

¶1 **Organizational Structure and Guidelines for the CDC Cooperative Studies in the Prevention of Bleeding Disorder Complications through Hemophilia Treatment Centers**

I. OVERALL GOALS AND PURPOSE

- ¶2 **DATA:** This document addresses the collection and release of the data gathered collaboratively throughout the cooperative agreements between Hemophilia Treatment Centers (HTCs) and the Centers for Disease Control and Prevention (CDC) in the Prevention of the Complications of Bleedings Disorders Through Hemophilia Treatment Centers (hereafter, the *cooperative agreement*).
- ¶3 The data collected and analyzed in this cooperative agreement is that of the Universal Data Collection (UDC) through which the HTCs participate in a public health surveillance system established to monitor health outcomes for people with bleeding and clotting disorders receiving care in federally funded hemophilia treatment centers.
- ¶4 **INVESTIGATORS:** The agreement will assure that participating investigators will be appropriately represented and have the opportunity to be involved in data analysis and the preparation of papers and presentations.
- ¶5 **QUALITY:** It is the commitment of all parties involved that prior to dissemination, the data will be scientifically reviewed for the quality and integrity of the content and methodologies of collection; openly evaluated among all principal investigators and the CDC; and that due attention will be paid to the confidentiality of all patients enrolled in these studies.
- ¶6 **OVERSIGHT:** The oversight and guidelines established in this agreement will assure that public data releases, abstracts, interviews, presentations, and publications are accurate and objective in order to strengthen the validity and integrity of the collaborative study.
- ¶7 **SUMMARY:** It is the intent of this cooperative agreement and its investigators to improve the health and well being of all individuals with coagulation disorders through these studies. Therefore, all investigators, Regional PIs and CDC, through the cooperative agreement, will endeavor to facilitate these goals to optimize research efforts and to facilitate the expeditious dissemination of data in articles, reports and presentations which will result in improved long term outcome and quality of life for all patients with bleeding disorders.

TERMINOLOGY

(Abbreviations and Definitions in Appendix G)

- ¶8 **The Cooperative Agreement Regional Principal Investigator (PI):** Regional PIs are the principal investigators for the 12 regional sites that were awarded funds through the cooperative agreement.
- ¶9 **The Coordinating Committee (CC)** is composed of the Regional PIs and the CDC Scientific Collaborator (voting) plus the regional coordinators and/or regional directors (non-voting).
- ¶10 **Hemophilia Treatment Center Network (HTCN)** refers to the collective group of HTCs that receive funding (either as an awardee or subcontractor) and participate in these activities through the cooperative agreement.
- ¶11 **HTCN Principal Investigators** are those individuals listed as the principal investigators (PIs) for each HTCN site within the twelve Regions.
- ¶12 **HTCN co-investigators** include any co-investigators or project staff documented in the grant file or HTC staff as verified by the local PI, indicating an official involvement with the HTCN project. . A current list of all co-investigators will be maintained on the project website: hemophiliaoutcomes@pbworks.com
- ¶13 **Outside investigators** are investigators who do not have any official role noted in the grant file for an HTCN site or within the working groups formed under the direction of the Coordinating Committee.

II. SCOPE OF THE PUBLICATION GUIDELINES

- ¶14 This policy covers analyses, presentations, abstracts, posters, press releases, and papers/manuscripts utilizing unpublished surveillance data from the UDC pooled dataset or that combine data from more than one region of the 12 grantees. In addition, this policy addresses proposals for data analysis, papers, abstracts, and presentations from *outside* the network, that involve unpublished data and surveillance information collected as part of the UDC and compiled at the CDC. Surveillance data elements that contain individual identifiers and are collected using CDC funding for a single region and its own HTC network, or an individual HTC's own such data, are considered Individual Site Data and are not covered by these guidelines.
- ¶15 **Exemptions:** Reports made by CDC that constitute public health practice are exempt from this policy and include, but are not limited to, periodic surveillance reports and electronic data summaries, reports to Congress, and reports to other government agencies as part of public health investigations (e.g., investigations of possible disease transmission through blood products). Unless prohibited, pertinent aspects these reports will be provided to the Coordinating Committee upon release.

HTCs can include UDC data in government peer-reviewed grant applications (marked "Preliminary HTCN data which is strictly confidential and for this grant application only"). Any UDC data that have not been previously published can not be reported in non-government proposals without the approval of the Coordinating Committee.

¶16 These guidelines are implemented through the Coordinating Committee and its major focus area working groups and subcommittees in order to optimize and prioritize the efforts of all investigators in the HTCN and CDC. These policies will remain in force until the Coordinating Committee is formally dissolved.

III. THE COORDINATING COMMITTEE AND ITS SUBSIDIARIES

A. Coordinating Committee

¶17 **Role:** The Coordinating Committee is the final decision-making body for data use and release under the scope of these guidelines. It is the role of the Coordinating Committee to set the research agenda for each competitive grant cycle once the cooperative agreements are awarded and to facilitate the successful completion of these specific aims. It is responsible for the planning and implementation of the cooperative aspects of the study. The CC addresses issues of common concern throughout the life of the project and is responsible for all aspects of the data collection, release, and publication. The CC may create, and dissolve, working groups and subcommittees to facilitate its work and has the final say on recommendations made by these groups.

¶18 Coordinating Committee will accomplish these tasks during two meetings per year and monthly teleconferences. The CC will establish working groups to focus on priority areas of research which will report back to the CC . The CC and the major working groups in their respective areas will:

- (1) Make recommendations on the UDC study protocol, major areas of study with associated subcommittees, and data collection approaches;
- (2) Discuss common protocols as they relate to all data and established research priorities;
- (3) Discuss the target populations that have been or will be recruited;
- (4) Identify and recommend solutions to unexpected study problems;
- (5) Discuss ways to efficiently coordinate study activities and best practices; and
- (6) Take decision on approval of manuscripts and presentations, proposals for analysis and other recommendations from working groups and committees.

Composition: The Coordinating Committee consists of the Cooperative Agreement Regional Principal Investigators (PI) and the CDC Scientific Collaborator (voting) plus the regional coordinators and/or regional directors (non-voting). A chair and a chair-elect are elected from the membership. The chair will serve for two years; the chair elect will serve for one year prior to assuming the Chair. The ex-Chair will serve on various committees (listed below) and be available to the chair to aid in transition as needed in the year after stepping down from the chairmanship.

¶19 **Practices:** The Coordinating Committee meets annually or semi-annually and holds monthly teleconferences. Decisions are made by vote when a quorum is in attendance.

Voting Rules:

Voting Members: Principal Investigators (12) or alternate/proxy; CDC representative (1). Total (13) voting members may designate alternates to vote in their absence. A quorum must be in attendance to conduct business (Roberts Rules, 10 ed p334-335.)

Alternates: A proxy vote may be assigned by the PI to the regional director /coordinator from their region which is determined before the vote and is recorded during the initial role call for each meeting or teleconference.

Quorum: 2/3 of voting members (9)

Approval: If quorum in attendance, a simple majority of all voting members present required to carry. (50%+1)

Revision of guidelines: Proposals to revise any of the guidelines herewith shall be submitted to the CC 3 weeks in advance of a subsequent meeting/call.

Approval of guidelines revisions: A 2/3 majority of voting members present is required to carry.

Proxy documents are maintained and kept current by each PI and the Chair of the CC. They will also be posted on hemophiliaoutcomes@pbworks.com. Coordination with working groups and subcommittees occurs through shared membership and through regular reports to the CC from the working groups and subcommittees.

B. Major Focus Working Groups

Role: The major focus working groups are created by the Coordinating Committee. They represent major areas of focus for research during the current funding cycle. These groups aid the CC in the coordination and quality assurance of UDC-based research; coordinate those research projects related to their focus area; and also provide an arena in which HTC/N staff can participate and contribute to the project. Group members participate in review of analysis proposals related to their area and guide projects through analysis.

¶20 The major focus working groups are:

1. Joint Outcome Group (JOG)
2. Socio-Economic Determinants of Health Status (SEDHS)
3. Healthy Weight (HW)
4. Rare Bleeding Disorders (RBD)
5. Inhibitor Studies Group (proposed)
6. Women and Bleeding Disorders

¶21 **Composition:** Membership is drawn from interested HTC and CDC staff. Two co-chairs (usually CC members) lead each working group. Comprehensive professional representation is not required but may be sought ad hoc.

¶22 **Practices:** Meeting/teleconference schedules may vary as needed to accomplish the work. Co-chairs participate in monthly teleconferences with the Coordinating Committee. Coordination with the CC and all committees (major working groups and subcommittees listed below) occurs through shared membership and participation and through regular reports to the CC. The Co-chairs are responsible for posting updated rosters, minutes, ongoing projects, drafts, and other working documents on hemophiliaoutcomes@pbworks.com which has been established to facilitate communications and is password protected.

C. Subcommittees

¶23 1. Selection of Subcommittee Members

Candidates will be solicited directly from HTCN staff. Each candidate should submit their CV and a personal statement describing their experience and interest. These will be posted online for others to review. The candidates will be evaluated and voted upon by the CC and the chairs of the working groups at the annual meeting in executive session.

¶24 2. Membership terms

Members who initially populate the committee will choose terms of 2, 3 or 4 years. Once rotation begins, members will serve a first term of 3 years with an option of 2 more years (pending re-approval by the CC). Members may serve again after being off for 2 years. Members can participate on other committees or working groups without the 2-year hiatus. Members may be removed by the CC upon recommendation of the subcommittee based on performance, for example, if unable to participate or complete assigned tasks.

3. Communication and Collaboration between Subcommittees

¶25 The Project Review and Informatics/Data Quality subcommittees have distinct functions but interdependent objectives. Close integration of their work is desired. Coordination with the CC and committees occurs through shared membership and participation and through quarterly reports to the CC. Monthly subcommittee minutes will be emailed to all CC members as they are approved and posted on hemophiliaoutcomes@pbworks.com. The Project Review Committee and the Informatics/Data Quality Committee shall meet together at least once a year.

4. Project Review Committee (PRC)

Role: The Project Review Committee evaluates all new non-exempt proposed data analysis projects from the working groups, CDC, HTCN and outside investigators. The PRC may also refer projects to the appropriate working group(s) or provide oversight and assistance to projects in areas not covered by a working group. The PRC may initiate coordination between working groups for projects with overlapping focus areas.

Importantly, the PRC will also track the progress of ongoing projects that are not already designated to a specific WG and report same to the CC on a quarterly basis. Projects approved by this committee based on scientific merit must then be sent to the

Coordinating Committee for final approval who must then base their final approval on available resources.

¶26 **Composition:** Membership is interdisciplinary and shall consist of:

One co-chair of each working group or their delegate	(5)
The chair-elect of the Coordinating Committee	(1)
A representative from the Informatics/Data Quality Committee	(1)
A physician in addition to those drawn from other committees/working groups	(1)
A social worker	(1)
Two nurses, representing a variety of demographics	(2)
A physical therapist	(1)
A consumer	(1)
CDC staff (1-2)	(+)
Total # members	13+

Ad hoc members may be utilized as necessary. The chair of the PRC will either be the ex-chair of the CC committee or the chair elect of the CC to provide maximum input during the review of the proposal relative to its novel approach, synergy with current major focus working groups or potential redundancy with ongoing projects.

5. Informatics/Data Quality Committee (IDC)

¶27 **Role:** The Informatics/Data Quality Committee focuses on the data collection tools, methodology, data quality and data access in collaboration with the Division of Birth Defects (DBD) Informatics Team to monitor and improve data consistency and completeness; provide reports quarterly to the CC; facilitate the establishment of new datasets and questionnaires; coordinate and design data forms revisions; harmonize data collection forms; improve access to data for HTC investigators; and develop novel analytic approaches to the data. Revisions and new procedures proposed by this committee must be approved by the CC.

¶28 **Composition:** The Informatics/Data Quality Committee is comprised of no fewer than 7 and no more than 10 individuals with data evaluation and use skills. One member should be from the DBD Informatics Team or their designee; another member from ATHN WebTracker/Technology Committee, the remaining members should be comprised of individuals with multidisciplinary background and ad hoc members may be utilized as needed.

6. Presentation and Publication Committee (PPC)

Role: The Presentation and Publication Committee evaluates for approval all non-exempt manuscripts, abstracts, press releases and presentations. Items approved by this committee will be sent to the Coordinating Committee for final approval.

¶30 **Composition:** The Presentation and Publication Committee is a pool of 15-20 persons who have published, have other writing experience (such as dissertations) and/or have significant experience in reviewing articles and who represent various professions within

the HTCs. Ad hoc members may be used for specific expertise. The immediate past chair of the Coordinating Committee will chair this committee and one individual from each working group shall serve.

- ¶31 As the workload dictates (such as preceding meetings in which the PPC and CC members are strongly involved), it may be necessary to form ad hoc committees to review abstracts in a timely manner. These groups would rotate in membership in order to assure that the proposed abstract is reviewed fairly and so that the effort is shared by all members of the committee. Recommendations for acceptance, acceptance with revision, or rejection are made by the PPC and sent to the CC for review and final notification of the authors.

IV. PROCEDURES

A. Approval of Proposals for Analyses

- ¶32 1. The investigator completes and submits via email a Proposal for UDC Data Analysis (Appendix B) to the CDC administrative designee and the Chair of the Coordinating Committee prior to being sent to the Project Review Committee.
- ¶33 2. After the project is deemed acceptable, if the initiating investigator is not a Regional Principle Investigator or CDC staff, but is an HTC sub-awardee or a team member at an affiliated HTC, the Regional PI will certify that the investigator is a member of the network and ask that any non-member sign the confidentiality agreement listed in appendix E.
- ¶34 3. The administrative designee forwards the proposal to the chair of the Project Review Committee, who assigns a primary and a secondary reviewer and forwards the proposal to all members of the PRC. When applicable, the primary reviewer will be chosen from the major focus area working group to which the analysis pertains. The chairs of the working groups may designate a reviewer from their group.
- ¶35 4. Each proposal will have a primary and a secondary reviewer. The primary and secondary reviewers read the proposal, complete a standard scoring sheet (Appendix C) and the primary reviewer will provide a written paragraph discussing the strengths and weaknesses of the proposed analysis.
- ¶36 5. Within 4 weeks of receipt of the proposal, the Project Review Committee will discuss the proposal to determine whether it is scientifically sound, whether the scope of the analysis is reasonable, and that there are no conflicts with existing analyses being conducted by other HTCN investigators or the major focus area working groups. The Project Review Committee may approve or disapprove a proposal and will make suggestions, if needed, to improve the scientific aspects of the study or suggest collaboration with other HTCN investigators. The PRC will vote on the proposal as 1) Accepted and may proceed, 2) Requires refinement by author in response to review, 3) Additional information is needed or 4) Not approved in current format. Modifications (refinement) should only be required for reasons of scientific or programmatic appropriateness or validity.

- ¶37 6. Proposals that are accepted by the PRC are forwarded to the CC for discussion at their next call. The CC will vote on each proposal as 1) Accept as is, 2) Accept with recommended changes, 3) Reject with comments. A proposal will be approved by the CC if at least 2/3 of the members present approve the proposal. The chair of the PRC will provide a final written response to the investigator.
- ¶38 7. Proposals that are judged to need additional work, as in 2) above, “Accept with recommended changes”, will be returned to the author with a written critique and request for a response within 90 days.
- ¶39 8. After approval of the research proposal by the CC, the CDC will work with the initiating investigator to provide the data management and epidemiologic support needed for the project. In the event that the proposal is aligned or complimentary to the work of a major focus working group, the working group co-chairs will ensure that the proposal and lead investigator be incorporated into the established working group. The investigator is not required to take part in working group activities unrelated to his/her analysis.
- ¶40 9. Proposals that are disapproved may be revised and resubmitted to the Project Review Committee.

¶ **B. Approval of Abstracts, Presentations, Press Releases, and Manuscripts**

- ¶41 1. Abstracts for presentations at scientific meetings, press releases, and manuscripts will be sent to the PPC and then the Coordinating Committee for approval prior to submission to an outside organization. It is the responsibility of the submitting author to see that all co-authors have already reviewed the document prior to submission to the PPC.
- ¶42 2. Submissions will be emailed to the administrative designee or sent to the PPC chair. The chair will assign three individuals to review the abstracts for an upcoming meeting. A press release can be reviewed by the chair and the CDC scientific collaborator to expedite and then sent on to the CC for final approval via teleconference for urgent material. Manuscripts will have two reviewers and a summary review will be written by the primary to be sent on with the submission to the remainder of the PPC members who will then discuss the submissions during monthly teleconference.
- ¶43 3. Submissions will be reviewed to determine that they are scientifically sound and that they meet the guidelines for authorship. A standard form with the guidelines for authorship will be signed and submitted with the paper by the each author (Appendix F). Reviewers will complete a standard evaluation form (Appendix D) and vote on each proposal as 1) Accept as is, 2) Accept with recommended changes, 3) Reject with comments.
- ¶44 4. The PPC will complete reviews within one week of submission for abstracts, press releases or presentations and within four weeks of submission for manuscripts. If necessary, a monthly review date will be established so that manuscripts received by the

first of the month will be reviewed and responded to by the end of the month. The PPC can recommend changes from the authors prior to sending completed review to the CC.

- ¶45 5. The PPC will forward its recommendations to the CC for final decision and to the DBD Associate Director for Science to initiate CDC clearance. For abstracts, presentations and press releases, the CC has 48-72 hours to respond; for manuscripts, one week.

A submission will be approved for publication if at least 2/3 of the responding CC members approve the submission. As the manuscripts represent work previously presented by the WGs, ongoing proposals, and projects familiar to the CC, little time should be required to review and approve the manuscript following the recommendations of the PPC.

The papers will be reviewed and approved via monthly teleconference. The CC will provide a written response to the authors immediately following the teleconference.

- ¶46 6. Under very limited circumstances, the author may call for an expedited review of an abstract or manuscript. Requests for an expedited review will be submitted to the CC chair and chair-elect with justification for the need to expedite the review.
- ¶47 7. Any presentations or publications with CDC personnel as an author or using UDC data must have CDC clearance prior to submission. One week should be allowed for clearance of an abstract; six weeks should be allowed for clearance of a manuscript.
- ¶-48 8. Abstracts containing UDC data will be authored only when a Proposal for UDC Data Analysis (Appendix B) has been approved request for analysis and publication has been approved.
- ¶49 9. Abstracts or manuscripts that are disapproved by the PPC or the Coordinating Committee may be revised and resubmitted.
- ¶50 10. Final authorship or acknowledgement status of an investigator will be determined in accordance with the Uniform Requirements for Manuscripts Submitted to Biomedical Journals, developed by the International Committee of Medical Journal Editors, and available at www.ICMJE.org.
- ¶51 11. It is the responsibility of the lead investigator/senior author to determine if a re-review of a manuscript by the PPC /Coordinating Committee is necessary because of substantial revision of a paper in response to peer reviewers.
- ¶52 12. A copy of each accepted abstract and manuscript will be sent to the Coordinating Committee and then posted to hemophiliaoutcomes@pbworks.com to track productivity of the group and to share published data with the HTCN within a week of acceptance.

- ¶53 12. For all data or information that has been collected or analyzed using CDC funding and/or the HTC� surveillance methodology or tools, references to the funding source(s), including CDC, and the collaboration of the *HTCN* must be made.
- ¶54 13. For publications, journal articles, etc. considered public health practice and produced under a CDC grant-supported project, an acknowledgment and disclaimer must be displayed such as: This publication (journal article, etc.) was supported by Grant/Cooperative Agreement from the Centers for Disease Control and Prevention. Its contents are solely the responsibility of the authors and do not necessarily represent the official views of (name of awarding agency).
- ¶55 14. For publications, journal articles, etc. considered research that include data collected from public health practice produced under a CDC grant-supported project, an acknowledgment must be displayed such as: The analysis and writing represented in this publication (journal article, etc.) was supported by Grant/Cooperative Agreement *Prevention of Bleeding Disorder Complications through Regional Hemophilia Treatment Centers*. This requirement also applies to the surveillance data collected under the Grant/Cooperative Agreement from the Centers for Disease Control and Prevention.
- ¶56 15. Any use of the “CDC” or “HHS” logo must first be approved through official CDC and HHS clearance processes.

V. STATUTE OF LIMITATIONS ON ANALYSIS AND REPORTING

- ¶57 Any approved analyses, abstracts/presentations, or manuscripts should be completed in a timely manner. If the approved project is not initiated within a reasonable amount of time (a first draft or preliminary results submitted to the Coordinating Committee within 1 year or less), other HTC� investigators may submit an application to the Coordinating Committee to allow other investigators access to those data or reporting privilege. In the case of a dispute over use and reporting of data in a timely manner, the issue will be brought to the Coordinating Committee for a vote.

VI. DATA POSTED ON THE CDC WEBSITE

- ¶58 Currently, reports of aggregate data from the UDC and associated questionnaires are posted on the CDC website. This data should be reviewed on at least an annual basis by the members of the CC, prior to posting, to help assure accuracy. Once compiled by the CDC and/or associated working groups, these data may be reviewed at the spring meeting or reviewed via teleconference prior to posting. A review of which data should be posted should also be discussed on at least an annual basis.

VII. AVAILABILITY AND ANALYSIS OF DATA BY OUTSIDE INVESTIGATORS

- ¶59 1. The use of the UDC pooled data will initially be limited to HTC� PIs, co-invest - igrators, CDC and the Regional PIs. If HTC� PIs wish to collaborate on a project with

non-HTCN investigators (“outside investigators”), they may submit a proposal to the Coordinating Committee and allow other HTCN PIs the first opportunity to serve as collaborators on the particular project. Outside investigators must sign a Confidentiality and Data Use Agreement (Appendix E).

- ¶60 2. At a point to be determined by CDC Guidelines and the Coordinating Committee, the network data may become available to outside researchers in the form of a limited access dataset. The availability of the data to outside investigators will be in accordance with CDC policies on public use datasets and Data Sharing (www.cdc.gov/od/ads/pol-385.htm). Requests for UDC data and their analyses will be submitted to the Coordinating Committee as described in the guidelines.

Appendix A

Cooperative Agreement Regional Principal Investigators

Region I

Doreen B. Brettler, MD
UMASS Memorial Hospital
Worcester, MA

Region II

Christopher E. Walsh, MD, PhD
Mount Sinai Medical Center
New York, NY

Region III

Regina Butler, RN
Childrens Hospital of Philadelphia
Philadelphia, PA

Region IV-North

Paul E. Monahan, MD
University of North Carolina at Chapel Hill
Chapel Hill, NC

Region IV-South

Christine Kempton MD
Emory University
Atlanta, GA

Region V-East

Mr. Ivan C. Harner, FACHE
Hemophilia Foundation of Michigan
Ypsilanti, MI

Region V-West

Sandy Lampson
Great Lakes Hemophilia Foundation
Milwaukee, WI

Region VI

Debra Brown, MD
University of Texas – Houston
Houston, TX

Region VII

Brian Wicklund, MD
The Children's Mercy Hospital
Kansas City, MO

Region VIII

Marilyn J. Manco-Johnson, MD
University of Colorado at Denver and Health
Sciences Center
Aurora, CO

Region IX

Diane J. Nugent, MD
Children's Hospital of Orange County
Orange, CA

Region X

Robina E. Ingram-Rich, BSN, MS, MPH
Michael Recht, MD
Oregon Health & Science University
Portland, OR

Appendix B

PROPOSAL FOR ANALYSIS OF UDC DATA

Before beginning, please note:

- *The UDC data set contains persons of both genders and all ages; persons with hemophilia and von Willebrand disease, as well as some with other clotting factor deficiencies. Please be specific when stating your population of interest.*
- *Please review the data collection forms to make sure your proposed data elements exist, and that the data collected are appropriate to your analysis. Copies of the data forms can be requested from Meredith Oakley at moakley@cdc.gov or (404) 498-6729.*
- *Complete all items on this proposal.*

- **Date**

- **Title**

- **Initiating Investigator**

Name:

Institution:

Address:

Phone #:

Fax #:

E-Mail Address:

- **Co-Investigators and Collaborations**

- **Identified member.**

Is the initiating investigator an identified member of an HTC UDC project and listed with the IRB monitoring the UDC project? Yes/No

If Not, this initiating investigator must sign and return Prevention of Bleeding Disorder Complications through Regional Hemophilia Treatment Centers/UDC Confidentiality and Data Use Agreement, Appendix E , prior to receipt of data. The initiating investigator will be bound by the agreement.

- **I understand** that a working group will be assigned to assist me in the development of this concept. I agree to work with this group including participating on conference calls about my project and providing regular updates about the progress.

Signature: _____

- **Proposed Audience/Journal**

- **Background and Rationale** (Include any preliminary data)

- **Specific Aims of the Study**

- **Study Plan**

1. Hypothesis
2. Overall design (i.e., cohort study, case-control, if known)
3. Study population (inclusion/exclusion criteria)
4. Study outcomes or end points
5. Study variables
6. Statistical considerations (sample size, power calculations, special analyses) and analysis plan (table shells, model equations, test statistics)
7. Staged timeline for completion of project

- **Data Requirements**

1. UDC data elements that will be used, including the form on which they are collected, the item number, and the text of the question. **Example:**

Form Name	Item #	Text of Question
Annual Visit	10	Highest inhibitor titer since and including the last annual visit
Annual Visit	14	Home infusion

2. For variables which must be calculated or transformed (ex. - BMI, age, decrease in range of motion), explain how they will be calculated or transformed.
3. Additional data not in UDC that will be required.
4. If additional data are needed, how do you propose to obtain it?

- **Data presentation**

Include a format such as a table or figure that demonstrates how you anticipate the final data will be presented. For example if you wish to compare the annual number of joint bleeds and soft tissue hematomas in babies with hemophilia A broken down by severity your table might look like the following:

	Median # joint bleeds/yr	Median # soft tissue hematomas/yr
Severe hemophilia A		
Moderate hemophilia A		
Mild hemophilia A		

(This is only an example. Depending upon your analysis, your data presentation may be quite different.)

- **Resources**

1. What components can the investigator accomplish (i.e., data collection, analysis)?
2. What components would require assistance from CDC?
3. Will funding be available to the investigator for work on this study (e.g., K12 Career Development Award, grant)?

Please address any questions and submit proposals via email to the contact listed below. You will receive acknowledgment of your submission within one week.

APPENDIX C
UDC Data Analysis Proposal Review

Title of Proposal:

Initiating Investigator:

Sponsoring PI:

Center:

Date reviewed:

Reviewed by:

1. Background with relevant references YES NO NA
Comment:

2. Study plan

- hypothesis YES NO NA
- overall design YES NO NA
- study population YES NO NA
- outcomes/end points YES NO NA
- study variables YES NO NA
- analysis plan with power calculations YES NO NA
- other data collection or record matching YES NO NA

Comment:

3. Data Requirements

- UDC data elements YES NO NA
- calculated/transformed variables YES NO NA
- additional data required YES NO NA
- plan to obtain additional data YES NO NA

Comment:

4. Resources

- Assistance required from CDC YES NO NA
- Funding available YES NO NA

5. Scope of analysis is reasonable YES NO NA
Comment:

6. Conflicts with existing research YES NO NA
Comment:

7. Suggestions for collaboration YES NO NA
Comment:

**8. Need for additional IRB approval
Comment:**

YES NO NA

9. Other Comments:

- Accepted
- Requires Refinement in response to review
- Additional information needed
- Not Approved in Current Format

AUTHOR MUST RESPOND TO THE FOLLOWING COMMENTS:

Appendix D

UDC Manuscript Review Form

Title of Document:

First Author:

UDC Analysis Project #:

Reviewer:

Date reviewed:

1. Author list includes acknowledgement of HTCN YES NO
Comment:

2. Participating Centers and funding source acknowledged YES NO NA
Comment:

3. Comments on other issues (e.g. authorship, conflict with other UDC research, etc):

Recommendation:

APPROVE AS IS

APPROVE WITH MINOR REVISIONS

REVISE AND RESUBMIT, REVIEWER REQUESTS TO SEE REVISED MANUSCRIPT

REVISE AND RESUBMIT, REVIEWER DOES NOT WISH TO SEE REVISED MANUSCRIPT

DISAPPROVE (*please explain*):

Please provide comments on scientific aspects of the manuscript (*use as much space as necessary*):

Appendix E

Prevention of Bleeding Disorder Complications through Regional Hemophilia Treatment Centers/UDC

CONFIDENTIALITY AND DATA USE AGREEMENT

Each Center for Prevention of the Complications of Bleedings Disorders Through Hemophilia Treatment Centers has been awarded a Certificate of Confidentiality from the Centers for Disease Control and Prevention (CDC). In accordance with Section 301(d) of the Public Health Service (PHS) Act (42 U.S.C. 241(d)), I, as a _____ (Centers employee, CDC employee, scientist, colleague), am permitted access to personally identifiable data. As a condition of this access and my participation in this project, I am required to comply with the following safeguards and policy commitments for individuals against invasions of privacy.

1. I agree to be bound by the following :

In accordance with Section 301(d) of the PHS Act (42 U.S.C. 241(d)), all respondents are assured that the confidentiality of their responses in this study will be maintained, and that the privacy of research subjects is protected by the withholding of, from all persons not connected with the study, any personally identifying characteristics of the research subjects.

2. I agree to maintain the following safeguards to assure that confidentiality is protected and to provide for the physical security of the records:

To preclude observation of confidential information by persons not authorized to have access to the information on this project, I shall maintain all records that identify individuals, or from which individuals could be identified, in locked containers or protected computer files, when not under immediate supervision by me or another authorized member of the project. The keys or means of access to these containers or files are not to be given to anyone other than HTC authorized staff. I further agree to abide by any additional requirements imposed by CDC for safeguarding the identity of individuals.

3. The Coordinating Committee must approve uses of the UDC data. No analysis of data or dissemination of findings from the UDC may occur without approval from the committee for a specific research purpose. Instructions for submission of research proposals are specified in the Analysis and Publication Guidelines document available from each Center.
4. The Principal Investigator from each Center is responsible for tracking the use of the UDC data at their Center and assuring that each person who has access to the data has read and signed this agreement.

5. I understand that the Coordinating Committee must approve any manuscripts, abstracts, or public presentations based on the analyses before they can be submitted for consideration.
6. I agree not to attempt to identify any individual person whose information is contained in the UDC data.
7. I agree not to distribute, copy, or share the data with any person(s) other than those designated by the Principal Investigator of the Center.
8. At the conclusion of the research covered by this agreement, I agree to promptly return to the Center from which the data were obtained, any documentation and manuals about the UDC proposal, and to remove (delete) any electronic files containing data or output from any computer equipment which I have used to gain access to and/or to analyze UDC data.

My signature below indicates that I have carefully read and understand this agreement which pertains to the confidential nature of all records to be handled in regard to this project. As a _____ (Center employee, CDC employee, scientist, colleague), I understand that I am prohibited from disclosing any such confidential information that has been obtained under this project to anyone other than authorized staff of the Center. I understand that any disclosure in violation of this Confidentiality agreement may lead to termination of my employment, as well as other penalties.

(Typed/Printed Name)

(Signature)

(Date)

(Center PI)

(Date)

Appendix F

HTC NETWORK AUTHORSHIP CREDIT FORM

Title of Project: _____

Please check each task to which you have made a contribution, and sign at end of form. This form will be checked by the lead author to confirm your role as author of this paper.

A. PROPOSAL CONCEPT AND DESIGN

1. Development of the structure and proposal for this specific analysis or paper

B. STUDY CONCEPT AND ACQUISITION OF DATA

1. Initial conception and Design of the study represented in this paper
2. Development of the study design for data collection represented in this paper
3. Acquisition of Data
4. Case Review

C. ANALYSIS AND INTERPRETATION

1. Statistical consultation
2. Statistical analysis
3. Production of graphs and tables
4. Participation in discussions of analyses

D. WRITING

1. Authorship of first draft
2. Authorship of segments of paper

- 3. Reviewing and critiquing drafts
- 4. Review of literature, provision of citations

E. OTHER

- 1. Obtaining Funding
- 2. Administrative, technical, or material support
- 3. Supervision
- 4. Other

Signature _____ Date: _____

Print Name _____

Form adapted from

Paneth N, Separating authorship responsibility and authorship credit: a proposal for biomedical journals. *Am J Public Health*, 1998; 88(5):824-6

“Policies and Procedures for the Development of Publications from the ELGAN Study”, 1/26/05 draft.

JAMA Authorship Responsibility, Financial Disclosure, Copyright Transfer, and Acknowledgement form.

APPENDIX G

Abbreviations of Terminology

CC	Coordinating Committee
CDC	Centers for Disease Control and Prevention
DBD	Division of Blood Disorders
HHS	Health and Human Services
HTC/HTCN	Hemophilia Treatment Center/Hemophilia Treatment Center Network
IDC	Informatics/Data Quality Committee
JOG	Joint Outcome Group
PHS	Public Health Service
PI	Cooperative Agreement Regional Principal Investigator
PPC	Presentation and Publication Committee
PRC	Project Review Committee
RBD	Rare Bleeding Disorders
SEDHS	Socio-Economic Determinants of Health Status
UDC	Universal Data Collection