

Sharing best practices on integrative approach to rare diseases in different countries



The Netherlands (NL)

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MoH= Minister of Health, OD= orphan drugs, RD= rare diseases,
R&D= research and development

Strategies for rare diseases



- Official definition of RDs in NL: European definition of 5:10,000 (off-label use: 1:150,000)
- National measures for RD and OD
 - The Dutch Steering Committee on OD (WGM)
 - The OD Designation Support programme
 - Dutch registration fee for a medicinal product for RD may be waived (in case of 1:200,000)
- National measures that may stimulate OD development
 - Policies in general to stimulate enterprises, like tax reductions for R&D (WBSO measure)
 - Several programmes from the Ministry of Economic Affairs to facilitate start-ups

Funding of actions, regarding rare diseases



Actions	Funding
Prenatal screening	
Down syndrome and fetal defects	health insurance
Hemolytic Disease of the Newborn	government funding
Neonatal screening	government funding
Diagnostic genetic services	health insurance
Treatment (clinical management)	health insurance
Orphan drugs, extramural treatment	health insurance
Orphan drugs, intramural treatment	health insurance/government funding
number of ODs available in the country	46
Rehabilitation	health insurance
Social care	government funding

Provision of information for rare diseases

- Availability of website-based information: Yes
- Help Lines: Yes

However, information is quite scattered.

- Availability of an official list of RDs: No

However, WGM uses an incomplete list of RDs for (theoretical) considerations on specific issues on health insurance and expertise in the eight university hospitals



Provision of information for rare diseases: websites and helplines

Examples

Websites	Information
CG-Raad (umbrella org for chronically ill and handicapped people)	Living, work, school, medical devices in general for the target group
Orphanet (NL)	RD information (not in Dutch)
MEB	Databank of all registered drugs
Websites and helplines	Information
Welder	Work, social security, insurances
Patient organisations	Specific information on rare diseases
Erfocentre	Specific information on inherited rare diseases
Farmanco	Specific practical information on EU registered OD
WGM	Information on RD and OD



Research on rare diseases



Existing research programmes for RDs

- STIGON-WGM programme: orphan product developer and PhD student stimulating and studying orphan drug development
- Programme Expensive and orphan medicines: research on effectiveness and health technology assessment of OD
- Participant in the EC projects E-Rare and Europlan
- Several other research programmes fund research on rare diseases

Research programme proposal for RDs (April 2007)

- Specific to rare diseases and orphan drugs with the ultimate goal to develop therapies for rare diseases. A decision of MoH on funding is pending

Prevention

- Folic acid
- Prenatal screening
- Neonatal screening (17 diseases since 2007)
- Delay of diagnosis
 - Clinic genetic centers in all eight university hospitals
- Other preventive practices?
 - Pre-conception counseling (in preparation)
 - Early detection in childcare (child health doctors, 0 – 19 yrs)



Empowerment of patients organisations

- Support to the activities of patient organisations
 - Financed by the MoH
- Representation and consultation of patient organisations

Examples:

- in Dutch Steering Committee on OD (WGM)
- in committees of the Netherlands Organisation for Health Research and Development
- initiatives on patient participation in (RD) research
- consultation by the MoH (umbrella patient organisations)

Specialised social services



In general, not specific for RD patients and families, but they may use these social services

- Respite Care Services
 - In-home respite, emergency respite, sitter-companion services, therapeutic day care ('dagopvang'), specialized facilities
- Therapeutic Recreational Programmes
 - Therapeutic day care ('dagverblijf')
- Services aimed at the integration of patients in daily life
 - Social support
 - Supported employment
 - Support in travelling