Belfast Health and Social Care Trust
caring supporting improving together

FOI/34658

17 September 2025

Rare Diseases

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

Paediatric Service

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

Adult Services

• **Fabry(-Anderson) disease (ICD10 code E75.21)**	58
• **Gaucher disease (ICD10 code E75.22)**	<5
• **Pompe disease (ICD10 Code E74.02)**	<5
• **Pompe disease (ICD10 Code E74.02) infantile onset (patients diagnosed before age 1)**	0
• **Hunter syndrome (MPS II) (ICD10 code E76.1)**	0

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

Paediatric Service

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

Adult Services

• **Fabry(-Anderson) disease (ICD10 code E75.21)**	0
• **Gaucher disease (ICD10 code E75.22)**	0
• **Pompe disease (ICD10 Code E74.02)**	<5
• **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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3. How many patients have been treated in the last 3 months with the following products:

Paediatric Service

• **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
• **Myozyme (alglucosidase alfa)**	0
• **Nexviadyme (avalglucosidase alfa)**	0
• **AT-GAA (cipaglucosidase alfa/miglustat)**	0

Adult Services

• **Replagal (agalsidase alfa)**	6
• **Fabrazyme (agalsidase beta)**	10
• **Galafold (migalastat)**	12
• **Elfabrio (pegunigalsidase alfa)**	<5
• **VPRIV (velaglucerase alfa)**	0
• **Cerezyme (imiglucerase)**	<5
• **Cerdelga (eliglustat)**	0
• **Zavesca (miglustat)**	0
• **Myozyme (alglucosidase alfa)**	0
• **Nexviadyme (avalglucosidase alfa)**	0
• **AT-GAA (cipaglucosidase alfa/miglustat)**	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 the small numbers involved could make patients personally identifiable. Disclosure would constitute a breach of the principles of the General Data Protection Regulation 2018.

4. 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?

Paediatric Service

No

Adult Services

Adult Service registry participation only with Follow Me registry (#23) and Genzyme Rare Disease Registry ~49

5. 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?

Paediatric Service

No

Adult Services

No