PARD 1

Orphan Medicinal Products to the Service of Patients affected by Rare Disorders

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Objectives (1)

• To strengthen collaboration at Community level among associations on rare disorders
• To develop new national alliances in European countries, around the theme of orphan medicinal products
• To strengthen existing national alliances
Objectives (2)

- Share best practice and knowledge
- Identification of needs and problems
- Link patient groups across pathologies and borders
- Give patients a voice
- Create Synergies around the theme: OMP’s
Objectives (3)

- Elaborate action plans
- Report evaluation needs
- Produce recommendations at national and European level
Methodology

Phase I : Kick-off meeting in Brussels - October 2000
Phase II : Explore the situation at national level - Workshops in 7 member states- Jan. 2001
Phase III : Develop common understanding - Workshop European level- June 2001
Phase IV : Implement different actions - workshops at national level- October 2001
Phase V : Final phase – report & recommendations for EC- March 2002
Results (1)

- Country by country situation described
- « SWOT » analysis of organizations and alliances performed
- Exchange of experiences and « best practice » at national and European level
Results (2)

- Common understanding and terminology across Europe
- Improved understanding of OMP-legislation
- Contact and dialogue with other stakeholders
- Setting up of new alliances
- Recommendations and action plans on national and European level
Major Findings (1)

- Unequal access to diagnosis and treatment across Europe
- Limited access to information on RD’s and OMP’s
- Insufficient structures
- Inadequate level of cooperation on national and European level
Major Findings (2)

OMP’s and innovative drugs:
- Research: Methodology of clinical trials, access to studies, abandoned molecules
- Lack of national incentives
- Delays in actual access after MA
- Insufficient data collection on side effects
- Limited access to information
- Reimbursement
Recommendations

OMP’s:
• Registry of OMP’s and clinical trials
• Adapt methodology of clinical trials
• Reduce delays in access
• Reinforce data collection on side effects
• Look into reimbursement issues
Recommendations (2)

Structures:

- Establish centres of excellence
- Support training and exchange
- Support creation of information networks
- Improve information on OMP-legislation
- Support further development of European collaboration between alliances
- Set up monitoring mechanisms
Recommendations (3)

Best practices:
• Stimulate consensus on health care guidelines for RD’s from diagnosis to treatment

Epidemiology:
• Coordinate pan European studies
Conclusion

PARD 1 has succeeded in creating a common understanding and platform for addressing issues regarding OMP’s among patient groups for RD’s in Europe. A number of specific recommendations have been identified and will need follow up. Patient group involvement will be crucial to ensure success.