

Freedom of Information Act 2000 - Request Reference Fol/21/019

Inherited Metabolic Disorders

Request Details

I have a freedom of information request related to the incidence and treatment of some inherited metabolic disorders. I would be grateful if you could help and provide some answers to the following four questions?

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

- **Fabry Disease**
- **Gaucher Disease**
- **MPS II (Hunter Syndrome)**

The below table represents Paediatric Patients, none of which have been diagnosed within the last 3 months.

Fabry Disease	7
Gaucher Disease	0
Hunter Disease	0

The below table represents adult patients, none of which have been diagnosed within the last 3 months.

Fabry Disease	47
Gaucher Disease	13
Hunter Disease	0

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

- **Fabry Disease**
- **Gaucher Disease**
- **MPS II (Hunter Syndrome)**

Please refer to the above.

3. 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)**
- **Fabrazyme (algalsidase beta)**
- **Galafold (migalastat)**
- **VPRIV (velaglucerase alfa)**
- **Cerezyme (imiglucerase)**
- **Cerdelga (eliglustat)**
- **Zavesca (miglustat)**
- **Any other treatment for Fabry Disease (Please state which treatments)**
- **Any other treatment for Gaucher Disease (Please state which treatments)**

No Paediatric patients are currently on any treatment for these diseases

The below table represent Adult Patients

Replagal (agalsidase alpha)	10
Fabrazyme (algalsidase beta)	6
Galafold (migalastat)	11
VPRIV (velaglucerase alfa)	3
Cerezyme (imiglucerase)	6
Cerdelga (eliglustat)	3
Zavesca (miglustat)	0
Any other treatment for Fabry Disease (Please state which treatments)	N/A
Any other treatment for Gaucher Disease (Please state which treatments)	N/A

4. Of the patients being treated for Gaucher Disease, can you please provide the numbers of patients being treated in hospital versus through homecare?

No patients are currently being treated in hospital, all are homecare.