Dear Jeremy

Thank you for giving me the opportunity to comment on the NICE appraisal for mannitol. I was impressed by the level of interest and commitment from the NICE committee and in particular the very fair handling of the appraisal meeting.
I have concerns about the current conclusions of the appraisal because the advice as it stands would prejudice against the group of CF patients who are experiencing a more rapid decline in lung function despite standard best therapies which would include a nebulised antibiotic , rHDNase and in some cases hypertonic saline. In that situation it would appear that a patient should receive a trial of mannitol
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Two things have changed since the time of the mannitol appraisal which may allow the committee to review things further. A national commissioning policy has been developed for CF medicines which essentially has a stepwise approach for high cost drugs related to decline in lung function and /or increase in exacerbations.
This document which has just been agreed by the Clinical Reference Group for CF Specialist commissioning may well help frame the use of Mannitol to avoid what the NICE committee do not feel would be cost effective ie all patients with CF receiving this medication.

I would suggest that as there is evidence of efficacy from the studies in patients who are already on best standard care that this new medication is targeted at patients who are failing on that best care. ie that a framework delineating a more rapid decline ie greater than 2 or 3 % per year or with more than 2 exacerbations requiring IV therapy are considered for a trial of therapy.

CF adults do not wish to take medicines that do not result in improvement and I believe that a 6 week trial would give an indication of patients with a response. Subsequent to the NICE meeting we have presented data at the ECFS and have an abstract accepted for the NACF (the US scientific meeting ) demonstrating that a response at 6 weeks is highly predictive of longer term response and so allows a stopping rule. As there is a close association between the lung function response and the exacerbation response I believe that a trial of therapy for a patient who is declining is a legitimate way to assess the medicine and prevents unnecessary long term use.

Both these developments are post the appraisal committee meeting and I wished to ensure that they are considered.

The national commissioning policy for CF drugs is just being ratified by the commissioners so that I am not at liberty today to send a copy through , however it will be available nationally within the next two weeks and I would urge the committee to review their decision and suggest that mannitol can fit as a key therapy for the patients unresponsive or intolerant of previous airway therapies.

Yours sincerely

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