

Clinical trial for atypical hemolytic uremic syndrome (aHUS).

Recent advances in the basic science of atypical HUS indicate that approximately half of the patients have mutations in various complement regulatory proteins that lead to dysregulation and excessive activation of this pathway for tissue injury. Based on this finding, a number of new anticomplement agents are being developed and moving forward to clinical testing. Eculizumab (Soliris) is approved for the treatment of aHUS.

Other biotechnology companies are also developing agents that can block the alternate pathway of complement. Celldex has produced a drug, TP10, based on the soluble complement receptor 1 (sCR1) that would be given intravenously every few days. They are performing a pilot study involving one or two patients to ascertain the feasibility of administering this agent in patients with aHUS and collecting preliminary information about its efficacy.

Patients interested in participating in this study are invited to come to the NYU Langone Medical Center in New York, New York for a two-week stay in the Clinical Research Center to receive this agent and ascertain whether it will ameliorate the activity of their disease. If you have a patient who might be interested in this study, please contact Dr. Howard Trachtman (914-563-1580, e-mail: howard.trachtman@nyumc.org).