



FOR ALL WE CALL

MIZZOU

CAMPAIGN NEWSLETTER

UNIVERSITY OF MISSOURI • WINTER 2008

Leading the fight against a killer of infants

Although many people have never heard of it, spinal muscular atrophy (SMA) is the leading genetic cause of death among infants. The neurodegenerative disease does not discriminate; it affects all ethnic groups and occurs in approximately one in 6,000 live births.

“It’s a remarkably common ‘rare’ disease,” says Chris Lorson, associate professor of veterinary pathobiology. “Most kids who develop the disease live two to five years. Treatments have been developed to extend that lifespan, but there is still no cure.”

Lorson, PhD '97, a researcher in the Christopher S. Bond Life Sciences Center, is leading the national effort to change that. With joint appointments in the College of Veterinary Medicine and the School of Medicine, Lorson has been collaborating with researchers on campus and across the country to learn more about what causes the disease and what can be done to stop it.

“The genetics are very clear,” Lorson says. “A single gene is responsible for all

clinical forms of the disease, and about 1 in 35 people carries the gene.”

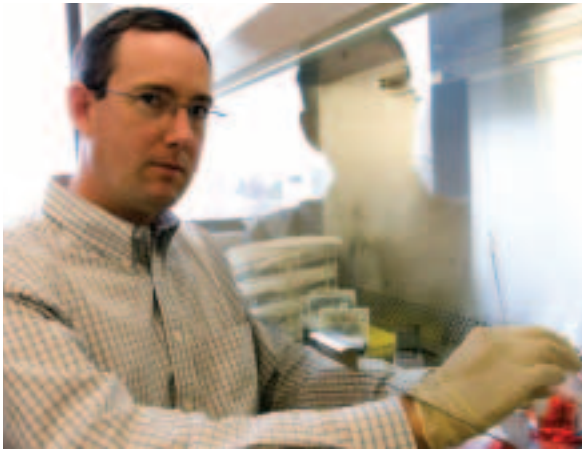
When it functions normally, this particular gene, known as survival motor neuron 1 (SMN1) creates a protein necessary for motor neuron activity. Children born with a defective SMN1 gene suffer from neurodegeneration, which leads to severe

muscle weakness and difficulty crawling, walking, controlling their head and neck, swallowing and even breathing.

Lorson, who is also scientific director for the nonprofit organization Fight SMA, says his team is working on a multifaceted approach that includes drug development, supportive care and a

gene therapy program to replace the defective gene. Their work could offer insights into treating other diseases such as Parkinson’s, Alzheimer’s and muscular dystrophy.

“SMA represents an outstanding platform for modeling neurodegenerative disorders,” Lorson says. “It is truly an exciting time to be working in this area. Obtaining funding, however, has become a full-time job.”



Chris Lorson fights spinal muscular atrophy in and out of the lab. He’s been working with the U.S. Congress to enhance federal support for SMA research.

For All We Call Mizzou is published quarterly to report news of the For All We Call Mizzou campaign to alumni and friends of the University. Direct inquiries to:

David Housh

Vice Chancellor for Development and Alumni Relations

301 Reynolds Alumni Center

Columbia, MO 65211

Telephone: 573-882-7703

E-mail: HoushD@missouri.edu

National Campaign Steering Committee Co-Chairs:

Cynthia Brinkley, BJ '91

Larry McMullen, BA '53, JD '59

William S. Thompson Jr., BS CiE '68

Designer: Dawn Sees

Writers: Sona Pai, Beth Hammock

Photographers: Rob Hill,

Nicholas Benner

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Communication, University Affairs

Winter 2008

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