

Supplementary Table 7. Administered questionnaires

Age	1y – 4y	5y	6y – 7y	8y – 15y	16y – 17y	18y+	18y+ Representative
Socio-demographics	<i>Parent proxy:</i> Socio-demographic questionnaire	<i>Parent proxy:</i> Socio-demographic questionnaire	<i>Parent proxy:</i> Socio-demographic questionnaire	<i>Parent proxy:</i> Socio-demographic questionnaire	<i>Parent proxy:</i> Socio-demographic questionnaire	<i>Self:</i> Socio-demographic questionnaire	<i>Proxy:</i> Socio-demographic questionnaire
PROMIS Mental health				<i>Self:</i> Anxiety CAT V2.0* Depressive symptoms CAT V2.0* Cognitive function SF7a V1.0#	<i>Self:</i> Anxiety CAT V2.0* Depressive symptoms CAT V2.0* Cognitive function SF7a V1.0#	<i>Self:</i> Anxiety CAT V1.0* Depression CAT V1.0* Cognitive function SF8a V2.0#	<i>Proxy:</i> Cognitive function SF8a V2.0#
		<i>Parent proxy:</i> Anxiety CAT V2.0\$ Depressive symptoms CAT V2.0\$ Cognitive function SF7a V1.0\$	<i>Parent proxy:</i> Anxiety CAT V2.0\$ Depressive symptoms CAT V2.0\$ Cognitive function SF7a V1.0\$	<i>Parent proxy:</i> Anxiety CAT V2.0\$ Depressive symptoms CAT V2.0\$ Cognitive function SF7a V1.0\$	<i>Parent proxy:</i> Anxiety CAT V2.0\$ Depressive symptoms CAT V2.0\$ Cognitive function SF7a V1.0\$		
PROMIS Physical health				<i>Self:</i> Physical function CAT V2.0\$ Fatigue CAT V2.0*	<i>Self:</i> Physical function CAT V2.0\$ Fatigue CAT V2.0*	<i>Self:</i> Physical function CAT V2.0* Fatigue V1.0 SF8a*	<i>Proxy:</i> Physical function CAT V2.0*
		<i>Parent proxy:</i> Physical function UE SF8a V2.0\$ Fatigue CAT V2.0\$	<i>Parent proxy:</i> Physical function UE SF8a V2.0\$ Fatigue CAT V2.0\$	<i>Parent proxy:</i> Physical function UE SF8a V2.0\$ Fatigue CAT V2.0\$	<i>Parent proxy:</i> Physical function UE SF8a V2.0\$ Fatigue CAT V2.0\$		
PROMIS Social health				<i>Self:</i> Peer relationships CAT V2.0*	<i>Self:</i> Peer relationships CAT V2.0*	<i>Self:</i> Ability to participate in social roles and activities CAT V2.0* Satisfaction with social roles and activities CAT V2.0* Companionship SF6a V2.0# Emotional support SF8a V2.0# Social isolation SF8a V2.0#	<i>Proxy:</i> Ability to participate in social roles and activities CAT V2.0* Companionship SF6a V2.0#
		<i>Parent proxy:</i> Peer relationships CAT V2.0\$	<i>Parent proxy:</i> Peer relationships CAT V2.0\$	<i>Parent proxy:</i> Peer relationships CAT V2.0\$	<i>Parent proxy:</i> Peer relationships CAT V2.0\$		
TNO Questionnaires (and subscales)	<i>Proxy:</i> TAPQOL - Sleep - Appetite	<i>Parent proxy:</i> TAPQOL - Sleep - Appetite	<i>Parent proxy:</i> TACQOL - Physical symptoms	<i>Self + Parent proxy:</i> TACQOL - Physical symptoms	<i>Self:</i> TAAQOL - Gross motor - Fine motor	<i>Self:</i> TAAQOL - Gross motor - Fine motor	

Supplementary Table 7. Administered questionnaires

Age	1y – 4y	5y	6y – 7y	8y – 15y	16y – 17y	18y+	18y+ Representative
	- Lungs	- Lungs	- Motor function	- Motor function	- Pain	- Pain	
	- Stomach	- Stomach	- Autonomy	- Autonomy	- Sleeping	- Sleeping	
	- Skin	- Skin	- Cognitive	- Cognitive	- Cognitive	- Cognitive	
	- Motor function (≥ 1.5 years)	- Motor function	- function	- function	- function	- function	
	- Social function (≥ 1.5 years)	- Social function	- Social function	- Social function	- Social function	- Social function	
	- Problem (≥ 1.5 years)	- Problem	- Positive	- Positive	- Daily activities	- Daily activities	
	- Problem behavior	- Communication behavior	- Negative emotions	- Negative emotions	- Sexuality	- Sexuality	
	- Communication (≥ 1.5 years)	- Anxiety	- Negative emotions	- Negative emotions	- Vitality	- Vitality	
	- Anxiety	- Positive mood			- Happiness	- Happiness	
	- Positive mood	- Liveliness			- Depressive moods	- Depressive moods	
	- Liveliness				- Aggressiveness	- Aggressiveness	

Notes. y = years, m = months, PROMIS = Patient-Reported Outcome Measurement Information System, CAT = Computerized Adaptive Tests, SF = Short form, UE = Upper Extremity. * Compared to the Dutch general population, # Compared to the US general population, § Compared to a subset of the US general population and a clinical population.

PROMIS pediatric and proxy-report measures and corresponding normative data: Anxiety – CAT V2.0 (1, 2), Depressive symptoms – CAT V2.0 (1, 3), Cognitive Function – SF7a V1.0 (4), Fatigue – CAT V2.0 (5, 6), Physical Function Upper Extremity (UE) Function – CAT V2.0 and Physical Function Upper Extremity (UE) Function proxy-report SF8a V2.0 (7) and Peer relationships – CAT V2.0 (8, 9).

PROMIS adult measures: Anxiety – CAT V1.0 (10), Depression – CAT V1.0 (10), Cognitive Function – SF8a V2.0 (4), Fatigue – SF8a V1.0 (11), Physical Function – CAT V2.0 (12), Ability to Participate in Social Roles and Activities – CAT V2.0 (12), Satisfaction with Social Roles and Activities – CAT V2.0 (12), Companionship – SF6a V2.0 (13), Emotional Support – SF8a V2.0 (14) and Social isolation – SF8a V2.0 (15)). TAPQOL = TNO-AZL Questionnaire for Preschool Children's Health-Related Quality of Life (16), TACQOL = TNO-AZL Questionnaire for Children's Health-Related Quality of Life (17), TAAQOL = TNO-AZL Questionnaire for Adult Health-Related Quality of Life (16).

References

1. Klaufus L, Luijten M, Verlinden E, Van der Wal M, Haverman L, Cuijpers P, et al. Psychometric properties of the Dutch-Flemish PROMIS® pediatric item banks Anxiety and Depressive Symptoms in a general population. *Qual Life Res.* 2021;30(9):2683-95.
2. PROMIS. Anxiety scoring manual. 2022.
https://staging.healthmeasures.net/images/PROMIS/manuals/Scoring_Manual_Only/PROMIS_Anxiety_Scoring_Manual_03June2022.pdf. Accessed 01 Sep 2022.
3. PROMIS. Depression scoring manual. 2022.
https://staging.healthmeasures.net/images/PROMIS/manuals/Scoring_Manual_Only/PROMIS_Depression_Scoring_Manual_03June2022.pdf. Accessed 01 Sep 2022.
4. PROMIS. Cognitive function scoring manual. 2022.
https://staging.healthmeasures.net/images/PROMIS/manuals/Scoring_Manual_Only/PROMIS_Cognitive_Function_Scoring_Manual_03June2022.pdf. Accessed 01 Sep 2022.
5. Peersmann SH, Luijten MA, Haverman L, Terwee CB, Grootenhuis MA, van Litsenburg RR. Psychometric properties and CAT performance of the PROMIS pediatric sleep disturbance, sleep-related impairment, and fatigue item banks in Dutch children and adolescents. *Psychol Assess.* 2022;34(9):860.
6. PROMIS. Fatigue scoring manual. 2022.
https://staging.healthmeasures.net/images/PROMIS/manuals/Scoring_Manual_Only/PROMIS_Fatigue_Scoring_Manual_28June2022.pdf. Accessed 01 Sep 2022.
7. PROMIS. Physical function scoring manual. 2022.
https://staging.healthmeasures.net/images/PROMIS/manuals/Scoring_Manual_Only/PROMIS_Physical_Function_Scoring_Manual_26May2022.pdf. Accessed 01 Sep 2022.
8. Luijten MA, van Litsenburg RR, Terwee CB, Grootenhuis MA, Haverman L. Psychometric properties of the Patient-Reported Outcomes Measurement Information System (PROMIS®) pediatric item bank peer relationships in the Dutch general population. *Qual Life Res.* 2021;30:2061-70.
9. PROMIS. Social relationships (peer, family) scoring manual. 2022.
https://staging.healthmeasures.net/images/PROMIS/manuals/Scoring_Manual_Only/PROMIS_Social_Relationships_Scoring_Manual_03June2022.pdf. Accessed 01 Sep 2022.
10. Elsmann EB, Flens G, de Beurs E, Roorda LD, Terwee CB. Towards standardization of measuring anxiety and depression: Differential item functioning for language and Dutch reference values of PROMIS item banks. *PLoS One.* 2022;17(8):e0273287.
11. Terwee CB, Elsmann EB, Roorda LD. Towards standardization of fatigue measurement: Psychometric properties and reference values of the PROMIS Fatigue item bank in the Dutch general population. *Research Methods in Medicine & Health Sciences.* 2022;3(3):86-98.
12. Terwee CB, Roorda LD. Country-specific reference values for PROMIS® pain, physical function and participation measures compared to US reference values. *Ann Med.* 2023;55(1):1-11.
13. PROMIS. Companionship scoring manual. 2022.
https://staging.healthmeasures.net/images/PROMIS/Differences_Between_PROMIS_Measures/PROMIS_Companionship_Measure_Differences_24Jan2022.pdf. Accessed 01 Sep 2022.
14. PROMIS. Emotional support scoring manual. 2021.
https://staging.healthmeasures.net/images/PROMIS/manuals/Scoring_Manuals_/PROMIS_Emotional_Support_Scoring_Manual.pdf. Accessed 01 Sep 2022.
15. PROMIS. Social isolation scoring manual. 2021.
https://staging.healthmeasures.net/images/PROMIS/manuals/Scoring_Manuals_/PROMIS_Social_Isolation_Scoring_Manual.pdf. Accessed 01 Sep 2022.
16. TNO. Vragenlijsten kwaliteit van leven. 2021. Available from:
https://www.tno.nl/media/5004/vragenlijsten_01032012.pdf. Accessed 01 Sep 2022.
17. Bosch AM, Grootenhuis MA, Bakker HD, Heijmans HS, Wijburg FA, Last BF. Living with classical galactosemia: health-related quality of life consequences. *Pediatrics.* 2004;113(5):e423-8.