MassBiologics receives orphan drug status from FDA for hepatitis C treatment

December 4 2013

MassBiologics of the University of Massachusetts Medical School (UMMS) has received an orphan drug designation from the U.S. Food and Drug Administration (FDA) for MBL-HCV1, a monoclonal antibody developed to prevent hepatitis C virus (HCV) recurrence in patients receiving a liver transplant.

Complications from chronic HCV infection are the most common indications for liver transplantation today. For patients with end-stage liver disease or hepatocellular carcinoma resulting from HCV infection, liver transplantation is often the only treatment option, but it is not a cure for the disease. In almost all cases, the new donor liver becomes infected with HCV soon after transplantation.

MassBiologics' monocloncal antibody, currently in a Phase 2 clinical trial, is intended to prevent HCV from damaging the transplanted liver.

"Being granted orphan drug status facilitates the goal of bringing this investigational product to patients," says Deborah C Molrine, MD, deputy director of clinical affairs at MassBiologics and professor of pediatrics at University of Massachusetts Medical School. "The economic incentives available to MassBiologics and potential commercial partners through the Orphan Drug Act will contribute greatly to bringing this monoclonal antibody to market as a treatment option for patients receiving liver transplants as a result of HCV infection."

The Orphan Drug Act was established by Congress in 1983 to aid the development of new therapies for rare medical conditions or diseases that affect less than 200,000 patients annually. To help stimulate new drug development for these less common conditions, the FDA provides financial benefits to companies that achieve orphan drug designation, including market exclusivity for 7 years, tax incentives, fee waivers and potential grant support.

Developed by MassBiologics, MBL-HCV1 is a fully human monoclonal antibody that targets a region of the hepatitis C virus on its surface envelope, preventing it from infecting liver cells. MBL-HCV1 has been shown to be safe in healthy human subjects and is currently being studied in patients with chronic hepatitis C infection undergoing liver transplantation.

"Infusions of the monoclonal antibody have been well-tolerated in transplant patients and allow for delivery of the targeted HCV treatment to begin just before the removal of the diseased liver and to continue through the early post-transplant period," said Dr. Molrine. "A Phase 2 study is underway in liver transplant patients that combines the monoclonal antibody with one of the first two oral HCV direct acting anti-virals to be licensed by the FDA. We anticipate having data to present soon on the effect of this treatment on HCV detection after liver transplantation."

Provided by University of Massachusetts Medical School