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

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

<table>
<thead>
<tr>
<th>Websites</th>
<th>Information</th>
</tr>
</thead>
<tbody>
<tr>
<td>CG-Raad (umbrella org for chronically ill and handicapped people)</td>
<td>Living, work, school, medical devices in general for the target group</td>
</tr>
<tr>
<td>Orphanet (NL)</td>
<td>RD information (not in Dutch)</td>
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<tr>
<td>MEB</td>
<td>Databank of all registered drugs</td>
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</tbody>
</table>

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<th>Websites and helplines</th>
<th>Information</th>
</tr>
</thead>
<tbody>
<tr>
<td>Welder</td>
<td>Work, social security, insurances</td>
</tr>
<tr>
<td>Patient organisations</td>
<td>Specific information on rare diseases</td>
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<tr>
<td>Erfocentre</td>
<td>Specific information on inherited rare diseases</td>
</tr>
<tr>
<td>Farmanco</td>
<td>Specific practical information on EU registered OD</td>
</tr>
<tr>
<td>WGM</td>
<td>Information on RD and OD</td>
</tr>
</tbody>
</table>
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