Foundation Fighting Blindness (FFB) Consortium

Governance Document

Version 7.0

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List of Abbreviations

ABBREVIATION	DEFINITION
AAO	American Academy of Ophthalmology
AE	Adverse event
ARVO	Association for Research in Vision and Ophthalmology
CFR	US Code of Federal Regulations
CRF	Case report form
eCRF	Electronic case report form
DSMC	Data Safety Monitoring Committee
EC	Ethics Committee
ERG	Electroretinograph
EU	European Union
FAF	Fundus autofluorescence
FFB	Foundation Fighting Blindness
FDA	Food and Drug Administration
FFB	Foundation Fighting Blindness
GCP	Good Clinical Practices
HIPAA	Health Insurance Portability Act of America
ICH	International Committee of Harmonization
IRB	Institutional Review Board
IRDs	Inherited Retinal Diseases
JCHR	Jaeb Center for Health Research, Tampa, FL
MRT	My Retina Tracker
OCT	Optical Coherence Tomography
ROC	Research Oversight Committee at FFB
US	United States

Glossary of Terms

Clinical Center: Any site or institution participating in a Consortium Study.

Data: Information related to the Study, including images, testing reports, CRFs, and data collected directly from devices.

Investigator: A physician or other qualified person who assists Principal Investigator by performing critical study-related procedures and/or making important study-related decisions.

IRB (Institutional Review Board): The ethics committee responsible for ensuring the protection of the rights, safety and well-being of human subjects involved in a study. Also known as an independent ethics committee (IEC), ethical review board (ERB), or research ethics board (REB). They may be independent or affiliated with the Clinical Center.

Protocol: The IRB-approved description of the study.

Study: The work performed by a Clinical Center’s investigators and other personnel in connection with the protocol.

Participant: As is defined in 21 CFR §312.3(b), means a person who participates in a Study.

Chapter 1: Background Information

1.1 Mission Statement

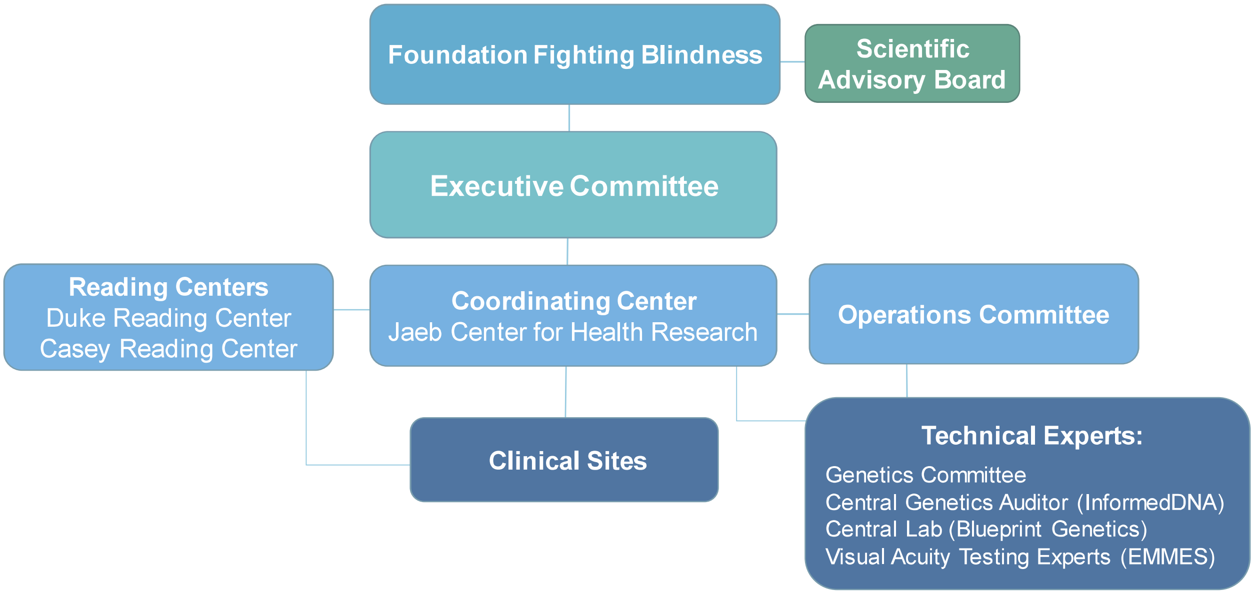
To accelerate the development of treatments for inherited retinal diseases (IRDs) through collaborative and transparent clinical research.

1.2 Amendments to this Policy Document

This is a controlled document for which the Executive Committee is accountable. Changes to the governance of the Consortium may be proposed by any Consortium Member and discussed and voted on by the Executive Committee. Changes to the document, date for the change and rationale for the change will be summarized in the Summary of Changes.

1.3 Organizational Structure

FFB is accountable for the FFB Consortium. The Consortium is comprised of an Executive Committee, an Operations Committee and the investigators of each Clinical Center. The FFB Scientific Advisory Board (SAB) will provide scientific advice to the Executive Committee; the Executive Committee may also reach out to other experts to provide specific advice. The Jaeb Center for Health Research (JCHR) is the Coordinating Center for the FFB Consortium, accountable for all operational activities.



1.3.1 Consortium Chair

The Consortium Chair (or 2 co-chairs) assumes overall scientific responsibility and direction for Consortium protocols, assisting the Coordinating Center with managing day-to-day Consortium activities. The Chair also serves as a spokesperson for the Consortium to the public. As a member of the Executive Committee, the Chair attends the Executive Committee meetings and has the same length of term and possibility for re-appointment. The Chair selection process may require input from FFB Science, ROC members, SAB members and the other Executive Committee members, as well as a formal Request for Application (RFA) process.

A chair-elect will be included 1 year in advance of the appointment of a new chair whenever possible. At the conclusion of the chair-elect term, there may be an informal evaluation by the EC as well as feedback from the chair-elect to mutually confirm the appointment. In the event of two co-chairs, following confirmation, a division of responsibilities will be determined. Co-chairs will be rotated off the committee at staggered time points.

1.3.2 Executive Committee

1.3.2.1 Membership

The Executive Committee will provide leadership to the Consortium. Membership will include, but may not be limited to:

1. The Consortium Chair (or two co-chairs)
2. Two or more clinical scientists, with at least one clinical scientist representing a site outside the United States
3. One or more clinical trialist expert (e.g., epidemiologist or biostatistician)
4. One or more representatives of the FFB Scientific Advisory Board or Research Oversight Committee, bringing expertise in translational research to the Executive Committee
5. An FFB liaison and one or more representatives of FFB leadership
6. The Director of the Coordinating Center at Jaeb Center for Health Research (JCHR)

1.3.2.2 Roles and Responsibilities

The Executive Committee will be responsible assuming overall scientific responsibility and direction for Consortium protocols, which includes, but is not limited to the following responsibilities: a) Identify novel, innovative and high impact endeavors or areas of research for the Consortium, b) Review all new study ideas and ancillary study ideas, c) Inform decisions around Study Chair selection, clinical center selection, vendor selection, d) Ensure the continuing scientific integrity and rigor of Consortium-conducted studies, e) Identify potential funding sources and help create and maintain relationships with existing funding sources, f) Review and approve manuscripts, presentations, press releases or other publicity, and g) Propose and review policy revisions to this governance document.

1.3.2.3 Appointment and Reappointment of Members

FFB will invite persons to participate in the Executive Committee based on recommendations from FFB Science, ROC members, SAB members and the Consortium Executive Committee. A request for application (RFA) process may be required to appoint new members, at the discretion of FFB. When mutually agreeable, Executive Committee members may be reappointed to serve an additional term. Reappointments will be based on re-evaluation of qualification and review of past activities and the special knowledge the member brings to the Executive Committee and the Foundation.

1.3.2.4 Term

The term for members of the Executive Committee will generally be 3-4 years to allow for rotation while ensuring institutional memory; the FFB liaison and Coordinating Center Director may change as needed. This is done so that there are less than 50% new members in any year.

1.3.2.5 Meetings

Meetings will be convened by teleconference, web, or face-to-face. Meetings during the initial year may be monthly as appropriate and no less than quarterly after that. Face-to-face meetings will be planned to coincide with other major events (e.g., AAO, ARVO or FFB-sponsored meeting) as much as feasible. Agenda items will be solicited in advance of the meeting and circulated to attendees.

For each decision or approval, at least 80% of voting members in attendance will constitute a quorum. For decisions or approval requested over email, at least 80% of responses are required before the decision is considered final. The Consortium chair's vote is required for decisions made in meetings or remotely. In the event of a tie, the Consortium chair(s) will cast the final vote. In the event of disagreement on a decision between two co-chairs, an FFB representative will cast the final vote. Executive Committee members are responsible to disclose any conflicts of interest related to decisions made that affect their other roles, such as for committee members who are also investigators. In these cases, members must recuse themselves and not participate in those specific discussions or attempt to influence the decision-making process. Any conflict of interest disclosure must occur at the beginning of an Executive Committee meeting.

Investigators from the Consortium, FFB Science, SAB members, and external advisors may be invited to Executive Committee meetings to discuss specific agenda items on an as-needed basis when the Committee desires additional scientific or other input.

Decisions and action items from Executive Committee meetings will be documented and archived by the Coordinating Center; members responsible for action items will be notified.

1.3.3 Operations Committee

1.3.3.1 Membership

The Operations Committee will be comprised of the FFB Liaison, Coordinating Center Director, and the Consortium Chair, and will be attended by additional support from the Coordinating Center as needed. Study Chairs and subject matter experts may also be invited to Operations Committee meetings.

1.3.3.2 Roles and Responsibilities

The Operations Committee will drive the execution of study protocols and be responsible for keeping the Executive Committee informed of any issues. Communications within the Operations Committee will consist of telephone calls, e-mails and in-person meetings. Operations Committee teleconferences may be weekly or bi-weekly.

1.3.4 Clinical Centers and Consortium Investigators

1.3.4.1 Membership

Clinical Centers and investigators will be invited to participate in the Consortium by FFB, in collaboration with the Executive Committee, based on the knowledge of inherited retinal diseases and ability to participate in and contribute to Consortium studies. Clinical Centers and investigators will be reviewed for Consortium requirements based on standard application forms to assess staffing, facilities, training, patient population, and experience. Additional Clinical Center certification and personnel certification requirements will need to be completed for participation in each study. All investigators must disclose any conflicts of interest that could present a bias in the design, conduct or reporting of a protocol.

1.3.4.2 Roles and Responsibilities

The Consortium investigators will be responsible for adhering to the process and policies in this governance document. Consortium investigators will provide ideas for studies, input to study protocols and analyses and be active contributors to support the Consortium mission. Investigators are encouraged to participate in Consortium-led studies; however, there may be instances that preclude their participation.

1.3.4.3 Minimum Clinical Center Activity

Clinical Centers are generally expected to enroll in studies to remain active in the Consortium. The Executive Committee will review activity levels periodically and may consider implementing minimum activity level goals to remain active in the Consortium.

In the unlikely event that the Executive Committee determines a Clinical Center is not making efforts to attempt minimum activity level goals, steps to deactivate the Center may be pursued. Any ongoing participation in studies would remain in place until complete, but interest in future studies would require reapplication to be in the Consortium.

1.3.5 Study Chairs

A Study Chair is often selected for a study because he or she initially submitted that protocol idea. Upon submission of a new protocol idea, the Executive Committee will review the submission and decide if it will move forward, prioritizing it among other current and upcoming studies. The Executive Committee will then decide who should be designated as Study Chair. While the submitter would be the likely candidate in most cases, occasionally the Executive Committee will nominate a different investigator because he or she has more experience with that particular study topic.

Instances may arise in which a new study idea is initiated from a source other than a single Consortium investigator; for instance, an industry partner or external advocacy group may propose the idea, or the idea may be generated from an interest poll sent to sites. In these instances, the Operations Committee will identify one or more Study Chair candidates. The identification of candidates may be informed by input from members of the Executive Committee, the FFB SAB, or the external research partner. The Operations Committee will nominate one or more candidates based primarily on subject matter expertise in the disease or genetic area relevant to the study idea. However, if there are no obvious candidates, the Operations Committee may solicit other interest from other internal or external groups. The potential Study Chair(s) will be proposed to the Executive Committee, which will make the final decision. Any investigator selected as Study Chair would be expected to join the Consortium (for the current study and potentially future studies). Study Chairs must disclose any conflicts of interest that could present a bias in the design, conduct or reporting of a protocol.

1.3.6 Coordinating Center

The Coordinating Center will coordinate activities (calls, meetings, communications) of all Consortium committees and members, coordinate development and maintain version control of all study documents, oversee conduct of all aspects of study protocols (including training, certification, IRB coverage, recruitment, retention, adverse event monitoring, closeout), develop and maintain a multi-functional study website and data management system for supporting Consortium activities (including online system for validated data entry/edit/signoff of data collection forms), develop and implement a quality assurance program that includes monitoring of protocol adherence as well as quality control of data at all stages of each study (both remote and on-site), and manage all aspect of Consortium publications and presentations (including overall production as well as statistical analyses, committee reviews, verifications, and submissions).

1.3.7 Reading Centers and Other Vendors

The FFB Liaison and the Coordinating Center Director will collaborate on selecting vendors to support the Consortium clinical studies. The activities of the reading centers and other vendors will be defined by study protocols and contracts/service agreements.

1.3.8 Data Safety Monitoring Committees

Each interventional clinical study will have a separate Data Safety Monitoring Committee (DSMC) that will be responsible for reviewing the ethical conduct of the study and monitoring the data for evidence of adverse or beneficial treatment effects. The DSMCs are advisory to the Executive Committee. The DSMCs will operate under a single written charter describing standard operating procedures for the Consortium, and details of study specific oversight or interim analyses will be described in each interventional study protocol and/or statistical analysis plan. The DSMCs will typically include an independent expert in each of the following areas: clinical trials, biostatistics, and the disease being studied. A minimum of three persons will be on the DSMC; these persons may not participate in the study in any other way.

Chapter 2: Adherence to Good Clinical Practices (GCP)

2.1 Good Clinical Practices (GCP)

All Consortium-led studies are to be conducted in accordance with applicable GCP regulations and guidelines per the International Committee on Harmonization (ICH) and US Code of Federal Regulations (CFR), including compliance with electronic records and electronic signatures (21 CFR, Part 11).

2.1.1 IRB/Ethics Committee Review and Approval

All protocols are to be conducted in accordance with IRB regulations (US 21 CFR Part 56.103) or applicable International Ethics Committee regulations. Investigators at each Clinical Center must obtain approval from a properly constituted/accredited IRB/EC prior to initiating the study and a re-approval on at least an annual basis.

2.1.1.1 Central IRB is Required for US Clinical Centers

Consortium studies starting after January 1, 2020, will require US Clinical Centers to use a central IRB for the review and approval for each study to ensure oversight across all Clinical Centers. For multi-center studies with a coordinating center at the JCHR, JCHR's Institutional Review Board (IRB) is able to enter into an IRB Reliance Agreement to serve as the IRB of record for institutions participating as Clinical Centers.

2.1.2 Informed Consent

Written informed consent/assent is to be obtained from each patient prior to any study-related activities or procedures in a study, and/or from the patient's legally authorized representative as per US 21CFR Part 50 and relevant country regulations.

2.1.3 Adverse Events

Adverse events will be assessed, documented, and recorded in the appropriate case report form throughout each study. Specific reporting and monitoring requirements and procedures for each study will be documented in the study protocol and procedures. Intervention studies will have adverse events monitored by a Medical Monitor, either internal or external to JCHR; this will be defined for each protocol.

2.1.4 Documentation and Record Retention

Source documents may include a patient's medical records, hospital charts, clinic charts, the investigator's patient study files, as well as the results of diagnostic tests such as ERGs, optical imaging, and laboratory tests. The investigator's access to the electronic CRFs on the study website serves as part of the investigator's record of a patient's study-related data.

For each study, the following information should be entered into the patient's medical record: patient's name and contact information; date the patient entered the study; study protocol title or number; dates of all visits; occurrence and status of any adverse events; vital signs; laboratory findings; visual acuity worksheets; results of any abnormal findings from any examination; printouts of any digital imaging/testing (e.g., FAF, OCT, fundus photos, etc.) and back-up copies of electronic records; date the patient exited the study, and if early discontinuation, the reason for early exit.

All study related correspondence, patient records, consent forms, patient privacy documentation, records of the distribution and use of all investigational products, and all CRFs (electronically on the website) should be maintained on file and at the Clinical Center.

Each center will archive all relevant study data records and keep them on file for a period of time that covers all minimums specified by each governing office/agency for that center and the given study as a whole, whichever is the greatest. Record retention will be defined for each study in adherence to the Coordinating Center's SOPs. This will include a requirement for clinical centers to contact the Coordinating Center prior to planned document destruction.

2.1.5 Policy for Email and Website Use

All investigators and coordinators must have a unique email address that they check regularly. All study personnel must log onto the study website only using their individually created password and must not share their password with others. An electronic signature on an electronic case report form indicates that the data have been reviewed and accepted by the signatory. Electronic signatures will consist of the combination of the individual's study website user identification number and password individually assigned by JCHR. It is unlawful to forge an electronic signature.

2.1.6 Adherence to Protocol and Study Procedures

All study investigators and their staff must adhere to protocols and study procedures to the best of their ability. The investigator must not implement any deviation from or changes to a protocol without approval by the Coordinating Center and prior review and documented approval/favorable opinion from the IRB/EC of a protocol amendment, except where necessary to eliminate immediate hazards to study participants, or when the changes involve only logistical or administrative aspects of the study (e.g., change in monitors, change of telephone numbers; in these cases, Coordinating Center must still be informed of the change).

Investigators will recruit participants in Consortium-led studies meeting the protocol-specified criteria and without prejudice of gender and ethnicity.

2.1.7 Protection of Patient Privacy and Confidentiality

The Clinical Centers and investigators will protect patient privacy and take appropriate precautions to maintain confidentiality of medical records and confidential information. However, as part of the quality assurance and legal responsibilities of an investigator, Clinical Centers must permit representatives of the Coordinating Center, authorized representatives, and/or the FDA or other appropriate governmental or regulatory authorities to examine at any reasonable time during normal business hours (a) the facilities where the Study is being conducted; (b) raw Study data including original subject records; (c) medical records in paper and electronic format supporting eligibility criteria and/or safety assessments; and (c) any other relevant information (and to make copies) necessary for the Coordinating Center to confirm that the Study is being conducted in conformance with the protocol and in compliance with applicable FDA or any national or governmental laws and regulations and the ICH guidelines as adopted by the FDA (where relevant). The Clinical Center and investigator must agree to take reasonable actions requested by the Coordinating Center to cure deficiencies noted during an audit or inspection. In addition, the Coordinating Center has the right to review and comment on any correspondence to a governmental authority generated as a result of an inspection or audit relating directly to the Study prior to submission by Institution or Principal Investigator, so long as such review does not unduly delay such response. During an on-site audit or inspection, the Coordinating Center may check to ensure that the informed consent was properly completed, including printed names, dates, and signatures, and therefore would be able to read the participant name. However, identifying information would be redacted prior to transmitting to the Coordinating Center for remote documentation or inspection. Study data are considered confidential until presented at a national meeting or published as an abstract or manuscript.

Written authorization and other documentation in accordance with the relevant country and local privacy requirements (where applicable) is to be obtained from each patient prior to enrollment into the study, and/or from the patient's legally authorized representative in accordance with the applicable privacy requirements (e.g., the Health Insurance Portability and Accountability Act Standards for Privacy of Individually Identifiable Health Information ("HIPAA")). For Clinical Centers in the European Economic Area (EEA), personal data of EEA citizens will be handled pursuant to the General Data Protection Regulation ("GDPR"). The Coordinating Center will honor any reasonable request by a study subject, pursuant to the GDPR, for access to or erasure, transfer, rectification, or accounting of personal data gathered as a part of any FFB Consortium protocol, or for withdrawal of consent to personal data processing. As applicable, the Coordinating Center will undertake all reasonable efforts to procure study participants' explicit, opt-in consent for data processing pursuant to Article 9 of the GDPR.

Only de-identified, pseudonymized patient data will be shared or appear in any publication.

The investigators will maintain the highest degree of confidentiality permitted for the clinical and research information obtained from participants in Consortium-led studies. Medical and research records will be maintained in the strictest confidence.

2.1.8 Data Quality Assurance and Monitoring

2.1.8.1 Clinical Center Staff Training

Clinical Centers and investigators are expected to maintain training records for staff participating in studies. This includes certification of visual acuity technicians, ocular imaging technicians, coordinators, perimetrists, genetic counselors, and others as specified in study protocols.

Good Clinical Practices (GCP) training is required every three years by investigators and coordinators. In addition, for each protocol, investigators and study staff will be required to be trained in study specific procedures prior to initiating the study at their Clinical Center. Requirements will be defined for each protocol.

2.1.8.2 Remote Monitoring and Audits of Clinical Centers

Clinical Centers are expected to have their own system to ensure quality of data entered into the eCRFs. The Coordinating Center will use remote data monitoring on a routine basis to identify potential inconsistencies in data as well as on-site data monitoring for assessment of potential issues.

Clinical Centers are to notify the Coordinating Center if they have been selected by the FDA or other government inspection agency that they are to be audited for an FFB Consortium-sponsored study.

Chapter 3: Conflicts of Interest and Investigator Conduct

3.1 Financial Disclosure and Conflict of Interest

All Consortium investigators, coordinators, committee members, and other key personnel will be required to disclose all financial interests and working relationships with any entity whose financial interests potentially could be affected by the conduct or outcome of Consortium-led research. This disclosure will be required separately for each protocol and will require an update according to criteria set for the given protocol. Financial disclosures must be updated within 30 days when there is a new financial disclosure due to a change in a Consortium protocol, or a change in the Consortium investigator or staff's finances.

Any person serving as a member of the Executive Committee (or other committees as applicable) who has financial disclosures relevant to a company involved in discussions to collaborate with the Consortium will forego discussion and voting privileges regarding decisions on the collaboration. This policy will prevent putting any Consortium investigator in an inappropriate position and will ensure that financial biases are eliminated when voting takes place. FFB is responsible to manage conflicts for Executive Committee members, while conflicts for other consultants and independent contractors (under contract with the Coordinating Center) will be managed by the Coordinating Center.

3.2 Potential Investigator Misconduct and Issue Escalation

3.2.1 Serious Breach of GCP and Protocol Adherence

Major protocol deviations (e.g., related to eligibility, informed consent, recording of adverse events, or study treatments) may jeopardize patient privacy, safety and integrity of a study and are not acceptable at any Consortium Clinical Center. This is monitored by the Coordinating Center and becomes a concern when a clinic is making more mistakes than expected, particularly major ones (e.g., entering ineligible participants).

3.2.2 Assessment and Reporting

Assessment of any potential investigator or staff serious misconduct will be done via an on-site monitoring visit. Potential issues will be discussed by the Operations Committee first and then escalated to the Executive Committee if there is evidence of serious misconduct. If GCP violations are serious, they will be reported to the governing IRB/EC and may also be reported to the FDA or other regulatory agency. The Executive Committee, and potentially the DSMC will make a decision regarding suspension or halting of study activity at that Clinical Center.

3.2.3 Corrective and Preventative Actions

A written corrective action and preventative action plan for any case of serious misconduct will be put into place by the Coordinating Center in collaboration with the Operations Committee.

3.2.4 Issue Escalation

Each FFB Consortium protocol will have an Escalation Plan in place to address potential problems as they arise. Escalation Plans are intended to specify the measures implemented (for instance, the levels of Operations Committee or Executive Committee involvement) when there are problems to address with a Clinical Center that may negatively impact the study but are not serious enough to be considered breaches of GCP.

Chapter 4: Editorial Policy

4.1 Editorial Policy

The following policies relate to publications and publicity produced by the Consortium. These activities will be managed by the Coordinating Center and overseen by the Executive Committee. Investigators wishing to publish or present Consortium data without scientific collaboration with the Consortium should follow the policies outlined in section 5.1.3.

4.1.1 Manuscripts and Presentations

All manuscript and presentation ideas related to any aspect of a Consortium-led study, including but not limited to the study protocol, study results, and study conduct that is not already information in the public domain, must receive the approval of the Executive Committee. The topic for a manuscript or presentation may be initiated by the Executive Committee, or by any investigator, who may submit a manuscript idea to the Coordinating Center for Executive Committee consideration.

Typically, the “primary” manuscript for a study will refer to the manuscript that contains the analysis of the primary outcome of the study, and all other manuscripts will be considered “secondary” manuscripts. There may be studies with multiple objectives that will result in multiple publications to address them, in which case there might be more than one primary manuscript (or no primary versus secondary designations). The Executive Committee will make the determination of whether a manuscript is primary or secondary.

The Executive Committee will approve all manuscripts about the study or any ancillary study in a timely fashion (e.g., 1-2 weeks) prior to submission for publication. The manuscripts will also be submitted to FFB for comment prior to submission. Primary manuscripts must also be approved by the DSMC (if there is a DSMC). The DSMC will be sent secondary manuscripts for comment, but approval will not be required.

All investigators at Clinical Centers participating in the relevant study will receive a draft of the manuscript for review. Prior to submission, each PI will also have an opportunity to approve the final version of the manuscript.

4.1.2 Authorship

Since every investigator cannot have an active role in writing a paper, the Operations Committee will establish a Writing Committee for each paper with the advice of the Executive Committee. Investigators may volunteer for these writing assignments. Writing Committees may also include representatives from Reading Centers, consultants who were involved in the implementation or monitoring of the protocol, or vendors with ownership or intellectual property related to the procedures performed. The Operations Committee will also determine the first author for each paper; typically, this will be the Study Chair for primary manuscripts.

For all manuscripts and presentations, the writing committee members will be listed by name followed by “for the FFB Consortium Investigator Group.” For all abstracts, the presenter will be the only author listed followed by “for the FFB Consortium Investigator Group.” Each Clinical Center with an investigator who enrolled at least one patient along with the study personnel at that site will be listed in at least one manuscript for each study (it may be referenced in other manuscripts for the same study) in descending order of recruitment, if this meets with journal approval. Each PI will be given the opportunity to review and sign off on the site listing as it will appear in the appendix, where applicable. Sources of support for the study will be listed. Members of the Writing Committee, Executive Committee, DSMC, reading centers, relevant independent consultants/experts and Clinical Centers will be listed.

To qualify for authorship, each author must meet at least one criterion in each of the three categories. Each author must also provide approval of the final version of the manuscript.

Category 1

- Conception and design
- Acquisition of data
- Analysis and interpretation of data

Category 2

- Drafting of the manuscript
- Critical revision of the manuscript for important intellectual content (this does not include reviewing the manuscript for journal submission approval)

Category 3

- Statistical analysis
- Obtaining funding
- Administrative, technical, or material support
- Supervision
- Other (specify)

4.1.3 Publicity

The Executive Committee and FFB must give approval prior to any press release or other publicity about the study using information not already in the public domain.

Chapter 5: Collaboration and Transparency

5.1 Collaboration and Transparency

5.1.1 Multi-centered studies

The Consortium-led studies will be conducted as multi-centered studies to increase the robustness of study results and enable individuals from different regions to participate.

5.1.2 Availability of Study Protocols and Procedures

To further the mission of the Consortium, sharing of study protocols and procedures will be allowed; requests will go through the Executive Committee.

5.1.3 Data Sharing Policy

Sharing study data is an integral component of the Consortium's mission. Unless otherwise approved by the Executive Committee, the policies below will be relevant for all data sharing circumstances. These policies address the processes by which valid and accurate study-specific data and general information can be accessed in a timely manner. Statements regarding the data sharing plans for each study will be posted on ClinicalTrials.gov during study registration and will be included with relevant manuscript submissions in accordance with journal standards.

5.1.3.1 Public Datasets

Individual, de-identified, study participant data will be made available as a "public dataset" after the study is completed and all manuscripts addressing the protocol-defined objectives have been published. These two activities will typically occur within one year of the last study participant's last visit. A study will be considered "completed" when all the following activities (as applicable) have been completed:

- Scheduled study visits;
- Exams and assessments;
- Image grading and interpretation;
- Genetic testing interpretation and adjudication;
- Quality assurance reviews;
- Data reconciliation;
- Medical coding;
- Documentation of known data anomalies and data handling rules; and
- Database lock.

5.1.3.2 Requests to Use Study Data from a Public Dataset

The following policy applies to situations when a study dataset has been made publicly available.

Persons wishing to use a public dataset must submit a request form to the Coordinating Center (ffb@jaeb.org).

Data sharing will be contingent upon executed confidentiality, data transfer and processing agreements, GDPR compliance, and compliance with the Coordinating Center's Data Transfer Agreements with Clinical Centers outside the United States. The content of these agreements may add more details and requirements than are included in this policy document.

Additionally, the author should be explicit when presenting their analyses in any forum that they do not speak for, nor represent, the opinions of the Consortium. Use of these Consortium data requires that the following disclaimer be added to any paper, review, presentation or other distribution of the data exactly as follows:

"The source of the data is the Foundation Fighting Blindness Consortium, but the analyses, content and conclusions presented herein are solely the responsibility of the authors and may not reflect the views of the Foundation Fighting Blindness."

5.1.3.3 Requests to Use or Access Study Data Before it is Publicly Available

The following policies apply to situations when a study dataset has not yet been made publicly available.

5.1.3.3.1 Academic Researchers Seeking Data Access (Aggregate or Individual Observations) – with Scientific Collaboration

Academic researchers wishing to scientifically collaborate with the Consortium on an idea using Consortium study data (either aggregate or individual data) not yet released must submit the idea to the Coordinating Center for Executive Committee consideration according to the Consortium editorial policy (Section 4.1).

5.1.3.3.2 Requests for Aggregate Data – without Scientific Collaboration

Persons requesting tabulated or summary data without scientific collaboration with the Consortium on an idea using Consortium study data not yet released (via public dataset) or already published must submit the request to the Coordinating Center for Executive Committee approval. The Executive Committee will determine whether analysis and presentation or publication of the data would negatively impact the Consortium study objectives or any planned or pending reporting on the study dataset.

Note: If the origin of the request is a company, the request will also be routed to FFB leadership to determine if it may be related to existing or possible future industry collaboration.

If approved, the following stipulations will apply:

- Use of Consortium data or images requires that the following disclaimer be added to any paper, review, presentation or other distribution of the data exactly as follows: "The source of the data is the Foundation Fighting Blindness Consortium, but the analyses, content and conclusions presented herein are solely the responsibility of the authors and may not reflect the views of the Foundation Fighting Blindness."
- If Executive Committee approval depends on any specific conditions, this will be communicated and will be required to be followed.

- If the aggregate data are to be disseminated in a publication or presentation, the draft manuscript, abstract, poster, or presentation must be submitted for Coordinating Center review for adherence to stipulations, with at least two weeks' time allotted for response.
- The final version of any manuscript, abstract, poster, or presentation must also be provided to the Coordinating Center.

5.1.3.3.3 Academic Researchers Seeking to Use Their Own Study Data– without Scientific Collaboration

Academic researchers wishing to pursue publication or presentation of Consortium data to which they already have access but is not yet publicly available (e.g., [1] Consortium data obtained from an investigator's own patients or [2] a reading center's graded data), without scientific collaboration with the Consortium must submit a data use request form to the Coordinating Center for Executive Committee consideration. The Executive Committee will determine whether analysis and presentation or publication of the data would negatively impact the Consortium study objectives or any planned or pending reporting on the study dataset.

Note: The only exception to this is the unlikely scenario that study data are not made public (via a public dataset) within 12 months following formal closeout of the study. In this case, the investigator would have the right to report or present Consortium data obtained from his or her own patients without prior Executive Committee approval.

If approved, the following stipulations will apply:

- Use of Consortium data or images requires that the following disclaimer be added to any paper, review, presentation or other distribution of the data exactly as follows: "The source of the data is the Foundation Fighting Blindness Consortium, but the analyses, content and conclusions presented herein are solely the responsibility of the authors and may not reflect the views of the Foundation Fighting Blindness."
- If Executive Committee approval depends on any specific conditions, this will be communicated and will be required to be followed.
- The draft manuscript, abstract, poster, or presentation must be submitted for Coordinating Center review for adherence to stipulations, with at least two weeks' time allotted for response.
- The final version of the manuscript, abstract, poster, or presentation must also be provided to the Coordinating Center.

5.1.3.3.4 Any Request for Data Access to Individual Observations – without Scientific Collaboration

The Consortium may consider providing academic researchers or companies early access to de-identified, pseudonymized participant-level study data in accordance with the following policies.

1. The request must be made in writing (to ffb@jaeb.org) and must specify the planned use of the data and specific datapoints requested
2. The Executive Committee must approve the written requests

3. The intended use must be consistent with the mission of the Consortium, as determined by the Executive Committee
4. The Executive Committee will determine whether any plans for presentation or publication of the requested data would negatively impact the Consortium study objectives or any planned or pending reporting on the study dataset
5. Any approved data sharing will be contingent upon executed confidentiality, data transfer and processing agreements, GDPR compliance, and compliance with the Coordinating Center's Data Transfer Agreements with Clinical Centers outside the United States. The content of these agreements may add more details and requirements than are included in this policy document.
6. Executive Committee approval may be conditional on additional stipulations beyond what is in this policy document
7. If approved by the Executive Committee, plans to publicly present or publish data **for the purpose of regulatory submissions and potential associated investor/public relations** must adhere to the following, as bound in the contract, which requires the researcher certify that:
    (a) No conclusion of the Data shall be used, shared, publicly presented or published that conflicts with the conclusions drawn by the FFB Consortium.
    (b) Any conclusions of the Data must include the following disclaimer: "The source of the data is the Foundation Fighting Blindness Consortium, but the analyses, content and conclusions presented herein are solely the responsibility of the authors and may not reflect the views of the Foundation Fighting Blindness."
    (c) Researcher will only submit conclusions that have been drawn in good faith.
    (d) High level conclusions should be provided to the Coordinating Center for review to support alignment of such conclusions with enough time to provide a review and feedback for consideration.
8. If approved by the Executive Committee, plans to publicly present or publish data **in a scientific journal or conference** must adhere to the following:
    (a) Any draft manuscript, abstract, poster, or presentation must be provided to the Coordinating Center in writing at least thirty (30) calendar days prior to submission for publication.
    (b) The final version of any manuscript, abstract, poster, or presentation must also be provided to the Coordinating Center.
    (c) All conclusions of the data must include the following disclaimer: "The source of the data is the Foundation Fighting Blindness Consortium, but the analyses, content and conclusions presented herein are solely the responsibility of the authors and may not reflect the views of the Foundation Fighting Blindness."

5.1.3.4 Requests to Use Information that does not Require Study Data

5.1.3.4.1 General Consortium Information

Persons wishing to publish or present general information about the Consortium with no study data included may do so without formal approval. Examples of general information include the number and identity of participating centers, information (e.g., study design and milestones) about planned and current studies, and summaries of publications and presentations. Since information about the Consortium changes frequently, presenters are encouraged to use frequently-updated slides from the Coordinating Center and send a courtesy notification to the Coordinating Center (ffb@jaeb.org) about the intended publication or presentation.

5.1.3.4.2 Independent Ancillary Study Data

Persons wishing to publish or present data from Consortium participants who are in an independent (not coordinated by the Consortium) ancillary study, where no study data will be used, may do so without formal approval. Since information about the Consortium changes frequently, a courtesy notification to the Coordinating Center (ffb@jaeb.org) about the intended publication or presentation is requested. The following disclaimer must be included.

“These data were collected as an independent ancillary study to a Foundation Fighting Blindness Consortium protocol. Data collection, analyses, content and conclusions presented herein are solely the responsibility of the authors and may not reflect the view of Foundation Fighting Blindness.”

Chapter 6: New and Competing Studies

6.1 New Studies

6.1.1 New Protocols

Protocol ideas may be submitted by individuals inside or outside the Consortium. A Consortium Protocol Idea Form can be used to propose a new study idea. Ideas will be first reviewed with the Executive Committee for merit, feasibility, and prioritization. All protocol ideas that are favorably reviewed by the Executive Committee will also be reviewed by Consortium Members for additional input and interest, and by the FFB's Clinical Subcommittee to the Research Oversight Committee for ultimate approval to proceed to full protocol development process.

6.1.2 Ancillary Studies

An ancillary study is one in which research procedures not part of the primary protocol is performed on a subject participating in a current Consortium protocol.

There are two main types of ancillary studies, Consortium ancillary studies and independent ancillary studies.

6.1.3 Consortium Ancillary Studies

A Consortium ancillary study is one that is coordinated by the Coordinating Center with oversight by the Executive Committee. This type of ancillary study would follow all of the same governance policies and oversight as a Consortium protocol, including the following:

1. The ancillary study idea must be submitted for review by the Executive Committee according to the same review process as described above for new protocols, section 6.1.1. An Ancillary Study Idea Form should be submitted for this review.
2. Use of Consortium ancillary study data would follow the data use policy noted in section 5.1.3, Data Sharing Policy.
3. The editorial policy for a Consortium ancillary study is the same as for any other Consortium manuscript as noted in section 4.1, Editorial Policy.

6.1.4 Independent Ancillary Studies

An independent ancillary study is one in which study resources and the Coordinating Center are not involved. The operations and funding would be the responsibility of the investigator(s). Although the independent ancillary study would not be coordinated or overseen by the Consortium, it must adhere to the following requirements:

1. The independent ancillary study idea must be reviewed and approved by the Executive Committee. The primary purpose of this review would be to determine that the ancillary study objectives do not interfere with the objectives of the primary protocol. The Coordinating Center should be contacted to propose an independent ancillary study idea.
2. Use of the independent ancillary study data that is not collected as part of any Consortium protocol can be used/published according to the policy in section 5.1.3.

3. Use of any Consortium study data that was collected in conjunction with the ancillary study data (i.e., even just for an investigator's own patients) would follow the data sharing policy noted in section 5.1.3.

6.2 Competing Studies

A 'competing' study is defined as one in which subject eligibility criteria overlap with that of a Consortium study. Clinical Centers are required to inform the Coordinating Center of studies in which they are participating that have eligibility criteria that overlap with a Consortium protocol in which they are concurrently participating. Clinical Centers should determine a management plan for competing studies internally. Assistance from the Operations Committee will be available for Clinical Centers that would like advice on how to manage their competing studies. Clinical Centers should ensure that any funding received, such as travel reimbursement for study visits, is managed and monitored appropriately in cases where participants are enrolled in more than one concurrent study.

Chapter 7: Funding

7.1 Funding of Consortium Studies and Clinical Centers

7.1.1 Funded through Private Donations

The Consortium is funded through private donations made to the Foundation Fighting Blindness for the purpose of finding treatments for inherited retinal diseases. Care must be taken to conserve resources to ensure highly efficient usage of the funding.

7.1.2 Contracts

Each Consortium-led study will have its own budget and contract between FFB and the Coordinating Center and between the Coordinating Center and Clinical Centers and vendors. The Coordinating Center will enter into a Master Agreement with each Clinical Center for their participation in the Consortium; each protocol will have an individual numbered Addendum. Additional funding to cover institutional indirect cost rates or overhead fees will not be available.

Funding of the Consortium is expected to produce data leading to development of treatments for IRDs. Contracts with the Clinical Centers will be based on a fee-for-service based on the number of participants enrolled into the study and the number of examinations completed. Clinical Centers will also receive funding not tied to specific study visits, intended to offset the certification and administrative tasks associated with each protocol; these payments will be distributed to Clinical Centers once certification requirements are completed.

Depending on the study, all study visits, including but not limited to screening, baseline and follow-up, and any standard of care appointments, may be charged to the study participant or their insurance carrier or health care system as permitted according to each country's laws and regulations. Depending on the study, the study participant may also be responsible for any deductible or co-payments as defined by their particular insurance carrier. Consortium rates for each procedure and visit are developed based on the "research rate" and are intended to cover the full cost without requiring any reimbursement from the patient or his/her insurance. Certain study procedures, including obtaining informed consent and non-standard examination, will not be incurred by the study participant and will be covered by the study. Participation of the study coordinator will be paid on a by-patient/by-visit basis, as will the investigator to ensure adequate compensation for completed work.

Traveling to Clinical Centers can be challenging for patients with IRDs; to assist with transportation, study participants will be offered a stipend on a by-visit basis for transportation and their participation. The amount and the mechanism for payment will be described in the informed consent form.

7.2 My Retina Tracker

My Retina Tracker Registry (MRTR) is a patient-driven registry for patients with IRDs sponsored by FFB. Consortium Clinical Members are expected to actively encourage their clinic patients to register and participate in MRTR and inform patients that they can request their physician/genetic counselor to put data into MRTR on the patient's behalf.

Summary of Changes

Version	Author(s)	Approver	Effective Date	Revision Description
1.0	J. Cheetham, A. Ayala	P. Zilliox	May 13, 2016	First Version of Document
2.0	J. Cheetham, A. Ayala	P. Zilliox	March 30, 2017	• Clarification: Financial disclosure requirements tied to each protocol • Clarification: FFB CRI Consortium will not pay indirect fees • Clarification: billing to insurance “may” be required instead of “will” be required for SOC tests, depending on the study • New policy: new protocol ideas and ancillary studies
3.0	A. Ayala, J. Cheetham	S. Rose	November 26, 2018	• Modified data sharing policy for use of Consortium data to the public to require a disclaimer • Modified ancillary studies policy to define Consortium sponsored ancillary vs independent ancillary study • Removed CRI references • Added section on GDPR • Updated site/staff training requirements
4.0	A. Ayala, R. Sitten	T. Durham	July 8, 2019	• Updated the figure in the Organizational Structure section • Added a subsection for Executive Committee reappointments • Expanded Study Chair selection policy to included instances where a new protocol idea is submitted by someone who is not an investigator in the Consortium • Added more explicit language with regards to access to records at site visits • Added collaborators to list of possible Writing Committee members

5.0	A. Ayala, R. Parsons	T. Durham	January 24, 2020	• Revised the data release and data use sections • General updates and minor corrections throughout • Added Central IRB as a requirement • Added section about Escalation Plans
6.0	A. Ayala, R. Parsons	Executive Committee	January 15, 2021	• Added a new section on Consortium Chair responsibilities and selection process • EC Membership- Added a new role for representative(s) of the FFB SAB or ROC • Added the percent of votes required for EC decision-making • Added additional detail throughout the Committee descriptions
7.0	A. Ayala, J. Shah	Executive Committee	April 11, 2022	• Added confirmation step in Chair selection process • Clarified abstract authorship • Added Minimum Site Activity