



Health Technology Assessment for Rare Disease: Lessons Learned from Thailand

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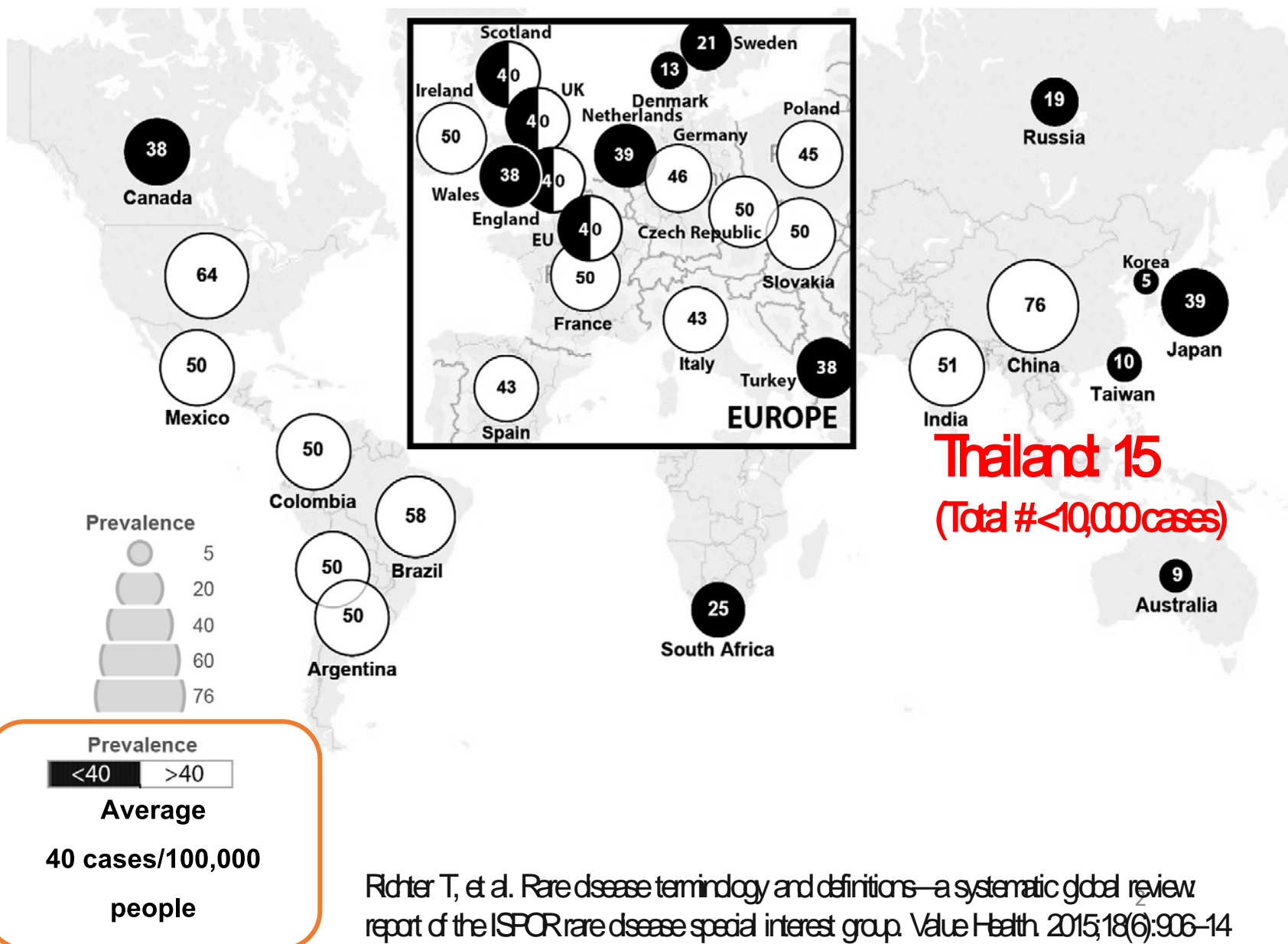


Rare disease terminology and prevalence threshold: Review literature

| Search term | Number of definitions | Proportion of total (%) |
|---|-----------------------|-------------------------|
| Rare disease* | 112 | 38 |
| Orphan drug | 79 | 27 |
| Orphan disease | 18 | 6 |
| Orphan medicinal product | 16 | 5 |
| Rare disorder | 11 | 4 |
| Ultrarare disease | 10 | 3 |
| Highly specialized technologies | 9 | 3 |
| Rare condition | 9 | 3 |
| Neglected disease | 5 | 2 |
| Ultraorphan drug | 4 | 1 |
| Orphan product | 4 | 1 |
| Very rare disease | 4 | 1 |
| Orphan indication† | 2 | 1 |
| Low-frequency disease† | 2 | 1 |
| Pharmacological therapies of high complexity† | 2 | 1 |
| Rare disability† | 2 | 1 |
| Ultraorphan disease | 2 | 1 |
| Priority review drugs† | 1 | <1 |
| Orphan pharmaceutical product† | 1 | <1 |
| Syndrome without a name | 1 | <1 |
| Rare and neglected disease | 1 | <1 |
| Extremely rare disease† | 1 | <1 |
| Orphan subset† | 1 | <1 |
| Rare medicinal technology† | 1 | <1 |

* Includes definitions that use qualifiers in addition to "rare disease" such as "intractable," for example, in definitions used in Japan and Korea.

† Terms included by local researchers for use during searches but not included in the original list of search terms.



Richter T, et al. Rare disease terminology and definitions—a systematic global review report of the ISPOR rare disease special interest group. Value Health. 2015;18(6):906-14

Using HTA for coverage decision on Thailand

Non-medical benefit package

- New Technology and interventions
- Effective Coverage for existing packages



Stakeholder
engagement

1. Topic selection

Working Groups of Topic selection

2 Priority setting of topics

3 HTA
Technical Health
Economics Working Group

Subcommittee of
Universal Coverage
Schemes Benefit
Package

4 Making
coverage
decisions

Medical benefit package

- New medicines



Working Groups of Medical expert

Subcommittee of
National List of
Essential Medicine

Step 1 Stakeholder Engagement for Topic selection



Criteria for development **non-medical** reimbursement package

Criteria for development **medical** reimbursement package

✓ *Rare disease* topics (medical and non-medical) were nominated from medical expert group*

Topic Selection

Step 2

1. Burden of disease
2. Severity of disease ✓
3. Efficacy and/or effectiveness ✓
4. Difference among health benefit schemes
5. Catastrophic illness and household economic impact ✓
6. Social and Equity consideration ✓

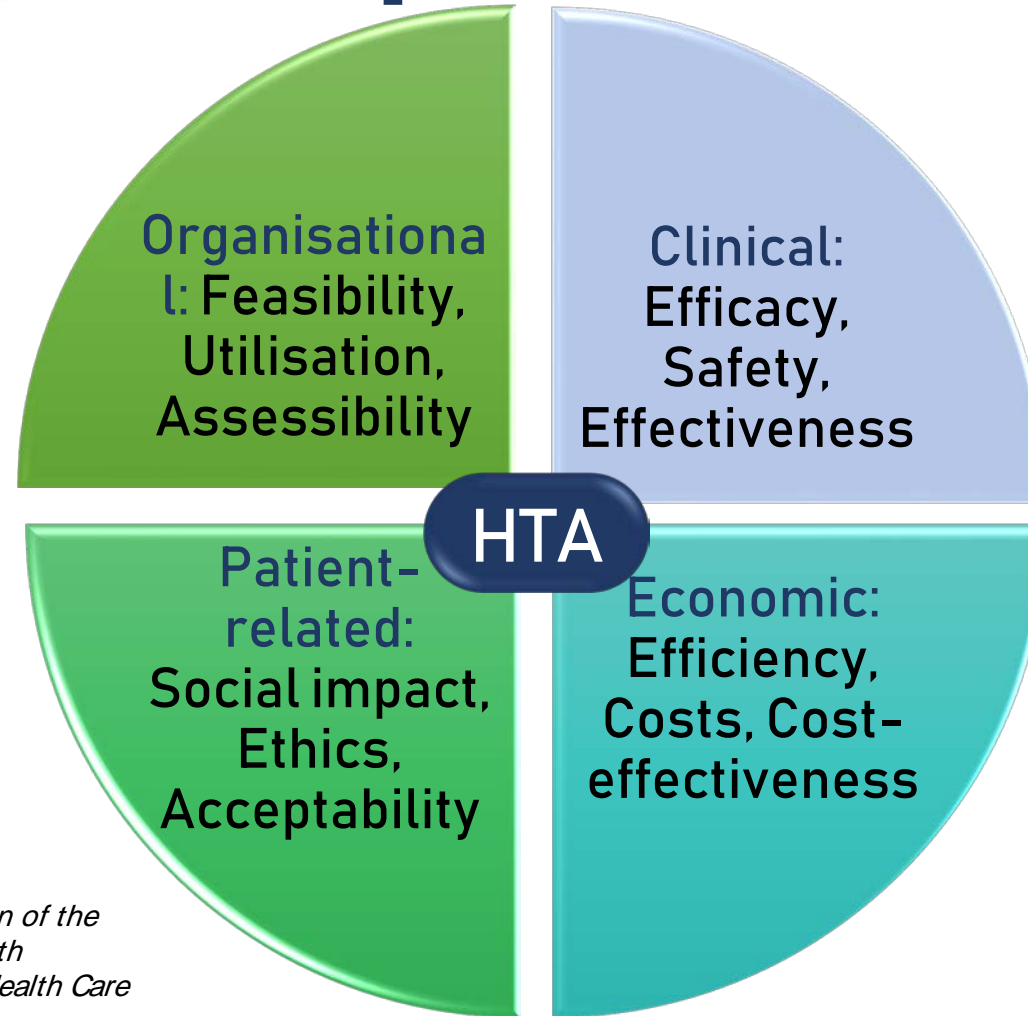
Topic Selection

Step 2

1. Burden of disease
2. Severity of disease ✓
3. Safety and Efficacy ✓
4. Clinical practice guideline ✓
5. Budget Impact ✓
6. Other supporting evidence e.g. **co-dependence technology and feasibility**

Step 3 HTA

✓ *Rare disease topics (medical and non-medical) were selected for HTA*



1. **Medical: Imiglucerase for Gaucher disease type I**
2. **Non-medical: Neonatal Screening for Inborn Errors of Metabolism Using Tandem Mass Spectrometry**

Source: Draborg et al. International comparison of the definition and the practical application of health technology assessment *Int J Technol Assess Health Care* 2005 Winter; 21(1):89-95.

Criteria for development **non-medical** reimbursement package

Making decision

Step 4

1. Cost-effectiveness
2. Clinical practice guideline
3. Feasibility and preparedness of health services
4. Affordability of public health insurance
5. Social and ethical issues
6. Other consideration e.g. **medical reimbursement package**

✓ *Bone marrow transplantation: curative treatment for Gaucher Type I*

Criteria for development **medical** reimbursement package

Making decision

Step 4

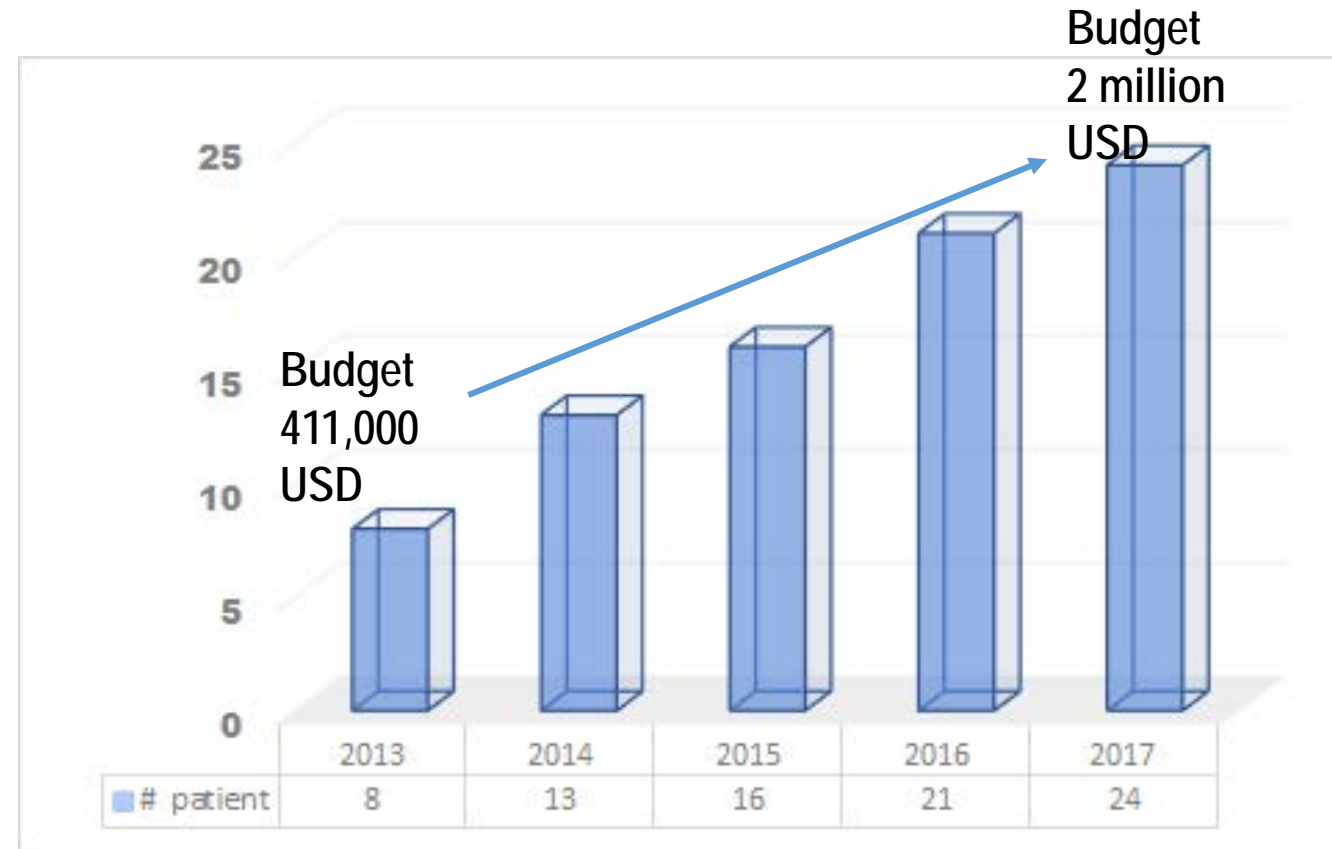
1. Cost-effectiveness and price negotiation
2. Clinical practice guideline
3. Feasibility and preparedness of health services
4. Affordability of public health insurance
5. Social and ethical issues
6. Other consideration e.g. **non-medical reimbursement package**

✓ *Imiglucerase before undergoing Bone marrow transplantation*

Coverage decision for rare disease

Imiglucerase for Gaucher type 1

- ✓ It was **not cost-effective** due to very high cost treatment (ICER 197,000 USD/QALY, threshold 5,000 USD/QALY).
- ✓ The drug is life-saving, and it is **needed for bone marrow transplant** (curative treatment covered by UHC).
- ✓ **Equity concern** : if this medicine is not included in the list but the bone marrow transplant is in the benefit package under UCS. This means only the rich can access the treatment as they are able to pay for imiglucerase (around 120,00 USD/year)
- ✓ Inclusion in the NLEM after price negotiation



*Only 2 patients could undergo bone marrow transplantation

Sources: Teerawattananon Y et al. The use of economic evaluation for guiding the pharmaceutical reimbursement list in Thailand. Zeitschrift für Evidenz, Fortbildung und Qualität im Gesundheitswesen, 2014,108.7:397-404.

In process: Topic nominated

| Nb. | Technology | Indication | Estimated number of patient |
|-----|---|--|-----------------------------|
| 1 | Agalsidase beta | Fabry disease | 4 |
| 2 | Alglucosidase alfa | Pompe disease | 6 |
| 3 | Idursulfase | Mucopolysaccharidosis type II (Hunter syndrome) | 6 |
| 4 | Laronidase | Mucopolysaccharidosis type I Hurler (MSP-IH) & Hurler-Scheie (MSP-IHS) | 6 |
| 5 | Neonatal Screening Using Tandem Mass Spectrometry | 30 indications of Inborn Errors of Metabolism | ... |

Challenges of HTA for rare disease

- Estimate of total number of rare disease patients and budget impact that should be calculated from all relevant technologies (co-dependent technology)
- Clinical and other evidence needed for HTA e.g. efficacy, cost, health quality of life
- Uncertainty of result
- Feasibility and preparedness of health services e.g. medical specialist, registry system, payment mechanism