

The Charlotte CF Family Advisory Board (FAB) is a group of Cystic Fibrosis parents and caregivers who provide feedback and partner with the Atrium Health CF Care Team to improve patients' and families' experiences and care during office visits and at the hospital. The FAB is committed to enhancing medical care and quality of life for children and adults with Cystic Fibrosis and their families. Our CF Seasonal Newsletter is a tool for you to know what's happening at your CF center and hospital, and to discover upcoming educational and connection opportunities.

FDA Approves Symdeko[®] for Children Ages 6 to 11

ARTICLE FROM CFF.ORG • June 21, 2019

The U.S. Food and Drug Administration (FDA) has approved the use of tezacaftor/ivacaftor (Symdeko[®]) for children with cystic fibrosis ages 6 to 11 who have two copies of the F508del mutation or a single copy of one of 26 specified mutations. An additional 2,000 children will now qualify for the CFTR modulator, which was first approved in 2018 for people with CF ages 12 and older.

Tezacaftor/ivacaftor provides an alternative option for people with qualifying mutations, including individuals who cannot tolerate the CFTR modulator lumacaftor/ivacaftor (Orkambi[®]).

The drug is a combination of two CFTR modulators which work in tandem to improve the function of the CFTR protein. It works by enabling the protein to form the right shape and allowing it to move to the surface of cells lining the lungs and other organs. With a functioning protein, chloride is then able to flow in and out of the cells, thereby reducing the symptoms of CF.

"We are pleased that more children with CF will have an additional modulator which will allow them to benefit from a treatment for the underlying cause of their disease," said Michael P. Boyle, MD, senior vice president of therapeutic development for the Cystic Fibrosis Foundation. "This is an important step forward, as research has shown that taking modulators at a young age has the potential to slow or even prevent the progression of the disease."



What Are the Different Types of CFTR Modulators?

Drugs that target the underlying defect in the cystic fibrosis transmembrane conductance regulator (CFTR) protein are called CFTR modulators. The three main types of modulators are potentiators, correctors, and amplifiers.

Potentiators

The first type of CFTR modulator is called a "potentiator." Potentiators help chloride flow through the CFTR protein channel at the cell surface. The CFTR protein is shaped like a tunnel that can be closed by a gate. Potentiators hold the gate open so chloride can flow through. The drug ivacaftor (Kalydeco[®]) is a potentiator. This drug can help patients with gating and conduction mutations in CFTR. It also works on residual function and splice mutations where an insufficient amount of normal protein is present. In all of these mutations, some CFTR protein reaches the surface of the cell. However, either not enough protein reaches the cell surface, or the protein does not allow enough chloride to flow through. By holding the gate on the CFTR protein open, potentiators allow more chloride to flow through and reduce the symptoms of CF.

(Continued on Next Page)

High Calorie Options for Back-to-School (or anytime!)

By Julie Matel, RD

ARTICLE FROM STANFORD CYSTIC FIBROSIS CENTER NEWS • Summer 2012

Maintaining adequate weight gain and growth is essential for children with CF in order to prevent declining lung function. Achieving a high calorie high fat diet is recommended to support this goal.

Heading back to school in the Fall can present challenges when it comes to offering high calorie options. Mornings are often hectic for families to fit in time for necessary respiratory treatments as well as ensuring a healthy high calorie breakfast. Once kids head off to school it can be difficult to consume enough calories with shortened lunch times and the need to fit in enzymes. Try these tips and aim for a goal of 3 high calorie meals and 3 high calorie snacks each day.



BREAKFAST

Breakfast is indeed the most important meal of the day. Try these quick and healthy high calorie suggestions to start the day. If time does not allow for a leisurely meal at the breakfast table, many of these items can be eaten on the way to school. Just remember to take along enzymes for the ride!

- Breakfast cookie
- Peanut butter banana smoothie
- Bagel with butter, cream cheese, or peanut butter
- Peanut butter and jelly sandwich on raisin toast
- Toaster waffle sandwich (try cinnamon toaster waffles with peanut butter and bananas)
- Trail mix (mix of nuts, raisins or dried cranberries, and chocolate chips)
- Toasted cheese and apple sandwich
- Oatmeal with added brown sugar, half and half, dried fruit and/or nuts
- Breakfast burrito (can add scrambled eggs, ham, cheese, veggies if desired and roll in a tortilla)
- Muffins (blueberry or any fruit/nut variety)
- Send a thermos of Carnation Instant Breakfast® and whole milk and add to any of the above breakfast ideas to provide more calories

MORNING SNACK

Take advantage of the time allotted to eat at morning recess by packing a designated snack in your kid's lunch each day. Kids are not given much time at their midday break to both eat a full lunch and play during recess, making it even more important to include a morning snack. The following options include healthy high calorie items that can be eaten quickly.

- Supplements such as PediaSure® or Boost Kids Essentials® -- try the higher calorie 1.5 versions (can pack these beverages in a thermos to keep cold or to help kids feel less self-conscious)
- Granola bar or oatmeal cookies and chocolate milk
- Pudding cup
- Full fat yogurt and sliced fruit or raisins
- Cheese stick and crackers
- Mini bagels with cream cheese

LUNCH

Kids may only get 15 to 20 minutes for lunch at school, by the time they leave their classroom and head for the lunch room. Kids are often in a hurry to finish their lunches during this time frame so they have time to play before returning to class. Talk to your child's school about allowing extra time for your child to finish his lunch or allowing him to finish his lunch in class. Lunch monitors can be enlisted to help encourage your child to eat, which may help with the urge for kids to ditch their lunch in the trash. Check with your school regarding their policy for medications. It may save time if your child can be allowed to keep her enzymes with her at the lunch table rather than going to the office. If a trip to the office is required, check to see if your child can be dismissed for lunch a few minutes earlier. Ask your child to help you with grocery shopping for high calorie items and have them help pack lunches the night before. Helping with shopping and food preparation can help him have a vested interest in eating lunch the next day. Check out the list below for some high calorie lunch ideas. Remember to pack perishable items with a cold/hot pack.

- Chicken or tuna salad on dense wheat bread or a croissant for extra calories
- Opt for full-fat yogurts and/or chocolate milk. Freeze the night before. By lunch time they will be the right temperature.
- Send Mac and cheese (make with half and half instead of milk) or cream based soups in a thermos
- Almond butter, honey and banana sandwich in pita bread
- Include carrots with hummus or ranch dip (look for single serving options in your grocery store)
- Try dried fruit for a higher calorie option in place of fresh fruit
- Remember an extra slice of cheese and plenty of mayonnaise added to lunch meat sandwiches will add extra calories

Achieving a high calorie lifestyle during the school year can be a daunting task! Aim for 3 healthy high calorie meals and snacks each day. Remember to involve your child in grocery shopping and in lunch preparation. Ask your child's school for help in ensuring that he has the opportunities needed to ensure successful enzyme use and adequate time for snacks and lunch.

What Are the Different Types of CFTR Modulators? (cont.)

Correctors

The next type of CFTR modulator is called a “corrector.” Correctors help the CFTR protein to form the right 3-D shape so that it is able to move -- or traffic -- to the cell surface. The corrector drugs lumacaftor and tezacaftor help the CFTR protein to form the right shape, traffic to the cell surface, and stay there longer. But, even with lumacaftor and tezacaftor, only about a third of the CFTR protein reaches the cell surface, so by itself it can't reduce the symptoms of CF. Additionally, the proteins that do reach the cell surface do not open sufficiently to allow chloride to pass out of the cell. But, if a corrector is used in combination with a potentiator -- such as ivacaftor -- to hold the gate on the CFTR protein open, enough chloride can then flow to reduce the symptoms of CF. The combinations of lumacaftor/ivacaftor (Orkambi®) and tezacaftor/ivacaftor (Symdeko®) are therefore used to treat people with two copies of the F508del mutation. (Tezacaftor/ivacaftor also can be used to treat people with a single copy of one of 26 specified mutations -- regardless of their other mutation.)

Amplifiers

The last type of CFTR modulator is called an “amplifier.” Amplifiers increase the amount of CFTR protein that the cell makes. Many CFTR mutations produce insufficient CFTR protein. If the cell made more CFTR protein, potentiators and correctors would be able to allow even more chloride to flow across the cell membrane. Amplifiers, which are being developed and tested, are not yet available.

Summer Hydration Recipes

Lemon-Lime Sports Drink

¼ cup of fresh squeezed lime juice
¼ cup fresh squeezed lemon juice
1½ -2 cups filtered water (or coconut water)
1/8 teaspoon of sea salt
1-2 tablespoons sugar/honey/agave/maple syrup (to taste)
Blend together

Watermelon Coconut Water

1 cup watermelon, cubed
1 cup coconut water
Fresh lime juice to taste
1/8 teaspoon salt
Blend together

Strawberry Recovery Drink

2 cups filtered water
½ cup orange juice
2 teaspoons honey/sugar/agave (to taste)
¼ teaspoon salt
Approximately 10 medium strawberries
Mix all ingredients in a large container and chill in the fridge for at least 5 hours (overnight is preferred). Strain liquid to remove the berries, or puree if you prefer a thicker drink. Enjoy!

SAVE THE DATE

CF Education Night

Wednesday, July 24 • 6:30-8:00pm
Gorelick Auditorium • Levine Children's Hospital

CF Education Nights are an opportunity for caregivers in our local CF community to connect with each other while expanding their knowledge on various aspects of CF care.

Our Summer topic is CF and School. The presentation will include: understanding the difference between an IEP and a 504 (with sample plans for you to review), suggestions on discussing CF with your child's school and teacher, and practical ideas for managing CF during the school day. We will focus on different age groups and how each segment manages CF at school: Preschool (0-5), Elementary (5-10), and Older Children / Self-Management (11-17+). The evening will wrap up with Q&A and breakout sessions.

Virtual Option available.

LEARN MORE & RSVP ONLINE:
charlotteCFfamilies.org/education-nights

FUN EASY RECIPE

NUTELLA BANANA SHAKE

INGREDIENTS

8 oz whole milk*
2 Tbsp Ovaltine
1 banana
2 Tbsp Nutella
Ice

DIRECTIONS

- (1) Place all ingredients in a blender.
- (2) Pulse until desired consistency is reached.
- (3) Pour into a cup and enjoy!

NUTRITION

Makes one serving. Calories:471 | Fat: 25g
Sodium: 119mg | Protein: 15g | Calcium: 297mg

**For even more calories, try with
half & half or heavy cream!*

Financial Assistance Programs & Resources

CF Foundation Compass

Personalized insurance, financial, and legal service
Call 844-COMPASS (844-266-7277) M-F, 9 a.m. until 7 p.m. ET
compass@cff.org

HealthWell Foundation

Financial support for co-pays when health insurance is not enough.
healthwellfoundation.org/fund/cystic-fibrosis-treatments
healthwellfoundation.org/fund/cystic-fibrosis-vitamins-supplements

AbbVie: CREON® CF Care Forward Patient Support Program

Offers nutritional services to eligible patients, as well as financial and educational support for patients and families.
creon.com/CFCareForward, 1-855-227-3493

Allergan: Zenpep Live 2 Thrive

Offers copay assistance, free vitamins and supplements, and nutritional information for eligible patients.
live2thrive.org 1-888-936-7371

Chiesi USA: BETHKIS® (Tobramycin Inhalation Solution) and

PERTZYE® (pancrelipase) 1-888-865-1222
patient.bethkis.com/savings-support
pertzycf.com/chiesi-caredirect

Genentech, Inc.: Pulmozyme® Access Program

genentechaccesssolutions.com/portal/site/AS/
1-866-4-ACCESS

Gilead: Cayston® Access Program

cayston.com/programs/cayston-access-program
1-877-7CAYSTON (877-722-9786)

Novartis Pharmaceuticals: Tobi Nebs and Podhaler

Co-pay assistance program:
1-877-999-TOBI (8624).

PARI: Kitabis (Tobramycin)

kitabis.com/patient-access

Vertex Pharmaceuticals

Vertex GPS: Guidance & Patient Support
(Kalydeco® or ORKAMBI®)
vertexgps.com
1-877-752-5933

Abbott Nutrition Patient Assistance Program

866-801-5657.



The Carolinas Chapter - Charlotte Office of the Cystic Fibrosis Foundation has many opportunities for you to get involved. Your participation in any way will help the CFF achieve their mission – finding a cure for all people with CF. For complete list of fundraising events & volunteer opportunities: charlotte.cff.org



Charlotte CF Cycle for Life 2019

Get your wheels in motion and join us for the 8th Annual CF Cycle for Life (CFL)! We will be starting and ending the ride at The Foundry, right beside Bank of America Stadium, and riding through the cycle friendly outskirts of Charlotte. CFL is a fully-supported ride with 15-mile, 35-mile, and 50-mile route options. **LEARN MORE: cycle.cff.org**

WHEN: August 3, 2019 @ 6:30am

WHERE: The Foundry/Draught Restaurant & Bar



Rocktoberfest 2019

Rockin' for a Cure has been the most ROCKIN' event in town for 13 years, and now as Rocktoberfest it's the same great event, just with MORE beer and MORE food! Can you say yum? **LEARN MORE & BUY TICKETS: events.cff.org/rocktoberfest**

WHEN: September 21, 2019 @ 6:30pm

WHERE: The Fillmore Charlotte

CONNECT WITH CHARLOTTE CF FAMILIES

Be the first to know about upcoming Charlotte CF Families events and Atrium Health CF Program announcements. Join our email list at: charlotteCFfamilies.org/stay-connected



charlotteCFfamilies.org



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