25 August 2017

Andy McKeon
Vice Chair
National Institute for Health and Care Excellence
10 Spring Gardens
London SW1A 2BU

Dear Mr McKeon,

Re: Final Appraisal Determination – Inotuzumab ozogamicin for treating relapsed or refractory B-cell acute lymphoblastic leukaemia [ID893]

Leukaemia CARE hereby gives notice to the National Institute for Health and Care Excellence (henceforth referred to as NICE) that it would like to appeal against the Final Appraisal Determination (henceforth referred to as FAD) of Inotuzumab ozogamicin for treating relapsed or refractory B-cell acute lymphoblastic leukaemia [ID893] on the following grounds:

Ground one: In making the assessment that preceded the recommendation, NICE has:

a) failed to act fairly

Ground two: The recommendation is unreasonable in the light of the evidence submitted to NICE.

We submit that the decision not to recommend inotuzumab ozogamicin for treating relapsed or refractory B-cell acute lymphoblastic leukaemia [ID893] was both unfair and unreasonable.

Ground 1a: In making the assessment that preceded the recommendation, NICE has: failed to act fairly

1a.1 Inotuzumab ozogamicin should not have been appraised through the Single Technology Appraisal (STA) process

Leukaemia CARE submits that the Single Technology Appraisal (STA) process was not a fair mechanism for appraising inotuzumab ozogamicin for treating relapsed or refractory B-cell acute lymphoblastic leukaemia [ID893].

As Sir Andrew Dillon, Chief Executive of NICE stated: *"NICE takes into account a greater range of criteria about the benefits and costs of highly specialised technologies than is the case with its appraisals of mainstream drugs and treatments. We do this because applying our standard approach to treatments for very small groups of patients would result in us always recommending against their use. This would be unfair."*

To address this unfairness, NICE set up the Highly Specialised Technologies (HST) programme. The Highly Specialised Technologies Interim Process and Methods of the Highly Specialised Technologies Programme[1] sets out a number of criteria for inclusion in the programme. We submit that these criteria are unfair and discriminatory. In particular, the criteria requiring that *"the condition is chronic and severely disabling"* discriminates unfairly against rare acute conditions, such as acute lymphoblastic leukaemia.

The company estimated that around 117 people in England and Wales each year for whom inotuzumab ozogamicin may be an appropriate option. This is consistent with the population sizes of treatments that have previously been appraised under the HST programme[2]. Access to inotuzumab ozogamicin is a life or death decision for these patients. As such, the population size is

[1] https://www.nice.org.uk/Media/Default/About/what-we-do/NICE-guidance/NICE-highly-specialised-technologies-guidance/HST-interim-methods-process-guide-may-17.pdf
[2] https://www.nice.org.uk/guidance/published?type=hst

sufficiently small that NICE acted unfairly by not considering inotuzumab ozogamicin through the HST process or an alternative mechanism which considers the "greater range of criteria" required to fairly appraise treatments for very small groups of patients.

Ground 2: The recommendation is unreasonable in the light of the evidence submitted to NICE

2.1 Number of courses of treatment

Leukaemia CARE do not have access to the confidential health economic modelling. However, as made public by the company during the consultation on the ACD[3] the recommendations were based on clinicians using six courses of inotuzumab ozogamicin. To the best of our knowledge this was not further discussed by the committee, despite it having an impact on the ICER.

In UK clinical practice, the goal of treatment in this setting is to achieve complete remission (CR) and enable the patient to have a potentially curative stem cell transplant (SCT). The recommended number of courses of inotuzumab ozogamicin prior to transplant is two (but patients may occasionally receive three). If patients are ineligible for transplant they can have up to six courses of treatment. However, only a small minority of patients would have more than three courses. There would also be a proportion of patients (approximately 20%) who do not respond to the treatment, in whom treatment with inotuzumab is stopped, often after only one course.

On this basis, we submit that basing the ICER on six courses of treatment is unreasonable in the light of the evidence submitted to NICE.

[3] https://www.nice.org.uk/guidance/gid-ta10091/documents/committee-papers, comment 15, page 10 of 11

Conclusion

For the reasons listed above, we believe that the appraisal of inotuzumab ozogamicin was both unfair and unreasonable. It is on this basis that we wish to appeal the FAD through this written appeal.

We urge you to make inotuzumab ozogamicin available to all of those who could benefit from it.

Yours Sincerely,

[redacted]

[redacted]

Leukaemia CARE