

 **ANTI-INFECTIVE | ENROLLING**

Study to evaluate the standardizing of treatment for pulmonary exacerbations in children and adults with CF ages 6 and older. | STOP360-IP-22 >

This study will look at pulmonary exacerbations, which are a worsening of respiratory symptoms, in people with CF who need to be treated with intravenous (IV) antibiotics. It will compare treatment with one antibiotic (a beta-lactam) to treatment with two antibiotics (tobramycin and a beta-lactam) to learn whether there is a difference in lung function and symptom improvement between the two groups. This study is for people ages 6 and older.

AGE	MUTATION(S)	FEV1% PREDICTED	NUMBER OF VISITS	LENGTH OF PARTICIPATION
6 Years and Older	No Mutation Requirement	No FEV1 Limit	3	48 days

- Tucson
- CHOC
- Miller's Children
- UC Davis
- UC San Diego
- Colorado
- University of Texas SouthWestern – Dallas

 **ANTI-INFECTIVE | ENROLLING**

Study to evaluate inhaled AR-501 in healthy adults and adults with cystic fibrosis and Pseudomonas aeruginosa | Aridis AR-501-001 MAD cohorts >

This study is testing inhaled AR-501, a drug intended to treat infections in the lung. The study will measure the drug's safety, how well it works, and how the body processes it. Multiple doses of AR-501 will be tested in both healthy adults and adults with cystic fibrosis who are infected with Pseudomonas aeruginosa to find the best dose.

AGE	MUTATION(S)	FEV1% PREDICTED	NUMBER OF VISITS	LENGTH OF PARTICIPATION
18 Years and Older	No Mutation Requirement	45% or greater	11	6 weeks

- Tucson
- Miller's Children
- Utah
- Colorado

 **OBSERVATIONAL** | **ENROLLING**

A study of strength and muscle development to assess nutrition and lung function in people with CF ages 18 and older. | [STRONG-CF >](#)

This observational study will look at physical measurements, including body mass index and body composition, and compare them to bone density scans. This study includes measurements of arm circumference, hand-grip strength, distance walked in 6 minutes, and lung function.

AGE	MUTATION(S)	FEV1%	NUMBER OF VISITS	LENGTH OF PARTICIPATION
18 Years and Older	No Mutation Requirement	PREDICTED No FEV1 Limit	5	12 months
<ul style="list-style-type: none">TucsonBaylor				

 **OBSERVATIONAL** | **ENROLLING**

PREDICT: NTM observational study
| [NTM-OB-17 \(PREDICT\) >](#)

This study is taking place at multiple care centers across the U.S. It will evaluate the current standard of diagnosing nontuberculous mycobacteria (NTM) in people with CF.

AGE	MUTATION(S)	FEV1%	NUMBER OF VISITS	LENGTH OF PARTICIPATION
6 Years and Older	No Mutation Requirement	PREDICTED No FEV1 Limit	20	5 years
<ul style="list-style-type: none">ColoradoDallasCHLAUCSUC San Diego				

 **OBSERVATIONAL** | **ENROLLING**

Study to evaluate the effects of CFTR modulators in infants and young children (BEGIN Part B) | BEGIN-OB-19 Part B >

This two-part observational study will look at the effects of CFTR modulators on growth in young children with CF. These drugs are intended to help CFTR protein function closer to normal.

AGE	MUTATION(S)	FEV1% PREDICTED	NUMBER OF VISITS	LENGTH OF PARTICIPATION
Less than 6 Years	Mutation Requirement	No FEV1 Limit	6	2 years
<ul style="list-style-type: none">• Colorado• Baylor• Dallas• Utah				

 **OBSERVATIONAL** | **ENROLLING**

Sweat chloride observational study | CHEC-OB-17 >

This study is taking place at multiple care centers across the U.S. It will look at sweat chloride concentration in people who are currently taking CFTR modulators.

AGE	MUTATION(S)	FEV1% PREDICTED	NUMBER OF VISITS	LENGTH OF PARTICIPATION
4 Months and Older	No Mutation Requirement	No FEV1 Limit	1	1 days
<ul style="list-style-type: none">• Colorado• Baylor• Dallas• Utah• CHLA• UC San Diego				

 **OBSERVATIONAL** | **ENROLLING**

Rare mutation cell collection (RARE) | RARE-OB-16 >

This study is taking place at six regional care centers across the U.S. Researchers will collect and make available for study cells from people with rare CFTR mutations.

AGE	MUTATION(S)	FEV1% PREDICTED	NUMBER OF VISITS	LENGTH OF PARTICIPATION
12 Years and Older	One Copy F508del or No Copies F508del	No FEV1 Limit	1	2 days

- Colorado
- Stanford


 **OBSERVATIONAL** | **ENROLLING**

MAYFLOWERS: Study of pregnancy in women with cystic fibrosis | MAYFLOWERS-OB-20 >

This observational study will evaluate the effects of CFTR modulators on women with CF during and after pregnancy. CFTR modulators are intended to help CFTR protein function closer to normal.

AGE	MUTATION(S)	FEV1% PREDICTED	NUMBER OF VISITS	LENGTH OF PARTICIPATION
16 Years and Older	No Mutation Requirement	No FEV1 Limit	9	35 months

- Colorado
- Baylor
- Dallas
- Utah
- UCS
- UC San Diego


 **GENETIC THERAPY | ENROLLING**

Study to evaluate VX-522 in adults 18 years and older with cystic fibrosis. | Vertex VX21-522-001 SAD >

This study will evaluate the safety and tolerability of VX-522, an investigational inhaled messenger RNA (mRNA) therapy, in adults with CF whose mutations are not responsive to CFTR modulator therapy.

AGE	MUTATION(S)	FEV1% PREDICTED	NUMBER OF VISITS	LENGTH OF PARTICIPATION
18 Years to 65 Years	No Copies F508del	40% or greater	8	6 months

- Colorado
- Stanford
- Baylor

 **GENETIC THERAPY | ENROLLING**

Study of 4D-710 in Adults with Cystic Fibrosis | 4DMT 4D-710-C001 >

This study will test the safety and tolerability of 4D-710, an investigational gene therapy, in adults with CF who are not eligible for or are unable to tolerate CFTR modulator therapy.

AGE	MUTATION(S)	FEV1% PREDICTED	NUMBER OF VISITS	LENGTH OF PARTICIPATION
18 Years and Older	No Mutation Requirement	50 to 100%	11	2 years

- UC San Diego
- Colorado
- University of Texas SouthWestern – Dallas

Observational study of adults with cystic fibrosis for colorectal cancer screening (NICE-CF) | NICE-CF >

This observational study will compare stool-based testing to colonoscopy for colorectal cancer screening in people with CF. The study includes the collection of stool samples at home and a clinical screening colonoscopy.

AGE	MUTATION(S)	FEV1% PREDICTED	NUMBER OF VISITS	LENGTH OF PARTICIPATION
18 Years to 75 Years	No Mutation Requirement	No FEV1 Limit	2	6 months
<ul style="list-style-type: none">• Colorado• Stanford• Dallas• Utah• UCLA				

+ OTHER | ENROLLING

Study of SPI-1005 in people with CF ages 18 and older | Sound Pharma SPI-3005-501.2 >

This study will test the safety and tolerability of SPI-1005, a drug intended to prevent and treat hearing loss caused by aminoglycosides.

AGE	MUTATION(S)	FEV1% PREDICTED	NUMBER OF VISITS	LENGTH OF PARTICIPATION
18 Years and Older	No Mutation Requirement	40% or greater	6	49 days
<ul style="list-style-type: none">• Dallas• UC San Diego				

ABATE: Study to evaluate IV gallium in adults with cystic fibrosis who have nontuberculous mycobacteria (NTM) | [ABATE-IP-18 >](#)

This study is taking place at multiple care centers across the U.S. It will look at the safety and tolerability of IV gallium, a drug intended to treat infections in the lung.

AGE	MUTATION(S)	FEV1% PREDICTED	NUMBER OF VISITS	LENGTH OF PARTICIPATION
18 Years and Older	No Mutation Requirement	25% or greater	8	20 weeks

- Colorado
- Dallas