

EXCLUSIVE REPORTS

From the September 1, 2000 Print Edition

Myoblast therapy inventor expands research globally

Scott Shepard

30 years ago Peter Law came up with the concept of myoblast cell therapy, and a little over a decade ago he was on TV during the annual Muscular Dystrophy Association Labor Day telethon, as an example of the research that MDA supports with gifts from the public.

Soon after, when Law wanted to push aggressively for more human trials of myoblast on Duchenne Muscular Dystrophy, he was compelled to leave the University of Tennessee, Memphis and set up a private entity, the Cell Therapy Research Foundation. Scientists affiliated with MDA said frequently at the time that Law's results couldn't be replicated by anybody else, and that myoblast was a quaint but archaic concept, soon to be eclipsed by targeted gene therapy.

UT, which jointly owned rights to myoblast with Law, signed away all interests in the technology. At the time, university officials said they believe myoblast was a scientific dead end and they were concerned about liability as Law continued to use and expand human trials.

Today, Law is opening myoblast clinics from countries ranging from China to Sweden, and myoblast culture labs in others. As myoblast is further defined, it has become an accepted treatment for muscular dystrophy and is being researched for other applications, ranging from treatment for hardening of the arteries to adult onset diabetes. Further, Law's global patent on myoblast is regarded as one of the tightest in the world for a biotech product.

"The patent is on the manufacture, use and sale of myoblast, the techniques and the instruments we've developed," Law says. "Any time anybody in the world injects myogenic cells into muscle, it's covered. We have the enabling technology; we have been injecting myoblast into humans for 10 years."

Myoblasts works by culturing immature cells, with complete genetic sequences, and injecting the cells into target muscles. In muscular dystrophy patients, the myoblast cells

fuse with diseased cells and deliver the missing gene. In other applications, the myoblast cells adopt the identity of surrounding cells and then mature.

Injected into heart muscle, for example, myoblasts conform themselves to the characteristics of heart cells.

“Peter is the entrepreneur, the driving force in getting this work done,” says interventional radiologist Scott Williams, medical director of the Medical Education and Research Institute and co-investigator with Law on using myoblast to regenerate heart tissue. The third investigator is interventional cardiologist Joseph Weinstein, with Cardiovascular Physicians of Memphis.

After studying Law’s research, Williams says he immediately saw both the potential and why early results were both erratic and hard to duplicate. Primarily it’s been because Cell Therapy has been working almost entirely alone, without most of the academic support other research has.

“There’s a lot of microscopic details that need to be examined; there’s the technical parameters of how to deliver the cells, the depth of the injections, the tissue, the operating conditions,” Williams says. “There are many, many steps from taking these cells from a petri dish to these cells becoming viable myocytes in the heart.”

“I have no doubt that the technical issues will be worked out,” he says. “The experiments just have to be done.”

Cell Therapy is investing about \$1 million to upgrade its 6,500-square foot surgical suite to meet FDA requirement for human trials. When complete in late October, the suite will have the filtration and sterility standards only otherwise found in orthopedic surgery centers. The improvements are in preparation for Phase III human trials of myoblast for muscle regeneration in both skeletal muscle and the heart.

Other domestic locations lined up for the trials are at Washington University School of Medicine in St. Louis and UT-Chattanooga. Law is still negotiating with other, but hopes to bring the count to 10 sites in the United States.

He’s in the final stages of opening two myoblast centers in South Korea, two in government hospitals in China, plus Malaysia. The Asian centers equipped with myoblast technology, will be supplied with myoblast cells from a central lab in Singapore.

“At the beginning, I didn’t want to put both technologies in the same country,” Law says. “I was afraid that if one country had both technologies too early, they’d kick me out. Outside of the U.S. we have an accepted treatment for muscular dystrophy; the Brazilian government has been paying us for years, as have the Hungarians, the Russians, and the Germans.

Law owns a 95% stake in the 6,000-square-foot myoblast clinic at a hospital in the South China city of Guantong. The other is a hospital in Beijing that serves as the headquarters hospital for the People's Liberation Army.

"The quality of the facility is top of the line, and comparable to any hospital in America," Law says.

In Europe, Law has established a myoblast center in Hungary, and is negotiating sites in Greece, France, and Sweden. Negotiations also are in the works in Mexico and Brazil. All the centers worldwide will have monitors to assure that the clinical procedures meet FDA standards.

"Ideally we would do it all in the U.S., but many MD patients cannot travel here, and this technology will benefit those places in the future," Law says. "These are also human beings who will otherwise go into a wheelchair and die. Parents already go to an extreme in a place like China just to get their child to a center."

Muscle cells are kissing cousins of bone and nerve cells. Since immature cells have all the genetic information they need to become almost any kind of cells, the uses of myoblast technology are wide reaching. In 1995, for example, Danek Medical, Inc. – now part of Medtronic, Inc- licensed the technology involving bone protein, and now is in clinical trials of Bone Morphogenic Protein, a growth factor with potential uses in spinal fusion and other places where accelerated bone growth would be useful.

Clinical trials of BMP are in Phase III study at seven locations and should be concluded by next spring, says Medtronic spokesman Joe McGrath.

"Ideally it is best for muscle, but I truly believe it will have a role down the road in rejuvenating blood vessels," Law says. "A new concept in hardening of the arteries says it is caused by aging with the walls of the blood vessels"

"Instead of going into the leg for an old vein, I believe we'll be able to grow a brand new one," he says.

By the end of the year Law and others expect to publish early findings in the treatment of solid tumors with myoblast. Such tumors require huge amounts of vascularization and oxygen. Like rambunctious, immature children, myoblast cells also have a huge appetite.

"They're so active and they consume so much nutrient that the cancer cells die and the tumor shrinks," Law says. "We are suffocating tumors to the point that they can be removed surgically."

Culturing myoblast with skin cancer cells shows that those cancer cells are also starved by the presence of myoblast.

In treating adult onset diabetes, Law believes it's possible to inject into muscle myoblast cells equipped with insulin receptors, allowing the hormone to react directly with muscle. There's even the potential of culturing extra cells for cosmetic surgery.

"Imagine," Law says, "No more silicone."

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