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## TRANSAVE COMPLETES SUCCESSFUL PHASE II CLINICAL PROGRAM FOR ARIKACE IN THE TREATMENT OF PSEUDOMONAS LUNG INFECTIONS

*Final Phase II results further validate ARIKACE™ efficacy and tolerability in the treatment of CF patients and non-CF bronchiectasis patients*

MONMOUTH JUNCTION, NJ, Oct. 17, 2009 – Transave Inc., today reported additional positive clinical trial results on its lead investigational drug, ARIKACE™ (liposomal amikacin for inhalation), an antibiotic that aims to treat chronic lung infections. The latest results along with previously reported data demonstrate significant clinical benefits and complete the company's Phase II program in two indications – for the treatment of lung infections due to the bacterium, *Pseudomonas aeruginosa* in cystic fibrosis (CF) patients and non-CF bronchiectasis patients.

The new data from the Phase II clinical trial program in CF patients with *Pseudomonas* lung infections indicate that ARIKACE, delivered at a dose of 560 mg once daily for 28 consecutive days, demonstrated superior clinical benefit compared to placebo as measured by a significant and sustained improvement in lung function, improvement in patient reported outcomes and reduction in *Pseudomonas* density. In addition, ARIKACE was well-tolerated with overall adverse events comparable to placebo. The Phase II program results were presented today at the 23rd annual North American Cystic Fibrosis Conference in Minneapolis, Minn., by JP Clancy, M.D., of the Children's Hospital of Alabama and the University of Alabama at Birmingham. The Phase II results were from a prospectively designed pooled analysis of two randomized, double-blind and placebo-controlled studies of 105 patients at 33 European and U.S. sites. Preliminary data from a third CF trial which is an on-going open label study where ARIKACE is being dosed in multiple cycles were also presented, showing continued improvement in lung function over repeat cycles.

"We now have consistent positive results from three placebo controlled and three open label studies of ARIKACE in CF and non-CF bronchiectasis patients," said Tim Whitten, Transave's President and Chief Executive Officer. "To date, studies suggest that ARIKACE has the potential to improve upon the standard of care in the treatment of chronic *Pseudomonas* lung infections, and further studies are merited in an effort to bring this medicine to market."

In the U.S., ARIKACE was administered once daily for 28 days at 70 mg, 140 mg and 560 mg doses using a novel inhalation device, the eFlow® Electronic Nebulizer (PARI Pharma GmbH). This study included member institutions of the Cystic Fibrosis Foundation's Therapeutics Development Network. In Europe, ARIKACE was administered once daily for 28 days at 280 mg and 560 mg dosages, also using the eFlow® Electronic Nebulizer. Improved lung function was dose-related with the higher 560 mg dose resulting in the greatest improvement in lung function which was sustained for 28 days after treatment ended. Adverse events reported were consistent with those expected in a population of CF patients receiving inhaled medicine, and there were no differences between groups in the overall rates of adverse events.

"Once-a-day ARIKACE, providing sustained improvement even after therapy ends, suggests that it is possible to treat these stubborn *Pseudomonas* lung infections while reducing the

treatment burden that these patients currently endure,” said Dr. Clancy. “Further, the potential for ARIKACE to penetrate the biofilm and achieve a sustained improvement in lung function would be an important advance in this field.”

Study results have consistently demonstrated significant reduction in *Pseudomonas* bacterial density in both mucoid and non-mucoid strains of *Pseudomonas*. Mucoid strains of *Pseudomonas* are associated with the presence of biofilms. A biofilm is a protective gel-like barrier formed by colonies of the common and often chronic *Pseudomonas* bacteria. The bacterial cells encased within the biofilm are difficult to kill because many antibiotics either stick to the surface of the biofilm and/or are cleared away before they can act on the bacteria inside the biofilm. ARIKACE was designed with small (0.3 micron), neutral liposomes that enable significant drug penetration into the biofilm, which may be an important advantage for improving treatment of lung infections due to *Pseudomonas*.

“ARIKACE development shows great promise for CF patients,” said Robert J. Beall, Ph.D., President and CEO of the Cystic Fibrosis Foundation. “We have supported its development in hopes that trials would demonstrate the potential of ARIKACE to improve lung function and quality of life for CF patients.”

Cystic Fibrosis Foundation Therapeutics, the nonprofit affiliate of the Cystic Fibrosis Foundation, has provided a total award of \$3.9 million to support the development of ARIKACE. The Foundation is the leading organization devoted to curing and controlling cystic fibrosis.

#### **About ARIKACE™**

ARIKACE is a form of the antibiotic amikacin, which is enclosed in nanocapsules of lipid called liposomes. This advanced pulmonary liposome technology prolongs the release of amikacin in the lungs while minimizing systemic exposure. The treatment uses biocompatible lipids endogenous to the lung that are formulated into small (0.3 micron), neutral liposomes that enable penetration of the biofilm. ARIKACE can be delivered through nebulization, which enables the small aerosol droplet size (1 to 5 microns) to facilitate more effective distribution in the lungs. ARIKACE has been granted orphan drug status in the United States by the FDA, and has received an orphan drug designation in Europe by the European Medicines Agency for the treatment of *Pseudomonas* infections in patients with CF. ARIKACE has also been granted orphan drug status by the FDA for the treatment of bronchiectasis in patients with *Pseudomonas* or other susceptible pathogens.

#### **About The Cystic Fibrosis Foundation**

The Cystic Fibrosis Foundation, the leading organization in the United States devoted to curing and controlling cystic fibrosis, has invested more than \$320 million in drug research with biotech companies since 1998 to develop therapies to fight CF. As a result, the Foundation has built a drug pipeline with more than 30 promising therapies in development. Virtually every approved CF therapy available today was made possible because of the support of the Foundation. Based in Bethesda, MD, the Foundation has 80 chapters and branch offices, and supports and accredits a nationwide network of 110 CF care centers that provide treatment and vital resources to patients and families. For more information, visit [www.cff.org](http://www.cff.org).

### **About PARI Pharma and the eFlow® Electronic Nebulizer**

ARIKACE is delivered by an eFlow® Nebulizer System developed by PARI Pharma GmbH. The eFlow® Nebulizer System uses eFlow Technology to enable highly efficient aerosolization of medication including liposomal formulations via a vibrating, perforated membrane that includes thousands of laser drilled holes. Compared to other nebulization technologies, eFlow® Technology produces aerosols with a very high density of active drug, a precisely defined droplet size, and a high proportion of respirable droplets delivered in the shortest possible period of time. Combined with its silent mode of operation, small size (it fits in the palm of the hand), light weight, and battery use, products incorporating eFlow® Technology reduce the burden of taking daily, inhaled treatments. The eFlow® Nebulizer System and eFlow® Technology are proprietary to PARI Pharma and can be optimized to specific drug formulations.

### **About Transave, Inc.**

Transave, Inc., is a biopharmaceutical company focused on the development of innovative inhaled pharmaceuticals for the site-specific treatment of chronic lung diseases. The company's major focus is on developing antibiotic therapy delivered via proprietary advanced pulmonary liposome technology in areas of high unmet need in lung diseases. The Transave team is dedicated to leveraging its development and commercialization expertise, along with its intellectual property, to bring life-extending and life-enhancing medicines to patients. For more information about Transave's technology and development programs, visit <http://www.transaveinc.com/>.