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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

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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

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Objectives (3)

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

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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

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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

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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

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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

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Major Findings (2)

OMP's and innovative drugs:

- **Research: Methodology of clinical trials, access to studies, abandonned molecules**
- **Lack of national incentives**
- **Delays in actual access after MA**
- **Insufficient datacollection on sideeffects**
- **Limited access to information**
- **Reimbursement**

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Recommendations

OMP's:

- **Registry of OMP's and clinical trials**
- **Adapt methodology of clinical trials**
- **Reduce delays in access**
- **Reinforce datacollection on sideeffects**
- **Look into reimbursement issues**

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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**

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Recommendations (3)

Best practices:

- **Stimulate consensus on health care guidelines for RD's from diagnosis to treatment**

Epidemiology:

- **Coordinate pan European studies**

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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.