

Freedom of Information request 292-23

Request

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

- Fabry Disease (ICD10 code E75.21)
- Gaucher Disease (ICD10 code E75.22)
- Pompe Disease (ICD10 Code E74.02)
- Pompe Disease (ICD1 Code E74.02) Infantile-onset (Patients Diagnosed before age 1)
- MPS II (Hunter Syndrome) (ICD10 code E76.1)

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

- Fabry Disease (ICD10 code E75.21)
- Gaucher Disease (ICD10 code E75.22)
- Pompe Disease (ICD10 Code E74.02)
- Pompe Disease (ICD1 Code E74.02) Infantile-onset (Patients Diagnosed before age 1)
- MPS II (Hunter Syndrome) (ICD10 code E76.1)

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

- Replagal (agalsidase alfa)
- Fabrazyme (algalsidase beta)
- Galafold (migalastat)
- VPRIV (velaglucerase alfa)
- Cerezyme (imiglucerase)
- Cerdelga (eliglustat)
- Zavesca (miglustat)
- Myozyme (alglucosidase alfa)
- Nexviazyme (avalglucosidase alfa)
- AT-GAA (cipaglucosidase alfa/miglustat)

Q4. How many patients have been treated with Zavesca (miglustat) for the following diseases:

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

Q5. Do you participate in any clinical trials for Fabry Disease? If so, can you please provide the name of each trial along with the number of patients taking part?

Q6. 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?

Clarification:

Q1 and Q2 Unfortunately, NHS Borders cannot provide diagnosis data. However, we can provide data on the number of patients treated for the named diseases, if held.

Q4 We are not able to provide data regarding drugs supplied for a specific diagnosis, however, we can provide data on the number of patients treated with the medications, if held.

Just wanted to confirm that I would be happy to receive the suggested data for Q1, Q2 and Q4.

Response

Q1. Within NHS Borders, the number of patients currently being treated is:

- Fabry Disease / Gaucher Disease <5
- Pompe Disease 0
- Pompe Disease - Infantile-onset 0
- MPS II (Hunter Syndrome) 0

Q2. Of the patients above, the number of patients who have been newly diagnosed is:

- Fabry Disease 0
- Gaucher Disease 0
- Pompe Disease 0
- Pompe Disease - Infantile-onset 0
- MPS II (Hunter Syndrome) 0

Q3. The number of NHS Borders patients who have been treated is:

- Replagal (agalsidase alfa) 0
- Fabrazyme (algalsidase beta) 0
- Galafold (migalastat) 0
- VPRIV (velaglucerase alfa) 0
- Cerezyme (imiglucerase) 0
- Cerdelga (eliglustat) 0
- Zavesca (miglustat) 0
- Myozyme (alglucosidase alfa) 0
- Nexviazyme (avalglucosidase alfa) 0
- AT-GAA (cipaglucosidase alfa/miglustat) 0

Q4. No NHS Borders patients have been treated with Zavesca (miglustat).

Q5. NHS Borders is not currently participating in any clinical trials in Fabry disease and has not been approached to undertake trials for this condition.

Q6. NHS Borders is not currently participating in any clinical trials in Gaucher Disease and has not been approached to undertake trials for this condition.

As the number of events in some areas are very small and in accordance with the Code of Practice for Official Statistics any number that is less than five, actual numbers and potentially identifiable information is withheld to help maintain patient confidentiality due to potential risk of disclosure. Further information is available in the [ISD Statistical Disclosure Control Protocol](#).

If you are not satisfied with the way your request has been handled or the decision given, you may ask NHS Borders to review its actions and the decision. If you would like to request a review please apply in writing to, Freedom of Information Review, NHS Borders, Room 2EC3, Education Centre, Borders General Hospital, Melrose, TD6 9BS or foi.enquiries@borders.scot.nhs.uk.

The request for a review should include your name and address for correspondence, the request for information to which the request relates and the issue which you wish to be reviewed. Please state the

reference number **292-23** on this request. Your request should be made within 40 working days from receipt of this letter.

If following this review, you remain dissatisfied with the outcome, you may appeal to the Scottish Information Commissioner and request an investigation of your complaint. Your request to the Scottish Information Commissioner should be in writing (or other permanent form), stating your name and an address for correspondence. You should provide the details of the request and your reasons for dissatisfaction with both the original response by NHS Borders and your reasons for dissatisfaction with the outcome of the internal review. Your application for an investigation by the Scottish Information Commissioner must be made within six months of your receipt of the response with which you are dissatisfied. The address for the Office of the Scottish Information Commissioner is, Office of the Scottish Information Commissioner, Kinburn Castle, Doubledykes Road, St Andrews, Fife.