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

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## EUCERD Joint Action / Work Package 4
## EUROPLAN National Conference

# Workshop Theme 5
# ORPHAN MEDICINAL PRODUCTS & THERAPEUTICS FOR RARE DISEASES

EUCERD
Joint Action

*Co-funded by the* European Commission

Executive Agency for Health and Consumers

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# Relevant extracts from the

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

## 8 June 2009

# COUNCIL RECOMMENDATION

(The Council of the EU) “HEREBY RECOMMENDS that Member States:

V. GATHERING THE EXPERTISE ON RARE DISEASES AT EUROPEAN LEVEL

Gather national expertise on rare diseases and support the pooling of that expertise with European counterparts in order to support:

- (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.”

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# EUCERD RECOMMENDATIONS ON CORE INDICATORS FOR RD NATIONAL PLANS / STRATEGIES

EUCERD Core Indicators, full version:
http://www.eucerd.eu/wp-content/uploads/2013/06/EUCERD_Recommendations_Indicators_adopted.pdf

# Core Indicators

N°15. Number of Orphan Medical Products (OMPs) with a EU marketing authorisation and available in the country (i.e. priced and reimbursed or directly supplied by the national health system)

**Identify access to OMPs in your country compared to all OMPs being granted an EU marketing authorisation**

N°16. Existence of a governmental system for compassionate use of medicinal products

**Does a national system exist to provide medicines to RD patients prior to marketing autorisation?**

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# GUIDELINES FOR DISCUSSION

## Workshop Theme 5
## ORPHAN MEDICINAL PRODUCTS & THERAPEUTICS FOR RARE DISEASES

# 1. Support to Orphan Drugs (OD) Development

- What type of support is provided to Small Medium Enterprises (SMEs) after designation of their products as Orphan Drug (OD)?
- Are there **specific programmes that foster further developments of designated ODs**?
- Discuss additional incentives at national level to strengthen research into rare diseases leading to the development of ODs.
- What mechanisms need to be put in place to facilitate the set-up of **clinical trials for small populations** run by academics in Centres of Expertise?

# 2. Access to Treatments

- How to improve and speed up national procedures for pricing and reimbursement of OD so as to minimise delays and improving access to OD?

- The **CAVOMP** (**Clinical Added Value for Orphan Medicinal Products**) helps MS make informed decisions on the added value of an OD:

– Is your country aware of the EUCERD Recommendation on the CAVOMP?

– Is your country ready to support the mechanism of exchange of knowledge between Member States and EU authorities on the scientific assessment of the CAVOMP?

# 2. Access to Treatments

The mechanism of the ‘CAVOMP Information flow’ includes **4 times points** to ensure the best possible ‘information flow’ and to allow sharing assessment reports with other EU Member States and EU authorities.

- How will your country participate to these time points:

  - Time point 1: Early dialogue

  - Time point 2: Compilation Report and evidence definition / Evidence Generation Plan

  - Time point 3: Follow-up of the EGP

  - Time point 4: Assessment of relative effectiveness

# 2. Access to Treatments

- Discuss about the importance of adopting a policy on **conditional pricing and reimbursement** with regular revisions, based on revised and updated assessment reports.
- Do national **HTA agencies** send representatives to participate in:

- EMA (European Medicines Agency) Protocol assistance/ scientific advice?
- Dialogue mechanisms with the EMA or other HTA agencies, notably in the context of the EUnetHTA?

- What measures should be put in place to ensure their involvement?

# 2. Access to Treatments

- Are HTA bodies in my country participating to the permanent cooperation mechanism for HTA as laid down in the EU Cross-Border Health Care Directive?

  What measures should be put in place to ensure their involvement?

- Is my country participating to the voluntary ad hoc Working Group **"MOCA" (Mechanisms for Coordinated Access to ODs)** set up between EU MS on a voluntary basis to discuss the value of new OD based on a common EU transparent value framework ?

# 2. Access to Treatments

- What measures are in place to support the availability and access to orphan drugs through Centres of Expertise?
- In particular, discuss the **value of budget allocation for OD at national/central level** (or coordinated at nat. level)

  *(so as to avoid that OD budget be managed by hospitals / Centres of Expertise alone, without overall coordination)*

- **Information and access to OD**: Is the information about treatment for rare diseases disseminated in the most effective way ?

  *(so as to avoid delays in access to treatments)*

# 3. Compassionate Use

- How to foster access to compassionate use* programmes?
- How to best inform patients, their organisations and healthcare professionals of compassionate use opportunities?
- How to adopt a compassionate use programme when one does not exist?

(**compassionate use = a treatment option for a patient 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)*

# 3. Compassionate Use

- As for **clinical trials**, a Compassionate Use Programmes Facilitation Group could be created in the context of the activities of the Heads of National Medicines Agencies, to coordinate actions.

  Discuss of the opportunity of supporting the creation of this group and joining it.

- Does your country respect the EU legislation and the related obligation to notify the EMA on compassionate use programmes?

# 3. Compassionate Use

- How to best support companies that have difficulties in putting in place compassionate use programmes?
- When drug supply is limited, is there a guideline on how to distribute a limited supply when the demand is greater than the offer?
- How to involve patients in the setting up and management of compassionate use programmes?

# 4. Off Label Use of Medicinal Products

- Can drugs be reimbursed when the possibility of a benefit for the patients exists?

- How to improve and simplify procedures for the off-label uses and related reimbursement?

# 4. Off Label Use of Medicinal Products

- What measures can be put in place to allow the adoption of **temporary protocols** for cohorts of patients treated with drugs outside their authorised use? Please discuss both cases:

  1) when clinical trials to better document the efficacy and the safety of the off-label use in question are in progress and a variation of the marketing authorisation can be envisaged at a later stage; *and*

  2) when such trials are not running and are not likely to be conducted, as there are too few patients/off label prescriptions.

# 4. Off Label Use of Medicinal Products

- How to decide **which post-authorisation efficacy and safety studies** are needed to document the off-label use in question?
- In particular, is there a threshold (in terms of volume) where such studies should become more systematic?
- Discuss the possibility of Centres of Expertise taking over the task of assessing benefits and risks of drugs currently prescribed off label for the patients they are following.

# 4. Off Label Use of Medicinal Products

- What measures could encourage the collection of data regarding off-label use of approved drugs and existing medications?

- Discuss the involvement of different stakeholders in the optimisation and adaptation of (existing) registries to support the collection of data for off-label use of approved drugs.

# 5. Pharmacovigilance

## Compliance with the EU Pharmacovigilance Regulation

- **Spontaneous reports of adverse drug reactions**: how to encourage patients and healthcare professionals to report to their national authorities?
- What measures are being put in place to facilitate the reporting of suspected adverse reactions to medicinal products by both healthcare professionals and patients?
- How to make methods for such reporting available to them?
- What measures are being adopted to ensure the establishment of a reporting mechanism, national contact points, reporting tools?

# 5. Pharmacovigilance

- What strategy can be set up to involve industry, academia and regulators to **expand data collection** and embed it in disease-specific registries to serve the requirements of post-marketing surveillance?

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# PROPOSALS

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