EURORDIS
Rare Diseases Europe

EUROPLAN
European Project for Rare Diseases National Plans Development
Coordinated by the Italian National Centre for Rare Diseases
Italian National Institute of Health
2012-2015

Best Practices in the Management, Funding, Indicators and Monitoring of a National Plan

Dorica Dan
EURORDIS - EUCERD
on behalf of
the EURORDIS-EUROPLAN
Advisors Committee

EURORDIS Membership Meeting 31 May 2013 Dubrovnik

ARE THERE BEST PRACTICES?

- As of today, few national plans on rare diseases have been implemented → difficult to make a thorough analysis
- **Nevertheless**, patients' advocates have gathered experience in getting involved in the process of developing National Plans/ Strategies

→ **Sharing of experience in different EU countries over the last 3 years**

→ **Identification of key common issues in NP/ NS**

→ **Leading us to provide « hints » on practices that can work**

A DYNAMIC COMMUNITY

- **We have seen that rare disease patients' representatives have been the driving force in:**
  - Raising awareness of rare diseases
  - Advocating at national level and European levels for RD patients' equal access to diagnosis and care
- **An EU framework is in place (Council & EUCERD Recommendation) → strong support**
- **Working towards national measures to be adopted and / or sustained**

MANAGEMENT / MONITORING

- **Bring together all relevant stakeholders involved**

  → EUROPLAN Conferences succeeded in bringing stakeholders around the same table:

  Dialogue established

- **IMPORTANT TO HAVE AN OFFICIAL NATIONAL DECISION THAT SUPPORTS THE ESTABLISHMENT OF NP/ NS**

  → Support the **commitments** of all necessary Institutions/ national bodies in the implementation phase

MANAGEMENT / MONITORING

- **IMPORTANT TO HAVE A NATIONAL ADVISORY COMMITTEE FOR THE MONITORING AND EVALUATION OF THE PLAN** → **Governance**

RD PATIENT REPRESENTATIVES MUST BE INVOLVED AT ALL STAGES OF THE PLAN DEVELOPMENT AND IMPLEMENTATION

MANAGEMENT / MONITORING

- **RD Patients' representatives / National Alliances are not always accepted in different governmental / institutional committees or working groups**
- **HOWEVER, RD patients' representatives are the EXPERTS on their diseases – their real life experience is invaluable**
- **It is – and always has been - the duty of RD patients' representatives to make their voice heard at national level and defend their seat on a national RD committee / working group**

MANAGEMENT / MONITORING

- **To increase effective involvement of patient representatives it is essential to work on « individual and collective empowerment »**
- **Key success factor to make RD emerge in the societal space, promote research activities, generate new knowledge on rare diseases and their management;**
- **Based on awareness, information, education, experience exchange, networking, benchmarking and good practices.**

(from the document "Common Goals & Mutual Commitments between EURORDIS & National Alliances in Europe", May 2013)

MANAGEMENT / MONITORING

- **Providing concrete results on specific actions and projects led by patients is another way to build credibility with policy makers and getting involved in national policy management**

Case study - *«Building a Community for RD»*, UNIAMO FIMR

“Towards a shared model of quality assessment of Centres of Expertise for RD”

- Developed training / information path addressed to people with RD, families, physicians, GPs, pediatricians, aimed at their participation in consultation processes on public health matters.
- The quality assessment model launched with the project was taken on board by public authorities and is becoming
- **Patients, successful initiators of the process, are closely involved**

MANAGEMENT / MONITORING

- **Building credibility with policy makers and getting involved in national policy management**

Case study – FEDER

The Advisory Committee of the Ministry of Health, Social Services and Equity MSPSI has created a Working Group on Rare Diseases. FEDER participates in this Working Group WG and has participated in the first kick-off meeting, presenting (insisting on) **13 proposal for the 2013 Year.**

MANAGEMENT / MONITORING

- **IMPORTANT TO « define a limited number of priority actions within their plans or strategies, with objectives and follow-up mechanisms »** (Council Recommendation)
- **Few actions feasible to implement in short, medium term**
- **Take stock of key common issues to be taken into in National Plan / Strategy**
- **Use existing EU legal and policy documents (EUCERD Recommendations, EU Regulations & Directives, projects ..) supporting the identified key common issues, measures**

MANAGEMENT / MONITORING

- **The main actions feasible to implement in short, medium term**

Case study – RONARD

A partnership between RONARD and MoH Romania has been signed during the RDD Campaign 2013 and a Roadmap of the main actions for 2013 has been agreeded.

Periodicly meetings of the NCRD with the MoH will monitor the implementation of these activities.

KEY COMMON ISSUES

KEY COMMON ISSUES :

1. Centres of Expertise
2. European Reference Networks
3. Improved Access to Orphan Medicinal Products
4. Registries and data collection
5. Research: international initiatives & national issues
6. Coding and Classification
7. Access to diagnosis for all

SOME PIECE OF ADVICE

From real life experience

- **Proposal to have a national / regional information and coordination unit for RD patients**
- **Call for a survey collecting information on the treatments used by the RD patients: drugs, surgery, physiotherapy, etc.;**
- **Organizing patients’ pathway in the health and social care system (cooperation of CoE and social services);**
- **Education and training of professionals, patients and caretakers;**

FUNDING

Public funding, public commitment

- **Public funding is pre-requirement - it means commitment from national/public authorities**
- **Minister for Health is in the frontline**, but also **Ministers for Research and Social Affairs, Education** are involved
  - ➔ **their funding should be coordinated**
- **Need to identify key measures, priorities needing dedicated funding**

FUNDING

Public funding, cost-effectiveness

- **Optimise existing resources: information must be shared about which structures already exist**
- **Re-distribution of allocated funds in an optimal manner**
- **Making the case of initial investments for RD – If part of the general budget allocated to best solution for patients as most cost-effecitve solutions in the long-run.**

FUNDING

Public funding, universal vs dedicated budget

- Universal systems vs dedicated budgets for the Plan **– often a mix of the two approaches**
  - Need to integrate specific measures in the overall healthcare system but certain actions for PLWRD need dedicated support

- **RD healthcare embedded in the system:**
  - Access to all
  - …but risk to dilute expertise and focus

FUNDING

Private funding, an essential complement

- **Public- private partnership is essential, including publicly led approach to capital investment**
- **More attractive in time of crisis**

➔ However necessary to maintain control mechanisms to ensure common 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, 2012

MONITORING - INDICATORS

- **INDICATORS developed in the EUROPLAN project (2008-2011) to evaluate the achievements of RD initiatives**
  - 59 EUROPLAN indicators have been identified accross the 6 themes of the Council Recommendation

21 Core indicators for Monitoring RD National Plans/Strategies

  - Ongoing work on Indicators within the EUCERD Joint Action on Rare Diseases amongst the original 59 Indicators
  - Expected adoption as EUCERD Reco. in June

MONITORING - INDICATORS

Case study - The French experience - Monitoring 2 National Plans for RD

- **For each action of the French Plan:**
  - **A « pilot » a « co-pilot » are clearly identified at the Ministry for Health or at a public agency**
  - **Partners including patient groups are clearly identified**
  - **Follow-up indicators and deliverables are also clearly identified, with a detailed agenda 2011-2014**

MONITORING - INDICATORS

- **However, in most countries evaluation mechanisms and criteria could not be established**
  - e.g. Germany – A dedicated Workshop «Monitoring» at EUROPLAN National Conference (2010)
  - Yet the process resulted just too time-consuming & it was considered that there was not enough experience
  - However the coordination platform NAMSE should continue to exist and should monitor the implementation process of the NP
- **Essential to pursue advocacy**

CONCLUSIONS

- **Never give up**
- **Do not lose what has been already achieved**
- **The process to push for national measures for rare diseases is a dynamic one → it needs to be constantly stimulated**

- **THANK YOU!**