We understand NICE's position. We also now have a much better understanding of the "many complex rules surrounding any discussion of the price or cost of a medicine" including The Pharmaceutical Price Regulation Scheme 2014 (PPRS2014) and as a result we have some further queries.

Before setting these out I would like to say that the trustees of aHUSUK appreciate having been asked to participate in the HST process and are grateful for the courtesy and fairness with which we have been treated.

Our current objectives are firstly, to make sure that all relevant information is in the Committee's possession before the meeting on 24 7 2014, at which we will be represented as observers only, and secondly, if price is still a problem, that all parties involved have made every possible effort to resolve it, thus allowing the Committee to make a positive final recommendation.

We also appreciate that at least some of the delay in the process has been due to external forces. For us at least, PPRS2014 came out of a clear blue sky [redacted] and at first we did not appreciate its full significance for the HST evaluation process. As we understand it PPRS 2014 was agreed finally between ABPI and DH and published in December 2013. It applies for 5 years from January 2014 but only to existing medicines. New medicines will be subject to VBP. Each participating ABPI member has to pay a levy to DH for the right to supply branded medicines to NHS. This takes the form of a rebate to DH, payable quarterly, calculated as a percentage of each member's net sales of qualifying medicines in the period. The percentage for the first year (2014) has been fixed at 3.74% on estimated sales. It will be adjusted on actual but will rise to an estimated 7.13% in Year 2 and 9.92% in each of Years 3, 4 and 5. These estimated percentages will vary year on year depending on whether and by how much NHS exceeds its branded medicines budget, the concept being that the total sum collected by the levy will equate to any overspend by NHS, allowing for budget growth of 0% in years 1 and 2, 1.8% in 3 and 4 and 1.9% in year 5. Effectively capping the budget for 5 years, it is a very sophisticated risk share deal and we understand that over 90% of ABPI members, including Alexion Pharma UK, are already taking part. Nothing like this featured in the PPRS 20009 or any previous versions and we doubt that it was even public information in late 2013 when the Committee was finalising the ECD. There is certainly no reference to it in that document. If it was not considered by the Committee because of timing we would like to ensure it is in July. The financial effects will be considerable. Using the aHUSUK and NICE estimates of costs of eculizumab over the next 5 years and applying the actual and estimated percentage levy currently envisaged in PPRS2014 to each, the rebate will be as undernoted.

NB 1 The estimated costs of the drug are extracted from the table in Para 13 of the Annexe to the aHUSUK Response to the ECD. As you can see, depending on whose figures turn out to be right the savings to DH generated by the rebate will will be either £22.3m or £29.8m or somewhere in between over the 5 year period.
2 The actual incidence from 1 4 2013 –31 3 2014 was 23.
3 I attach the original Annexe 1 to the aHUSUK ECD Response for reference, entitled Notes on Assumptions and Working for Budget Projection. .

Period	aHUSUK Base + 25 estimated incidence Computed 1200mg doses £m	Rebate %	Rebate £m	NICE Estimated 1200mg doses £m	Rebate %	Rebate £m
Base	33			58		

2014/15	36	3.74	1.3	58	3.74	3
2015/16	45	7.13	3.2	63	7.13	4.5
2016/17	52	9.92	5.2	69	9.92	6.8
2017/18	59	9.92	5.9	75	9.92	7.4
2018/19	68	9.92	6.7	82	9.92	8.1
Period total	261		**22.3**	347		**29.8**

PPRS 2014 also provides for Patient Access Schemes (PASs) post 2013 but under Sections 5.37- 5.40 complex PAS schemes may only be initiated either at the start of the submission to NICE, and only then once approval has been received from DH, or at the very end of the process, that is, after appeal. In our case you could say that the first of these opportunities arose on 1 4 2013, that is 9 months before PPRS 2014 even existed, and the second has yet to take place. There has therefore been no chance so far the manufacturer to submit a proposal for a PAS under PPRS 2014, so one possible opportunity to improve the cost effectiveness of the drug, in addition to the savings obtained from the PPRS levy above, has been missed.

Finally we are concerned that there has been no response from NICE to our analysis of numbers and budget costs contained in Annexe 1 to our response to the ECD, attached, given the difference between aHUSUK and NICE estimates of cost, which even after the estimated PPRS discount above is £78.5m over 5 years.

In summary therefore, we would like to be assured that the Committee will have all the relevant information about the financial effects of the PPRS levy on the net cost of eculizumab over the next 5 years, that it will be aware of the current impossibility of effecting a PAS under PPRS2014, and that it will be aware of the aHUSUK analysis of future numbers and costs.

[redacted]

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