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Supplementary Material to "Safety and Efficacy of Elosulfase Alfa in Australian Patients with Morquio A Syndrome: A Phase 3b Study"

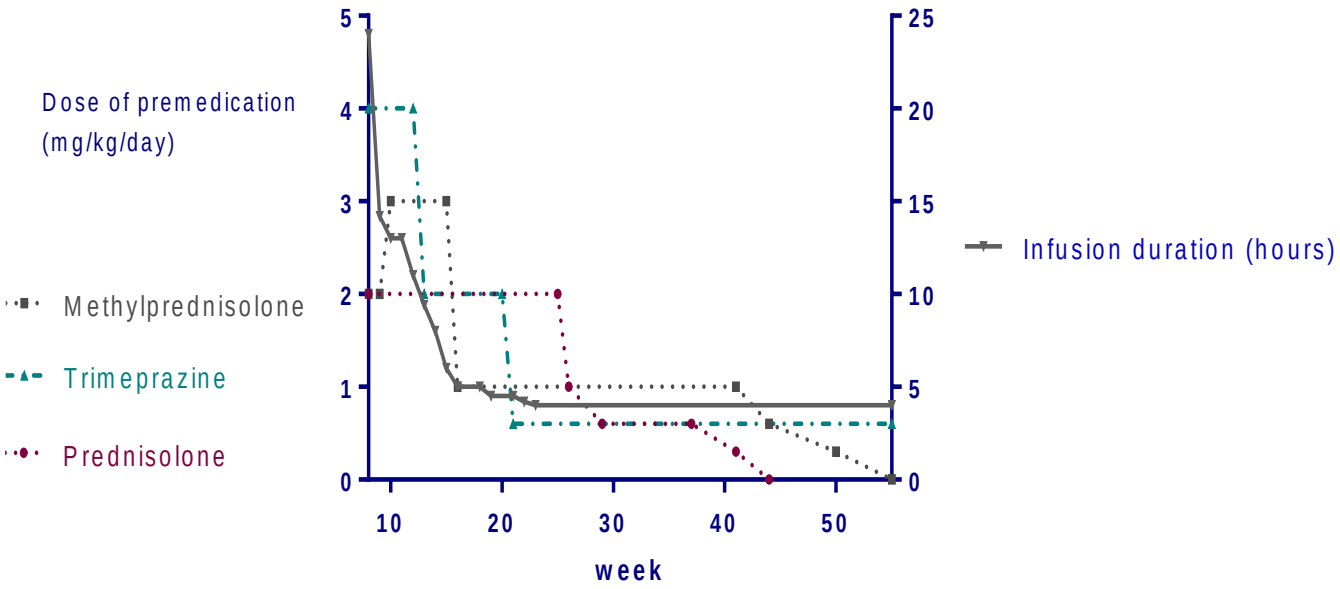
Supplementary file 2. Details on infusion associated reactions (IAR)

Details on treatment infusion interruptions requiring medical treatment

Two patients had instances of infusion interruption due to IARs that were managed by medical intervention. In the first patient, these infusion interruptions occurred in week 5 (abdominal pain, managed with ondansetron, paracetamol, and intravenous hyoscine butylbromide), week 6 (dyspnea, managed with oxygen and intravenous hydrocortisone and promethazine), week 7 (non-patent cannula dysfunction, interrupted vascular access, and dyspnea, managed by paracetamol, oxygen, and intravenous promethazine), and week 10 (hypotension, managed by intravenous normal saline and oxygen). This patient received pre-medication with intravenous hydrocortisone on week 7. The second patient required infusion interruption in week 12 due to pyrexia, despite premedication with antihistamines (loratadine) and paracetamol. All these IARs were resolved without sequelae.

Narrative of patient with significant IARs to elosulfase alfa

One individual aged 6 years at recruitment developed significant IARs. The individual had mild abdominal pain after the fifth infusion. After 25 minutes of the sixth infusion there was desaturation to 78%, tachycardia with heart rate of 149 beats per minute and hypertension with blood pressure of 132/69 mm Hg. The symptoms settled with 5 mg/kg hydrocortisone and promethazine. The following week with premedication using both these agents and loratadine, a similar event occurred 6 minutes into the infusion when the infusion rate was 6 mL/h (maximum rate 30 mL/h). For the eighth infusion, 2 mg/kg of prednisolone was administered the night before infusion with 5 mg cetirizine. One hour before the infusion, 1 mg/kg of methylprednisolone was administered and 2 mg/kg of trimeprazine. The infusion ran at 1 mL/h for 30 minutes, 3 mL/h for 210 min and 6 mL/h for 20 h. Methylprednisolone and trimeprazine doses were repeated at 12 h. The infusion was tolerated. Over the subsequent year, the rates and premedication were gradually reduced such that all steroids were discontinued and standard infusion rates were achieved.


Dose of premedication (mg/kg/day)
Methylprednisolone
Trimeprazine
Prednisolone
Infusion duration (hours)
week
0
1
2
3
4
5
0
5
10
15
20
25
10
20
30
40
50