

Adverse events reported in juvenile/adult patients treated with miglustat 200 mg three times daily p.o.

Adverse event*	Number (%) of patients treated with miglustat (N=20)				Overall
	Time interval (weeks)				
	>0-13	>13-26	>26-39	>39	
Number of patients with at least 1 treatment-emergent AE during time interval	20 (100)	20 (100)	20 (100)	18 (100)	20 (100)
Diarrhea	17 (85)	12 (60)	10 (50)	7 (39)	17 (85)
Flatulence	13 (65)	10 (50)	9 (45)	9 (50)	14 (70)
Weight decrease	1 (5)	6 (30)	10 (50)	12 (67)	13 (65)
Abdominal pain	9 (45)	2 (10)	4 (20)	3 (17)	10 (50)
Headache	2 (10)	2 (10)	4 (20)	3 (17)	9 (45)
Tremor	8 (40)	7 (35)	7 (35)	5 (28)	8 (40)
Nausea	2 (10)	2 (10)	2 (10)	2 (11)	7 (35)
Nasopharyngitis	2 (10)	1 (5)	2 (10)	2 (11)	7 (35)
Fatigue	3 (15)	3 (15)	4 (20)	5 (28)	7 (35)
Vomiting	4 (20)	2 (10)	3 (15)	0	6 (30)
Insomnia	2 (10)	3 (15)	5 (25)	5 (28)	6 (30)
Gait spastic	0	1 (5)	4 (20)	4 (22)	5 (25)
Appetite decrease	2 (10)	5 (25)	5 (25)	5 (28)	5 (25)
Depression	3 (15)	3 (15)	3 (15)	4 (22)	4 (20)
Tremor aggravated	4 (20)	3 (15)	1 (5)	2 (11)	5 (25)
Paresthesia	2 (10)	1 (5)	3 (15)	2 (11)	4 (20)
Dysphagia	1 (5)	1 (5)	3 (15)	4 (22)	4 (20)
Abdominal distension	4 (20)	2 (10)	1 (5)	0	4 (20)
Laceration	0	3 (15)	0	2 (11)	4 (20)

AE=adverse event; data collected from clinical trial 007

Adverse events reported in the juvenile/adult patients receiving standard care.

Adverse event*	Number (%) of patients receiving standard care (N=9)				Overall
	Time interval (weeks)				
	>0-13	>13-26	>26-39	>39	
Number of patients with at least 1 study emergent AE during time interval	9 (100)	8 (89)	7 (88)	8 (100)	9 (100)
Gait abnormal	2 (22)	4 (44)	4 (50)	3 (38)	4 (44)
Diarrhea	0	3 (33)	1 (13)	2 (25)	4 (44)
Dysphagia	3 (33)	4 (44)	4 (50)	4 (50)	4 (44)
Headache	2 (22)	0	1 (13)	0	3 (33)
Dizziness	1 (11)	1 (11)	1 (13)	2 (25)	3 (33)
Tremor	0	1 (11)	1 (13)	2 (25)	2 (22)
Nasopharyngitis	1 (11)	1 (11)	1 (13)	1 (13)	3 (33)
Fall	2 (22)	2 (22)	1 (13)	1 (13)	2 (22)
Pain in limb	1 (11)	0	0	1 (13)	2 (22)
Eyelid ptosis	1 (11)	2 (22)	1 (13)	1 (13)	2 (22)
Deafness	0	1 (11)	1 (13)	2 (25)	2 (22)

AE=adverse event; \*data collected from clinical trial 007

Adverse events reported in the paediatric patients receiving miglustat (dose adjusted according to body surface area)

Adverse event*	Number (%) of paediatric patients receiving miglustat (N=12)				Overall
	Time interval (weeks)				
	>0-13	>13-26	>26-39	>39	
Number of patients with at least one AE during time interval	11 (92)	11 (100)	10 (91)	10 (100)	12 (100)
Diarrhea	8 (67)	8 (73)	7 (64)	6 (60)	8 (67)
Fatigue	3 (25)	3 (27)	3 (27)	2 (20)	5 (42)
Gait abnormal	1 (8)	3 (27)	3 (27)	3 (30)	4 (33)
Flatulence	4 (33)	1 (9)	1 (9)	1 (10)	4 (33)
Vomiting	2 (17)	0	1 (9)	2 (20)	4 (33)
Nasopharyngitis	3 (25)	3 (27)	2 (18)	2 (20)	4 (33)
Cough	2 (17)	3 (27)	2 (18)	0	4 (33)
Ataxia	1 (8)	2 (18)	3 (27)	3 (30)	3 (25)
Hyperreflexia	0	0	3 (27)	3 (30)	3 (25)
Dysphagia	1 (8)	1 (9)	3 (27)	3 (30)	3 (25)
Sinusitis	1 (8)	2 (18)	0	1 (10)	3 (25)
Weight decrease	0	1 (9)	1 (9)	3 (30)	3 (25)

AE=adverse event; \*data collected from clinical trial 007