



Axial Biotherapeutics Announces Publication of Preclinical Data Highlighting the Link Between Human Gut Microbiota and Behavioral Symptoms of Autism Spectrum Disorder in Mouse Models

WALTHAM, Mass., May 31, 2019 – Axial Biotherapeutics, a biotechnology company dedicated to building a unique class of gut-targeted programs for neurodegenerative diseases and neurodevelopmental disorders, today announced the publication of new findings in *Cell* by Sarkis K. Mazmanian, Ph.D., the Company’s scientific co-founder, and California Institute of Technology (Caltech) researchers, demonstrating that the colonization of germ-free mice with gut microbiota from human donors with Autism Spectrum Disorder (ASD) induces hallmark behaviors of ASD as compared to control, typically-developing (TD) gut microbiota.

The publication, entitled “Human Gut Microbiota from Autism Spectrum Disorder Promote Behavioral Symptoms in Mice,” is the first to suggest a direct link between gut microbiota and the pathophysiology and behavioral outcomes of ASD. This link further supports Axial Biotherapeutics’ foundational scientific rationale, suggesting that the gut microbiome regulates behavior in mice via the production of neuroactive metabolites.

“We are pleased to see these new preclinical insights from Sarkis and his team, which further enhance our understanding of the link between the human gut microbiome and the brain as it relates to ASD and other CNS diseases. It is exciting to build upon our hypothesis rooted in this innovative research as evidence continues to show that non-genetic, yet heritable gut microbiota may contribute to human behaviors,” said David Donabedian, Ph.D., and Co-founder and Chief Executive Officer of Axial Biotherapeutics. “As we continue to advance our clinical-stage candidate, AB-2004, we look forward to drawing on these foundational insights to better understand the complexities of altered ASD human behaviors.”

“ASD manifests as changes in complex human behaviors, and is likely the outcomes of genetic and environmental factors. Through our research, we have been able to see a clear induction of hallmark ASD behaviors in germ-free mice that harbor human ASD gut microbiota, which supports Axial’s approach to ASD-symptom treatment,” said Dr. Mazmanian, the Luis & Nelly Soux Professor of Microbiology in the Division of Biology and Biological Engineering at Caltech and scientific co-founder of Axial Biotherapeutics. “Our findings represent additional evidence for a new scientific paradigm in which specific bacterial metabolites produced by gut bacteria modulate behaviors in experimental animals, and potentially in people. While more work is needed, this study and several other recent publications suggest that gut-brain connections contribute to the pathophysiology of some forms of ASD.”

In November 2016, Axial Biotherapeutics entered into a license agreement with Caltech for worldwide exclusivity to related intellectual property in applications for neurological diseases and disorders. Patient screening for the Phase 1b/2a clinical trial of AB-2004 was initiated in the first quarter of 2019.

About Autism Spectrum Disorder



According to the Center for Disease Control (CDC) about 1 in 59 children has been identified with ASD. Core symptoms of ASD include impairments in social interaction, communication and the presence stereotyped repetitive behaviors. Comorbidities are extensive and diverse, and include gastrointestinal (GI) dysfunction, metabolic abnormalities, allergies, autoimmune disorders, neuroinflammation and epilepsy. GI dysfunction is estimated to occur in 40-70% of individuals with ASD. The medical severity of core and non-core ASD symptoms, and the lack of safe and effective long-term treatments argue strongly for pursuing fundamentally new measures to address this significant unmet medical need.

About Axial Biotherapeutics

Axial Biotherapeutics is a clinical stage biopharmaceutical company pioneering novel science focused on the interaction between the brain and the gut to mitigate the causes and symptoms of CNS and other gut-derived diseases. The Company has built a pipeline of novel small-molecules and live biotherapeutics with lead programs to address the significant unmet patient needs associated with Parkinson's Disease and Autism Spectrum Disorder.

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