

# LIFE SAVING DRUGS PROGRAM EXPERT PANEL

## FIFTH MEETING: 16-17 April 2020

### AGENDA

#### 1. Standing business

- Welcome & apologies
- Declaration of potential or actual Conflict of Interest
- Correspondence
- Update from Secretariat

#### 2. Reviews of existing LSDP medicines

##### TRANCHE 1

- Consideration of Fabry disease medicines final report
- Consideration of Gaucher disease medicines draft report

##### TRANCHE 2 – consideration of draft reports

- Idursulfase (Elaprase®) for treatment of Mucopolysaccharidosis type II
- Alglucosidase alfa (Myozyme®) for treatment of Pompe disease
- Eculizumab (Soliris®) for treatment of Paroxysmal Nocturnal Haemoglobinuria

##### TRANCHE 3 – updates

- Laronidase (Aldurazyme®) for treatment of Mucopolysaccharidosis type I
- Galsulfase (Naglazyme®) for treatment of Mucopolysaccharidosis type VI
- Nitisinone (Orfadin®) for Hereditary Tyrosinaemia type I

#### 3. Applications for new LSDP medicines

#### 4. Other business