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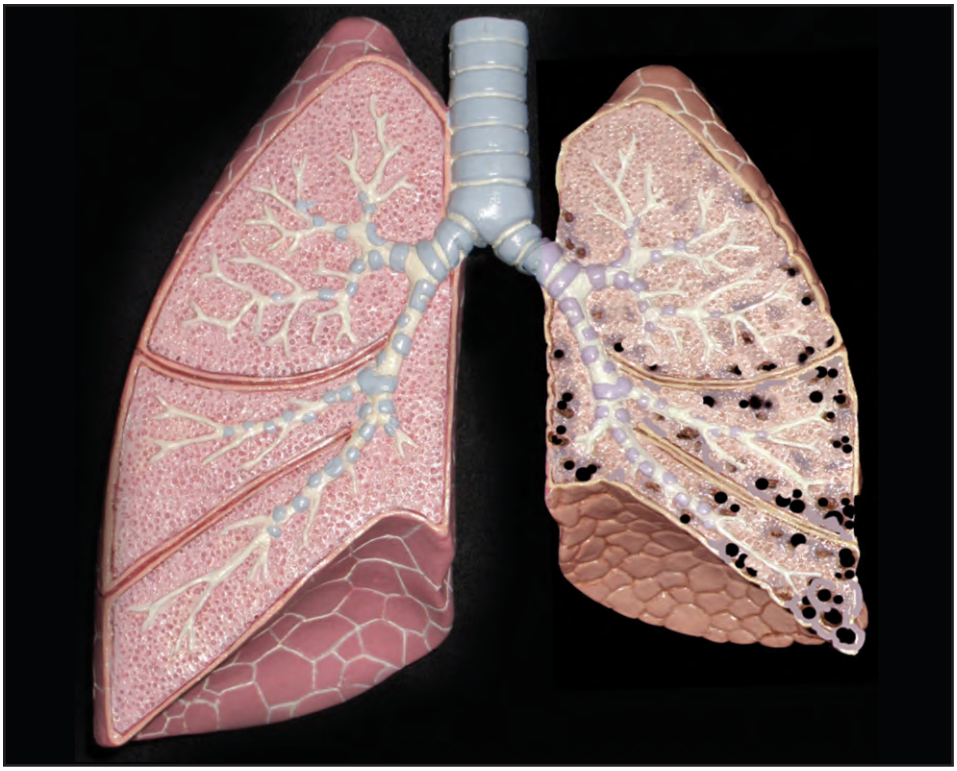
Coalition Works To Reverse Idiopathic Pulmonary Fibrosis 'Death Sentence'

Studies With Stem Cells Prove Positive At Experimental Level



This image compares a healthy lung, at left, with a lung afflicted with idiopathic pulmonary fibrosis. The disease, which kills about 40,000 people each year in the United States, is basically the scarring of the lung. Excess fibrous tissue forms in the lung, reducing the organ's capacity and thus making it more difficult to breathe. (Image courtesy of Dr. Kevin Leslie)

By **Tiffany Rider**
Staff Writer

It starts with a seemingly innocent cough, or maybe shortness of breath. Within a few years – depending on how quickly it's diagnosed and the availability of treatment – a patient with pulmonary fibrosis is dead.

Pulmonary fibrosis (PF), which translates to fibrous growth in the lungs, is a disease that kills just as many patients in the United States as the number of women who die from breast cancer – 40,000 deaths each year – and has no cure. PF is caused by an irritation or scraping of the internal wall of the lungs, such as from breathing in sharp or acidic material. As a reaction to the damage, the body begins to produce scar tissue the same way it would form around a cut on the skin. However, this tissue forms in and around the lungs in an uncontrollable fashion and patients gradually lose the ability to breathe.

Since 1999, the number of patients with PF has increased by 156 percent, according to the Coalition for Pulmonary Fibrosis. A number of things can cause PF, such as: asbestos exposure; mold; bad acid reflux; contact with birds; being in the dust storms of Iraq; or having been a first responder surrounded by the clouds of debris after 9/11. Those causes may be more easily attributed to causing the disease, but many patients diagnosed are idiopathic. Idiopathic pulmonary fibrosis (IPF) is a diagnosis in which doctors cannot determine the exact cause of the disease.

Dr. Arthur F Gelb, a pulmonologist at Lakewood Regional Medical Center, works with about 25 PF patients of all ages at any given time. "It's not a disease you want to wish on anybody," he said. Although the coalition's statistics show that of the number of people diagnosed with PF, only half of them will be alive after three years, Gelb said, "I have a number of IPFs that are going 10 years. My own experience has been that some people do well and go along for a number of years and then they do poorly."

Even so, the story of the disease is dramatic as the patient population continues to grow, according to Mishka Michon, chief executive officer of the Coalition for Pulmonary Fibrosis. "We are in for a huge increase in patient numbers and families affected," she said. "The challenge is to get to treatments with some haste by supporting advocacy and research and sharing the message as broadly as possible. We lose someone every 13 minutes, so we really can't afford to drag our feet."

Michon told the Business Journal that this deadly disease affects 128,000 people each year and that four times as many people have pulmonary fibrosis than have Lou Gehrig's disease or Cystic Fibrosis. PF can strike anyone, typically between the ages of 30 to 80, and more women are being diagnosed with the disease today than ever before. "We're where cancer was 30 years ago. . . .You would rather get a cancer diagnosis than this," she said, noting that even the lung cancer survival rate is better than that of pulmonary fibrosis.

Celebrities Marlon Brando, Robert Goulet and Evel Knievel all died from idiopathic pulmonary fibrosis. In a case more close to home, a former councilmember and vice mayor for Long Beach, Wallace Edgerton, is living with the disease today as the mayor of the City of Menifee. His doctor found the beginnings of pulmonary fibrosis after a CT-scan in 2005.

Doctors classified his form of the disease as idiopathic because he was exposed to multiple potential causes throughout his life. "I worked in Los Angeles when smog was at its worst," Edgerton said, also noting that he grew up across from orchards and breathed in a lot of dust and dirt. In addition, his grandfather worked with asbestos and as a child Edgerton and his friends would play with, and likely breath in, the thin fibrous crystal material.

Edgerton, who served Long Beach for 17 years, had his diagnostic work done at UCLA, and by late 2007 was told he had a rare, slow-growing form of the disease. He was 73 at the time. "He stated that he was confident I would die of something else," he said. Buoyed by a new lease on life, Edgerton decided to run for office for what would be the newly incorporated City of Menifee.

Within a year, however, the disease progressed and he was sent to San Diego Medical Center for treatment. By December 2010, Edgerton was referred to Cedars-Sinai, a nonprofit hospital in Los Angeles, as a possible candidate for a lung transplant. He was cleared to be on the transplant wait list on February 11, 2011, and had a lung within about three weeks. Edgerton said the doctor decided to forego the lung and wait for another. "He just decided not to use it," he said, and was put back on the wait list until he got another call on May 10.

In the very early hours of May 11, Edgerton's transplant was completed. He received the lung of an 18-year-old and said he is thankful to the donor and family for helping save his life. He recently surpassed the three-month milestone in his recovery, and was able to swing dance with his wife again. "At 77 years of age, I am the oldest transplant ever done at Cedars and maybe the oldest in the country," Edgerton said.

Edgerton is fortunate to have received a lung transplant, Michon said, since it is currently the only treatment option to extend survival. But 50 percent of patients waiting for a lung die before they are able to receive a transplant. "A lot of our patients die the day before they get the phone call for the lung," she said. "It's really horrifying."

Research Continues To Find PF Cure

With a disease like PF that hasn't received mainstream attention the way that breast cancer or AIDS has, government organizations consider it rare and fund it less than others. According to the coalition, idiopathic pulmonary fibrosis receives approximately $18 million per year in federal funding, compared to the annual $85 million for cystic fibrosis and $48 million for amyotrophic lateral sclerosis, for finding a cure.

In addition, the U.S. Food and Drug Administration (FDA) has denied approval for a handful of experimental drugs for treating PF, either for poor results or a lack of sufficient test subjects. According to Michon, the coalition has been working with various organizations to help get the FDA to look at drugs that treat rare diseases differently.

"If you have a huge disease, you've got a huge pool of patients. You can test like crazy," Michon said. "If you have a rare disease, to find a number of patients who are at the same stage of the disease, who have the same possible trajectory of disease, it's really hard. You never get a cohort that is as balanced as you would be with a much larger one." Since it takes about 10 times as long to get enough patients tested, Michon said that the National Organization of Rare Diseases and a number of other organizations are working with the FDA to help fast track the testing process for rare diseases.

Mishka Michon is the chief executive officer of the Coalition for Pulmonary Fibrosis. Michon has been working in fundraising her whole life, she said, and now dedicates her talents to raising awareness of pulmonary fibrosis and funding for research to find a cure. (Photograph by the Business Journal's Thomas McConville)

While waiting for the FDA to approve a pharmaceutical treatment, various medical experts are working on experimental treatments to help patients now. Dr. Joyce Lee of the University of California at San Francisco and her colleagues recently published research that shows the use of medication for gastroesophageal reflux disease may improve the survival time of patients with idiopathic PF. Patients who used the drugs had a median survival time of about 65 months, compared to about 29 months for those who did not.

Meanwhile, an interesting feature about this disease is that West Highland White (Westie) terriers get pulmonary fibrosis, Michon said. In theory, because of the short lifespan of a dog, the development of the disease may be much faster. "There is a group of people working on research with them . . . looking at whether this is going to be a way to come out with some questions and answers about what happens in the lungs on a much shorter track," she said.

Dr. Richard Vulliet from the University of California at Davis Veterinary Medicine program, has been working with rodents afflicted with idiopathic PF and treating them with stem cells injected into their tracheas. This process, according to his research, has been shown to reduce collagen deposition and scarring of lung tissue after anti-cancer drug treatment.

More recently, Vulliet has attempted a stem cell experiment on these diseased Westie terriers by injecting the cells into the dog's blood stream. According to his research called Cytotherapeutics in Veterinary Medicine, Vulliet has seen "Good laboratory evidence that this can be repaired." This experiment process is different from other trials happening using stem cells in humans, Michon said. "What they've been doing is mixing stem cells with their cells and then they are actually injecting them into the lung," she said.

The American Journal of Respiratory and Critical Care Medicine published a study in 2003 demonstrating that adult human stem cell transplantation results in spontaneous cell regeneration in damaged lung tissue. "Many of the body's tissues once thought to be locally regenerative may, in fact, be actively replaced by circulating stem cells after hematopoietic or blood-forming stem cell transplantation," according to the study's lead author, Dr. Benjamin Suratt, assistant professor of medicine at the University of Vermont College of Medicine. "This finding is of note not only for its novelty as a regenerative mechanism of the lung, but also for its vast therapeutic implications for any number of lung diseases."

Michon said a doctor in Greece has been performing stem cell experiments with his patients for some time. "What this guy has been doing in Greece, and I think he's the first person in the world to be doing this, is he is treating patients ad-hoc because they were all terminal and very close to the end," she said. "I give this doctor a lot of credit for saying to the patient, 'I can't promise you anything. The government has not approved this, but I am not going to get in trouble,'" she said.

"The Greek government is not like the United States," Michon continued. "No country is like the U.S. If you did that in the U.S., it's like 50 people bang your door down and arrest you. He told the Greek government what he was doing, and they said, 'If these patients agree to it and you don't report any immediate harm from the first experiments, by all means because it's the patient's choice. In this country, you can't do that." ■