

Dear

### FREEDOM OF INFORMATION – PRESCRIBING

I write in response to your request for information in relation to prescribing.

Question:

1. Within your trust, how many patients currently have a diagnosis for:
  - a. Fabry(-Anderson) disease (ICD10 code E75.21)
  - b. Gaucher disease (ICD10 code E75.22)
  - c. Pompe disease (ICD10 Code E74.02)
  - d. Pompe disease (ICD10 Code E74.02) infantile onset (patients diagnosed before age 1)
  - e. Hunter syndrome (MPS II) (ICD10 code E76.1)

Answer:

a.	7
b.	5<
c.	5<
d.	5<
e.	5<

Question:

2. Of the patients above, how many patients have been newly diagnosed within the past 3 months for:
  - a. Fabry(-Anderson) disease (ICD10 code E75.21)
  - b. Gaucher disease (ICD10 code E75.22)
  - c. Pompe disease (ICD10 Code E74.02)
  - d. Pompe disease (ICD10 Code E74.02) infantile onset (patients diagnosed before age 1)
  - e. Hunter syndrome (MPS II) (ICD10 code E76.1)

Answer:

a.	5<
b.	5<
c.	5<

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Chair Professor John Connaghan CBE  
Chief Executive Professor Caroline Hiscox  
*Lothian NHS Board is the common name of Lothian Health Board*

d.	5<
e.	5<

Question:

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

- a. Replagal (agalsidase alfa)
- b. Fabrazyme (agalsidase beta)
- c. Galafold (migalastat)
- d. Elfabrio (pegunigalsidase alfa)
- e. VPRIV (velaglucerase alfa)
- f. Cerezyme (imiglucerase)
- g. Cerdelga (eliglustat)
- h. Zavesca (miglustat)
- i. Myozyme (alglucosidase alfa)
- j. Nexviadyme (avalglucosidase alfa)
- k. Pombiliti + Opfolda (cipaglucosidase alfa + miglustat)

Answer:

a.	5<
b.	5<
c.	5<
d.	5<
e.	5<
f.	5<
g.	5<
h.	5<
i.	5<
j.	5<
k.	5<

To protect the identity of the individuals involved any figure of 5 or less has not been shown in the table above. Since we do not have their consent to release this data from their records, the information is exempt under section 38(1)(b) of the Freedom of Information (Scotland) Act i.e. to provide it would breach the Data Protection Act (2018).

Question:

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?

Answer:

We have no trials recruiting participants with Fabry disease.

Question:

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?

Answer:

We have one (1) trial entitled "RAPSODI, entitled "remote assessment of Parkinsonism supporting ongoing development of interventions in Gaucher's disease". This study is being run by University College London and is a basic science study. The study is split into 2 cohorts: RAPSODI (recruiting participants with Gaucher's disease who may go on to develop Parkinson's disease (dedicated trial website [www.rapsodistudy.com](http://www.rapsodistudy.com)) and PD FRONTLINE (recruiting participants with Parkinson's disease that may carry the gene for Gaucher's disease (dedicated trial website [www.pdf frontline.com](http://www.pdf frontline.com)). In Lothian we are currently only recruiting to the PD FRONTLINE cohort and we have recruited 61 participants.

I hope the information provided helps with your request.

If you are unhappy with our response to your request, you do have the right to request us to review it. Your request should be made within 40 working days of receipt of this letter, and we will reply within 20 working days of receipt. If our decision is unchanged following a review and you remain dissatisfied with this, you then have the right to make a formal complaint to the Scottish Information Commissioner within 6 months of receipt of our review response. You can do this by using the Scottish Information Commissioner's Office online appeals service at [www.itspublicknowledge.info/Appeal](http://www.itspublicknowledge.info/Appeal). If you remain dissatisfied with the Commissioner's response you then have the option to appeal to the Court of Session on a point of law.

If you require a review of our decision to be carried out, please write to the FOI Reviewer at the email address at the head of this letter. The review will be undertaken by a Reviewer who was not involved in the original decision-making process.

FOI responses (subject to redaction of personal information) may appear on NHS Lothian's Freedom of Information website at: <https://org.nhsllothian.scot/FOI/Pages/default.aspx>

Yours sincerely

**ALISON MACDONALD**  
**Executive Director, Nursing**  
Cc: Chief Executive