Belfast Health and Social Care Trust
caring supporting improving together

FOI/29110

26 April 2023

Rare Diseases

I would be grateful if you could please answer the following questions:

1. **Within your trust, how many patients currently have a diagnosis for the following:**

   Paediatric Service

   - Fabry Disease (ICD10 code E75.21) 5
   - 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

   Adult Services

   - Fabry Disease ICD10 code (E75.21) 50
   - 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

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

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.

26 April 2023

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

	Paeds	Adults
• Replagal (agalsidase alfa)		5
• Fabrazyme (algalsidase beta)		14
• Galafold (migalastat)		10
• VPRIV (velaglucerase alfa)		0
• Cerezyme (imiglucerase)		0
• Cerdelga (eliglustat)		0
• Zavesca (miglustat)	<5	0
• Myozyme (alglucosidase alfa)		0
• Nexviazyme (avalglucosidase alfa)		0
• AT-GAA (cipaglucosidase alfa/miglustat)		0

4. How many patients have been treated with Zavesca (miglustat) for the following diseases?

- Niemann Pick Disease (ICD10 code E75.24) <5
- Gaucher Disease (ICD10 code E75.22) 0

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

No

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

No.