

A stylized world map in a golden-brown color is set against a dark blue background at the top of the slide. The map shows the continents and is centered horizontally.

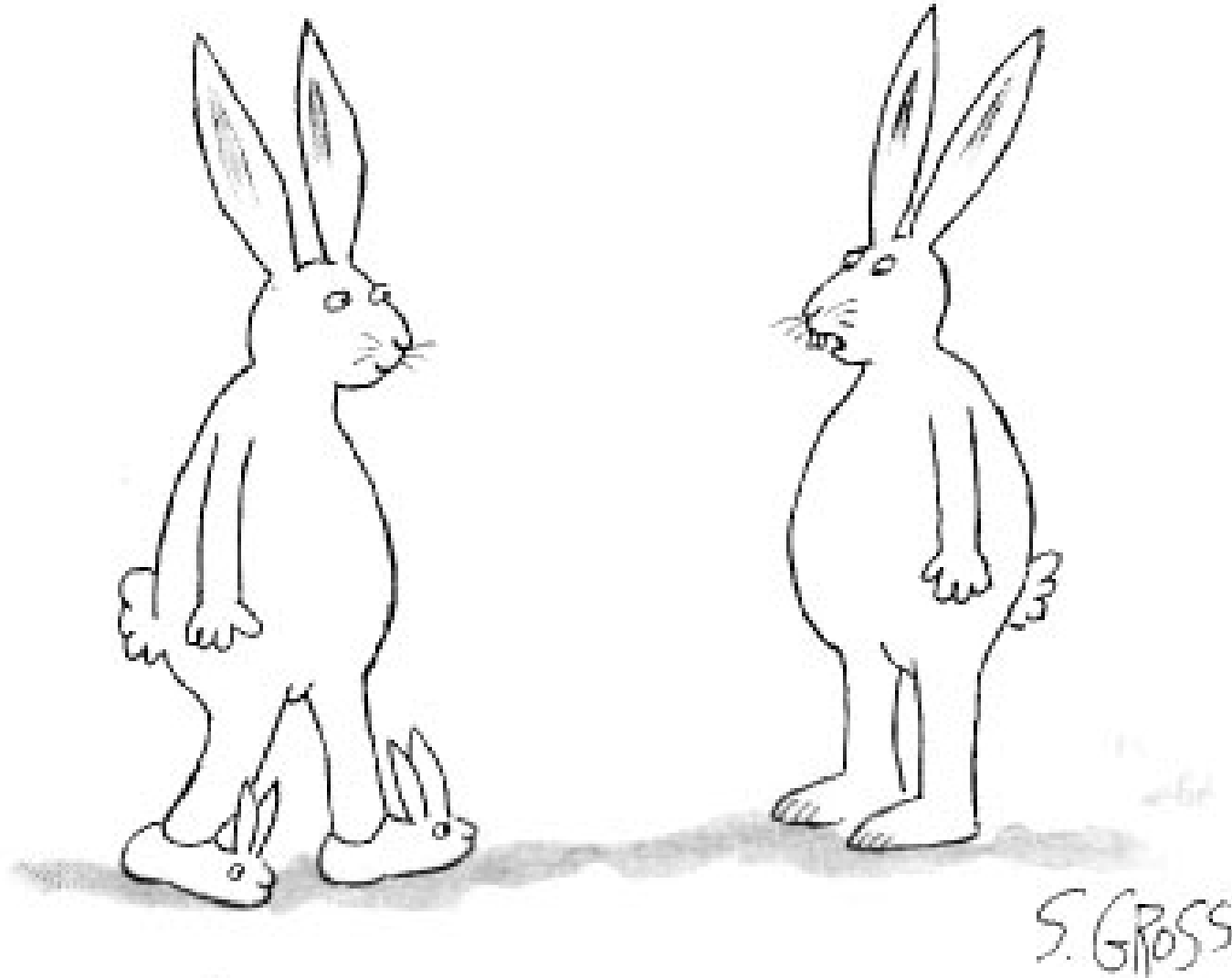
Muscle Study Group

**MSG Meeting  
Update on HYP-Hop**

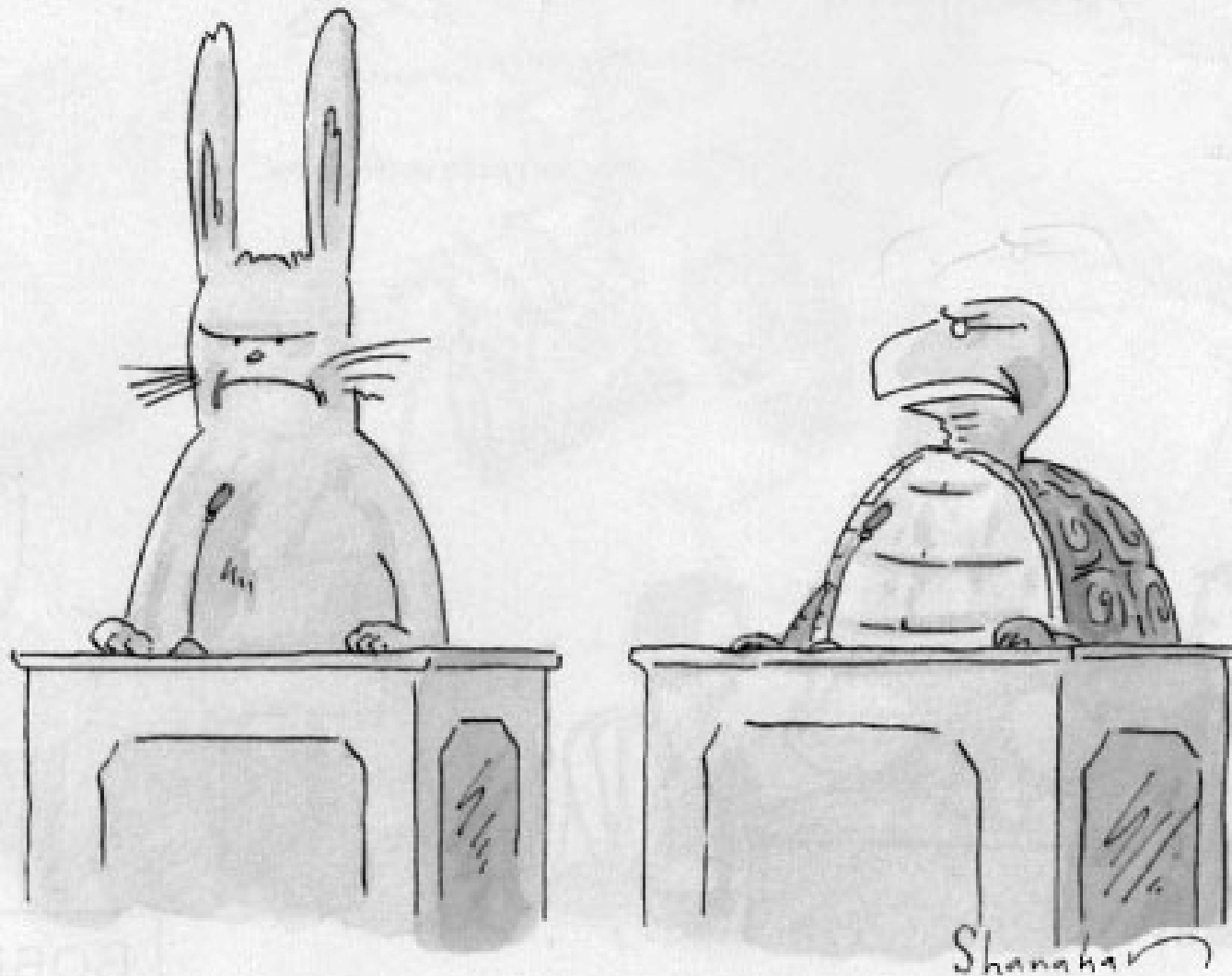
Robert C. Griggs, M.D.

Beaver Hollow Conference Center

September 2009



**“You are one sick rabbit.”**



**“And now my opponent accuses me of playing the race card.”**

# Modified Protocol (1)

New Title: Dichlorphenamide vs Placebo for Periodic Paralysis

Acetazolamide arm removed

Study design revised to 9 week double-blind, placebo controlled phase (DCP vs placebo) followed by 1 year open-label DCP extension.

Addition of new sites

Total enrollment decreased to 80 HYP participants and 60 HOP participants

Allergy to sulfonamides removed from exclusion criteria

## Modified Protocol (2)

Removed Week 04 and Week 35 visits to make the visit burden less for the participants.

Decreased length of baseline visit from 4 days to 3 days to decrease burden and required overnight stay only if participants were previously on a treatment or have a history of recent severe attacks. Drug naïve participants may be discharged at end of 1<sup>st</sup> day if stable.

Subjects who are actively enrolled in the original three-arm trial (DCP vs. ACZ vs. Placebo) when enrollment in the new trial begins will be given the option of transferring to the current trial and receiving open-label DCP or withdrawing participation.

# Time table

- August 10, 2009 DSMB Meeting
- September TBD, 2009 Receive DSMB approval and minutes
- Sept/Oct TBD, 2009 Submit IND amendment to FDA
- Sept/Oct TBD, 2009 Send to RSRB for approval
- October TBD, 2009 Aptuit to submit to EU regulatory authorities
- October TBD, 2009 Send amended protocol to sites
- October – Dec., 2009 Sites receive IRB approval
- **December 2009** **Enrollment re-starts!**
- September, 2011 Enrollment complete (80 HYP, 60 HOP)
- October, 2012 Last participant completes study
- November 30, 2012 Data analysis complete
- March 31, 2013 Blind revealed, participants notified of results, manuscript submitted

# Sites recruiting

## United States

Brigham & Women's Hospital (Boston, MA, USA), Anthony Amato, MD

Columbia-Presbyterian Medical Center (New York, NY, USA), Jinsy Andrews, MD

Mayo Clinic (Rochester, MN, USA), Brian Crum, MD

Ohio State University (Columbus, OH, USA), John Kissel, MD

University of California - San Francisco (San Francisco, CA, USA), Jeffrey Ralph, MD

University of Kansas Medical Center (Kansas City, KS, USA), Richard Barohn, MD

University of Rochester School of Medicine (Rochester, NY, USA), Emma Ciafaloni, MD

University of Texas Southwestern (Dallas, TX, USA), Stephen Cannon, MD, PhD

## European Sites

Hopital de la Salpetriere (Paris, FRANCE), Bertrand Fontaine, MD

Institute of Neurology (London, UK), Michael Hanna, MD

University of Milan (Milan, ITALY), Giovanni Meola, MD

## Potential New Sites

Johns Hopkins University (Baltimore, MD, USA), Kathryn Wagner, MD

University of Florida (Jacksonville, FL, USA), Michael Pulley, MD

St. Louis Washington University (St. Louis, MO, USA), Alan Pestronk, MD