UCF, UF receive $5.5 million NIH grant for hemophilia research

Published on September 3, 2011 at 5:03 AM

At a time when research funding is hard to come by, a University of Central Florida and University of Florida partnership has landed almost $5.5 million in National Institutes of Health highly competitive grants for hemophilia research.

The first grant worth $3.6 million over five years is aimed at determining whether a green technique pioneered at UCF will help make treatment of hemophilia A more effective. The second grant worth $2 million for four years is for similar research for hemophilia B. The grants were awarded to UCF and UF, which applied for funding jointly thanks to a long-time collaboration between scientists at both institutions. Duke University also is a partner for the research on hemophilia A.

Hemophilia is an incurable bleeding condition that affects about 400,000 adults and children worldwide. Hemophilia is characterized by defects in the gene that produces proteins required for blood to clot. Hemophilia A, the most common type of hemophilia, is characterized by prolonged or spontaneous bleeding, especially in the muscles, joints or internal organs.

Treating hemophilia is challenging and dangerous because many patients suffer fatal allergic reactions to the protein that doctors use to facilitate blood clotting. The scientists are working on a way to make patients resistant to any deadly allergic reactions caused by the protein.

Treatments with the protein are also expensive. They must be provided in a hospital setting under supervision, and they can cost up to $1 million over a patient's lifetime because of the required hospital stays and blood transfusions. Average annual treatment costs are $60,000 to $150,000, according to the National Hemophilia Foundation.

"I am confident we will achieve success sooner than you think," said UCF Professor Henry Daniell. "We are hopeful that this technique will potentially save thousands of lives."

The researchers are using genetically modified plants to encapsulate a tolerance-inducing protein within plant cells so the protein could be ingested and safely travel through the stomach before being released into the small intestines, where the immune system can act on it.

In mice with hemophilia B, when blood clotting factor IX bio-encapsulated within plant cells was delivered to the gut, it prevented fatal anaphylactic shock and complex immune reactions. The new NIH funding, which came through the National Lung, Blood and Heart Institute, will help propel the research to determine if the technique can work in other models and potentially to clinical trials thereafter.

"The collaboration has an excellent chance of developing treatments that improve the lives of people with hemophilia and, at the same time, help lower health care costs," said Roland Herzog, a professor at UF.

After Daniell mentored Herzog at Auburn University, Herzog went on to develop his career. He has received multiple awards for his research in hematology including several NIH grants, a career development award from the National Hemophilia Foundation, an outstanding investigator award from the American Society of Gene Therapy and a Bayer Hemophilia Award.

Both researchers are hopeful that if future research bears out, this approach would be much safer and potentially deliver less expensive treatments to thousands who live with this disease.

While the approach is cutting edge, the NIH funding has come after Daniell and Herzog's research was featured last year in Proceedings of the National Academy of Sciences, a highly acclaimed scientific journal. Bayer Healthcare of Germany, the world's largest funder of hemophilia research, also gave Daniell a $200,000 grant in 2010 for research exploring the novel concept.

Daniell is conducting similar research on a polio vaccine funded by the Bill and Melinda Gates Foundation and on diabetes funded by the Juvenile Diabetes Research Foundation.

"The clinical translation of our work is closely tied with other projects in our lab, and all are showing promising

results. So I am very hopeful that this concept and technology will move forward soon," Daniell said.

Source: University of Central Florida