ARIZONA PEDIATRIC PULMONARY CENTER

# NEWSLETTER

## FDA EXPANDS APPROVED USE OF KALYDECO TO TREAT ADDITIONAL MUTATIONS OF CYSTIC FIBROSIS

The U.S. Food and Drug Administration today expanded the approved use of Kalydeco (ivacaftor) for treating cystic fibrosis. The approval triples the number of rare gene mutations that the drug can now treat, expanding the indication from the treatment of 10 mutations, to 33. The agency based its decision, in part, on the results of laboratory testing, which it used in conjunction with evidence from earlier human clinical trials. The approach provides a pathway for adding additional, rare mutations of the disease, based on laboratory data.

"Many rare cystic fibrosis mutations have such small patient populations that clinical trial studies are not feasible," said Janet Woodcock, M.D., director of the FDA's Center for Drug Evaluation and Research. "This challenge led us to using an alternative approach based on precision medicine, which made it possible to identify certain gene mutations that are likely to respond to Kalydeco.

Results from an in vitro cell-based model system have been shown to reasonably predict clinical response to Kalydeco. When additional mutations responded to Kalydeco in the laboratory test, researchers were thus able to extrapolate clinical benefit demonstrated in earlier clinical trials of other mutations. This resulted in the addition of gene mutations for which the drug is now indicated.

Kalydeco, available as tablets or oral granules taken two times a day with fat-containing food, helps the protein made by the CFTR gene function better and as a result, improves lung function and other aspects of cystic fibrosis, including weight gain. If the patient's genotype is unknown, an FDA-cleared cystic fibrosis mutation test should be used to detect the presence of a CFTR mutation followed by verification with bi-directional sequencing when recommended by the mutation test instructions for use.

Cystic fibrosis is a rare disease that affects about 30,000 people in the United States. Kalydeco is indicated for patients aged 2 and older who have one mutation in the CFTR gene that is responsive to drug treatment based on clinical and/or in vitro (laboratory) data. The expanded indication will affect another 3 percent of the cystic fibrosis population, impacting approximately 900 patients.

## CYSTIC FIBROSIS CAREGIVER SUPPORT GROUP

Our CF Caregivers Support Group continues to meet once a month and is open for all caregivers to attend. It is a positive, uplifting group that meets to hang out, discuss relative topics for CF caregivers, and swap information and ideas. Dinner is include!

The group meets one Monday a month from 6-8PM. The meeting dates for the year are as follows:

July 17, 2017
August 21, 2017
September 18, 2017
October 16, 2017
November 20, 2017
December 18, 2017

**Please note there is no support group meeting in June.

Meetings are held at Playformance -
119 E. Toole Ave
Tucson, AZ 85701

If you want more information or to RSVP, please contact Anna:
anna@plyformancetucson.com
520-850-5218

Check out the Facebook page for updates: https://www.facebook.com/groups/272827716217202/

ARIZONA® PEDIATRIC PULMONARY CENTER

## ONLINE RESOURCES

**www.uappc.peds.arizona.edu**
The Pediatric Pulmonary Center's website

**www.CysticLife.org**
A social network for the cystic fibrosis community

**www.CFF.org**
The Cystic Fibrosis Foundation's website

## CONTACT INFORMATION

**Medical Questions**
(520)694-5132

**Newsletter Questions**
(520)626-2962

The University of Arizona
Pediatric Pulmonary Center
1501 N Campbell Avenue
Tucson, AZ 85724-5073
520-626-2962/Fax 520-626-5942
0709-3014450 PPC

# CF EDUCATION DAY A HUGE SUCCESS

On Saturday April 29th, the UAPPC hosted a CF Family Education Day. This educational day was planned and facilitated by our CF Patient and Family Advisory Committee (CFPFAC). The agenda included clinic updates, vendors, a parent panel question and answer session, and Ronnie Sharpe, from CysticLife, as our keynote speaker.

Evaluations provided positive feedback on all components, with high ratings on the parent panel and keynote speaker. We also were able to provide Spanish translation services for our Spanish speaking parents, and live stream the event for those unable to attend in person.

We had a great turn out with 43 attendees, including parents, speakers, and vendors. We look forward to our next CF education day in the spring of 2018. Thank you to all who attended and helped make this a great education day for families!