

26 June 2025

Rare Diseases

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

- Fabry(-Anderson) disease (ICD10 code E75.21)
9
- Gaucher disease (ICD10 code E75.22)
0
- Pompe disease (ICD10 Code E74.02)
<5 (via genetics only)
- Pompe disease (ICD10 Code E74.02) infantile onset (patients diagnosed before age 1)
0
- Hunter syndrome (MPS II) (ICD10 code E76.1)
<5

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

- Fabry(-Anderson) disease (ICD10 code E75.21)
0
- Gaucher disease (ICD10 code E75.22)
0
- Pompe disease (ICD10 Code E74.02)
<5 (as above)
- Pompe disease (ICD10 Code E74.02) infantile onset (patients diagnosed before age 1)
0
- Hunter syndrome (MPS II) (ICD10 code E76.1)
0

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

- **Replagal (agalsidase alfa)**
0
- **Fabrazyme (agalsidase beta)**
0
- **Galafold (migalastat)**
0
- **Elfabrio (pegunigalsidase alfa)**
0
- **VPRIV (velaglucerase alfa)**
0
- **Cerezyme (imiglucerase)**
0
- **Cerdelga (eliglustat)**
0
- **Zavesca (miglustat)**
<5 (for none of the above indications)
- **Myozyme (alglucosidase alfa)**
0
- **Nexviadyme (avalglucosidase alfa)**
0
- **AT-GAA (cipaglucosidase alfa/miglustat)**
0

Q4. Do you participate in any clinical trials for Fabry(-Anderson) disease? If so, can you please provide the name of each trial along with the number of patients taking part?

No

Q5. 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

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Where numbers are low we have used <5 rather than state the exact figure. This is to ensure our patients remain non identifiable.