Axial Biotherapeutics Announces Positive Topline Results from Phase 1b/2a Clinical Trial of AB-2004 for the Treatment of Autism Spectrum Disorder

WALTHAM, Mass.--(April 2, 2020)-- Axial Biotherapeutics, a biotechnology company dedicated to building a unique class of gut-targeted programs for neurodegenerative diseases and neurodevelopmental disorders, today announced positive topline data from its Phase 1b/2a clinical trial of AB-2004, a first-in-class therapeutic that removes certain metabolites from the gastrointestinal (GI) tract for the treatment of Autism Spectrum Disorder (ASD). The study met the primary endpoint of safety, tolerability and adherence with no drug-related adverse events (AE) reported. Initial signs of efficacy were also observed across exploratory endpoints, including signs of target engagement of AB-2004 in the GI lumen with significant reductions of several key neuroactive microbial metabolites (NMMs™), measured in plasma and urine.

“These topline results mark an important milestone for Axial as we have now observed positive preliminary efficacy and safety data for AB-2004, further supporting our novel hypothesis of targeting the GI to address core and non-core symptoms of ASD in children,” said David H. Donabedian, Ph.D., Co-founder and Chief Executive Officer of Axial Biotherapeutics. “These initial data support the continued advancement of this program, further de-risking our future development plans. We look forward to presenting the full results from this study at an upcoming medical meeting and plan to initiate the Phase 2, placebo-controlled study of AB-2004 in ASD later this year.”

“Worldwide, it is estimated that 1 in 160 children has ASD. In the U.S., ASD affects about 1 in 59 children and there are currently no approved therapies to treat core behavioral symptoms and limited options for non-core symptoms. Axial’s extensive preclinical work, coupled with its initial clinical data, represents a new and potentially promising approach to treating ASD via the gut-brain axis,” said Dr. Robert Hendren, Professor of Psychiatry and Behavioral Science; Division of Child and Adolescent Psychiatry, UCSF Weill Institute for Neurosciences. “I am encouraged by these early clinical findings and look forward to working with Axial on the design and execution of their Phase 2 placebo-controlled study.”

Topline Results

The Phase 1b/2a study was designed to assess the primary endpoints of safety, tolerability and adherence of AB-2004 in up to 26 male adolescent subjects with ASD. Additional exploratory endpoints included changes in key microbial metabolites, target engagement and changes in core and non-core ASD behaviors including irritability, anxiety, socialization, communication and GI symptoms. Results include:

- AB-2004 was safe and well-tolerated with no drug related AEs reported.
- Adherence to the three-times-per-day dosing regimen was greater than 90%.

- Significant reductions in plasma and urinary levels of several key microbial metabolites over the 8-week dosing period were also observed, demonstrating target engagement of AB-2004 in the GI lumen.
- Significant improvements in irritability scores based on the Aberrant Behavior Checklist were observed, particularly in subjects with more severe baseline scores.
- Significant improvement in anxiety based on the Pediatric Anxiety Rating Scale were observed in subjects with more severe baseline scores.
- Improvements in social withdrawal scores based on the Aberrant Behavior Checklist were observed, particularly in subjects with more severe baseline scores.

About AB-2004

AB-2004 is a first-in-class, orally administered therapeutic, targeting Autism Spectrum Disorder (ASD). In a preclinical mouse model of ASD, AB-2004 reduced urinary levels and neuroactive microbial metabolites (NMMs™), repaired leaky gut and improved repetitive behavior, anxiety and sensorimotor gating deficits.

About Autism Spectrum Disorder

According to the Center for Disease Control (CDC), about 1 in 59 children has been identified with Autism Spectrum Disorder (ASD). Core symptoms of ASD include impairments in social interaction, communication and the presence of stereotyped repetitive behaviors. Comorbidities are extensive and diverse, and include gastrointestinal (GI) dysfunction, irritability, ADHD, metabolic abnormalities, allergies, autoimmune disorders, neuroinflammation, anxiety 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 the central nervous system (CNS) and other gut-derived diseases. The Company has built a pipeline of novel small molecules with lead programs to address the significant unmet patient needs associated with Autism Spectrum Disorder (ASD) and Parkinson's Disease (PD).

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