EURORDIS
Rare Diseases Europe

Inspiring actions & good measures from National Plans/Strategies - Conferences

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Discussion on optimising results from EUROPLAN National Conferences

→ **Decision** to identify **1) good practices 2) inspiring proposals** on governance, research, access to care (CEs & ERNs), orphan drugs and social care

Mainly from EUROPLAN Conference Reports + current RD National Plans + State of the Art of Rare Disease activities in Europe.

Membership Meeting 2015

Team members produced a very first draft of good practices / proposals stemming from Conferences, current plans…

Research, Centres of Expertise, ERNs, Orphan Drugs, social care → to be discussed throughout EMM 2015
→ EUROPLAN Advisors will input.

Theme 5: Access to OMPs and therapies for RDs patients

→ to be discussed today

Access to RDs therapies

Objective → Sharing of good practices so National Alliances can be well equipped for the decision-making process around implementation / monitoring NPRDs

First Document circulated for comments is the "**Good Practice Report EUROPLAN II - Theme 5 – Access to Orphan Medicinal Products and therapies for Rare Diseases patients**

Access to OMPs and therapies for RDs patients

Good Practice Report → 5 chapters

5.1 Support to OMPs Development

- Additional incentives on top of OMP Regulation

5.2 Access to treatments

- Measures / systems on P&R
- Exchange of clinical added value
- (International) Cooperation of HTA bodies
- Innovative approaches to MA
- Existence of information centres/helplines
- Existence of Centres of Expertise

Access to OMPs and therapies for RDs patients

Good Practice Report → 5 chapters

5.3 Access to non-approved OMPs: Compassionate use

5.4 Access to non-approved OMPs: Off label use

5.5 Pharmacovigilance, including reporting tools of adverse reactions

Your feedback and comments today are absolutely essential

Additional incentives to OMP development

Best Practice: “Fondo AIFA 5%” in ***Italy***. AIFA established an innovative funding scheme operational since 2005, by which pharma companies are required to donate 5% of their promotional expenditure to an independent research fund.

The fund collects 45 millions every year → ½ for the reimbursement of orphan or life saving drugs waiting market entry and ½ support independent research, drug information programme and PHV. But, in 2008, RDs and OMPs were not listed as a priority area for research.

Additional incentives to OMP development

Best Practice: Since 2006 in ***Belgium***, the revenues of OMPs are no longer subject to the so-called “pharmaceutical taxes” on sales of reimbursable drugs.
BUT since 2013, the government has reintegrated a tax, but lower than the one for non-OMPs.
Best practice: Dutch pharmaceutical SMEs are given a subsidy in ***the Netherlands*** for the costs of submitting a dossier for designation and MA to the EMA.

Measures on P&R

Best practice (proposal?): when the therapeutic value is not clear, in the ***Netherlands*** the reimbursement should be conditional for a period of 4 years (this period could/should be shorter)

→ Conditional MA for producers, reduces prices for payers and ensures reimbursement / access to patients.

→ During this period, LT data generation post-conditional MA. Strong link with establishment of registries.

Measures on P&R

Best practice: “Balduzzi Law” (2012): pharmaceutical companies can apply in ***Italy*** for P&R to AIFA as soon as CHMP gives a positive opinion → Quicker decision-making starting before the EC has granted MA.

Best practice: OMPs have to show therapeutic added-value but there is no obligation for pharmaceutical companies to provide pharmaco-economic data/evaluation for OMPs to address a rare disease with no alternative treatment exit. This is valid in ***Belgium and in the Netherlands***.

Exchange on clinical added-value

Best Practice the elaboration of the **Therapeutic Product Reports (TPRs)** directed by the Agency for MPs and MDs in ***Spain*** (AEMPS) and developed by different agents of the health system (AEMPS, Ministry of Health, Social Services and Equality, regional governments, medical experts, different medical and HC professionals and POs) → provide **single assessment of the added value of MPs for the entire territory to secure equal access to MPs in all regions of Spain.**

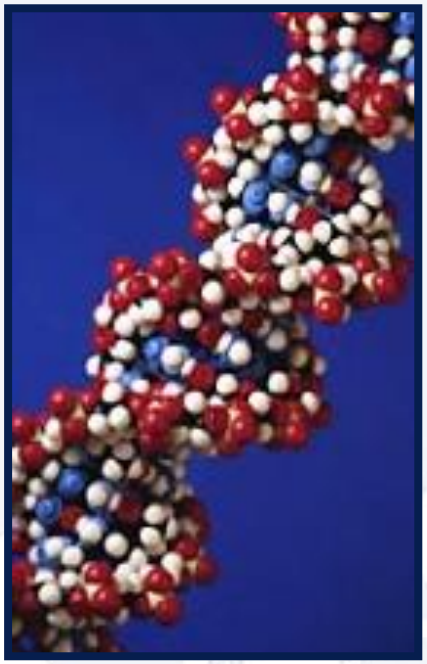
The role of the TPRs should be consolidated as they are binding on all Autonomous Communities and need to be updated on a regular basis in the light of new scientific evidence and real-life use data.

This stimulates **coordination** between all Ministries involved and the Autonomous Communities based on **scientific** rather than purely economic grounds.

Innovative approaches to MA

Best Practice: Decree 2013:

In ***Italy***, AIFA assesses OMPs applications as a priority → fast track authorisation (max 100 days).
Fast-track mechanism for ODs to enter Pricing / reimbursement negotiation before MA is granted at EMA level.



Access to non-approved OMPs: Compassionate use

Best Practice : ATU (Temporary Authorisation of Use) exists in ***France*** since 1994, valid for one year, whereby a MP without MA can be prescribed under exceptional circumstances for severe and life-threatening diseases, either for cohorts (group, phase III) or for individuals (phase II, no therapeutic option, under the responsibility of prescribing doctor). It is a system of compassionate use / early access. Conditional pricing and conditional Market Access.

Proposal: currently, ATU does not include obligation to collect data on efficacy in real life → need to link this to data generation and risk management plans.

Access to non-approved OMPs: Compassionate use

Best Practice:

Based on an unofficial procedure within the Division of Pharmacy and Drugs in ***Luxembourg***, all Medicinal Products that are reimbursed in Belgium are automatically reimbursed and accessible in Luxembourg. Sometimes, it is possible to get the needed medicines from Germany, but this is till an expensive procedure.

Proposal: establishment of a Platform on RDs could solve some of the access issues by offering a unique access point to patients, families and clinicians.

Access to non-approved OMPs: Compassionate use / off-label

Best practice - When a MP is not authorised in ***Italy*:**

1. Use of OMP on a national basis (Law 648): **compassionate use** allows for prescription – **paid by the producer** – of drugs not yet authorised (Phase II or III CT) but where favourable evaluation expected in terms of efficacy/safety.

2. Off-label: in hospital, by a doctor, personal basis, no other treatment option. Prescription on an individual ad hoc basis for one patient through the **Fondo AIFA 5%, where** ½ of the Fund goes to purchase OMPs and drugs representing a hope for treatment for severe pathologies. The applications to the Fund are forwarded to AIFA through the Regions and Centres dealing with RDs (or directly from the CoE copy to the Region).

In spite of this, there has been emergency situations with sudden shortage of essential drugs for RDs → AIFA asked to the **Military Chemical Pharmaceutical Factory** in Firenze (public not for profit plant for drug production) to produce drugs that are not available. This role as State Pharmaceutical Shop to solve drug shortages was provided for by the Cooperation Agreement signed with AIFA in March 2012.

Access to non-approved OMPs: Compassionate use/off-label

Best practice

Compassionate Use programmes or Medical Need Programme (off-label) are provided for in ***Belgium*** since 2006.

Also exists the possibility to reimburse OMPs which are not covered by the basket of benefit through the **Special Solidarity Fund (SSF)** (OMP = 35%).

Pharmacovigilance, including reporting tools on adverse reactions

Best practice 8: opening of the PHV system to direct reporting of undesired effects from patients, ***in France.***

There is an online form.

Proposal to improve the BP → the forms should be translated in EN and made available.

Not comprehensive list, subjective, with the aim to nourish the debate and inspire Good Practices.