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 months 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
Phase 3 study of lumacaftor/ivacaftor (Orkambi®) in babies with two copies of the F508del CFTR mutation	Vertex VX-16-809-122	3	Enrolling	This open-label study will evaluate the safety of the drug lumacaftor/ivacaftor (Orkambi®) and its effect on the body. Primary measurement: adverse events Length of participation: 38 weeks	CFTR Mutation: F508del homozygous	1 to less than 2 years
Phase 2 study of PTI-428 drug in people with CF ages 18 and older who have two copies of the F508del CFTR mutation	Proteostasis PTI-428-06	2	Enrolling	This randomized, placebo-controlled study will look at the safety and tolerability of the drug PTI-428 and how it is processed by the body. It is for people with CF ages 18 and older who have two copies of the F508del CFTR mutation and are already taking tezacaftor/ivacaftor.	CFTR Mutation: F508del homozygous Other: Patients must be stable on tezacaftor/ivacaftor for at least one month before Day 1.	18 years and older

Click the Study Name for more information about the study and site locations.
Report Date: 11/8/2018
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Intervention Type: CFTR Modulation						
RESTORE-CF: Phase 1/2 study of MRT5005 drug in adults with cystic fibrosis (Parts A & B)	Translate Bio MRT5005-101	1 and 2	Enrolling	This randomized, placebo-controlled study will look at the safety and tolerability of different doses of the nebulized drug MRT5005 in adults with CF. Primary measurement: adverse events Length of participation: 12 months	Other: (see list below)	18 years and older
Phase 1 study of PTI-808 drug and triple combination in healthy adults and then in adults with cystic fibrosis	Proteostasis PTI-808-01	1	Enrolling	Part 3 of this randomized, placebo-controlled study will look at the safety, tolerability, and how the body processes the drug in multiple ascending doses of PTI-808 and PTI-808 together with PTI-428 and PTI-801. Primary measurement: changes in physical exams, ECGs, vital signs, and safety labs Length of participation: 56 days	CFTR mutation: F508del homozygous	18 years and older
Phase 3 study of VX-445 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	Vertex VX17-445-102	3	Enrolling	This randomized, placebo-controlled study will evaluate the effectiveness, safety, and effect on the body of the drug VX-445 in combination with tezacaftor and ivacaftor. Primary measurement: FEV1 Length of participation: 32 weeks	CFTR mutation: One copy of F508del and one copy of an eligible mutation, listed below.	12 years and older
Phase 3 study of VX-445 triple combination drug in people with CF ages 12 years and older who have two copies of the F508del mutation	Vertex VX17-445-103	3	Enrolling	This randomized, controlled study will look at the effectiveness and safety of the drug VX-445 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)						
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
SHIP CT: Study of hypertonic saline in preschoolers	SHIP002	2	Enrolling	This placebo-controlled study will look at the safety and effectiveness of hypertonic saline compared to isotonic saline (normal saline). Primary measurement: LCI Length of participation: 54 weeks	N/A	3 years to 5 years
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 with IV antibiotics.	12 years and older

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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)						
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)						
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.

Eligibility for the MRT5005-101 Study

Patients who have a Class III, IV, or V CFTR gene mutation in at least 1 allele or who are receiving treatment with ivacaftor monotherapy are ineligible. Patients who are receiving lumacaftor/ivacaftor combination drug are eligible for the study; however, patients must have been on stable treatment with this medication for at least 28 days prior to the screening visit, and should remain on it for the duration of the study preferably at a stable dose.

Mutations eligible for the VX-445-102 Study

A list of the eligible mutations can be found at https://www.cff.org/PDF-Archive/Study-VX-445-102-Eligible-Mutations-April-2018.pdf