Freedom of Information Act 2000 - Request Reference FoI/23/407
Pompe disease diagnosis and treatment

Information Requested:

Q1. Within your trust, how many patients currently have a diagnosis for:

- Fabry Disease (ICD10 code E75.21): 60
- Gaucher Disease (ICD10 code E75.22): 13
- Pompe Disease (ICD10 Code E74.02): 14
- Pompe Disease (ICD10 Code E74.02) Infantile-onset (Patients Diagnosed before age 1): 0
- MPS II (Hunter Syndrome) (ICD10 code E76.1): 0

Q2. Of the patients above, how many patients have been newly diagnosed within the past 3 months for:

- Fabry Disease (ICD10 code E75.21): 0
- Gaucher Disease (ICD10 code E75.22): 0
- Pompe Disease (ICD10 Code E74.02): 0
- Pompe Disease (ICD10 Code E74.02) Infantile-onset (Patients Diagnosed before age 1): 0
- MPS II (Hunter Syndrome) (ICD10 code E76.1): 0

Q3. How many patients have been treated in the last 3 months with the following products:

- Replagal (agalsidase alfa): 11
- Fabrazyme (algalsidase beta): 9
- Galafold (migalastat): 16
- Elfabrio (pegunigalsidase alfa): 0
- VPRIV (velaglucerase alfa): 2
- Cerezyme (imiglucerase): 5
- Cerdelga (eliglustat): 5
- Zavesca (miglustat): 0
- Myozyme (alglucosidase alfa): 2
- Nexviazyme (avalglucosidase alfa): 5
- AT-GAA (cipaglucosidase alfa/miglustat): 5 (EAMS patients)

Q4. Do you participate in any clinical trials for Pompe Disease? If so, can you please provide the name of each trial along with the number of patients taking part?

Rare disease registry- 12 patients recruited alongside an additional pregnancy sub study.