

## Autistic Disorders and Medical Biotechnology

Autistic Disorders (ADs) are neuro-developmental disorders in the category of pervasive developmental disorders chiefly described by three main deficits: 1) deviant communication, 2) impaired reciprocal social interaction, and 3) limited, repetitive and stereotypic patterns of behaviors or interests<sup>1</sup>. The world-wide prevalence of ADs is estimated to be 62/10,000<sup>1</sup>. Although various treatment regimens have been proposed for improving overall function of autistic patients, a definite efficient treatment which can target both core and associated symptoms in ADs has not been established so far. For example, current approved drugs by the Food and Drug Administration (FDA) such as risperidone and aripiprazole have not been proven to pose significant effect on the core features of this disorder<sup>2-4</sup>.

While the absolute pathophysiological mechanism of ADs is still debated, several genetic, environmental and neurobiological factors such as immune dysfunction, oxidative stress and imbalance of the inhibitory-excitatory systems are implicated in the pathogenesis of these disorders<sup>5-7</sup>. Neurobiological models have become research areas of interest for development of novel therapeutic agents in this regard<sup>1</sup>. Increased neuronal excitation in various central pathways has been proposed as one of the main neurobiological dysregulations in autistic patients<sup>1</sup>. Indeed, biotechnology and in particular gene therapies plays an important role in the future of research in autism<sup>1</sup>.

**Neurexin 1:** Part of family of genes that play a role with the neurotransmitter glutamate which is linked to autism. Gene chip technology was used to scan for genetic similarities in people with autism. DNA was scanned to search for copy number variations (CNVs), or insertions and deletions of genetic material linked to autism<sup>1</sup>.

**Adult Form of Fragile X Syndrome:** Expression of toxic RNA leads to Fragile X Tremor Ataxia Syndrome is modifiable by gene therapy<sup>1</sup>.

**Fragile X Syndrome:** Caused by loss of a gene called **FMR1** which acts as a break on a protein synthesis in specific area of the brain. This allows another protein, mGluR5 **Metabotropic Glutamate Receptor**, to function unchecked resulting in over activity in the brain. Reducing **mGluR5** reduces symptoms associated with fragile x syndrome<sup>1</sup>.

**MECP2** Activation of the gene reversed Rett syndrome. The MECP2 gene mutation is present in 95 percent of individuals with the disease<sup>1</sup>.

**Keywords:** Autistic disorder, Biotechnology, Genetic diseases

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