation documents** to prevent errors during its handling.

3. Project title

Use the title (preferably no longer than 200 characters) as indicated in the submitted proposal. Minor corrections are possible if agreed during the preparation of the grant agreement.

4. Starting date

Unless a specific (fixed) starting date is duly justified and agreed upon during the preparation of the Grant Agreement, the project will start on the first day of the month following the entry info force of the Grant Agreement (NB : entry into force = signature by the Commission). Please note that if a fixed starting date is used, you will be required to provide a detailed justification on a separate note.

5. Duration

Insert the duration of the project in full months.

6. Call (part) identifier

The Call (part) identifier is the reference number given in the call or part of the call you were addressing, as indicated in the publication of the call in the Official Journal of the European Union. You have to use the identifier given by the Commission in the letter inviting to prepare the grant agreement.

7. Activity code

Select the activity code from the drop-down menu.

8. Free keywords

Use the free keywords from your original proposal; changes and additions are possible.

9. Abstract

10. The month at which the participant joined the consortium, month 1 marking the start date of the project, and all other start dates being relative to this start date.

11. The number allocated by the Consortium to the participant for this project.

12. Include the funding % for RTD/Innovation – either 50% or 75%

13. Indirect cost model

A: Actual Costs
S: Actual Costs Simplified Method
T: Transitional Flat rate
F :Flat Rate

Workplan Tables

Project number

305690

Project title

RARE-Bestpractices—Platform for sharing best practices for management of rare diseases

Call (part) identifier

FP7-HEALTH-2012-INNOVATION-1

Funding scheme

Coordination and support action

WT1
List of work packages

Project Number [1]	305690	Project Acronym [2]	RARE-Bestpractices

LIST OF WORK PACKAGES (WP)

WP Number [53]	WP Title	Type of activity [54]	Lead beneficiary number [55]	Person-months [56]	Start month [57]	End month [58]
WP 1	Scientific coordination, networking	COORD	1	45.70	1	48
WP 2	Platform infrastructure	COORD	2	20.40	1	48
WP 3	Agree upon methodology for production of guidelines on clinical management of RD	COORD	3	74.20	2	42
WP 4	Collection of BP and research recommendations on RD	COORD	4	22.30	1	48
WP 5	RD technologies and value assessment	COORD	5	20.20	1	22
WP 6	Dissemination	OTHER	1	24.30	1	48
WP 7	Collaboration with the International Rare Diseases Research Consortium (IRDiRC)	COORD	1	5.00	1	48
WP 8	Project management	MGT	6	11.10	1	48
			Total	223.20		

WT2:
List of Deliverables

Project Number [1]	305690	Project Acronym [2]	RARE-Bestpractices

List of Deliverables - to be submitted for review to EC

Delive-rable Number [61]	Deliverable Title	WP number [63]	Lead benefi-ciary number	Estimated indicative person-months	Nature [62]	Dissemi-nation level [63]	Delivery date [64]
D1.1	Project Internal General meeting #1	1	1	1.00	O	RE	2
D1.2	Project Internal General meeting #2	1	1	1.00	O	RE	12
D1.3	Project Internal General meeting #3	1	1	1.00	O	RE	24
D1.4	Project Internal General meeting #4	1	1	1.00	O	RE	36
D1.5	Project Internal General meeting #5	1	1	1.00	O	RE	48
D1.6	Report on training initiatives	1	8	8.00	R	CO	46
D1.7	Sustainability strategic plan report	1	1	8.00	R	CO	44
D2.1	Project Website	2	1	2.00	O	PU	6
D2.2	BP Guideline and research recommendation databases infrastructures and user documentations	2	2	12.00	O	PU	24
D2.3	Resource Review Report	2	2	1.00	R	CO	36
D2.4	Web Community and System Support	2	2	4.00	O	RE	20
D3.1	Report on the Ethical regulations applicable at the Survey	3	8	1.50	R	CO	2
D3.2	State of the art of BP guidelines	3	8	12.50	R	PP	10

WT2:
List of Deliverables

Delive-rable Number [61]	Deliverable Title	WP number [53]	Lead benefi-ciary number	Estimated indicative person-months	Nature [62]	Dissemi-nation level [63]	Delivery date [64]
D3.3	Methodology for BP guidelines on RD	3	9	18.50	R	PU	20
D3.4	Method of representation of BP guideline through BPMN	3	6	10.00	O	PU	36
D3.5	Pilot BP guideline	3	3	22.00	O	PU	36
D3.6	Patient version of the pilot BP guideline	3	7	6.50	O	PU	42
D4.1	Procedures manual for collection of guidelines and research recommendations	4	4	3.00	O	PU	24
D4.2	Collection and evaluation of existing guidelines and research recommendations #1	4	3	5.00	O	PU	24
D4.3	Collection and evaluation of existing guidelines and research recommendations #2	4	12	4.00	O	PU	36
D4.4	Collection and evaluation of existing guidelines and research recommendations #3	4	4	3.00	O	PU	44
D4.5	Evidence monitoring protocol published	4	12	3.00	R	PU	40
D4.6	Meta guideline on evidence translation in RD	4	4	3.00	R	PU	46
D5.1	Identification of BP	5	5	5.00	O	RE	22
D6.1	Logo, leaflet	6	1	0.50	O	PU	4
D6.2	Newsletter #1	6	1	1.50	O	PU	12

WT2:
List of Deliverables

Deliverable Number [61]	Deliverable Title	WP number [53]	Lead beneficiary number	Estimated indicative person-months	Nature [62]	Dissemination level [63]	Delivery date [64]
D6.3	Newsletter #2	6	1	1.00	O	PU	24
D6.4	Newsletter #3	6	1	1.00	O	PU	48
D6.5	Web-site content	6	1	2.00	O	PU	6
D6.6	Training tools #1	6	8	1.50	O	PU	25
D6.7	Training tools #2	6	8	1.00	O	PU	36
D6.8	Training tools #3	6	8	0.50	O	PU	48
D6.9	Dissemination plan events-special session	6	1	2.00	O	CO	8
D6.10	Periodic electronic journal #1	6	10	2.00	O	PU	12
D6.11	Periodic electronic journal #2	6	10	1.00	O	PU	16
D6.12	Periodic electronic journal #3	6	10	1.00	O	PU	20
D6.13	Periodic electronic journal #4	6	10	1.00	O	PU	24
D6.14	Periodic electronic journal #5	6	10	1.00	O	PU	28
D6.15	Periodic electronic journal #6	6	10	1.00	O	PU	32
D6.16	Periodic electronic journal #7	6	10	1.00	O	PU	36
D6.17	Periodic electronic journal #8	6	10	1.00	O	PU	40
D6.18	Periodic electronic journal #9	6	10	1.00	O	PU	44
D6.19	Periodic electronic journal #10	6	10	1.00	O	PU	48
D6.20	Final International Symposium	6	1	1.50	O	PU	48

WT2:
List of Deliverables

Delive-rable Number [61]	Deliverable Title	WP number [53]	Lead benefi-ciary number	Estimated indicative person-months	Nature [62]	Dissemi-nation level [63]	Delivery date [64]
D7.1	Report from IRDiRC meetings #1	7	1	0.50	R	RE	12
D7.2	Report from IRDiRC meetings #2	7	1	0.50	R	RE	24
D7.3	Report from IRDiRC meetings #3	7	1	0.50	R	RE	36
D7.4	Report from IRDiRC meetings #4	7	1	0.50	R	RE	48
D8.1	Management plans and tools	8	6	2.00	O	CO	3
D8.2	IPR policy	8	1	2.00	O	CO	12
			Total	166.50			

WT3:
Work package description

Project Number [1]	305690	Project Acronym [2]	RARE-Bestpractices

One form per Work Package

Work package number [53]	WP1	Type of activity [54]	COORD
Work package title	Scientific coordination, networking		
Start month	1		
End month	48		
Lead beneficiary number [55]	1		

Objectives

WP1 is responsible for providing the overall scientific strategy and the coordination of the project, with a view towards the long term sustainability issue. It is also responsible for the organization of training activities. Specific objectives addressed in the present WP:

1. to support appropriate communication within the Consortium and with external stakeholders
2. to plan and manage training initiatives

Description of work and role of partners

Task 1.1 (1 - 48) – Scientific Coordination - The strategic direction of all scientific and technical activities will be assured. Close coordination with management activities is envisaged. In particular, a detailed Role Assignment Matrix (RAM) will be defined at the start of the project, and constantly updated, to allocate single researchers to each specific activity with defined terms of reference. The scientific Coordination will be assured by:

o a Steering Committee constituted by the Project Coordinator, the Project Manager and by WP leaders; it will coordinate the networking activities and define the strategies of the platform; moreover it will ensure awareness on scientific advances and key emerging issues in the field of clinical management of RD also by a periodic electronic journal (see WP6).

o An Advisory Board composed of international experts representing the major stakeholders involved in the clinical management of patients with RD, in Evidence Based Medicine, and in developing evidence-based health care policies; it will give advice on major topics and will evaluate the project progress and the sustainability plan.

o A General Assembly constituted by a representative for each Beneficiary; it is in charge for contractual issues.

The SC will meet annually (including the Kick-off Meeting - KoM).

ISS is in charge of this task with the collaboration of all Beneficiaries.

Task 1.2 (24 - 48) - Manage training initiatives –This task it is recognized as one of the pillars of a self-sustainable platform.
With the overall aim to promote the development of high quality BP guidelines and their use across Europe, training courses will be available providing participants with the opportunity to learn about the basics of BP guideline development. Training will be targeted to the anticipated needs of all relevant stakeholders involved in RD, primarily BP guidelines developers of EU organizations and of European Reference Networks. Courses will based on the quality methodological standards for BP guidelines development elaborated in WP3 and criteria for evaluation elaborated in WP4- Skills and expertise will be involved within the consortium and promote contacts and relationships with other initiatives and subjects in concerned areas (G-I-N, GRADE working group).

AREAS-CCI is in charge of this task, with the support of ISS and EURORDIS.

Task 1.3 (32 – 48) - Definition of strategic plan to ensure sustainability of networking platform - To ensure sustainability in the long term for the networking platform it is essential to define an action plan for its governance. In this task operating procedures for sustainability will be explored including juridical form, funding

mechanisms as well as procedures to assure transparency and accountability of the networking platform. The strategic plan will be realised through a series of meetings actively involving all of the beneficiaries in order to obtain agreement on the proposed models of cooperation beyond the duration of the project.

ISS is charge of this task with the support of CNR. All Beneficiaries are involved.

Person-Months per Participant

Participant number [10]	Participant short name [11]	Person-months per participant
1	ISS	12.00
2	JAMARAU	4.10
3	KI	3.10
4	HIS	1.00
5	LSE	2.00
6	CNR	2.00
7	EURORDIS	10.00
9	UKLFR	1.00
10	BAPES	1.00
11	FUNCIS	2.00
12	UM	1.00
13	UNEW	4.00
14	EAP/UEMS-SP	0.50
15	ISCIII	2.00
	Total	45.70

List of deliverables

Delive-rable Number [61]	Deliverable Title	Lead benefi-ciary number	Estimated indicative person-months	Nature [62]	Dissemi-nation level [63]	Delivery date [64]
D1.1	Project Internal General meeting #1	1	1.00	O	RE	2
D1.2	Project Internal General meeting #2	1	1.00	O	RE	12
D1.3	Project Internal General meeting #3	1	1.00	O	RE	24
D1.4	Project Internal General meeting #4	1	1.00	O	RE	36
D1.5	Project Internal General meeting #5	1	1.00	O	RE	48
D1.6	Report on training initiatives	8	8.00	R	CO	46
D1.7	Sustainability strategic plan report	1	8.00	R	CO	44
		Total	21.00			

Description of deliverables

D1.1) Project Internal General meeting #1: Kick-off Meeting - Linked to Task 1.1 [month 2]

D1.2) Project Internal General meeting #2: General Assembly and Steering Committee Meetings - Linked to Task 1.1 [month 12]

D1.3) Project Internal General meeting #3: General Assembly and Steering Committee Meetings - Linked to Task 1.1 [month 24]

D1.4) Project Internal General meeting #4: General Assembly and Steering Committee Meetings - Linked to Task 1.1 [month 36]

D1.5) Project Internal General meeting #5: General Assembly and Steering Committee Meetings - Linked to Task 1.1 [month 48]

D1.6) Report on training initiatives: Training modules tool and report on the participation will be delivered - Linked to Task 1.2 [month 46]

D1.7) Sustainability strategic plan report: Report with description of the operating procedures approved by consortium will be delivered - Linked to Task 1.3 [month 44]

Schedule of relevant Milestones

Milestone number [60]	Milestone name	Lead beneficiary number	Delivery date from Annex I [60]	Comments
MS1	Meetings #1 agenda sent to beneficiaries	1	1	Document available for consultation
MS2	Meetings #2 agenda sent to beneficiaries	1	11	Document available for consultation
MS3	Meetings #3 agenda sent to beneficiaries	1	23	Document available for consultation
MS4	Meetings #4 agenda sent to beneficiaries	1	35	Document available for consultation
MS5	Meetings #5 agenda sent to beneficiaries	1	47	Document available for consultation
MS6	Training activities plan available	8	20	Training activities plan ready
MS7	Validation from beneficiaries of strategic sustainability plan	1	40	Validation available

WT3:
Work package description

Project Number [1]	305690	Project Acronym [2]	RARE-Bestpractices

One form per Work Package

Work package number [53]	WP2	Type of activity [54]	COORD
Work package title	Platform infrastructure		
Start month	1		
End month	48		
Lead beneficiary number [55]	2		

Objectives

The following objectives are addressed in the present WP:
1. to develop, implement and maintain a project website that enables the network to publicise its activities/results and communicate the availability and purpose of the network's resources
2. to set up, implement and maintain an online database facilitating collection, development, dissemination, and revision of BP guidelines, enabling access
3. to develop, implement and maintain an online database that enables collection, prioritization and communication of research recommendations, enabling access
4. to develop and maintain a web community for developing the pilot guideline

Description of work and role of partners

WP2 is responsible for developing, implementing and maintaining the network platform infrastructure to support the activities of the network. The following tasks are pursued in this WP:

Task 2.1 (1 - 48) – BP Guidelines Database - Set up, development and implementation of BP Guidelines Database which will facilitate the collection, development, dissemination and revision of BP guidelines (see WP4). It will also collect statistical data that will aid the network to evaluate implementation (i.e. are guidelines being accessed). The task will consist of the following activities:

o Database Set-up - An instance of the database will be created and configured to address network needs, be tested for functionality, and any bugs fixed.
o Database Development – Preliminary content, as agreed with KI and HIS-UK, will be provisioned (i.e. web pages developed) prepared and uploaded to the site.
o Consultation Launch – Jamarau will apply the consultation interface to support WP3 and WP4 to undertake surveys (see Task 3.1 and 4.1), agree methodologies (see Task 3.2) and develop guidelines (see Task 3.3, 3.5, 4.5). Jamarau will lead and undertake all technical activities involved including web-authoring.
o Database Updates – Site content will be updated to include standards set for collection criteria, as defined by WP4 (see Task 4.1).
o Guideline Collection - Jamarau will populate the site with existing guidelines as identified by KI/HIS (see Task 4.2), cross-referenced by database for ease of access.
o Sanity Check and Launch – The system, populated with data and information, will be subjected to a final sanity check by all partners, refinements undertaken if necessary and then officially launched. Jamarau will aid ISS in compiling launch publicity.
o Assess Efficacy - Jamarau and KI will develop a strategy to evaluate the impact of the platform infrastructure through the application of available tools and identify any technical issues, solutions that might be applied to fill gaps.

Jamarau is in charge of this task with the support of HIS, KI and ISS. Collaboration is envisaged from all Beneficiaries. (Tweek! Pty ltd), third party, is involved too.

Task 2.2 (1 - 48) - Research Recommendations Database - Design, development and implementation of the Research Recommendations Database (see WP4). Task involves:

o Database Design – Database requirements will be researched in consultation with HIS (see 4.1) and initial database design completed.
o Database Development – Once designs are complete jamarau will oversee development with the support of (Tweek! Pty ltd), third party.
o Prototype Released – The database prototype will be released for a period of 1 month, allowing partners to test the functionality and utility from a user perspective and to provide feedback regarding the system and its features.
o Research Recommendations Collection - Jamarau will assist in populating the site with research recommendations as identified by HIS (see Task 4.2).
o Sanity Check and Launch – The system, populated with data and information, will be subjected to a final sanity check by all partners, refinements undertaken if necessary and then officially launched. Jamarau will aid ISS in compiling launch publicity.

Jamarau is in charge of this task with the support of HIS-UK, KI and ISS. (Tweek! Pty ltd), third party, is involved too. Collaboration is envisaged from all Beneficiaries.

Task 2.3 (1 - 48) - Realize and manage the project Website - Development, implementation and maintenance of the project website, providing information to keep the community apprised of news, events and progress, support dissemination of information, promote discussion and advertise Network tools and services. The task will involve:
o Consultation with CO / relevant PP to determine scope, coverage and features.
o Website design, construction and testing.
o Website population (initial project information) and launch.
o Provide on-going maintenance and user support.

ISS is in charge of this task with the support of Jamarau and of a subcontractor (webmaster).

Task 2.4 (6 – 36) - User Documentation and Agreements- Development of user documentation and agreement for the two database applications, involving:

o Two user manuals will be developed and published. Manuals and system will be reviewed by users. Feedback evaluated and findings/recommendations reported.
o System access and support – A service agreement will be established, in association with task 1.6, that addresses the provision of access, maintenance and support for the databases beyond the end of the grant period.

Jamarau is in charge of this task. Collaboration is envisaged from all Beneficiaries.

Task 2.5 (6 – 48) - Develop and Support Web-community - Develop and support the private web-community platform aiding to build and maintain a network of experts that use the platform, enabling them to contribute to discussions, project work (e.g. development of the pilot guidelines, including use of Delphi method as per task 3.3-Develop a new BP guideline on RD) and ongoing sustainability (e.g. revision of guidelines over time).

Jamarau is in charge of this task, with the support of KI and ISS. Collaboration is envisaged from all Beneficiaries.

Person-Months per Participant

Participant number [10]	Participant short name [11]	Person-months per participant
1	ISS	4.40
2	JAMARAU	13.00
3	KI	2.00
4	HIS	1.00
	Total	20.40

WT3:
Work package description

List of deliverables

Deliverable Number [61]	Deliverable Title	Lead beneficiary number	Estimated indicative person-months	Nature [62]	Dissemination level [63]	Delivery date [64]
D2.1	Project Website	1	2.00	O	PU	6
D2.2	BP Guideline and research recommendation databases infrastructures and user documentations	2	12.00	O	PU	24
D2.3	Resource Review Report	2	1.00	R	CO	36
D2.4	Web Community and System Support	2	4.00	O	RE	20
		Total	19.00			

Description of deliverables

D2.1) Project Website: Public launch of project web site - Linked to Task 2.3 [month 6]

D2.2) BP Guideline and research recommendation databases infrastructures and user documentations: Launch of DB for the collection of BP guidelines and research recommendations identified in the Task 4.2 - Linked to Task 2.1 [month 24]

D2.3) Resource Review Report: Review system and user documentation to ensure utility - Linked to Task 2.1, 2.2, 2.3 [month 36]

D2.4) Web Community and System Support: Develop and mantain a web-community - Linked to Task 2.5 [month 20]

Schedule of relevant Milestones

Milestone number [59]	Milestone name	Lead beneficiary number	Delivery date from Annex I [60]	Comments
MS8	BP guideline database prototype ready	2	4	Website & report
MS9	RR database prototype ready	2	22	Website & report
MS10	Collections underway	2	23	Website & report
MS11	Sanity check of databases	2	23	Website & report

WT3:
Work package description

Project Number [1]	305690	Project Acronym [2]	RARE-Bestpractices

One form per Work Package

Work package number [53]	WP3	Type of activity [54]	COORD
Work package title	Agree upon methodology for production of guidelines on clinical management of RD		
Start month	2		
End month	42		
Lead beneficiary number [55]	3		

Objectives

WP3 is responsible for leading work to agree on methodological quality standards for the development of guidelines on clinical management of RD.
The following objectives are addressed in the present WP:
1. to delineate the current state of existing institutional programmes, as well as processes and tools for developing, updating, adapting guidelines in the field of RD
2. to define methodological quality standards for BP guidelines on RD
3. to develop one new BP guideline for a specific rare condition implementing the methodological quality standards and utilizing a web community (see WP2)
4. to develop a methodology to implement through Business Processes Modelling Notation (BPMN) implicit processes subtended by the guidelines' activities

Description of work and role of partners

Task 3.1 (2 - 10) – Analyse the state of the art of BP guidelines - The following activities will be conducted:

o a survey to gain information on BP guidelines and programmes dedicated to RD in EU MS.
o A study to investigate methods in use for the development of BP guidelines and reports on RD by developers.

Results of the survey and of the study will be summarized in a report (see D3.1) which will contribute to Consortium discussion (see Task 3.2).
Before beginning the survey, when requested by laws and regulations, the notifications and /or opinions will be submitted and/or required to the competent legal Bodies. In such cases relevant communication with such bodies and any approval will be submitted to the EC and reported as a deliverable (see D3.1). Such deliverable will be ready 2 months before the launch of the survey in order to be in time to take any applicable measure to respect the identified regulations / recommendations.

AREAS-CCI in charge of this task with the support of KI, HIS. Collaboration is envisaged from all the Beneficiaries.

Task 3.2 (11 - 20) – Agree on common methodological quality standards for developing BP guidelines on RD - The best methodology for assessing the confidence in estimates of effect (also known as "quality of evidence") and for developing recommendations based on a multi-stage process will be developed in this task. This process will be informed by a review of current methods and procedures for development of guidelines and recommendations for RD. The process will involve working through prepared examples based on the GRADE approach that the GRADE Working Group has applied successfully in the development of its methodology. For this purpose we will identify five challenges in guidelines for a specific RD. That is we will use five RD and prepare evidence summaries and recommendations as examples. These five examples will be explored and challenges and advantages identified during a one-day workshop in Freiburg with key participants of this proposal. During this workshop the examples will be finalized and key questions for external advisors identified. These key questions and finalized examples will then be submitted for input to the GRADE Working Group that consists of over three hundred individuals with interest in guideline development. They will be discussed at a major GRADE Working Group meeting and presented by the investigators at the University of Freiburg. This work is feasible because Dr. Schünemann is co-chair of the GRADE Working Group and responsible for the agenda at the GRADE meetings. After obtaining feedback from the GRADE Working Group, this material

will be finalized. Any methodological challenges will be worked through and an additional five examples will be identified and prepared prospectively with evidence profiles and mock recommendations. In a second workshop with participants of this proposal we will finalize the methodology and involve all relevant stakeholders for final feedback before the second workshop. During the second workshop we will prepare mock recommendations with all participants by assigning roles of relevant stakeholders to participants in the workshop and by simulating commonly used guideline recommendations development processes such as described in various publications by WHO and the GRADE Working Group.

UKLFR is in charge of this task with the support of ISS, KI, HIS, AREAS-CCI, FUNCIS, ISCIII, , BAPES, CNR, EURORDIS. Collaboration is envisaged from all the Beneficiaries.

Task 3.3 (21 – 36) – Develop a new BP guideline on a specific RD - A pilot will be conducted to test the methodological quality standards in the collaborative development of a guideline for a specific rare condition, implementing the methodological quality standards and common tools/formats set up in Task 3.2. Any criticisms observed will be discussed among all project partners in order to ameliorate the guideline development protocol. Task 3.3. is strictly linked with Task 3.4 which is in charge of the representation of processes embedded in the guidelines to be developed.

KI is in charge of this task with the support of Jamarau, CNR, AREAS-CCI, BAPES, FUNCIS, ISCIII, EURORDIS.

Task 3.4 (21 - 36) – Represent the processes defined in the new BP guideline on a specific RD (see Task 3.3) A pilot will be conducted to test the methodological representation of standards, set up in Task 3.2, using the Business Process Model Notation (BPMN 2.0) in the collaborative development of a guideline for a specific rare condition. Also in this case, any criticisms observed will be discussed among all project partners in order to ameliorate the guideline development protocol. The work will be carried out in 3 phases,in collaboration with the partners involved in Task 3.3:

o selection of possible processes to implement the guideline to be developed;
o development of the process model; and
o submission of the results to a joint revision to the project Beneficiaries.

CNR is in charge of this task with the support of and KI, UKLFR, AREAS-CCI, BAPES, FUNCIS, ISCIII, EURORDIS. Collaboration is envisaged from all the Beneficiaries

Task 3.5 (36 - 42) – Patient version of the pilot BP guideline developed in Task 3.3 - A patient version will be elaborated as a complement to the pilot BP guideline developed in Task 3.3. The patient version of the pilot BP guideline will provide a summary of recommendations made for health professionals, in a language adapted for patients and their families.
Patients and their families will be involved in the elaboration process of the patient version of the pilot BP guideline.

EURORDIS is in charge of this task, with the support of KI.

Person-Months per Participant

Participant number [10]	Participant short name [11]	Person-months per participant
1	ISS	8.00
2	JAMARAU	3.00
3	KI	7.00
4	HIS	2.00
6	CNR	12.30
7	EURORDIS	11.50
8	AREAS-CCI	4.40
9	UKLFR	3.50

WT3:
Work package description

Person-Months per Participant

Participant number [10]	Participant short name [11]	Person-months per participant
10	BAPES	2.00
11	FUNCIS	10.00
14	EAP/UEMS-SP	0.50
15	ISCIII	10.00
	Total	74.20

List of deliverables

Deliverable Number [61]	Deliverable Title	Lead beneficiary number	Estimated indicative person-months	Nature [62]	Dissemination level [63]	Delivery date [64]
D3.1	Report on the Ethical regulations applicable at the Survey	8	1.50	R	CO	2
D3.2	State of the art of BP guidelines	8	12.50	R	PP	10
D3.3	Methodology for BP guidelines on RD	9	18.50	R	PU	20
D3.4	Method of representation of BP guideline through BPMN	6	10.00	O	PU	36
D3.5	Pilot BP guideline	3	22.00	O	PU	36
D3.6	Patient version of the pilot BP guideline	7	6.50	O	PU	42
		Total	71.00			

Description of deliverables

D3.1) Report on the Ethical regulations applicable at the Survey: A detailed research of current legislations will be done and reported. In case notifications and/or authorizations are requested, copies of such communications will be included in the present deliverable - Linked to Task 3.1 [month 2]

D3.2) State of the art of BP guidelines: A report summarized the survey and the study information conducted will be delivered - Linked to Task 3.1 [month 10]

D3.3) Methodology for BP guidelines on RD: Report on standard methodology suitable for developing and updating BP care guidelines on RD - Linked to Task 3.2 [month 20]

D3.4) Method of representation of BP guideline through BPMN: Guide on the application of the BPMN for the representation of BP guideline - Linked to Task 3.4 [month 36]

D3.5) Pilot BP guideline: Using the methods agreed upon for how to develop BP guidelines (D3.2), and the platform developed to aid in developing care guidelines (WP 2), a pilot BP guideline will be developed - Linked to Task 3.3, 3.4 [month 36]

D3.6) Patient version of the pilot BP guideline: Patient version of the pilot BP guideline developed in task 3.3 - Linked to Task 3.5 [month 42]

Schedule of relevant Milestones

Milestone number [59]	Milestone name	Lead beneficiary number	Delivery date from Annex I [60]	Comments
MS12	Survey to gain information on BP guidelines or HTA programmes dedicated to RD in EU MS	8	5	Survey available on the Project WEB site for on-line access
MS13	First workshop held to identify challenges in developing RD guidelines	9	6	Minutes of the workshop available
MS14	Second workshop held to finalize BP guidelines methodology	9	15	Minutes of the workshop available
MS15	Specific rare condition identified for the development of a new pilot BP guideline	3	24	Report on motivations available

WT3:
Work package description

Project Number [1]	305690	Project Acronym [2]	RARE-Bestpractices

One form per Work Package

Work package number [53]	WP4	Type of activity [54]	COORD
Work package title	Collection of BP and research recommendations on RD		
Start month	1		
End month	48		
Lead beneficiary number [55]	4		

Objectives

The following objectives are addressed in the present WP:

1. to capitalize on existing BP(guidelines, documents, etc.)
2. to identify gaps in scientific knowledge (uncertainties), elaborating related research needs, and recommend relevant research initiatives

Description of work and role of partners

WP4 will collect and capitalize on existing BP guidelines currently scattered across various RD databases and websites and make them accessible through a single point of access. WP4 will also coordinate the collection, prioritization and communication of RD research recommendations. The approach should guarantee effectiveness, reproducibility and transparency. WP4 will take advantage of existing collection of data (National Guideline Clearing House, G-I-N, Orphanet, PHGEN II). WP4 will also monitor the emerging evidence from basic science (genomics) to identify future research and policy needs to improve the clinical and health management of RD. WP4 will develop meta level BP guidelines on the timely and effective and efficient translation of the evidence into patient oriented strategies for RD.
The following tasks are pursued in this WP:

Task 4.1 (1 - 18) - Develop collection criteria for existing guidelines and research recommendations - With over 5000 rare diseases in existence, initial agreement must be reached on the scope and organisation of the information gathering, monitoring and evaluation activities to be undertaken in WP4. Task 4.1 will involve the identification and investigation of existing sources of guidelines and research recommendations; an information needs analysis of participants and key stakeholders to inform the collection development criteria; proposal of collection criteria; and validation of the criteria by partners.

HIS is in charge of this task, with the support of ISS and UM. Collaboration is envisaged from all the Beneficiaries.

Task 4.2 (18 - 48) - Collection of existing guidelines and research recommendations - The development and delivery of a collection process will involve:

o Specification of search strategy (resources, search terms and timing of searches).
o Validation of search strategy.
o Development of model collections on selected topics.
o Evaluation of model collections.
o Development of plan for coordination of ongoing collection.
o Creation of a collection procedures manual.
o Implementation and embedding of ongoing collection of guidelines and research recommendations.

KI and HIS are jointly in charge of this task with the support of ISS, Jamarau and UM. Collaboration is envisaged from all Beneficiaries.

Task 4.3 (18- 48) - Evaluation of guidelines on RD – The evaluation of existing RD guidelines will involve:

o Establishing evaluation requirements with participating partners

o Identification of existing evaluation methods and appraisal instruments.
o Adaptation or development of evaluation method.
o Validation of evaluation method.
o Application of evaluation method to selected RD topics.
o Creation of an evaluation procedures manual.

HIS is in charge of with the support of ISS and UKLFR of this task. Collaboration is envisaged from all Beneficiaries.

Task 4.4 (12 - 48) - Horizon scanning to monitor emerging evidence on RD (LAL model) - This task involves development and delivery of methods to monitor emerging evidence from basic science, particularly genomics, to identify future research and policy needs and support improved clinical management of RD and health. This task will involve:

o Identification and investigation of existing information sources and monitoring activities.
o Development and validation of search methods.
o Creation of an evidence monitoring (horizon scanning) protocol.
o Development of plan for coordination of ongoing evidence monitoring.
o Development and application of a methodological tool for translation and dissemination of emerging evidence from basic sciences into healthcare systems (LAL model).
o Implementation and embedding of ongoing evidence monitoring.

UM is in charge of this task with the support of HIS.

Task 4.5 (25 - 48) - Development of meta level guideline on evidence translation for RD - This task involves development and delivery of a meta level guideline to encourage best practices in the timely, effective and efficient translation of evidence into patient oriented strategies for RD. This task will involve:

o Formation of meta level guideline development group and agreement of working methods.
o Development of guideline scope and key questions.
o Supporting literature search.
o Development and validation of draft meta level guideline.
o Finalisation and dissemination of draft meta level guideline.

HIS is in charge of this task with the collaboration of UKLFR, UM, and KI.

Person-Months per Participant

Participant number [10]	Participant short name [11]	Person-months per participant
1	ISS	4.00
2	JAMARAU	2.00
3	KI	4.00
4	HIS	6.30
9	UKLFR	2.00
12	UM	4.00
	Total	22.30

WT3:
Work package description

List of deliverables

Deliverable Number [61]	Deliverable Title	Lead beneficiary number	Estimated indicative person-months	Nature [62]	Dissemination level [63]	Delivery date [64]
D4.1	Procedures manual for collection of guidelines and research recommendations	4	3.00	O	PU	24
D4.2	Collection and evaluation of existing guidelines and research recommendations #1	3	5.00	O	PU	24
D4.3	Collection and evaluation of existing guidelines and research recommendations #2	12	4.00	O	PU	36
D4.4	Collection and evaluation of existing guidelines and research recommendations #3	4	3.00	O	PU	44
D4.5	Evidence monitoring protocol published	12	3.00	R	PU	40
D4.6	Meta guideline on evidence translation in RD	4	3.00	R	PU	46
		Total	21.00			

Description of deliverables

D4.1) Procedures manual for collection of guidelines and research recommendations: Document that formalises search strategies, sources, and procedures for ongoing collection of best practice guidelines and research recommendations including procedure for evaluation of guidelines -Linked to Task 4.1, 4.2 and Task 4.3 [month 24]

D4.2) Collection and evaluation of existing guidelines and research recommendations #1: Guidelines and research recommendations are identified and uploaded into each specific databases. The application of evaluation methods it is applied to specific RD topics. Linked to Task 4.1, 4.2 and task 4.3 [month 24]

D4.3) Collection and evaluation of existing guidelines and research recommendations #2: Guidelines and research recommendations are identified and uploaded into each specific databases. The application of evaluation methods it is applied to specific RD topics. Linked to Task 4.1, 4.2 and task 4.3 [month 36]

D4.4) Collection and evaluation of existing guidelines and research recommendations #3: Guidelines and research recommendations are identified and uploaded into each specific databases. The application of evaluation methods it is applied to specific RD topics. Linked to Task 4.1, 4.2 and task 4.3 [month 44]

D4.5) Evidence monitoring protocol published: Formalised methods to monitor emerging evidence from basic science, particularly genomics, to identify future research and policy needs and support improved clinical management of RD - Linked to Task 4.4 [month 40]

D4.6) Meta guideline on evidence translation in RD: A document that captures the evidence base on the timely, effective and efficient translation of evidence into patient oriented strategies for RD and provides recommendations on BP - Linked to Task 4.5. [month 46]

WT3:
Work package description

Schedule of relevant Milestones

Milestone number [59]	Milestone name	Lead beneficiary number	Delivery date from Annex I [60]	Comments
MS16	Approval of criteria	4	15	Criteria will be validated by the General Assembly
MS17	Approval of the draft of the metalevel guidelines	4	42	Metalevel guidelines will be validated by the General Assembly

WT3:
Work package description

Project Number [1]	305690	Project Acronym [2]	RARE-Bestpractices

One form per Work Package

Work package number [53]	WP5	Type of activity [54]	COORD
Work package title	RD technologies and value assessment		
Start month	1		
End month	22		
Lead beneficiary number [55]	5		

Objectives

The following objectives are addressed in the present WP:
1. to understand the criteria used by competent authorities in selected Member States in assessing the value of orphan medicines relying on evidence-based medicine (EBM) and cost-effectiveness analysis (CEA) in each jurisdiction
2. by drawing on a selection of orphan drug appraisals in a number of EU countries over the January 2006 to December 2012 period and across therapeutic classes, to determine the criteria used to evaluate these drugs and the way clinical practice guidelines are developed across the selected sample of drugs;and
3. to explore BP based on the evidence collected and share these across EU Member States

Description of work and role of partners

Regulation (EC) No. 141/2000 on orphan medicinal products states that 'patients suffering from rare conditions should be entitled to the same quality of treatment as other patients'. The Council Recommendation of 8 June 2009 (29/C 151/02) recommends that EU MS establish and implement plans or strategies for RD aiming to ensure that patients with RD have access to high quality care, including diagnostics, treatments, and, if possible, effective orphan drugs. These plans should also support the 'sharing of the MS assessment reports on the therapeutic added value of orphan drugs at community level where the relevant knowledge and expertise is gathered, in order to minimise delays in access to orphan drugs for rare disease patients'. Current evidence suggests that patient access to these drugs varies greatly from one EU MS to another, for a variety of reasons, including the high annual treatment costs and unacceptable incremental cost effectiveness ratios. Drug priority setting in most jurisdictions within the EU relies heavily on EBM and CEA; this approach is helpful in that it provides useful information that supports three key values (evidence, benefit, and efficiency), but limited because these are not the only values relevant to drug priority setting. Other values relevant to drug priority setting decisions include: equity, equality, need, precedent, and solidarity.
The following tasks are pursued in this WP:

Task 5.1 (1-5) – Review of the available literature and jurisdiction-specific guidelines on value assessments of orphan drugs – The aim of this first phase is to map out the current processes of EBM and CEA for orphan drugs in EU MS. To do so, the following will be carried out:

o To review the available evidence on clinical assessment in general and for orphan drugs in particular.
o To review the processes in place for orphan drugs in a sample of countries referred to as "the study countries" (e.g. Sweden, France, England, Scotland, Germany, Italy, Spain and Poland).
o To identify current practices (e.g. within clinical and HTA-specific guidelines to apply for the reimbursement of (orphan) drugs) for appraising orphan drugs in the study countries, and identify whether this is done in the same manner as all other technologies or whether additional considerations are accounted for in the assessments (e.g. disease severity, need, quality of life to the patient, availability of other treatment alternatives, life-threatening conditions, etc.).

LSE will be responsible for this task in collaboration with all the Beneficiaries.

Task 5.2 (6 – 11) – Selection of orphan drug appraisals in the EU study MS over the 2006-2012 period and across therapeutic classes – This is the data collection phase, where all orphan drugs appraised between January 2006 and December 2012 in the study jurisdictions will be listed in a central database. Orphan drugs

included will be based on the European designation of an orphan drug (Regulation (EC) No 141/2000). They will be recorded as "drug-indication pairs", since this is what coverage decisions usually apply to. A sub-sample of drug-indication pairs (referred to as the "study drugs") will be selected for an in-depth analysis as follows:

o Drugs appraised by a sufficient number of jurisdictions (the "sufficient number" will depend on the number of study countries included and the corresponding number of appraisals collected).
o Drug-indication pairs that have received different value assessments from different jurisdictions. These will be most interesting to analyze, as it will enable us to identify the critical factors driving contrary decisions.
A sample of drugs, common across the study countries will be selected and this is estimated to be between 5 and 10 - Submitted clinical and economic evidence related to each of these drugs will be identified, collected and analysed. Sources of this information will be peer reviewed publications, relevant government websites, including the HTA agencies. This evidence will be analyzed both quantitatively and qualitatively to systematically identify decisions made relative to value assessment, as well as best practices.

LSE will be responsible for this task in collaboration with all the Beneficiaries.

Task 5.3 (12 – 18) – Synthesis of value assessment criteria and identification of BP based on clinical and economic evidence - Following on from the previous task - the critical factors leading to value assessment and bestpractice(s) across the study countries will be identified and cross compared.
Those factors leading to positive value assessments and outcomes will be considered as BP, whereas those leading to negative value assessments will further be investigated to understand whether these are weaknesses in the processes. If so, alternatives will be proposed. Cross country comparisons will be conducted in order to understand why there may be differences across countries in the interpretation of the clinical and, if applicable, the economic evidence.

LSE will be responsible for this task in collaboration with all the Beneficiaries.

Task 5.4 (19 – 22) – Explore best practice (BP) based on the evidence collected and share these across EU MS - The applicability to different settings of the BP identified previously will be explored, to determine whether they can be harmonized across jurisdictions. Findings will include, for example, whether certain elements (e.g. disease severity, need) may favour or not the outcome of an orphan drug assessment in a particular country, and explain how BP can be implemented in such settings.

LSE will be responsible for this task in collaboration with all the Beneficiaries.

Person-Months per Participant

Participant number [10]	Participant short name [11]	Person-months per participant
1	ISS	3.00
5	LSE	17.20
	Total	20.20

List of deliverables

Delive-rable Number [61]	Deliverable Title	Lead benefi-ciary number	Estimated indicative person-months	Nature [62]	Dissemi-nation level [63]	Delivery date [64]
D5.1	Identification of BP	5	5.00	O	RE	22
		Total	5.00			

Description of deliverables

D5.1) Identification of BP: Synthesis of value assessment criteria and identification of BP - Linked to Task 5.4. [month 22]

WT3:
Work package description

Schedule of relevant Milestones

Milestone number [59]	Milestone name	Lead beneficiary number	Delivery date from Annex I [60]	Comments
MS18	Review of the available literature on value assessment in general and specifically to RD completed	5	5	Intermediate report available
MS19	Orphan drug appraisals across the EU study MS over the 2006 – 2012 period selected and DB realised	5	11	Creation of a database
MS20	Synthesis available of value assessment criteria and identification of BP	5	18	Final report

WT3:
Work package description

Project Number [1]	305690	Project Acronym [2]	RARE-Bestpractices

One form per Work Package

Work package number [53]	WP6	Type of activity [54]	OTHER
Work package title	Dissemination		
Start month	1		
End month	48		
Lead beneficiary number [55]	1		

Objectives

WP 6 is responsible for preparing and supporting the dissemination of the results of the project, laying the foundation for further collaborations, sustainability and expansion of the network. Consequently the present work package has to achieve the following objectives:
1. to plan and manage external communication activities
2. to widely disseminate the project concept, developments and findings to all key actors in the field in an interactive way, integrating their feedback at key points of development and evaluation work
3. to promote acceptance and implementation of the output project by scientific and patient community as well as integration of the results of the project in sustainable policies and collaboration

Description of work and role of partners

Task 6.1 (1 - 48) Develop dissemination tools -The following dissemination tools will be developed:

o Logo: the first step for a good communication is to deliver a project logo and to issue some recommendations and templates for project documents. This will be done by ISS during the first month of the project.
o Leaflet with project aims, objectives and participants will be produced at the start of the project.
o Newsletter: in the course of the project, a short electronic yearly newsletter will be published. It will provide dedicated information about the project results and achievements. Three issues are planned.
o Web site content: the project web site, designed and implemented by ISS in collaboration with Jamarau (see WP2), will include public documents, deriving from the project work. Content will be regularly updated, offering links to other relevant sites and to partners' web sites. The objectives of the website are to provide general information in English language about the project and to communicate about the results of the project.

ISS is in charge of this task. Collaboration is envisaged from all the Beneficiaries.

Task 6.2 (1 - 48) - Scientific Communication - To increase the interest in the project results and to enhance the dissemination of project results, dedicated communication activities will be implemented. This will be done by the organisation of special sessions during existing Conferences or Congresses. Particular attention will be given to the invitation of key stakeholders to attend project internal meetings (WP1). The idea is to exchange knowledge and feedback with these stakeholders during the sessions and to include the results of these exchanges in the project's global thinking. With respect to the organization of special sessions during existing Conferences or Congresses a plan will be prepared. Some events have already been targeted for example: G-I-N, EUCERD, EURORDIS, PAHO-WHO, EAHIL, EUPHA, EHFG, HTAi conferences; International Congress on Medical Librarianship and Cochrane Collaboration conferences and Colloquium.
Partners will be encouraged to publish articles in scientific journals, based on or referring to the work achieved in the project.

ISS is in charge of this task. Collaboration is envisaged from all the Beneficiaries.

Task 6.3 (1 - 48) - Establish interaction with relevant EU initiatives -The objective of this task is to maximize impact of the project results on the European health care provision and health care policy-making, and assure that project output will gain acceptance by scientific and patient community so that they are embedded into the ongoing definition of health policies. To this purpose RARE-Bestpractices will pursue interaction with EURORDIS, EUCERD and EUCERD joint action, and within the EUCERD joint action it will ensure specific

links and cooperation with EUROPLAN II, establishing effective synergy. To achieve this RARE-Bestpractices is facilitated by the strategic involvement of key partners (EURORDIS, UNEW is leader of EUCERD joint action, and ISS is the coordinator of EUROPLAN II).

ISS is in charge of this task with support of UNEW and EURORDIS. Collaboration is envisaged from all the Beneficiaries

Task 6.4 (20 - 48) - Training tools preparation and dissemination -RARE-Bestpractices tools and resources to assist in BP guidelines development will be elaborated on the basis of the WP3 and WP4 results for guidelines developers. Tools for online learning will be prepared and they will be collated and disseminated via the website.

AREAS-CCI is in charge of this task with the support of ISS.

Task 6.5 (12 - 46) – Periodic Electronic Journal – An electronic, open access scientific journal will be established to foster further interaction between the research community and the other RD stakeholders. It will build on the project's general communication and dissemination channels, especially targeting RD researchers, since there are not journals dedicated to RD research. Rare Diseases Epidemiology and Best Practice (the draft title) will be intended to be a vehicle for the exploration and discussion of current issues on RD epidemiology and best practice, and in particular will be aimed at enhancing communication between key stakeholders. The Editorial Board will consist of members of the project's Consortium, thus ensuring sustainable development of the journal after the end of the project.

BAPES is in charge of this task with the support of ISS. Collaboration is envisaged from all Beneficiaries.

Task 6.6 (36 - 48) - Final international symposium - The final conference will be important for the dissemination of the project outputs. It will involve the participation of representatives of organizations involved in BP guidelines development, patients, researchers, experts, health professionals and other professionals involved in the field of RD, scientific societies and academic institutions, policy-makers, public entities, including regulatory bodies, general public, and industry.
ISS is in charge of this task with the support of EURORDIS. Collaboration is envisaged from all Beneficiaries

Person-Months per Participant

Participant number [10]	Participant short name [11]	Person-months per participant
1	ISS	11.10
6	CNR	1.00
7	EURORDIS	0.50
8	AREAS-CCI	3.70
10	BAPES	7.00
14	EAP/UEMS-SP	1.00
	Total	24.30

List of deliverables

Deliverable Number [61]	Deliverable Title	Lead beneficiary number	Estimated indicative person-months	Nature [62]	Dissemination level [63]	Delivery date [64]
D6.1	Logo, leaflet	1	0.50	O	PU	4
D6.2	Newsletter #1	1	1.50	O	PU	12
D6.3	Newsletter #2	1	1.00	O	PU	24

WT3:
Work package description

List of deliverables

Delive-rable Number [61]	Deliverable Title	Lead benefi-ciary number	Estimated indicative person-months	Nature [62]	Dissemi-nation level [63]	Delivery date [64]
D6.4	Newsletter #3	1	1.00	O	PU	48
D6.5	Web-site content	1	2.00	O	PU	6
D6.6	Training tools #1	8	1.50	O	PU	25
D6.7	Training tools #2	8	1.00	O	PU	36
D6.8	Training tools #3	8	0.50	O	PU	48
D6.9	Dissemination plan events-special session	1	2.00	O	CO	8
D6.10	Periodic electronic journal #1	10	2.00	O	PU	12
D6.11	Periodic electronic journal #2	10	1.00	O	PU	16
D6.12	Periodic electronic journal #3	10	1.00	O	PU	20
D6.13	Periodic electronic journal #4	10	1.00	O	PU	24
D6.14	Periodic electronic journal #5	10	1.00	O	PU	28
D6.15	Periodic electronic journal #6	10	1.00	O	PU	32
D6.16	Periodic electronic journal #7	10	1.00	O	PU	36
D6.17	Periodic electronic journal #8	10	1.00	O	PU	40
D6.18	Periodic electronic journal #9	10	1.00	O	PU	44
D6.19	Periodic electronic journal #10	10	1.00	O	PU	48
D6.20	Final International Symposium	1	1.50	O	PU	48
		Total	23.50			

Description of deliverables

D6.1) Logo, leaflet: Logo and Leaflet with project's aims and objective and partners involved - Linked to Task 6.1. [month 4]

D6.2) Newsletter #1: Periodic newsletter of achievements and events of the project disseminated via-mailing list - Linked to Task 6.1. [month 12]

D6.3) Newsletter #2: Periodic newsletter of achievements and events of the project disseminated via-mailing list - Linked to Task 6.1. [month 24]

D6.4) Newsletter #3: Periodic newsletter of achievements and events of the project disseminated via-mailing list - Linked to Task 6.1. [month 48]

D6.5) Web-site content: Content development and periodic update - Linked to Task 6.1 [month 6]

D6.6) Training tools #1: Development and dissemination via website of online learning modules in the field of BP guideline development and evaluation - Linked to Task 6.4. [month 25]

D6.7) Training tools #2: Development and dissemination via website of online learning modules in the field of BP guideline development and evaluation - Linked to Task 6.4. [month 36]

D6.8) Training tools #3: Development and dissemination via website of online learning modules in the field of BP guideline development and evaluation - Linked to Task 6.4. [month 48]

D6.9) Dissemination plan events-special session: Dissemination plan of presentation of project achievements to target conference and events - Linked to Task 6.2. [month 8]

D6.10) Periodic electronic journal #1: A Periodic Scientific Journal will be established to foster further interaction between the research community and the other RD stakeholders (three numbers per year are envisaged) - Linked to Task 6.5. [month 12]

D6.11) Periodic electronic journal #2: A Periodic Scientific Journal will be established to foster further interaction between the research community and the other RD stakeholders (three numbers per year are envisaged) - Linked to Task 6.5. [month 16]

D6.12) Periodic electronic journal #3: A Periodic Scientific Journal will be established to foster further interaction between the research community and the other RD stakeholders (three numbers per year are envisaged) - Linked to Task 6.5. [month 20]

D6.13) Periodic electronic journal #4: A Periodic Scientific Journal will be established to foster further interaction between the research community and the other RD stakeholders (three numbers per year are envisaged) - Linked to Task 6.5. [month 24]

D6.14) Periodic electronic journal #5: A Periodic Scientific Journal will be established to foster further interaction between the research community and the other RD stakeholders (three numbers per year are envisaged) - Linked to Task 6.5. [month 28]

D6.15) Periodic electronic journal #6: A Periodic Scientific Journal will be established to foster further interaction between the research community and the other RD stakeholders (three numbers per year are envisaged) - Linked to Task 6.5. [month 32]

D6.16) Periodic electronic journal #7: A Periodic Scientific Journal will be established to foster further interaction between the research community and the other RD stakeholders (three numbers per year are envisaged) - Linked to Task 6.5. [month 36]

D6.17) Periodic electronic journal #8: A Periodic Scientific Journal will be established to foster further interaction between the reasearch community and the other RD stakeholders (three numbers per year are envisaged) - Linked to Task 6.5. [month 40]

D6.18) Periodic electronic journal #9: A Periodic Scientific Journal will be established to foster further interaction between the research community and the other RD stakeholders (three numbers per year are envisaged) - Linked to Task 6.5. [month 44]

D6.19) Periodic electronic journal #10: A Periodic Scientific Journal will be established to foster further interaction between the research community and the other RD stakeholders (three numbers per year are envisaged) - Linked to Task 6.5. [month 48]

D6.20) Final International Symposium: Organization of final international symposium - Linked to Task 6.6. [month 48]

Schedule of relevant Milestones

Milestone number [59]	Milestone name	Lead beneficiary number	Delivery date from Annex I [60]	Comments
MS21	Periodic electronic journal structure approved	10	6	Structure of the Journal approved by the SC

WT3:
Work package description

Project Number [1]	305690	Project Acronym [2]	RARE-Bestpractices

One form per Work Package

Work package number [53]	WP7	Type of activity [54]	COORD
Work package title	Collaboration with the International Rare Diseases Research Consortium (IRDiRC)		
Start month	1		
End month	48		
Lead beneficiary number [55]	1		

Objectives

The International Rare Diseases Research Consortium (IRDiRC) was launched in April 2011 to foster international collaboration in rare diseases research. IRDiRC will team up researchers and organisations investing in RD research in order to achieve two main objectives, namely to deliver 200 new therapies for RD and means to diagnose most rare diseases by the year 2020. Maximising scarce resources and coordinating research efforts are key elements for success in the rare diseases field. Worldwide sharing of information, data and samples to boost research is currently hampered by the absence of an exhaustive RD classification, standard terms of reference and common ontologies, as well as harmonised regulatory requirements.
WP7 is responsible for strengthening the collaboration between RARE-Bestpractices and IRDiRC. To date some of the RARE-Bestpractices partners are already involved in IRDiRC: ISS and BAPES are members of IRDiRC Interdisciplinary Scientific Committee and NIH-ORDR and OPHG, Project Advisors, are members of the Executive Committee. The close partnership and collaboration with IRDiRC is crucial, as it would deliver great opportunities for RARE-Bestpractices to achieve its objectives and increase the project added value in long term globally.

Description of work and role of partners

Task 7.1 (1 - 48) – Participate in IRDiRC meetings – RARE-Bestpractices Consortium will collaborate with IRDiRC and will be represented, in IRDiRC meetings, by ISS and BAPES; this participation will allow to actively exchange information.

ISS and BAPES are in charge of this task in collaboration with Project Advisory Institutions NIH-ORDR and OPHG. Contributions are also awaited also from all the other beneficiaries.

Task 7.2 (6 - 48) - Disseminate "best practice" documents – The collaboration with IRDiRC will allow to facilitate global sharing of best practices, standard operating procedures on RD research and BP guidelines, and encouraging harmonized policies.

ISS and BAPES are in charge of this task in collaboration with Project Advisory Institutions NIH-ORDR and OPHG. Contributions are envisaged from all Beneficiaries.

Task 7.3 (1 - 12) - Identify common communication channels – RD disease-orientated scientific journals, researchers, professional societies, and patient advocacy groups need to be engaged in discussing potential policies to support public data release and the sharing of best practices resources. Specific criteria and methods will be identified in collaboration with IRDiRC while communication strategies will be implemented under WP6.

ISS and BAPES are in charge of this task in collaboration with Project Advisory Institutions NIH-ORDR and OPHG. Contributions are envisaged from all Beneficiaries

Task 7.4 (6 - 48) – Contribute to the creation of a working group of representatives of all projects funded within the scope of IRDiRC - RARE-Bestpractices will contribute, in the ambit of its participation in IRDiRC, to ensure synergies between all research projects within the scientific area by exchanging results, expertise, experiences, resources (such as study designs, data analysis, data management tools, etc) and information.

ISS and BAPES are in charge of this task in collaboration with Project Advisory Institutions NIH-ORDR and OPHG. Contributions are envisaged from all Beneficiaries.

Person-Months per Participant

Participant number [10]	Participant short name [11]	Person-months per participant
1	ISS	3.00
10	BAPES	2.00
	Total	5.00

List of deliverables

Delive-rable Number [61]	Deliverable Title	Lead benefi-ciary number	Estimated indicative person-months	Nature [62]	Dissemi-nation level [63]	Delivery date [64]
D7.1	Report from IRDiRC meetings #1	1	0.50	R	RE	12
D7.2	Report from IRDiRC meetings #2	1	0.50	R	RE	24
D7.3	Report from IRDiRC meetings #3	1	0.50	R	RE	36
D7.4	Report from IRDiRC meetings #4	1	0.50	R	RE	48
		Total	2.00			

Description of deliverables

D7.1) Report from IRDiRC meetings #1: A report from attended IRDiRC meetings will be submitted-Linked to task 7.1 [month 12]

D7.2) Report from IRDiRC meetings #2: A report from attended IRDiRC meetings will be submitted-Linked to task 7.1 [month 24]

D7.3) Report from IRDiRC meetings #3: A report from attended IRDiRC meetings will be submitted-Linked to task 7.1 [month 36]

D7.4) Report from IRDiRC meetings #4: A report from attended IRDiRC meetings will be submitted-Linked to task 7.1 [month 48]

Schedule of relevant Milestones

Milestone number [59]	Milestone name	Lead benefi-ciary number	Delivery date from Annex I [60]	Comments
MS22	Contribution to the Communication Strategy (WP6) delivered	1	12	Document available and transmitted to WP6 members

WT3:
Work package description

Project Number [1]	305690	Project Acronym [2]	RARE-Bestpractices

One form per Work Package

Work package number [53]	WP8	Type of activity [54]	MGT
Work package title	Project management		
Start month	1		
End month	48		
Lead beneficiary number [55]	6		

Objectives

WP8 is responsible for providing the technical management of the project in terms of people, processes and resources.

Description of work and role of partners

Task 8.1 (1 - 48) - Perform Daily Management – This task will be under the responsibility of a Project Management Team (PMT), an operative team acting as a staff body of the Steering Committee to guarantee operational and daily support to all the Beneficiaries in technical, economic and administrative issues. The following activities will be performed:

o work plan management (control and update);
o timely submission of deliverables (after verification of completeness and uniformity);
o facilitating communication among partners;
o support to meetings organization and meeting minutes production;
o support to partners in economic and administrative issues – this will be assured also through the production of short and comprehensive guides and personalized templates for economic reporting (based on the documents produced by the Commission);
o management of contractual and other legal issues related to the project (in particular, Grant Agreement (GA) and Consortium Agreement (CA) implementation and amendments, but also subcontracts and external collaborations).
A Document Management System will be set up to categorize, store, management and share of documents, amendments to allow collaborative working (see WP2);

CNR is in charge of this task with the support of ISS.

Task 8.2 (1 - 48) - Prepare reports and manage administrative issues - It is devoted to:

o Economic Management: cost controlling and justification, budget management, EC contribution distribution;
o Periodic Reporting: all the appropriate instruments will be set up to allow an efficient collection and organization of project data to fulfill in the request of the Commission in terms of reporting; this will include internal periodic interim reporting.

CNR is in charge of this task with the support of ISS. All Beneficiaries are involved too.

Task 8.3 (1 - 48) - Manage intellectual property rights (IPR) issues - IP and exploitation of results will be addressed within the CA in accordance with the indications reported in the Rules for Participation and in the EC model Grant Agreement with a specific attention to:

o Access to background knowledge – the internal access to any knowledge produced prior to the project starting will be ruled within the Consortium Agreement;
o Property and Use of foreground - the project results in terms of deliverables will be freely accessible while additional services of training and consultancy will be one of the pillars for the sustainability of the platform. Specific rules will be defined.

ISS will be responsible for this task with the support of CNR. Collaboration is envisaged from all the Beneficiaries.

Person-Months per Participant

Participant number [10]	Participant short name [11]	Person-months per participant
1	ISS	5.10
6	CNR	6.00
	Total	11.10

List of deliverables

Delive-rable Number [61]	Deliverable Title	Lead benefi-ciary number	Estimated indicative person-months	Nature [62]	Dissemi-nation level [63]	Delivery date [64]
D8.1	Management plans and tools	6	2.00	O	CO	3
D8.2	IPR policy	1	2.00	O	CO	12
		Total	4.00			

Description of deliverables

D8.1) Management plans and tools: This will include the Activity Monitoring and Document Approval Procedure, the Change Management Plan, the Risk Management Plan, the internal Communication Plan - Linked to Task 8.1. [month 3]

D8.2) IPR policy: Specific addendum to the Consortium Agreement for IPR management - Linked to Task 8.3. [month 12]

Schedule of relevant Milestones

Milestone number [59]	Milestone name	Lead benefi-ciary number	Delivery date from Annex I [60]	Comments
MS23	Role Assignment Matrix (RAM) defined (including all the managing bodies)	6	3	RAM fully completed and approved by the SC
MS24	Templates available (also for interim reports)	6	3	Document available

WT4:
List of Milestones

Project Number [1]	305690	Project Acronym [2]	RARE-Bestpractices

List and Schedule of Milestones

Milestone number [59]	Milestone name	WP number [53]	Lead beneficiary number	Delivery date from Annex I [60]	Comments
MS1	Meetings #1 agenda sent to beneficiaries	WP1	1	1	Document available for consultation
MS2	Meetings #2 agenda sent to beneficiaries	WP1	1	11	Document available for consultation
MS3	Meetings #3 agenda sent to beneficiaries	WP1	1	23	Document available for consultation
MS4	Meetings #4 agenda sent to beneficiaries	WP1	1	35	Document available for consultation
MS5	Meetings #5 agenda sent to beneficiaries	WP1	1	47	Document available for consultation
MS6	Training activities plan available	WP1	8	20	Training activities plan ready
MS7	Validation from beneficiaries of strategic sustainability plan	WP1	1	40	Validation available
MS8	BP guideline database prototype ready	WP2	2	4	Website & report
MS9	RR database prototype ready	WP2	2	22	Website & report
MS10	Collections underway	WP2	2	23	Website & report
MS11	Sanity check of databases	WP2	2	23	Website & report
MS12	Survey to gain information on BP guidelines or HTA programmes dedicated to RD in EU MS	WP3	8	5	Survey available on the Project WEB site for on-line access
MS13	First workshop held to identify challenges in developing RD guidelines	WP3	9	6	Minutes of the workshop available
MS14	Second workshop held to finalize BP guidelines methodology	WP3	9	15	Minutes of the workshop available
MS15	Specific rare condition identified for the development	WP3	3	24	Report on motivations available

WT4:
List of Milestones

Milestone number [59]	Milestone name	WP number [53]	Lead beneficiary number	Delivery date from Annex I [60]	Comments
	of a new pilot BP guideline				
MS16	Approval of criteria	WP4	4	15	Criteria will be validated by the General Assembly
MS17	Approval of the draft of the metalevel guidelines	WP4	4	42	Metalevel guidelines will be validated by the General Assembly
MS18	Review of the available literature on value assessment in general and specifically to RD completed	WP5	5	5	Intermediate report available
MS19	Orphan drug appraisals across the EU study MS over the 2006 – 2012 period selected and DB realised	WP5	5	11	Creation of a database
MS20	Synthesis available of value assessment criteria and identification of BP	WP5	5	18	Final report
MS21	Periodic electronic journal structure approved	WP6	10	6	Structure of the Journal approved by the SC
MS22	Contribution to the Communication Strategy (WP6) delivered	WP7	1	12	Document available and transmitted to WP6 members
MS23	Role Assignment Matrix (RAM) defined (including all the managing bodies)	WP8	6	3	RAM fully completed and approved by the SC
MS24	Templates available (also for interim reports)	WP8	6	3	Document available

WT5:
Tentative schedule of Project Reviews

Project Number [1]	305690	Project Acronym [2]	RARE-Bestpractices

Tentative schedule of Project Reviews

Review number [66]	Tentative timing	Planned venue of review	Comments, if any

WT6:
Project Effort by Beneficiary and Work Package

Project Number [1]	305690	Project Acronym [2]	RARE-Bestpractices

Indicative efforts (man-months) per Beneficiary per Work Package

Beneficiary number and short-name	WP 1	WP 2	WP 3	WP 4	WP 5	WP 6	WP 7	WP 8	Total per Beneficiary
1 - ISS	12.00	4.40	8.00	4.00	3.00	11.10	3.00	5.10	50.60
2 - JAMARAU	4.10	13.00	3.00	2.00	0.00	0.00	0.00	0.00	22.10
3 - KI	3.10	2.00	7.00	4.00	0.00	0.00	0.00	0.00	16.10
4 - HIS	1.00	1.00	2.00	6.30	0.00	0.00	0.00	0.00	10.30
5 - LSE	2.00	0.00	0.00	0.00	17.20	0.00	0.00	0.00	19.20
6 - CNR	2.00	0.00	12.30	0.00	0.00	1.00	0.00	6.00	21.30
7 - EURORDIS	10.00	0.00	11.50	0.00	0.00	0.50	0.00	0.00	22.00
8 - AREAS-CCI	0.00	0.00	4.40	0.00	0.00	3.70	0.00	0.00	8.10
9 - UKLFR	1.00	0.00	3.50	2.00	0.00	0.00	0.00	0.00	6.50
10 - BAPES	1.00	0.00	2.00	0.00	0.00	7.00	2.00	0.00	12.00
11 - FUNCIS	2.00	0.00	10.00	0.00	0.00	0.00	0.00	0.00	12.00
12 - UM	1.00	0.00	0.00	4.00	0.00	0.00	0.00	0.00	5.00
13 - UNEW	4.00	0.00	0.00	0.00	0.00	0.00	0.00	0.00	4.00
14 - EAP/UEMS-SP	0.50	0.00	0.50	0.00	0.00	1.00	0.00	0.00	2.00
15 - ISCIII	2.00	0.00	10.00	0.00	0.00	0.00	0.00	0.00	12.00
Total	45.70	20.40	74.20	22.30	20.20	24.30	5.00	11.10	223.20

WT7:
Project Effort by Activity type per Beneficiary

Project Number [1]	305690	Project Acronym [2]	RARE-Bestpractices

Indicative efforts per Activity Type per Beneficiary

Activity type	Part. 1 ISS	Part. 2 JAMARAU	Part. 3 KI	Part. 4 HIS	Part. 5 LSE	Part. 6 CNR	Part. 7 EURORDI	Part. 8 AREAS-C	Part. 9 UKLFR	Part. 10 BAPES	Part. 11 FUNCIS	Part. 12 UM	Part. 13 UNEW	Part. 14 EAP/ UEM
3. Consortium Management activities														
WP 8	5.10	0.00	0.00	0.00	0.00	6.00	0.00	0.00	0.00	0.00	0.00	0.00	0.00	0.00
Total Management	5.10	0.00	0.00	0.00	0.00	6.00	0.00	0.00	0.00	0.00	0.00	0.00	0.00	0.00
Work Packages for Coordination activities														
WP 1	12.00	4.10	3.10	1.00	2.00	2.00	10.00	0.00	1.00	1.00	2.00	1.00	4.00	0.50
WP 2	4.40	13.00	2.00	1.00	0.00	0.00	0.00	0.00	0.00	0.00	0.00	0.00	0.00	0.00
WP 3	8.00	3.00	7.00	2.00	0.00	12.30	11.50	4.40	3.50	2.00	10.00	0.00	0.00	0.50
WP 4	4.00	2.00	4.00	6.30	0.00	0.00	0.00	0.00	2.00	0.00	0.00	4.00	0.00	0.00
WP 5	3.00	0.00	0.00	0.00	17.20	0.00	0.00	0.00	0.00	0.00	0.00	0.00	0.00	0.00
WP 7	3.00	0.00	0.00	0.00	0.00	0.00	0.00	0.00	0.00	2.00	0.00	0.00	0.00	0.00
Total Coordination	34.40	22.10	16.10	10.30	19.20	14.30	21.50	4.40	6.50	5.00	12.00	5.00	4.00	1.00
4. Other activities														
WP 6	11.10	0.00	0.00	0.00	0.00	1.00	0.50	3.70	0.00	7.00	0.00	0.00	0.00	1.00
Total other	11.10	0.00	0.00	0.00	0.00	1.00	0.50	3.70	0.00	7.00	0.00	0.00	0.00	1.00
Total	50.60	22.10	16.10	10.30	19.20	21.30	22.00	8.10	6.50	12.00	12.00	5.00	4.00	2.00

WT7:
Project Effort by Activity type per Beneficiary

Activity type	Part. 15 ISCIII	Total
3. Consortium Management activities		
WP 8	0.00	11.10
Total Management	0.00	11.10
Work Packages for Coordination activities		
WP 1	2.00	45.70
WP 2	0.00	20.40
WP 3	10.00	74.20
WP 4	0.00	22.30
WP 5	0.00	20.20
WP 7	0.00	5.00
Total Coordination	12.00	187.80
4. Other activities		
WP 6	0.00	24.30
Total other	0.00	24.30
Total	12.00	223.20

WT8:
Project Effort and costs

Project Number [1]	305690	Project Acronym [2]	RARE-Bestpractices

Project efforts and costs

Beneficiary number	Beneficiary short name	Estimated eligible costs (whole duration of the project)						Total receipts (€)	Requested EU contribution (€)
		Effort (PM)	Personnel costs (€)	Subcontracting (€)	Other Direct costs (€)	Indirect costs OR lump sum, flat-rate or scale-of-unit (€)	Total costs		
1	ISS	50.60	260,562.00	23,000.00	348,800.00	121,872.40	754,234.40	0.00	675,016.00
2	JAMARAU	22.10	168,262.00	0.00	28,000.00	39,252.40	235,514.40	0.00	210,000.00
3	KI	16.10	95,387.00	0.00	44,800.00	28,037.40	168,224.40	0.00	150,000.00
4	HIS	10.30	82,950.00	0.00	29,200.00	22,430.00	134,580.00	0.00	120,000.00
5	LSE	19.20	98,050.00	0.00	14,100.00	22,430.00	134,580.00	0.00	120,000.00
6	CNR	21.30	80,186.00	0.00	8,600.00	54,894.00	143,680.00	0.00	95,000.00
7	EURORDIS	22.00	139,810.00	0.00	17,200.00	31,402.00	188,412.00	0.00	168,000.00
8	AREAS-CCI	8.10	46,259.00	0.00	47,200.00	18,691.80	112,150.80	0.00	100,000.00
9	UKLFR	6.50	28,275.00	0.00	27,800.00	11,215.00	67,290.00	0.00	60,000.00
10	BAPES	12.00	38,876.00	0.00	28,400.00	13,455.20	80,731.20	0.00	71,984.00
11	FUNCIS	12.00	38,876.00	0.00	17,200.00	11,215.20	67,291.20	0.00	60,000.00
12	UM	5.00	23,784.00	0.00	13,600.00	29,533.00	66,917.00	0.00	40,000.00
13	UNEW	4.00	27,784.00	0.00	9,600.00	7,476.80	44,860.80	0.00	40,000.00
14	EAP/UEMS-S	2.00	10,838.00	0.00	17,200.00	5,607.60	33,645.60	0.00	30,000.00
15	ISCIII	12.00	38,875.00	0.00	17,200.00	11,215.00	67,290.00	0.00	60,000.00
	Total	223.20	1,178,774.00	23,000.00	668,900.00	428,727.80	2,299,401.80	0.00	2,000,000.00

1. Project number

The project number has been assigned by the Commission as the unique identifier for your project. It cannot be changed. The project number **should appear on each page of the grant agreement preparation documents (part A and part B)** to prevent errors during its handling.

2. Project acronym

Use the project acronym as given in the submitted proposal. It cannot be changed unless agreed so during the negotiations. The same acronym **should appear on each page of the grant agreement preparation documents (part A and part B)** to prevent errors during its handling.

53. Work Package number

Work package number: WP1, WP2, WP3, ..., WPn

54. Type of activity

For all FP7 projects each work package must relate to one (and only one) of the following possible types of activity (only if applicable for the chosen funding scheme – must correspond to the GPF Form Ax.v):

• **RTD/INNO =** Research and technological development including scientific coordination - applicable for Collaborative Projects and Networks of Excellence

• **DEM =** Demonstration - applicable for collaborative projects and Research for the Benefit of Specific Groups

• **MGT =** Management of the consortium - applicable for all funding schemes

• **OTHER =** Other specific activities, applicable for all funding schemes

• **COORD =** Coordination activities – applicable only for CAs

• **SUPP =** Support activities – applicable only for SAs

55. Lead beneficiary number

Number of the beneficiary leading the work in this work package.

56. Person-months per work package

The total number of person-months allocated to each work package.

57. Start month

Relative start date for the work in the specific work packages, month 1 marking the start date of the project, and all other start dates being relative to this start date.

58. End month

Relative end date, month 1 marking the start date of the project, and all end dates being relative to this start date.

59. Milestone number

Milestone number:MS1, MS2, ..., MSn

60. Delivery date for Milestone

Month in which the milestone will be achieved. Month 1 marking the start date of the project, and all delivery dates being relative to this start date.

61. Deliverable number

Deliverable numbers in order of delivery dates: D1 – Dn

62. Nature

Please indicate the nature of the deliverable using one of the following codes

R = Report, **P** = Prototype, **D** = Demonstrator, **O** = Other

63. Dissemination level

Please indicate the dissemination level using one of the following codes:

• **PU =** Public

• **PP =** Restricted to other programme participants (including the Commission Services)

• **RE =** Restricted to a group specified by the consortium (including the Commission Services)

• **CO =** Confidential, only for members of the consortium (including the Commission Services)

• **Restreint UE =** Classified with the classification level "Restreint UE" according to Commission Decision 2001/844 and amendments

• **Confidentiel UE =** Classified with the mention of the classification level "Confidentiel UE" according to Commission Decision 2001/844 and amendments

• **Secret UE =** Classified with the mention of the classification level "Secret UE" according to Commission Decision 2001/844 and amendments

64. Delivery date for Deliverable

Month in which the deliverables will be available. Month 1 marking the start date of the project, and all delivery dates being relative to this start date

65. Review number

Review number: RV1, RV2, ..., RVn

66. Tentative timing of reviews

Month after which the review will take place. Month 1 marking the start date of the project, and all delivery dates being relative to this start date.

67. Person-months per Deliverable

The total number of person-month allocated to each deliverable.

Platform for sharing best practices for management of rare diseases
RARE-Bestpractices

Coordination and Support Actions
(COORDINATING)

Ref. Number: 305690
Description of Work – Part B