### Therapeutic Strategy to Remedy Synaptic Deficiencies that Cause the Symptoms of Rett Syndrome and Other Autism Spectrum Disorders

Technology #11841-12720

#### Applications

- Treatment of Rett Syndrome
- Treatment of other autism spectrum disorders
- Enhancement of recovery of patients with damaged nervous system

#### Problem Addressed

A great variety of neurodevelopment diseases are caused by genetic mutation in the MECP2 gene. As an example, Rett Syndrome is a rare genetic neurological disorder that predominantly affects 1 out of 10,000 to 15,000 newborn girls. Mutations in the MECP2 gene in Rett patients delay neural and synaptic maturation leading to extended brain plasticity. This condition is characterized by developmental delay in newborns that is accompanied by certain motor, behavioral and intellectual disabilities. Currently, there is no cure for this disease. This research has shown that it is possible to study brain plasticity in subjects with Rett Syndrome, and affect the progression of the disease by administering plasticity-modifying agents.

#### Technology

This research has developed methods for identifying genetic pathways involved in the development of different neurodevelopmental diseases and means for modification of the course of the diseases. Particularly, IGF-1, Insulin-like Growth Factor 1, has been shown to be safe and effective for managing the progression of Rett Syndrome. IGF-1’s efficacy stems from its ability to promote neuronal growth and maturation of synapses. IGF-1 accelerates the process of visual cortex maturation, which is, as a rule, slowed down in patients with Rett Syndrome.

This discovery set the foundation for an IGF1 Phase 1 clinical trial in children with Rett syndrome. The positive results from Phase 1 led to the Phase 2 trial that is currently underway.

#### Advantages

- IGF-1 is a well-studied hormone with insulin-like molecular structure and function
- IGF-1 is approved and marketed for treatment of growth failure

#### Categories For This Invention:

- Life Sciences
- Therapeutics
- Other (Therapeutics)
Intellectual Property:
Identifying and modulating molecular pathways that mediate nervous system plasticity
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Treatment of rett syndrome
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Inventors:
Mriganka Sur
Emanuela Giacometti
Nathan Wilson
Daniela Tropea
Gabriel Kreiman
Rudolf Jaenisch

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