Policy for reimbursing orphan drugs

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The Rare Disease Prevention and Medication Act
Organizations and statutory duties for rare disease prevention
Designation of rare disease and orphan drugs
Benefits of orphan drugs designation
Reimbursement and logistic for orphan drugs
Challenge and future vision
The Rare Disease Prevention and Medication Act

- Promulgated in 2000 and became the 5th country to make a law especially for rare disease prevention in the world

- Aims of the act
  - To prevent the rare disease
  - To early detect rare disease
  - To enhance healthcare for rare disease patients
  - To support rare disease patients for necessary treatment and nourishment
  - To promote and protect the aforementioned R&D and suppliers
## Organizations and statutory duties for rare disease prevention

<table>
<thead>
<tr>
<th>MOHW</th>
<th>Health Promotion Administration</th>
<th>Food and Drug Administration</th>
<th>National Health Insurance Administration</th>
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<tbody>
<tr>
<td></td>
<td>Designation</td>
<td>Designation</td>
<td>Reimbursement</td>
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<td></td>
<td>Prevention &amp; promotion</td>
<td>Approval of orphan drugs</td>
<td>List in catastrophic disease field (Subsidized co-payment)</td>
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<td></td>
<td>Medical subsidization</td>
<td>Publication for special nutrient food</td>
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<td></td>
<td>R&amp;D</td>
<td>Research promotion for orphan drugs research</td>
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<td>Regulation</td>
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Determination of rare disease and orphan drugs

Committee of Rare Disease and Orphan Drugs (CRDOD)

- Designate rare disease
- Comments on approval of orphan drugs and special nutrient food

Medical Section, CRDOD

Comments on designate rare disease
  • 201 rare diseases designated by 2013

Pharmaceutical Section, CRDOD

Comments on approval of orphan drugs and special nutrient food
  • 86 orphan drugs listed and 40 special nutrient drugs published by 2013
Benefits of orphan drugs designation

- Reduce the fee for registration and review
- Simplify approval procedures, ex. cancel the adopting certificate of A–10 countries
- Drug certificate valid for 10 years, registration exclusive for same ingredients within terms
- Special application for prior use is allowed before approval, NHI reimbursement is applicable as well
Processes of listing orphan drug

Proprietor requests for orphan drug appraisal

Pharmaceutical Section, Committee of Rare Disease and Orphan Drugs evaluates submission

Committee of Rare Disease and Orphan Drugs review

TFDA orphan drug designation

TFDA notifies proprietor to apply for NHI reimbursement

Proprietor submits for NHI reimbursement

PBRS Joint Meeting discussed by all stakeholders

NHIA
Orphan drug listed & reimbursed
## Logistics for reimbursing orphan drugs

### Particular budget for health care

<table>
<thead>
<tr>
<th>Year</th>
<th>Particular Budget (million)</th>
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<tbody>
<tr>
<td>2008</td>
<td>44.9</td>
</tr>
<tr>
<td>2009</td>
<td>50.5</td>
</tr>
<tr>
<td>2010</td>
<td>57.9</td>
</tr>
<tr>
<td>2011</td>
<td>69.2</td>
</tr>
<tr>
<td>2012</td>
<td>87.1</td>
</tr>
<tr>
<td>2013</td>
<td>101.2</td>
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### Beneficial pricing for orphan drugs

- **Pricing rule for new drugs**
- **Cost based pricing (up to extra 25% of marketing fee)**
- **International reference pricing**

#### Monthly claimed expenditure (US$)

<table>
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<tr>
<th>Upper limit</th>
<th>Medium price of A-10 countries*1.x</th>
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</thead>
<tbody>
<tr>
<td>≤16,667</td>
<td>Medium price of A-10 countries*1.2</td>
</tr>
<tr>
<td>&gt; 16,667 &amp; ≤33,333</td>
<td>Medium price of A-10 countries*1.1</td>
</tr>
<tr>
<td>&gt; 33,333</td>
<td>Medium price of A-10 countries</td>
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### Co-payment

- Co-payment subsidized

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75 orphan drugs are reimbursed by NHI, 27 items of them (36%) without approval.

The claimed expense of those items without approval is around 50 million, which is half of the total expense for orphan drugs.
There are 6,783 rare disease patients in 2013, which is 0.029% of the insured (around 23 million population).
Reimbursement of orphan drugs(3)

- The expense of orphan drugs is around 100 million in 2013, which is 1.2% of total healthcare expenditure.
- If we calculate the average personal premium as $650 (NTD), we have already pulled premiums from 150 thousands insured to cover the expense of treating rare disease in 2013.
Once the orphan drugs are designated, they are allowed to apply for reimbursement before approval. The suppliers are unwilling to complete the registration.

27 items of 75 orphan drugs reimbursed by NHI are still without approval. Only 4 of the 27 items are not approved in US, EU, Canada or Australia.
Challenges (2)

- Physicians and pharmacists cannot get enough information of therapeutic effect and adverse effect of certain orphan drugs since the registration is not completed.
- The Drug Injury Relief Act is not applied for unapproved drugs, patient’s rights may be diluted in such case, and medical disputes may occur then.
Challenges (3)

- Monopoly market of orphan drugs
  - the drug company usually offers compassionate therapy before getting NHI reimbursed, and then cut supply afterward to raise humanity issues
  - Drug company appeals for increasing drug price.
Future vision

- Horizontal collaboration between medicine and insurance authorities
  - Simplify procedures to facilitate completing registration

- Principles for reimbursement
  - Reallocate budget
  - Conduct cost-effectiveness (ICER) analysis
Thanks for your attention!