

November 2021

## Treatment of Rare Diseases

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

- Fabry Disease ICD10 code (E75.21) - **57**
- Gaucher Disease (ICD10 code E75.22) - **2**
- MPS II (Hunter Syndrome) (ICD10 code E76.1) - **2**

**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**
- MPS II (Hunter Syndrome) (ICD10 code E76.1) - **0**

**Q3. How many patients (including any patients whose treatment is on hold due to COVID-Shielding) are being treated at this point in time with:**

- Replagal (agalsidase alpha) - **2**
- Fabrazyme (algalsidase beta) - **15**
- Galafold (migalastat) - **11**
- VPRIV (velaglucerase alfa) - **1**
- Cerezyme (imiglucerase) - **2**
- Cerdelga (eliglustat) - **0**
- Zavesca (miglustat) - **1**
- Any other treatment for Fabry disease [please state which treatments] - **0**
- Any other treatment for Gaucher disease [please state which treatments] - **0**

**Q4. Can you please provide the split of patients being treated between homecare patients and hospital treated patients for the following two diseases?**

Fabry Disease (ICD10 code (E75.21))

- Hospital treated Patients - **15**
- Homecare Patients - **11**

Gaucher Disease (ICD10 code E75.22)

- Hospital treated Patients - **1**
- Homecare Patients - **1**

**Q5. Do you have any Fabry Disease or Gaucher Disease patients on clinical trials? If so can you please advise how many patients and the name of the trial for each of the following two diseases?**

- Fabry Disease ICD10 code (E75.21) - **No**
- Gaucher Disease (ICD10 code E75.22) - **No**