CFFT TDN Welcome Packet for CF Clinical Study Sponsors

Welcome to the CFFT Therapeutics Development Network (TDN). We are a nationwide clinical trials network funded by Cystic Fibrosis Foundation Therapeutics, Inc. (CFFT) for the purpose of facilitating safe, rapid and coordinated evaluation of new treatments for cystic fibrosis. The TDN includes 82 clinical research centers that are geographically distributed across the United States, seven specialized laboratories and over-reading/interpretation centers, and a coordinating center. Each of our clinical research centers is independent, and a key role of the coordinating center (TDNCC) is to provide training, tools and templates to encourage and facilitate quality, efficiency and consistency within the network.

We have developed this welcome packet to provide you with tools and information that we hope will help you understand how we work as a network and facilitate the execution of your current and future clinical studies with the TDN. This information is divided into the following broader categories, each with related attachments:

1. Facilitation of Site Selection, Quality Improvement and Crisis Communication
2. Facilitation of Site Study Budgets
3. Facilitation of Recruitment

We hope that you find these resources helpful. If you have any questions or comments please don't hesitate to contact us:

- Jill Van Dalfsen, jill.vandalfsen@seattlechildrens.org, (206-884-7509), Director of Network and Clinical Trial Operations, CFFT Therapeutics Development Network Coordinating Center; or:
- Kelsie Pearson, kelsie.pearson@seattlechildrens.org(206-884-7553), Project Manager, CFFT Therapeutics Development Network Coordinating Center
- Amy Hoffman, amy.hoffman@seattlechildrens.org, (206-884-7540), Manager, Network Development Unit, CFFT Therapeutics Development Network Coordinating Center

Other Available Services

Keep in mind that in addition to the free services described in this packet, the TDN offers many other services (for a fee) that may be useful to you, including consultation on unique CF outcome measures or study design, clinical study materials (e.g. study manuals, consent templates), clinical trial management or monitoring, and specific laboratory/over-reading services. If you are interested in more information about other possible services and associated fees, please contact Jill Van Dalfsen or Kelsie Pearson.

The TDN has developed an online educational program that covers specific aspects of clinical research that are particular to cystic fibrosis. This program covers the basics of CF, CF treatment strategies and medications, a brief overview of the TDN Network and the CF Foundation, and some specific aspects of monitoring CF clinical trials. We encourage all sponsors and CROs to take this training program, especially if you are new to CF. The course costs $300 and you can access the program at https://cftdn.digitalchalk.com/dc/guest/login. Please contact Amy Hoffman if you have any questions about the program or feedback on the content.

We have provided a list of the contacts for the *TDN Standard Operating Procedures* for some of the CF specific outcome measures that you may be interested in using for your study. Note that not all TDN centers have been trained or have the equipment to perform all of the specialized procedures and some of these procedures may require services or training available from one of our National Resource Center (NRC) laboratories or over-reading centers. If you are interested in more information about TDN Standard Operating Procedures, please contact Kelsie Pearson.

Facilitation of Site Selection, Quality Improvement & Crisis Communication

Solicitation of Study Interest

Upon request, the TDNCC is happy to facilitate the process of site selection by determining which TDN centers have initial interest in participating in your study. We will send your non-confidential study summary to the primary contacts at TDN centers, collect and compile their responses, and send you a list of interested centers with appropriate contact information. This is a complimentary service for sponsors who submit protocols to the TDN for review. TDN centers are usually very responsive to a study solicitation e-mail sent from the TDNCC because it provides confirmation to the sites that the protocol is undergoing TDN review. **Given the number of CF studies that are in start up at any one time, most TDN investigators will ignore requests for feasibility information from parties that have not been identified by the TDNCC through the solicitation process**. If you are interested and have not already requested this service in your TDN Protocol Review Application, please contact Kelsie Pearson to get started.

Facilities Information on TDN Centers

The TDN maintains a database of information regarding the current research facilities of our centers. This database includes most of the generic information that is typically collected as part of study feasibility questionnaires, such as number of patients and IRB review schedules. We will be happy to provide this information to you for TDN centers that you may be interested in using as a study site. Using our pre-collected capabilities data minimizes redundant efforts for both the sponsor and the clinical sites, which can help to facilitate more rapid site selection. A sample *Site Research Facilities Report* is included in this packet. For more information or to request reports, please contact Amy Hoffman.

Site Selection Guidance

Given the unequal distribution of studies to TDN sites that we've seen to date, we would aim with sponsors' support to provide better, equitable distribution of clinical trials to all TDN centers and patients with cystic fibrosis throughout the United States. Equitable distribution will benefit sponsors as well by increasing capacity, accelerating clinical trial enrollment through faster start-up times and a larger geographical range for enrollment. For a list of sites that are currently underutilized please contact Amy Hoffman.

Quality Improvement: TDN Research Metrics

In an effort to improve the clinical trial performance of sites within the network, the TDN tracks site performance metrics, overall study milestones, and enrollment at TDN centers by storing this information in its database along with site contact and facilities information. In order to keep this information up-to-date,

CFFT requires that TDN sites provide certain information about their study activities on an ongoing basis. In addition, the TDNCC will contact sponsors of TDN studies once per quarter to obtain information about total enrollment in their studies and any changes in key study milestones. This information is used to project the total number of subjects anticipated to enroll and be active in CF studies in the future and to determine if the TDN needs to add additional resources to meet the needs of sponsors. More information about this database and how we would like to coordinate with you can be found in the attached document, *CCSM Fact Sheet for Sponsors*.

Quality Improvement: Sponsor and CRO Survey

The TDN Steering Committee has implemented an online survey process to collect feedback from study sites on their experiences with CF TDN clinical trials. The survey is divided into four main areas: protocol and study conduct, general study management questions, sponsor interactions, and CRO interactions. The purpose of the survey is to gather specific comments, both positive and negative, from study sites and provide this information back to sponsors. In addition, the TDNCC will use this information to help determine whether the CFFT TDN Protocol Review Committee's review accurately reflected the protocol's feasibility. The TDNCC will send a survey to sites at two time points, after start-up and at study closeout, and provide a survey summary to sponsors if requested with the goal of improving processes and study conduct. This information will remain confidential and will not be shared outside of the TDN Coordinating Center and TDN leadership. The survey questions can be found in the attached documents, *Start-Up Sample Survey* and *Close-out Sample Survey*. Sponsors should indicate whether they would like to receive the survey results at the time of protocol submission on the Protocol Review Application. For more information, please contact Kelsie Pearson.

CF Clinical Trial Crisis Plan for CFFT and Site Investigators

A clinical trial crisis plan has been developed by Cystic Fibrosis Foundation Therapeutics for the network and site investigators. This plan serves to ensure patient safety should a death or life threatening event occur in a CFFT funded clinical trial. The plan outlines the chain of communication between the site investigator, TDNCC medical monitor, and CFF leadership. Recognizing the confidentiality issue related to such events in industry sponsored studies, this plan reinforces the need for investigators to know and follow a sponsor's safety plan as outlined in the protocol. While we hope such a devastating event never occurs, the intent of this plan is to help investigators be prepared. If you would like to take advantage of the resources related to clinical trial crisis management please contact Amy Hoffman.

Facilitation of Site Study Budgets

Payments to Study Subjects

The CF patient population is a highly studied group of patients, many of whom participate in multiple studies during their lifetimes. The studies in which they participate require a large commitment of time and effort, while often providing no direct benefit since many are placebo-controlled. For that reason, it is typical for CF study subjects to be compensated for their participation in clinical studies. In an effort to standardize these payments across the network at a level that is both appropriate and ethical, the TDN has developed the *TDN Guidelines for Payments to Study Subjects*. The current guidelines are included in this packet. While IRBs have the ultimate say in how study subjects at each site are compensated, TDN centers are encouraged to follow the TDN guidelines as much as possible; therefore sponsors may want to use the dollar figures in these guidelines as the basis for the patient compensation costs in their initial budgets.

TDN Budget Template

Many sponsors and CROs use their own budget templates to develop and negotiate site budgets. CF trials can be unique in that many of the procedures routinely performed in CF clinical trials are not routine billable procedures with set costs, but rather represent staff time to complete. In addition, sponsors sometimes neglect to include line items for activities that require staff time that must be compensated (such as monitoring visits). The TDN has developed a site budget template that our sites generally use to determine their own budgets, and sponsors may also find it helpful (and efficient) to use the same budget template to establish the parameters of the site budget and to more accurately estimate site costs. Contact Amy Hoffman to obtain the most up-to-date copy of the template and for instructions on how to use it.

Site Labor Costs Estimation

Many of the other costs for CF studies are based on estimation of time required of the research coordinator and the investigator. The level of experience in budget estimation varies widely at the TDN centers. We believe that sponsors who provide appropriate time estimates to sites participating in the study can decrease the time required to negotiate mutually acceptable budgets. To obtain realistic time estimates, we recommend having a pilot group of two or three highly experienced centers who plan to participate in the study prepare their budget estimates first, using a detailed template that includes time for both study visits and study management. Ideally, this would be followed by a group conference call to discuss the time estimates and arrive at a range that the pilot group and the sponsor all believe is reasonable and which can then be shared with other study sites. The TDNCC is happy to provide the detailed budget template and facilitate the pilot group meeting for you. If interested, please contact Amy Hoffman.

Facilitation of Recruitment

Port CF Clinical Trial Query Tool

CFF-accredited care centers maintain key patient data in a common database called Port CF. For most studies, CFF is able to load a high-level query into Port CF to help sites do an initial screen for the number of potentially eligible subjects at their site. These queries are largely limited to age, FEV_1, genotype and major microbiology inclusion and exclusion criteria, but are still helpful to sites in creating a subset that can be evaluated further.

An approval checkbox for this complimentary service is included on the TDN Protocol Review Application. If you checked the box at the time of protocol submission, you may assume a query is in process for your study. Please contact Kelsie Pearson if you would like a query created but did not indicate that at the time of application, or if you already have a Port CF query but your inclusion/exclusion criteria have subsequently changed from the original protocol submission.

Research Subject Referral

Historically, a CF patient's access to trials has been limited to those studies conducted at their closest center. We have now entered an era of clinical trials in CF where many new studies are currently being initiated in the CF population and often these studies include restrictive eligibility requirements or esoteric procedures that may only be available at a smaller sub-set of study sites. Furthermore, the CFF has launched the successful "I am the Key" initiative to inspire patients and families to participate in clinical trials, and many patients nationwide are now asking about studies.

As a result, study sponsors are encouraged to plan for the need for research subject referrals to successfully enroll their studies. To facilitate such referrals, the TDN has created a series of documents called *Successful*

Research Subject Referral for CF Clinical Trials to provide tips to the referring site, the study site and the study sponsor. A synopsis of this information is attached.

TDN Publication Guidelines

It is particularly important to the CF community that study results for ALL studies conducted within CF are published for the following reasons:

- People with CF are asked to repeatedly participate in studies; thus, it is important that they believe that their time and effort in participating in the study contributed to the scientific knowledge about the disease, product, or product type regardless of whether the study had a positive or negative result.
- Investigators within the TDN are largely from academic institutions. Their ability to continue engaging in studies is hampered when their time and effort participating in a study is not recognized through a publication or presentation of the data at a national meeting.
- Negative studies may provide important information regarding potential safety issues or potential lack of efficacy of similar products within the same therapeutic class.

We realize an awareness of the TDN goals for publication is not likely to help recruitment on your current study, but it may be important for recruitment for your future studies. More information can be found in the attached document, *TDN Publication Guidelines for Sponsors*.

Study Subject Follow-up Communications

As noted earlier, the CF patient population is called upon repeatedly to participate in clinical studies. The CF research community believes that taking the time to acknowledge each patient's contribution and to provide them with information about what happened in the study is critical to preserving the goodwill of the CF patient population and maintaining their interest in clinical trials. To that end we have established the *TDN Guidelines for Study Subject Follow-up Communications*. Our sites have a responsibility to follow these guidelines but your cooperation is also appreciated. Again, this information is included here more to help facilitate recruitment for your future studies than your current study.

CFFT TDN Standard Operating Procedures

Pediatric Lung Function Testing

Contact: Dr. Stephanie Davis, Director, Center for Pediatric Lung Function, sddavis3@iupui.edu

Nasal Potential Difference

Contact: Dr. Steve Rowe, Director, Center for CFTR Detection, smrowe@uab.edu

Sputum Induction, Inflammatory Markers & Cytology

Contact: Dr. Mike Konstan, Director, Center for Interpretive Cytology, michael.konstan@case.edu

Contact: Dr. Frank Accurso, Director, Center for Biochemical Markers, frank.accurso@childrenscolorado.org

Sweat Testing

Contact: Dr. Frank Accurso, Director, Center for Sweat Analysis, frank.accurso@childrenscolorado.org

CF Microbiology

Contact: Dr. Lucas Hoffman, Director, Center for CF Microbiology, lhoffm@u.washington.edu

Diagnostic Imaging

Contact: Dr. Paul Guillerman, Co-Director, Center for Diagnostic Imaging, rpguille@texaschildrens.org

Contact: Dr. Scott Nagel, Co-Director, Center for Diagnostic Imaging, snagle@uwhealth.org

Current Organization Capabilities: Cleveland CC and Peds

Number of Patients Served by CF Program

Quantity Details

Number of Pediatric Patients (< 18 years)	116
Corrected for ineligible patients (< 18 years)	116
Number of Adult Patients (>= 18 years)	50
Corrected for ineligible patients (>= 18 years)	49

Administrative Facilities

Facility Details

Research Coordinator Resources (check all that apply):

- [x] Designated Work Area
- [x] Secured (locked) Storage for study material/records
- [x] Networked Computer
- [ ] Access to laptop computer
- [x] Individual User ID & Password

Administrative Facilities:

Medical Record Type
- [x] Paper

Pharmacy Services

Drug Storage, Services & Procedures

Investigational Drug Storage (check all that apply):

- [x] Separate, Dedicated IDS Pharmacy
- [ ] Regular Pharmacy
- [x] Secured (locked) Drug Storage

Pharmacy Personnel:

- [x] Investigational Drug Pharmacist Available

Backup Pharmacist Type
- [x] Regular Pharmacist

Refrigerated Temperature Monitoring (check all that apply):

- [x] Continuous Temperature Charts
- [x] Daily Recording from Thermometer
- [x] Temperature Alarms

Room Temperature Monitoring (check all that apply):

- [x] Continuous Temperature Charts
- [x] Daily Recording from Thermometer
- [x] Temperature Alarms

Pharmacy Capabilities (check all that apply):

- [x] Dye-marker Experience
- [x] Capsule Preparation
- [ ] System for error recording/tracking

Written Pharmacy Procedures (check all that apply):

- [x] Investigational Drug Storage

- [x] Investigational Drug Dispensing
- [x] Investigational Drug Return
- [x] Investigational Drug Destruction/Disposal

Pharmacy Comments

same peds and adult

Study Review and Approval

IRB & Contract

Usual IRB Type: [x] IRB Affiliated Institutions

IRB Name: Universities of Cleveland Institutional Review Board

IRB Schedule: [x] Every 2 Weeks

IRB Fees for Industry Studies ($): 0

IRB Fees for Non-Industry Studies ($): 0

Institutional HIPAA Requirement: [x] Separate from ICF

IRB Comment

Same peds and adult

IRB & Contract Review Coordination: [x] Conducted Concurrently

Contract Comments

- [ ] IRB Requires CRFs for Review
- [ ] IRB Requires DMC Documents for Review

GCRC

- [x] GCRC Affiliation

GCRC Meeting Schedule: [x] Every 2 Weeks

IRB & GCRC Review Coordination: [x] Conducted Concurrently

GCRC Comments

Same peds and adult

Special Procedure Capabilities

Specialized Procedures

Specialized Procedures Comment

Non-TDNCC Qualified Capabilities (check all that apply)

- [x] In-Patient Stays
- [x] Metabolic Kitchen
- [x] Protocol Specified Diets
- [x] 24-Hour Food Records
- [x] 72-Hour Stool Processing
- [x] Audiology

- [x] CT Scans
- [x] Bronchoscopy
- [x] Toddler PFT

TDNCC Qualified Site Capabilities (check all that apply)

- [x] Specialized Sweat Test
- [x] NPD
- [x] Infant PFT
- [x] Sputum Induction
- [x] Sputum Cytology
- [ ] GI-PD
- [ ] Mucocilliary Clearance

Study Visit Information

In-Patient Study Services

- [x] Ability to Conduct In-Patient Studies

Number of In-Patient Study Beds: 12

- [x] Pediatric Study Beds
- [x] Adult Study Beds

In-Patient Study Comments:

Pediatric and Adult same

Out-Patient Visit Location(s) (check all that apply)

- [x] CF Clinic
- [x] GCRC
- [ ] Other

Out-Patient Visit Location Comments:

Equipment, Labs & PFT Personnel

Site Equipment (check all that apply)

- [x] Refrigerated Centrifuge for 15 & 20 mL tubes (250 to 4000 G Spin)
- [x] -70 C Freezer
- [x] -20 C Freezer
- [x] 4 C Refrigerator
- [ ] Barcode Scanner (site owned)
- [x] Portable Spirometer
- [ ] Nouvag Nebulizer (site owned)
- [ ] Macroduct (site owned)

Lab Information

Research Specimen Processing Lab: [x] Designated On-Site Research Processing Laboratory

Standard Procedures (Hem/Chem) Lab ☒ On-Site Clinical Standard Procedures Laboratory

Research PFTs Personnel (check all that apply)

- ☒ Respiratory Therapist
- ☐ RC/Nurse
- ☐ Research Physician/Investigator

Research PFTs Personnel Comment

CFFT TDN Clinical Study Metrics Database
Fact Sheet for Sponsors

In an effort to improve the clinical trial performance of sites in the CFFT Therapeutics Development Network (TDN) and assist sponsors with the identification of sites that have the appropriate training, resources, and patient base needed for a new study, the TDN has developed a database that tracks site performance metrics, overall study milestones, and enrollment. In order to keep this information up-to-date, Cystic Fibrosis Foundation Therapeutics (CFFT) requires that sites that receive TDN research funding provide certain information about their study activities on an ongoing basis.

Note that all study milestone information and enrollment information is kept strictly confidential and is only provided to senior management at CFFT and the TDN Coordinating Center (TDNCC). Both the CFFT and TDNCC maintain confidentiality agreements with all corporate sponsors.

Specifically, the information collected from TDN sites about each study includes:

- Number of subjects site agreed to enroll
- Date the regulatory packet was received
- Date of GCRC (or Scientific Advisory Board approval) – if applicable
- Date of IRB approval
- Date of contract execution
- Date site was approved to enroll (i.e. date all regulatory documents were received and site was approved to receive study drug)
- Date first subject screened
- Date first subject enrolled
- Date last subject enrolled
- Number of patients enrolled

These metrics are used to provide sites with regular quality improvement reports that can help them identify bottlenecks at their site and also to give them some idea of how they are performing relative to other sites in the network. The sites are only provided with detailed information from their own site; they do not see data from other participating sites.

In addition, the TDNCC will contact sponsors of TDN studies once per quarter to obtain information about total enrollment in their studies and any changes to the following estimated study milestone timelines:

- Enrollment start: Date First Patient First Visit
- Enrollment end: Date Last Patient First Visit
- Study Complete: Date Last Patient Last Visit

This information will be used to project the total number of subjects anticipated to enroll and be active in CF studies in future and to determine if the TDN needs to add additional resources to meet the demands of sponsors.

Finally, at the conclusion of each study, the TDNCC will contact the sponsor to solicit information about the quality of the research conducted at each site in order to identify issues at the site that may require additional training or assistance.

If you have any questions about the database and security associated with it, please don't hesitate to contact Amy Hoffman, Manager, Network Development Unit, amy.hoffman@seattlechildrens.org. We sincerely hope that you will help us with our endeavor to provide the highest quality sites for cystic fibrosis research.

2013 Site Protocol and Sponsor Assessment Start-Up - Generic

1. Welcome

The CFFT TDN Steering Committee would like to provide feedback to the study sponsor for the xxxx clinical trial which recently completed start-up at your center. Please complete the following survey regarding your site's experience with the start-up activities. The TDNCC will collate all site responses and provide an anonymized summary to the sponsor.

The complete list of survey questions has been provided in the attachment so that your study team can review and discuss them before responding.

We would appreciate your completion of this survey by XX date. Please do not respond to this email directly.

We hope that by providing sponsors with a detailed assessment of your collective experiences, both positive aspects of study conduct and issues that could be improved upon will be discussed and considered when planning future clinical trials. We appreciate your help with this effort.

If you have any questions, please contact XX at xx@seattlechildrens.org

2. Site Information

***1. Please select the name of your site:**

***2. Please complete the following for the individual completing the survey (please note this information will remain anonymous):**

First Name:

Last Name:

Email:

3. General

3. Please rate the following areas related to general aspects of this study (relative to other sponsors that you have worked with) using this scale: 1 = Very Poor; 2 = Adequate; 3 = Good; 4 = Excellent

	1 (Poor)	2	3	4 (Excellent)	N/A
a) Ease of IRB submission using provided materials	◯	◯	◯	◯	◯
b) Ease of budget negotiations using provided templates	◯	◯	◯	◯	◯
c) Willingness to negotiate budget if there were unreasonable, unrealistic or missing line items	◯	◯	◯	◯	◯
d) Ease of contract negotiations	◯	◯	◯	◯	◯
e) Centralized and clear lines of communication defined at start of study	◯	◯	◯	◯	◯
f) Timeliness and reasonableness in reviewing site-specific consent forms	◯	◯	◯	◯	◯
g) Reasonableness of time frame given to complete start-up tasks	◯	◯	◯	◯	◯
h) Efficient and informative investigator meeting	◯	◯	◯	◯	◯
i) Efficient and clear study initiation visit and training	◯	◯	◯	◯	◯
j) Additional study specific training requirements were clear	◯	◯	◯	◯	◯
k) Protocol amendments did not delay study start-up	◯	◯	◯	◯	◯
l) Study website was easily navigable and contained all required documents	◯	◯	◯	◯	◯
m) Integration of sponsor and CRO as a team	◯	◯	◯	◯	◯

4. Sponsor Interactions

4. Please rate the following areas related to your interactions with the sponsor on this study (relative to other sponsors that you have worked with) using this scale: 1 = Very Poor; 2 = Adequate; 3 = Good; 4 = Excellent

	1 (Poor)	2	3	4 (Excellent)	N/A
a) Professionalism of sponsor staff (conversation or email interactions)	◯	◯	◯	◯	◯
b) Sponsor's respect of site staff	◯	◯	◯	◯	◯
c) Sponsor defined roles for the study clearly	◯	◯	◯	◯	◯
d) Sponsor's identification of their Project Manager or primary contact person	◯	◯	◯	◯	◯
e) Sponsor's promptness in responding to study questions	◯	◯	◯	◯	◯
f) Sponsor's consistency in advice and answers	◯	◯	◯	◯	◯
g) Sponsor's willingness to help solve problems with site	◯	◯	◯	◯	◯
h) Sponsor's willingness to help solve problems with CRO	◯	◯	◯	◯	◯

5. CRO Interactions

***5. Did you work with a CRO on this study during start-up?**

- ◯ Yes
- ◯ No

6. CRO Interactions

6. Which CRO is being used for this study?

7. Please rate the following areas related to your interactions with the CRO on this study (relative to other CROs you have worked with) using this scale: 1 = Very Poor; 2 = Adequate; 3 = Good; 4 = Excellent

	1 (Poor)	2	3	4 (Excellent)	N/A
a) Professionalism of CRO staff (conversation or email interactions)	◯	◯	◯	◯	◯
b) CRO's respect of site staff	◯	◯	◯	◯	◯
c) CRO defined roles for the study clearly	◯	◯	◯	◯	◯
d) CRO's promptness in responding to study questions	◯	◯	◯	◯	◯
e) CRO's consistency in advice and answers	◯	◯	◯	◯	◯
f) CRO's willingness to help solve problems with site	◯	◯	◯	◯	◯

7. Comments

8. Please provide any comments regarding things that went well during start-up:

9. Please provide any comments regarding things that did not go well during start-up:

10. Please provide recommendations for how things could be improved with sponsor:

11. Please provide recommendations for how things could be improved with this CRO:

12. Did you learn anything from this study/interaction that made you rethink or change any of your processes?

- ◯ Yes
- ◯ No

If so, what?

8. Thank You

Thank you for taking the time to complete this survey.

2013 Site Protocol and Sponsor Assessment Close-Out - Generic

1. Welcome

The CFFT TDN Steering Committee would like to provide feedback to the study sponsor for the xxxx clinical trial which was recently completed at your center. Please complete the following survey regarding your site's experience with the protocol, study conduct, and study management. The TDNCC will collate all site responses and provide an anonymized summary to the sponsor.

The complete list of survey questions has been provided in the attachment so that your study team can review and discuss them before responding.

We would appreciate your completion of this survey by XX date. Please do not respond to this email directly.

We hope that by providing sponsors with a detailed assessment of your collective experiences, both positive aspects of study conduct and issues that could be improved upon will be discussed and considered when planning future clinical trials. We appreciate your help with this effort.

If you have any questions, please contact XX at xx@seattlechildrens.org

2. Site Information

***1. Please select the name of your site:**

***2. Please complete the following for the individual completing the survey (please note this information will remain anonymous):**

First Name:

Last Name:

Email:

3. Protocol and Study Conduct

2013 Site Protocol and Sponsor Assessment Close-Out - Generic

3. Please answer the following questions regarding the protocol and study conduct:

	Yes	No	NA
a) Was the feasibility of the study accurately reflected in the PRC review and score?	◯	◯	◯
b) Were there any unanticipated enrollment barriers due to inclusion/exclusion criteria?	◯	◯	◯
c) Were there any unanticipated enrollment barriers due to study design (duration, number of visits)?	◯	◯	◯
d) Were there any unanticipated enrollment barriers due to perceived risks?	◯	◯	◯
e) Were there any unanticipated enrollment barriers due to competing studies?	◯	◯	◯
f) Were there any unanticipated enrollment barriers due to specific procedures?	◯	◯	◯
g) Was there confusion by patients/families about the product or study rationale?	◯	◯	◯
h) Were there excessive number of protocol amendments during the study?	◯	◯	◯
i) Was required training provided by knowledgeable staff?	◯	◯	◯
j) Was there real time support for issues with study specific procedures?	◯	◯	◯
k) Were patient enrollment goals realistic?	◯	◯	◯
l) Were the equipment and supplies provided adequate for study conduct?	◯	◯	◯
m) Were there excessive withdrawals due to difficulty in subject compliance with protocol demands?	◯	◯	◯

4. Please provide comments on things that went well in terms of feasibility and enrollment:

5. Please provide comments on issues with feasibility and/or enrollment:

6. Please provide recommendations for how these issues could have been avoided:

7. Please rate the following using this rating scale: 1 = Poor; 2 = Adequate; 3 = Good; 4 = Excellent

	1 (Poor)	2	3	4 (Excellent)	N/A
a) Level of interest from patients/families	◯	◯	◯	◯	◯
b) Level of interest from clinic and support staff	◯	◯	◯	◯	◯
c) Quality and quantity of tools provided (recruitment materials, consent form template, study diary, SOPs, study manual, websites, source documents)	◯	◯	◯	◯	◯

8. Did errors or ambiguity in the protocol lead to delayed IRB submissions, protocol violations, scheduling issues, or other problems? Please explain.

9. Please provide any additional comments on the information provided above:

4. General

10. Please rate the following areas related to general aspects of this study (relative to other sponsors that you have worked with) using this scale: 1 = Very Poor; 2 = Adequate; 3 = Good; 4 = Excellent

	1 (Poor)	2	3	4 (Excellent)	N/A
a) Low monitor turnover	◯	◯	◯	◯	◯
b) Knowledge and perceived competence of study monitors	◯	◯	◯	◯	◯
c) Adequate traning and guidelines for completing case report forms	◯	◯	◯	◯	◯
d) Reasonableness of time frame given to complete eCRF and query completion	◯	◯	◯	◯	◯
e) Ease of use of electronic data capture system	◯	◯	◯	◯	◯
f) Conduct of monitoring visits	◯	◯	◯	◯	◯
g) Access to Lead PI	◯	◯	◯	◯	◯
h) Access to Lead RC	◯	◯	◯	◯	◯
i) Timeliness of updates (enrollment, amendments)	◯	◯	◯	◯	◯
j) Ease of invoicing and and timeliness of payment	◯	◯	◯	◯	◯
k) Integration of sponsor and CRO as a team	◯	◯	◯	◯	◯

5. Sponsor Interactions

11. Please rate the following areas related to your interactions with the sponsor on this study (relative to other sponsors that you have worked with) using this scale: 1 = Very Poor; 2 = Adequate; 3 = Good; 4 = Excellent

	1 (Poor)	2	3	4 (Excellent)	N/A
a) Professionalism of sponsor staff (conversation or email interactions)	◯	◯	◯	◯	◯
b) Sponsor's respect of site staff	◯	◯	◯	◯	◯
c) Sponsor's promptness in responding to study questions	◯	◯	◯	◯	◯
d) Sponsor's consistency in advice and answers	◯	◯	◯	◯	◯
e) Sponsor's willingness to help solve problems with site	◯	◯	◯	◯	◯
f) Sponsor's willingness to help solve problems with CRO	◯	◯	◯	◯	◯
g) Availability of sponsor's Medical Monitor	◯	◯	◯	◯	◯
h) Sponsor's adherence to promised timelines (start-up and end of enrollment)	◯	◯	◯	◯	◯

6. CRO Interactions

***12. Did you work with a CRO on this study?**

◯ Yes

◯ No

2013 Site Protocol and Sponsor Assessment Close-Out - Generic

7. CRO Interactions

13. Which CRO was used for this study?

14. Please rate the following areas related to your interactions with the CRO on this study (relative to other CROs you have worked with) using this scale: 1 = Very Poor; 2 = Adequate; 3 = Good; 4 = Excellent

	1 (Poor)	2	3	4 (Excellent)	N/A
a) Professionalism of CRO staff (conversation or email interactions)	◯	◯	◯	◯	◯
b) CRO's respect of site staff	◯	◯	◯	◯	◯
c) CRO's promptness in responding to study questions	◯	◯	◯	◯	◯
d) CRO's consistency in advice and answers	◯	◯	◯	◯	◯
e) CRO's willingness to help solve problems with site	◯	◯	◯	◯	◯
f) Availability of CRO's Medical Monitor	◯	◯	◯	◯	◯

8. Comments

15. Please provide any comments regarding things that went well:

16. Please provide any comments regarding things that did not go well:

2013 Site Protocol and Sponsor Assessment Close-Out - Generic

17. Please provide recommendations for how things could be improved in the future with this sponsor:

18. Please provide recommendations for how things could be improved in the future with this CRO:

19. Did you learn anything from this study/interaction that made you rethink or change any of your processes?

- ◯ Yes
- ◯ No

If so, what?

20. Did you receive any patient feedback regarding the study that would be important to share with the sponsor?

- ◯ Yes
- ◯ No

If so, please provide:

21. Would you be interested in working with this sponsor again?

- ◯ Yes, definitely
- ◯ Maybe
- ◯ No

22. Would you be interested in working with this CRO again?

- ◯ Yes, definitely
- ◯ Maybe
- ◯ No

9. Thank You

Thank you for taking the time to complete this survey.

TDN Guidelines for Payment to Study Subjects

Purpose and Scope

This procedure provides guidelines for a unified approach to the payment of study subjects for participation in studies conducted through the CFFT Therapeutics Development Network (TDN). Although the Institutional Review Board (IRB) at each participating site will have final responsibility for careful review and approval of payments to study subjects, the TDN has created these guidelines to assist sites in collaborating with their IRBs. While consistency among sites participating in TDN trials is desirable, the TDN will honor the site-specific IRB approval for each study.

Approvals

The *TDN Guidelines for Payment to Study Subjects*, including the recommended payment schedule, will be reviewed annually by the TDN Steering Committee and revised as necessary. This review will examine payment information from studies that have been conducted or are currently ongoing, as well as feedback from the various IRBs involved with those studies. This level of review will assist the TDN in making recommendations about payments to subjects that are as unbiased and non-coercive as possible.

Responsibilities

Each site participating in a TDN trial should adhere to these guidelines and the payment schedule whenever possible. It is the responsibility of the Steering Committee to review these guidelines.

Additionally, all TDN members must adhere to the FDA *Payment to Research Subjects Information Sheet*, included in the Guidance for Institutional Review Boards and Clinical Investigators materials found on the FDA's website.

It is recommended that each site participating in a TDN trial (where payment is being provided to study subjects) notify both their IRB and the subjects being recruited for the trial of the potential loss of benefits from Supplemental Security Income (SSI) and Medicaid due to trial payments that exceed the current $2,000 per calendar year exclusion. Please see the *Guidance for Study Sites on Implementation of the Improving Access to Clinical Trials Act (IACT)* document for further information and instructions for how patients can claim this exclusion.

Process

Budgeting

Study sites participating in TDN trials should use the payment schedule included in this document to estimate costs of payment to study subjects, which will be included in the budget for the trial. Study sites should also ensure that during budget and contract negotiations, the additional costs of reimbursing study subjects for expenses such as their actual costs for meals, mileage and parking are addressed and that provision is made to pass these costs through to sponsors.

IRB Approval

Study sites should use the TDN guidelines in collaborating with their IRBs and inform their IRBs that such guidelines have been developed for the TDN sites. The IRB at each site will have final approval of the schedule for payment to study subjects.

Payment Schedule

Individuals (families) volunteering to participate in research studies deserve to be reasonably paid for their time and expenses incurred during participation. Such payment should not be excessive and should not be perceived as an inducement to participate. Current recommendations of the CFFT Therapeutics Development Network (TDN) for appropriate payment follow.

Compensation for Time Spent at Study Visits

Study Visits

Generally, study subjects (or parents who need to take time off of work to bring a child in for a study visit) should be paid a maximum of $30/hour for participating in a study up to maximum of $250 for a single visit (up to 24 hour total study visit duration). These amounts may be adjusted in areas where the cost of living is significantly higher compared to the rest of the country.

It is expected that all subjects will be compensated for the *anticipated average amount of time for each study visit*. The determination of the anticipated average amount of time each study visit will take is site specific and should be determined at the time that the study budgets are being negotiated with the sponsor and prior to finalizing the informed consent. Anticipated average visit time should be calculated to the nearest hour.

Special Considerations for Compensation When Study Subject is a Minor

When a study subject is a minor, there may be additional considerations regarding reimbursement for time and expenses incurred in study participation. Please review your institutional practices and guidelines or discuss with your IRB chair if additional guidance is needed.

Reimbursement for Expenses

Subjects should be reimbursed for actual costs incurred for mileage, parking, meals, etc. Reimbursement for actual costs incurred does NOT affect SSI benefits and Medicaid, and is important to keep it separate for these reasons.

Mileage

Reimbursement for mileage should be provided at the current federal business mileage rate.

Other Expenses

Reimbursement for parking fees and meals should be made at the rates applicable for the participating study site.

Guidelines for Long-Distance (>200 miles) Participation

In some instances, subjects traveling long distances (>200 miles) may require air transportation. If participation by subjects who travel long distance is required, the sponsor of the study should underwrite

these costs, *but prior approval from the sponsor should be obtained before subjects are recruited or payment is arranged*.

For minor children, travel expenses should be paid for the study subject and one parent/legal guardian. For adult study subjects, travel expenses should be paid for the subject. Consideration may be given to defraying travel costs (partial or complete) of a travel companion for adults.

Additional expenses related to study subjects traveling long distances (air fare, housing, meal expenses, and local transportation) should be negotiated and submitted as pass-through costs to the study sponsor. Since subject reimbursement for these expenses is for incurred costs, it will NOT affect SSI benefits and Medicaid. All arrangements should be made in the most cost-efficient manner possible.

Guidelines for Filing Payment Records with Institution

Compensation for participation in a clinical study is ordinarily reported to the Internal Revenue Service (IRS) as *miscellaneous non-employee income* on an IRS Form 1099. The IRS requires such reporting if payment for services is made in the amount of $600/year or more. The hospital should not include on the 1099 form any amount that was paid to the individual for reimbursement of expenses. Please determine with your hospital accounting department what records they require to ensure that study visit payments are kept separate from receipts or other documentation required to obtain payment for reimbursable expenses.

Consent Form Considerations

For clarity to potential subjects and the IRB who will be reviewing your center's study material and for calculating budgets, it is important that the consent form include the specific amounts to be paid for each visit. The consent form should not include blanket statements like "you will be paid $30/hour for participating in this study" for two reasons: 1) if a visit exceeds the time that you estimated, the subject may expect to be paid for the additional time; and 2) it is difficult for an IRB to calculate the total compensation that is being offered and thus evaluate the proposed compensation appropriately. An example of consent language that is clear and provides complete information for both subjects and the IRB is found below:

Example Consent Language

Payments to Participants

All subjects will be paid up to $600.00 to cover the expenses and time for participating in the study. The following amounts will be paid for each completed visit:

Visit 1	$90	Visit 6	$90
Visit 2	$90	Visit 7	$60
Visit 3	$60	Visit 8	$60
Visit 4	$60	Visit 9	$60
Visit 5	$60	Visit 10	$60

If the study is not completed, subjects will be paid for those visits that are completed and will be prorated accordingly, plus $60 for an Early Termination Visit.

All subjects will receive reimbursement for parking and mileage (at the current federal business mileage rate). Mileage will be determined using a computer program called Streets and Trips.

CYSTIC FIBROSIS FOUNDATION THERAPEUTICS

Successful Research Subject Referral for CF Studies: Tips for the Study Sponsor

A. Introduction & Overview

Many new studies are currently being initiated in the CF population and often these studies include restrictive eligibility requirements or esoteric procedures that may only be available at a smaller sub-set of study sites. As a result, study sponsors are encouraged to plan for the need for research subject referrals to successfully enroll their studies. This document provides tips for sponsors to successfully facilitate the referral process to meet enrollment goals.

B. Definitions

Study site: The site conducting the study.

Referring site: The site or physician that is not conducting the study but that provides care to CF patients who may be eligible to participate in the study.

C. Tips for the Sponsor for Successful Partnership

Site Selection Process

Notify study sites during the site selection process that you will support referral of subjects from other sites and provide a summary of the support that you will provide. This enables study sites to consider how they will be able to support referrals and to include referral recruitment numbers in their enrollment estimates.

Study Information Packet

Provide the study site with a packet of **non-confidential** information that can be shared with potential referring sites. This packet ideally will include:

- A synopsis of the protocol, including eligibility criteria
- Study-specific subject recruitment materials (e.g. scripts, recruitment letter)
- Travel and Other Expenses for Referred SubjectsIf airfare or overnight hotel stays are required these should be paid directly by the company rather then expecting patients to pay for these expenses out-of-pocket and wait for reimbursement. It is strongly advised that the sponsor contract with a travel agent for the highest level concierge service to assist potential subjects with their travel arrangements as study site research staff generally do not have time for this activity. If study site staff are required to make the arrangements, expect to pay them for any additional time required to perform these services.
- For other types of out of pocket expenses (e.g.; mileage, food, taxi, parking) it is imperative that subjects are reimbursed in a timely manner.
- Please utilize the Greenphire ClinCard System, or similar type of clinical trial subject payment system, to ensure out-of-pocket expenses are paid for in real-time making payments simpler, faster and easier for study participants and study sites.

IRB Materials for the Study Site

- Provide study sites with written instructions regarding travel arrangements that can be included in their initial IRB applications. These instructions should include how arrangements for travel for referred subjects are to be made, how reimbursement of subject expenses will be made, what items will be reimbursed, what reimbursement rates are and requirements for documentation. Include details explaining which expenses will be paid directly by the sponsor and which will require that the subject retain receipts to receive reimbursement.
- Additionally, the informed consent template provided to the study sites should include template language to accommodate referral subjects.

Study Site Budget and Contract

The following costs should be included in the study site budget if referral subjects are anticipated:

- Compensation for the extra coordinator time involved in obtaining and reviewing medical records for referred subjects (eligibility criteria, AEs, SAEs and concomitant medications throughout the study).
- Any pass-through expenses not directly paid by the sponsor.

Finder Fees

It is **NEVER** acceptable to offer a finder fee for referral.

Publications Guidelines for Industry Sponsors of CFFT TDN Studies

A. Purpose and Scope

Rapid dissemination of knowledge gained from clinical studies performed within the CFFT Therapeutics Development Network (TDN) is a fundamental principle of the network, TDN investigators, people with CF who participate in the trials, and the Cystic Fibrosis Foundation. For this reason, the TDN has formed a Publications and Presentations Committee (PPC) and has developed operational guidelines for industry- and investigator-sponsored clinical trials.

The following guidelines provide industry sponsors with details about the process and associated expectations for review of manuscripts and other publications and presentations that present primary study results from TDN studies. These guidelines were created to ensure the reliability, quality, and integrity of such publications.

TDN members are required to follow the related policies and procedures described in the *TDN Study Publication Authorship Guidelines* and the *TDN Guidelines for Submission to the PPC*.

B. General Information

TDN Study

TDN studies are defined as multi-center (two or more centers) studies that have been evaluated by the TDN Protocol Review Committee and/or the TDN Clinical Research Executive Committee. TDN studies can be investigator-initiated or industry-sponsored, and may be clinical or observational in nature. TDN study data refers to the aggregate data from the participating sites and not to the individual site data.

Goals for Communication of Study Results

Sponsors of TDN studies are expected to promote transparency and timely dissemination of clinical study results by using best efforts, in good faith, to publish the data and results of the study in accordance with generally accepted academic, medical and scientific standards and practices, regardless of whether the results support or present negative data about the study or the medical treatment.

Communication of study results may include publication of a paper in a peer-reviewed journal, abstract submission with a poster or oral presentation at a scientific meeting, or making results public by some other means. In all cases, the study results should be reported in an objective, accurate, balanced and complete manner, with a discussion of the strengths and limitations of the study.

It is particularly important to the investigators within the TDN that study results for ALL studies conducted within CF are published for the following reasons:

- People with CF are asked to repeatedly participate in studies and as such, it is important that they feel like their time and effort in participating in the study contributed to scientific knowledge regardless of whether or not the study had a positive or negative result.
- The investigators within the TDN are largely from academic institutions. Their ability to continue to participate in studies is hampered when their time and effort in participating in a study is not made evident by a culminating publication or presentation of the data.
- Negative studies may provide information regarding potential safety issues or potential lack of efficacy issues of similar products in the same class.

Sponsor and Investigator Responsibilities

Industry sponsors of TDN studies are strongly encouraged to submit their manuscripts to the PPC for review before submission to a journal. TDN Lead PIs are expected to encourage sponsors in this activity. The PPC can be contacted by e-mail: TDN_PPC@seattlechildrens.org.

For industry-sponsored studies, at minimum it is expected that the sponsor will provide the Lead PI/First Author the opportunity to review relevant statistical tables, figures, and reports for the entire study.

Additionally, in accordance with the Uniform Requirements for Manuscripts Submitted to Biomedical Journals (available at http://www.icmje.org) it is expected that all manuscripts, abstracts, posters, and presentations derived from the aggregate data of multi-center TDN studies will be reviewed by the Lead PI/First Author and other listed authors prior to their public presentation at scientific meetings or submission to a journal or other publisher.

Timelines

Timelines for communication of the study results should be established by joint agreement of the study sponsor and Lead PI at the conclusion of the study.

Sponsors are requested to make a summary of the study results available to the study investigators prior to public dissemination of the data.

C. Review of Manuscripts

Sponsor Review

Unless otherwise specified by contract, TDN First Authors are responsible for submitting manuscripts to industry sponsors for review at least 30 days in advance of submission for publication. Sponsors are asked to perform that review in a timely manner, generally within 15 days for presentations and 30 - 60 days for manuscripts. The PPC should also be notified at that time and will arbitrate any disagreements with the sponsor.

PPC Review Process

The primary focus of PPC review of manuscripts is to ensure, at minimum:

- That interpretation of the data, to the best of their ability to determine it, is accurate
- That interpretation of the data is not overstated
- That appropriate parties are acknowledged
- That appropriate acknowledgement of funding sources is made

The process for PPC review is as follows:

1. Whether submitted directly from an industry sponsor or by a TDN First Author, manuscripts should be submitted by e-mail to TDN_PPC@seattlechildrens.org.
1. Three PPC members, including one TDN statistician, are selected and provided with the manuscript.
2. Assigned PPC members review the manuscript and return comments within two weeks of receipt.
3. The reviewers' comments are summarized and provided to the First Author (or sponsor as appropriate) within one week of receipt. There are two possible outcomes following PPC review:
    a. The manuscript is endorsed, with no additional review needed; or
    b. Revisions and PPC review of revised manuscript are requested before endorsement.

i. If revisions are requested, the First Author (or sponsor) is responsible for revising the manuscript or providing a point by point response to revisions requested but not made. In an effort to rapidly disseminate study results, the PPC requests resubmission within one month of receipt of PPC response.

ii. The PPC Chair and reviewer(s) requesting revisions will review the revised manuscript and respond to the First Author within one week of receipt.

iii. If, after discussion with the TDN Steering Committee, the PPC does not endorse a manuscript, the PPC will notify all authors and provide the final review document.

TDN MANUAL OF OPERATIONS

TDN Guidelines for Study Subject Follow-up Communications

Introduction

Purpose and Scope

This guideline provides for a unified approach for follow-up with study subjects. Individual study subjects are an integral and valuable part of CF studies. In the past, subjects have not always been thanked for their participation nor received any further communications about the study once their individual participation ended.

Responsibilities

It is the responsibility of each Therapeutics Development Center (TDC) to follow the guidelines contained in this document. It is the responsibility of the TDN Coordinating Center (TDNCC) to provide study specific follow-up template letters to participating sites for CFFT-funded, TDNCC-managed studies. TDC staff is encouraged to notify industry sponsors of their desire to provide appropriate study subject follow-up and ask for their cooperation in doing so.

Steering Committee Oversight

All significant revisions to these guidelines or to the standard "TDN Study Subject Thank You Letter" template must be reviewed and approved by the TDN Steering Committee.

Process

Study Start-Up

CFFT Funded/TDNCC Managed Studies

1. The TDNCC clinical study manager will customize the TDN "Study Subject Thank-You Letter" template by adding study specific information.
2. The TDNCC clinical study manager will distribute the study specific template to participating sites along with the protocol and other regulatory packet materials.
3. The RC at each study site should then adds site-specific information to the templates and submit them for IRB approval (if required).

Industry Sponsored Studies

1. The TDNCC will provide sponsors who have their protocols reviewed by the TDN PRC with a copy of the Study Subject Thank You Letter template. Sponsors will be informed that the TDN expects sites to send thank you notes to all subjects in all studies at the end of their participation, and will encourage them to customize the document and provide it to all participating sites in their start-up packets.

2. If a subject thank you letter template is not provided by the sponsor, the RC at each study site is **strongly encouraged** to modify the standard TDN thank-you letter template to be both study and site specific and include it in their initial IRB submission.
3. Note that for some studies, the content of the "Thank You" letter may need to be less specific in the second paragraph of the template letter with regard to the availability of additional information at study completion. (i.e., unless the sponsor has agreed to provide a study summary and a treatment assignment list, the thank you letter should not mention that additional information will be available at study completion.)

End of Individual Study Subject Participation

When each study subject at a site completes his or her study participation, the RC at that site should individualize the study/site-specific, IRB-approved "Thank You" letter and send it to the study subject.

End of Study Follow-up

CFFT Funded/TDNCC Managed Studies:

1. The Lead PI is expected to prepare a summary of the study results in lay language once the final statistical report is completed and submit it through the TDNCC to the TDN Publications and Presentations Committee (PPC) for review. The approved summary will be forwarded to the CFF for posting on cff.org.
2. If a summary of study results is not provided, the staff at the TDNCC will prepare a summary based on published presentation of results by the Lead PI at a national meeting and submit it to the PPC for review. The approved summary will be forwarded to the CFF for posting on cff.org.
3. The TDNCC clinical study manager will request a list of all treatment assignments from the TDNCC Biostatistics and Clinical Data Management (BCDM) group or from the vendor who provided these services.
4. At a time deemed appropriate by consensus of the Lead PI and the study statisticians (typically after the results of the study have been presented to participating investigators and the scientific community), the TDNCC clinical study manager will forward the site-specific treatment assignment information to all participating sites.
5. The RC at each participating site is expected to provide treatment assignment information to study subjects upon request.

Industry Sponsored Studies

1. A summary of study results in lay language will be prepared by staff at the TDNCC based on sponsor press releases or published presentation of results by the Lead PI at a national meeting. The summary will be submitted to the TDN Publications and Presentations Committee (PPC) for review. The approved summary will be forwarded to the CFF for posting on cff.org.
2. When a peer-reviewed publication is available, staff at the TDNCC will compare the results with the original summary, make appropriate revisions, and submit the revision the TDN PPC for review. The approved summary and publication citation will be forwarded to the CFF for posting on cff.org.
3. If a site specific treatment assignment list is not provided by the sponsor, the RC at each participating site may request this information from the sponsor and provide it to study subjects upon request. Usually this information will only be provided to study sites after the results of the study have been presented to participating investigators and to the scientific community.

Study Result Summaries

Study result summaries that are posted to cff.org are written for the lay audience. A factual, objective review that provides the key findings of the study outcomes is provided in the following format:

- Primary Efficacy: Brief description of study and results of the Primary Efficacy endpoint, if applicable
- Secondary Efficacy: Brief summary of the other efficacy endpoint results
- Safety: Brief summary of key safety outcomes
- Citation: Journal reference

Sample Results Summary

TRIAL RESULTS:

Primary Efficacy:

This study enrolled 151 CF patients to evaluate the safety, tolerability and efficacy of three dose levels of inhaled Aeroquin (120mg given once daily, 240 mg given once daily and 240 mg given twice daily) administered for 28 days. The trial met the primary endpoint of a reduction in sputum Pseudomonas aeruginosa density at Day 28, the end of treatment. All three doses of Aeroquin demonstrated statistical significance ($p < 0.01$ for each group versus placebo), with the highest dose (240 mg BID) showing the greatest effect of approximately 1 log reduction.

Secondary Efficacy:

Statistically significant improvements in respiratory function and reduction in the need for other antibiotics were also demonstrated in the trial.

Safety:

The percent of patients with adverse events (AEs) was similar across all treatment groups, with no evidence of increasing incidence or severity of AEs with increasing Aeroquin dose.

Citation:

Am J Respir Crit Care Med 2011;183(11):1510-1516

Template

A copy of the Subject Thank You Letter template text is included below for reference. A customizable version of this document (in MS Word format) can be found in the Clinical Research Toolbox on CF ClinicalResearchNet.

Date

<Subject's_ Name>
<Address_1>
<Address_2>
<City_State_Zip>

Dear <Greeting>:

On behalf of <institution>, the CFFT Therapeutics Development Network, and < Sponsor Name>, we want to extend our sincere thanks for your participation in the <Trial_Name> research study/clinical trial. Your contribution of time and commitment to this research helps us find ways to improve care for people with cystic fibrosis and make progress towards treatments that will help ensure longer and healthier lives.

Results of this study should be available on the CF Foundation's Web site (www.cff.org) a few months after its completion. If you have any questions regarding this study, please contact our Research Office at <Research_Address> <Research Office Phone Number>, or <TDN Site PI for Trial Name with contact information>.

Include the following sentence for blinded, controlled studies: After study results have been posted, please contact our Research Office if you are interested in learning more about the study, including your treatment assignment.

Sincerely,

PI

RC