LIFE SAVING DRUGS PROGRAM EXPERT PANEL
THIRD MEETING: 28 June 2019

AGENDA

1. Standing business
   1.1. Welcome & apologies
   1.2. Declaration of potential or actual Conflict of Interest
   1.3. Correspondence
   1.4. Report from the Secretariat

2. Reviews of existing LSDP medicines
   TRANCHE 1 – status update
   2.1. Gaucher disease
   2.2. Fabry disease

   TRANCHE 2 – consideration of draft Review Protocols
   2.3. idursulfase (Elaprase®) for treatment of Mucopolysaccharidosis type II
   2.4. alglucosidase (Miglustat®) for treatment of Pompe disease
   2.5. eculizumab (Soliris®) for treatment of Paroxysmal Nocturnal Haemoglobinuria

   TRANCHE 3 – consideration of draft Terms of Reference
   2.6 laronidase (Aldurazyme®) for treatment of Mucopolysaccharidosis type I
   2.7 galsulfase (Nagalazyme®) for treatment of Mucopolysaccharidosis type VI
   2.8 nitisinone (Orfadin®) for Hereditary Tyrosinaemia type I

3. Applications for new LSDP medicines
   Update from Secretariat on status of new LSDP medicine applications

4. Other business