

Lurie Children's Campaign

for every child

Advancing Care and Research for Children with Cystic Fibrosis

Prepared for Liv For A Cure
Spring 2021



 Ann & Robert H. Lurie
Children's Hospital of Chicago®

Stanley Manne
Children's Research Institute™

Ann & Robert H. Lurie Children's Hospital of Chicago is honored by Liv For A Cure's dedication to improve the lives of children with Cystic Fibrosis. Your support is especially meaningful to the entire care team because it represents your commitment to better outcomes for patients of all ages and families like yours.

Life-Changing Care

Trikafta has transformed the lives of patients with the most common CF mutation after the medication was FDA-approved in October 2019 for patients 12 and older. Many patients are incredibly grateful that they were able to receive this new drug before the COVID-19 pandemic. Lurie Children's is dedicated to bringing this drug to more children, and support from donors such as Liv For a Cure helps us to stay highly involved in clinical trials with the hope of transforming the lives of younger pediatric patients.

Division Head Dr. Terri Laguna and Dr. Susanna McColley continue to play a pivotal role in bringing Trikafta to more patients and remain very

encouraged by recent developments in new Trikafta studies. In clinical trials for children age 12 and older, Dr. McColley served as the Lurie Children's principal investigator, and now she is the global co-principal investigator for the current trial of the drug for patients 6-11 years old.

Results of this trial showed that the drug was well-tolerated and produced meaningful improvements in lung function, sustaining body weight, and other indicators that demonstrated benefits of the drug.



Dr. Laguna with 3-month-old Madelyn Sherman who has Class I CF, and mom, Bridget.

It is estimated that Trikafta will help 90 percent of children with CF, but this still leaves a substantial number of children with no effective therapies because their CF is caused by different genetic mutations. This dilemma is especially pronounced at Lurie Children's, where 30 percent of patients fall into this category and tend to be from minority populations, particularly Black and Latinx children. Drs. Laguna and McColley are involved in additional scientific studies targeting patients with different mutations who still have no beneficial therapies. The entire CF team is committed to supporting families through multidisciplinary resources, including economic and mental health assistance, so they can better cope with their child's condition and remain vigilant in meeting their child's needs.

Exemplary Leadership During Difficult Times

We were fortunate that pulmonologist Maria Dowell, MD, joined us in March 2020 as Director of Lurie Children's Cystic Fibrosis Center and Clinical Practice Director for the entire Division of Pulmonary and Sleep Medicine. Dr. Dowell joined us around the time the pandemic developed in the Chicago area and has been essential in working with Drs. Laguna and McColley to ensure patient care continues and that Lurie Children's locations are safe for patients. Dr. Dowell joined us from the University of Chicago Comer Children's Hospital, where she was medical director of the pulmonary clinic and ambulatory subspecialty clinics. She earned her medical degree from the University of North Carolina Chapel Hill and completed a pediatric pulmonary fellowship at Lurie Children's.

Even as we face a pandemic, the division is united in serving young people and moving research forward. The COVID-19 pandemic has made it difficult for some patients to travel to the main hospital or outpatient facilities, especially patients who need mechanical ventilation to breathe. When innovation became essential to serve our patients, physicians and nurses quickly became certified in telehealth to conduct private video visits online with patient families in their homes, including a single telehealth visit staffed by several multidisciplinary medical providers. As the pandemic begins to subside, we will continue to provide telehealth visits when appropriate for certain patients.

To allow clinical trials to continue during this time, researchers arranged to have experimental drugs sent directly to patients' homes and for patients to have blood samples taken closer to their homes in safe facilities—all in compliance with clinical trial regulations. A grant from the Cystic Fibrosis Foundation also made it possible to send spirometers to patients' homes.

Thank You For Making Progress Possible!

Your support has been essential to advancing research and patient care at Lurie Children's for children with CF and will impact future generations. Lurie Children's is grateful for Liv For A Cure's belief in our mission and confidence in our work. Thank you.