

Date 29/05/2025
Your Ref
Our Ref 10036

Enquiries to Richard Mutch
Extension 35687
Direct Line 0131 465 5687
loth.freedomofinformation@nhs.scot
richard.mutch@nhs.scot

Dear

FREEDOM OF INFORMATION - DUCHENNE MUSCULAR DYSTROPHY

I write in response to your request for information in relation to Duchenne Muscular Dystrophy.

Question:

- Can you please provide copies of all correspondence between Scottish Government ministers and/ or officials and NHS Lothian officials / clinicians that relate to the Duchenne Muscular Dystrophy drug treatment GIVINOSTAT (also known as Duvyzat) and the associated Early Access Program. Time frame – November 2024 to present.

Answer:

Enclosed is the available information to answer your request. Please note that NHS Lothian has a policy of not releasing the names and details of staff below a senior level and non-NHS Lothian staff. This information is considered exempt under Section 38(1)(b) of the Freedom of Information (Scotland) Act 2002 – personal information.

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. 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.

Headquarters
Mainpoint
102 West Port
Edinburgh EH3 9DN

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

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

[REDACTED]
[REDACTED]

Hiscox, Caroline

Dear Lorraine,

Further to your conversation with Caroline yesterday and our call earlier this morning, I thought it would be helpful to follow up in writing regarding this.

The timescales that have been provided are in relation to starting the medicine

That will be preceded in the May, June and July by clinical meetings with every individual family to explain in detail what is involved including the commitment needed and the side effects to consider

The consultant has reviewed all possible patients and applied the broadest definition possible to the eligibility criteria which has increased the numbers slightly but makes the medicine available to the largest number of boys, prioritising those closest to the threshold to "miss out". There is no agreed tool for prioritisation.

The consultant will be able to run the appointments for medicine and monitoring in blocks, so minimising the impact on other children with neurological conditions. This will be better than adding in ad hoc patients

The consultant in touch regularly with clinicians in other centres and doesn't believe we are significantly out of step (in a way that would disadvantage our population)

With best wishes,

[REDACTED]
[REDACTED]
Chief Executive Associate

[REDACTED].scot.nhs.uk

From: [REDACTED]
Sent: Wednesday, April 30, 2025 6:26 PM
To: [REDACTED]
Cc: Hiscox, Caroline; John Burns
Subject: Re: Givinostat - Access - Response for Cabinet Secretary

Hi [REDACTED]

Given Caroline's diary commitments tomorrow, I expect 3-5pm would be the best window. Would that be OK?

[REDACTED]

Sent from Outlook for Android
From: Lorraine Cowie
Sent: Wednesday, April 30, 2025 5:01:19 PM
To: [REDACTED]
Cc: Hiscox, Caroline ; John Burns
Subject: Re: Givinonstat - Access - Response for Cabinet Secretary
Thanks again [REDACTED].

I have spoken to John this afternoon and would like to discuss further with Caroline around timelines.

Can you advise if Caroline has 15 mins tomorrow please?

Alternatively Caroline my mobile is below and can take a call anytime.

Kind regards
Lorraine

[REDACTED]

NHS Scotland Professional Lead for Sustainability/Health Planning

Directorate of Chief Operating Officer, Scottish Government

Support to Professional Lead: [REDACTED], Project Manager [REDACTED]@nhs.scot)

[REDACTED]

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From: [REDACTED]
Sent: 30 April 2025 15:28
To: [REDACTED]
Cc: Hiscox, Caroline ; John Burns
Subject: Re: Givinonstat - Access - Response for Cabinet Secretary
Dear [REDACTED],

Please accept Caroline's apologies for the late return of this response - Caroline had thought this response had already been submitted.

Please find attached Word document in response to the questions set out in the table in your original email. There would be a need to increase staffing in order to deliver this service, and we are working through the consequences of that internally.

To note that the return includes an end date of January, which would be the end of the increased monitoring period for this group only.

Please let me know if you require anything further.

With best wishes,

[REDACTED]

[REDACTED]

Chief Executive Associate

[REDACTED]

.scot.nhs.uk

From: [REDACTED]

Sent: Tuesday, April 29, 2025 9:45 AM

To: [REDACTED] (NHS Grampian); GRAM GRAMPianchiefexecutive; Hiscox, Caroline; [REDACTED]
[REDACTED] (NHS Grampian)

Cc: [REDACTED]

Subject: Re: Givinsonstat - Access - Response for Cabinet Secretary

Good morning,

I appreciate there was a quick turnaround requested for this response (yesterday at 3pm) but can you advise when this will be available please?

Have removed NHST and NHS GG&C from this circulation as I have received their responses.

Thank you

[REDACTED]

NHS Scotland Professional Lead for Sustainability/Health Planning

Directorate of Chief Operating Officer, Scottish Government

Support to Professional Lead: [REDACTED], Project Manager [REDACTED] nhs.scot)

[REDACTED]

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From: [REDACTED]

Sent: 24 April 2025 15:51

To: [REDACTED] (NHS Grampian) ; GRAM GRAMpianchiefexecutive ; [REDACTED] ; Hiscox, Caroline ; [REDACTED] (NHS Grampian) ; [REDACTED]

Cc: [REDACTED]

Subject: Givinostat - Access - Response for Cabinet Secretary

As discussed yesterday, Givinostat (Duvyzat) is a new licensed medicine used to treat Duchenne muscular dystrophy (DMD) resulting from a genetic defect that affects normal muscle function in boys aged 6 and over. This is a drug which has been requested by families due to the benefits presented by them in a meeting with the Cabinet Secretary and therefore it is imperative that we can take a population planning approach and ensure equity of access and outcomes across Scotland.

As you are aware this medicine is currently being provided through a free of charge early access programme agreed by NHS NP. The company has indicated that they will be submitting to the SMC for appraisal at the end June. The company has confirmed that the medicine will remain free of charge for a further 90 days if the SMC accepts the medicine for routine use and in the event it is not recommended by the SMC they will continue to make the medicine available free of charge as long as a child continues to benefit. Until the medicine has been approved by the SMC then it cannot be considered for the Risk Share agreement. We will continue to work through this with [REDACTED].

We acknowledge there are some key areas that we collaboratively and/or from a Scottish Government need to address in order to gain equity of access and outcomes. We will work on these in the coming weeks.

As agreed we are keen to understand your implementation plans and attach a table below to give high level detail of this and would ask you complete this by 3pm on the 28th of April 2025 in order for Cabinet Secretary to provide a response to the families.

Patient Access

Number/Timetable

Comments/Response

What is your timetable for access for these patients?

Example –

Patient A – May 25

Patient B – May 25

Patient G – Aug 25

We appreciate there will be clinical prioritisation and therefore if this is not known due to this then please indicate this.

If you are cohorting patients please indicate these in terms of first patient date and last patient access date.

Have any of your patients already been involved in the clinical trial and therefore excluded?

From the EAP criteria are there any patients from this number who will be excluded?

Number

Have these families been contacted?

If you are a regional centre then please indicate number of patients by region?

Number

Supplier Aspects

Number

Comments

Have you had discussions with the supplier?

Yes/No

At what level have these discussions got to?

If so how many patients would be covered by FOC agreement with supplier given the timelines above

Number of patients

Please send the above directly to myself and I will collate the response for Cabinet Secretary.

Thank you for your support in this matter and should you wish to discuss anything further then please contact me. I have copied in those who were in attendance as deputies yesterday.

Kind regards

[REDACTED]

[REDACTED]

NHS Scotland Professional Lead for Sustainability/Health Planning

Directorate of Chief Operating Officer, Scottish Government

Support to Professional Lead: [REDACTED] Project Manager [REDACTED] nhs.scot)

[REDACTED]

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[REDACTED]

Gillies, Tracey;

[REDACTED]

[REDACTED];

Medicaldirector Tayside;

Hugh Bishop (NHS Grampian)

[REDACTED]

Hiscox, Caroline;

[REDACTED]

John Burns;

[REDACTED]

[REDACTED]

GRAM GRAMPianchiefexecutive;

[REDACTED]@gov.scot

Dear colleagues,

Many thanks for the information you have shared with Lorriane, setting out your plans for access to givinostat for boys with Duchenne Muscular Dystrophy (DMD) through a company Early Access programme (EAP) after BCE's met with the Cabinet Secretary the other week. [REDACTED] and I have met and discussed.

Timelines

As you are aware, the Cabinet Secretary had stated a preference that all four Boards commence children on treatment at the same time or as close to that as possible. However, having considered the timelines you have all shared, and the rationale for those dates, we do not believe there is any merit in asking you to try and align further, as this is only likely to displace other clinical activities.

It would be helpful, however, if we could have an agreed timeline by which you would all communicate to the families outlining your individual timelines and providing the appropriate level of detail about what will happen next (such as baseline bloods, treatment initiation, prioritisation approach, inductive timescale etc as much as you can).

Prioritisation of cohorts

The Cabinet Secretary and I also heard very clearly from the families when we met them that they were concerned about cohorting or prioritising children. However, given the need to safely introduce this medicine, the operational impacts, and that this is the approach identified by clinicians (who we understand plan to start with the least ambulatory children), we are supportive of this approach; this is based on maintaining the primacy of clinically driven decision making. The Cabinet Secretary has acknowledged that as being critical in all discussions on this subject.

Action

Given that NHS GGC plan to start treatment during the last week in May, we would like to be able to state in correspondence that Health Boards will be communicating with all families with ambulatory children who are covered by the EAP week commencing 19 May.

Can colleagues, except NHS Tayside given the small number of children involved there, please confirm by return if undertaking to commit to this is achievable.

I expect that once we commence this work the next push will be for the non-ambulatory cohort, but that can be for another day.

I hope that this is helpful and provides us all with a consistent narrative on how we are progressing this work in Scotland.

Happy to discuss if useful.

Kindest regards

[REDACTED]

[REDACTED]

Chief Pharmaceutical Officer and Deputy Director

Pharmacy and Medicines Division | Directorate for Chief Medical Officer | Scottish Government
| St Andrews House | Regent Road | Edinburgh | EH1 3DG | Tel: [REDACTED]
[REDACTED]@gov.scot | Twitter: [REDACTED]

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RE: Access to givinostat via an Early Access programme (EAP)

[REDACTED]

[REDACTED]

behalf of Hiscox, Caroline

[REDACTED]

John Burns;

[REDACTED]

Dear Alison

Thank you for your email, please see the attached response.

Kind regards

Caroline

Professor Caroline Hiscox

Chief Executive

NHS Lothian Board

Mainpoint

102 West Port

Edinburgh

EH3 9DN

[REDACTED]

[REDACTED]

[REDACTED]

[REDACTED]

[REDACTED]@nhs.scot

From: [REDACTED]@gov.scot

Sent: 08 May 2025 16:02

To: Gillies, Tracey ; [REDACTED] ; Medicaldirector Tayside ; [REDACTED]
(NHS Grampian)

Cc: [REDACTED] ; Hiscox, Caroline ; [REDACTED] ; John Burns ; [REDACTED]
; GRAM GRAMpianchiefexecutive [REDACTED]@gov.scot

Subject: Access to givinostat via an Early Access programme (EAP)

Importance: High

Dear colleagues,

Many thanks for the information you have shared with Lorriane, setting out your plans for access to givinostat for boys with Duchenne Muscular Dystrophy (DMD) through a company Early Access programme (EAP) after BCE's met with the Cabinet Secretary the other week. [REDACTED] and I have met and discussed.

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Can colleagues, except NHS Tayside given the small number of children involved there, please confirm by return if undertaking to commit to this is achievable.

I expect that once we commence this work the next push will be for the non-ambulatory cohort, but that can be for another day.

I hope that this is helpful and provides us all with a consistent narrative on how we are progressing this work in Scotland.

Happy to discuss if useful.

Kindest regards

[REDACTED]

[REDACTED]

Chief Pharmaceutical Officer and Deputy Director

Pharmacy and Medicines Division | Directorate for Chief Medical Officer | Scottish Government
| St Andrews House | Regent Road | Edinburgh | EH1 3DG | Tel: [REDACTED]
[REDACTED]

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Sent by Email

[Redacted]

Date 9 May 2025

Your Ref

Our Ref

CH/GS

Enquiries to Caroline Hiscox

Extension

Direct Line

Email

EA

Caroline.Hiscox@nhs.scot

[Redacted]

Dear [Redacted]

ACCESS TO GIVINOSTAT VIA AN EARLY ACCESS PROGRAMME (EAP)

Thank you for your email yesterday.

In this email you have helpfully confirmed acceptance of our plan, submitted to John's office last week, to safely introduce givinostat via the Early Access Programme (EAP) for ambulatory patients being cared for in NHS Lothian. As previously communicated the consultant has reviewed all patients and applied the broadest definition possible to the eligibility criteria to ensure that all eligible patients set out in the EAP can access the medicine. There is no agreed prioritisation tool however those closest to the threshold in the EAP have been identified to ensure they are considered first.

You have specifically asked about our timescales for communication to all families with ambulatory children covered by the EAP. I can confirm that there will be clinical meetings with every individual family to discuss in detail what is involved, including the commitment needed, and the side effects to consider. These meetings will start w/c 19 May and will be concluded by w/c 2 June 25.

The Consultant is in regular contact with his peers across the country to ensure there is a sharing of best practice and a consistency of approach to the introduction of this medicine.

I hope this information is helpful.

Yours sincerely



PROFESSOR CAROLINE HISCOX
Chief Executive