

Myostatin Story reported by Daily Camera.com
Doctors discover a toddler muscle man
By Linda A. Johnson, Associated Press
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Somewhere in Germany is a baby Superman, born in Berlin with bulging arm and leg muscles. Not yet 5, he can hold seven-pound weights with arms extended, something many adults cannot do. He has muscles twice the size of other kids his age and half their body fat. DNA testing showed why: The boy has a genetic mutation that boosts muscle growth.

The discovery, reported in Thursday's New England Journal of Medicine, represents the first documented human case of such a mutation.

Many scientists believe the find could eventually lead to drugs for treating people with muscular dystrophy and other muscle-destroying conditions. And athletes would almost surely want to get their hands on such a drug and use it like steroids to bulk up.

The boy's mutant DNA segment was found to block production of a protein called myostatin that limits muscle growth. The news comes seven years after researchers at Johns Hopkins University in Baltimore created buff "mighty mice" by "turning off" the gene that directs cells to produce myostatin.

"Now we can say that myostatin acts the same way in humans as in animals," said the boy's physician, Dr. Markus Schuelke, a professor in the child neurology department at Charite/University Medical Center Berlin. "We can apply that knowledge to humans, including trial therapies for muscular dystrophy."

Given the huge potential market for such drugs, researchers at universities and pharmaceutical companies already are trying to find a way to limit the amount and activity of myostatin in the body. Wyeth has just begun human tests of a genetically engineered antibody designed to neutralize myostatin.

Dr. Lou Kunkel, director of the genomics program at Boston Children's Hospital and professor of pediatrics and genetics at Harvard Medical School, said success is possible within several years.

"Just decreasing this protein by 20, 30, 50 percent can have a profound effect on muscle bulk," said Kunkel, who is among the doctors participating in the Wyeth research.

Muscular dystrophy is the world's most common genetic disease. There is no cure and the most common form, Duchenne's, usually kills before adulthood. The few treatments being tried to slow its progression have serious side effects.

Muscle wasting also is common in the elderly and patients with diseases such as cancer and AIDS.

"If you could find a way to block myostatin activity, you might slow the wasting process," said Dr. Se-Jin Lee, the Johns Hopkins professor whose team created the "mighty mice."

Lee said he believes a myostatin blocker also could suppress fat accumulation and thus thwart the development of diabetes. Lee and Johns Hopkins would receive royalties for any myostatin-blocking drug made by Wyeth.

Dr. Eric Hoffman, director of Children's National Medical Center's Research Center for Genetic Medicine, said he believes a muscular dystrophy cure will be found, but he is unsure whether it will be a myostatin-blocking drug, another treatment or a combination, because about a dozen genes have some effect on muscles.

He said a myostatin-blocking drug could help other groups of people, including astronauts and others who lose muscle mass during long stints in zero gravity or when immobilized by illness or a broken limb.

Researchers would not disclose the German boy's identity but said he was born to a somewhat muscular mother, a 24-year-old former professional sprinter. Her brother and three other close male relatives all were unusually strong, with one of them a construction worker able to unload heavy curbstones by hand.

In the mother, one copy of the gene is mutated and the other is normal; the boy has two mutated copies. One almost definitely came from his father, but no information about him has been disclosed. The mutation is very rare in people.

The boy is healthy now, but doctors worry he could eventually suffer heart or other health problems.

In the past few years, scientists have seen great potential in myostatin-blocking strategies.

Internet marketers have been hawking "myostatin-blocking" supplements to bodybuilders, though doctors say the products are useless and perhaps dangerous.

Some researchers are trying to turn off the myostatin gene in chickens to produce more meat per bird. And several breeds of cattle have natural variations in the gene that, aided by selective breeding, give them far more muscle and less fat than other steer.