



NGO COMMITTEE FOR  
**RARE DISEASES**

# EVERY LIFE COUNTS



[www.mymlda.com](http://www.mymlda.com)

# HOPE

by Malaysia Lysosomal Diseases Association



A Committee of **CoNGO**



CoNGO  
The Conference of NGOs  
in Consultative Relationship  
with the United Nations



# Why MALAYSIA LYSOSOMAL is formed? DISEASES ASSOCIATION



By TAN SHION CHIN

starhealth@thestar.com.my

**I**N THE not-too-distant past, it was common for parents to pray for boys to carry on the family line.

Nowadays, most parents just hope that their newborn child will be healthy, with all their fingers, toes and everything else in the right place.

And rightfully so, because there can be no harder blow than when a child is born with a defect or abnormality.

But it is far worse when the baby turns out to have a rare disease, simply because of the difficulty in obtaining a diagnosis and treatment, as well as the general lack of knowledge about such conditions.

### Two in the family

Engineer Lee Yee Seng, 37, and stay-at-home mum Chia Kian Poon, 41, have three children. The eldest, 11-year-old Yen Ling, and youngest, Wei Ling, 7, are girls, with the middle one is Sze Hong, a nine-year-old boy.

While Wei Ling was always a sickly child from birth, it wasn't until her first birthday that it dawned on her parents that there was something seriously wrong with her.

Says Chia: "At one year of age, Wei Ling was losing out on all the gross motor development skills."

"Normally, at one, kids can stand (with support), but she couldn't stand or even crawl. And she fell sick very easily, and it would drag on for a long time."

In addition, she had very poor weight gain, despite a good appetite, and was prone to choking on food easily.

Around that time, she was also suffering from a respiratory infection. As it had dragged on for almost two months without improvement, despite oral antibiotics, she was admitted to the Seremban General Hospital.

Upon taking a chest X-ray, it was discovered that she had an enlarged heart, and later,

# A hard, lonely road

Two families share their experiences with rare diseases.



An energetic Sze Hong runs ahead of (from left) Yen Ling, Lee, Wei Ling and Chia, as the family goes for a walk around the neighbourhood. He was fortunate enough to miss out on the genetic lottery that gave his sisters Pompe disease. - UU BAN/The Star



Chia holding the then 20-month-old Wei Ling, who has obviously weak muscle tone - a typical symptom of Pompe disease.

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Before ERT



2005



After ERT – Wei Ling life takes a quick u-turn...



2006



2007



2008



2011

The Miracle of  
MYOZYME



玮玲就医后获得重生



Before ERT



2001



2003



After ERT – Yen Ling life was back to normal...



2007



2008



2011

燕林过着平凡人的生活

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# 1<sup>st</sup> MLDA Protemp Committee on April Fool 2011

Malaysia Lysosomal Diseases Association (MLDA) was founded by 7 families.

## Our Vision

To ensure that all patients who suffering from LSD receive proper medical treatment and enjoy equal opportunities in life regardless of race gender, age, education and religion.

## Our Mission

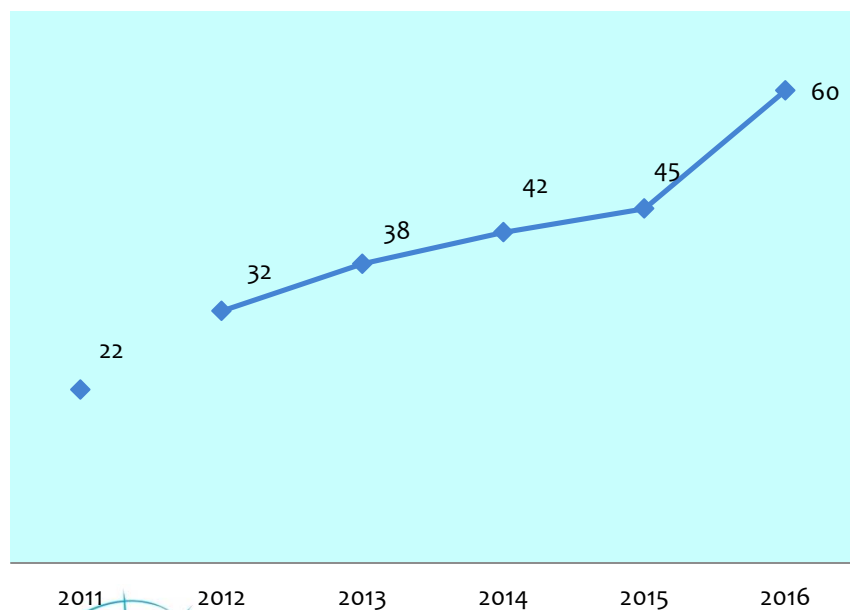
1. Educate and increase public awareness on the existence of LSD
2. Advocate and work with policy makers towards better suuport for LSD patients.
3. Support the welfare of LSD patient and their families.



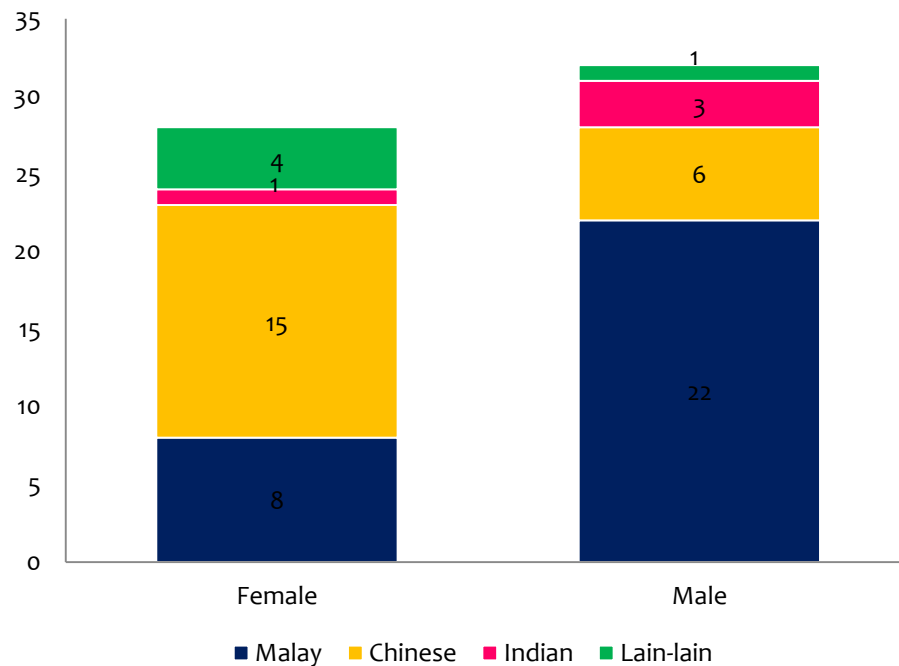
# Statistics

## Patients Count

—◆— 2011 —◆— 2012 —◆— 2013 —◆— 2014 —◆— 2015 —◆— 2016



## Ethnicity and Gender Distribution





# ACTIVITIES HIGHLIGHT

❖ Ice Bucket Challenge Sep 2014 Subang Jaya



# ACTIVITIES HIGHLIGHT

❖ Every Life Counts Charity Dinner Empire Hotel 2016  
Fund Raised 116k USD





# ACTIVITIES HIGHLIGHT

❖ Provided 11 families with money allocation every 2 month.

1 Muhamad Shapingi bin Hashim	Sunsidising	200.00
2 Salbiah binti Mohd. Razali	Sunsidising	400.00
3 Subaramaniam A/L Nagappan	Sunsidising	200.00
4 Paezah binti Awang Puteh	Sunsidising	200.00
5 Bong Siaw Fui	Sunsidising	400.00
6 Norhaiwati binti Che Wil	Sunsidising	200.00
7 Sunarti binti Che Wil	Sunsidising	400.00
8 Faizal bin Sait	Sunsidising	200.00
9 Nur Nadia Bt Shamsudin	Sunsidising	200.00
10 Jussena Ata Ak Jaboh	Sunsidising	360.00
11 Mohd Najmudin bin Yasok	Sunsidising	200.00

**Total**

**RM 2,960.00**



# PATIENT SUPPORT & CARE

MLDA procured 3 mini Ipad, 5 sofa bed(USD 1100) and freezer +Data net (USD 4100) for Hospital Kuala Lumpur children.



Aldurazume  
RM 30,000  
Before BMT



CPAP  
(USD1,000)  
machine



5 sofa bed  
(USD 1,000)



Freezer  
+Datanet  
USD 4,100



Bipap  
(USD11,000)  
machine



# International Collaboration





# International Collaboration

Singapore 2012 – Asia Orphan Drug Congress



Osaka Japan 2013- international MPS Meeting



Bahia Brazil, 2014 – International MPS Meeting



Singapore 2015 – First Asia Rare Disease Conference



Bonn, German 2016 – International MPS Meeting



Today



Next Week



Patient Advocacy Group Panel - Landscape of the Support Networks in Asia

17<sup>th</sup> Sep 2016

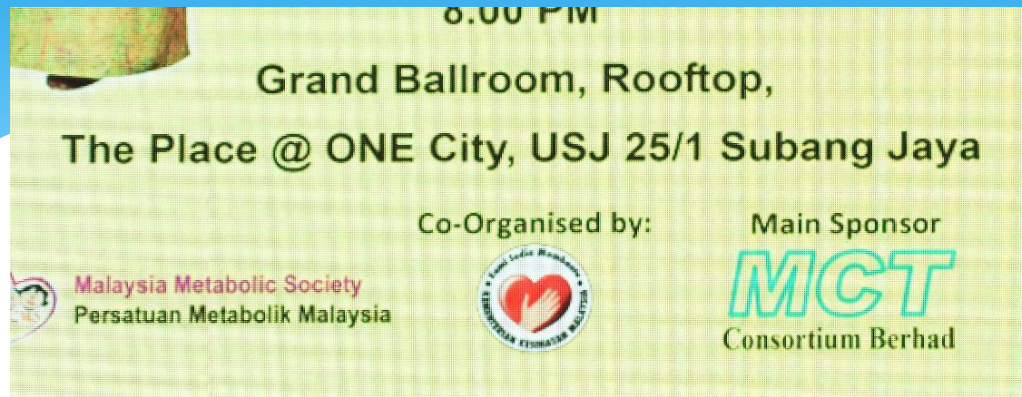


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# Every Life Counts Charity Dinner 2014 One City, USJ

Fund Raised 163K USD



## 2014 Malaysia Charity Dinner





Every Life Counts Charity Dinner 2014 One City, Malaysia  
Health Minister granted us RM 2.5mil .  
We use 2 mil to procure drugs to save 7 life.  
Another 0.5 mil for genetic screening for MPS II



*The King receives a gift of one of the members of MLDA*



*The Minister of Health welcomed by Haziq a MPS type 6 patient*

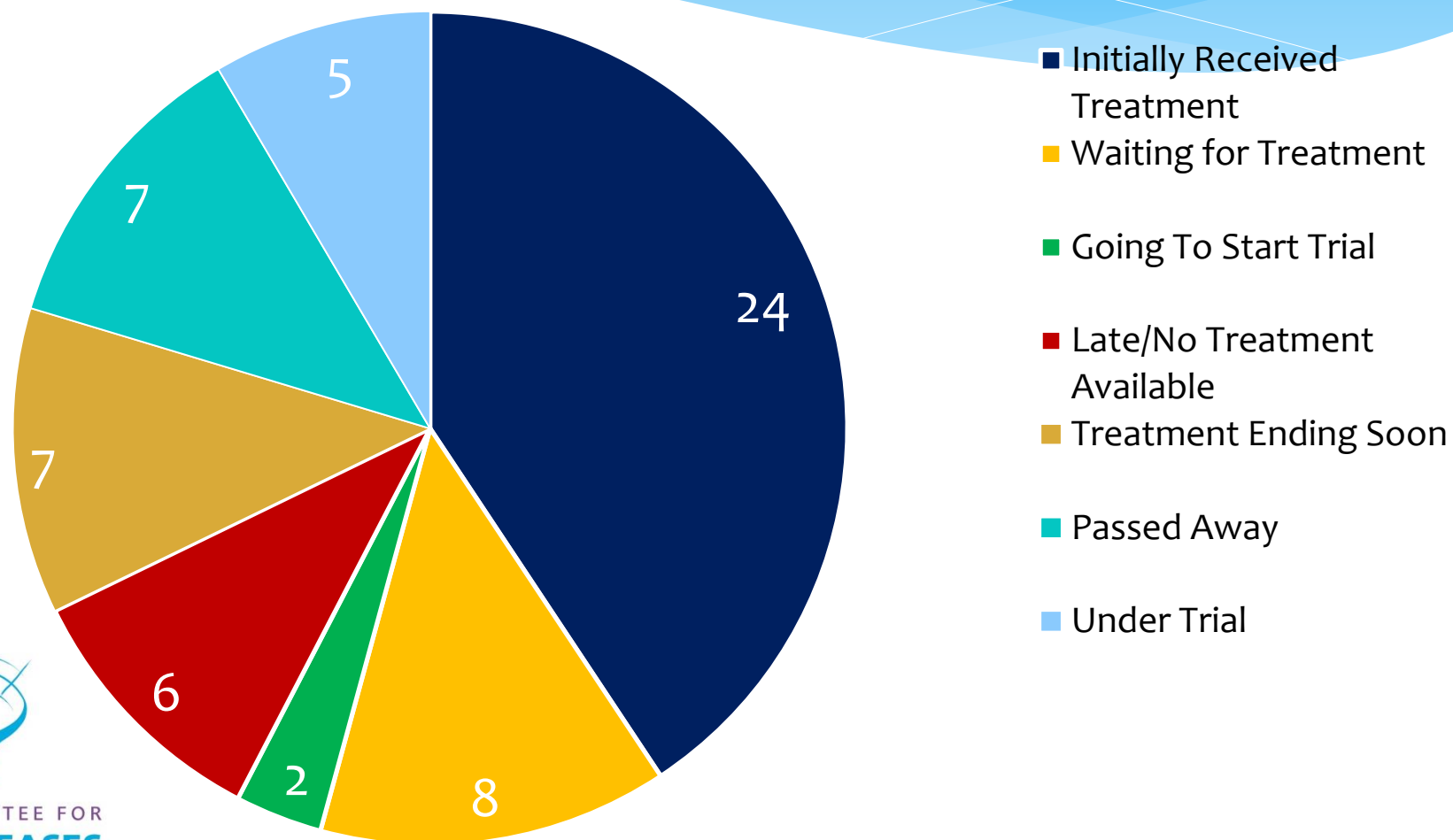


# Our Challenges







# Statistics

## Patient Treatment Status








# TREATMENT ENDING by Jan 2017

No	Name of Patients	Age (years)	Location	Disease	ERT Treatment Status	Estimation Cost per Year	ERT Start Date	ERT End Date	Efficiency of ERT	Medication
1	Muhammad Hazim Imran Bin Faizal 	11	Shah Alam, Selangor	MPS Type II Hunter	Ending by Apr 16	RM 994,500.00	15 Apr 2015	14 Apr 2016	<ul style="list-style-type: none"> <li>- Finger less stiff</li> <li>- Appetite Improved</li> <li>- Taller</li> </ul>	Elaprase or Hunterase
2	Liew May Yee @ Joel 	28	Ipoh, Perak	GSD Type II Pompe	Ending by Apr 16	RM 1,000,480.00	16 Apr 2015	15 Apr 2016	<ul style="list-style-type: none"> <li>- Able to walk around unaided</li> <li>- Neck is stronger</li> <li>- Able to stand up unaided</li> </ul>	Myozyme
3	Maclean Korinth Anak Malken 	7	Kuching, Sarawak	MPS Type II Hunter	Ending by Apr 16	RM 976,083.00	29 Apr 2015	28 Apr 2016	<ul style="list-style-type: none"> <li>• Able to walk up the staircase unaided</li> <li>- Increased in immunity</li> <li>- Improved in balancing</li> </ul>	Elaprase or Hunterase
4	Afiq Luqman Bin Mad Nawi 	12	Kelang, Selangor	MPS Type II Hunter	Ending by Jun 16	RM 979,766.00	2 Jun 2015	1 Jun 2016	<ul style="list-style-type: none"> <li>- More active in sports and physical activities</li> <li>- Less snoring</li> <li>- Stomach no more swollen</li> </ul>	Elaprase or Hunterase

# TREATMENT ENDING by Jan 2017




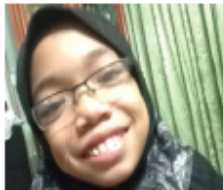
No	Name of Patients	Age (years)	Location	Disease	ERT Treatment Status	Estimation Cost per Year	ERT Start Date	ERT End Date	Efficiency of ERT	Medication
5	Darmash A/L Subaramanlam 	16	Mentakab, Pahang	MPS Type II Hunter	Ending by Aug 16	RM 913,466.00	6 Aug 2015	5 Aug 2016	- Stomach no more swollen - Fingers less stiff - Facial features have soften	Elaprase or Hunterase
6	Lim Wei-Jann 	7	Puchong, Selangor	MPS Type II Hunter	Ending by Sep 16	RM 1,042,383.00	23 Sep 2015	22 Sep 2016	- Stomach no more swollen - Fingers less stiff - Facial features have soften - Able to walk up the staircase unaided - More verbal	Elaprase or Hunterase
7	Muhammad Shafwan Bin Mohd Salim 	8	Tanah Merah, Kelantan	MPS Type II Hunter	Ending by Mar 17	RM 699,833.00	1 Apr 2016	31 Mar 2017	- Just started	Elaprase or Hunterase
						RM 6,606,511.00				

# In memorial of Darmash








# STILL WAITING FOR TREATMENT

No	Name of Patients	Age (years)	Location	Disease	ERT Treatment Status	Waiting Period	Estimation Cost per Year	ERT Start Date	ERT End Date	Efficiency of ERT	Medication
W 1	Aaron Ng Yik Fung 	4	Ampang, KL	MPS Type VI Maroteaux-Lamy	Waiting	2 years	RM 842,400.00	-	-	-	Naglazyme
W 2	Adnin Bt Baharudin 	16	Subang Jaya, Selangor	MPS Type IVA Morquio	Waiting	2 years	RM 1,347,840.00	-	-	-	Vimizim
W 3	Ahmad Adam Bin Sharin 	7	Petaling Jaya, Selangor	MPS Type II Hunter	Waiting	2 years	RM 773,500.00				Elaprase or Hunterase
W 4	Aqilah Bt Baharudin 	14	Subang Jaya, Selangor	MPS Type IVA Morquio	Waiting	2 years	RM 1,198,080.00	-	-	-	Vimizim

# STILL WAITING FOR TREATMENT


No	Name of Patients	Age (years)	Location	Disease	ERT Treatment Status	Waiting Period	Estimation Cost per Year	ERT Start Date	ERT End Date	Efficiency of ERT	Medication
W 5	Muhammad Arman Bin Sharin 	3	Petaling Jaya, Selangor	MPS Type II Hunter	Waiting	2 years	RM 552,500.00	-	-	-	Elaprase or Hunterase
W 6	Siti Nur Shahira Bt Muhd Nizam 	12	Rawang, Selangor	MPS Type IVA Morquio	Waiting	2 years	RM 1,123,200.00	-	-	-	Vimizim
W 7	Woon Jia Huey 	17	Cheras, KL	MPS Type IVA Morquio	Waiting	2 years	RM 1,572,480.00	-	-	-	Vimizim
W 8	A male patient	23	Ipoh, Perak	Pompe	Waiting	3 years	RM 811,200.00				
						Total:	RM 8,221,200.00				
						Grand Total:	RM 14,827,711.00				

RESEARCH

Open Access



# State of rare disease management in Southeast Asia

Asrul Akmal Shafie<sup>1\*</sup> , Nathorn Chaiyakunapruk<sup>2,3,4,5</sup>, Azuwana Supian<sup>1</sup>, Jeremy Lim<sup>6</sup>, Matt Zafra<sup>6</sup> and Mohamed Azmi Ahmad Hassali<sup>1</sup>



**Fig. 1** Rare disease journey-Progress of SEA and benchmark countries



**Table 1** National initiatives to support rare diseases and access to orphan drugs

	Indicator	Philippines	Singapore	Malaysia	Indonesia	Vietnam	Thailand
Health system	GDP per capita (USD)	2,588	54,007	10,440	3,551	1,755	5,480
	Health expenditure per capita (USD)	119	2,426	410	108	103	215
	Total health expenditure (% of GDP)	4.6	4.7	3.9	3.0	6.6	3.9
	Health coverage (%) [37]	76	100	100	48	55	98
	Out of pocket health expenditure (% of total expenditure on health)	52.0	58.6	35.6	45.3	48.8	13.1
	Life expectancy at birth (years) [40]	69	82	75	70	73	75
	Mortality rate, infant (per 1,000 live births) [40]	22	1.8	7	32	16	11
Governance	Presence of national strategy	Yes	No	In progress	No	No	No
	Definition of rare disease	Yes	Yes	Yes	No	No	No
	Comprehensive rare disease legislation	Yes (2015)	No	No	No	No	No
Awareness	Orphan drug act/ legislation	Pending approval	Yes (1991)	No	No	No	No
	Patient support/advocacy groups	PSOD	RDSS	MLDS, MMA, MRDS	No	NPH RD club	Genetic LSD Foundation
	Patient support group activity	Yes (since 2010)	Yes (since 2013)	Yes (since 2010)	No	Yes (since 2013)	Yes (since 2011)
	Media attention (Based on Factiva search from 2009–2015)	72 articles	48 articles	33 articles	13 articles	26 articles	12 articles
Clinical expertise	Presence of patient registries	Largely institution specific			No	No	Planned, LSD diseases
	Presence of centre of expertise	Yes (1)	Yes (2)	Yes (3)	No	Yes (1)	Yes (2)
	Presence of national guidelines for treatment	No	No	No	No	No	No
	Professional societies to support specialist	No	No	Limited	No	No	No
Funding	Government funding for rare disease treatments	Limited	Limited	Limited	No	No	Limited
	Government funding for rare disease research	No	Yes	No	No	No	No
	Non-government access to rare disease treatment	Some charitable and industry funds	Some charitable and industry funds	Some charitable and industry funds, employer benefits	No	No	Some charitable and industry funds
New born screening	National neonatal screening programs	Yes	Yes	Yes	No	Yes	Yes
	Percentage of new born screened	28 %	100 %	>95 %	<1 %	31 %	>97 %
	Source of payment for the new born screening	OOP/insurance	OOP (40 %)	Gov./ OOP	OOP	Government	

Abbreviations: OOP Out of Pocket; Gov Government; PSOD Philippine Society for Orphan Disorders; RDSS Rare Disorders Society (Singapore); MLDS Malaysia Lysosomal Diseases Association; MMA Malaysian Medical Association; MRDS Malaysian Rare Disorders Society; NPH RD National Pediatric Hospital (rare disease); LSD Lysosomal storage diseases





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# "I have a dream" .....

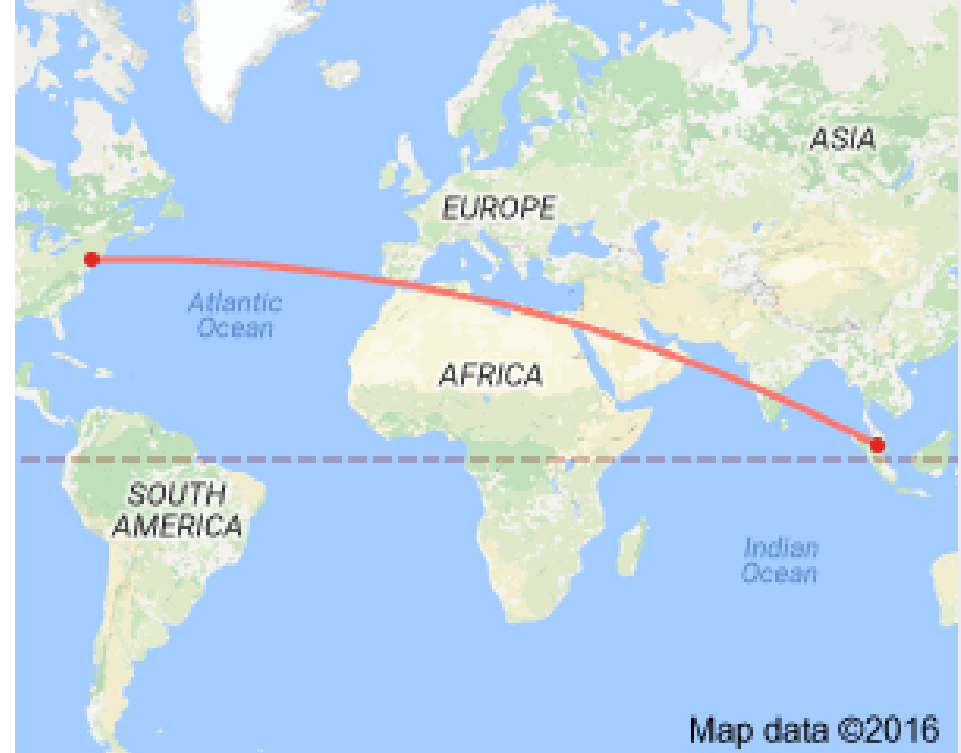
- \* Firstly: To formulate an International Policy to protect the right for rare disease patients across the globe.
- \* 2nd: To mark Rare Disease as one of the key issue in UN meeting.
- \* 3rd: UN Economic & Social Council to file up any discrimination cases in certain countries when the code of ethic has been violated.
- \* 4<sup>th</sup>: UN to help collaborate medical assessment in countries with unmet medical needs. Help governing and regulate orphan drug pricing to avoid overpricing that could deprive of the chance for treatment.





# 15,106 km

Distance from Kuala Lumpur to  
United Nations Headquarters



To fight for the children is a long journey  
Until we get to know about ConNGO  
You are the light at the end of the tunnel for us  
Today is a beautiful day because

Love travel 15,106KM away and unite us together  
Let's not leave anyone behind...



# Thank You So Much!

