



# LSD Registry: A Philippine Experience

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# 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





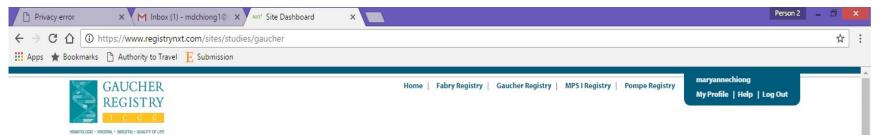


### **Number of Patients**

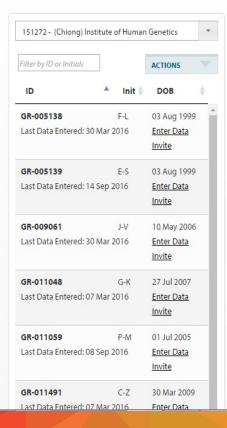
- Gaucher registry 9
- Pompe registry 2
- Fabry registry -1
- MPS 1 registry -1

### 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

NOTIFICATIONS

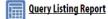
#### Featured Reports

#### Site Data Download

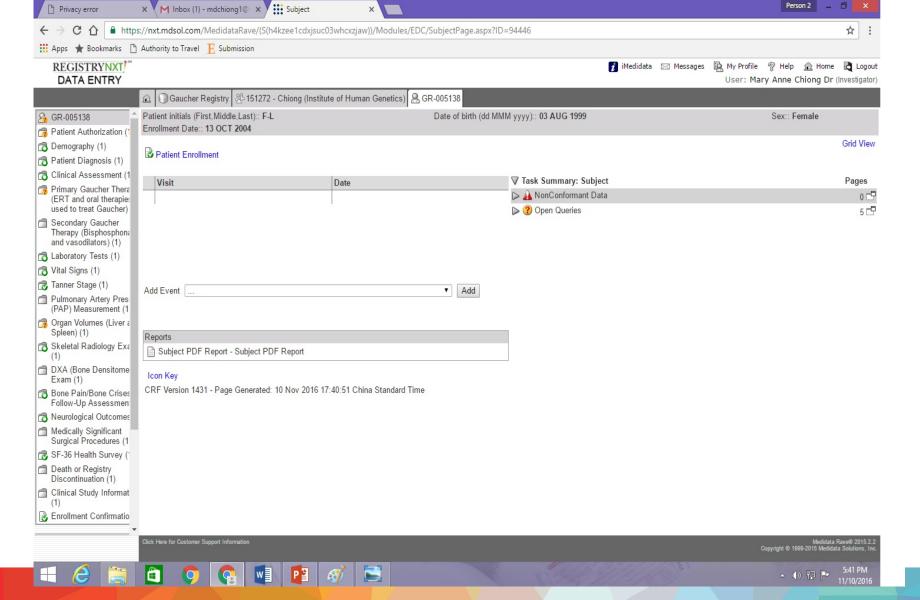
This site data download provides you the ability to download clinical summary data for a select patient population. You are able to select which patients you would like to include and the sections of the clinical summary to include. The data download generates an excel spreadsheet containing the aggregate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



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







## 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

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# 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