Understanding the Scout Program

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What is the Scout Program? Developed by Rettsyndrome.org in 2013, the Scout Program is a drug screening program that uses a mouse model to test a drug's potential to be a future treatment for Rett syndrome. The Scout Program was designed to aggressively accelerate the testing of compounds through preclinical trials* that are standardized so that every compound screened is studied under similar conditions.

The mice used in the Scout Program have a mutation in the *Mecp2* gene and have been established as a model for Rett syndrome. The Scout Program uses only female mice, ensuring a "medically relevant model ". While studies could be performed in male mice, the severity of their symptoms complicates the screening and yields lower quality data.

What is the benefit of the Scout Program to those with Rett syndrome? When the Scout Program was started, there was only one drug in a clinical trial for Rett syndrome. Today there are a number of clinical trials and through the Scout Program we have been able to accelerate the rate that drugs move to clinical trials. This program has given the pharmaceutical industry an opportunity to more quickly know whether their drug has potential or not. This saves them significant time and money. The Rett syndrome community benefits because drugs can be screened quickly and the preclinical data can help bring the drugs forward to the human clinical trials

How many drugs have been screened? To date over twenty compounds have been screened and another dozen are scheduled to be tested over the next year. The Scout Program has seen 3 compounds move forward to clinical trials or the planning stages of clinical trials. One is Newron's compound Sarizotan, whose Phase2/3 trial is the single largest clinical trial ever attempted in Rett syndrome. This clinical trial is in 5 sites in the U.S., 3 sites in Europe, 4 sites in India, and 2 sites in Australia. The second is Anavex's compound, Anavex 2-73, which will be starting a clinical trial in 2018 and is planned in at least 10-12 U.S. sites. The third is AMO's compound AMO-04, which is scheduled to be in a future clinical trial.

Who participates in the Scout Program? Rettsyndrome.org developed the Scout Program to serve both the academic community (universities) and industry (bio-technology companies and pharmaceutical companies). The industrial use of this program has exceeded our hopes. Bio-tech and pharmaceutical companies see that the Scout Program provides a rigorous platform for the companies to test compounds that they believe might be useful in treating Rett syndrome.

Who performs the preclinical trials of the Scout Program? Rettsyndrome.org provides the testing environment through an established partnership with PsychoGenics, a Contract Research Organization (CRO). PyschoGenics ensures that the Scout Program testing is done rigorously to provide high-quality preclinical trial data, which is required to commence any FDA approved clinical trial. Dr. Taleen Hanania, Senior Vice President of Behavioral Pharmacology leads the testing team.

*** What is a preclinical trial?** A preclinical trial is an animal study to test a drug, procedure, or medical treatment and collect data in support of the safety of a new treatment. Preclinical studies are required before clinical trials in humans can be started.

For more information on the Scout Program, visit https://www.rettsyndrome.org/for-researchers/scout-program