



The 2nd
**Rare Disease Asia
Conference 2016**

17 - 19 November 2016
Hotel Pullman Bangsar,

ORGANISED BY



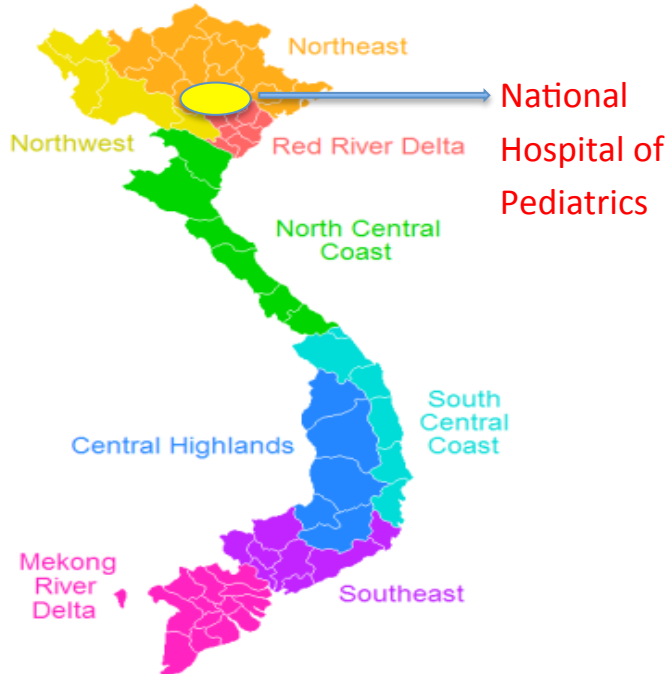
MEDICAL PANEL: Definition & Challenges in Rare Disease Management



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Rare Diseases in Vietnam Priority



~92 M inhabitants 14th in the world & 3 in ASEAN.

Population by age distribution 2016 (Million):

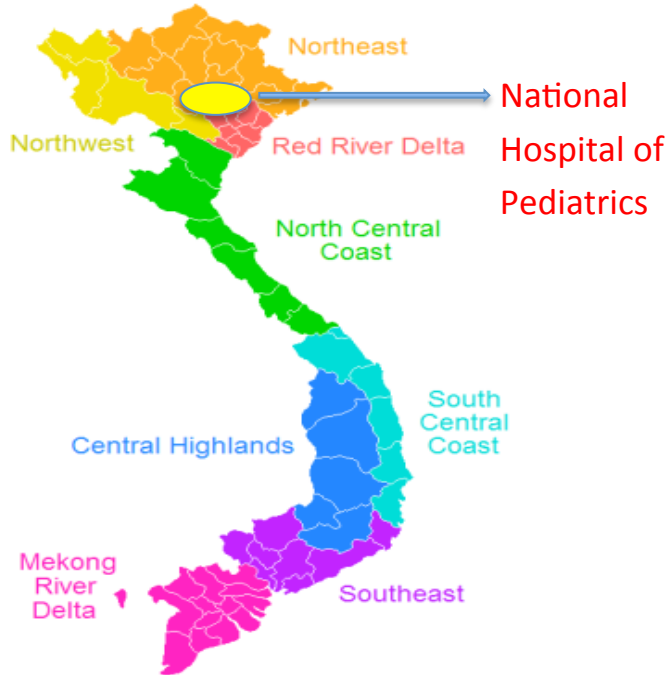
0-14:	20.99
15- 64:	66.85
65+:	6.60

Congenital anomalies including rare diseases accounted about 22% of causes of deaths in children < 5 (2010)

The prevalence (per 1000 live birth) of common serious birth defects: congenital heart defects: 7.9, neural tube defects: 1.9, pathological hemoglobin disorder: 0.9.



National Hospital of Pediatrics



The main tertiary hospital for children in Vietnam.

Serving for a population of approximately 30 million people:

28% referral from Hanoi

72% referral from provinces

1 675 staffs, 1 500 beds, 3 000 pts/day:
outpatients

National or Local Registry

- National /Regional registry is available

for several rare diseases including:

Congenital adrenal hyperplasia,

Duchenne muscular dystrophy,

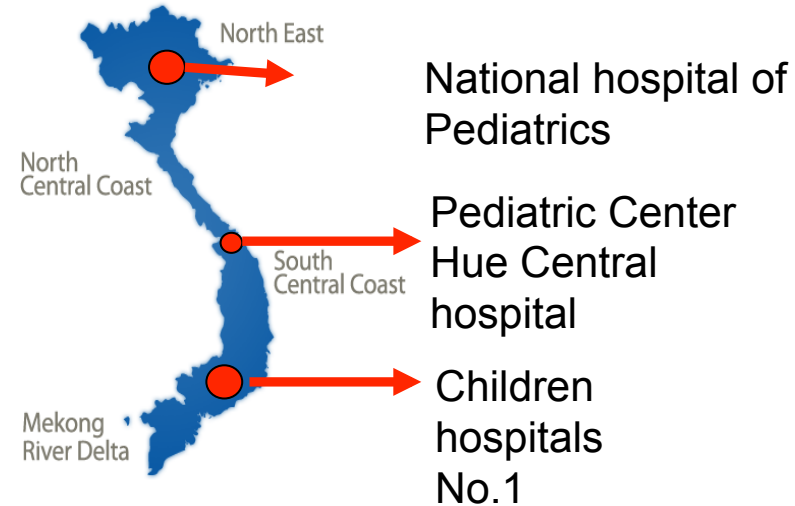
Ambiguous genitalia, Osteogenesis imperfecta,

Turner syndrome, Beta cell disorders,

Mucopolysaccharidosis (MPS), Pompe diseases

(Endocrinology- Medical genetics, Metabolism Dept)

Gaucher disease (Hematology Dept.)



Serving rare diseases in NHP

- International rare diseases day from 2013.
- Introduce: diagnosis, management, activities, supportive care, health care.
- Rare diseases clubs: congenital adrenal hyperplasia, MPS, Duchenne muscular dystrophy...

Challenges

1. Low rare disease awareness even amongst physicians: frequent misdiagnosis or failure to diagnose, inadequate or no treatment, lack of support services.
2. The class of treatment for rare diseases: High cost.
3. No insurance for rare diseases.
4. Lack of registration system for all center.
5. Lab diagnostic system

Solutions

- Training for doctors, nurses: increase medical knowledge.
- Build the scientific data system for rare disease which will be used as evidence based to making rare disease policies .
- Learning best practice from other countries should be advance and saving time to Vietnam .
- Centre of expertise for testing, test may be sent oversea.
- Guidelines for treatment.
- Clinical expertise and patient management.
- Government funding and other sources of funding for rare diseases.

THANK YOU FOR YOUR ATTENTION