The 2nd
Rare Disease Asia
Conference 2016
17 - 19 November 2016
Hotel Pullman Bangsar,
ORGANISED BY
Rainbow Across Borders

Definition & Challenges in Rare Disease Management

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THAILAND

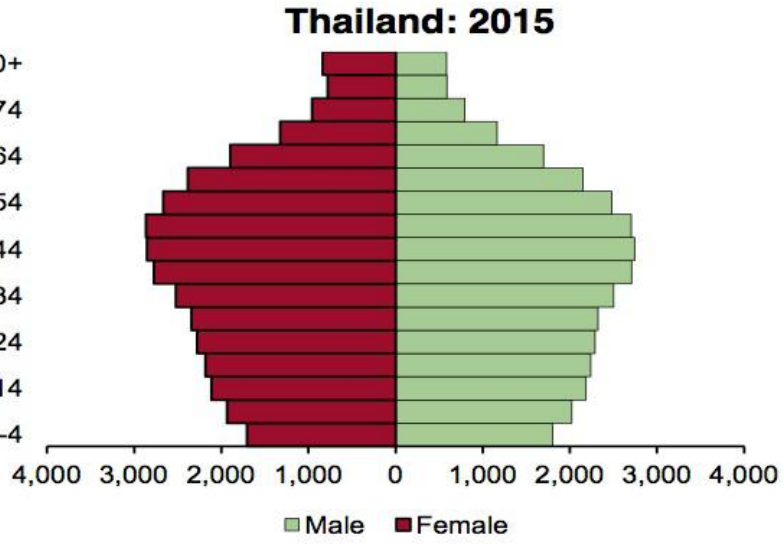
The 2nd Rare Disease Asia Conference 2016

67 M people (2015)
Aging society: 10% of ≥65y

Needs of
Healthy elderly &
Health children

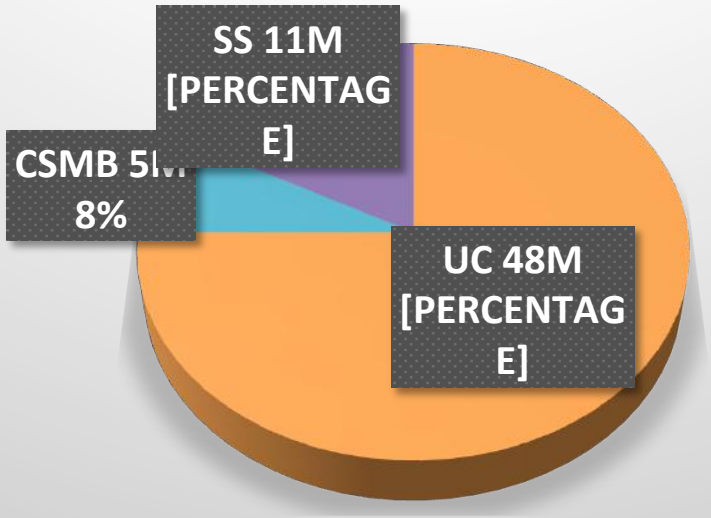
Population pyramid of Thailand, 2015

In thousands



Source: UN, Credit Suisse

Major Health Schemes in Thailand



92% of Children - UC

UC, Universal Health Coverage
SS, Social Security (only working adults)
CSMB, Civil Servant Medical Benefit

Thailand: Definition of Rare Disease

- by health authority **"No"**

- by Office of The Royal Society **"Yes"**
  - incidence 1 in 2,500 or less
  - serious condition
  - hard to find medicine
  - difficult to treat -

Medical Challenges in Managing Rare Diseases

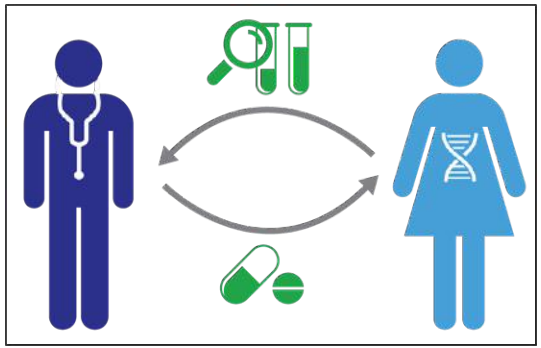
1. **Delayed Diagnosis**
2. **Limited Resources** – human, lab, orphan drugs
3. **Reimbursement issue**

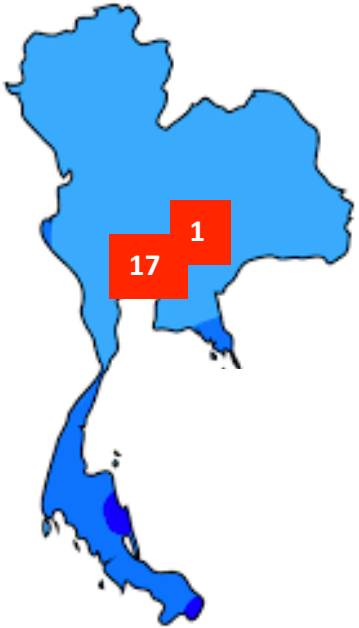
1. Delayed Diagnosis

- Lack of Awareness
- Lack for initial lab for inborn metabolic disorder: ammonia level
- No national NBS to aid early diagnosis for emergency IEM

2. Limited Resources (A)

Geneticists & Confirmatory Tests
– University in Bangkok
Patients – Public Hospitals (MOPH)



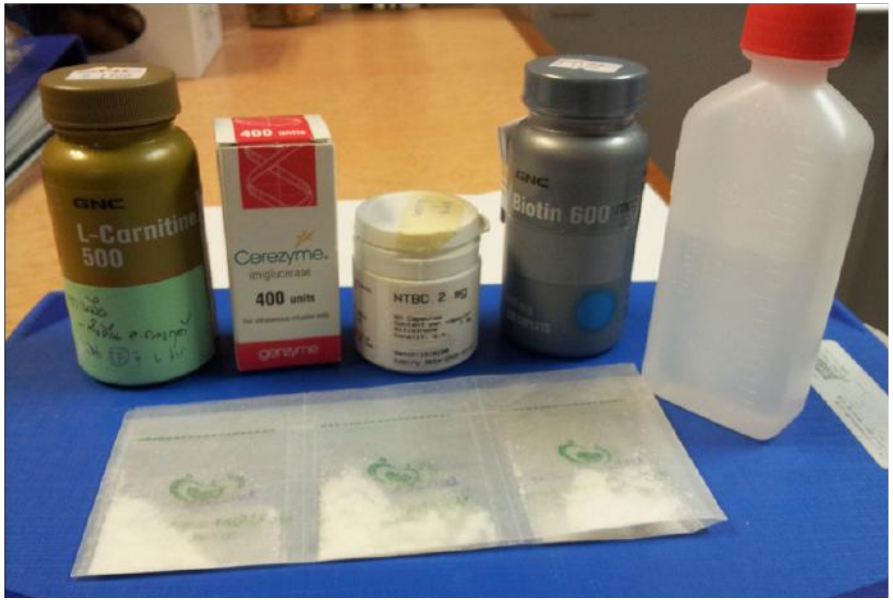


1 for 3.7M

2. Limited Resources (B)

- Most orphan drugs for RD not supplied (except for Hemophilia & Gaucher)

Substitute medicine
Food grade NOT medical grade
No IV form available for critical patients



Donation

3. Reimbursement issue

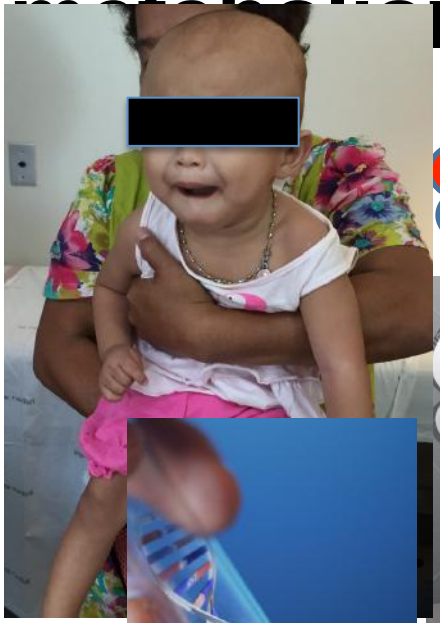
- Most specific **test** (chromosome & gene) not covered by UC & SS

- Amino acid & organic acid are covered, but only with the presence of the patients at the referral center

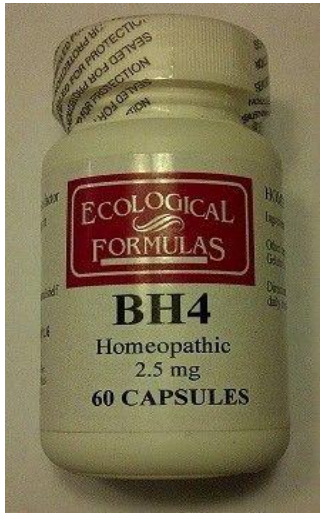
- Most specific treatment & orphan drugs for RD not listed in NLEM, therefore not covered by UC & SS (except for Hemophilia & Gaucher)

- Prevention of reoccurrence: genetic test is not covered by UC & SS

movement
? Dx. BH4
metabolism defect

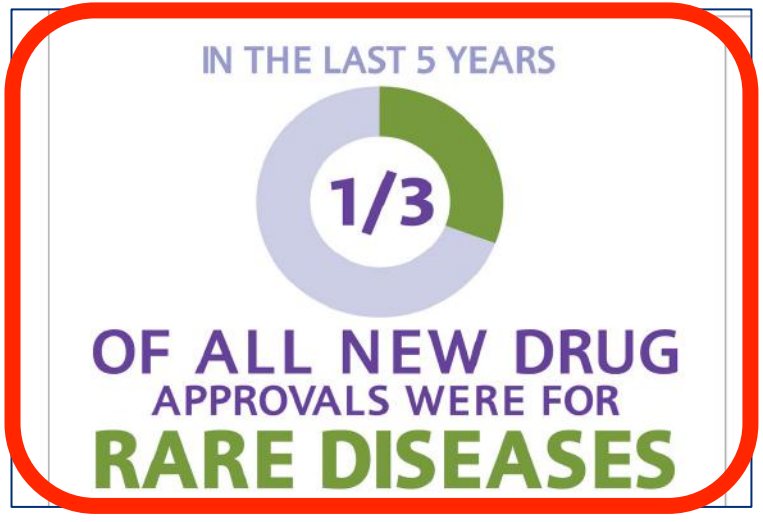


Mutation analysis 8,000 Bahts



BH4 - 66,000 Bahts/y

5-HTP 2,000 Bahts/y



- More of rare disease become treatable
- We are now paying a lot already for supportive care in RD,
  why not diagnose them earlier,
  treat them more specifically and effectively
  to achieve better outcome with less morbidity & mortality
  and to prevent reoccurrence

Solutions

1. Increase Awareness
2. Resource Sharing
3. Capacity Building

1. Increase Awareness

Campaign events
Rare Dis Day Thailand
2011-2016

- Health professional/Student
- PH policy maker
- Population
- Patients

Medical students on the sky train

พิธีกรจิตอาสา: นพ.กฤษฎา ศิรามพุช และคุณดิก กลิ่นสี

คหายาก : ร่วมร้องเพลง “แตกต่างเพื่อสร้างสรรค์”

ศิลปะดนตรีคนพิการเอื้อเฟื้อบทเพลงจากอัลบั้ม Station I)

น้องอิงฟ้า เล่านิทานลูกหมู 3 ตัว

(ผู้ป่วยโรคกล้ามเนื้ออ่อนแรง : SMA)

Ramathibodi Hospital, Mahidol University
Medical Genetics Network, Genetic Society of Thailand
Present

Round Table Meeting on

Rare Disease

Learning from France

Date Friday 11th September 2015
Venue Rm 907, Medical Learning Resource Center
and Ramathibodi School of Nursing Building
Ramathibodi Hospital, Bangkok

Program

13.30 Opening
Assoc. Prof. Thanya Subhadrabandhu
Deputy Dean for Service, Faculty of medicine Ramathibodi Hospital

13.35 Health care policy and strategy for rare disease, The experience of setting up French National Rare disease Plan, Overview of Orphanet
Prof. Odile Kremp
General Directorate for Health, Ministry of Health, France
Professor of Paediatrics, Faculty of Medicine, Catholic University, Lille, France

15.00 Sharing Thai experiences on rare disease policy: part I
Dr. Chuchai Sornchamni
Deputy Secretary General National Health Security Office (Thai UC)

15.30 Sharing Thai experiences on rare disease policy: part II
Dr. Chulaluck Kuptanon
Pediatrician, Queen Sirikit National Institute of Child Health,
Department of Medical Service, Ministry of Public Health

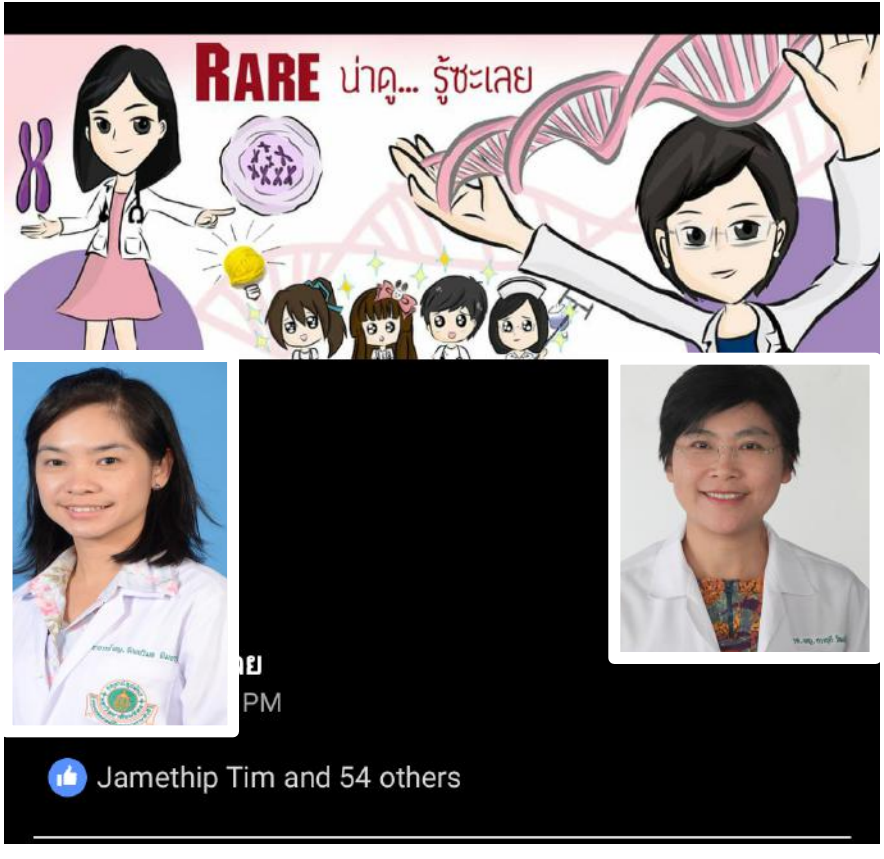



16.00 Open discussion
16.30 Closing

Modulator
Prof. Duangrurdee Wattanasirichaigoon

2015

FB@rarenadoo



Discussion with Policy Maker is Mandatory

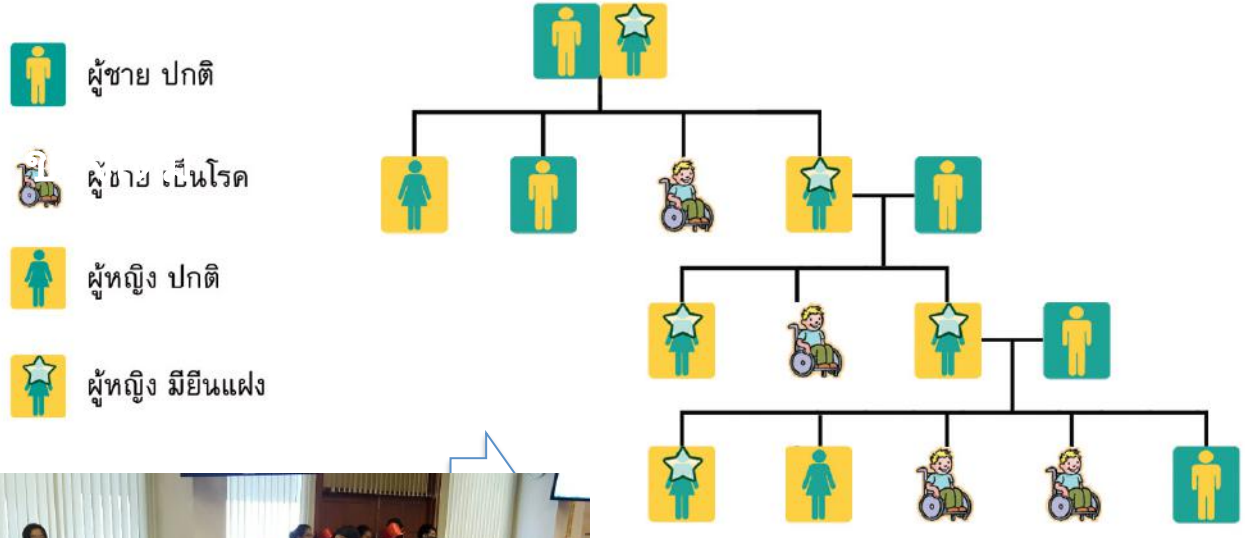
Help from country friends: Prof. Tarun Weeramanthri
Special Seminar on Rare Disease Policy, November 14th, 2016

- Thai MOPH, NHSO, Clinical geneticists, Patient groups
- Pediatricians from public hospitals, MOPH **"Proposal for Thailand RD Network"**

โรคกล้ามเนื้อเสื่อมดูเชน

ตัวอย่าง แผนภูมิครอบครัวโรคกล้ามเนื้อเสื่อมพันธุกรรมในเด็ก (ดูเชน)

Patient Group



6th Rare Disease Day Thailand 2016 → Thai Rare Disease Foundation (ThaiRDF)

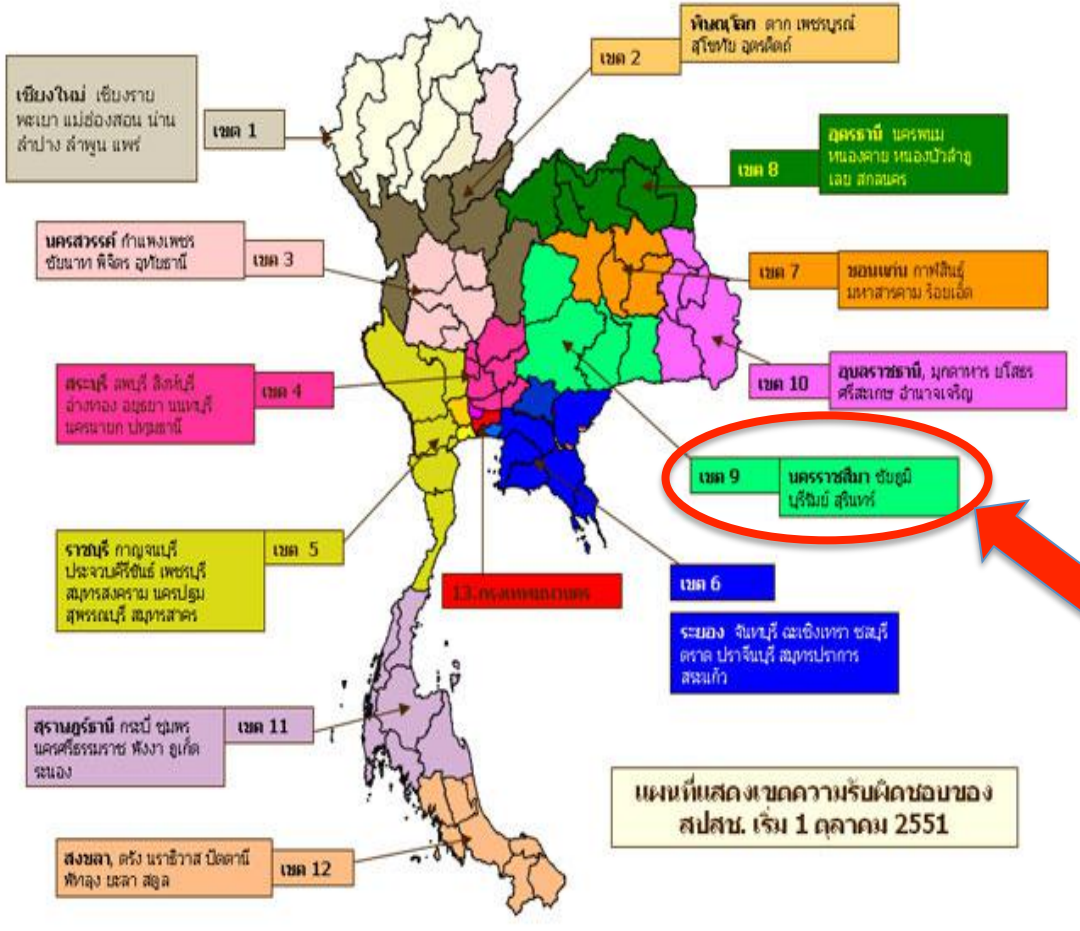
2. Resource Sharing

- Create service platform for resource sharing "Network"
  - To use limited & expensive resources - Wisely, Effectively, Fairly
  - Specialist, Lab test, Orphan drugs
- Tools
  - Patient registry and benefits
  - Expert panel
  - Effective management & coordination supported by Policy
  - Pool and share data → national experience → better guideline
- Orphan drugs registration system and marketing incentive
- Mustistakeholders
  - Doctors, Scientist, Patient group, Policy, Pharma, etc.

Birth Defect Prevention and Treatment led by Prof.Pornswan Wasant

แผนที่แสดงการแบ่งเขตพื้นที่ของ สปสช.

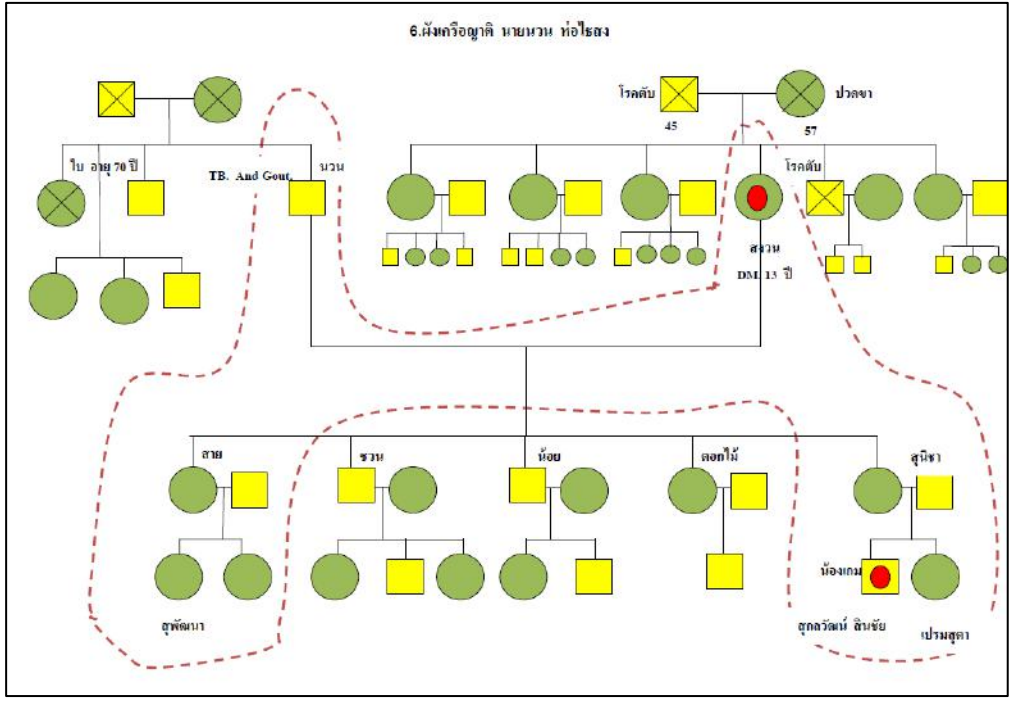
เริ่ม 1 ตุลาคม 2551



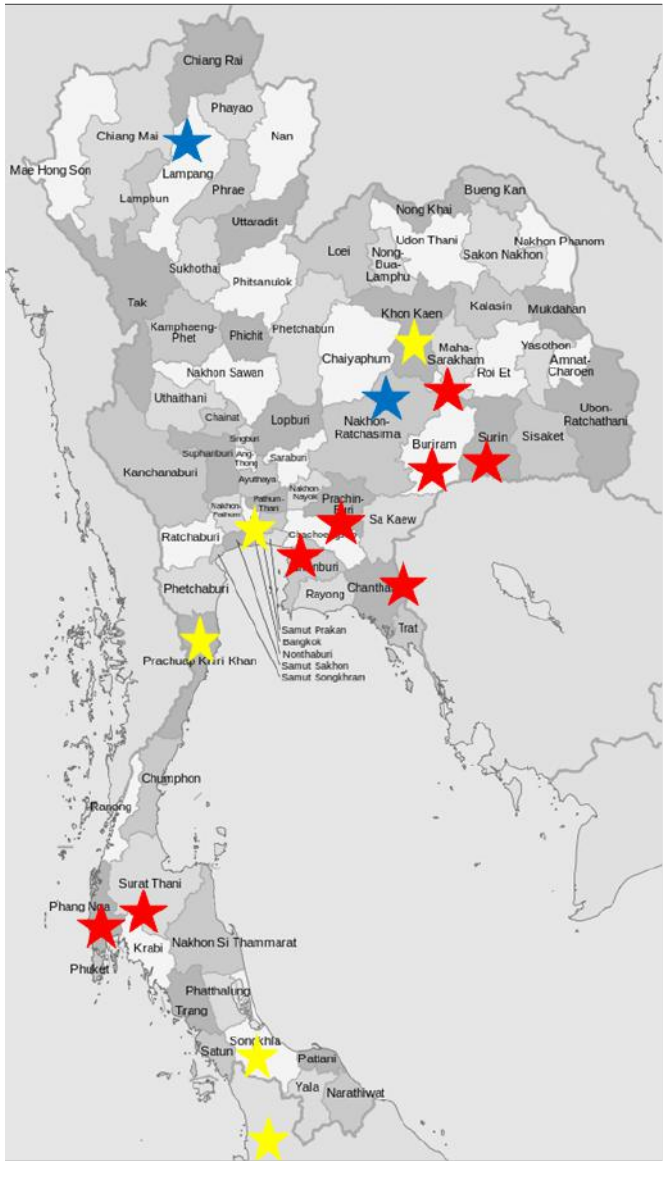
Pilot Project
Birth Defect Prevention Program
(led by Prof.Wasant)

Ramathibodi Hospital

Integration into any MOPH Existing Program
Quality Family Visit: ROF (Relation On Family Tree)
for Noncommunicating Diseases (DM, Hypertension, Stroke)



- Multiple birth defects
- Mental retardation
- Muscle weakness
  (Duchenne muscular dystrophy)
- Recurrent problems in
  multiple family members
- Serious illness of unknown cause
  in children



Duchenne muscular dystrophy model

Provincial Hospital

- Family tree
- Blood collection

Ramathibodi Hospital

- Mutation analysis

- Genetic counseling

- Result report

3. Capacity Building

- Training:
  Specialty: Clinical genetics subboard: 2-yr program
  approved by Royal College of Pediatricians 2016
  Genetic counsellor??
  Laboratory specialist??
  Allies: General Ped-Med-OB, etc, Family physicians
- Clinical Service Center for RD, Undiagnosed Program (UDP)
  & Newborn Screening for Metabolic Disorder
- Research: Each region & country has different problems in detail
  Baseline national data
  Clinical guidelines: Treatment & Diagnosis

ERT for Gaucher Disease by Thai UC: How??

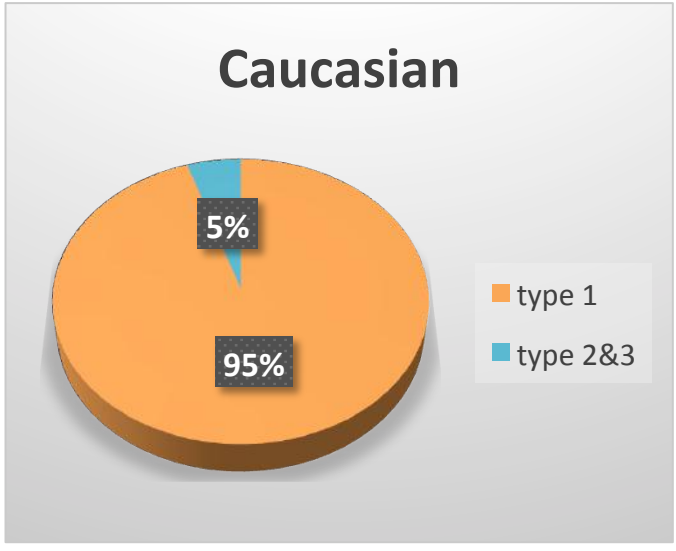
2008 2010
Donated ERT & BMT

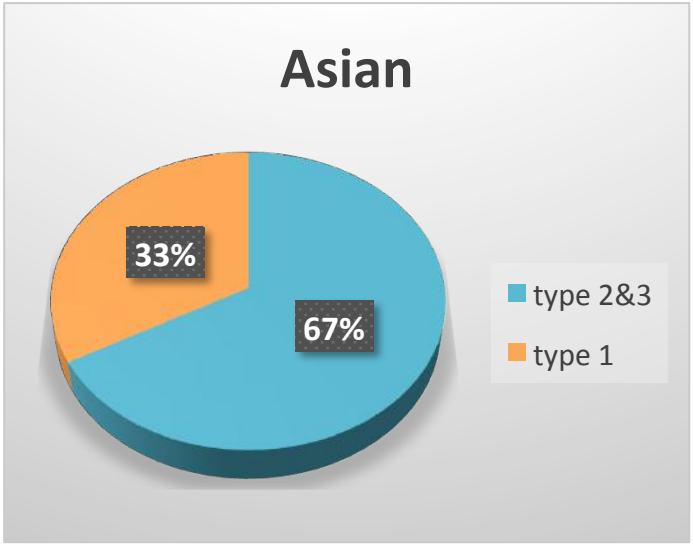
2008 2013
Donated ERT & BMT

2011 to UC

2014 2015
1st Pt ERT by Thai UC
& BMT (half-matched)

Research is needed for each country: Gaucher disease subtype





- More of neurologic subtype 2&3
- Mutation analysis should be done to predict Gaucher subtype
- ERT cannot prevent neurologic manifestation
- Bridging therapy is proposed “ERT followed by BMT before neurologic symptoms present”

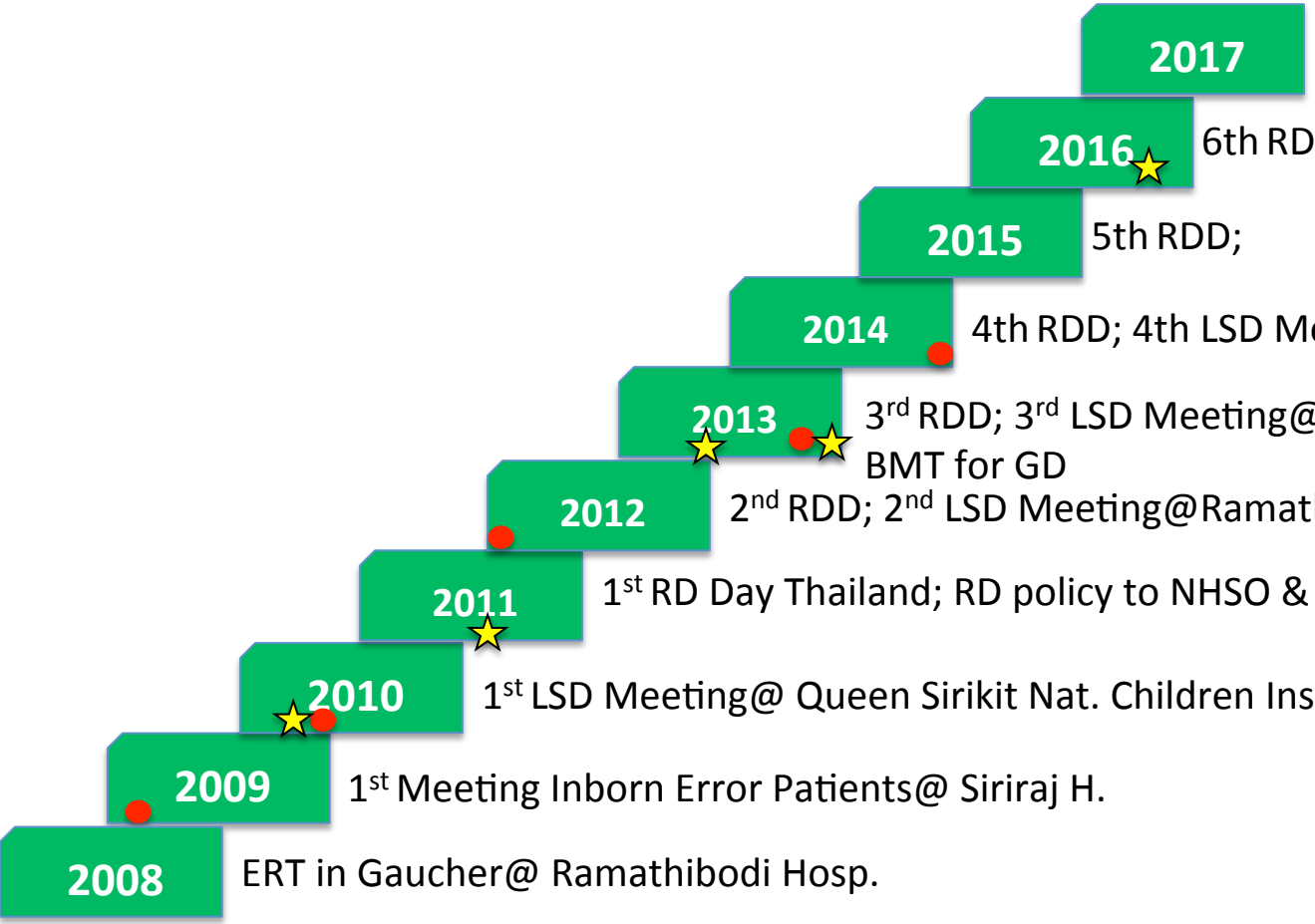
"Proposal for Thailand Rare Disease Network"

Smart

Sustainable

Inclusive

1. Increase Awareness
2. Resource Sharing
3. Capacity Building



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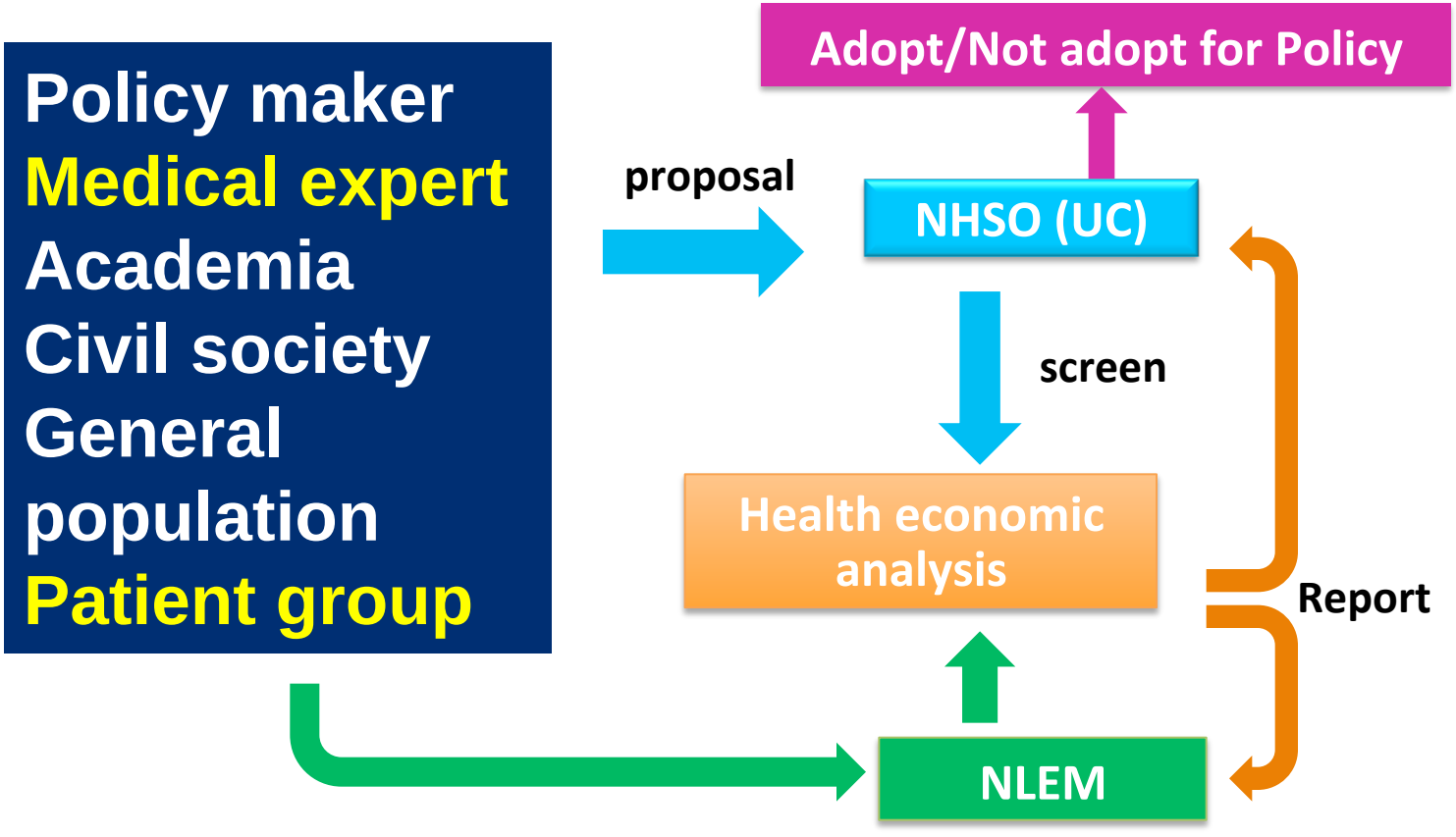
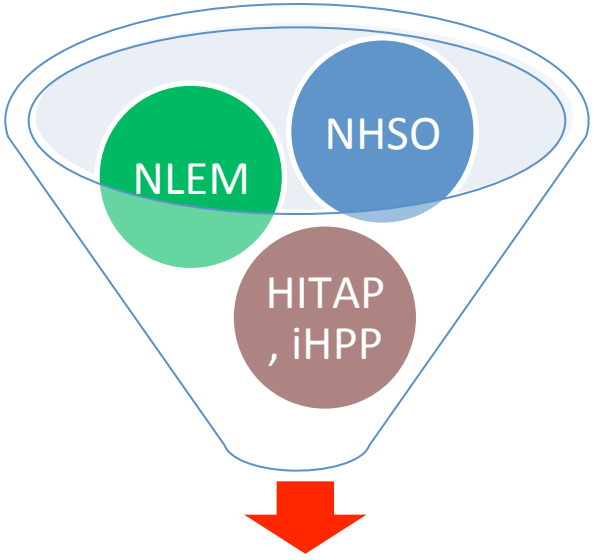
Wisdom of the Land

Development of Thai UC Benefit Package





Medical Benefit Package for UC
หลักประกันสุขภาพถ้วนหน้า

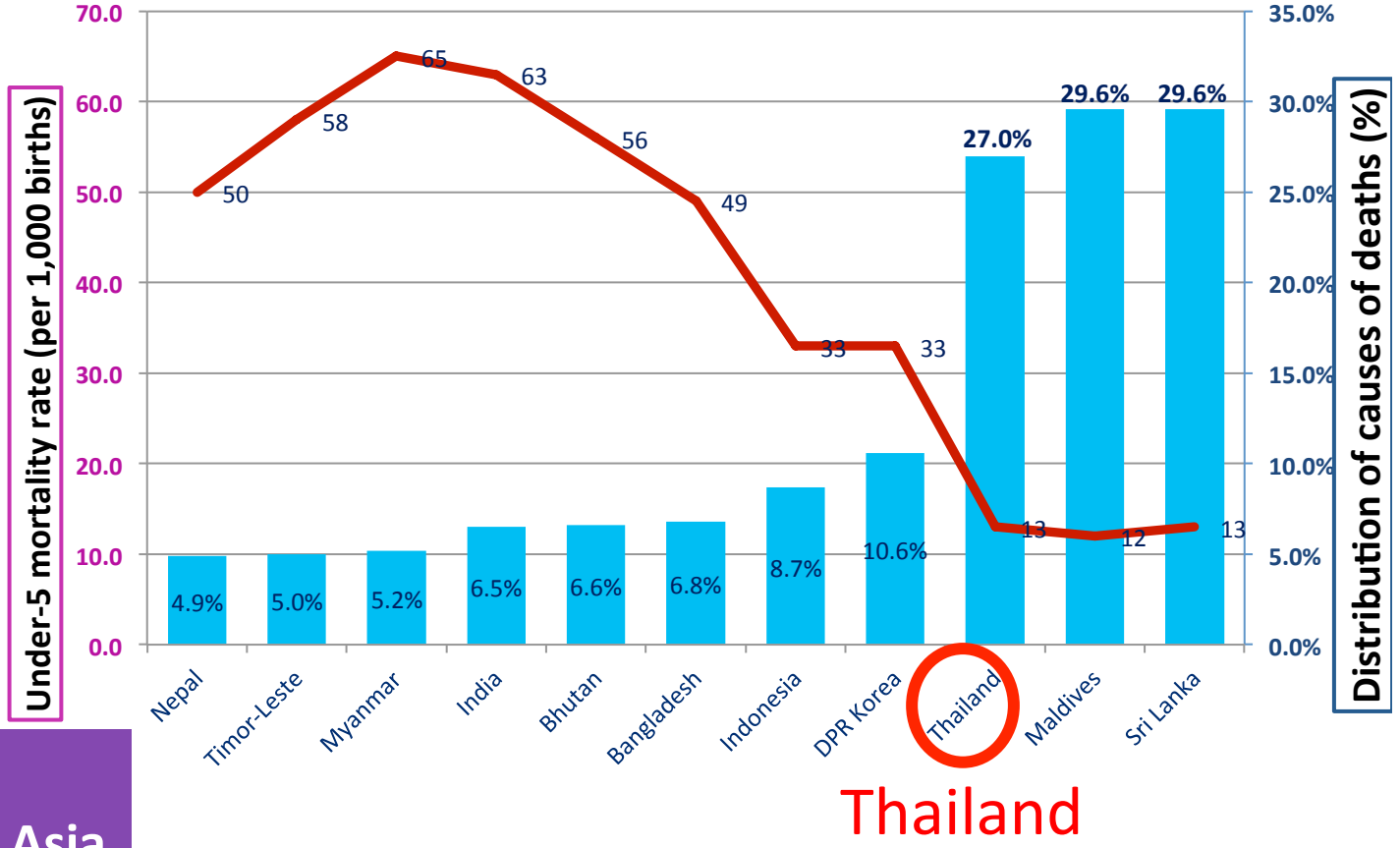
NLEM, National List of Essential medicine บัญชียาหลักแห่งชาติ

NHSO, National Heath Security Office: subcommittee for development of medical benefit อนุกรรมการพัฒนาชุดสิทธิประโยชน์

HITAP, Health Intervention Technology Assessment Program

iHPP, International Health Policy Planning

Mortality rate in children under 5 yr. (red line)
Distribution of the causes of death due to birth defects (blue bar)

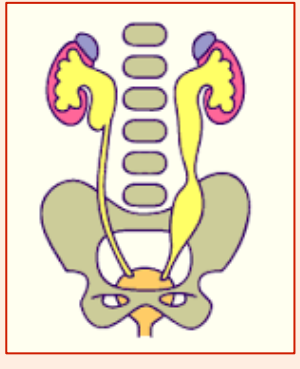


WHO-South East Asia

Thailand

Birth Defects

Structural defect



Functional defect

Environmental factors

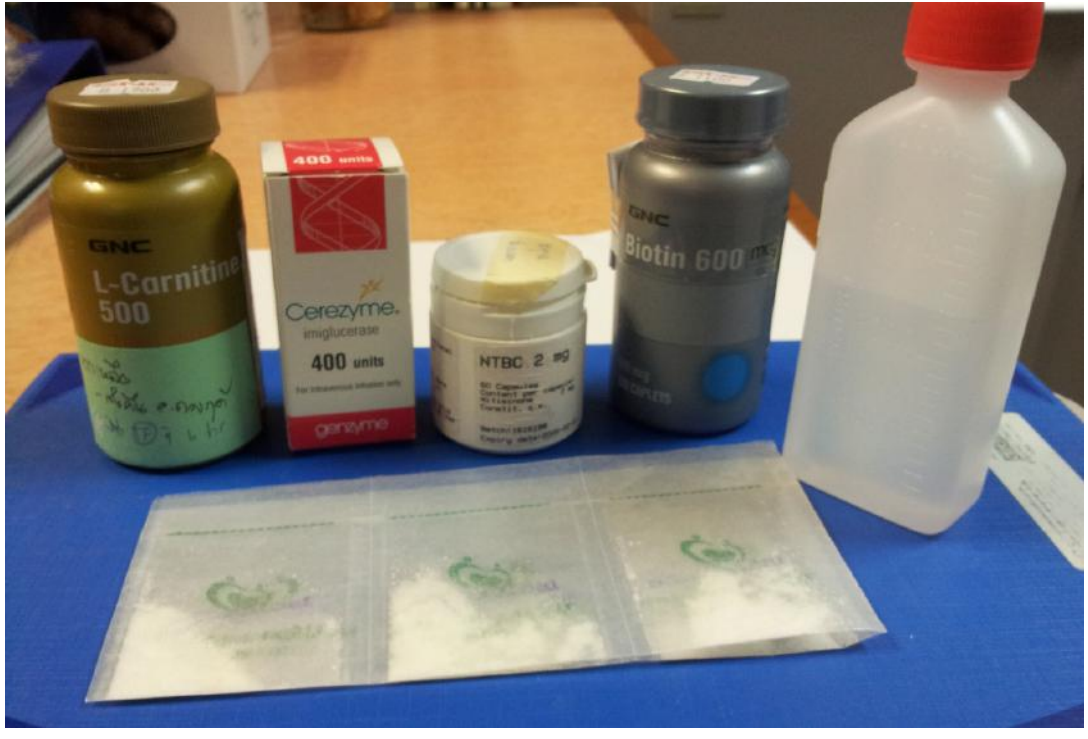
Chromosome defect

Genetic disorder

Substitute medicine

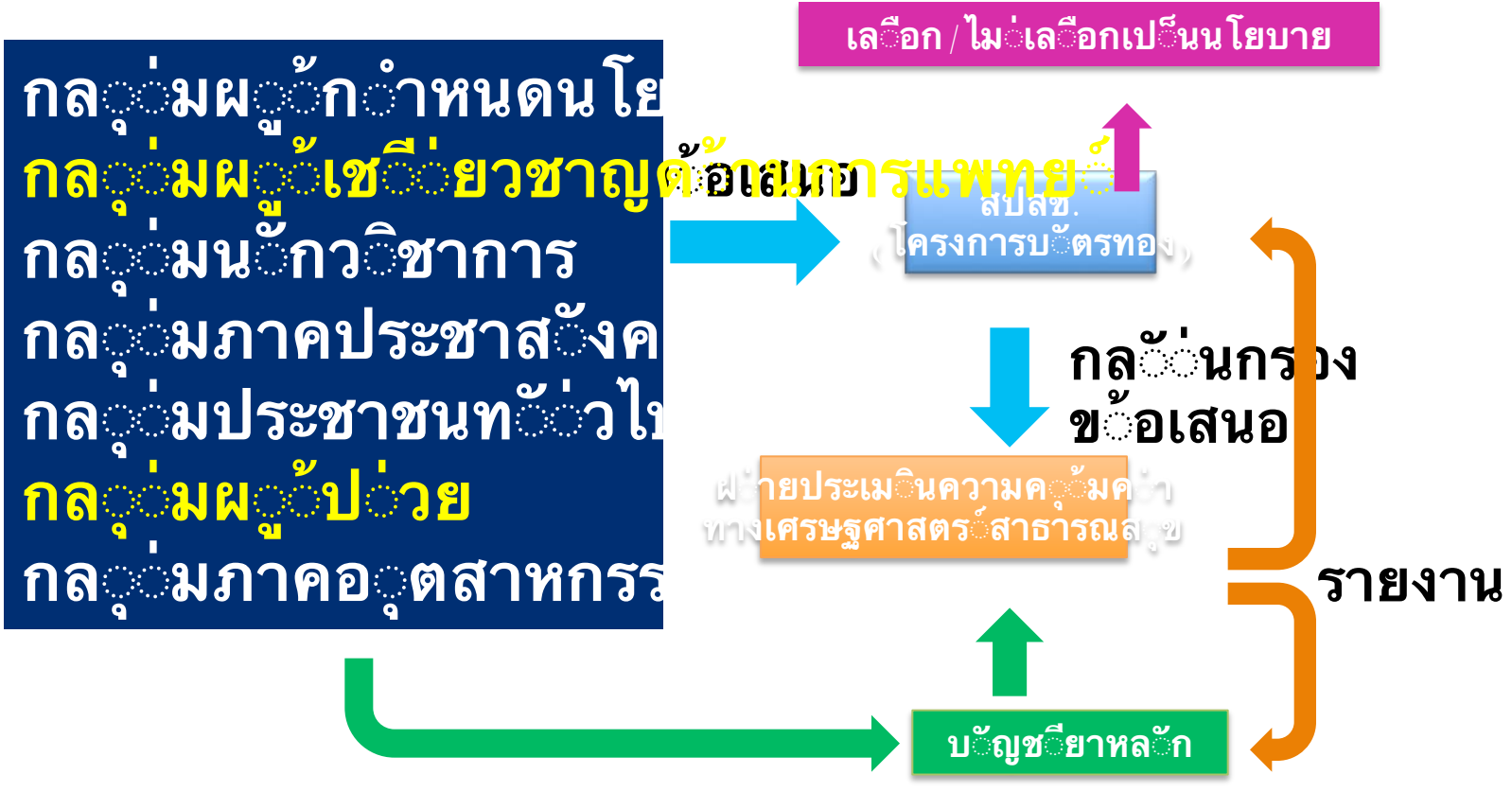
Food grade NOT medical grade

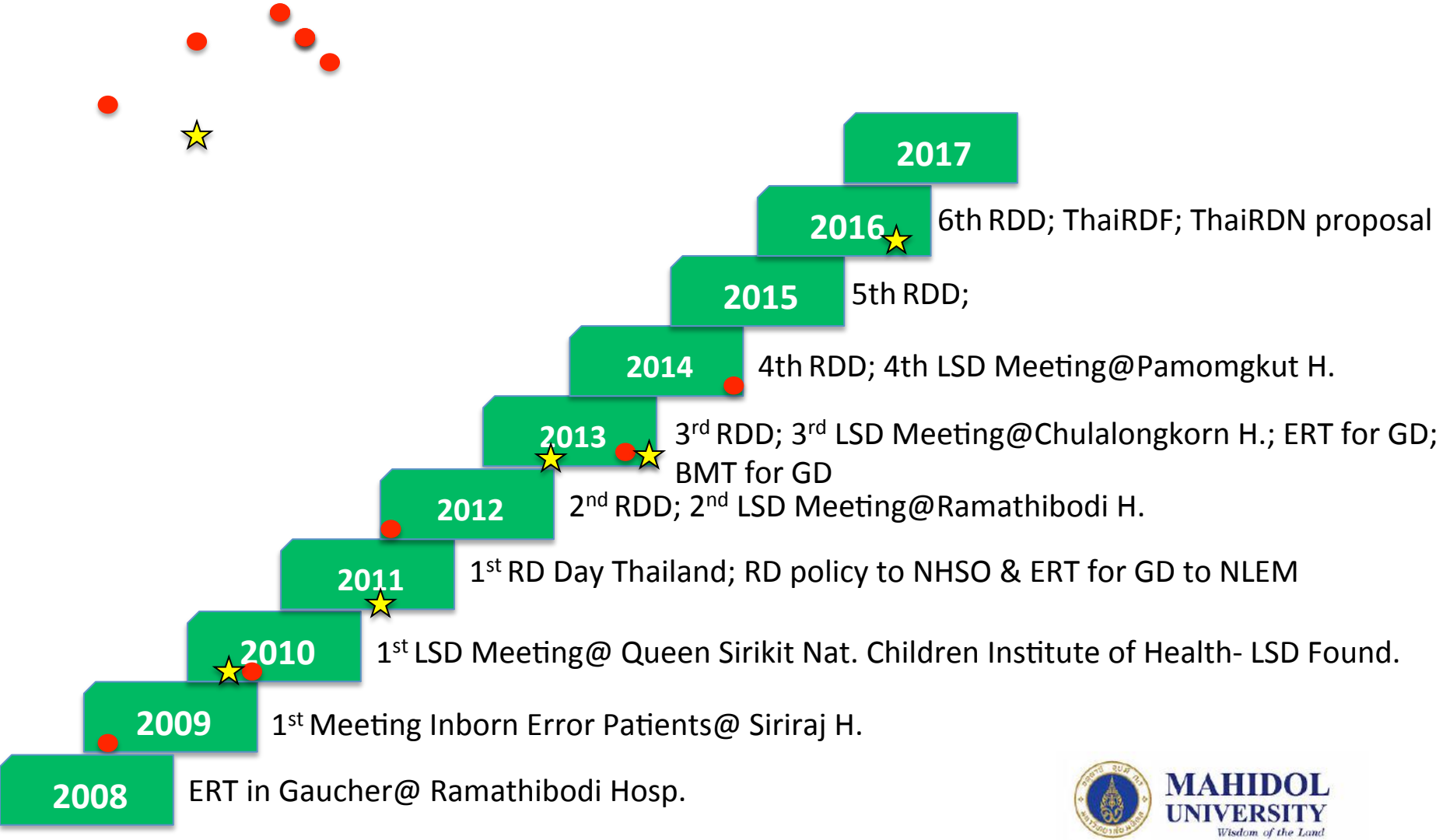
No IV form available for critical patients



Donation

การพัฒนาชุดอนุสิทธิประโยชน์บัตรทอง





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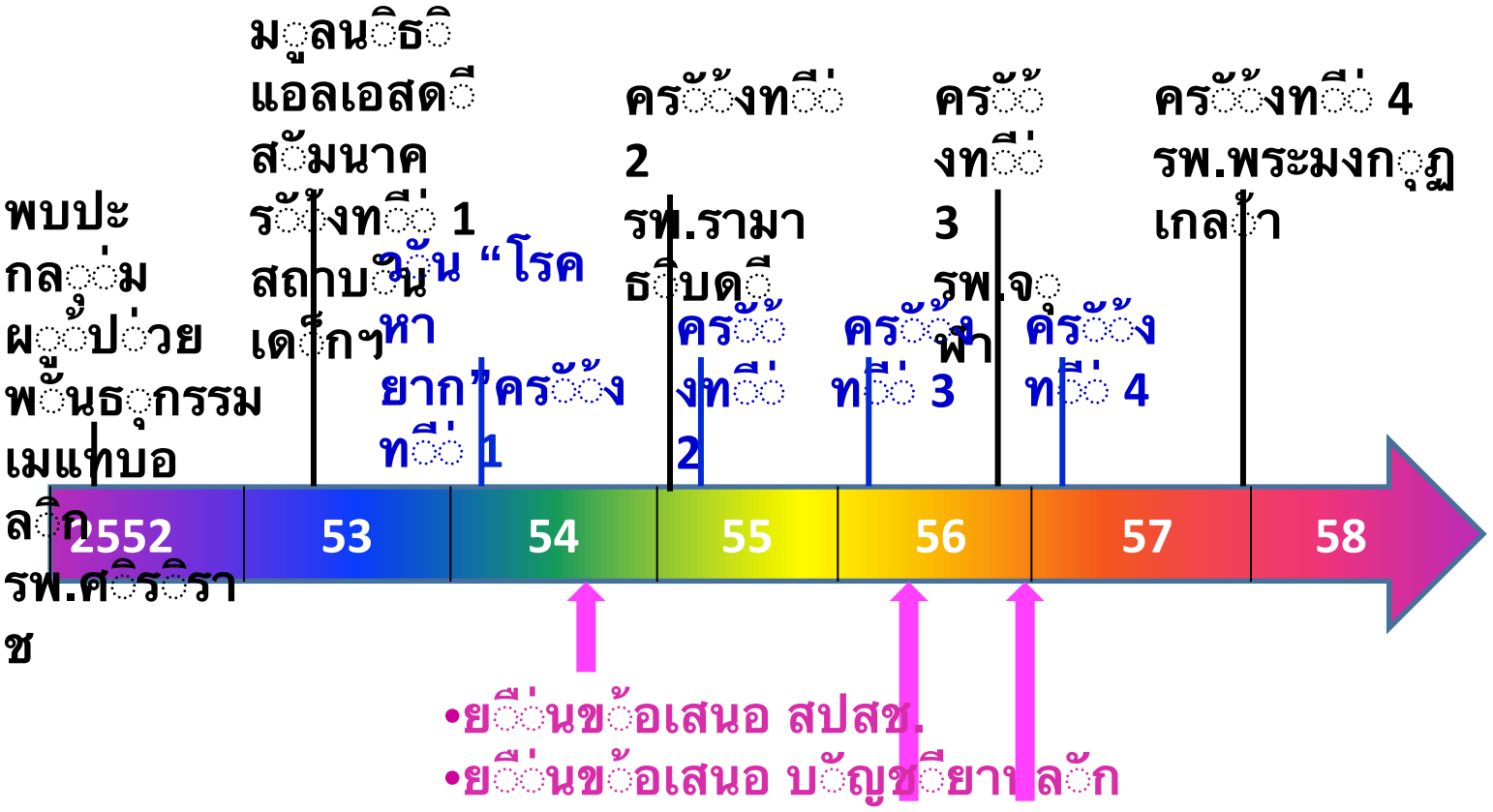
พบปะกลุ่มผู้ป่วยพันธุกรรมเมแทบอลิก รพ.ศิริราช
มูลนิธิแอลเอสดีสัมนาครั้งที่ 1 สถาบันเด็กฯ
วัน “โรคหายาก” ครั้งที่ 1
ครั้งที่ 2 รพ.รามาธิบดี
ครั้งที่ 2
ครั้งที่ 3
ครั้งที่ 3 รพ.จุฬาฯ
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•ยื่นข้อเสนอ บัญชียาหลัก
•ได้ยาโรคกอเชส์
•ปลูกถ่ายไขกระดูก