CHOC
Specialty Pharmacy Services

Treatment Overview for Cystic Fibrosis

When a child or adolescent is diagnosed with cystic fibrosis (CF), the entire family is affected. That's why at CHOC, we specialize in providing the very best CF treatment and care to ensure your child lives a long, healthy life.

CF is an inherited disease in which the body makes very thick, sticky mucus. The mucus causes problems in the lungs, pancreas and other organs. In people with CF, changes in the cystic fibrosis transmembrane conductance regulator (CFTR) gene prevent water from getting to certain cells. This causes the mucus in some organs to become thick and sticky.

While there is no cure for CF, the goals of treatment are to ease symptoms, prevent and treat complications, and slow the progress of the disease.

The main treatments of CF are:

- Airway clearance to help loosen and get rid of the thick mucus that can build up in the lungs.
- Inhaled medicines to open the airways or thin the mucus. These are liquid medicines that are made into a mist or aerosol and then inhaled through a nebulizer.
- Pancreatic enzyme supplement capsules to improve the absorption of vital nutrients. These supplements are taken with every meal and most snacks. People with CF also usually take multivitamins.
- An individualized fitness plan to help improve energy, lung function, and overall health.
- Medications called CFTR modulators to target the underlying cause of cystic fibrosis. There can be different kinds of changes to the CFTR gene and the medications that have been developed so far are effective only in people with specific changes

Specialty CF Medications at CHOC

CFTR Modulators

KALYDECO™ (Ivacaftor)

Kalydeco™ is a CFTR modulator used for the treatment of CF in children and adults who have at least one mutation in their CF gene that is responsive to Kalydeco.
Patient Package Insert: https://pi.vrtx.com/files/patientpackageinsert_ivacaftor.pdf

ORKAMBI™(lumacaftor/ivacaftor)

Orkambi™ is a CFTR modulator used for the treatment of CF in children and adults who have two copies of the F508del mutation in the CFTR gene.
Patient Package Insert: https://pi.vrtx.com/files/patientpackageinsert_lumacaftor_ivacaftor.pdf

SYMDEKO™ (tezacaftor/ivacaftor)

Symdeko™ is a CFTR modulator used for the treatment of CF in children and adults who have two copies of the F508del mutation, or who have at least one mutation in the CFTR gene that is responsive to treatment of Symdeko.
Patient Package Insert: https://pi.vrtx.com/files/patientpackageinsert_tezacaftor_ivacaftor.pdf

TRIKAFTA™ (elexacaftor/ivacaftor/tezacaftor)

Trikafta™ is a CFTR modulator used for the treatment of CF in children and adults who have at least one copy of the F508del mutation in the CFTR gene of another mutation that is responsive to the treatment of Trikafta.
Patient Package Insert: https://pi.vrtx.com/files/patientpackageinsert_elexacaftor_tezacaftor_ivacaftor.pdf

Inhaled Medications

CAYSTON™ (Aztreonam Nebulization Solution)

Cayston is an antibiotic that is used with a nebulizer to treat people with cystic fibrosis who have a lung infection with a certain type of bacteria (Pseudomonas aeruginosa). The medication is usually taken on a 28-day cycle using a special type of nebulizer.

How to give this medicine:

1. Make sure the handset is on a flat, stable surface.
2. Remove the rubber stopper from the vial, then pour all the mixed CAYSTON and saline into the Medication Reservoir of the handset (Figure 1). Be sure to completely empty the vial, gently tapping the vial against the side of the Medication Reservoir if necessary. Close the Medication Reservoir (Figure 2).
3. Begin your treatment by sitting in a relaxed, upright position. Hold the handset level and place the Mouthpiece in your mouth. Close your lips around the Mouthpiece (Figure 3).
4. Breathe in and out normally (inhale and exhale) through the Mouthpiece. Avoid breathing through your nose. Continue to inhale and exhale comfortably until the treatment is finished.
5. The empty vial, stopper and saline ampule should be thrown away in household garbage when finished.

FIGURE 1

FIGURE 2

FIGURE 3

Patient Information:
www.gilead.com/~/media/files/pdfs/medicines/respiratory/cayston/cayston_patient_pi.pdf

COLISTIN NEBULIZATION SOLUTION

This medication is an antibiotic that is used to treat people with cystic fibrosis who have a persistent lung infection with resistant bacteria (Pseudomonas aeruginosa or Stenotrophomonas). The medication is usually taken on a 28-day cycle.

How to give this medication:

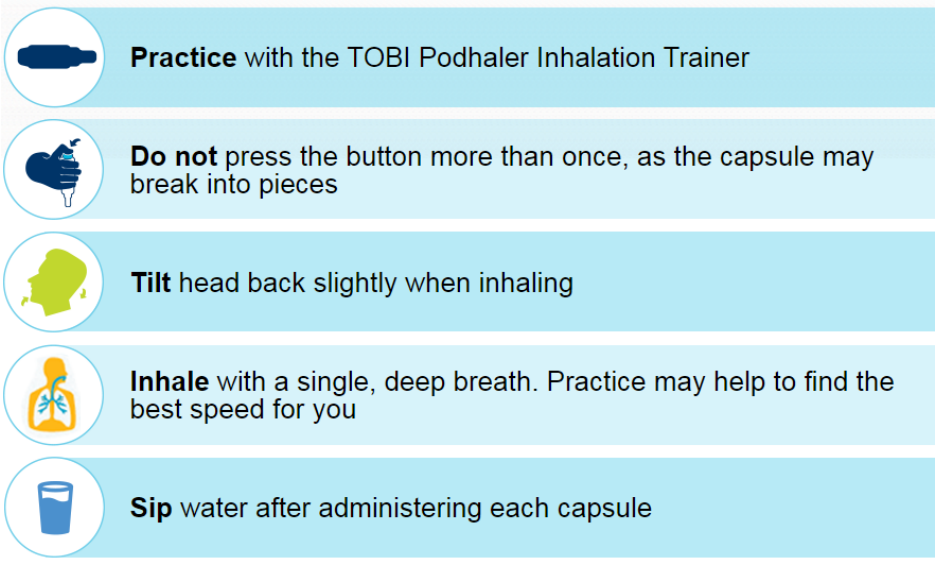
- Colistin comes as a powder in small vials which needs to be mixed with sterile water before giving it. You will be shown how to make up the solution and how to nebulize it.

TOBI PODHALER™ (Tobramycin Inhalation Powder)

This medication is the powder form of the inhaled antibiotic tobramycin. It is used to treat people with cystic fibrosis who have a lung infection with a certain type of bacteria (Pseudomonas aeruginosa). The medication is usually taken on a 28-day cycle.

How to give this medication:

- Capsules are for oral inhalation only
- Each dose of 4 capsules should be taken as close to 12 hours apart as possible. Do not take doses less than 6 hours apart.



Patient Information: https://dailymed.nlm.nih.gov/dailymed/fda/fdaDrugXsl.cfm?setid=625a4499-4e46-4f5a-8d0c-d104f520d97e&type=display#section-17

TOBRAMYCIN NEBULIZATION SOLUTION

This medication is an antibiotic that is used with a nebulizer to treat people with cystic fibrosis who have a lung infection with a certain type of bacteria (Pseudomonas aeruginosa). The medication is usually taken on a 28-day cycle.

How to give this medication:

- Give using a hand-held nebulizer cup reusable nebulizer with a compressor over about 15 minutes.
- Doses should be taken as close to 12 hours apart as possible. Do not take doses less than 6 hours apart.

Patient Information: https://dailymed.nlm.nih.gov/dailymed/drugInfo.cfm?setid=dad13445-f591-4829-96f9-1598ba03dff8&audience=consumer

PULMOZYME™ (Dornase Alfa)
Pulmozyme is a medication that is used with a nebulizer to help break down the excess pulmonary secretions in children and adults with cystic fibrosis.

How to give this medication:

At a glance:

How to Take Pulmozyme

***Remember:** Pulmozyme must be stored in its protective foil pouch in the refrigerator at 2-8°C (36-46°F). This helps to protect Pulmozyme from excessive heat and strong light.*

Sanitize your hands, work area, and equipment to help prevent the spread of infection.
- Wash your hands to reduce the risk of infection
- Clean the table surface before you lay out your nebulizer and compressor pieces
- Clean your nebulizer and tubing after every use and disinfect once a day
  – *Always follow the manufacturer's instructions for how to clean your equipment*

Test the compressor.
- You should be able to feel air flowing from the "air out" port if it is working properly
- Turn off compressor

Inspect the ampule of Pulmozyme.
- Remove 1 ampule of Pulmozyme from its pouch
- Do not use Pulmozyme if it is expired, cloudy or discolored, or leaking

Connect the compressor to your nebulizer.
- The tubing is attached to the "air out" port that you tested on the compressor
- Connect the other end of the tubing to the port on the bottom of the nebulizer by pushing up firmly

Pour entire contents of Pulmozyme ampule into the nebulizer.
- Twist off the top of the ampule by firmly holding the tab located at the base — avoid squeezing the ampule at this time
- Turn the ampule upside down and then squeeze its entire contents into the nebulizer cup; snap the cap on to the nebulizer cup and throw away the empty ampule
- Attach the mouthpiece to the nebulizer

Take your medicine by inhaling the mist through your mouth.
- Turn on the compressor and make sure that mist is coming out of the nebulizer
- Breathe through the mouthpiece until the nebulizer is empty or stops making mist. Condensation may collect in the tubing during treatment
- For more details, watch the ***How to take Pulmozyme*** video on Pulmozyme.com

Clean and put away your equipment.
- When you are finished, clean the equipment as instructed by the manufacturer
- Put the equipment away, preferably in a place where you can easily see and access it

Want to see a demonstration of the full step-by-step process? Watch the* How to take Pulmozyme *video on Pulmozyme.com

Please see the Indication and Usage and Important Safety Information on the next page and see the accompanying full Pulmozyme Prescribing Information for additional Important Safety Information.

Pulmozyme® dornase alfa INHALATION SOLUTION

Patient Resources

CHOC Pediatric Pulmonology: www.choc.org/programs-services/pulmonology/cystic-fibrosis-center/
CF Foundation: www.cff.org/Get-Involved/Connect/
KidsHealth: https://kidshealth.org/en/parents/cf.html