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Building Consensus and Synergies for the EU Registration of Rare Disease Patients

JA2015 - GPSD [705038]

START DATE: 16/04/2011

END DATE: 16/04/2014

DURATION: 36 month(s)

CURRENT STATUS: Finalised

PROGRAMME TITLE: Second Programme of Community action in the Field of Health 2008-2013

PROGRAMME PRIORITY: -

CALL: Promote Health (Hp-2010)

TOPIC: PROMOTE HEALTH (HP-2010)

EC CONTRIBUTION: 661402 EUR

KEYWORDS: Diagnostic, Health system, Patients registries, Quality evaluation, Rare diseases and disorders

PORTFOLIO: Rare diseases

General objectives

The general objective of this initiative is to build consensus and synergies to address regulatory, ethical and technical issues associated with the registration of RD patients and to elaborate possible policy scenarios. Specific attention will be given to the scenario of the creation of a EU platform for the collection of data on RD patients and their communication among qualified users, based on a feasibility study. To this aim, the project will define the options for the preparation of a legal basis, the possible scopes to achieve most effective synergies, the corresponding governance framework and possible options for sustainability. The feasibility of registration of a minimum data set common to all rare diseases, designed to inform policy-making, the conditions to admit research-driven disease or treatment-specific modules and the ways to ensure a sustainable data flow will be assessed.

Strategic relevance and contribution to the public health programme

The development of guiding reports, including the legal and organizational framework, for the registration of RD patients is strategic to build up an evidence base for Community, public health policies, health service management, clinical research and the assessment of orphan drugs effectiveness and appropriateness of use. The successful establishment of a EU registration of health data, made for RDs, may represent an important example paving the way to the EU-wide registration of data regarding other health conditions. The project prepares the ground for the collection of an agreed common set of data from RD patients providing also rules to ensure data quality and data validation and improve as far as possible comparability of data among countries. The adoption of an EU legal basis may provide more statistical power to evidence for EU policies, epidemiological investigations and research. The project intends to act as the Scientific Secretariat of an EUCERD WG on Registries and DataBases to integrate the project in policy development for RD. The ISS and EURORDIS will co-chair the EUCERD WG.

Methods and means

(1) A survey will be carried out through the existing registries located at universities, and among the patients associations, to identify the needs and interests. (2) The most suitable legal instrument will be analyzed to provide sound operational indications for the EU Commission to start the process leading to the adoption of an EU-level legal basis. (3) The sustainability of EU RD patients registration will be pursued by defining the aims and the scope of the platform and appropriate governance models. (4) A common data set is developed consistently with the public health aims; use of different data sets is considered. (5) The services to be made available through the platform will be identified; offering practical instruments to

carry out usual tasks for the registry users and facilitate communication with the platform; providing public reports; and fulfilling specific needs of the registry users, as appraised from the surveys. Special attention is given to the involvement of all relevant stakeholders to foster the implementation and sustainability of its recommendations.

Expected outcomes period

The dissemination strategy, the link with a specific Working Group (WG) of the EUCERD and the project deliverables will set in motion a mechanism for the approval of the legal base for the EU registration of RD patients. Indeed it will provide the European Commission with all the elements to draft a legislative proposal and submit it to the Council and Parliament. The advantages are obvious. If the legislation enters into force it will allow the pooling of data from the EU member states. However, even in the case that the legislation is not approved, the project deliverables and the consensus built up with this project will result in beneficial effects. Indeed, the project will provide common ground, resulting from wide ranging debate and consensus, for the national health authorities to adopt a legal basis, which is for sure in their legal remit. Moreover, the European Commission could develop and provide the services and facilities described in the project deliverables and tailored to the national health institutions establishing a registry and to those of current “private” registries and databases.

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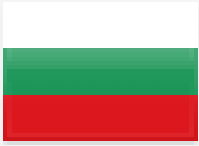


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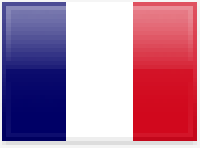
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D01 - The current situation, the challenges and the expectations on Patient registries and Database (EN)

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http://azorina.cc.cec.eu.int:8082/publications/20101202/20101202_d01_d05_oth_en_ps.pdf

D02 - Best practice model of a legal instrument for a European Platform of EU RD registries (EN)

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D03 - Possible policy scenarios on Scope, Common data set, Governance and Sustainability for RD Patients registration (EN)

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D04 - Guidelines for data sources and quality for RD Registries in Europe (EN)

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D05 - Identification and characterization of services and facilities of a European Platform of EU RD Registries (EN)

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D06 - Scientific secretariat of an EUCERD WG on registries and databases (co-chair ISS and EURODIS) (EN)

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D07 - Promotional material website, and activities intended for dissemination at conferences and distributed to stakeholders (EN)

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D08 - European workshop with stakeholders to discuss the possible policy scenarios (EN)

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http://azorina.cc.cec.eu.int:8082/publications/20101202/20101202_d8_00_oth_en_ps.pdf

D09-01 - Report on common data sets and disease treatment and other specific modules (EN)

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http://azorina.cc.cec.eu.int:8082/publications/20101202/20101202_d09_02_ir1_en_is.pdf

D09-02 - Analysis of survey on registry data elements (EN)

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http://azorina.cc.cec.eu.int:8082/publications/20101202/20101202_d09_02_oth_en_ps.pdf

D10-01 - Final report (EN)

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http://azorina.cc.cec.eu.int:8082/publications/20101202/20101202_d10_01_fr_en_ps.pdf

D10-02 - Evaluation report (EN)

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D10-03 - Summary final report (EN)

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Final report (EN)

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