Belfast Health and Social Care Trust
caring supporting improving together

FOI/28523

13 January 2023

Rare Diseases

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

Paediatric Service:

- **Fabry Disease ICD10 code (E75.21)** 6
- **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)** 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, particularly as the diseases in question are rare. 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:**

Paediatric Service:

- **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

Adult Services:

- **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

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

Paediatric Service:

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

Adult Services:

- **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

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Cardiac Genetics:

- **Replagal (agalsidase alpha)** 0
- **Fabrazyme (algalsidase beta)** 0
- **Galafold (migalastat)** 0

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

Paediatric Services:

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

Adult Services:

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

5. **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.

6. **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).