



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

17 - 19 November 2016
Hotel Pullman Bangsar,

ORGANISED BY



LSD Registry: A Philippine Experience

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Rare Disease Asia Conference
KL, Malaysia 2016



History

- Started around 2003
- From “Quick Study Capture” to “RegistryNxT”
- Joined the following LSD registries
 - Gaucher Registry in 2003
 - Pompe Registry in 2004
 - Fabry Registry in 2014
 - MPS1 Registry in 2014





Number of Patients

- Gaucher registry – 9
- Pompe registry – 2
- Fabry registry -1
- MPS 1 registry -1

Team of Doctors

- 2 clinical geneticists/metabolic specialists and 1 genetics nurse handle our registry
- We are also the ones directly involved in the diagnosis and management of patients with LSDs in our country



Site Dashboard

LIBRARY | RESOURCES | 0 NOTIFICATIONS

151272 - (Chiong) Institute of Human Genetics

Filter by ID or Initials

ID	Init	DOB
GR-005138	F-L	03 Aug 1999
Last Data Entered: 30 Mar 2016		
Enter Data		
Invite		
GR-005139	E-S	03 Aug 1999
Last Data Entered: 14 Sep 2016		
Enter Data		
Invite		
GR-009061	J-V	10 May 2006
Last Data Entered: 30 Mar 2016		
Enter Data		
Invite		
GR-011048	G-K	27 Jul 2007
Last Data Entered: 07 Mar 2016		
Enter Data		
Invite		
GR-011059	P-M	01 Jul 2005
Last Data Entered: 08 Sep 2016		
Enter Data		
Invite		
GR-011491	C-Z	30 Mar 2009
Last Data Entered: 07 Mar 2016		
Enter Data		

Data Entry Activity (Past 90 Days)

ID	Initials	Date	Details	ID	Initials	Date	Details
GR-205304	PGM	28-Oct-2016	View	GR-011059	P-M	08-Sep-2016	View
GR-005139	E-S	14-Sep-2016	View				

Featured Reports

Site Data Download

This site data download provides you the ability to download clinical summary data for a select patient population. You are able to select which patients you would like to include and the sections of the clinical summary to include. The data download generates an excel spreadsheet containing the aggregate data for you to use in your own analysis.

Patient Listing

The Patient Listing provides internal team members with the ability to view all patients enrolled in the Registry. It includes information such as patient ID number, initials, DOB, gender, site, physician, authorization date, whether a patient is living or deceased, and treatment status and type.

Query Listing Report

The Query Listing Report is to inform data managers of forms of Open, Answered and Non-Conformant queries.

GR-005138 Patient initials (First.Middle.Last):: F-L Date of birth (dd MMM yyyy):: 03 AUG 1999 Sex:: Female
Patient Authorization (1) Enrollment Date:: 13 OCT 2004

- Demography (1)
- Patient Diagnosis (1)
- Clinical Assessment (1)
- Primary Gaucher Therapy (ERT and oral therapies used to treat Gaucher)
- Secondary Gaucher Therapy (Bisphosphonates and vasodilators) (1)
- Laboratory Tests (1)
- Vital Signs (1)
- Tanner Stage (1)
- Pulmonary Artery Pressure (PAP) Measurement (1)
- Organ Volumes (Liver and Spleen) (1)
- Skeletal Radiology Examination (1)
- DXA (Bone Densitometry Exam) (1)
- Bone Pain/Bone Crises Follow-Up Assessments
- Neurological Outcomes
- Medically Significant Surgical Procedures (1)
- SF-36 Health Survey (1)
- Death or Registry Discontinuation (1)
- Clinical Study Information (1)
- Enrollment Confirmation

Patient Enrollment

Grid View

Visit	Date	Task Summary: Subject	Pages
		NonConformant Data	0
		Open Queries	5

Add Event [Dropdown] [Add]

- Reports
- Subject PDF Report - Subject PDF Report

Icon Key

CRF Version 1431 - Page Generated: 10 Nov 2016 17:40:51 China Standard Time



What has the registry done for us?

- Easily accessible
- Easier to see clinical summaries of patients and see their progress
 - Monitoring guidelines
 - Therapeutic goals
- Helped us generate local publications
- Use of resources in the registry
- Contribute/compare data to/with other sites



Challenges encountered

- Minor technical difficulties
 - Definitions of data sets
 - Required tests in the monitoring guidelines not locally available/ too costly for patients
- Implications of external factors to accuracy of data:
 - Enzymes for ERT not continuously available
 - Low compliance of patients (socioeconomic status, geographical distances of residence to ERT sites)
 - Limitations - laboratory investigations
- Regulatory compliance
 - Ethics approval
 - Re-consenting
 - More stringent checking of documents by CRAs
- Interface of registry is not-so user-friendly to new end-users