Northern Treatment Advisory Group

Northern (NHS) Treatment Advisory Group

Treatment Appraisal: Decision Summary

Date	9th April 2015
Appraisal & Details	**Rituximab for the treatment of Immune (Idiopathic) Thrombocytopenic Purpura** The Northern (NHS) Treatment Advisory Group considered an appraisal of rituximab (MabThera®, Roche) for use outside its licensed indication as second-line treatment for Immune (Idiopathic) Thrombocytopenic Purpura (ITP) in adults and children.
Recommendation	**The Northern (NHS) Treatment Advisory Group recommends the use of rituximab as an option for second line treatment in adults or children with immune (idiopathic) thrombocytopenic purpura. (ITP)** The off-label use of rituximab as a second-line therapy for ITP has been recommended in two recent international guidelines, and has been acknowledged in the NICE appraisal of romiplostim for ITP as being acceptable current practice. Despite the limited data available as would be expected for an unlicensed preparation; the group had no reason to disagree with this position.
Clinical evidence summary	The group noted that the majority of evidence for the efficacy of rituximab in ITP is derived from observational studies, with no comparator arm. Only a few randomised controlled studies have been performed. The populations included in these studies varied, as did the definition of the primary outcome measures, concomitant medications, and the point at which rituximab was used in the treatment pathway. The results of studies included in this review are inconsistent, and efficacy compared to other treatments could not be determined. However the limited data suggest that rituximab treatment can induce a significant and durable response in many patients with ITP. In adults with ITP, response rates with rituximab ranged from 44% to 63%, and complete responses were seen in 30% to 44% of patients. However, as with other treatments, relapse frequently occurs.
Safety	The safety data on the use of rituximab in the treatment of ITP is poorly reported. However rituximab has been available as a licensed medicine in the UK since 1998 and the overall safety and tolerability has been well described. Adverse events associated with rituximab were generally mild to moderate in severity; with infusion-related reactions and infections the most frequently reported adverse events.
Patient Perspective	Patients may prefer a one off four week course of treatment compared to regular treatments and/or surgery for a splenectomy. However patients would need to be informed of the unlicensed nature of rituximab for this indication so an informed decision can be made.
Cost analysis summary	Rituximab is typically given as a one off four week course with the aim of inducing a long-term remission. Using a dose of 375 mg/m2 body surface area once a week for four weeks. The estimated cost for an adult (BSA 1.82 m2) is £4,889.63, and for a child (BSA 0.9 m2) is £2,444.80 per patient (assuming wastage and ex VAT). A lower fixed dose of rituximab (100 mg weekly for 4wks) appears to provide similar efficacy to the standard four week regimen. The estimated cost using this fixed dose would be £698.50 per patient (ex VAT). The cost of splenectomy (the only other one off treatment) is £3,500 to £4,500 depending upon the complexity of the procedure. It is estimated (using the NICE costing tool for romiplostim) that around 209 patients in NE & C may be eligible for treatment with rituximab or other second line option treatments.
Financial impact PbR: excluded	Rituximab is a high cost PbR excluded drug. However it is likely that some of the cost of rituximab could be offset against potential savings realised through a reduction in blood transfusions, nursing time and rescue therapy compared to other second-line options. The financial impact of this recommendation is expected to be low.