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THINKING OUT OF THE BOX: NATIONAL PLANS WITHOUT DEDICATED FUNDING

YANN LE CAM
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FUNDING RD NATIONAL PLANS: WHAT THEY SAY

"Improving the health care of rare disease patients implies ***allocation of funds for the delivery of improved health and social services over the long term****, as well as funding to implement the adaptation of the health system, including the establishment of new structures and/or new tasks,* ***to respond to the unfulfilled needs of rare disease patients."***

EUROPLAN Recommendations, 2010

BUT …

"The financial crisis in Greece is continuing to progressively undermine many aspects of the public sector, including healthcare*, and since December 2012 there have already been some changes with respect to certain financial and legal aspects relating to healthcare, hospitalization, medicinal products etc."*

Final Report of the 2nd EUROPLAN Greek National Conference, December 2012

"The crisis has left a few countries with little or no choice but to introduce cuts"

WHO Report "Health policy responses to the financial crisis in Europe", 2012

AND IF WE TRIED TO THINK "OUT OF THE BOX"?

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TURNING THE QUESTION

→ In Europe: overall financial crisis affecting the public sector, specifically health systems

→ National policy responses: all seems somehow to put in danger :

- Overall the "*allocation of funds for the delivery of improved health and social services over the long term*"
- Specifically the development and/or the implementation of Rare Disease National Plans

> *"Improving the health care of rare disease patients implies* ***allocation of funds for the delivery of improved health and social services over the long term****, as well as funding to implement the adaptation of the health system, including the establishment of new structures and/or new tasks,* ***to respond to the unfulfilled needs of rare disease patients****."*
>
> EUROPLAN Recommendations, 2010

→ Turning the question in a different way :

How ***"to respond to the unfulfilled needs of rare disease patients"*** without additional sources of funding ?

(A) "DOING SMARTER, COOPERATE BETTER"

*"The time of crisis is **a time for reflection**, a time to stop and see what does not work and **set the foundation for a more effective and efficient work in the future**"*

Rosa Sánchez de Vega, EURORDIS Vice President, FEDER Federacion Española de Enfermedades Raras

"Not every step of progress needs funding.
*It is also a question of **doing smarter** & **cooperate better**"*

Birthe Holm, Rare Disorders Denmark

(A) "DOING SMARTER, COOPERATE BETTER"

"Surely ***we want National Plans for Rare Disease with actions that are properly financed*** *to implement them.*

However ***a National Plan without a visible "dedicated funding" can still be a very good National Plan with proper financing*** *for the actions adopted.*

We can promote ***measures making a better use of existing public money****.*

We can promote important ***measures which are less visible in term of budget impact*** *so easier to adopt by policy makers & top officials.*
The key success factor for that is in our ability to work closely and trustfully with willing and dedicated civil servants in the national health authorities"

Yann Le Cam, EURORDIS Chief Executive Officer & EUCERD Vice President & EUROPLAN Partner

DOING SMARTER (1) – HOW TO?

→ During crises, everything is under scrutiny. An opportunity for our health and social authorities to evaluate how money is spent and how to spend it better. As citizen & tax payers it is a good thing. As RD patient advocates, it's often though but can create new opportunities.

→ The following are essentials for "thinking & doing smarter":

- **Stimulate the Commitment & Willingness to take action to address the challenges of people living with rare diseases** – despite the negative economic downturn there should be commitment of health and social authorities to take possible actions within current situation
- **Be ready to do less now and focus on your building blocks** – rather on focus on few areas of actions & measures which are essential for patients, within the EU policy framework & EUCERD recommendations, and useful to build the case of RD policy
- **Prepare for the Future** – we do not expect all actions to be done now, but it is necessary to plan and organise for the future with clear steps and time schedule.

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DOING SMARTER (2) – HOW TO?

→ **Monitoring mechanisms**

→ **Cost-effectiveness and accountability**

“WHO Report – Health policy response to the financial crisis in Europe”
Policy options “that are **likely to enhance efficiency** and will not have an adverse impact on, or **may even promote, other health system goals**”:

- Mechanisms including **quality indicators, patient-reported outcome measures** and other forms of feedback
- **HTA to assist in setting priorities**, combined with **accountability, monitoring and transparency measures**
- **Price reductions for pharmaceuticals combined with cost–effectiveness evidence** and other measures to promote rational prescribing and dispensing
- **Reducing administrative costs** while maintaining capacity to manage the health system

DOING SMARTER (3) – USE THE TRENDS

→ **Community-care**

→ **Integration of health & social services**

“WHO Report – Health policy response to the financial crisis in Europe”

Policy options “that are likely to **enhance efficiency** and will not have an adverse impact on, or may even promote, other health system goals”:

[…]

- **Shifting from inpatient to day-care or ambulatory care**
- **Integration and coordination of primary care & secondary care, and of health & social care**

also:

- « To preserve quality of care, it is clear that reducing acute care capacity must be accompanied by **investment in alternative services, such as community-based care** (or “hospital reconfiguration ”) »

POLICY SUMMARY 5

Health policy responses to the financial crisis in Europe

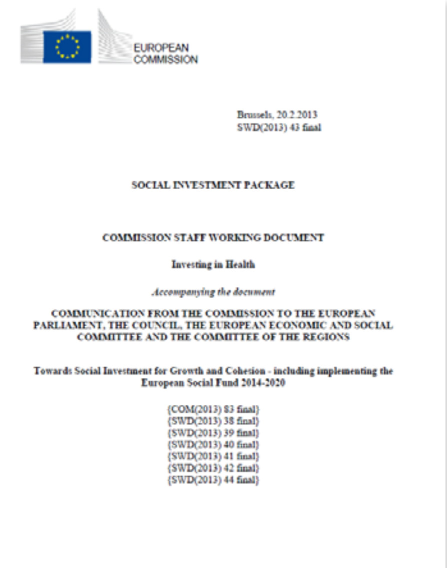
Philipa Mladovsky, Divya Srivastava, Jonathan Cylus, Marina Karanikolos, Tamás Evetovits, Sarah Thomson, Martin McKee

→ **WHO REPORT**: http://www.euro.who.int/__data/assets/pdf_file/0009/170865/e96643.pdf

DOING SMARTER (3) – NEW FINANCING

→ **Community-care**

→ **Integration of health & social services**



EUROPEAN COMMISSION

Brussels, 20.2.2013
SWD(2013) 43 final

SOCIAL INVESTMENT PACKAGE

COMMISSION STAFF WORKING DOCUMENT

Investing in Health

Accompanying the document

COMMUNICATION FROM THE COMMISSION TO THE EUROPEAN PARLIAMENT, THE COUNCIL, THE EUROPEAN ECONOMIC AND SOCIAL COMMITTEE AND THE COMMITTEE OF THE REGIONS

Towards Social Investment for Growth and Cohesion - including implementing the European Social Fund 2014-2020

{COM(2013) 83 final}
{SWD(2013) 38 final}
{SWD(2013) 39 final}
{SWD(2013) 40 final}
{SWD(2013) 41 final}
{SWD(2013) 42 final}
{SWD(2013) 44 final}

“SOCIAL INVESTMENT PACKAGE - Commission Staff Working Document “Investing in Health”

« The **EU Cohesion and Structural Funds should co-finance investments in health by the Member States** that follow a coherent, strategic policy approach by:

- investing in health infrastructure that fosters a **transformational change in the health system**, in particular reinforcing the **shift from a hospital-centred model to community-based care and integrated services** »

→ **INVESTING IN HEALTH**: http://www.euro.who.int/__data/assets/pdf_file/0009/170865/e96643.pdf

DOING SMARTER (3) – BEING LESS VISIBLE

→ Exemple 1: Financing for Centres of Expertise

"Asking visible budget to create Centres of Expertise for RDs may not the best way to obtain maximum financing" Yann Le Cam

The financing of CoE for RDs can be justified and promoted with four measures:

- **Shifting from hospital inpatient to hospital day-care or ambulatory care** (this saves a lot of public money)
- **Financing RD multidisciplinary consultations thanks to their coordination on same day so to pay with hospital day-care rate** (this brings money to the hospital & the RD Centre of Expertise to pay for staff and longer consultations)
- **Asking for the cost of diagnosis & care of all complex diseases & multidisciplinary medical consultation to be paid at higher rates than regular hospital consultations** (idem + better health outcome & reduction of healthcare costs)
- **Integration and coordination of primary care & secondary care, and of health & social care** (this optimise the use of public resources)

DOING SMARTER (3) – BEING LESS VISIBLE

→ Exemple 2: Financing patients’ access to Orphan Medicinal Products

“This was probably the most important financing measures in the 1st French National Plan for Rare Diseases” **Yann Le Cam**

The financing of orphan drugs should not be on the hospital pharmacist’s budget:

- The cost of orphan drugs is a burden for the hospital pharmacist who has a limited budget to mange
- It raise unethical questions of deciding for which patients to pay drugs or not
- It is a des-incentive for hospital to create RD CoE

The financing of orphan drugs should be on regional or national budgeting:

- Solidarity of healthcare budget should pay for orphan drugs
- Financing should come from national level or, at worst, regional level with a national coordination – not county or hospital

-> This measure is recommended in EUROPLAN and in the EU Report of Mechanisms for a Coordinated Access to Orphan Medicinal Products, approved by all EU Members States in April 2013

DOING SMARTER (4) – PPP

→ **Public private partnerships**

> ***"We have no doubt that any progress in developing a National Plan for Ireland*** *under the EUCERD Recommendations and those ascertained thought the consultation process on our National Plan development in 2012,* ***will have to be a public/private partnership with emphasis on private"***
>
> Avril Daly, Fighting Blindness, GRDO - Genetic and Rare Disorders Organisation, Ireland, EURORDIS Vice President

→ Involving private funding seems to be unavoidable in many countries

→ However :

- It is necessary to **maintain control mechanisms to ensure that the public interest is preserved**
- "PPP may act as a barrier to collaboration between facilities offering complementary services to a defined population, leading to fragmentation and duplication"

WHO Report – Health policy response to the financial crisis in Europe

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DOING SMARTER (4) – PPP

→ **Public private partnerships**

3 Exemples

→ **HELPLINES** : run by National Alliances with the support of public or corporate funding

→ **THERAPEUTIC EDUCATION OF PATIENTS AND FAMILIES**

→ **CO-FINANCING OF CLINICAL RESEARCH ACTIVITIES PUBLIC - INDUSTRY-** based on specifications and rules

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DOING SMARTER (4) – PPP

→ **Public private partnerships**

3 Counter Exemples

→ **ENZYME THERAPY REPLACEMENT** : with a nurse service at home provided by the hospital or by the company?

→ **GENE TESTING OUTSOURCED:** to platform in the USA or through Internet in China… our information out there without any control…

→ **DATA COLLECTION:** NIH to outsource to private companies:

Risks: - Use of data : no or little control

- for profit business model
- lack sustainability

DOING SMARTER (5) - PRESERVING

→ **Preserving what has already been achieved**

> *"In Denmark, most fo the specialised counselling services in the social field that we had have been integrated into 'mainstream' initiatives, with little left of the original expertise, focus and dedicated staff"*
>
> Terkel Andersen, Danish Haemophilia Society, EURORDIS President,

→ What we are experiencing:

- Cuts to existing investments or budget engagements for specififc RD actions
- Slimmed down actions
- Absorption of dedicated services into the general realignment of all services so that they become les distinct

→ However, **at a very least, we should expect not lo lose what we have achieved so far => be clear about what is specific & what is achieved**

→ The long-term effectiveness of interventions for health and social care for PLWRD should be regularly restated

COOPERATE BETTER

> *"The participation of patients is essential in the designation of Centres of Expertise, because professionals, hospital managers and even regional health administrations have other very different interests when designating a CE. I insist, the participation of patients is essential in every level."*
>
> Rosa Sánchez de Vega, EURORDIS Vice President, FEDER Federacion Española de Enfermedades Raras

→ Patients know better what is worth doing and makes a difference in their daily life

→ Cooperating better with patients means greater efficacy and cost-effectiveness of policy choices

→ RD Patients' advocates are the catalysts of the partnership between all stakeholders and they are triggering the awareness & policy process

→ So far, our best achievements in RD policy and current policy development are when there is good cooperation and partnership

(B) "NOT EVERY STEP NEEDS FUNDING"

> *"**Not every step of progress needs funding**.*
> *It is also a question of doing smarter & cooperate better"*
>
> Birthe Holm, Rare Disorders Denmark

→ ***Not every step needs funding***

Specific measures that are virtually cost free

VIRTUALLY COST-FREE MEASURES

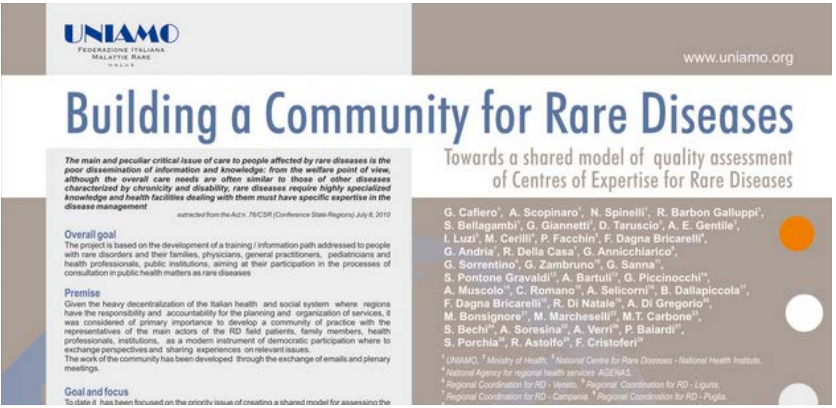
1. National Working Group for the designation of CEs

→ A WG at national level with the participation of the NA

→ Aimed to identify criteria and real expertise in different RDs & eventually develop a model of quality assessment for RD CEs

→ First steps for later designation with quality criteria:

- Mapping expertise by crossing data: Orphanet + patient information + hospital information
- Information could be collected via a survey
- Starting from a limited group of RDS as pilot and then extended to other pathologies. Exemple: UNIAMO 'Community' project



UNIAMO
FEDERAZIONE ITALIANA
MALATTIE RARE

www.uniamo.org

Building a Community for Rare Diseases

Towards a shared model of quality assessment of Centres of Expertise for Rare Diseases

G. Cafiero, A. Scopinaro, N. Spinelli, R. Barbon Galluppi, S. Bellagambi, G. Giannetti, D. Taruscio, A. E. Gentile, I. Luzi, M. Cerilli, P. Facchin, F. Dagna Bricarelli, G. Andria, R. Della Casa, G. Annicchiarico, G. Sorrentino, G. Zambruno, G. Sanna, S. Pontone Gravaldi, A. Bartuli, G. Piccinocchi, A. Muscolo, C. Romano, A. Selicorni, B. Dallapiccola, F. Dagna Bricarelli, R. Di Natale, A. Di Gregorio, M. Bonsignore, M. Marcheselli, M.T. Carbone, S. Bechi, A. Soresina, A. Verri, P. Baiardi, S. Porchia, R. Astolfo, F. Cristoferi

UNIAMO, Ministry of Health, National Centre for Rare Diseases - National Health Institute, National Agency for regional health services AGENAS, Regional Coordination for RD - Veneto, Regional Coordination for RD - Liguria, Regional Coordination for RD - Campania, Regional Coordination for RD - Puglia

The main and peculiar critical issue of care to people affected by rare diseases is the poor dissemination of information and knowledge: from the welfare point of view, although the overall care needs are often similar to those of other diseases characterized by chronicity and disability, rare diseases require highly specialized knowledge and health facilities dealing with them must have specific expertise in the disease management

extracted from the Act n. 76/CSR (Conference State Regions) July 8, 2010

Overall goal
The project is based on the development of a training / information path addressed to people with rare disorders and their families, physicians, general practitioners, pediatricians and health professionals, public institutions, aiming at their participation in the processes of consultation in public health matters as rare diseases

Premise
Given the heavy decentralization of the Italian health and social system where regions have the responsibility and accountability for the planning and organization of services, it was considered of primary importance to develop a community of practice with the representatives of the main actors of the RD field patients, family members, health professionals, institutions, as a modern instrument of democratic participation where to exchange perspectives and sharing experiences on relevant issues.
The work of the community has been developed through the exchange of emails and plenary meetings.

Goal and focus
To date it has been focused on the priority issue of creating a shared model for assessing the

Best practice:
'**Community for RD**' project: originally funded and developed by **UNIAMO FIMR** , then extended and taken on board by the National Agency for Regional Health Services

VIRTUALLY COST-FREE MEASURES

1. Other low cost measures for RD Hospital care

→ Evaluate the capacities of **diagnosis laboratories** so to organise provision of diagnosis at national level: reduce fragmentation, rather specialise and share the work in order to apply quality control procedure and maximise the use of available equipments with a critical mass of samples

→ Promote the use of **OrphaCode in hospital system** in addition to ICD…

→ Registries & Data collection by RD CoE should use the **core common data elements** (under development) to all RDs + the **specialised common data elements by disease or group of diseases**. All registries should be inter-operable

→ Publish National **Good Clinical Practice Guidelines for Diagnosis & Care** of RDs. Using & adapting the ones developed at EU level or by other EU MSs, or developping their own guidelines by the RD CoE using a common methodology (Exemple of the French HAS methodology + Ongoing EC project Rare-Best Practices by ISS with Eurordis)

→ Encourage RD CoE and national experts to be part of the **European Reference Networks for Rare Diseases**

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2. Working Group at the National Medicines Agency on OMPs or Rare Disease Treatments

→ With the participation of RD patients, notably the National Alliance, and possibly all stakeholders

→ To learn and find out about problems of access to OMPS, distribution, availability, coverage, shortages …

→ To discuss issues around off label use such as survey to list all medicines used off label for PLWRDs, or, collection of safety & efficacy data on off-label use

→ To stimulate participation of your MS in CAVOMP and MoCA for exchange of information on clinical added value of OMP, post-marketing research activities, common effectiveness reports, coordinate on the value and negotiations of price…

3. National survey to explore overall treatments and care that PLWRD need

→ Beyond OD, include physiotherapy, surgery, sanitary products

→ Argument: the first step in view of the coverage of a treatment is to know which treatments RD patients needs. We still lack information on this!

→ Potentially MoH funded: it a low-cost initiative

4. Training of professionals

→ Training solution for healthcare professionals are most needed in order to create awareness on RD and increase diagnostic , prognostic and care for PLWRD

→ Many low-cost solutions exist e.g.:

- Dedicated sessions on RD in initial curriculum at Medicine Schools & University
- Dedicated RD trainings at Medicine Schools and / or Continuing Medical Education
- Summer Camps; Exemple: Agrenska (Sweden) and NoRo (Romania)
- …

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4. Social Services and Integration of PLWRD

- → **Working groups at Ministry for Social Affairs & Labour** are necessary to create platforms for recognition of needs of social services for RD, discussing specificities of PLWRD and develop adequate services
- → **Working groups Social Affairs & Education** would be necessary, also in view of promoting child inclusion
- → Elaborate **good practices** between CoE and local authorithies in charge of the **evaluation of disabilities**: what to write in a medical certificate, check lists of issues to be evaluated by the disability authorithies for each disease or group of diseases
- → Make a **better use of Orphanet**: promote the use of available information on consequences of the disease in daily life + use the Orpha disability data (as soon as will be available)

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THANK YOU

QUESTIONS & ANSWERS

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