Current and Upcoming TDN Sanctioned Clinical Trials

Study Name	Protocol Number	Study Phase	Status	Brief Study Description	Inclusion / Exclusion Criteria	Age Range
Intervention Type: CFTR Modulation (Drugs designed to correct the cystic fibrosis transmembrane conductance regulator (CFTR) protein, which allows chloride and sodium (salt) to move properly in and out of cells lining the lungs and other organs)						
Phase 3 study of long-term ivacaftor in babies who have a CFTR gating mutation	Vertex VX-770-126	3	Enrolling	This open-label study will look at the safety and effectiveness of long-term ivacaftor in babies who have a CFTR gating mutation. Primary measurement: adverse events Length of participation: 128 weeks	CFTR Mutation: (see list below)	0 moths to 24 months
Phase 3 study of ivacaftor in babies who have a CFTR gating mutation	Vertex VX15-770-124	3	Enrolling	This two-part study will look at the safety and effectiveness of ivacaftor, as well as how the body processes the drug, in babies younger than 2 years old. Primary measurement: Parts A & B: concentration level in the body, adverse events and other safety measures Length of participation: Part A: 10 weeks Part B: 6 months	CFTR Mutation: At least one copy of a CFTR gating mutation (see list below)	0 months to 24 months
Phase 1/2 study of PTI-801 drug in healthy adults and then in adults with cystic fibrosis	Proteostasis PTI-801-01	1 and 2	Enrolling	This study will look at the safety and tolerability of the drug PTI-801. Primary measurement: concentration level in the body and adverse events Length of participation: 30 days	Other: Patients must be stable on ivacaftor/lumacaftor dosing for both label indication and per label dosing for a minimum of 3 months on Day 1	18 years and older

Click the Study Name for more information about the study and site locations.
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Intervention Type: CFTR Modulation						
Phase 1/2 study of VX-445 combination drug in healthy adults and then in people with cystic fibrosis	VX-445-001	1 and 2	Enrolling	This study will look at the safety and effectiveness of the drug VX-445. Primary measurement: FEV1 and adverse events Length of participation: 12 weeks	CFTR Mutation: F508del homozygous or heterozygous (see list below)	18 years and older
A study to evaluate tezacaftor/ivacaftor and ivacaftor in adults with cystic fibrosis and two copies of the F508del mutation	Vertex VX16-661-114	3	Enrolling	This randomized, placebo-controlled study will look at the safety, effectiveness, and tolerability of the drug tezacaftor/ivacaftor in combination with ivacaftor. Primary measurement: adverse events Length of participation: 84 days	CFTR Mutation: F508del homozygous Other: Must have previously discontinued treatment with Orkambi	12 years and older
Phase 3 study of VX-659 triple combination drug in people with CF 12 years and older who have one copy of the F508del mutation and one copy of a minimal function mutation	VX17-659-102	3	Enrolling	This randomized, placebo-controlled study will be taking place at multiple care centers across the U.S. It will evaluate the effectiveness, safety, and effect on the body of the drug VX-659 in combination with tezacaftor and ivacaftor. Primary measurement: FEV1 Length of participation: 32 weeks	CFTR Mutation: F508del heterozygous (see list below)	12 years and older
Phase 3 study of VX-659 triple combination drug in people with CF ages 12 years and older who have two copies of the F508del mutation	VX17-659-103	3	Enrolling	This randomized, placebo-controlled study will look at the effectiveness and safety of the drug VX-659 in combination with ivacaftor and tezacaftor. Primary measurement: FEV1 Length of participation: 10 weeks	CFTR Mutation: F508del homozygous	12 years and older

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Intervention Type: Mucociliary Clearance (Drugs that target proteins other than CFTR to improve the movement of salt in and out of cells, which helps to hydrate the mucus so it can be cleared more easily)						
Phase 1b study of inhaled AZD5634 in adults with CF	AstraZeneca D6600C00002	1b	Enrolling	This study will look at the safety and tolerability of the inhaled drug AZD5634. Primary measurement: LCI Length of participation: 4 months	Other: Patients currently being treated with ivacaftor monotherapy at screening or received ivacaftor monotherapy within 30 days before screening are excluded. Note: Patients receiving ivacaftor/lumacaftor combination therapy may continue therapy during the study.	18 years to 60 years
Cohorts 1 and 2 of a study to evaluate inhaled QBW276 in adults with CF	Novartis CQBW276X2201	2	Enrolling	This randomized, placebo-controlled study will look at the safety and tolerability of the inhaled drug QBW276 as well as how the body processes the drug. Primary measurement: Adverse events Length of participation: 14 days	FEV1% Predicted: 40 to 100%	18 years and older
Cohort 3 of a study to evaluate inhaled QBW276 in adults with CF	Novartis CQBW276X2201	2	Enrolling Soon	This randomized, placebo-controlled study will look at the safety, effectiveness, and tolerability of the inhaled drug QBW276. Primary measurement: FEV1 Length of participation: 3 months	FEV1% Predicted: 40 to 100% CFTR Mutation: F508del homozygous	18 years and older
Intervention Type: Anti-Inflammatory (Drugs designed to reduce inflammation in the lungs of people with CF, which should help decrease chronic damage to lung tissue)						
APPLAUD: Phase 2 study of LAU-7b in adults with CF	Laurent LAU-14-01	2	Enrolling	This study will look at the safety and effectiveness of the anti-inflammatory drug LAU-7b. Primary measurement: FEV1 and adverse events Length of participation: 189 days	Other: Must have had history of pulmonary exacerbation, defined as at least one (1) pulmonary exacerbation in the year prior to screening which resulted in documented IV antibiotics.	12 years and older
Phase 2 study of lenabasum in people with CF ages 12 and older	JBT101-CF-002	2	Enrolling	This study will look at the safety and effectiveness of the anti-inflammatory drug lenabasum. Primary measurement: Rate of pulmonary exacerbations Length of participation: 32 weeks	Other: Must have been treated with antibiotics for a new exacerbation two or three times in the last year, including at least one treatment v	12 years and older

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Study Name	Protocol Number	Study Phase	Status	Brief Study Description	Inclusion / Exclusion Criteria	Age Range
Intervention Type: Anti-Infective (Drugs designed to fight acute and chronic lung infections by destroying infection-causing bacteria that enter into the airways and colonize)						
Phase 2 study of inhaled nitric oxide in people with cystic fibrosis	Novoteris NO-CF-02E	2	Enrolling	This study will look at the effectiveness of inhaled nitric oxide in adults with cystic fibrosis who are taking an inhaled antibiotic. Primary measurement: FEV1 Length of participation: 7 weeks	FEV1% Predicted: 35 to 85 Culture: Chronic colonization with Pseudomonas aeruginosa, Staphylococcus aureus or Stenotrophomonas maltophilia in at least two sputum cultures in the past year. Other: Ongoing chronic inhaled antibiotic therapy for at least three months prior to screening or baseline.	18 years and older
Treatment of Pulmonary Exacerbations in people with CF (STOP 2)	STOP2-IP-15	4	Enrolling	This study will look at the safety and effectiveness of different lengths of IV treatment for pulmonary exacerbations in people with CF. Primary measurement: FEV1 Length of participation: 35 days	Other: Must experience a pulmonary exacerbation and be planning to receive IV antibiotic treatment	18 years and older
TEACH: Testing the effect of adding oral azithromycin to inhaled tobramycin in people with CF	TEACH-IP-15	2	Enrolling	This study will determine if azithromycin affects the previously recognized clinical benefits of inhaled tobramycin. It is for people with CF who have a Pseudomonas aeruginosa airway infection. Primary measurement: FEV1 Length of participation: 4 months	FEV1% Predicted: 25 to 100 Culture: At least two positive Pseudomonas aeruginosa cultures within last 12 months	12 years and older
Phase 3 study of inhaled vancomycin in adults and children 6 years and older with cystic fibrosis	Savara SAV005	3	Enrolling	This study will look at the effectiveness of the inhaled drug vancomycin in adults and children 6 years and older with cystic fibrosis and positive cultures for methicillin-resistant Staphylococcus aureus (MRSA). Primary measurement: FEV1 Length of participation: 12 months	FEV1% Predicted: 30 to 90% Culture: Positive sputum culture or a throat swab culture for MRSA at Screening.	6 years and older

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Study Name	Protocol Number	Study Phase	Status	Brief Study Description	Inclusion / Exclusion Criteria	Age Range
Intervention Type: Nutrition (Enzymes that increase both fat and vitamin absorption, allowing better nutrition for people with CF, who can become malnourished as a result of thick mucus clogging the pancreas)						
GROW: Phase 2 Study of oral glutathione in children with cystic fibrosis	GROW-IP-16	2	Enrolling	This study will look at the safety and effectiveness of oral glutathione in children with cystic fibrosis. Primary measurement: Weight-for-age (Z-scores) Length of pariticipation: 6 months	Other: Must use pancreatic enzyme replacement therapy (PERT), be clinically stable, and have a weight-for-age between the 10th and 50th percentiles at screening.	24 months to 10 years
Intervention Type: Behavioral (These studies are designed to identify and understand how factors such as self-management, mental health and social support are associated with health outcomes.)						
Intervention Type: Observational (Observational trials do not test new drugs, but they are very important to understanding new ideas about cystic fibrosis and the best ways the disease might be treated)						
TOBI Podhaler Observational Study	Novartis CTBM100C2407	4	Enrolling	This is a prospective observational study for patients who have chronic respiratory Pseudomonas aeruginosa infection. This study is for people with CF who are treated with TOBI® Podhaler™ or other FDA-approved inhaled antipseudomonal antibacterial drugs. Primary measurement: FEV1 and baseline P. aeruginosa colony forming units in sputum Length of participation: 5 years	FEV1 % Predicted: 25-80 Culture: Established diagnosis of chronic Pseudomonas aeruginosa (two positive cultures in the previous year)	6 Years and older
RARE: Rare mutation cell collection	RARE-OB-16	N/A	Enrolling	In this study, researchers will collect and make available for study cells from people with rare CFTR mutations. Primary measurement: N/A Length of participation: 1 day	Other: At this time, participants are required to have two pre-mature stop codon ("nonsense") mutations.	2 years and older
CHEC-SC: Sweat chloride observational study	CHEC-OB-17	N/A	Enrolling	This study will look at sweat chloride concentration in people who are currently taking CFTR modulators. Primary measurement: Sweat chloride concentration Length of participation: 1 day	Other: Must be enrolled in the CF Foundation Patient Registry and have been taking a CFTR modulator for at least three months before enrolling.	4 months and older
PREDICT: NTM observational study	NTM-OB-17	N/A	Enrolling	This study will evaluate the current standard of diagnosing nontuberculous mycobacteria (NTM) in people with CF. Primary measurement: diagnostic NTM disease Length of participation: 5 years	Other: Must be enrolled in the CF Foundation Patient Registry and have a positive culture for NTM within the past two years that has never been treated.	10 years and older

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Mutations Eligible for the VX15-770-124 Study:

G551D, G178R, S549N, S549R, G551S, G1244E, S1251N, S1255P, or G1349D. Subjects who have an R117H-CFTR mutation will be eligible in regions where ivacaftor is approved for use in subjects 2 through 5 years of age with an R117H-CFTR mutation.

Mutations Eligible for the VX-770-126 Study:

Subjects with CF who have 1 of the following CFTR mutations on at least 1 allele: G551D, G178R, S549N, S549R, G551S, G1244E, S1251N, S1255P, or G1349D. Subjects who have an R117H-CFTR mutation will be eligible in regions where ivacaftor is approved for use in subjects 2 through 5 years of age with an R117H-CFTR mutation.

Mutations Eligible for the VX-445-001 Study:

There are over 195 minimal function mutations that are eligible for this study. Some of the most common eligible mutations include: G542X, N1303K, W1282X, R553X, 621+1G->T, 1717-1G->A, 3120+1G->A, I507del, R1162X, 3659delC, 1898+1G->A, G85E, R560T, R347P, 2184insA, 2184delA, Q493X. This is not a complete list of eligible mutations.

Mutations Eligible for the VX17-659-102 Study

A list of the eligible mutations can be found at https://www.cff.org/PDF-Archive/Study-659-102-protocol-feb-2018/

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