



KU researchers explore human disease through tiny roundworm (图)

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[摘要] Dec. 1, 2006, LAWRENCE — University of Kansas researchers are learning a lot about humans from a tiny roundworm. Researchers commonly use the worm, *Caenorhabditis elegans*, or *C. elegans*, to study a range of human diseases, from Alzheimer's to muscular dystrophy.

[关键词] human disease; roundworm; muscular dystrophy



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Researchers commonly use the worm, *Caenorhabditis elegans*, or *C. elegans*, to study a range of human diseases, from Alzheimer's to muscular dystrophy.

Lisa Timmons, assistant professor of molecular biosciences, uses the worm to study genes that are involved with drug resistance in cancers.

"A number of geneticists use these organisms to discover basic mechanisms found in all organisms, including humans, and to study gene function," said Timmons. "We apply that knowledge directly to the human condition, directly improve it." As most people are surprised to hear, researchers say the worm has a similar body organization to humans. This means their research can eventually be translated to larger organisms for study, including humans.

Erik Lundquist, associate professor of molecular biosciences, says *C. elegans* is a simple organism with 959 cells. It has many of the same genes and gene functions as humans.

"All the cells can be identified unambiguously," said Lundquist. "While they're simple, they have all of the tissues or many of the tissues that we have. So they have neurons, they have muscles and they have skin cells."

Lundquist and Timmons said because *C. elegans* reproduces so quickly, they are able to perform more experiments within a given time frame. To study the molecular basis of disease on mice, for example, would take longer and be more complex.

Specifically, Timmons studies how a cell can stop a gene, or silence it, from performing its normal function, when a molecule called double-stranded RNA is introduced into the cell. This process is called RNAi, for RNA Interference.

The researchers who discovered RNAi did so using *C. elegans* in their work. They were recently awarded the Nobel Prize for the discovery.

"The way to study how a gene functions is to knock out that gene and look at what happens to a cell or organisms in the absence of that gene function," said Timmons.

Timmons said her lab is trying to find the link between this process and the progression or development of certain kinds of cancer.

Lundquist uses the worm to study nervous system development and disorder or disease, such as mental retardation, spinal cord diseases or motor neuron disease.

Through his research with *C. elegans*, Lundquist has found a gene mutation affecting the development of certain neurons in the head of the worm.

The same gene that is affected by this mutation in humans is called Six5. Six5 is associated with a type of muscular dystrophy in humans, called myotonic dystrophy.

"Many of the defects that we've seen in the Six5 mutant worms somewhat mirror the defects in myotonic dystrophy patients," said Lundquist. "Many have mental retardation or anterior brain problems, muscular problems and sterility due to it."

Lundquist said researchers already know the complete sequence of the human genome, but it is more like a parts list than an instruction set.

"That just tells us what parts go into making a human, it doesn't say anything about how those parts interact with each other," he said.

But this line of research using a tiny roundworm will eventually lead to more knowledge about how genes work together to make up the human body.

