

Appendix for MGTX Extension Study	Page
Table A1: Demographic and clinical characteristics of MGTX subjects	2
Acknowledgements	3
MGTX Study Group	4

Table A1. Demographic and clinical characteristics of MGTX subjects

Characteristic	Did not enter extension study (N=58)	Entered extension study (N=68)	P Value [#]
Gender – Count (%)			
Female	38 (66)	51 (75)	0.24
Ethnicity – Count (%)			
Asian	2 (3)	8 (12)	0.0003
Black/African American	8 (14)	5 (7)	
Hispanic	7 (12)	27 (40)	
White, not Hispanic origin	38 (66)	23 (34)	
Other (Mixed/Native American/Alaskan)	3 (5)	5 (7)	
Therapy at enrollment* – Count (%)			
Pyridostigmine	51 (88)	65 (96)	0.18
Corticosteroids	46 (85), (N=54)	50 (74)	0.12
Prior intravenous immunoglobulin	16 (30) , (N=54)	9 (13)	0.0259
Prior plasma exchange	7 (13) , (N=54)	9 (13)	0.96
MG Foundation of America Class at enrollment[†] – Count (%)			
Class IIa	26 (45)	24 (35)	0.34
Class IIb	15 (26)	17 (25)	
Class III	17 (29)	24 (35)	
Class IV	0 (0)	3 (4)	
Median age in years at enrollment (range)	32.5 (18.0-64.0)	32.0 (18.0-63.0)	0.80
Median disease duration in years at enrollment (range)	1.09 (0.02-4.41)	1.11 (0.15-3.97)	0.40
Baseline measures*			
Quantitative MG score: Mean ± SD	10.81 ± 5.08 (N=54)	12.66 ± 4.85 (N=68)	0.0430
Alternate day prednisone dosage, mg: Mean ± SD	57.0 ± 33.7 (N=49)	47.3 ± 31.5 (N=68)	0.11
MG Activity of Daily Living score: Mean ± SD	5.24 ± 3.48 (N=54)	5.43 ± 3.22 (N=68)	0.76
Month 36 measures			
Quantitative MG score: Mean ± SD	5.71 ± 4.93 (N=42)	7.18 ± 5.75 (N=67)	0.17
Alternate day prednisone dosage, mg: Mean ± SD	20.0 ± 28.6 (N=41)	28.3 ± 33.0 (N=67)	0.19
MG Activity of Daily Living score: Mean ± SD	3.00 ± 3.14 (N=42)	1.91 ± 2.52 (N=67)	0.0483
Treatment-Associated Complications: Mean no. ± SD	1.02 ± 1.32 (N=42)	0.36 ± 0.57 (N=67)	0.0031
Treatment-Associated Symptoms: Mean no. ± SD	8.57 ± 6.44 (N=42)	6.79 ± 5.71 (N=67)	0.13

*Four subjects dropped out without completing baseline therapy at enrollment and primary outcome information.

[†]Myasthenia Gravis Foundation of America Class (II mild weakness, III moderate weakness, IV severe weakness, (“a” denotes predominantly limb/axial, “b” denotes predominantly bulbar).

[#]Chi-square test or Fisher’s exact test if applicable for categorical outcome, 2 sample t-test for continuous outcome

Acknowledgements

A special thanks is extended to Audrey Penn, M.D., of the NINDS, Johan Aarli, M.D., Robert Griggs, M.D., Robert Lisak, M.D., and Lewis Rowland, M.D., who were strong advocates for the trial in its early stages. The investigators would also like to thank the patients who participated in this trial and were willing to be randomized between a surgical and non-surgical arm and comply with a study protocol lasting years.

MGTX Study Executive Committee

Data Coordinating Center: Gary R. Cutter, Inmaculada B. Aban, Greg Minisman, Michelle Feese, Hui-Chien Kuo (Dept. of Biostatistics, University of Alabama at Birmingham, Birmingham, AL).

Trial Leadership: John Newsom-Davis (Department of Neurology, Oxford University, Oxford, U.K.); Gil I. Wolfe (Department of Neurology, University at Buffalo Jacobs School of Medicine and Biomedical Sciences); Henry J. Kaminski (Department of Neurology, George Washington University School of Medicine and Health Sciences); Alfred Jaretzki III, Joshua R. Sonett (Section of General Thoracic Surgery, Columbia University Medical Center, New York, NY).

MGTX Study Group

Argentina: Claudio Mazia, Valeria Saluto, Moises Rosenberg, Valeria Alvarez, Lisa Rey (University of Buenos Aires, Buenos Aires).

Australia: John King, Helmut Butzkueven, John Goldblatt, John Carey (University of Melbourne, Melbourne); John Pollard, Stephen Reddel, Nicholas Handel, Brian McCaughan, Linda Pallot (University of Sydney, Sydney).

Brazil: Márcia Waddington-Cruz, Ricardo Novis, Carlos Boasquevisque (Federal University of Rio De Janeiro, Rio De Janeiro); Elza Dias-Tosta, Rubens Morato-Fernandez, Manoel Ximenes (Hospital de Base do Distrito Federal, Brasília); Lineu Werneck, Rosana Scola, Paulo Soltoski (Universidade Federal do Paraná; Curitiba).

Canada: Colin Chalk, Fraser Moore, David Mulder, Lisa Wadup (McGill University, Montreal, QC); Joel Oger, Michele Mezei, Kenneth Evans, Theresa Jiwa, Anne Schaffar (University of British Columbia, Vancouver, BC); Chris White, Cory Toth, Gary Gelfand, Susan Wood (University of Calgary, Calgary, AB); Elizabeth Pringle, Jocelyn Zwicker, Donna Maziak, Farid Shamji, Sudhir Sundaresan, Andrew Seely (University of Ottawa, Ottawa, ON).

Chile: Gabriel Cea, Renato Verdugo, Alberto Aguayo (University of Chile, Santiago).

Germany: Sebastian Jander, Philipp Zickler, Michael Klein (University of Düsseldorf, Düsseldorf); Alexander Marx, Philipp Ströbel, Cleo-Aron Weis (University of Heidelberg, Mannheim); Arthur Melms, Felix Bischof, Hermann Aebert, Gerhard Ziemer (University of Tübingen, Tübingen); Wilfred Nix, Björn Thümmler, Thomas Wilhem-Schwenkmezger, Eckhard Mayer (Johannes-Gutenberg University, Mainz); Berthold Schalke, Peter Pöschel, Gisela Hieber (University of Regensburg, Regensburg); Karsten Wiebe (University of Münster, Münster).

Italy: Giovanni Antonini, Alessandro Clemenzi, Vanessa Ceschin, Erino Rendina, Federico Venuta, Stefania Morino, Elisabetta Bucci (University of Rome “Sapienza,” Rome); Luca Durelli, Alessia Tavella, Marinella Clerico, Giulia Contessa, Piero Borasio (University of Torino, Torino); Amelia Evoli, Serenella Servidei, Pierluigi Granone (Catholic University, Rome); Renato Mantegazza, Emilia Berta, Lorenzo Novellino, Luisa Spinelli (National Neurological Institute “Carlo Besta,” Milan).

Japan: Masakatsu Motomura, Hidenori Matsuo, Takeshi Nagayasu (Nagasaki University, Nagasaki); Hiroaki Yoshikawa, Masaharu Takamori, Makoto Oda, Isao Matsumoto, Yutaka Furukawa, Daisuke Noto, Yuko Motozaki, Kazuo Iwasa, Daisuke Yanase (Kanazawa University, Kanazawa).

Mexico: Guillermo Garcia Ramos, Bernardo Cacho, Lorenzo de la Garza (Instituto Nacional de la Nutrición, Tlalpan).

Poland: Anna Kostera-Pruszczyk, Marta Lipowska, Hubert Kwiecinski, Anna Potulska-Chromik (Medical University of Warsaw, Warsaw); Tadeusz Orłowski (Institute of Tuberculosis and Lung Disease, Warsaw).

Portugal: Ana Silva, Marta Feijo, António Freitas (Porto University, Porto).

South Africa: Jeannine Heckmann, Andrew Frost, Edward Lee Pan, Lawrence Tucker, Johan Rossouw, Fiona Drummond (University of Cape Town, Cape Town).

Spain: Isabel Illa, Jorge Diaz, Carlos Leon (H. Sant Pau, Universitat Autònoma de Barcelona, Barcelona).

Taiwan: Jiann-Horng Yeh, Hou-Chang Chiu, Yei-San Hsieh (Fu-Jen Catholic University, Taipei).

Thailand: Rawiphan Witoonpanich, Supoch Tunlayadechanont, Sukasom Attanavanich (Ramathibodi Hospital, Mahidol University, Bangkok).

The Netherlands: Jan Verschueren, Chiara Straathof, Maarten Titulaer, Michel Versteegh, Arda Pels, Yvonne Krum (Leiden University, Leiden).

United Kingdom: Camilla Buckley, M. Isabel Leite, Angela Vincent, David Hilton-Jones, Chandi Ratnatunga, John Newsom-Davis (University of Oxford, Oxford); Maria Elena Farrugia, Richard Petty, James Overell, Alan Kirk (Queen Elizabeth University Hospital, Glasgow); Andrew Gibson, Chris McDermott, David Hopkinson (University of Sheffield, Sheffield); Bryan Lecky, David Watling, Dot Marshall, Sam Saminaden, Deborah Davies, Charlotte Dougan, Siva Sathasivam, Richard Page, Dot Marshall (Walton Centre for Neurology and Neurosurgery, Liverpool Heart and Chest Hospital, Liverpool); Jon Sussman, John Ealing, Peter Krysiak (University of Manchester, Manchester).

United States: Anthony Amato, Mohammad Salajegheh, Michael Jaklitsch, Kristen Roe (Brigham and Women's Hospital, Boston, MA); Tetsuo Ashizawa, Robert Glenn Smith, Joseph Zwischenberg, Penny Stanton (University of Texas Medical Branch, Galveston, TX); Alexandru Barboi, Safwan Jaradeh, William Tisol, Mario Gasparri, George Haasler, Mary Yellick, Cedric Dennis (Medical College of Wisconsin, Milwaukee, WI); Richard Barohn, Mamatha Pasnoor, Mazen Dimachkie, April McVey, Gary Gronseth, Arthur Dick, Jeffrey Kramer, Melissa Currence, Laura Herbelin (University of Kansas, Kansas City, KS); Jerry Belsh, Geoge Li, John Langenfeld, Mary Ann Mertz (Robert Wood Johnson University, New Brunswick, NJ); Michael Benatar (University of Miami, Miami, FL); Taylor Harrison, Seth Force, Sharon Usher (Emory University, Atlanta, GA); Said Beydoun, Frank Lin, Steve DeMeester, Salem Akhter, Ali Malekniazi, Gina Avenido (University of Southern California, Los Angeles, CA); Brian Crum, Margherita Milone, Stephen Cassivi, Janet Fisher (Mayo Clinic, Rochester, MN); Emma Ciafaloni, Chad Heatwole, Thomas Watson, James Hilbert, Alexis Smirnow (University of Rochester, Rochester, NY); B. Jane Distad, Michael Weiss, Douglas Wood, Joanna Haug (University of Washington, Seattle, WA); Raina Ernstoff, Jingyang Cao, Gary Chmielewski, Robert Welsh, Robin Duris (William Beaumont Hospital, Royal Oak, MI); Laurie Gutmann, Gauri Pawar, Geoffrey Marc Graeber, Patricia Altemus, Christopher Nance, Ludwig Gutmann (West Virginia University, Morgantown, WV); Carlayne Jackson, Patrick Grogan, John Calhoon, Pamela Kittrell, Deborah Myers (University of Texas Health Science Center, San Antonio, TX); Henry Kaminski, Ghazala Hayat, Keith Naunheim, Susan Eller, Eve Holzemer (St. Louis University, St. Louis, MO); Bashar Katirji, Amer Alshekhlee, Jason Robke, Brenda Karlinchak (Case Western Reserve University, Cleveland, OH); Jonathan Katz, Robert Miller, Ralph Roan, Dallas Forshaw (California Pacific Medical Center, San Francisco, CA); John Kissel, Bakri Elsheikh, Patrick Ross, Sharon Chelnick (The Ohio State University Wexner Medical Center, Columbus, OH); Richard Lewis, Agnes Acsadi, Frank Baciewicz, Stacey Massee (Wayne State University, Detroit, MI); Janice Massey, Vern Juel, Mark Onaitis, James Lowe, Bernadette Lipscomb (Duke University, Durham, NC); Tahseen Mozaffar, Gaby Thai, Jeffrey Milliken, Veronica Martin, Ronnie Karayan (University of California, Irvine, CA); Suraj Muley (Barrow Neurological Institute, Phoenix, AZ); Gareth Parry, Sara Shumway (University of Minnesota, Minneapolis, MN); Shin Oh, Gwen Claussen, Liang Lu, Robert Cerfolio, Angela Young, Marla Morgan (University of Alabama at Birmingham, Birmingham, AL); Robert Pascuzzi, John Kincaid, Kenneth Kesler, Sandy Guingrich, Angi Michaels (Indiana University, Indianapolis, IN); Lawrence Phillips, Ted Burns, David Jones, Cindy Fischer (University of Virginia, Charlottesville, VA); Michael Pulley, Alan Berger, Harry D'Agostino, Lisa Smith

(University of Florida, Jacksonville, FL); Michael Rivner, Jerry Pruitt, Kevin Landolfo, Demetric Hillman (Augusta University, Augusta, GA); Aziz Shaibani, Angelo Sermas, Ross Ruel, Farah Ismail (Nerve and Muscle Center of Texas, Houston, TX); Mark Sivak, Martin Goldstein, Jorge Camunas, Joan Bratton (Mount Sinai Hospital, New York, NY); Rup Tandan, Hill Panitch, Bruce Leavitt, Marilee Jones (University of Vermont, Burlington, VT); Gil Wolfe, Srikanth Muppidi, Steven Vernino, Sharon Nations, Dan Meyer, Nina Gorham (University of Texas Southwestern, Dallas TX).