

21 September 2022

## Drug Treatments for Rare Diseases

I have a freedom of information request I would like to raise with the Trust, I would be grateful if you could please answer the following questions:

**1. Within your Trust, how many patients currently have a diagnosis for:**

- Fabry Disease ICD10 code (E75.21) 49
- Gaucher Disease (ICD10 code E75.22) <5
- Pompe Disease (ICD10 Code E74.02) <5
- Pompe Disease (ICD1 Code E74.02) Infantile-onset (Patients Diagnosed before age 1) 0
- MPS II (Hunter Syndrome) (ICD10 code E76.1) <5

**Use of < 5 (less than five):** We are unable to provide an exact figure - exempt from release under Section 40(2) of the Freedom of Information Act - as this could make patients personally identifiable. Disclosure would constitute a breach of the principles of the General Data Protection Regulation 2018.

**2. 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 (ICD1 Code E74.02) Infantile-onset (Patients Diagnosed before age 1) 0
- MPS II (Hunter Syndrome) (ICD10 code E76.1) 0

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

Pharmacy:

- Replagal (agalsidase alpha) 5
- Fabrazyme (algalsidase beta) 13
- Galafold (Migalastat) 12
- VPRIV (velaglucerase alfa) 0
- Cerezyme (imiglucerase) <5
- Cerdelga (eliglustat) 0
- Zavesca (miglustat) <5
- Myozyme (Alglucosidase alfa) <5
- Nexviazyme (Avalglucosidase alfa) 0
- AT-GAA 0

Cardiac Genetics:

- Replagal (agalsidase alpha) 6
- Fabrazyme (algalsidase beta) 12
- Galafold (migalastat) 12

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- 4. 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?**

Belfast Trust is not participating in any clinical trials for Pompe Disease.

- 5. Do you participate in any clinical trials for MPS II (Hunter Syndrome)? If so, can you please provide the name of each trial along with the number of patients taking part?**

Belfast Trust is not participating in any clinical trials for MPS II (Hunter Syndrome).