Muscular dystrophy: New focus on myoblasts

A controversial scientific report claims that a new type of cell therapy boosts the muscle strength of boys suffering from Duchenne's muscular dystrophy, the most severe form of this muscle-wasting disease. However, the new report already has drawn vocal criticism from a number of scientists who urge people to view the findings with extreme caution.

The study will be published in the May CELL TRANSPLANTATION, a new peer-reviewed scientific journal published by Pergamon Press. The principal investigator, Peter K. Law, left the University of Tennessee in Memphis in January 1991 to start his own privately funded institute, the Memphis-based Cell Therapy Research Foundation.

Scientists now know that people with Duchenne's muscular dystrophy have a defective gene that causes the devastating muscle weakness. The disease mostly strikes males and usually kills them by age 20.

To correct the genetic flaw, Law and other researchers have turned to myoblast-transfer therapy, a treatment in which doctors inject diseased muscles with healthy but immature muscle cells. These cells, called myoblasts, carry the crucial gene for dystrophin, a key muscle protein. The myoblasts fuse with the defective muscle cells, a process that yields the missing dystrophin.

In 1990 Law used that technique to treat Sam Looper, a boy with Duchenne's muscular dystrophy who was 9 years old at the time. After receiving myoblast injections in one big toe, Looper could wriggle his toe more vigorously, Law reported (SN: 6/16/90, p.380).

Now, Law claims more convincing proof of the treatment's efficacy. He and his colleagues injected Looper and 20 other boys who have Duchenne's muscular dystrophy with solutions containing billions of healthy myoblasts. This time, Law injected the cells into 69 major muscle groups in the legs and buttocks.

To prevent rejection of the myoblasts, which are obtained from healthy male donors, the team gave the boys the immunosuppressant drug cyclosporine.

The team documented the strength of each muscle three months prior to the injection period and again three months later. A sampling of preliminary data revealed that 43 percent of the treated muscles increased in strength, 38 percent stayed the same and 19 percent continued to lose strength.

While making no claims for a cure, Law says some of the treated boys experienced a dramatic improvement in muscle power.

Other researchers remain skeptical of Law's report. "There are some concerns with this paper," comments Donald S. Wood, director of science and technology for the Tucson-based Muscular Dystrophy Association (MDA). Wood says that Law's group failed to document each child's long-term decline in muscle strength, raising the possibility that the observed benefit is not real. Although the muscles of boys with muscular dystrophy do deteriorate over time, individual muscles can exhibit a temporary increase in strength even without treatment, Wood observes. Law disagrees, saying that boys show a steady muscle decline after age 6.

Neurologist Robert G. Miller at the California Pacific Medical Center in San Francisco cites another flaw in the study. He says Law's group failed to control for the effect of cyclosporine. Miller's own unpublished research, funded in part by MDA, suggests that cyclosporine temporarily improves muscle strength in boys with Duchenne's muscular dystrophy.

Law counters that his earlier research showed that myoblast transfer, not cyclosporine, produced an improvement in muscle might.

Despite the disagreements on some aspects of the new study, all those interviewed say the future for myoblast transfer remains one of great promise. "All of us working in this field are extremely optimistic," Miller says.

– K.A. Fackelmann

Budding scientists earn top scholarships

Westinghouse Electric Corp.

Thorn *Madras* *Agney*

What do clam shells, tennis balls and a desktop computer have in common? These disparate objects – plus hard work and imagination – helped the top three winners of this year's Westinghouse Science Talent Search earn a total of $90,000 in scholarships.

Last week, Kurt Steven Thorn of Shoreham-Wading River H.S. in Shoreham, N.Y., Claudine Deborah Madras of The Winsor School in Boston and Michael Shayne Agney of Melbourne (Fla.) H.S. accepted first, second and third place in the 51st annual science competition sponsored by Westinghouse Electric Corp. and administered by Science Service, Inc.

Thorn, 16, won the top prize, a $40,000 scholarship, for detecting trace elements in clam shells. A physics teacher helped him gain access to Brookhaven National Laboratory in Upton, N.Y., where he used X-rays from a synchrotron to measure levels of strontium and iron in the clam shells. Thorn found that the levels of these trace elements in the shells correlated with their concentrations in the seawater in which the clams grew.

Second-place winner Madras, 17, won a $30,000 scholarship for her predictions of the rotation rate of the asteroid 951 Gaspra, which the Galileo space probe observed at close range during a flyby last year (SN: 11/23/91, p.326). Madras predicted the egg-like shape of Gaspra months before the Galileo encounter by matching the light-reflection pattern of the real asteroid with those of models she constructed using tennis balls coated with moldable plastic. NASA used her calculations in planning Galileo's photography of Gaspra.

Neural networks were the topic of Agney's third-prize project, which earned him a $20,000 scholarship. The 17-year-old used his own desktop computer to simulate a neural network that directed a simulated robotic arm to "see" and catch a simulated bouncing ball.

Scholarships of $15,000 each went to fourth-place winner Leonid Natanovich Reyzin of Sinai Academic Center in New York City, who simulated walking robots; fifth-place winner Patricia R. Bachiller of Scotch Plains-Fanwood H.S. in Scotch Plains, N.J., who studied bird songs; and sixth-place winner Christopher Marshall Linn Bouton of Saint Ann's School in New York City, who found a protein involved in mammalian heat regulation.

The judges awarded four $10,000 scholarships. Seventh-place winner Erica Beth Goldman of Hunter College H.S. in New York City won hers for discovering that oxygen-starved sea stars turn upside down to breathe. Eighth-place winner Peter Gabriel Khalifah of Shawnee Mission (Kan.) South H.S. received his for developing artificial red blood cells. Benjamin Che-Ming Jun of Montgomery Blair H.S. in Silver Spring, Md., won ninth place for building a computer-controlled room-mapping system. And Robin Ann Niles of Commack (N.Y.) H.S. placed 10th for splicing a nerve-cell protein into liver cells.

Winners were selected from 40 finalists during a week-long visit to Washington, D.C. The remaining 30 finalists won $1,000 scholarships.

– C. Ezzell