



Draft of the Portuguese Programme on Rare Diseases

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DRAFT OF THE PORTUGUESE PROGRAMME WORKING GROUP

- The programme was developed by a workgroup of experts from these fields:
 - health care,
 - medical research,
 - clinical laboratories,
 - orphan drug agency,
 - administration.



NATIONAL HEALTH PLAN 2004 - 2010





DRAFT OF THE PORTUGUESE PROGRAMME

- This programme was on public consultation until January 31st, 2008
- Workshops were held with the stakeholders (health care professionals, researchers, drug industry and patient associations).



DRAFT OF THE PORTUGUESE PROGRAMME

- The final draft incorporates all the inputs from the public consultation and from the stakeholders.



MAIN GOALS

- Improve the national response to non-satisfied health needs of people with rare diseases and their families;
- Improve health care quality for people with rare diseases.



SPECIFIC GOALS

- Establish the national network of reference centres for rare diseases;
- Improve access to adequate care for people with rare diseases;
- Improve the mechanism of integrated management of rare diseases;
- Improve knowledge and research on rare diseases;
- Promote therapeutic innovation on medicines for rare diseases;
- Ensure the trans-national cooperation in the EU and CPLP (Community of Portuguese Language Countries).



TARGET

All individuals affected by rare diseases, considered within their families and communities.



ACTION PLAN

- **Implementation: 2008 – 2010**
- **Consolidation: 2010-2015**



STRATEGIES

Three main directions:

- Intervention
- Training
- Collect data and information analysis



INTERVENTION STRATEGIES

- Propose specific financial rules for accredited reference centres;
- Define criteria for early diagnosis of rare diseases;
- Guarantee long term, health and social care to people with rare diseases;
- Propose specific support and inclusion to children with rare diseases;
- Create and disseminate guidelines of “best practices” for the promotion of clinical quality and patient safety of people with rare diseases;
- Monitor efficiency and safety of the orphan drugs.



TRAINING STRATEGIES

- Promote specific training on rare diseases for health professionals;
- Create and disseminate self-assistance manuals;
- Disseminate scientific information and the available resources.



STRATEGIES DATA COLLECTION AND INFORMATION ANALYSIS

- Develop partnerships between health services, investigation centres and patient associations;
- Monitor health gains resulting from the programme implementation.



ACCOMPANIMENT AND EVALUATION

The Directorate-General of Health, supported by a national expert committee should develop and monitor a set of indicators, such as:

- Total number of accredited reference centres;
- Total number of rare diseases with integrated management;
- Total number of technical guidelines on “best practices” developed.



TIMELINE AND BUDGET

- The timeline for each strategy has been outlined in great detail.
- A strict budget is under development, considering the planned timeline of each strategy.



CONCLUSIONS

Thank you!

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