Characteristics of a medical care program for specific diseases in Japan

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History of “Nanbyo”

- 1955: a mysterious disease, in some respects resembling polio, made its appearance in Japan. The symptoms were a combination of diarrhea, internal bleeding and various signs of nerve degeneration.
- 1959: Reached the highest annual incidence of 2,814.
- 1964: the disease was given a formal name, "Sub-acute Myelo-Optic Neuropathy"—SMON.
- 1969: the Ministry of Health and Welfare decided to invest a large sum of money to support the patients’ expenses and to organize a major research project on SMON.
- 1972: The project team clarified the cause of SMON as use of Clioquinol.
→ SMON has decreased remarkably.
1972: The Diet discussed about measures against “Nanbyo” intensively.

1972: “Outline of the Measures against Intractable Disease” was established.
  - Office for Specific Disease Control was established in the Ministry of Health and Welfare.

130 diseases that fit the definition have been specified by an expert advisory board on the basis of research priorities.
Measures against “Nanbyo”
Medical care program for specific diseases

- Research grant for specific diseases
  - Total Budget for Research groups: $125 million in 2010.
  - Today, there are 275 research groups working for 130 specific diseases and 214 rare disease next to specific diseases.

- Medical cost subsidy for specific disease
  - Target: 56 specific diseases (650,000 patients)
  - Budgets: $343.8 million (government) and about $1 billion (local government) in 2010.

- US$1=¥80
Target of specific diseases program

- **Specific diseases with medical cost subsidy**: 56 diseases
- **Specific diseases without medical cost subsidy**: 74 diseases
- **Rare diseases next to specific diseases without medical cost subsidy**: 214 diseases
- **Other rare diseases**
Number of patients with specific diseases, 1973-2008

- Ulcerative Colitis
- Parkinson's disease
- Systemic Lupus Erythematosus

<table>
<thead>
<tr>
<th>Year</th>
<th>Number of patients</th>
</tr>
</thead>
<tbody>
<tr>
<td>1973</td>
<td>Number of patients with specific diseases, 1973-2008</td>
</tr>
<tr>
<td>1978</td>
<td>Number of patients</td>
</tr>
<tr>
<td>1983</td>
<td>Number of patients</td>
</tr>
<tr>
<td>1988</td>
<td>Number of patients</td>
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<tr>
<td>1993</td>
<td>Number of patients</td>
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<td>1998</td>
<td>Number of patients</td>
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<tr>
<td>2003</td>
<td>Number of patients</td>
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<tr>
<td>2008</td>
<td>Number of patients</td>
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</tbody>
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### Number of research target diseases with medical cost subsidy

<table>
<thead>
<tr>
<th>Name of 56 specific diseases</th>
<th>Number of registered patients</th>
<th>Cumulative number (% of Total)</th>
</tr>
</thead>
<tbody>
<tr>
<td>Ulcerative colitis</td>
<td>117,855</td>
<td>117,855 (17)</td>
</tr>
<tr>
<td>Parkinson's Disease and Related Neurodegenerative Disorders</td>
<td>106,637</td>
<td>224,492 (32)</td>
</tr>
<tr>
<td>Systemic lupus erythematodes</td>
<td>56,254</td>
<td>280,746 (40)</td>
</tr>
<tr>
<td>Scleroderma &amp; polymyositis</td>
<td>42,233</td>
<td>322,979 (46)</td>
</tr>
<tr>
<td>Crohn disease</td>
<td>31,652</td>
<td>354,631 (50)</td>
</tr>
<tr>
<td>Ossification of posterior longitudinal ligament</td>
<td>29,647</td>
<td>384,273 (54)</td>
</tr>
<tr>
<td>Retinitis pigmentosa</td>
<td>25,296</td>
<td>409,574 (58)</td>
</tr>
<tr>
<td>Spinocerebellar degeneration</td>
<td>23,290</td>
<td>432,864 (61)</td>
</tr>
<tr>
<td>Idiopathic thrombocytopenic purpura</td>
<td>22,220</td>
<td>455,084 (64)</td>
</tr>
<tr>
<td>Idiopathic dilated cardiomyopathy</td>
<td>22,123</td>
<td>477,207 (68)</td>
</tr>
<tr>
<td>Restrictive cardiomyopathy</td>
<td>18</td>
<td>706,720 (100)</td>
</tr>
<tr>
<td><strong>Total</strong></td>
<td>706,720</td>
<td>706,720 (100)</td>
</tr>
</tbody>
</table>

As of 2011, MHLW
Current status of specific diseases

Responsiveness to therapy

- Respond to therapy
  - Early diagnosis & prevention of complication
    - Idiopathic hyperlipidemia
    - Primary aldosteronism
  - Promote stage appropriate therapy
    - Ulcerative colitis
    - Parkinson’s diseases
    - Systemic lupus erythematoses

- Resistant for therapy
  - Early diagnosis & prevention of complication
    - Severe acute pancreatitis
    - Idiopathic Osteonecrosis of Femoral Head

- No specific therapy
  - Promote development of new drugs
    - IgA nephritis
    - Interstitial pneumonia
    - Bone marrow fibrosis

- Promote international research cooperation
  - Amyotrophic lateral sclerosis
  - Prion disease
  - Progeria

Rarity of patients with specific diseases

- More than 50,000
- Less than 50,000

Rarity of patients with specific diseases

- Amyotrophic lateral sclerosis
- Prion disease
- Progeria

Responsiveness to therapy
Government’s Role for Promotion of Research in RD

1. Grants for basic science and clinical research should be harmonized in well balance.

2. Government needs to coordinate the clinical trials in RD for its incidence.

3. Registry of RD should be expanded to the patients outside of medical subsidy.

4. Human resources for seeds seeking experts needs to be developed.

5. International cooperation needs to accelerate:
   1) Establishing International Platform for information exchange.
   2) Framework for joint clinical trials is mandate:
      Recruitment, Diagnosis, Standardization of medical treatment, Racial difference in response etc.