



Avaxia Biologics Receives FDA Orphan Drug Designation for AVX-470 for the Treatment of Pediatric Ulcerative Colitis

LEXINGTON, Mass. – November 21, 2014 – [Avaxia Biologics, Inc.](#), a clinical-stage biopharmaceutical company developing gut-targeted antibody therapeutics for gastrointestinal diseases, announced today that the US Food and Drug Administration (FDA) granted Orphan Drug Designation to AVX-470 for the treatment of pediatric ulcerative colitis. AVX-470 is a gut-targeted anti-TNF antibody, which earlier this year successfully completed a Phase 1b clinical trial in adults with active ulcerative colitis.

Pediatric ulcerative colitis (UC) is a serious chronic inflammatory disease of the colon. As with adult patients, children with acute UC have symptoms of rectal bleeding, diarrhea, weight loss, abdominal pain, and fever. In addition, pediatric UC patients face impaired growth and delayed puberty, as well as challenges to self-image and quality of life during the critical childhood years. Most pediatric UC patients experience more severe disease than adults, and the disease may progress more quickly in children. If drug therapy fails to control the disease, the only treatment is surgical removal of the colon (colectomy).

“We are pleased to receive Orphan Drug Designation for AVX-470 for treatment of pediatric ulcerative colitis,” said Barbara S. Fox, Ph.D., Chief Executive Officer. “The drawbacks of current anti-TNF drugs are especially problematic in children. These drugs are injected or infused and may have serious side effects caused by systemic immunosuppression. As a gut-targeted antibody drug, AVX-470 avoids these drawbacks and may allow use of anti-TNF therapy earlier in the treatment of pediatric UC, with the goal of improving patient outcomes.”

The US Orphan Drug Act is intended to assist and encourage companies to develop safe and effective therapies for the treatment of rare diseases and disorders that affect fewer than 200,000 individuals within the United States. Among other benefits, Orphan Drug Designation confers a seven-year term of market exclusivity upon FDA approval.

About AVX-470

AVX-470 is the first gut-targeted anti-TNF antibody in clinical development. The lead indication is inflammatory bowel disease (IBD), a serious disease involving chronic inflammation of the intestinal tract. Currently marketed anti-TNF drugs are highly effective for the treatment of IBD. However, despite their success, these drugs have two significant drawbacks. One is that they must be infused or injected, whereas patients prefer oral medications. More importantly, they cause rare but serious side effects, such as risk of serious infection, because they dampen the immune system throughout the body, not just in the intestines where the disease occurs. As a result, these drugs are used mostly as a second- or

third-line therapy, despite evidence that earlier use may improve patient outcomes. In contrast, AVX-470 is an orally administered antibody therapeutic that targets the gut, potentially improving safety and efficacy over existing anti-TNF therapies. Avaxia believes that AVX-470 has the potential to become a first-line therapy that transforms the treatment of IBD.

About Avaxia Biologics

Avaxia leads the field of gut-targeted antibody therapeutics — orally administered, minimally absorbed antibody drugs that are designed to act locally in the gastrointestinal tract. Avaxia’s clinical candidate, AVX-470, is an oral anti-TNF antibody for inflammatory bowel disease (IBD). This transformative product offers potentially improved safety and efficacy over existing anti-TNF therapies by focusing immune suppression only where needed in the diseased gut. Avaxia is focused primarily on gastrointestinal diseases such as IBD, celiac disease and necrotizing enterocolitis, though our proprietary gut-targeted antibody platform can address any disease that has a biological target accessible via the gastrointestinal tract. www.avaxiabiologics.com

Contact

Avaxia Biologics:

Barbara S. Fox, Ph.D.

Chief Executive Officer

or

Michael D. Rivard

VP Corporate Development

781-861-0062

media@avaxiabiologics.com

Media:

MacDougall Biomedical Communications

Charles Liles, 781-235-3060

cliles@macbiocom.com