Late-breaking clinical trials

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Results from C91 "Late-Breaking Clinical Trials" on Tuesday afternoon, May 17, will bring new light to clinical problems and potential treatments. While five examine possible new therapies for people with asthma (pregnant women), emphysema, lymphangioleiomyomatosis (LAM), TB and chronic obstructive pulmonary disease (COPD), a sixth looks at the safety of physician- vs. nurse-led transport teams for critically ill patients.

In one, researchers from Australia identified a way for pregnant women with asthma to avoid exacerbations. This randomized, controlled trial tested a management algorithm for asthma in pregnancy based on fractional exhaled nitric oxide (FENO) which indicates level of inflammation, and symptoms, and compared this to standard, guideline-based care.

Heather Powell, MMedSci, and colleagues enrolled 242 pregnant asthmatic women before 20 weeks' gestation. They measured FENO, symptoms and lung function at monthly visits. For the women randomized to the algorithm-based treatment, FENO was used to increase or decrease their ICS medications. Long-acting beta agonists were used to treat symptoms when FENO was not elevated.

The researchers found that for every six women receiving treatment adjustment by the FENO-based algorithm, one was prevented from having a severe exacerbation, making FENO-based care an effective way to reduce asthma exacerbations in pregnant women.

Another trial, submitted by Alexis Rames, MD, of Switzerland, investigated the use of a selective retinoid agonist in the treatment of emphysema. Palovarotene reduces inflammation, and promotes structural and functional improvement in animal models of emphysema. This was a two-year Phase-2 double-blind, randomized, placebo-controlled multi-center study to assess the safety and efficacy of a 5 mg/day regimen of palovarotene in 492 patients with cigarette smoke-induced emphysema. In addition to the study treatment, all patients were given standardized inhaled treatment with an inhaled steroid, long-acting bronchodilator and tiotropium. After two years, while there was no significant effect of palovarotene overall, the researchers found that in patients with lower lobe emphysema, palovarotene significantly reduced lung function decline, and may have a disease-modifying effect.

A third study submitted by Erik van Lieshout, MD, of the Netherlands, examined the effect of physician-versus qualified nurse-based critical care transport by ambulance. The researchers prospectively assigned critical care patients to be transported by physician- or nurse-led transport teams and used stored digital monitoring data to determine whether patients underwent critical events during transport. They found that nurse-led transport groups had outcomes that equaled the physician-led groups, suggesting that, at least among less severely critical ill patients, nurse-led transport is a safe option.

In a fourth study, Francis X. McCormack, MD, and colleagues from the University of Cincinnati School of Medicine, conducted a two-year double-blind trial at 13 National Institutes of Health Rare Lung Disease Consortium sites to determine whether sirolimus improves lung function in patients with LAM. Patients were given sirolimus or placebo for the first year and monitored for changes in 6-minute walk distance, serum levels of vascular endothelial growth factor (VEGF-D) and quality of life scores. During the treatment phase, they found a 1 ml/month improvement in FEV1 of treated patients and a decrement of 12 ml in

the placebo group. Over the course of the first year, they found that LAM patients on sirolimus had a mean improvement of 20 ml in their FEV1, whereas the placebo group saw a drop of 130 ml. FVC and serum VEGF-D, Quality of life scores also showed significant changes favoring the treatment group. In the subsequent observation year, both groups showed similar loss of lung function, and there were no significant differences between the groups in terms of serious adverse events. The authors conclude that sirolimus may be useful in certain patients with LAM.

Another study, submitted by Susan Dorman, MD, of Johns Hopkins University, examined the efficacy of rifampin vs. rifapentine in treating TB. The investigators found that rifapentine administered without food was safe and generally well-tolerated as rifampin, and appeared to be as effective.

Finally, a study from Richard Albert, MD, of Denver Health, examined reducing the frequency of acute exacerbations of COPD with a macrolide antibiotic, azithromycin, taken daily for one year in addition to usual therapy. Treatment significantly decreased frequency of exacerbations and improved quality of life, but it caused decrements in hearing in a small fraction of patients.

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