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Safety, tolerability and preliminary efficacy of AB-2004, a gut-restricted molecule targeting gut metabolites, in an adolescent population with ASD

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Research has shown that the gut flora of autistic children is different compared with non-autistic children. Axial Therapeutics and collaborators have identified certain bacteria-derived metabolites (neuroactive microbial metabolites) that are produced in the gut and have been shown in preclinical models to reach the brain resulting in altered myelination patterns and certain behavioral characteristics associated with autism. Axial is developing AB-2004, a novel gut-restricted therapeutic that removes these metabolites from the gastrointestinal (GI) tract, in an effort to help manage co-occurring conditions in those with autism.

A study of AB-2004 was conducted as an open label, single cohort, 8-week multiple dose escalation study in 12- to 17-year-olds designed to establish safety, tolerability, and adherence to the three-times-per-day dosing regimen. Thirty subjects were enrolled at three sites in Australia and New Zealand and 26 successfully completed the study. Safety was assessed by spontaneously reported adverse events as well as physical exams, blood samples, and urine samples. Exploratory endpoints included changes in key neuroactive microbial metabolites, changes in core and non-core autistic traits, and GI symptoms.

AB-2004 was found to be safe and well-tolerated with no drug-related adverse events. Significant reductions in plasma and urinary levels of several neuroactive microbial metabolites over the 8-week treatment were also observed, demonstrating target engagement of AB-2004 in the GI lumen. Additionally, irritability and anxiety assessment scores, as measured by the Aberrant Behavior Checklist-Irritability Subscale and Pediatric Anxiety Rating Scale, respectively, showed significant improvement over the 8-week treatment. Taken together, these data support that AB-2004 was safe and well-tolerated and informed the design of a currently enrolling global Phase 2 double-blind, randomized, placebo-controlled trial targeting irritability associated with autism in adolescents.

Biography

Dr. Campbell joined Axial in 2016 and was appointed the company's CEO in February 2021. Dr. Campbell has played a fundamental role in company's growth and strategy over the last 5 years. He brings more than 25 years of experience leading and building teams that have strong track records of discovering and developing novel therapeutics for the patients who need them. Prior to joining Axial, Dr. Campbell held various leadership roles at biopharmaceutical and chemical manufacturing companies including Corden Pharma, Surface Logix and Insmed Inc. Prior to Corden Pharma Dr. Campbell served as Vice President of R&D at Ancora Pharmaceuticals, successfully triaging the company through the acquisition and integration process by Corden Pharma. Throughout his carrer Dr. Campbell played an integral role in the discovery and/or development of seven clinical stage drug candidates, including the recently approved kinase inhibitor RUZUROCK™. During his career Dr. Campbell has been co-inventor on over 20 issued patents. He holds a BSc with Honors in Chemistry from St. Francis Xavier University, a Ph.D. in Organic Chemistry from the Queen's University (Canada) and did post-doctoral research at Duke University..

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