Health-Related Quality of Life and Fatigue in Children and Adults with Pyruvate Kinase Deficiency

SUPPLEMENTARY APPENDIX

Supplemental Table 1. List of Site Investigators (Enrolling sites)

Principal Investigator	Institution
United States, enrollment: 154	
Heather A. Bradeen, MD	The University of Vermont Children's Hospital, Burlington, VT
Sateesh Chonat, MD	Emory University School of Medicine , Aflac Cancer and Blood Disorders Center, Children's Healthcare of Atlanta, GA
Jenny M. Despotovic, DO	Texas Children's Hematology Center, Baylor College of Medicine, Houston, TX
Bertil Glader, MD, PhD	Lucile Packard Children's Hospital, Stanford University School of Medicine, Palo Alto, CA
Rachael F. Grace, MD	Dana-Farber/Boston Children's Cancer and Blood Disorders Center, Boston, MA
Christine M. Knoll, MD	Phoenix Children's Hospital, Phoenix, AZ
Melissa McNaull, MD	University of Mississippi Medical Center, Jackson, MS
D. Holmes Morton, MD	Central Pennsylvania Clinic for Special Children & Adults, Belleville, PA Lancaster General Hospital, Lancaster, PA
Peter E. Newburger, MD	University of Massachusetts Medical School, Worcester, MA
Janet L. Kwiatkowski, MD, MSCE	Children's Hospital of Philadelphia and Perelman School of Medicine of the University of Pennsylvania, Philadelphia, PA
Latika Puri, MD	St. Jude Children's Research Hospital, Memphis, TN
Yaddanapudi Ravindranath, MD, MBBS	Wayne State University School of Medicine, Children's Hospital of Michigan, Detroit, MI

Melissa J. Rose, DO	Nationwide Children's Hospital, The Ohio State University College of Medicine, Columbus, OH
Jennifer A. Rothman, MD	Duke University Medical Center, Durham, NC
Mukta Sharma, MD	Children's Mercy Hospital, Kansas City, MO
Sujit Sheth, MD	Weill Cornell Medical College, New York, NY
Alexis A. Thompson, MD	Ann & Robert H. Lurie Children's Hospital of Chicago, Chicago, IL
Heng Wang, MD, PhD	DDC Clinic for Special Needs Children, Middlefield, OH
Hassan M. Yaish, MD	Primary Children's Hospital, University of Utah, Salt Lake City, UT
Canada, enrollment: 14	
Vicky R Breakey, MD Madeleine Verhovsek, MD	McMaster University, Hamilton, ON, Canada
Kevin H.M. Kuo, MD, MSc, FRCPC	University of Toronto, University Health Network, Toronto, ON
Yves D. Pastore, MD	CHU Sainte-Justine, Montreal, Canada
Italy, enrollment: 25	
Wilma Barcellini, MD	Fondazione IRCCS Ca' Granda Ospedale Maggiore Policlinico, Milan
Netherlands, enrollment: 23	
Eduard J. van Beers, MD	Van Creveldkliniek, University Medical Center Utrecht, Utrecht University, Utrecht
Germany, enrollment: 31	
Stefan W. Eber, MD	Schwerpunktpraxis für Pädiatrische Hämatologie-Onkologie and Children's Hospital, Technical University,Munich, Germany

Susanne Holzhauer, MD	Charite, Berlin, Germany
Nina Kollmar, MD	Klinikum Kassel GmbH, Kassel, Germany
Joachim Kunz, MD, PhD	Zentrum für Kunder-und Jugendmedizin, Heidelberg, Germany
Marcin W. Wlodarski, MD, PhD	University of Freiburg, Freiburg, Germany
Czech Republic, enrollment: 7	
Dagmar Pospíšilová, MD	Fakultni nemocnice Olomouc, Czech Republic

Supplemental Table 2. Scoring and availability of quality of life measures by age group and language

	EQ-5D	FACT-An	PROMIS Fatigue SF-7a	PedsQL 4.0	pedsFACIT-F	PROMIS Fatigue SF
Age Group	≥18 y	≥18 y	≥18 y	Parent Proxy: children 2-17 y Child Self-report: 5-17 y	Child report: ages 8-17 y	Parent proxy 10a: children 5-17 y Child Self-report 10a: 8-17 y
Languages available for this study	English, Spanish, Canadian French, German, Italian, Dutch	English, French, German, Italian, Russian, Dutch, Spanish, Czech	English, Spanish	English, French, German, Italian, Dutch, Spanish	English, French, German, Italian, Russian, Dutch, Spanish, Czech	English, Spanish
Scoring	Visual Analog Scale: Higher score represents best health (0-100) Health Utility Score[1]: Higher score represents full health (0 to 1)	Higher score represents low fatigue (0-188)	Higher score represent more fatigue (T-scores: standard score mean of 50, SD 10)	Higher score represents full health (0-100)	Higher score represents low fatigue (0-52)	Higher score represent more fatigue (T-scores: standard score mean of 50, SD 10)

Completion rate at enrollment	Visual Analog Scale: 120/131 Health Utility Score: 86/131	126/131	60/131	Parent Proxy: 86/123 Child Self-report: 61/123	47/123	Parent proxy 10a: 48/123 Child Self-report: 36/123

[1] Health Utility Score could only be analysed for participants from the US, Germany, and the Netherlands