Response to:

NATIONAL INSTITUTE FOR HEALTH AND CARE EXCELLENCE

Immunosuppressive therapy for kidney transplantation in children and adolescents (review of technology appraisal guidance 99) [ID346]

Prepared by:

Novartis Pharmaceuticals UK Limited

05 June 2015

Response to the Assessment Report produced by Peninsula technology Assessment Group (PenTAG): Immunosuppressive therapy for kidney transplantation in children and adolescents (review of technology appraisal guidance 99) [ID346]

There is no commercial in confidence information or academic in confidence information in this document

Any reference to the Assessment Report is referred to in the format: [page X, Section X.X]

Medicinal products:	Mycophenolate sodium Everolimus
Brand names:	Myfortic® Certican®
Manufacturer:	Novartis Pharmaceuticals UK Ltd
Date:	05 June 2015

Letter of response

Dear Sirs,

We would like to take this opportunity to thank the National Institute for Health and Care Excellence (NICE) and its Assessment Group (PenTAG) for the time and consideration they have devoted to this appraisal to date.

Novartis Pharmaceuticals has previously commented on the PenTAG report on immunosuppressive therapy for kidney transplantation in adults (review of technology appraisal guidance 85, ID456). We appreciate that economic assessment in paediatrics is often associated with separate and different challenges from assessments in adults, such as paucity of randomized controlled trial data and availability of child specific utility data. We also note that the licenced indications for some of the Novartis medicines under review in this appraisal (i.e. Certican (EVL) and Myfortic (EC-MPS)) do not include the paediatric and adolescent population. However, in the context of the exceptional directive from the Department of Health for NICE and PenTAG to undertake this MTA (ID346) we consider it appropriate to comment on the disease modelling.

In this instance, we find that the areas of focus for comment are similar to the areas in which we commented on the previous report relating to ID456. Specifically, the method used by PenTAG to estimate health state utilities is flawed. As in ID456, PenTAG has estimated a baseline utility based on age and gender, and then applied a utility decrement for functioning graft, dialysis or new onset diabetes after transplantation (NODAT). All patients with a functioning graft are assumed to be at the same stage of disease; ie, the same utility value was assigned to all. We are unable to find specific evidence linking health utility to renal function in children, but there is evidence of this link in adults (Neri et al, 2012). The model should be re-designed to link health utility to renal function, measured by eGFR. It would be more clinically justifiable to make this change, as it is a more granular approach, captures the wide variation in quality of life associated with a functioning graft and the long-term nephrotoxicity of calcineurin inhibitors (which can affect kidney function) and, thus, enables the model to be more sensitive to changes in the patient's health.

As in the MTA of renal transplantation in adults, PenTAG has failed to consider the impact of specific patient sub-populations on cost effectiveness of immunosupressants such as those patients with poor initial renal function post-transplant, patients at higher risk of CMV or those experiencing GI adverse events on standard immunosuppressive regimens.

Novartis therefore proposes that PenTAG should adapt its model to link health utility to renal function, measured by eGFR, and should consider patient sub-population analysis.

We welcome continued dialogue with NICE on this technology appraisal.

Yours faithfully,

[redacted]

Novartis Pharmaceuticals UK Limited

References

Neri L, McEwan P, Sennfalt K, Baboolal K. Characterizing the relationship between health utility and renal function after kidney transplantation in UK and US: a cross-sectional study. Health Qual Life Outcomes 2012; 10: 139.