FDA approves trial for type 1 diabetes treatment

June 29 2010

The U.S. Food & Drug Administration (FDA) has granted Investigational New Drug (IND) regulatory clearance to initiate a Phase I/II clinical trial evaluating Alpha-1 Antitrypsin (AAT) in type 1 diabetics, based on research by Dr. Eli Lewis of Israel's Ben-Gurion University of the Negev.

This is the first time AAT will be evaluated in humans with type 1 diabetes. AAT is an FDA-approved off-patent drug currently used to treat pulmonary emphysema among youngsters and adults with an AAT genetic deficiency.

"We designed the trial with the same dose of AAT that has been used safely and effectively for over 20 years," explains Dr. Lewis, who is the director of Israel's only Clinical Islet Laboratory and a senior lecturer in the Department of Clinical Biochemistry at BGU in Beer-Sheva, Israel.

Dr. Lewis received a generous five-year Young Scientist Career Development Award from the Juvenile Diabetes Research Foundation (JDRF). He has also received the prestigious Yigal Alon Scholarship, the Wolf Foundation Krill Award, a Marc Rich Foundation award and two Israel Science Foundation grants.

Dr. Lewis conducted and published research studies using animal models to demonstrate that AAT may be effective in reversing type 1 diabetes during his post-doctoral fellowship at the University of Colorado and later in his own lab at BGU. He determined that eliminating

inflammation is the key to pancreatic islet beta cell survival and to restoring normal glucose levels.

This approach could potentially eliminate the need for daily insulin shots in recently diagnosed individuals, whose native circulating AAT molecules appear to be inactivated by high glucose. AAT is also potentially potent following pancreatic islet transplantation, sparing patients from intense immunosuppressive therapy. The procedure of islet transplantation is the only known approach today that affords continuous physiological glucose levels to patients with diabetes.

According to Dr. Lewis, "We found that targeting multiple inflammatory molecules by using a safe non-toxic and non-steroidal drug, we can block inflammation so effectively that we literally modify the immune response, which facilitates transplant acceptance to treat diabetes."

The study protocol provides for AAT administration during an eight-week treatment period in an initial group of 15 individuals with recently diagnosed diabetes, potentially expanding to up to 50 patients. As in most other diabetes trials, enrolled patients will be monitored for two years.

"Dr. Lewis is at the forefront of research in his field to find a treatment for type 1 diabetes and we're very excited that BGU is exporting its pioneering research once again to the U.S.," says Doron Krakow, executive vice president of American Associates, Ben-Gurion University of the Negev. "We wish Eli success with the trials and hope that his findings lead to a cure for a disease that affects millions of people around the world."

Provided by American Associates, Ben-Gurion University of the Negev