

Bone Marrow Transplant arrests symptoms in model of Rett Syndrome

We at IRSF want to make you all aware of a wonderful study that has come out of the University of Virginia under the guidance of Jonathan Kipnis, Ph.D.. This research was sponsored by the Rett Syndrome Research Trust, the National Institute of Child Health and Human Development, and the National Institute of Aging. In short, this is an excellent study that demonstrates that bone marrow transplantation in Rett mice (*Mecp2* null male mice) can arrest the course of the symptoms as they develop in the male mice. Dr. Kipnis' group demonstrated that replacing the microglial cells (a group of brain cells derived from bone marrow stem cells) in the brain that do not have MeCP2 with microglial cells that do express normal MeCP2 could arrest the developmental challenges in the male mice.

This study once again demonstrates how incredibly complex Rett syndrome is as a developmental disorder. The microglial cells are immune-derived and are the "street cleaners" in the central nervous system that work to help establish and maintain neuronal connections while "cleaning up" when neurons die. Please remember the microglial cells are only one cell type among many in the brain involved in Rett syndrome. As described by other scientists, Rett syndrome is principally a disorder of the neurons, and more recently astrocytes have also been shown to be contributors to the disorder.

This newest observation sets forward yet another branch of research that is needed for a better understanding of the Rett syndrome developmental story. It demonstrates the complexity of developmental systems affected by mutations in *MECP2*. This work will have to be repeated, done to FDA standards, as one starts to think about moving toward pre-clinical experiments. Dr. Kipnis' study continues to demonstrate the complex nature of this developmental disorder and now adds the intricacies of the immune system to better understand Rett development. However, we should all look at this as the cup is "half full", i.e. one more thing to understand with the possibility of many more ways forward.

As with the IRSF sponsored IGF-1 study, which by design is a treatment to modify the progression of the disease in Rett syndrome, any bone marrow transplantation study, too, will only modify the progression of Rett syndrome. These studies do not address all of the developmental systems involved in this complex disorder. The IGF-1 study and possible future stem cell studies should help us think through the challenges to better understand the multiple approaches needed to identify treatments for Rett.

Dr. Kipnis' study may not lead to a clinical trial and may not lead to a treatment, but it could. Moreover, the one thing it certainly has done is giving science world another avenue to explore, and maybe a new way to attack this Rett syndrome. This may entice others that work on the immune system or microglial cells or other related cells a reason to start pursuing research in Rett syndrome. Maybe it will lead the scientists to another way to treat some of the symptoms of Rett or maybe even a cure.

Each new finding, each little step gives us all hope that one day our girls will be able to live without Rett syndrome, but we have to continue to push the science to get there. Hopefully, this has gotten us one step closer to reducing the struggles that come with Rett syndrome.

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