Appendix 2 Coinvestigators

Name	Location	Role	Contribution
Adnar Manzur, FRCPCH	Great Ormond Street Hospital, UCL, London, UK.	Site investigator	Data acquisition and revised the manuscript for intellectual content
Anne Marie Childs, MD	Leeds Teaching Hospitals NHS Trust (LTHT), UK	Site investigator	Data acquisition and revised the manuscript for intellectual content
Ashutosh Kumar, MD	Penn State health Milton S Hershey Medical Centre	Site investigator	Data acquisition and revised the manuscript for intellectual content
Basil T. Darras, MD	Department of Neurology, Boston Children's Hospital and Harvard Medical School, Boston, MA	Site investigator	Data acquisition and revised the manuscript for intellectual content
Craig Campbell, MD, MSc, FRCPC	Department of Pediatrics, Clinical Neurological Sciences and Epidemiology, University of Western Ontario, London, Ontario, Canada	Site investigator	Data acquisition and revised the manuscript for intellectual content
Craig M. McDonald, MD	UC Davis Medical Center, USA.	Site investigator	Data acquisition and revised the manuscript for intellectual content
Elena Pegoraro, MD, PhD	University of Padova, Padova Italy	Site investigator	Data acquisition and revised the manuscript for intellectual content
Giovanni Baranello, MD, PhD	Foundation IRCCS Neurological Institute Carlo Besta, Milan, Italy.	Site investigator	Data acquisition and revised the manuscript for intellectual content
Giuseppe Vita, MD	ERN Neuromuscular Unit, Department of	Site investigator	Data acquisition and revised the

	Clinical and Experimental Medicine, University of Messina, Messina, Italy		manuscript for intellectual content
Hugh J McMillan, MD, MSc	Children's Hospital of Eastern Ontario, University of Ottawa, Ottawa, Ontario, Canada.	Site investigator	Data acquisition and revised the manuscript for intellectual content
lain Horrocks, MRCPUK, MRCPCH	Greater Glasgow and Clyde NHS Yorkhill Hospital, UK.	Site investigator	Data acquisition and revised the manuscript for intellectual content
Imelda Hughes, MB, BCh	Royal Manchester Children's Hospital, Manchester, United Kingdom	Site investigator	Data acquisition and revised the manuscript for intellectual content
Janbernd Kirschner, MD.	University Medical Center, Freiburg, Germany.	Site investigator	Data acquisition and revised the manuscript for intellectual content
James F. Howard, Jr, MD	University of North Carolina at Chapel Hill School of Medicine, Chapel Hill, North Carolina	Site investigator	Data acquisition and revised the manuscript for intellectual content
Jean K. Mah, MD, MSc	University of Calgary, Calgary, Alberta, Canada.	Site investigator	Data acquisition and revised the manuscript for intellectual content
Jeffrey M. Statland, MD	University of Kansas Medical Center, Kansas City, Kansas	Site investigator	Data acquisition and revised the manuscript for intellectual content
Kevin M. Flanigan, MD.	Nationwide Children's Hospital, Columbus Ohio, USA.	Site investigator	Data acquisition and revised the manuscript for intellectual content
Leslie Morrison, MD	University of New Mexico HSC,	Site investigator	Data acquisition and revised the

	Albuquerque, New Mexico		manuscript for intellectual content
Lorenzo Maggi, MD	Foundation IRCCS Neurological Institute Carlo Besta, Milan, Italy.	Site investigator	Data acquisition and revised the manuscript for intellectual content
Luca Bello, MD, PhD	University of Padova, Padova, Italy.	Site investigator	Data acquisition and revised the manuscript for intellectual content
Mathula Thangarajh, MD,PhD	Children's National Health System, Washington, DC, USA.	Site investigator	Data acquisition and revised the manuscript for intellectual content
Maja von der Hagen, , MD	Abteilung Neuropädiatrie, Medizinische Fakultät Carl Gustav Carus, Technische Universität Dresden, Germany	Site investigator	Data acquisition and revised the manuscript for intellectual content
Matthew Wicklund, MD	Penn State Health Milton S Hershey Medical Center, Hershey, Pennsylvania	Site investigator	Data acquisition and revised the manuscript for intellectual content
Monika Krzesniak- Swinarska, MD	University of New Mexico HSC, Albuquerque, New Mexico	Site investigator	Data acquisition and revised the manuscript for intellectual content
Nancy Kuntz	Ann & Robert H Lurie Children's Hospital of Chicago, Chicago, Illinois	Site investigator	Data acquisition and revised the manuscript for intellectual content
Peter B. Kang, MD	Department of Neurology, Boston Children's Hospital and Harvard Medical School, Boston, MA	Site investigator	Data acquisition and revised the manuscript for intellectual content
	Paul and Sheila Wellstone Muscular Dystrophy Center and Department of Neurology, University		

	of Minnesota Medical School, Minneapolis, Minnesota		
Richard J. Barohn, MD.	University of Missouri, Columbia Missouri, USA.	Site investigator	Data acquisition and revised the manuscript for intellectual content
Richard S. Finkel, MD.	St. Jude Children's Research Hospital, USA.	Site investigator	Data acquisition and revised the manuscript for intellectual content
Russell Butterfield, MD, PhD	Department of Pediatric Neurology, University of Utah, Salt Lake City, Utah	Site investigator	Data acquisition and revised the manuscript for intellectual content
Shieh Perry, MD, PhD	David Geffen School of Medicine, University of California at Los Angeles, Los Angeles, California	Site investigator	Data acquisition and revised the manuscript for intellectual content
Stefan Spinty, MMedSc	Alder Hey Children's Hospital, UK.	Site investigator	Data acquisition and revised the manuscript for intellectual content
Tiziana E. Mongini	Neuromuscular Center, AOU Città della Salute e della Scienza, University of Turin, Turin, Italy	Site investigator	Data acquisition and revised the manuscript for intellectual content
Taeun Chang, MD	Children's National Hospital, George Washington University, Washington, DC	Site investigator	Data acquisition and revised the manuscript for intellectual content
Ulrike Schara- Schmidt, MD.	University Clinic Essen, University of Duisburg-Essen, Germany.	Site investigator	Data acquisition and revised the manuscript for intellectual content
Volker Straub, MD, PhD.	John Walton Muscular Dystrophy Research Centre, Clinical and	Site investigator	Data acquisition and revised the

	translational Research Institute, Newcastle University and Newcastle Hospitals NHS Foundation Trusts, Newcastle, UK.		manuscript for intellectual content
William Burnette, MD, PhD	Vanderbilt Children's Hospital Nashville, Tennesse, US.	Site investigator	Data acquisition and revised the manuscript for intellectual content
Zoya Alhaswani, MD	University Hospitals Birmingham NHS Foundation Trust, Birmingham, United Kingdom	Site investigator	Data acquisition and revised the manuscript for intellectual content