Comments on the ECD Received from the Public through the NICE Website

Name	[redacted]
Role	[redacted], PHARMAC, New Zealand
Conflict	no
Notes	
Comments on individual sections of the ECD:	
Section 1 (Evaluation Committee's preliminary recommendations)	The Pharmaceutical Management Agency (PHARMAC) supports the NICE Evaluation Committees preliminary recommendation to seek a justification from Alexion Pharmaceuticals for the high cost per patient of eculizumab. PHARMAC is the New Zealand Government agency that decides which medicines and related products are funded for use in public hospitals and in the community. PHARMAC evaluated a funding application for eculizumab in paroxysmal nocturnal haemoglobinuria (PNH). In December 2013, PHARMAC made a decision to decline the funding application for eculizumab for PNH. The main reason for this decision is that the price being sought by Alexion Pharmaceuticals is too high for PHARMAC to justify funding in light of other available funding options. Detail regarding our decision for eculizumab in PNH can be found on our website: PHARMAC decision paper (http://www.pharmac.health.nz/assets/eculizumab-2013-11-board-decision-paper.pdf) Cost-utility analysis (http://www.pharmac.health.nz/assets/tar-209-eculizumab.pdf) Recommendations from clinical advisors (http://www.pharmac.health.nz/assets/ptac-and-haematology-combined-eculizumab-minutes.pdf)
Section 5 (Consideration of the Evidence)	Even if we assumed that eculizumab was 100% effective in PNH and guaranteed patients were restored to full health with normal life expectancy, at the price offered, eculizumab would still be about 20 times less cost effective than the average medicine funded by PHARMAC over the past two years. A 100% effectiveness assumption is not supported by clinical trials and the other evidence available. Alexion has been unwilling to offer a price that would bring eculizumab for PNH within an acceptable cost effectiveness range. PHARMAC has not received a funding application for eculizumab in atypical haemolytic uraemic syndrome (aHUS). We envisage that unless the pricing for eculizumab is reduced substantially, poor cost-effectiveness would again pose a barrier to funding of eculizumab for aHUS here in New Zealand.
Date	3/5/2014 7:43:00 PM

Name	[redacted]
Role	Academic economist
Conflict	no
Notes	
Comments on individual sections of the ECD:	
Section 5 (Consideration of the Evidence)	I understand that NICE is proposing to ask for information concerning the costs of development of the product. Cost information may be relevant; but in my view the most useful cost information is the average cost of development of this kind of product, rather than the costs of development of this specific product. For a general approach to using cost to help determine the maximum reasonable price, see Orphanet Journal of Rare Diseases 2013, 8:180 , at http://www.ojrd.com/content/8/1/180 . With best regards.
Date	3/4/2014 11:50:00 PM