NICE National Institute for Health and Care Excellence

10 Spring Gardens
London
SW1A 2BU
United Kingdom

+44 (0)845 003 7780

Sent by email

[redacted]
General Manager
Ferring Pharmaceuticals Ltd

12 May 2014

Dear [redacted]

Final Appraisal Determination: Degarelix for treating advanced hormone dependent prostate cancer

Thank you for lodging Ferring's appeal against the above Final Appraisal Determination.

Introduction

The Institute's appeal procedures provide for an initial scrutiny of points that an appellant wishes to raise, to confirm that they are at least arguably within the permitted grounds of appeal ("valid"). The permitted grounds of appeal are:

- 1(a) NICE has failed to act fairly,[1] or
- 1(b) NICE has exceeded powers;[2]
- (2) the recommendation is unreasonable in the light of the evidence submitted to NICE

This letter sets out my initial view of the points of appeal you have raised: principally whether they fall within any of the grounds of appeal, or whether further clarification is required of any point. Only if I am satisfied that your points contain the necessary information and arguably fall within any one of the grounds will your appeal be referred to the Appeal Panel.

[1] Formerly ground 1
[2] Formerly ground 3

You have the opportunity to comment on this letter in order to elaborate on or clarify any of the points raised before I make my final decision as to whether each appeal point should be referred on to the Appeal Panel.

I can confirm that there will be an oral hearing of the appeal.

Initial View

Ground 1 (a)

Ground 1.1(a) NICE failed to issue a second ACD following a substantial change to the preliminary recommendations that significantly reduces the number of eligible patients that can be treated with degarelix.

A valid ground 1(a) appeal point.

Ground 1.2(a) The decision in the FAD to restrict use of degarelix to patients with spinal metastases who have actual spinal compression (as opposed to those who are "at risk" of spinal compression) lacks transparency and fails to give adequate reasons

A valid ground 1(a) appeal point.

Ground 1.3(a) The FAD recommendation is not sufficiently clear, precise or understandable for the NHS and is therefore not in accordance with the STA Guide or principles of good administration.

You argue that in the context of a recommendation for use in a sub-group, it is vital to emphasise the need to adhere to dosing and administration requirements in the product licence. I believe that a specific concern is that when a patient passes from acute to community care, treatment might be discontinued. This is said to be contrary to the product licence. Your proposed solution is that the guidance should be added to to read "Once initiated, treatment should be continued until the initiating NHS clinician considers it appropriate to stop."

I doubt there can be any argument with the sentiment proposed, either that recommended treatment must be within the terms of the SPC or that treatment should continue until the responsible clinician recommends that it should stop (or, of course, until the patient wishes to stop, if sooner). I would expect those points to be universally understood, so that there is no

need to add them to an FAD. Even if that was not correct I could not agree that it was arguable that not spelling that understanding out made the guidance unclear.

As the issue here is not on the substance of the guidance or the process by which it was produced, but whether to make explicit something which I believe is universally regarded as implicit, it is a question of the wording of the FAD only. I would propose to pass your point to the Guidance Executive to consider before final publication, supposing the FAD is passed for publication which would depend on the outcome of the appeal.

I am not minded to agree this is a valid appeal point.

Ground 1(b)

Ground 1.1 (b) The failure to recommend the use of degarelix in patients at-risk of suffering cardiovascular events is in breach of the NICE Charter.

NICE's powers are set out in its governing legislation, statutory instruments, and to an extent European law and caselaw. NICE's charter is a document adopted by NICE. Acting inconsistently with the charter, if that was what happened here, would not of itself be acting outside NICE's powers.

In any case I am not persuaded that NICE has arguably acted outside its charter, or rather, it will not add anything to the appeal to argue that it has. The Appeal Panel will consider whether the guidance is reasonable in the light of the evidence on CVS risks (see below). If it is not, then the appeal would succeed on that basis. If it is, then the guidance is not a breach of an expectation to produce guidance that seeks to prevent and treat illness. On either result, consideration of the charter will not affect the outcome.

I should also add that it seems to me that the charter is intended to describe what NICE seeks to achieve and how it works at the global level. I am not persuaded it is intended to be applied paragraph by paragraph to each appraisal.

I am not currently persuaded this is a valid appeal point.

Ground 1.2 (b) The failure to recommend the use of degarelix in patients at-risk of suffering cardiovascular events is in breach of fundamental rights.

For the same reason given above in respect of the NICE charter, I am not persuaded this appeal point is valid. If the guidance is reasonable in light of CVS evidence, it is hard to see how it could be a breach of human rights. If it is not reasonable the appeal succeeds for that reason.

Further the Appeal Panel has in the past set out its understanding of the effect of the Human Rights Act on NICE in the appeal decision relating to "**dasatinib, high-dose imatinib and nilotinib for the treatment of imatinib-resistant chronic myeloid leukaemia (part review of NICE technology appraisal guidance 70) and dasatinib and nilotinib for people for whom treatment with imatinib has failed because of intolerance**" In light of the position taken in that letter I would doubt that your argument here could succeed.

I am not currently persuaded this is a valid appeal point.

Ground 1.3(b) The failure to provide a clear recommendation for a specific sub-group by omitting important initiation and maintenance information is in breach of the NICE Charter

For the reasons give under point 1.3 (a) and 1.1(b) I am not currently persuaded this is a valid appeal point.

Ground 2

Ground 2.1 The assumptions upon which the ERG and Appraisal Committee has based their assessment are unreasonable in light of the evidence of cardiovascular risk submitted.

A valid appeal point relating to the "assumption that the rate of cardiovascular events is the same for patients receiving degarelix and LHRH agonists is the same"

2.2 ***Ground 2.2 The failure to recommend degarelix for patients at risk of cardiovascular disease is unreasonable in light of the evidence submitted***

A valid ground 2 appeal point

As I agree some of your appeal points are valid they will be passed to an Appeal Panel for consideration. There will be an oral hearing. I would be grateful to receive your comments

on the points I am presently not minded to treat as valid within 14 days of this letter, no later than **Tuesday 27 May 2014,** whereupon I will take a final decision.

Yours sincerely

Dr Maggie Helliwell
Vice Chair of NICE
National Institute for Health and Care Excellence