



**Asia-Pacific  
Economic Cooperation**

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2018/SOM3/HLM-HE/019

## **Innovative Financing Models for Rare Diseases**

Purpose: Information  
Submitted by: Shire



**Eighth High Level Meeting on Health and  
the Economy  
Port Moresby, Papua New Guinea  
16-17 August 2018**

# Innovative Financing Models for Rare Diseases

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APEC High Level Meeting on  
Health and the Economy



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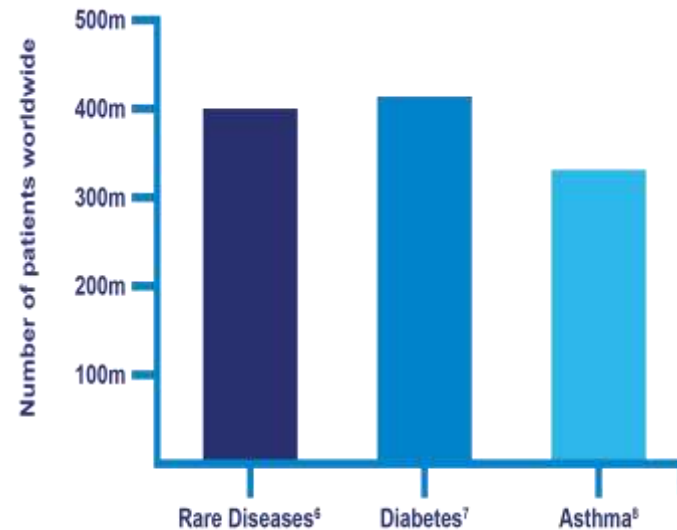
# Rare is not so rare

Rare disease are one of the largest overlooked patient populations in the world

~ 7,000 rare diseases<sup>1</sup> yet each only affects a small number of people

Rare Disease	Number of patients
Gaucher disease	1 in 40,000 (~10,000 worldwide) <sup>2</sup>
Fabry Disease	1 in 80,000 <sup>3</sup>
Hereditary Angioedema	1 in 40,000 (~10,000 worldwide) <sup>4</sup>
Hemophilia	1 in 5,000 (~400,000 worldwide) <sup>5</sup>

~6% of the population has a rare disease<sup>6</sup>



1. Global Genes Factsheet on Rare Diseases. 2016: <https://globalgenes.org/wp-content/uploads/2015/12/2016-WRDD-Fact-Sheet.pdf>
2. Sireesha K, et al. IJPRR 2014;3(2):79-84
3. [http://www.orpha.net/consor/cgi-bin/OC\\_Exp.php?Expert=324](http://www.orpha.net/consor/cgi-bin/OC_Exp.php?Expert=324)
4. <http://www.haea.org/what-is-hae/hae-the-disease>

5. <https://www.hemophilia.org/About-Us/Fast-Facts>
6. Vrueth R de, et al. Background Paper 6.19 Rare Diseases 2013
7. <https://www.diabetes.co.uk/diabetes-prevalence.html>
8. Global Asthma Report 2014

# Rare disease is a public health issue

Rare diseases are often life-threatening, life-debilitating conditions, affecting many children



Over **half** of all rare diseases begin in childhood<sup>1</sup>



**One in three** of these children will die before their fifth birthday<sup>1</sup>



**80%** of rare diseases have a genetic component that cannot be managed through lifestyle changes<sup>2</sup>



Rare diseases are often associated with **lifelong suffering** and **shorter life-spans**<sup>3</sup>

## The impact on those who suffer from rare disease and their families can be profound:<sup>3</sup>

- Low levels of quality of life
- High levels of disability
- Socially isolated
- Economically disadvantaged
- Reduced professional opportunities
- Stigmatized



1. Global Genes Factsheet on Rare Diseases. 2016: <https://globalgenes.org/wp-content/uploads/2015/12/2016-WRDD-Fact-Sheet.pdf>. Accessed February 2018.

2. Rhode J. 2005. Rare Diseases: Understanding the Public Health Priority. Eurordis.

3. Field M, Boat T, Editors. 2010. Rare Diseases and Orphan Products: Accelerating Research and Development. National Academy of Sciences.

# Innovative financing models in rare diseases:

## A Patient-Centric Approach that full of Public Private Partnership Opportunities



- 5-30 years delayed diagnosis:
- PPP: CDSS; AI; cloud;
  - Family doctor
  - Neonatal screening

- Often require highly coordinated, long-term care
- 'Shared Nurse' APP
  - PPP with cold-chain delivery service providers



- Per-patient treatment cost tends to be higher:
- Government coordination:
  - Payer innovation: mobilize medical savings account
  - NGO & industry; PAP (deductible and self pay)
  - Sin tax
  - Internet payment / credit

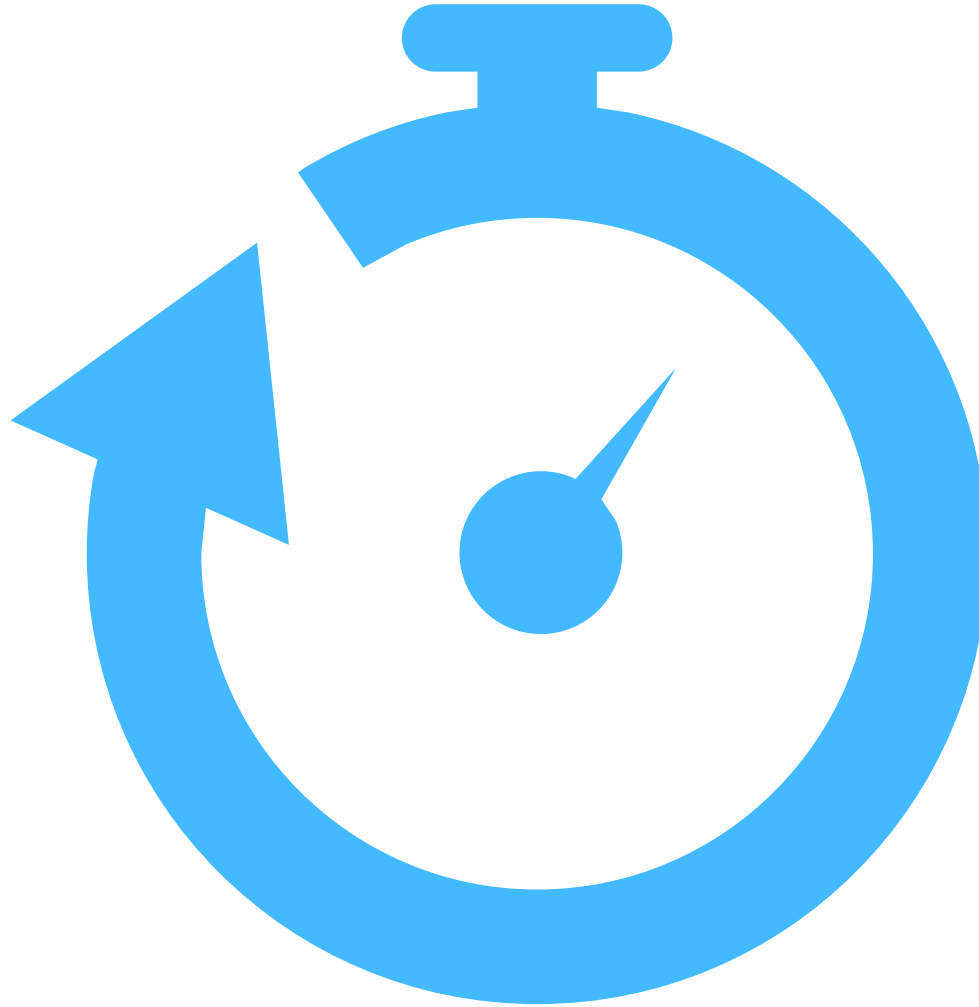
- Lack of data to demonstrate the value
- Registry + patient app
  - PPP: government endorsed utilization and outcome tracking



Eco-System



# Patients are waiting



# Our ambition

To work together with authorities and the rare disease community to ensure patient access to rare disease treatments upon regulatory approval

A proposed approach in rare diseases where the treatment meets one of 3 criteria

1

The only option for patients

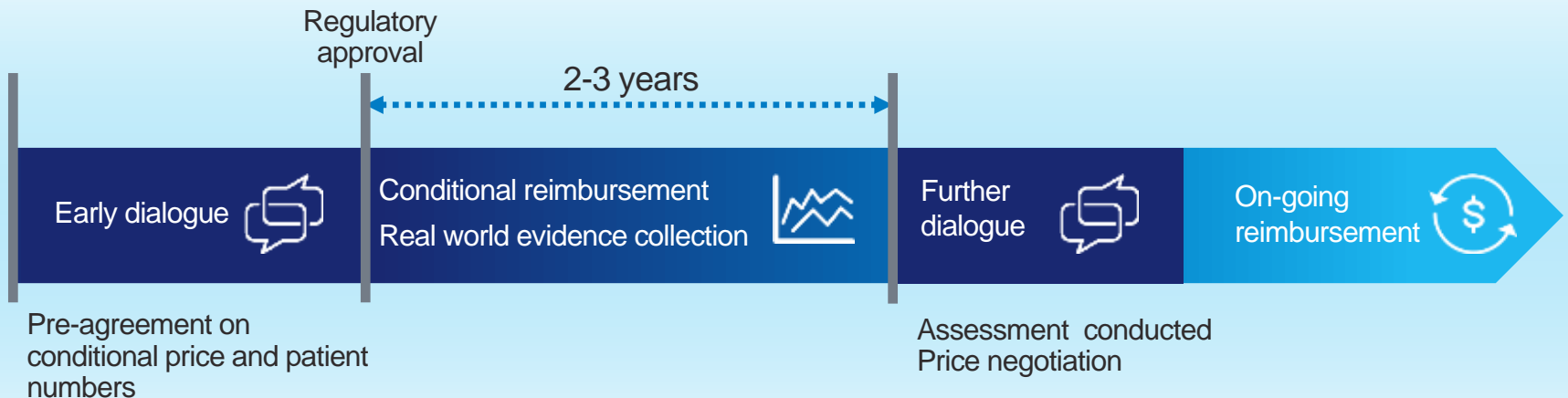
2


Patients experiencing high unmet need

3

Innovative therapeutic advance

## Conditional reimbursement for up to a 3-year period post-regulatory approval



 Existing approaches in France and Germany

# Thank You!

