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Patient-Reported Outcomes from a Randomized, Active-Controlled, Open-Label, Phase 3 Trial of Burosumab Versus Conventional Therapy in Children with X-Linked Hypophosphatemia

Journal name

Calcified Tissue International

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Electronic Supplementary Material

Table 2 Questions from the validated fixed-length short-form PROMIS pediatric self-report (≥ 8 years) and parent proxy report (5 to <8 years) used in this trial

PROMIS pediatric self-report questions	PROMIS parent report questions
Physical function mobility. In both questionnaires, response options were “with no trouble,” “with a little trouble,” “with some trouble,” “with a lot of trouble,” and “not able to do.”	
In the past 7 days...	In the past 7 days...
I could do sports and exercise that other kids my age could do.	My child could do sports and exercise that other kids his/her age could do.
I could get up from the floor.	My child could get up from the floor.
I could keep up when I played with other kids.	My child could keep up when he/she played with other kids.
I could walk up stairs without holding on to anything.	My child could walk up stairs without holding on to anything.
I have been physically able to do the activities I enjoy most.	My child has been physically able to do the activities he/she enjoys most.
I could carry my books in my backpack.	My child could carry his/her books in a backpack.
I could get down on my knees without holding on to something.	My child could get down on his/her knees without holding on to something.
I could get in and out of a car.	My child could get in and out of a car.
I could ride a bike.	My child could ride a bike.
I could walk more than one block.	My child could walk more than one block.
Fatigue. In both questionnaires, response options were “never,” “almost never,” “sometimes,” “often,” and “almost always.”	
In the past 7 days...	In the past 7 days...
Being tired made it hard for me to play or go out with my friends as much as I'd like.	Being tired made it hard for my child to play or go out with friends as much as he/she would like.

I got tired easily.	My child got tired easily.
I was too tired to do sports or exercise.	My child was too tired to do sports or exercise.
I was too tired to do things outside.	My child was too tired to do things outside.
I was too tired to enjoy the things I like to do.	My child was too tired to enjoy the things he/she likes to do.
I felt too tired to spend time with my friends.	My child felt too tired to spend time with his/her friends.
I needed to sleep during the day.	My child needed to sleep during the day.
I was too tired to go up and down a lot of stairs.	My child was too tired to go up and down a lot of stairs.
Pain interference. In both questionnaires, response options were “never,” “almost never,” “sometimes,” “often,” and “almost always.”	
In the past 7 days...	In the past 7 days...
I had trouble sleeping when I had pain.	My child had trouble sleeping when he/she had pain.
It was hard for me to run when I had pain.	It was hard for my child to run when he/she had pain.
It was hard for me to walk one block when I had pain.	It was hard for my child to walk one block when he/she had pain.
It was hard to stay standing when I had pain.	It was hard for my child to stay standing when he/she had pain.

PROMIS Patient-Reported Outcomes Measurement Information System

Table 3 Item response variability in PROMIS pain interference, physical function mobility, and fatigue domains for patients receiving burosumab or conventional therapy

PROMIS domain	Burosumab					Conventional therapy				
	<i>n</i> (%)	<i>n</i> (%)	<i>n</i> (%)	<i>n</i> (%)	<i>n</i> (%)	<i>n</i> (%)	<i>n</i> (%)	<i>n</i> (%)	<i>n</i> (%)	<i>n</i> (%)
Pain interference	Never	Almost never	Sometimes	Often	Almost always	Never	Almost never	Sometimes	Often	Almost always
Had trouble sleeping when I/they had pain										
Baseline	9 (60.0)	1 (6.7)	3 (20.0)	0	2 (13.3)	10 (50.0)	3 (15.0)	5 (25.0)	1 (5.0)	1 (5.0)
Week 64	10 (66.7)	3 (20.0)	2 (13.3)	0	0	12 (60.0)	3 (15.0)	3 (15.0)	2 (10.0)	0
Hard to run when I/they had pain										
Baseline	4 (26.7)	1 (6.7)	7 (46.7)	0	3 (20.0)	8 (40.0)	2 (10.0)	3 (15.0)	3 (15.0)	4 (20.0)
Week 64	5 (33.3)	6 (40.0)	3 (20.0)	1 (6.7)	0	7 (35.0)	4 (20.0)	6 (30.0)	3 (15.0)	0
Hard to walk when I/they had pain										
Baseline	4 (26.7)	2 (13.3)	6 (40.0)	1 (6.7)	2 (13.3)	10 (50.0)	2 (10.0)	3 (15.0)	2 (10.0)	3 (15.0)
Week 64	4 (26.7)	7 (46.7)	2 (13.3)	1 (6.7)	1 (6.7)	8 (40.0)	3 (15.0)	4 (20.0)	3 (15.0)	2 (10.0)

Hard to stand when I/they had pain										
Baseline	8 (53.3)	1 (6.7)	3 (20.0)	1 (6.7)	2 (13.3)	11 (55.0)	4 (20.0)	2 (10.0)	1 (5.0)	2 (10.0)
Week 64	5 (33.3)	6 (40.0)	3 (20.0)	0	1 (6.7)	10 (50.0)	4 (20.0)	3 (15.0)	3 (15.0)	0
Physical function mobility	Not able to do	With a lot of trouble	With some trouble	With a little trouble	With no trouble	Not able to do	With a lot of trouble	With some trouble	With a little trouble	With no trouble
I/they could do sports and exercise										
Baseline	0	1 (6.7)	6 (40.0)	3 (20.0)	5 (33.3)	1 (5.0)	2 (10.0)	0	9 (45.0)	8 (40.0)
Week 64	0	1 (6.7)	1 (6.7)	6 (40.0)	7 (46.7)	0	1 (5.0)	4 (20.0)	5 (25.0)	10 (50.0)
I/they could get up from the floor										
Baseline	0	0	0	1 (6.7)	14 (93.3)	0	0	0	4 (20.0)	16 (80.0)
Week 64	0	0	1 (6.7)	0	14 (93.3)	0	0	0	5 (25.0)	15 (75.0)
I/they could keep up when I/they played										
Baseline	0	2 (13.3)	4 (26.7)	5 (33.3)	4 (26.7)	0	3 (15.0)	6 (30.0)	4 (20.0)	7 (35.0)
Week 64	0	0	4 (26.7)	4 (26.7)	7 (46.7)	0	1 (5.0)	3 (15.0)	7 (35.0)	9 (45.0)

I/they could walk up stairs without holding										
Baseline	1 (6.7)	0	2 (13.3)	4 (26.7)	8 (53.3)	1 (5.0)	2 (10.0)	2 (10.0)	2 (10.0)	13 (65.0)
Week 64	0	0	3 (20.0)	4 (26.7)	8 (53.3)	0	1 (5.0)	2 (10.0)	4 (20.0)	13 (65.0)
I/they was/were able to do the activities I/they enjoy most										
Baseline	0	0	1 (6.7)	8 (53.3)	6 (40.0)	1 (5.0)	0	3 (15.0)	4 (20.0)	12 (60.0)
Week 64	0	0	1 (6.7)	2 (13.3)	12 (80.0)	0	0	3 (15.0)	4 (20.0)	13 (65.0)
I/they could carry my/their books in my/their backpack										
Baseline	0	1 (6.7)	0	5 (33.3)	9 (60.0)	1 (5.0)	1 (5.0)	2 (10.0)	1 (5.0)	15 (75.0)
Week 64	0	0	1 (6.7)	1 (6.7)	13 (86.7)	0	0	3 (15.0)	5 (25.0)	12 (60.0)
I/they could get down on my/their knees without holding										
Baseline	0	1 (6.7)	0	1 (6.7)	13 (86.7)	1 (5.0)	0	1 (5.0)	4 (20.0)	14 (70.0)
Week 64	0	0	1 (6.7)	2 (13.3)	12 (80.0)	1 (5.0)	0	1 (5.0)	4 (20.0)	14 (70.0)
I/they could get in and out of a car										
Baseline	0	0	0	2 (13.3)	13 (86.7)	0	0	1 (5.0)	3 (15.0)	16 (80.0)
Week 64	0	0	2 (13.3)	0	13 (86.7)	0	0	0	4 (20.0)	16 (80.0)

I/they could ride a bike										
Baseline	0	0	1 (6.7)	1 (6.7)	13 (86.7)	1 (5.0)	1 (5.0)	3 (15.0)	3 (15.0)	12 (60.0)
Week 64	0	0	1 (6.7)	2 (13.3)	12 (80.0)	2 (10.0)	0	3 (15.0)	3 (15.0)	12 (60.0)
I/they could walk more than one block										
Baseline	0	1 (6.7)	1 (6.7)	4 (26.7)	9 (60.0)	3 (15.0)	2 (10.0)	0	3 (15.0)	12 (60.0)
Week 64	0	1 (6.7)	1 (6.7)	3 (20.0)	10 (66.7)	2 (10.0)	3 (15.0)	1 (5.0)	5 (25.0)	9 (45.0)
Fatigue	Never	Almost never	Sometimes	Often	Almost always	Never	Almost never	Sometimes	Often	Almost always
Being tired made it hard for me/them to play										
Baseline	8 (53.3)	4 (26.7)	1 (6.7)	1 (6.7)	1 (6.7)	10 (50.0)	3 (15.0)	5 (25.0)	2 (10.0)	0
Week 64	7 (46.7)	4 (26.7)	4 (26.7)	0	0	11 (55.0)	6 (30.0)	3 (15.0)	0	0
I/they got tired easily										
Baseline	4 (26.7)	5 (33.3)	4 (26.7)	2 (13.3)	0	10 (50.0)	2 (10.0)	4 (20.0)	1 (5.0)	3 (15.0)
Week 64	7 (46.7)	3 (20.0)	4 (26.7)	1 (6.7)	0	9 (45.0)	4 (20.0)	6 (30.0)	1 (5.0)	0

I/they was/were too tired to do sports or exercise										
Baseline	6 (40.0)	4 (26.7)	2 (13.3)	2 (13.3)	1 (6.7)	11 (55.0)	4 (20.0)	1 (5.0)	2 (10.0)	2 (10.0)
Week 64	8 (53.3)	3 (20.0)	3 (20.0)	1 (6.7)	0	11 (55)	4 (20.0)	5 (25.0)	0	0
I/they was/were too tired to do things outside										
Baseline	10 (66.7)	2 (13.3)	3 (20.0)	0	0	11 (55)	4 (20.0)	2 (10.0)	3 (15.0)	0
Week 64	10 (66.7)	2 (13.3)	2 (13.3)	1 (6.7)	0	13 (65.0)	2 (10.0)	5 (25.0)	0	0
I/they was/were too tired to enjoy things I/they like										
Baseline	9 (60.0)	4 (26.7)	2 (13.3)	0	0	14 (70.0)	2 (10.0)	1 (5.0)	3 (15.0)	0
Week 64	10 (66.7)	4 (26.7)	1 (6.7)	0	0	15 (75.0)	1 (5.0)	4 (20.0)	0	0
I/they felt too tired to spend time with friends										
Baseline	10 (66.7)	4 (26.7)	1 (6.7)	0	0	13 (65.0)	5 (25.0)	1 (5.0)	1 (5.0)	0
Week 64	10 (66.7)	4 (26.7)	1 (6.7)	0	0	13 (65.0)	4 (20.0)	3 (15.0)	0	0
I/they needed to sleep during the day										
Baseline	6 (40.0)	6 (40.0)	3 (20.0)	0	0	14 (70.0)	3 (15.0)	3 (15.0)	0	0
Week 64	9 (60.0)	2 (13.3)	4 (26.7)	0	0	13 (65.0)	4 (20.0)	3 (15.0)	0	0

I/they was/were too tired to go up and down stairs										
Baseline	6 (40.0)	4 (26.7)	5 (33.3)	0	0	9 (45.0)	5 (25.0)	3 (15.0)	1 (5.0)	2 (10.0)
Week 64	10 (66.7)	2 (13.3)	3 (20.0)	0	0	11 (55.0)	4 (20.0)	4 (20.0)	0	1 (5.0)

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