



## **Axial Biotherapeutics Presents New Preclinical Data from AB-2004 Program Demonstrating Reduction of Microbiome-Derived Metabolites in Mouse Models with Autism Spectrum Disorder at the International Society for Autism Research (INSAR) 2019 Annual Meeting**

- *Preclinical results support development of AB-2004 as potential treatment for gastrointestinal dysfunction and associated behavioral symptoms of Autism Spectrum Disorder –*
- *Patient screening for Phase 1b/2a clinical trial of AB-2004 initiated in 1Q19 -*

**WALTHAM, Mass., May 2, 2019** – Axial Biotherapeutics, a biotechnology company dedicated to building a unique class of gut-targeted programs for neurodegenerative diseases and neurodevelopmental disorders, presented new preclinical data on AB-2004, its orally administered, drug candidate that has demonstrated the ability to repair leaky gut and improve repetitive behavior, anxiety, and ASD-related sensorimotor gating deficits by removing key microbial metabolites in animal models with Autism Spectrum Disorder (ASD). The Company presented the data in a poster presentation at the International Society for Autism Research (INSAR) 2019 Annual Meeting in Montreal, Canada.

“We are pleased to present additional preclinical data which substantiates the potential of our platform to remove microbial based metabolites, by which we can restore gastrointestinal (GI) function and improve behavior associated with CNS and gut-derived diseases,” said David H. Donabedian, Ph.D., Co-founder and CEO of Axial Biotherapeutics. “We believe that altered microbial metabolite profiles can drive ASD symptoms, including behavior abnormalities, leaky gut and other GI issues. We believe that AB-2004 has the potential to alter these symptoms and we look forward to continuing patient screening for our Phase 1b/2a program and progressing this product further into the clinic.”

The main highlights from the poster presentation titled, “Characterization of GI barrier integrity and gut microbiome-derived metabolites in BTBR, Shank3 and Cntnap2 mouse models of ASD and demonstration of AB-2004 as a potential mitigating therapeutic” include:

- The Cntnap2<sup>-/-</sup> mouse model accurately recapitulated the leaky gut phenotype and elevated levels of the gut microbiome-derived metabolite 4-EPS that have been reported in ASD patients
- Treatment with AB-2004 effectively restored GI integrity and reduced elevated 4-EPS levels in Cntnap2<sup>-/-</sup> mice
- The Cntnap2<sup>-/-</sup> model has been identified as a promising and translationally relevant animal model for the development of microbiome-inspired therapies for the effective treatment of GI and behavioral dysfunctions in ASD



- These data support the development of AB-2004 as a treatment for GI dysfunction in ASD and potentially behavioral symptoms through reduction of pathologically active microbiome-derived metabolites

Axial is currently screening ASD adolescents for its Phase 1b/2a clinical trial of AB-2004.

### **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. To learn more, visit <https://www.axialbiotherapeutics.com/>.

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