

SOUTHWESTERN NEWS

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UT SOUTHWESTERN RESEARCHERS BEGIN DRUG STUDY IN HOPE OF FINDING FIRST TREATMENT FOR SPINAL MUSCULAR ATROPHY

DALLAS – March 19 – Puzzled by his weak legs and awkward waddle, orthopaedists in the 1950s suspected that 12-year-old Dick Shlakman had severely shortened Achilles tendons. Their remedy was to put his legs in casts and hope for the best. But the treatment didn't work, and the doctors failed to identify the true culprit — spinal muscular atrophy (SMA), a rare incurable neuromuscular disease.

Though little research has been done on SMA, investigators at UT Southwestern Medical Center at Dallas are offering new hope to patients like Shlakman by initiating a yearlong study of gabapentin, a widely used epilepsy drug. Scientists think the drug may slow the progression of SMA by interfering with the formation of glutamate, a natural substance that can be toxic to nerve cells if overproduced or not processed properly — a condition that may lead to SMA.

"A study like this is long overdue," said Dr. Wilson Bryan, assistant professor of neurology at UT Southwestern. Bryan will head the study at UT Southwestern, which will involve eight other North American universities. "Our team of investigators is very passionate about finding a way to prevent the debilitating effects of SMA, and the only way we can do that is through research and more research," he said.

Investigators are seeking patients willing to participate in the study, which will be funded by the Muscular Dystrophy Association (MDA). Bryan is co-director of an MDA-funded clinic at UT Southwestern. In order to qualify for the clinical trial, patients must be 18 or over, have type II or III SMA (to be confirmed by the trial site's neurologist), have confirmation of their SMA diagnosis through genetic testing, have adequate respiratory function (determined by a test known as forced vital capacity) and be confined to a wheelchair. Participants in the double-blind

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study will receive gabapentin or a placebo.

"This is tremendously exciting for me," said Shlakman, who has volunteered for the study. "Although I would love to see gabapentin reverse the degenerative effects of the disease, the important thing — regardless of the results of this study — is that we keep searching for a treatment."

Gabapentin is manufactured by Parke-Davis and distributed under the name Neurontin.

Individuals who would like to participate or want more information on the SMA study should call Lori Camperlengo, a registered nurse and clinical research coordinator, at (214) 648-9038.

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