Reviewer No.1

APPLICATION FOR INCLUSION OF POLYVALENT HUMAN IMMUNOGLOBULIN FOR SUBCUTANEOUS ADMINISTRATION FOR INCLUSION IN THE WHO ESSENTIAL DRUG LIST FOR CHILDREN

This application is for the inclusion of polyvalent human immunoglobulin for subcutaneous administration in the management of a variety of disorders. There is strong support for this inclusion of this medicine from a range of medical societies and eminent individuals. Polyvalent human immunoglobulin administered by intravenous infusion is already included on the list for the treatment of:

(a) Immune deficiencies where the immunoglobulin is to replace the deficiency.
(b) Specific diseases usually thought to have an autoimmune aetiology where the immunoglobulin is therapeutic.

Polyvalent human immunoglobulin for subcutaneous administration is the favoured route of administration in children (for the management of Immune deficiencies where the immunoglobulin is to replace the deficiency). The application describes the only differences between subcutaneous immunoglobulin and intravenous immunoglobulin as being the concentration and the dosing regimen.

The application acknowledges the limited data on efficacy in children and many of the studies were conducted mostly adults. There is a larger series of 51 children, treated with subcutaneous immunoglobulin, presented by Dr Alison Jones from Great Ormond Street Hospital for Sick Children.

(1) Have all important studies that you are aware of been included?

Yes ☑ No ☐

(2) Is there adequate evidence of efficacy for the proposed use?

Yes ☑ No ☐

(3) Is there evidence of efficacy in diverse settings and/or populations?

Yes ☑ No ☐

(4) Are there adverse effects of concern?

Yes ☑ No ☐

Overall immunoglobulin can be administered safely under the supervision of an experienced physician. There is the serious risk is of an allergic reaction; however this is rare and, if it occurs, can usually be managed by a standard protocol. Local reactions are relatively common but easily managed.
As immunoglobulin is derived from pooled donors, there is a risk of transmission of communicable, blood born diseases such as hepatitis and HIV. This risk has been addressed in the previous application.

(5) Are there special requirements or training needed for safe/effective use?

Yes ☑ No ☐

Immunoglobulin should be administered under the supervision of an immunologist or other experienced physician.

(6) Is this product needed to meet the majority health needs of the population?

Unknown

This has not been detailed in the application. This application covers only a subsection of those included in the Intravenous Immunoglobulin application.

(7) Is the proposed dosage form registered by a stringent regulatory authority?

Yes ☑ No ☐

(8) What action do you propose for the Committee to take?

Given the previous approval of intravenous polyvalent human immunoglobulin, I propose that the committee accept this application and that polyvalent human immunoglobulin for subcutaneous administration is added to the EML for Children.

(9) Additional comment, if any.