Hemophilia of Georgia
It takes more than medicine...

Hogs Help People with Hemophilia – Again!

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Hemophilia of Georgia has had a hog as its mascot for many years. The large pink porker was chosen because the acronym for Hemophilia of Georgia – HoG – of course spells hog. But hogs have played an important role in treatment for people with hemophilia and they are poised to do an even greater service. Some people with hemophilia A (factor VIII deficiency) can't be treated effectively with factor VIII concentrate. Their immune system recognizes the factor concentrate as a foreign invader and develops inhibitors against the factor. Their body destroys factor concentrate soon after it is infused, before it can stop any bleeding.

For some years, one option for treating people who have an inhibitor to factor VIII has been to give them porcine factor VIII. Porcine factor VIII is taken from the blood of pigs. The pig factor VIII is close enough to human factor VIII to stop bleeding but is different enough so that the inhibitors don't destroy it. Unfortunately, with repeated use, people can develop inhibitors against the porcine factor.

Pigs are now playing another part in hemophilia treatment that has potential to help everyone with factor VIII and factor IX deficiency. The University of Nebraska-Lincoln has announced that it has received a $9.98 million grant from the National Institutes of Health to fund the research of a team led by William Velander, a chemical engineer. The team is working with transgenic pigs. These transgenic pigs are pigs that have been genetically engineered to produce human factor proteins in their milk. Scientists have been working on transgenic animals for over 15 years. The transgenic pigs developed by Dr. Velander's team started out as regular pig embryos. While still at the one- or two-celled stage, they were injected with either the human gene that controls the production of factor VIII or the one that controls factor IX. The human gene is combined with one that targets it to the mammary glands of the pig. The embryo is then put into a mother pig where it develops and is born. The transgenic pigs are able to start producing milk about a year after they are born. The milk they give is very rich in human factor proteins.

At first researchers thought that the only way the factor proteins could be used would be to remove them from the milk, purify them, and then inject them into the bloodstream, just as factor concentrate is used today. Even though it was milk, it didn't seem likely that you could get the factor proteins into your bloodstream by drinking it. It was believed that the acid in our stomachs would breakdown the factor proteins making them useless for clotting. Dr. Velander's team decided to test and see if drinking the pig milk might work. They took milk from a transgenic pig that had been engineered to make factor IX. The factor IX pig milk was then fed to mice that had hemophilia B.

Within 45 minutes after drinking the pig milk, the factor IX had passed through the digestive system of the mice into the bloodstream and was able to stop bleeding. Drinking factor proteins instead of injecting them has several advantages. Obviously it is much easier and less painful than using a syringe and needle. This is especially true for babies. It also will not be necessary to treat the factor to remove all other proteins. Research also suggests that drinking factor lowers the risk of developing inhibitors.

This research could be life-saving for the 80% of people with hemophilia in the world who presently get no treatment at all. A pig produces about 300 liters of milk in a year. Several hundred transgenic pigs could make enough milk to treat every person with hemophilia A or B in the world. Dr. Velander also estimates the cost of the treatment would be $2,000 to $10,000 per person each year, much less than the $30,000 to over $300,000 patients are currently paying.

The transgenic pigs are kept in Virginia in a facility where they can be protected from getting any diseases. A recipient of a Hemophilia of Georgia Clinical Scientist Research Grant, Dr. Paul Monahan of the University of North Carolina at Chapel Hill, is one of the researchers who is leading the testing on the animals with hemophilia.

With the NIH grant the researchers hope to be able to have clinical trials within five years. The first tests are being done with factor IX but Dr. Velander says, “Factor VIII is only three years behind the pace set by Factor IX.” He is very optimistic about the research. “While a lot of people with complex diseases are frustrated by the apparent slowness of the development of new therapies, it takes a lot of scientists working a long time to understand the required processes,” Velander said. “Even though the wait is long, there’s hope. There’s a glimmer of hope because we really can foresee clinical trials on this.”

This article contains information from the University of Nebraska-Lincoln press release dated September 12, 2005.