Autifony Therapeutics receives Orphan Drug Designation

for AUT00206 for Fragile X Syndrome

Stevenage, UK – 10 July 2017- Autifony Therapeutics Limited ("Autifony"), which is pioneering the development of novel pharmaceutical treatments for serious disorders of the central nervous system, today announced that the U.S. Food and Drug Administration (FDA) has granted AUT00206 an Orphan Drug Designation for the treatment of Fragile X Syndrome, the most common known cause of inherited learning disabilities.

Orphan Drug Designation is a special status granted to a drug intended to treat a rare disease or condition, which meets criteria specified in the Orphan Drug Act. Orphan designation qualifies the sponsor of the drug for various development incentives as well as seven years of market exclusivity following approval in the U.S.

The application was founded on positive results in a range of preclinical studies exploring the efficacy of AUT00206 in a genetic model of Fragile X in mice. Studies of multiple disease-relevant endpoints showed significant improvement in both cognitive and behavioural functioning of the mice following treatment with AUT00206. This work was supported by the FRAXA Research Foundation, a charity which encourages and funds research into Fragile X Syndrome.

Dr Charles Large, CEO of Autifony, said: "We believe that our promising findings in the preclinical model of Fragile X support investigation of AUT00206 in clinical trials, which we plan to initiate as rapidly as possible in 2018. Orphan Drug designation by the FDA provides us with further encouragement to address this important unmet medical need."

Dr Mike Tranfaglia, Medical Director and Chief Scientific Officer of FRAXA Research Foundation, said: "We are excited about the potential of AUT00206 as a treatment for Fragile X. With its novel mechanism of action, this compound has demonstrated its ability to rescue many disease-relevant phenotypes in Fragile X animal models. AUT00206 has the potential to be a disease-modifying therapy for people with Fragile X, and we are looking forward to clinical trials in Fragile X patients."

AUT00206 is also in development for schizophrenia. An experimental medicine study in patients with schizophrenia, involving electrophysiological biomarkers and imaging outcomes, was initiated in May 2017.

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About Autifony Therapeutics Ltd
Autifony Therapeutics is an independent UK based biotechnology company formed in 2011 as a spin-out from GSK, which retains equity in the company. The Company is focused on the development of high value, novel medicines to treat serious diseases of the central nervous system. It is funded by SV Health Investors, Touchstone Innovations plc, Pfizer Venture Investments, International Biotechnology Trust PLC, and UCL Business plc. www.autifony.com

About AUT00206
AUT00206 is a novel, orally active small molecule designed to selectively modulate Kv3 potassium channels.

The drug recently completed a first-in-human clinical trial in healthy volunteers and was safe and well tolerated. In mice with a targeted knockout of the FMR1 gene, AUT00206 treatment for 21 days improved both cognitive and behavioral abnormalities that are similar to those that occur in children with Fragile X syndrome.

In addition, preclinical studies using models relevant to the pathophysiology of schizophrenia suggest that AUT00206 has the potential to treat cognitive and negative symptoms of schizophrenia, as well as positive symptoms with fewer side effects than current anti-psychotic drugs. Cognitive and negative symptoms are poorly treated by antipsychotic drugs and are associated with significant functional impairment and reduced quality of life for patients.

About Fragile X Syndrome

Fragile X Syndrome is a genetic condition and is the most common known cause of inherited learning disabilities. There have been a number of studies aimed at determining the prevalence of FXS in males and females. The prevalence of FXS in males is approximately 1 in 3,600 to 4,000 and in females is approximately 1 in 4,000 to 6,000, and it occurs in all racial and ethnic groups. Disruption of the FMR1 gene causes a range of developmental problems, including learning disabilities and cognitive impairment. Almost all boys with Fragile X suffer from learning disabilities to varying degrees. Girls usually have milder learning disabilities than boys. Other symptoms include anxiety and hyperactive behaviour, and frequently autistic like behaviours such as avoiding eye contact, anxiety in social situations and insistence on familiar routines. Some children develop epilepsy. Speech and language are usually delayed, with continuing communication difficulties.

About FRAXA

Founded in 1994 by parents who have children with Fragile X, FRAXA is a nonprofit, tax-exempt organization based in Newburyport, Mass. Committed to finding a cure for Fragile X, FRAXA has funded more than $26 million in biomedical research, yielding discoveries that are changing the lives of families coping with Fragile X. FRAXA is one of the most efficient and effective charities in the world, with management and general expenses under 4 percent and research expenditures at 86 percent. Three Nobel Laureates sit on their Scientific Advisory Board.

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