

## Enc 6 Appx 3

### Clinical Expert Summary Eltrombopag (Revolade<sup>®</sup>) 25 mg, 50 mg film coated tablets and 25 mg powder for oral suspension

Eltrombopag (Revolade<sup>®</sup>) for chronic immune (idiopathic) thrombocytopenic purpura (ITP) patients aged 1 year to < 18 years who are refractory to other treatments (e.g. corticosteroids, immunoglobulins).

#### 1. Existing guidelines

One expert reported that local guidelines for ITP in children do not affect eltrombopag prescribing, and another said that current guidelines do not include the use of thrombopoietin mimetics in children because they have not been updated since the PETIT and PETIT II studies.

#### 2. Disease prevalence/incidence

It was estimated that around 8 to 15 new cases of chronic ITP in children are seen per year in Wales. One expert predicted that 10–20 children per year would need treatment for chronic ITP.

#### 3. Current treatment options

Experts said the main treatment options are:

- ‘watch and wait’ while offering supportive treatment, with tranexamic acid or steroids, for episodes of bleeding
- rituximab given by intravenous infusion in a day care unit; the associated risk of progressive multifocal leukoencephalopathy with this treatment was highlighted.

Other treatment options reported were immunosuppressive agents, such as mycophenolate mofetil (MMF), azathioprine, ciclosporin and vincristine.

#### 4. Unmet needs

Experts highlighted the lack of new and effective treatments for chronic ITP and the subsequent lifestyle restrictions and adverse effects on quality of life for children with chronic ITP and their families. Experts also referred to the ‘potentially devastating’ side effects of the immunosuppressive treatments.

#### 5. Knowledge of product in given indication

One expert referred to the “durable remissions” seen in clinical studies of eltrombopag in children and its “very few side effects”. They reported that their use of eltrombopag would follow the inclusion/exclusion criteria of the PETIT studies: prescribing eltrombopag for children aged between 1 year and 18 years, with a confirmed diagnosis of chronic ITP for at least one year, a peripheral blood smear or bone marrow examination to support the diagnosis of ITP (with no evidence of other causes of thrombocytopenia), who are refractory or have relapsed after at least one prior ITP therapy or are unable to continue other ITP treatments, and who have a platelet count < 30 Gi/L.

Another expert reported that they would prefer to offer eltrombopag as an alternative to rituximab (and avoid immunosuppressants) for children with chronic ITP with a bleeding phenotype who maintain platelet counts < 30 x 10<sup>9</sup> L, with a higher threshold for children who like to play contact sports.

Experts felt that eltrombopag would be appropriate for specialist only prescribing, or prescribing under specialist recommendation.

It should be noted that one expert involved in compiling this response declared a non-personal specific interest in relation to eltrombopag for the indication under consideration.