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# Cystic Fibrosis

# Update

**Cystic Fibrosis Update** is a regular newsletter featuring pertinent and interesting information related to cystic fibrosis care. The information presented reflects the view of the editor alone and does not represent medical advice. Please contact your physician to discuss any of the issues raised before starting any new therapies.

## Highlights from the 23rd NACF Conference

As I returned from Minneapolis from this year's annual CF conference, I had a chance to reflect on the past 17 years in terms of where we have come from. In 1992, I attended my first CF conference having just completed my medical training and everything presented at the conference sounded complicated and quite overwhelming. 17 years later, a little bit less overwhelmed but I am amazed at the progress that has been made. I find the new and novel ideas that my colleagues are trying to develop to be very exciting. Some new ideas meet up with a dead end but some make it through the process. As you read the following, keep in mind how a new therapy has to be created, undergo early proof of concept that it might actually work, and then journey through the stages of a clinical trial. Here is my top 10 list from the 2009 meeting.

**10. Human Alpha 1 Antitrypsin (Kamada, Israel):** This phase II study attempted to treat the imbalance that occurs in the inflammation response in the lungs of people with CF. This nebulized therapy with eFlow for 28 days was well tolerated with damaging chemicals and inflammatory white blood cells becoming significantly reduced. Novel and promising.

**9. Nebulized Amikacin (Amikace) (Transave Inc, New Jersey):** Amikacin is a widely used antibiotic belonging to a group called aminoglycosides (TOBI is another) with effectiveness against *Pseudomonas aeruginosa*. A special preparation made for eFlow delivery was evaluated in a Phase II study over a 28 day period. Results are promising with improved lung function and excellent tolerance to therapy.

**8. Overnight High Frequency Chest Wall Oscillations (The VEST):** Dr. Ran Anbar, CFI's friend and conference speaker from the 2007 Winter Retreat, conducted a trial using The Vest overnight during sleep. Out of 30 patients, 22 reported they were

able to do so. Preliminary data suggests that for some, improvement in lung function is noted. This type of study is a great example of optimizing what is already currently available for patient use.

**7. Nebulized Ceftazidime for Burkholderia Cepacia (Royal Brompton Hospital, UK):** While the off label use of this IV medication has been used for years, it makes the top 10 list because it is one of the few reports of using existing antibiotics to help fight *B.cepacia*—a bacteria associated with a marked deterioration in life span. In the 15 patients they studied, 11 showed improvement as measured by standard lung function testing.

**6. KB001 (KaloBios, California):** When you see the medicine name as initials and a number, you know that it is in early development. This pilot study looked at a new agent called KB001. This chemical is directed against a product made by *Pseudomonas aeruginosa* felt to cause marked lung damage. 24 patients received a single administration of one of two intravenous doses. At 8 weeks follow-up, both doses were well tolerated and there was a trend for reduction in markers of inflammation were seen as well as a trend towards reduction in *Pseudomonas aeruginosa*. Very preliminary but novel.

**5. Aerosolized Azithromycin (Zithromax)** Although limited to healthy subjects, the ability to attempt to nebulize pre-existing therapy always seems to be the way to go. This study showed the feasibility of creating a nebulized preparation of Zithromax.

**4. Inhaled Dry Powder Mannitol (Bronchitol) (Pharmaxis Lts, Australia):** This phase III trial evaluated 295 people with CF. Mannitol is an osmotic agent that increases water content of airway surface fluid. This dry powder inhalation was given twice a day over 26 weeks; appeared well tolerated with cough as the main side effect. Lung function improved significantly (6%) at 26 weeks. Very Promising.



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