

**Table S3.** TEAEs leading to study drug discontinuation.

	<b>CORE STUDY</b>		
	<b>Cohort 1</b>	<b>Cohort 2</b>	<b>Total</b>
	<b>≥7 to &lt;12 years</b>	<b>≥2 to &lt;7 years</b>	
	<b>(N = 28)</b>	<b>(N = 22)</b>	<b>(N = 50)</b>
<b>Any TEAE leading to study</b>	<b>2 (7.1)</b>	<b>1 (4.5)</b>	<b>3 (6.0)</b>
<b>drug discontinuation, n (%)</b>			
Gait disturbance	1 (3.6)	0 (0.0)	1 (2.0)
Irritability	1 (3.6)	0 (0.0)	1 (2.0)
Grand mal convulsion	0 (0.0)	1 (4.5)	1 (2.0)
Psychomotor hyperactivity	1 (3.6)	0 (0.0)	1 (2.0)
Tremor	1 (3.6)	0 (0.0)	1 (2.0)
Abnormal behavior	1 (3.6)	0 (0.0)	1 (2.0)
Emotional distress	1 (3.6)	0 (0.0)	1 (2.0)
	<b>EXTENSION</b>		
	<b>Cohort 1</b>	<b>Cohort 2</b>	<b>Total</b>
	<b>≥7 to &lt;12 years</b>	<b>≥2 to &lt;7 years</b>	
	<b>(N = 22)</b>	<b>(N = 19)</b>	<b>(N = 41)</b>
<b>Any TEAE leading to study</b>	<b>2 (9.1)</b>	<b>3 (15.8)</b>	<b>5 (12.2)</b>
<b>drug discontinuation, n (%)</b>			
Aggression	1 (4.5)	1 (5.3)	2 (4.9)
Gingival recession	0 (0.0)	1 (5.3)	1 (2.4)
Oral mucosal discoloration	0 (0.0)	1 (5.3)	1 (2.4)
Blood alkaline phosphatase increased	1 (4.5)	0 (0.0)	1 (2.4)

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Total bile acids increased	1 (4.5)	0 (0.0)	1 (2.4)
Urine bilirubin increased	1 (4.5)	0 (0.0)	1 (2.4)
Lethargy	0 (0.0)	1 (5.3)	1 (2.4)
Suicidal ideation	1 (4.5)	0 (0.0)	1 (2.4)

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For each row category, a patient with two or more TEAEs in that category is counted only once.

MedDRA: Medical Dictionary for Regulatory Activities; TEAE: treatment-emergent adverse event.