A Pilot Study of Etanercept in Dermatomyositis

Primary Outcome Measures:
- Frequencies of adverse events and abnormal laboratory test results, changes from baseline in continuous laboratory values and vital signs, ability to complete the trial, and ability to complete the trial on the originally assigned [ Time Frame: over the one year study period ]
- Cumulative dosage of prednisone over the one year study period. [ Time Frame: over the one year study period ]

Secondary Outcome Measures:
- Average daily dose of prednisone (cumulative dose / # of days in the study) [ Time Frame: over the one year study period ] [ Designated as safety issue: Yes ]

Contact: Anthony A. Amato, MD  aamato@partners.org for nearest participating site

Rituximab for the Treatment of Refractory Adult and Juvenile Dermatomyositis (DM) and Adult Polymyositis (PM)

Primary Outcome Measures:
- The time to achieve improvement will be compared between the two groups of IIM patients. [ Time Frame: Week 44 of treatment phase ]
- Secondary Outcome Measures: Response rates (proportion of improved patients) between Groups A (rituximab-treated) and B (placebo-treated) [ Time Frame: Week 8 of the treatment phase ]
- 20% improvement in Manual Muscle Testing (MMT) over baseline on two consecutive time points (muscle is the primary organ of involvement, and MMT is the one objective measurement of the Definition of Improvement [DOI]) [ Time Frame: Week 44 of treatment phase ] [ Designated as safety issue: No ]

Contact: Diane C. Koontz  412-383-8674  dik4@pitt.edu

A Phase 1B, Randomized, Double-Blind, Placebo-Controlled, Multicenter Study to Evaluate Safety of Multiple-Dose, Intravenously Administered MEDI-545, A Fully Human Anti Interferon-Alpha Monoclonal Antibody, In Adult Patients With Dermatomyositis or Polymyositis

Primary Outcome Measures:
- The primary endpoints of the study are safety and tolerability of multiple intravenous (IV) doses of MEDI-545 in adult patients with Dermatomyositis or
Polymyositis, assessed primarily by summarizing AEs assessing changes in viral cultures and titers. [ Time Frame: 12 months ]

Secondary Outcome Measures:
- The secondary endpoints of the study are the PK and IM of multiple IV doses of MEDI-545. [ Time Frame: 12 months ]
- The third endpoint of the study are the evaluations of disease activities. [ Time Frame: 12 months ]

For nearest participating sites contact:
Mick G. Ribeiro 301-398-4202 ribeirom@medimmune.com
Lisa Farace 301-398-4991 faracel@medimmune.com

Safety and Tolerability Trial of Arimoclomol for Sporadic Inclusion Body Myositis

Primary Outcome Measures:
- Adverse event reporting [ Time Frame: Every 2 weeks for 4 months ]

Secondary Outcome Measures:
- Muscle Strength Testing [ Time Frame: Monthly for 4 months ]
- IBM functional rating scale [ Time Frame: Monthly for 4 months ]
- Muscle biopsy [ Time Frame: pre and post treatment ]

This pilot study is only being done at Univ of Kansas (PI: Richard Barohn)

Testosterone Replacement for Older Men With Sarcopenia

Primary Outcome Measures:
- Changes in physical performance measured by an exercise testing regimen [ Time Frame: baseline and 6 months ]

Secondary Outcome Measures:
- Changes in disability, fatigue, affect, and sense of well being assessed by validated questionnaires [ Time Frame: baseline, 3 months, and 6 months ]

Contact: Lindsay Cloutier Lindsay.Cloutier@bmc.org

Phase II Therapeutic Trial of Mexiletine in Non-Dystrophic Myotonia
Primary Outcome Measures:
  • patient assessed stiffness [ Time Frame: weeks 2,3,7,8 ]

Secondary Outcome Measures:
  • patient assessed pain, weakness, fatigue, clinical myotonia assessment, quality of life, grip myotonia, CMAP [ Time Frame: week 4,5,9 ]

Contact: Richard Barohn, M.D. (rbarohn@kumc.edu) for closest participating sites

**Dichlorphenamide vs. Acetazolamide for Periodic Paralysis**

The trial consists of two 9-week studies—one study will enroll persons with hyperkalemic periodic paralysis and the other study will enroll persons with hypokalemic periodic paralysis. Participants will be randomly assigned to one of three treatment groups: acetazolamide, dichlorphenamide, or placebo (an inactive substance). During the studies, participants will be asked to keep a daily computer diary to record the time, length, and severity of each episode of weakness. The study coordinator will contact participants weekly to review the diary information.

The 9-week studies will be followed by 1-year extensions to compare the long-term effects of acetazolamide and dichlorphenamide on the course of periodic paralysis. Participants who initially received a placebo during the 9-week studies will be randomly assigned to receive either acetazolamide or dichlorphenamide during the extension studies.

Duration of the trial for participants is a maximum of 61 weeks, including the first 9-week treatment phase and the one-year extension phase.

Primary Outcome Measures:
  • The number of attacks/week

Secondary Outcome Measures:
  • Efficacy: severity-weighted attack rate; muscle strength and mass measures; intolerable increase in attack frequency or severity necessitating withdrawal from the treatment period.

Contact: Patty Smith  585-275-4339 for nearest participating site

**A Phase 2b Extension Study of Ataluren (PTC124) in Subjects With Nonsense-Mutation Mediated Duchenne and Becker Muscular Dystrophy**

Primary Outcome Measures:
Long-term safety of PTC124 in boys with nonsense-mutation mediated DMD/BMD, as determined by adverse events and laboratory abnormalities [ Time Frame: 2 years ]

Contact: Diane Goetz 908 912 9256 dgoetz@ptcbio.com

A Phase 2a Extension Study of PTC124 in Subjects With Nonsense-Mutation-Mediated Duchenne Muscular Dystrophy:

Primary Outcome Measures:
  • Long-term safety [ Time Frame: 2 years ]

Contact: Diane Goetz 908 912 9256 dgoetz@ptcbio.com