Understanding Clinical Trials in Males with MECP2 Mutations

Currently there are active clinical trials for Rett syndrome and plans for new trials in the future. At this time, all the clinical trials have been designed with eligibility criteria that are inclusive for females and have excluded males with MECP2 mutations from participating. This has generally occurred as part of the study design in attempt to have a uniform genetic background that will allow investigators to determine the safety and effectiveness of the medication for Rett syndrome. There are several factors involved in the design of clinical trials. The funding agency, typically a pharmaceutical company, will frequently be involved in the design of the clinical trial with the goal of maximizing the information that is learned from the clinical trial. The investigators involved in conducting the clinical trial may have some input into the design of the clinical trial, but are typically involved with conducting the trial as designed by the funding agency. There are also regulatory agencies, such as the FDA, which have stringent criteria for clinical trial design to ensure safety and proper conduct of trials. Lastly, the knowledge of the clinical condition being treated will impact the design of the trial and the selection of the compounds used in the trial.