MEMO

To: **EURORDIS National Alliances, Organisers of EUROPLAN National Conferences**

From: **EURORDIS Advisory Group**

Date: **27 November 2009**

Re: **CONTENT OUTLINE - Minimum requirements and recommended content for the WORKSHOPS of EUROPLAN National Conferences**

This memo introduces the Themes that will have to be dealt with in the Workshops of the National Conferences on Rare Diseases (RD) organised in the framework of the EUROPLAN project (WP8).

Each National Conference will be configured in Plenary Session and Workshops. The Workshops will be set up according to specific Themes and will be responsible to deliver concrete proposals for the Plenary.

The **Themes of the Workshops** are linked to the chapters in the EU Council Recommendation on Rare Diseases. There will be as many Workshops as Themes, as summarised below:

THEMES of Workshops	
MAIN THEMES	**Also includes** : These compulsory Sub-themes can be addressed in the Workshop or in a separate one
1. Methodology and Governance of a NP	
2. Definition, codification and inventorying of RD	**Information and training**
3. Research on RD	
4. Standards of care for RDs - Centres of Expertise / European Reference Networks	**Orphan Drugs and Provision of Treatment**
5. Patient Empowerment and Specialised Services	
HORIZONTAL THEMES These two additional Themes of the Council Recommendation are considered like "Horizontal Themes" and, as such, they may be addressed within the different Workshops 1 to 5. It is of course also possible to organise specific Workshops for Themes 6 and 7. The cells corresponding to Themes 6 and 7 are in grey.	
6. Sustainability	

7. Gathering expertise at the European level	

It is important to recall that these **Themes, together with the Sub-themes listed in the tables which follow, are compulsory, as they constitute the common outline that is required in all EUROPLAN National Conferences**. Nevertheless, within this outline there is some flexibility on how to organise Workshops: starting from a minimum of 5 Workshops (4 main Themes + one introductory Theme), additional Workshops can be organised on the Horizontal Themes or on the two specific Sub-themes (Orphan Drugs and Provision of Treatment; Information and Training) as mentioned above.

The following tables illustrate in detail the content of each Workshop with the following structure:

- **Sub-themes** which need to be addressed in each Workshop (**mandatory**);
- **Further specific guidelines for the discussion**. These are **not strictly mandatory, but are highly recommended**. They have been shaped to a large extent on the 'EUROPLAN Recommendations', the ongoing document which will provide practical guidelines on how to develop National Plans or Strategies on RD. They will link national efforts to a common strategy at EU level, ensuring that progress in the field of RDs is globally coherent and follows common orientations throughout Europe. The EUROPLAN Recommendations, based on the Council Recommendation on RD, provide practical details on how to implement the EU document. They also expand on topics which are not addressed in the Council Recommendations.

To help you prepare the conference, at the end of each table we added the part of the **Council Recommendation on RD** which is relevant to Theme dealt with, whereas the text of the **EUROPLAN Recommendations**, on the other side, will be provided when available.

Please note that the **Final Conference Report will be based on this content structure**. Each Conference organiser will have to account for how each Theme and Sub-Theme has been dealt with in the National Conference and what practical solutions or proposals emerged in that area.

Please note that **further guidelines for Workshops' Chairs and Moderators** will follow in due time.

Last but not least, please remember that Conference organisers will be able to use the **Factsheets for patient advocates** that EURORDIS is developing to help patients develop and familiarise with the content of the Conferences. We will inform you as soon as they are available online.

LEGEND
- RD = Rare Diseases
- NP = National Plan or National Strategy
- SC = Steering Committee (indicating a governing body in charge of the coordination of the plan, whatever is the official name chosen – governing body, coordinating committee, etc.)
- CoE = Centres of Expertise

- ERN = European Reference Networks
- OD = Orphan Drugs

THEMES for WORKSHOPS of the EUROPLAN National Conferences

1 - Governance and Monitoring of the National Plan

SUB-THEMES (mandatory)	Further guidelines for discussion (not strictly mandatory but highly recommended)
Mapping exercise before developing a NP	• Is there an awareness of the situation of RD in your country (epidemiologic figures, dimension of the problem)? • Is an inventory being made, or a report, of the existing resources and actions on RD (or of which RD patients can benefit) in the national health care and social system? • Are the unmet needs of RD patients being evaluated?
Development and structure of a National Plan / Strategy	• Is there in your country a legal/policy framework in the form of a national plan or strategy created to address the health care and social needs of patients with RD with specific actions? What are the steps to be taken? • Is the NP integrated and comprehensive so to respond to all patients' needs? • Is the NP created in the form of a written document with a clear structure? • What general and specific objectives or priority areas have been / can be identified in your country? • Are the specific actions envisaged in the NP accompanied by clear deliverables and measurable results? • Is there a timeline for the achievement of priority actions with specific deliverables?
Governance of a National Plan	• Is there a Steering Committee (SC), or coordination committee, governing panel... governing the implementation of the Plan? • Do all stakeholders participate in the governance of the NP – healthcare authorities, patients, healthcare professionals, academics, representatives of the industry, etc.? Do these stakeholders cover all areas of expertise relevant to the NP, such as pharmacology, regulatory, clinical, health and social services, epidemiology, administrative policies, etc. • Is the participation of patients envisaged to all phases of the NP so to ensure that patients are actors in the decision on health care measures directed to them? • Does the SC meet regularly? • Does the SC write a status for their activities and the responsibilities of its members? • Does the SC write a regular (yearly) report on the achievement of the objectives of the NP and deliverables? • Is the NP made public in the general content and specific actions?

Monitoring the National Plan	• Is there a monitoring system for the NP? • What type of Indicators is used to monitor its implementation? Are the EUROPLAN indicators used a basis for monitoring and evaluating the actions of the NP? • Is the evaluation of the Plan ensured by an external body, i.e. different from the SC? • Does the evaluation include also the collection of opinions and satisfaction surveys addressed to patients?
Sustainability of the National Plan	• Is there a specific budget attached to the NP? Does it ensure the long term sustainability of its actions? • What are the main sources of funding of the National Plan? • Are there specific budget provisions accompanying specific actions in certain priority areas e.g. orphan drugs, CoE, diagnosis, research, etc.? **Theme 6** - **Sustainability**

From the COUNCIL RECOMMENDATION of 8 June 2009 on an action in the field of rare diseases (2009/C 151/02)

"I. PLANS AND STRATEGIES IN THE FIELD OF RARE DISEASES
1. Establish and implement plans or strategies for rare diseases at the appropriate level or explore appropriate measures for rare diseases in other public health strategies, in order to aim to ensure that patients with rare diseases have access to high-quality care, including diagnostics, treatments, habilitation for those living with the disease and, if possible, effective orphan drugs, and in particular:
(a) elaborate and adopt a plan or strategy as soon as possible, preferably by the end of 2013 at the latest, aimed at guiding and structuring relevant actions in the field of rare diseases within the framework of their health and social systems;
(b) take action to integrate current and future initiatives at local, regional and national levels into their plans or strategies for a comprehensive approach;
(c) define a limited number of priority actions within their plans or strategies, with objectives and follow-up mechanisms;
(d) take note of the development of guidelines and recommendations for the elaboration of national action for rare diseases by relevant authorities at national level in the framework of the ongoing European project for rare diseases national plans development (EUROPLAN) selected for funding over the period 2008-2011 in the first programme of Community action in the field of public health."

2. Definition, codification and inventorying of RDs + Information and training

SUB-THEMES (mandatory)	Further guidelines for discussion (not strictly mandatory but highly recommended)
Definition of RD	• Is the EU official definition (RD are those affecting up to 5 out of 10 000 person) used in your country? Are there alternative or more specific definitions used instead or in addition?
Classification and traceability of RDs in the national health system	• What classification system is used in your country? ICD9, ICD10, SNOMED, OMIM, ORPHAN... • For which purpose is (are) the classification system(s) used, e.g. surveillance, reimbursement, provision of social support, etc. • Is your country prepared to adopt the WHO-led system, the ICD-11, recommended by the EU in the Council Recommendation on RD, when ready (2014)? • What level of awareness and knowledge do healthcare professionals have of the RD classification and codification? What can be done to improve it?
Inventories, registries and lists	• Are there official lists of RD in your country? Is there an official governmental RD registry? And/or specific RD databases e.g. held by CoE? Are there RD surveillance projects or programmes (e.g. sentinel programmes, surveys)? • What kind of initiatives should be taken or reinforced in your country?
	• Do these registries and programmes receive government support? • How to ensure, through appropriate funding mechanisms, the long-term sustainability of registries and databases? **Theme 6 – Sustainability**
	• Does your country participate to the development of a EU inventory of RD as recommended in the Council Rec. on RD? **Theme 7 – Gathering expertise**

2.1. Information and training (a separate Workshop may be organised if deemed useful)	
How to improve information on available care for RDs in general, for different audiences	• What are the existing information sources in the country? Are they of good quality? These include considering whether: o there is a national official website for RD in the country; o there are help lines for both patients and healthcare professionals, whether they are known to the public; o there are initiatives of centres of expertise and/or patient organisations or programme to stimulate the development of information and educational material for patients or specific publics (teacher, social workers, etc.); o if existing resources at European level, Orphanet and Eurordis, are used: information on diseases, specialised centres and patient groups, ongoing research projects, clinical trials. o there are initiatives to raise awareness on RD such as a RD Day.
	• Do these initiatives and activities receive public funding? Are they mainly promoted and funded by patient associations? **Theme 6 – Sustainability**
How to improve access to quality information on RDs	• How are these information sources and initiatives publicised? • How to make sure that they reach out to the target audiences? And to the wider public?
How to ensure adequate training of healthcare professionals on RDs	• Training for healthcare professionals may include : o training to make them aware of the existence of RDs and of resources available for their care; o medical training in fields relevant to diagnosis of RDs (e.g. genetics, oncology, immunology, neurology, paediatrics) o medical training of young doctors and scientists in the field of RD o exchange and sharing of expertise between centres within the country. • How to ensure that existing and validated international guidelines are used at national level to guide diagnosis and treatment of RD?

	• What mechanisms can be put in place to support the exchange of expertise at EU level and the adequate training for all healthcare professionals? • Is your country supporting the participation of national experts in developing international guidelines to guide diagnosis and treatment of RD at national level? **Theme 7 – Gathering EU expertise**

From the COUNCIL RECOMMENDATION of 8 June 2009 on an action in the field of rare diseases (2009/C 151/02)

"II. ADEQUATE DEFINITION, CODIFICATION AND INVENTORYING OF RARE DISEASES
2. Use for the purposes of Community-level policy work a common definition of rare disease as a disease affecting no more than 5 per 10 000 persons.
3. Aim to ensure that rare diseases are adequately coded and traceable in all health information systems, encouraging an adequate recognition of the disease in the national healthcare and reimbursement systems based on the ICD while respecting national procedures.
4. Contribute actively to the development of the EU easily accessible and dynamic inventory of rare diseases based on the Orphanet network and other existing networks as referred to in the Commission Communication on rare diseases.
5. Consider supporting at all appropriate levels, including the Community level, on the one hand, specific disease information networks and, on the other hand, for epidemiological purposes, registries and databases, whilst being aware of an independent governance."

3. Research on RDs

SUB-THEMES (mandatory)	Further guidelines for discussion (not strictly mandatory but highly recommended)
Mapping of existing research resources, infrastructures and programmes for RDs	• Evaluation of RD research resources and infrastructures across different disciplines and sources of funds, both public and private. Considering whether a combination of private and public support is feasible. • Does a specific national RD research programme with dedicated funds exist? Is there a scope for such programme? • What is the scope of patient-driven research? **Further suggestion** • ***Specific area: Biobanks and databases***
Needs and priorities for research in the field of RDs	• Assessing needs and priorities for basic, clinical and translational research, as well as priorities for social research.
Fostering interest and participation of national laboratories and researchers, patients and patient organisations in RD research projects	• How to make the link between basic and translational research and Centres of Expertise? *(could be also dealt with in the next Theme).* • Promoting interdisciplinary approaches to research. • Strengthening the exchanges among patient organisations. • Specific programmes for funding or recruiting young scientists on RD research.
Sustainability of research on RD	• How to ensure, through appropriate funding mechanisms, structural and long-term sustainability of research projects and research infrastructures in the field of RDs? In particular in respect of public health and social research, as well as transversal infrastructures. **Theme 6 - Sustainability**
EU collaboration on research on RD	• How to foster and support the participation of national researchers and laboratories, patients and patients organisations in EU-wide projects? **Theme 7 – Gathering EU expertise**

From the COUNCIL RECOMMENDATION of 8 June 2009 on an action in the field of rare diseases (2009/C 151/02)

"III. RESEARCH ON RARE DISEASES
6. Identify ongoing research and research resources in the national and Community frameworks in order to establish the state of the art, assess the research landscape in the area of rare diseases, and improve the coordination of Community, national and regional programmes for rare diseases research.
7. Identify needs and priorities for basic, clinical, translational and social research in the field of rare diseases and modes of fostering them, and promote interdisciplinary co-operative approaches to be complementarily addressed through national and Community programmes.
8. Foster the participation of national researchers in research projects on rare diseases funded at all appropriate levels, including the Community level.
9. Include in their plans or strategies provisions aimed at fostering research in the field of rare diseases.
10. Facilitate, together with the Commission, the development of research cooperation with third countries active in research on rare diseases and more generally with regard to the exchange of information and the sharing of expertise."

4. Standards of care for RD -Centres of Expertise + Orphan Drugs

SUB-THEMES (mandatory)	Further guidelines for discussion (not strictly mandatory but highly recommended)
Identification of national or regional CoE all through the national territory by 2013	• How to ensure that all patient living with a rare disease have access to a CoE in your country or abroad, and support the of CoE creation where necessary? • How to best apply in your country the criteria identified in the report of the RDTF (RD Task Force), which will possibly become part of the EC Cross-Border Healthcare Directive, art.15, for the designation of CoE? • In particular, how to make sure that CoE are –as much as possible- expert of the specific RD in both the clinical and the research field? • What are the best structures and solutions suitable to be a CoE in your country? Do they depend on the disease (or group of diseases)? Are they regional or national?
Sustainability of CoE	• How to ensure, through appropriate funding mechanisms, the long-term sustainability of healthcare infrastructures, in particular CoE? **Theme 6** - **Sustainability**
Participation in ERN	• How to foster the participation of CoE to European Reference Networks? • How to support the mobility of patients and/or professionals beyond the national borders? **Theme 7 – Gathering EU expertise**
How to shorten the route to diagnosis	• How to support the mobility of expertise and knowledge to facilitate the treatment of patients in their proximity (including mobility of information through ICT)? • How to map, network and support the laboratories at national level? **Further suggestion** • ***Specific area: Screening and genetic testing***
	• How to organise DNA and samples exchanges and reimbursement at European and international level? • How to support the development of European guidelines on diagnostic tests and population screening? • What mechanisms to develop in order to support common protocols and recommendations such as European reference opinions on diagnostic tools, medical care, education and social care? **Theme 7 – Gathering EU expertise**

How to offer suitable care and organise adequate healthcare pathways[1] for RD patients	• How to develop a system based on the adoption of clinical pathways for the provision of care for RD patients: provide funding for multidisciplinary consultations, cover patient and families transportation costs? • How to link medical expertise of the specialised centres to local medical, paramedical and social care? • How to establish good cooperation with relevant experts within the country or from abroad when necessary through European reference networks, with the aim to adopt common healthcare pathways based on the best evidence and expertise?
How to ensure in CoE multidisciplinary approaches and integration between medical and social levels	**Further suggestion** • ***Specific area: social counselling***
How to evaluate CoE	• How to envisage a system for the evaluation of CoE? Would it be based on clinical outcomes or patient satisfaction or both?

1.1. Orphan Drugs (a separate Workshop may be organised if deemed useful) **Future of Orphan Drugs**	• Number of OD on the market and number of patients treated. • Obstacles to the availability of OD approved in the EU within the timeframe requested by the regulations (180 days).
Access of RD patients to orphan drugs Pricing and Reimbursement	• How to improve and speed up national procedures for pricing and reimbursement of OD? • In particular, what mechanisms to put in place to use the "clinical added value of orphan drugs"[2] report developed at the EU level (EMEA) to base the national decision on pricing and reimbursement in order to minimise delays in access to OD? • How to promote a national policy on conditional pricing and reimbursement, based on the EU Pharma Recommendation "Improving access to orphan drugs"? • Access to orphan drugs through CoE.
	• Participating to the EU-level collaboration on the assessment of the clinical added value of orphan drugs at the EMEA. **Theme 7 – Gathering EU expertise**

[1] Healthcare pathways are structured, multidisciplinary plans of care designed to support the implementation of clinical guidelines and protocols

[2] The "clinical added value of orphan drugs" is the assessment of medical and scientific data on the right place of the orphan product on the therapeutic strategy of the RD.

Compassionate use and temporary approval of orphan drugs. Off label use	• How to foster access to OD through compassionate use[3]? • Can drugs be prescribed off label and reimbursed when the evidence of a benefit for the patients exists? • What measures can be studied and put in place to provide treatments other than medicinal products when the evidence of a benefit for the patient exists?

From the COUNCIL RECOMMENDATION of 8 June 2009 on an action in the field of rare diseases (2009/C 151/02)

"IV. CENTRES OF EXPERTISE AND EUROPEAN REFERENCE NETWORKS FOR RARE DISEASES
11. Identify appropriate centres of expertise throughout their national territory by the end of 2013, and consider supporting their creation.
12. Foster the participation of centres of expertise in European reference networks respecting the national competences and rules with regard to their authorisation or recognition.
13. Organise healthcare pathways for patients suffering from rare diseases through the establishment of cooperation with relevant experts and exchange of professionals and expertise within the country or from abroad when necessary.
14. Support the use of information and communication technologies such as telemedicine where it is necessary to ensure distant access to the specific healthcare needed.
15. Include, in their plans or strategies, the necessary conditions for the diffusion and mobility of expertise and knowledge in order to facilitate the treatment of patients in their proximity.
16. Encourage centres of expertise to be based on a multidisciplinary approach to care when addressing rare diseases."

[3] A treatment option for European patients suffering from a disease for which no satisfactory authorised alternative therapy exists and/or who cannot enter a clinical trial, may be the use of an unauthorised medicinal product in a compassionate use programme.

5. Patient empowerment and Specialised Services

SUB-THEMES (mandatory)	Further guidelines for discussion (not strictly mandatory but highly recommended)
Involvement of patients and their representatives in decision-making processes in the field of RDs	How to involve and empower patients , particularly: • in the elaboration of national plans; • in the provision of information; • in the establishment and management of CoE and ERN; • in the definition of the RD research policy; • and other areas such as the establishment and management of registries, clinical trials, the evaluation of clinical added value of drugs, therapeutic education programmes, medical, paramedical and social workers training…
	• How to ensure, through appropriate funding mechanisms, patient representativeness in decision-making processes relevant to RDs? **Theme 6** - **Sustainability**
Support to the activities performed by patient organisations	• How to support activities performed by patient organisations, such as awareness-raising, capacity-building and training, exchange of information and best practices, networking, outreach to very isolated patients.
	• What mechanisms can be put in place to support patients' empowerment activities and their representativeness in EU-wide instances? **Theme 7 – Gathering EU expertise**
Specialised social services: Respite Care Services[4]; Therapeutic Recreational Programmes[5]; Services aimed at the integration of patients in daily life	• What kind of programmes exist in your country to support patients and families with RD and/or disabilities in general? • What kind of schemes or programmes do exist supporting access of RD patients to Respite Care Services, Therapeutic Recreational Programmes and services aimed at the integration of patients in daily life ? • What can be done to improve their availability and accessibility of such services, including public funding?

[4] Respite Care Services are provided on a short-term basis for disabled people who usually live at home. It gives family members and carers time and temporarily relief, prevents burn out. For further information see www.rapsodyonline.eu

[5] Therapeutic Recreational Programmes give children the possibility to stop thinking about disease and treatment and to focus on fun and leisure, thus allowing personal development to thrive. For further information see www.rapsodyonline.eu

	• How are specialised social services financed? By government institutions and budget? By private initiative or patient associations? **Theme 6 – Sustainability**
Help Lines	• What kind of help lines (all diseases) exist in your country to assist RD patients and healthcare professionals? • How to develop or consolidate existing patient-run help line services for RD? • How to improve the service offered? How to improve their visibility esp. for patients? • National measures to establish the 116 European number[6].
	• How are help lines financed? By private initiative or patient associations? Is there any government funding? • How to ensure their long-term sustainability? **Theme 6 – Sustainability**

From the COUNCIL RECOMMENDATION of 8 June 2009 on an action in the field of rare diseases (2009/C 151/02)

"VI. EMPOWERMENT OF PATIENT ORGANISATIONS
18. Consult patients and patients representatives on the policies in the field of rare diseases and facilitate patient access to updated information on rare diseases.
19. Promote the activities performed by patient organisations, such as awareness-raising, capacity-building and training, exchange of information and best practices, networking and outreach to very isolated patients."

[6] The European Commission seeks to identify services of social value in Europe that could benefit from single European free phone numbers starting with 116. Once the 116 number has been assigned by the Commission, national procedures are necessary to make the free number work in each E U country. An application will be made for a 116 number for RD.

HORIZONTAL THEMES

As mentioned above, these two additional Themes of the Council Recommendation are considered like "Horizontal Themes" and, as such, they may be addressed within the different Workshops 1 to 4. It is of course also possible to organise specific Workshops for Themes 6 and 7. The cells corresponding to Themes 6 and 7 can be found are in the tables above, highlighted in grey. We therefore report below only the corresponding parts of the text of the Council Recommendation on RD.

6. Sustainability

From the COUNCIL RECOMMENDATION of 8 June 2009 on an action in the field of rare diseases (2009/C 151/02)

"VII. SUSTAINABILITY
20. Together with the Commission, aim to ensure, through appropriate funding and cooperation mechanisms, the long-term sustainability of infrastructures developed in the field of information, research and healthcare for rare diseases."

7. Gathering expertise at the European level

From the COUNCIL RECOMMENDATION of 8 June 2009 on an action in the field of rare diseases (2009/C 151/02)

"V. GATHERING THE EXPERTISE ON RARE DISEASES AT EUROPEAN LEVEL
17. Gather national expertise on rare diseases and support the pooling of that expertise with European counterparts in order to support:
(a) the sharing of best practices on diagnostic tools and medical care as well as education and social care in the field of rare diseases;
(b) adequate education and training for all health professionals to make them aware of the existence of these diseases and of resources available for their care;
(c) the development of medical training in fields relevant to the diagnosis and management of rare diseases, such as genetics, immunology, neurology, oncology or paediatrics;
(d) the development of European guidelines on diagnostic tests or population screening, while respecting national decisions and competences;
(e) the sharing Member States assessment reports on the therapeutic or clinical 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."