Supplementary Information

Safety and efficacy of chronic weekly rozanolixizumab in generalized myasthenia gravis: The randomized open-label extension MG0004 study

Vera Bril[1], Artur Drużdż[2], Julian Grosskreutz[3], Ali A. Habib[4], Henry J. Kaminski[5], Renato Mantegazza[6], Sabrina Sacconi[7], Kimiaki Utsugisawa[8], Tuan Vu[9], Marion Boehnlein[10], Maryam Gayfieva[11], Bernhard Greve[10], Franz Woltering[10], John Vissing[12] on behalf of the MG0004 study investigators

[1]Ellen and Martin Prosserman Centre for Neuromuscular Diseases, Toronto General Hospital, University of Toronto, Toronto, Ontario, Canada; [2]Department of Neurology, Municipal Hospital, Poznań, Poland; [3]Precision Neurology of Neuromuscular Diseases, Department of Neurology, University of Lübeck, Lübeck, Germany; [4]MDA ALS & Neuromuscular Center, Department of Neurology, University of California, Irvine, Orange, CA, USA; [5]Department of Neurology & Rehabilitation Medicine, George Washington University, Washington, DC, USA; [6]Emeritus and Past Director, Department of Neuroimmunology and Neuromuscular Diseases, Fondazione IRCCS, Istituto Nazionale Neurologico Carlo Besta, Milan, Italy; [7]Université Côte d'Azur, Peripheral Nervous System & Muscle Department, Pasteur 2 Hospital, Centre Hospitalier Universitaire de Nice, Nice, France; [8]Department of Neurology, Hanamaki General Hospital, Hanamaki, Japan; [9]Department of Neurology, University of South Florida Morsani College of Medicine, Tampa, FL, USA; [10]UCB, Monheim, Germany; [11]UCB, Slough, UK; [12]Copenhagen Neuromuscular Center, Department of Neurology, Rigshospitalet, University of Copenhagen, Copenhagen, Denmark

Journal: Journal of Neurology

Corresponding author: Dr Vera Bril

Email: vera.bril@utoronto.ca

Table of Contents

Supplementary Information

Online Resource 1 Plain language summary

Generalized myasthenia gravis (gMG) is a neuromuscular disease that causes unpredictable muscle weakness which may require lifelong treatment. A Phase 3 study called MycarinG (NCT03971422) showed that one 6-week cycle of once-weekly rozanolixizumab treatment improved disease symptoms compared with placebo in adult patients with gMG. After completing the MycarinG study, patients could enroll in the open-label extension study MG0004 (NCT04124965), which assessed the safety and efficacy of long-term weekly rozanolixizumab treatment. Patients received once-weekly rozanolixizumab 7 mg/kg or 10 mg/kg for up to 52 weeks, followed by an 8-week observation period.

In the MG0004 study, 70 patients received rozanolixizumab 7 mg/kg (n=35) or 10 mg/kg (n=35). Mean treatment duration was 22.9 and 23.7 weeks, respectively; study participation was low after Week 33 because patients had the option to enroll in a different open-label extension study to receive symptom-driven cycles of rozanolixizumab instead of continuous weekly treatment. In MG0004, side effects were reported in 85.7% of patients. Most side effects were mild or moderate. The most common side effects were headache (35.7% of patients), diarrhea (18.6% of patients) and decreased blood immunoglobulin G (15.7% of patients). There were no reports of any opportunistic infections, serious or severe infections, serious or severe hypersensitivity reactions, serious or severe injection-site reactions, any anaphylactic reactions or any side effects related to albumin or lipid abnormalities. Improvements from baseline in the Myasthenia Gravis Activities of Daily Living score were observed; the largest mean reduction from baseline was 3.1 in the 7 mg/kg group at Week 13 and 4.1 in the 10 mg/kg group at Week 21.

In this study, treatment with up to 52 weekly doses of rozanolixizumab was generally well tolerated and improvements across disease-specific outcomes were maintained, supporting the long-term use of rozanolixizumab for the treatment of patients with gMG.

Online Resource 2 Patient flow from MycarinG through MG0004 and MG0007
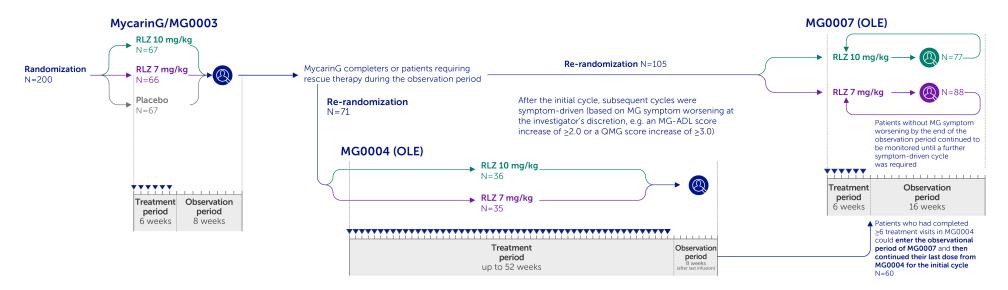

Figure adapted with permission from Bril V, Drużdż A, Grosskreutz J, Habib AA, Mantegazza R, Sacconi S, Utsugisawa K, et al (2025) Rozanolixizumab in generalized myasthenia gravis: Pooled analysis of the Phase 3 MycarinG study and two open-label extensions. J Neuromuscul Dis :22143602241305511. https://doi.org/10.1177/22143602241305511. MG, myasthenia gravis; MG-ADL, Myasthenia Gravis Activities of Daily Living; OLE, open-label extension; QMG, Quantitative Myasthenia Gravis; RLZ, rozanolixizumab.

Online Resource 3 MG0004 co-investigators and contributors

Name	Location	Role	Contribution
Rodrigo Álvarez-Velasco	Hospital de la Santa Creu i Sant Pau, Barcelona, Spain	Principal Investigator	Supported with the acquisition of data
Radwa Aly	Department of Neurology & Rehabilitation Medicine, George Washington University, Washington, DC, USA	Study Coordinator	Supported with the acquisition of data
Henning Andersen	Department of Neurology, Aarhus University Hospital, Aarhus, Denmark	Principal Investigator	Supported with the acquisition of data
Giovanni Antonini	Sapienza University of Rome, Department of Neurosciences, Mental Health and Sensory Organs (NESMOS), Italy	Principal Investigator	Supported with the acquisition of data
Aramide Balogun	Houston Methodist Hospital, Houston, TX, USA	Study Coordinator	Supported with the acquisition of data
Ruggero Barnabei	Fondazione Mondino Istituto Neurologico Nazionale a Carattere Scientifico – IRCCS, Pavia, Italy	Sub Investigator	Supported with the acquisition of data
Said Beydoun	University of Southern California Keck School of Medicine, Los Angeles, CA, USA	Principal Investigator	Supported with the acquisition of data
Franz Blaes	Klinik für Neurologie, Klinikum Oberberg, Gummersbach, Germany	Principal Investigator	Supported with the acquisition of data
Silvia Bonarino	Fondazione Istituto di Ricovero e Cura a Carattere Scientifico, Istituto Neurologico Carlo Besta, Italy	Sub Investigator	Supported with the acquisition of data
Anna Boss Soevang	Rigshospitalet, Denmark	Study Coordinator	Supported with the acquisition of data
Nazibrola Botchorishvili	LTD Simon Khechinashvili University Hospital, Georgia	Sub Investigator	Supported with the acquisition of data
Stephan A. Botez	Centre Hospitalier de l'Université de Montréal, Montreal, QC, Canada	Principal Investigator	Supported with the acquisition of data
Ivo Bozovic	Neurology Clinic, University Clinical Center of Serbia, Faculty of Medicine, University of Belgrade, Belgrade, Serbia	Sub Investigator	Supported with the acquisition of data
Paulina Budzinska	Miejskie Centrum Medyczne JONSCHER im. dr Karola Jonschera w Łodzi, Lodz, Poland	Sub Investigator	Supported with the acquisition of data
Pietro Businaro	Fondazione Mondino Istituto Neurologico Nazionale a Carattere Scientifico – IRCCS, Pavia, Italy	Sub Investigator	Supported with the acquisition of data

Lucia Campetella	PU A. Gemelli, Università Cattolica del Sacro Cuore, Italy	Sub Investigator	Supported with the acquisition of data
Ana Belen Cánovas	Hospital Universitari Vall D Hebron, Spain	Sub Investigator	Supported with the acquisition of data
Carlos Casasnovas	Hospital Universitari de Bellvitge-IDIBELL and CIBERER, Barcelona, Spain	Principal Investigator	Supported with the acquisition of data
Hou-Chang Chiu	Department of Neurology, Shin Kong Wu Ho-Su Memorial Hospital, Taipei, Taiwan	Sub Investigator	Supported with the acquisition of data
His-Chieh Chou	Taipei Veterans General Hospital, Taipei, Taiwan	Study Coordinator	Supported with the acquisition of data
Adam Comer	Indiana University Health Neuroscience Center, Indianapolis, IN, USA	Sub Investigator	Supported with the acquisition of data
Elena Cortés Vicente	Hospital de la Santa Creu i Sant Pau, Barcelona, Spain	Principal Investigator	Supported with the acquisition of data
Roberto D'Angelo	IRCCS Istituto delle Scienze Neurologiche di Bologna, Bologna, Italy	Sub Investigator	Supported with the acquisition of data
Lubna Daniyal	University Health Network, Toronto, ON, Canada	Study Coordinator	Supported with the acquisition of data
Annie Dionne	CHU de Québec – Université Laval, Hôpital Enfant-Jésus, Québec, QC, Canada	Principal Investigator	Supported with the acquisition of data
Péter Diószeghy	Szabolcs-Szatmár-Bereg County Hospitals and University Teaching Hospital, Nyiregyháza, Hungary	Principal Investigator	Supported with the acquisition of data
Laura Fionda	AOU Sant'Andrea Roma, Italy	Sub Investigator	Supported with the acquisition of data
Denis Flemm	UCB, Monheim, Germany	Operational study conduct	Supported with the acquisition of data
Rita Frangiamore	Fondazione Istituto di Ricovero e Cura a Carattere Scientifico, Istituto Neurologico Carlo Besta, Italy	Sub Investigator	Supported with the acquisition of data
Manuela Gambella	Université Côte d'Azur, Peripheral Nervous System and Muscle Department, Pasteur 2 Hospital, Centre Hospitalier Universitaire de Nice, Nice, France	Study Coordinator	Supported with the acquisition of data
Rachana K. Gandhi Mehta	Atrium Health Wake Forest Baptist, Winston-Salem, NC, USA	Principal Investigator	Supported with the acquisition of data
Matteo Garibaldi	AO S. Andrea, Università degli Studi di Roma La Sapienza, Italy	Sub Investigator	Supported with the acquisition of data

Matteo Gastaldi	Fondazione Mondino Istituto Neurologico Nazionale a Carattere Scientifico – IRCCS, Pavia, Italy	Principal Investigator	Supported with the acquisition of data
Christian Geis	Klinik für Neurologie, Universtitätsklinikum Jena, Jena, Germany	Principal Investigator	Supported with the acquisition of data
Hannah George	University of California, San Francisco Medical Center, San Francisco, CA, USA	Study Coordinator	Supported with the acquisition of data
Stefan Gingele	Medizinische Hochschule Hannover, Germany	Sub Investigator	Supported with the acquisition of data
Monica Grau Martin	Hospital Universitario de Bellvitge, Spain	Study Coordinator	Supported with the acquisition of data
Yuh-Cherng Guo	China Medical University Hospital, Taichung, Taiwan	Principal Investigator	Supported with the acquisition of data
Gerardo Gutiérrez Gutiérrez	Neurology Department, Hospital Universitario Infanta Sofía. Universidad Europea de Madrid. Department. of Medicine, Faculty of Biomedical and Health Sciences. Madrid, Spain	Principal Investigator	Supported with the acquisition of data
Francesco Habetswallner	Clinical Neurophysiology Unit, Cardarelli Hospital, Naples, Italy	Principal Investigator	Supported with the acquisition of data
Lina Hassoun	Klinik für Neurologie, Universitätsmedizin Göttingen, Göttingen, Germany	Sub Investigator	Supported with the acquisition of data
Sonja Holm-Yildiz	Copenhagen Neuromuscular Center, Department of Neurology, Rigshospitalet, University of Copenhagen, Copenhagen, Denmark	Sub Investigator	Supported with the acquisition of data
Faraz Hussain	University of Alberta, Edmonton, AB, Canada	Study Coordinator	Supported with the acquisition of data
Francisca Iniesta	Multiple Sclerosis and Clinical Neuroimmunology Unit, Neurology Department, Hospital Clínico Universitario Virgen de la Arrixaca, Murcia, Spain	Study Coordinator	Supported with the acquisition of data
Viktoriya Irodenko	University of California, San Francisco Medical Center, San Francisco, CA, USA	Sub Investigator	Supported with the acquisition of data
Marina Janelidze	Simon Khechinashvili University Hospital, Tbilisi, Georgia	Principal Investigator	Supported with the acquisition of data
Min Kang	University of California, San Francisco Medical Center, San Francisco, CA, USA	Sub Investigator	Supported with the acquisition of data
Chafic Karam	University of Pennsylvania, Philadelphia, PA, USA	Principal Investigator	Supported with the acquisition of data

Denis Korobko	State Novosibirsk Regional Clinical Hospital, Novosibirsk, Russia	Sub Investigator	Supported with the acquisition of data
Sergey Kotov	Vladimirsky Moscow Regional Research and Clinical Institute, Moscow, Russia	Principal Investigator	Supported with the acquisition of data
Michal Kretkowski	Clinical Research Center Sp. Z o.o., Medic-R Sp. K., Poland	Sub Investigator	Supported with the acquisition of data
Nana Kvirkvelia	LTD Petre Sarajishvili Institute of Neurology, Georgia	Sub Investigator	Supported with the acquisition of data
Antonio Lauletta	AO S. Andrea, Università degli Studi di Roma La Sapienza, Italy	Sub Investigator	Supported with the acquisition of data
Yi-Chung Lee	Taipei Veterans General Hospital, Taipei, Taiwan	Principal Investigator	Supported with the acquisition of data
Luca Leonardi	AOU Sant'Andrea Roma, Italy	Sub Investigator	Supported with the acquisition of data
Kore Liow	Hawaii Pacific Neuroscience, Honolulu, HI, USA	Principal Investigator	Supported with the acquisition of data
Arnau Llauradó Gayete	Hospital Universitari Vall D Hebron, Spain	Sub Investigator	Supported with the acquisition of data
Sara Llufriu	Servicio de Neurología ICN, Hospital Clinic de Barcelona, Universitat de Barcelona, Barcelona, Spain	Sub Investigator	Supported with the acquisition of data
Catherine Lomen-Hoerth	University of California, San Francisco Medical Center, San Francisco, CA, USA	Principal Investigator	Supported with the acquisition of data
Jan D. Lünemann	Klinik für Neurologie, Universitätsklinikum Münster, Münster, Germany	Sub Investigator	Supported with the acquisition of data
Lorenzo Maggi	Fondazione Istituto di Ricovero e Cura a Carattere Scientifico, Istituto Neurologico Carlo Besta, Italy	Sub Investigator	Supported with the acquisition of data
Eugenia Martínez Hernández	Servicio de Neurología ICN, Hospital Clinic de Barcelona, Universitat de Barcelona, Barcelona, Spain	Principal Investigator	Supported with the acquisition of data
Gianvito Masi	PU A. Gemelli, Università Cattolica del Sacro Cuore, Italy	Sub Investigator	Supported with the acquisition of data
Marion Masingue	Neurology Department, Pitié Salpêtrière-Charles Foix Hospital Group, AP-HP, Sorbonne University, Paris, France	Sub Investigator	Supported with the acquisition of data
Rami Massie	Clinical Research Unit, The Montreal Neurological Institute, Montreal, QC, Canada	Sub Investigator	Supported with the acquisition of data

Marco Masullo	Istituto delle Scienze Neurologiche, Italy	Sub Investigator	Supported with the acquisition of data
Federico Mazzacane	Fondazione Mondino Istituto Neurologico Nazionale a Carattere Scientifico – IRCCS, Pavia, Italy	Sub Investigator	Supported with the acquisition of data
Nora Möhn	Medizinische Hochschule Hannover, Germany	Sub Investigator	Supported with the acquisition of data
Stefania Morino	AOU Sant'Andrea Roma, Italy	Sub Investigator	Supported with the acquisition of data
Kelsey Moulton	University of Pennsylvania, Philadelphia, PA, USA	Study Coordinator	Supported with the acquisition of data
Tahseen Mozaffar	MDA ALS and Neuromuscular Center, University of California, Irvine, CA, USA	Sub Investigator	Supported with the acquisition of data
Elene Nebadze	LTD Petre Sarajishvili Institute of Neurology, Georgia	Sub Investigator	Supported with the acquisition of data
Velina Nedkova-Hristova	Hospital Universitari de Bellvitge-IDIBELL and CIBERER, Barcelona, Spain	Sub Investigator	Supported with the acquisition of data
Eduardo Ng	University Health Network, Toronto, ON, Canada	Study Coordinator	Supported with the acquisition of data
Ekaterina Novikova	Vladimirsky Moscow Regional Research and Clinical Institute, Moscow, Russia	Sub Investigator	Supported with the acquisition of data
Izabella Obál	Department of Neurology, Aalborg University Hospital, Aalborg, Denmark	Principal Investigator	Supported with the acquisition of data
Anita Palsgård	Aalborg Sygehus Nord, Denmark	Study Coordinator	Supported with the acquisition of data
Claudia Papi	PU A. Gemelli, Università Cattolica del Sacro Cuore, Italy	Sub Investigator	Supported with the acquisition of data
Lorena Pérez	Hospital Universitario Infanta Sofía, Spain	Study Coordinator	Supported with the acquisition of data
Stojan Peric	Neurology Clinic, University Clinical Center of Serbia, Faculty of Medicine, University of Belgrade, Belgrade, Serbia	Principal Investigator	Supported with the acquisition of data
Mikhail Petrov	St. Petersburg Regional Clinical Hospital, Russia	Sub Investigator	Supported with the acquisition of data
Nicolai Rasmus Preisler	Rigshospitalet, Denmark	Sub Investigator	Supported with the acquisition of data

Giorgia Querin	Neurology Department, Pitié Salpêtrière-Charles Foix Hospital Group, AP-HP, Sorbonne University, Paris, France	Sub Investigator	Supported with the acquisition of data
Konrad Rejdak	Department of Neurology, Medical University of Lublin, Lublin, Poland	Principal Investigator	Supported with the acquisition of data
Kourosh Rezania	University of Chicago, Chicago, IL, USA	Principal Investigator	Supported with the acquisition of data
Elena Rinaldi	Fondazione Istituto di Ricovero e Cura a Carattere Scientifico, Istituto Neurologico Carlo Besta, Italy	Study Coordinator	Supported with the acquisition of data
Rita Rinaldi	IRCCS Istituto delle Scienze Neurologiche di Bologna, Bologna, Italy	Principal Investigator	Supported with the acquisition of data
Michael H. Rivner	Department of Neurology, Augusta University, Augusta, GA, USA	Principal Investigator	Supported with the acquisition of data
Annekathrin Roediger	Klinik für Neurologie, Universtitätsklinikum Jena, Jena, Germany	Sub Investigator	Supported with the acquisition of data
Laura Rosow	University of California, San Francisco Medical Center, San Francisco, CA, USA	Sub Investigator	Supported with the acquisition of data
Simone Rossi	IRCCS Istituto delle Scienze Neurologiche di Bologna, Bologna, Italy	Sub Investigator	Supported with the acquisition of data
Elena Rossini	AOU Sant'Andrea Roma, Italy	Sub Investigator	Supported with the acquisition of data
Stephen Ryan	University of Kentucky, Lexington, KY, USA	Sub Investigator	Supported with the acquisition of data
Lotte Sahin Levison	Aarhus Universitetshospital, Denmark	Sub Investigator	Supported with the acquisition of data
Albert Saiz	Servicio de Neurología ICN, Hospital Clinic de Barcelona, Universitat de Barcelona, Barcelona, Spain	Principal Investigator	Supported with the acquisition of data
Maria Salvado	Vall d'Hebron University Hospital, Passeig de la Vall d'Hebron, Barcelona, Spain	Sub Investigator	Supported with the acquisition of data
Daniel Sánchez-Tejerina	Hospital Universitari Vall D Hebron, Spain	Sub Investigator	Supported with the acquisition of data
Margret Schwarz	Klinik für Neurologie, Universitätsmedizin Göttingen, Göttingen, Germany	Study Coordinator	Supported with the acquisition of data
María Sepúlveda	Servicio de Neurología ICN, Hospital Clinic de Barcelona, Universitat de Barcelona, Barcelona, Spain	Sub Investigator	Supported with the acquisition of data

Khema R. Sharma	University of Miami Miller School of Medicine, Miami, FL, USA	Principal Investigator	Supported with the acquisition of data
Sheetal Shroff	Houston Methodist Hospital, Houston, TX, USA	Principal Investigator	Supported with the acquisition of data
Olga Sidorova	Vladimirsky Moscow Regional Research and Clinical Institute, Moscow, Russia	Sub Investigator	Supported with the acquisition of data
Guilhem Solé	Neuromuscular Reference Center AOC, Nerve-Muscle Unit, Bordeaux University Hospitals, Bordeaux, France	Principal Investigator	Supported with the acquisition of data
Javier Sotoca	Vall d'Hebron University Hospital, Passeig de la Vall d'Hebron, Barcelona, Spain	Sub Investigator	Supported with the acquisition of data
Mads Stemmerik	Copenhagen Neuromuscular Center, Department of Neurology, Rigshospitalet, University of Copenhagen, Copenhagen, Denmark	Sub Investigator	Supported with the acquisition of data
Aleksandar Stojanov	Clinic for Neurology, Clinical Centre Nis, Nis, Serbia	Sub Investigator	Supported with the acquisition of data
Tanya Stojkovic	Neurology Department, Pitié Salpêtrière-Charles Foix Hospital Group, AP-HP, Sorbonne University, Paris, France	Sub Investigator	Supported with the acquisition of data
Kai Su	University of Kentucky, Lexington, KY, USA	Study Coordinator	Supported with the acquisition of data
Sebastian Szklener	Department of Neurology, Medical University of Lublin, Lublin, Poland	Sub Investigator	Supported with the acquisition of data
Alexander Tsiskaridze	Pineo Medical Ecosystem, Tbilisi, Georgia	Principal Investigator	Supported with the acquisition of data
Laura Tufano	AOU Sant'Andrea Roma, Italy	Sub Investigator	Supported with the acquisition of data
Michaela Tyblova	Department of Neurology and Center of Clinical Neuroscience, First Faculty of Medicine Charles University and General Hospital, Prague, Czech Republic	Principal Investigator	Supported with the acquisition of data
Eiko Uenaka	Department of Neurology, Osaka University Hospital, Osaka, Japan	Study Coordinator	Supported with the acquisition of data
Astrid Unterlauft	Klinik und Poliklinik für Neurologie, Universitätsklinikum Leipzig A.ö.R, Leipzig, Germany	Sub Investigator	Supported with the acquisition of data
Gabriel Valero	Multiple Sclerosis and Clinical Neuroimmunology Unit, Neurology Department, Hospital Clínico Universitario Virgen de la Arrixaca, Murcia, Spain	Sub Investigator	Supported with the acquisition of data
Fiammetta Vanoli	Fondazione Istituto di Ricovero e Cura a Carattere Scientifico, Istituto Neurologico Carlo Besta, Italy	Sub Investigator	Supported with the acquisition of data

Tamar Vashadze	Pineo Medical Ecosystem, Tbilisi, Georgia	Sub Investigator	Supported with the acquisition of data
Nuria Vidal Fernández	Hospital de la Santa Creu i Sant Pau, Barcelona, Spain	Study Coordinator	Supported with the acquisition of data
Marie-Hélène Violleau	Neuromuscular Reference Center AOC, Nerve-Muscle Unit, Bordeaux University Hospitals, Bordeaux, France	Study Coordinator	Supported with the acquisition of data
Nicolas Weiss	Neurology Department, Pitié Salpêtrière-Charles Foix Hospital Group, AP-HP, Sorbonne University, Paris, France	Sub Investigator	Supported with the acquisition of data
Nanna Witting	Copenhagen Neuromuscular Center, Department of Neurology, Rigshospitalet, University of Copenhagen, Copenhagen, Denmark	Sub Investigator	Supported with the acquisition of data
Jiann-Horng Yeh	Department of Neurology, Shin Kong Wu Ho-Su Memorial Hospital, Taipei, Taiwan	Principal Investigator	Supported with the acquisition of data
Leila Zaidi	Centre de Référence des Maladies Neuromusculaires NEIdF, Département de Neurologie, Hôpital de Hautepierre, Centre Hospitalier Universitaire de Strasbourg and ERO-NMD Strasbourg, Strasbourg, France	Study Coordinator	Supported with the acquisition of data
Leonid Zaslavskiy	St. Petersburg Regional Clinical Hospital, Russia	Principal Investigator	Supported with the acquisition of data
Jana Zschüntzsch	Klinik für Neurologie, Universitätsmedizin Göttingen, Göttingen, Germany	Principal Investigator	Supported with the acquisition of data