Policy for reimbursing orphan drugs

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Outline

- 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 especial 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

MOHW		
Health Promotion Administration	Food and Drug Administration	National Health Insurance Administration
• Designation • Prevention & promotion • Medical subsidization • R&D • Regulation	• Designation • Approval of orphan drugs • Publication for special nutrient food • Research promotion for orphan drugs research • Regulation	• Reimbursement • List in catastrophic disease field (Subsidized co-payment)

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

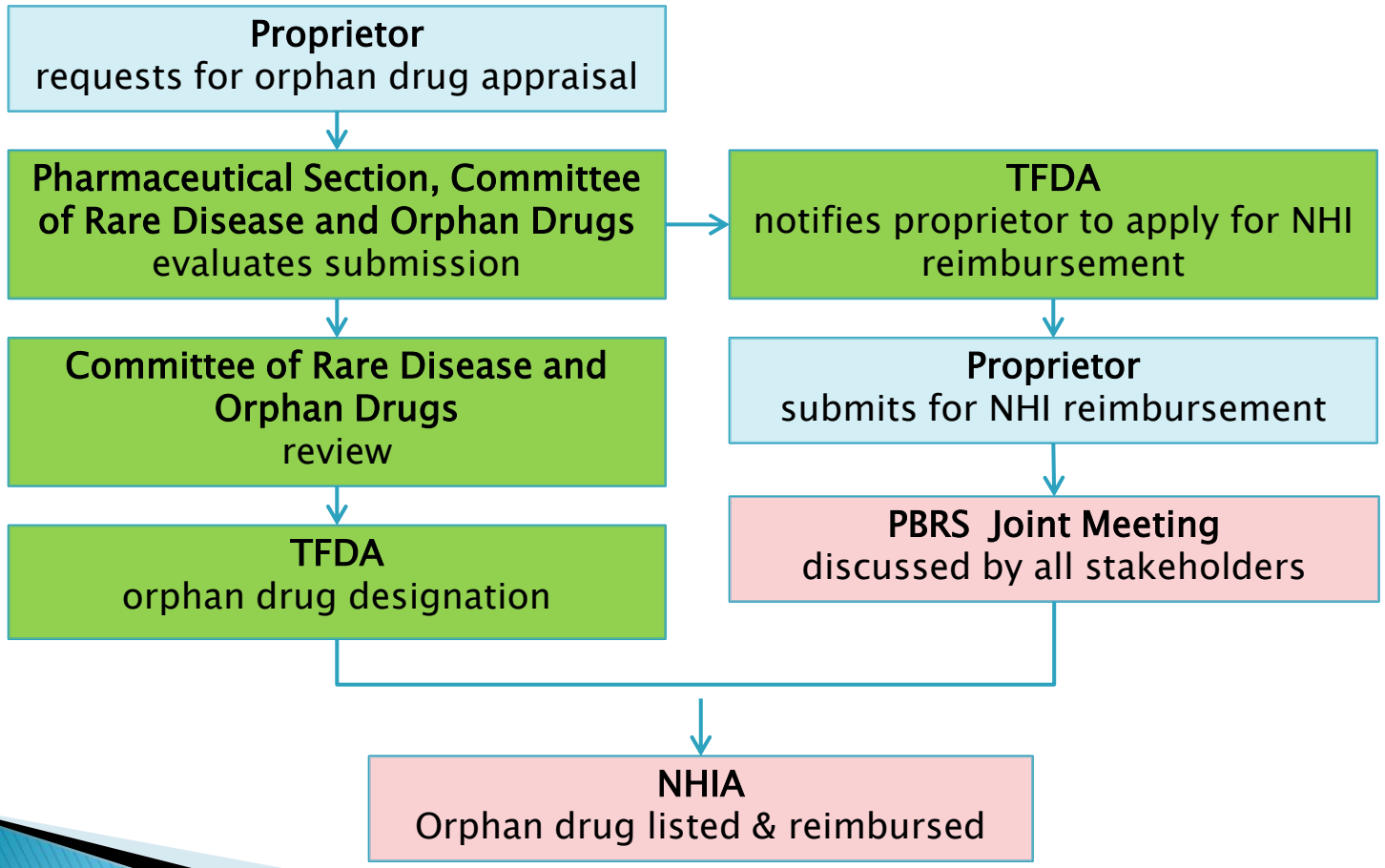
Comments on approval of orphan drugs and special nutrient food

- 86 orphan drugs listed and 40 special nutrient drugs published by 2013

Benefits of orphan drugs designation

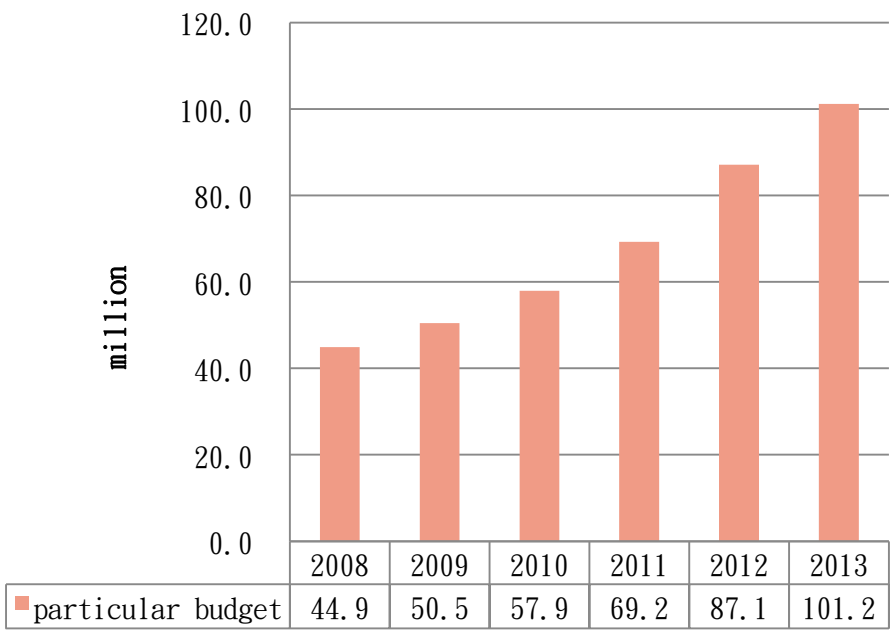
- Reduce the fee for registration and review
- Simplify approval procedures, ex. cancel the adopting certificate of A-10 countries
- Drug certificate valid for 10 years, registration exclusive for same ingredients within terms
- Special application for prior use is allowed before approval, NHI reimbursement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

Processes of listing orphan drug



Logistics for reimbursing orphan drugs

particular budget for health care



	2008	2009	2010	2011	2012	2013
particular budget	44.9	50.5	57.9	69.2	87.1	101.2

Co-payment

Co-payment subsidized

Beneficial pricing for orphan drugs

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

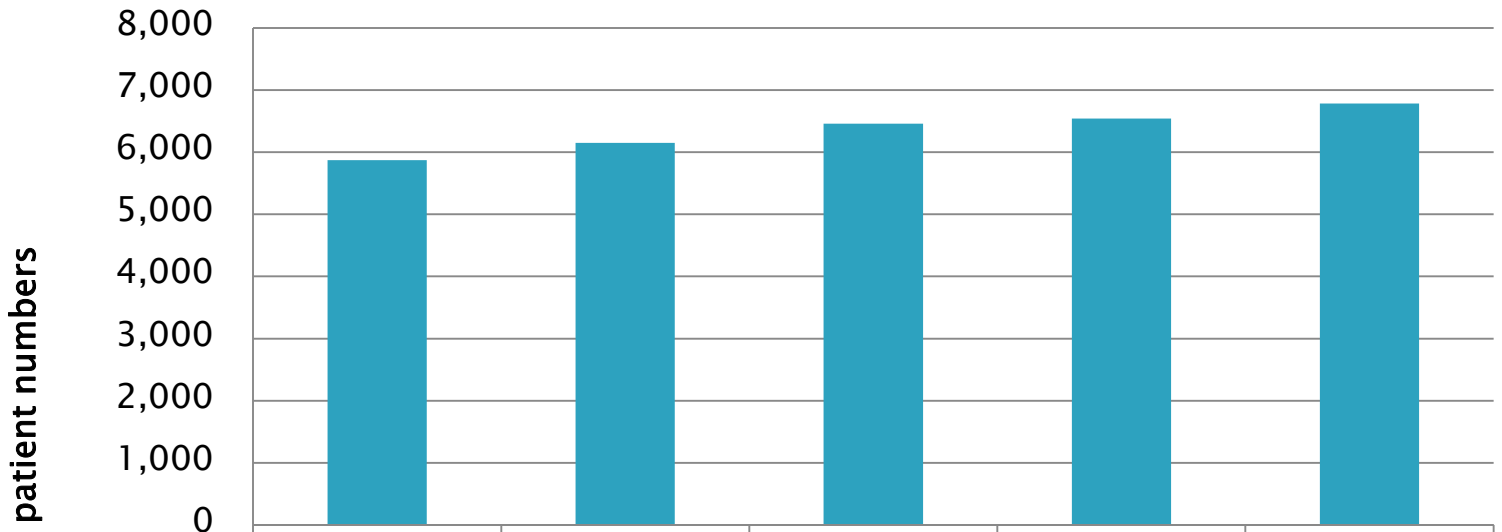
Monthly claimed expenditure ($US)	Upper limit
≦16,667	Medium price of A-10 countries*1.2
＞16,667 & ≦33,333	Medium price of A-10 countries*1.1
＞33,333	Medium price of A-10 countries

Reimbursement of orphan drugs(1)

- 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.

Reimbursement of orphan drugs(2)

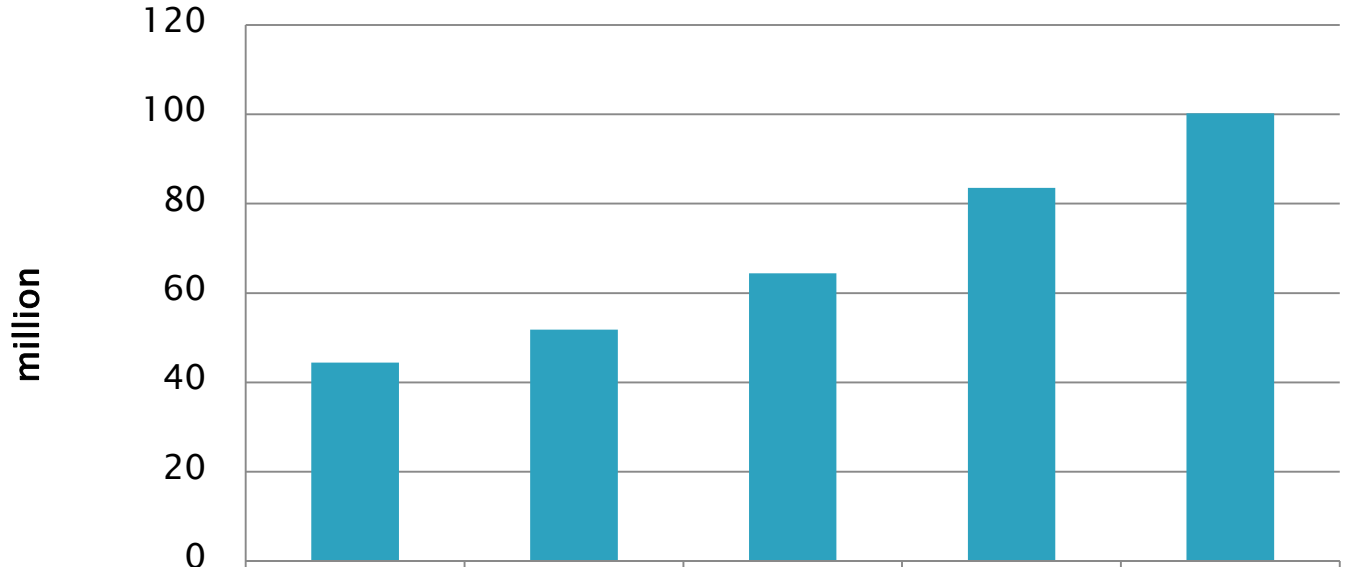
- There are 6,783 rare disease patients in 2013, which is 0.029% of the insured(around 23 million population)



	2009	2010	2011	2012	2013
patient numbers	5,869	6,151	6,459	6,543	6,783
growth rate		4.80%	5.00%	1.30%	3.70%

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.



	2009	2010	2011	2012	2013
expense of orphan drugs	44.4	51.8	64.4	83.5	100.2
growth rate		16.70%	24.40%	29.60%	20.00%

Challenges (1)

- 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 can not get enough information of therapeutic effect and adverse effect of certain orphans drugs since the registration is not completed.
- The Drug Injury Relief Act is not applied for unapproved drugs, patient's right may be diluted in such case, and medical dispute 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 afterword 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!