

LIFE SAVING DRUGS PROGRAM EXPERT PANEL

SIXTH MEETING: 25-26 June 2020

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 – review of final reports

- 2.1 imiglucerase (Cerezyme®), velaglucerase (VPRIV®) and taliglucerase (Elelyso®) for treatment of Gaucher disease

TRANCHE 2 – review of final reports

- 2.2 eculizumab (Soliris®) for treatment of Paroxysmal Nocturnal Haemoglobinuria
- 2.3 idursulfase (Elaprase®) for treatment of Mucopolysaccharidosis type II
- 2.4 alglucosidase alfa (Myozyme®) for treatment of Pompe disease

TRANCHE 3 – review of draft reports

- 2.5 laronidase (Aldurazyme®) for treatment of Mucopolysaccharidosis type I
- 2.6 galsulfase (Naglazyme®) for treatment of Mucopolysaccharidosis type VI
- 2.7 nitisinone (Orfadin®) for Hereditary Tyrosinaemia type I

3. Applications for new LSDP medicines

4. Other business