CRC-SCA Natural History Study Publication and Biospecimen Policy

Introduction

High quality research and productive publication and biospecimen collection of the CRC-SCA natural history dataset are the major goals of the CRC-SCA Publication Subcommittee and Biospecimen Subcommittee, and the study sponsors, National Institutes of Health (NIH) and National Ataxia Foundation. This document outlines CRC-SCA's policies for biospecimen, publication, and publication credits for all manuscripts, abstracts, and posters utilizing CRC-SCA natural history data and the dataset for U01 Clinical Trial Readiness for SCA1 and SCA3.

This policy is designed to maximize the number and quality of publications and presentations; facilitate and streamline collaborative development of manuscripts; allow tracking and coordination of projects using shared dataset and biospecimens; facilitate communication; allocate publication credit; and avoid potential conflicts.

Project Proposal Submission

1. Project Proposals are required for all manuscripts, research projects, data analysis, and biospecimen requests. This includes analysis of CRC-SCA data from participants evaluated at other sites. Note that internal investigators do not need to contact each site individually to obtain approval for analysis of CRC-SCA data from participants evaluated at other sites- only Project Proposal submission is required.
2. Project Proposals may be submitted by email to: Sheng-Han Kuo, sk3295@columbia.edu; Tetsuo Ashizawa, tashizawa@houstonmethodist.org. If biospecimen is requested, please also send an email to Puneet Opal: p-opal@northwestern.edu, Stefan Pulst: stefan.pulst@hsc.utah.edu, and Karla P. Figueroa: karlaf@genetics.utah.edu.
3. Project Proposals will be reviewed and approved by the CRC-SCA Publication Subcommittee before data are released. Publication Subcommittee Review and/or Biospecimen Subcommittee will be completed within 15 working days of submission. For the data of U01, the U01 Steering Committee will make the final approval of the dataset release, which will take additional 2 weeks.
4. The investigator submitting to the Publication Subcommittee has sole possession of the idea. Other groups can contribute but the original submitter of the sheet will take the lead. If more than one person submits the same or similar topic, the Publication Subcommittee helps decide who will assume the project lead, allocation of publication credit, and order of authorship.
5. In addition to submitting a Project Proposal prior to embarking on a project, investigators must submit the manuscript of any work arising from the approved project to the CRC-SCA Publication Subcommittee for approval *prior to* submission to a journal or conferences (see *Guidelines for Manuscripts* below).

CRC-SCA members

The original NIH-funded CRC-SCA natural history study from 2009-2012 constitutes of 14 principal investigators (alphabetical order according to investigator's last name)

Old list 2010-2012

1. Tetsuo Ashizawa, Houston Methodist Research Institute
2. Khalaf Bushara, University of Minnesota
3. Michael D Geschwind, University of California, San Francisco
4. Christopher M. Gomez, University of Chicago
5. Sheng-Han Kuo, Columbia University
6. Henry Paulson and Vikram Shakkottai, University of Michigan
7. Susan Perlman, University of California, Los Angeles
8. Stefan M. Pulst and Karla P Figueroa, University of Utah
9. Jeremy Schmahmann, Harvard University
10. S.H. Subramony, University of Florida, Gainesville
11. George Wilmot, Emory University
12. Sarah H Ying, Johns Hopkins University
13. Theresa Zesiewicz, University of South Florida

After 2012, the natural history enrollment continues with the support from the National Ataxia Foundation, with the original CRC-SCA members except for Liana Rosenthal and Chiadi Onyike replacing Sarah Ying as the principal investigators at Johns Hopkins University, and Puneet Opal at Northwestern University joining the natural history study team. Please note that not all of the sites below are funded by the National Ataxia Foundation for the natural history study but the funding is expected in the near future for those sites that are not currently funded.

New list 2012-

1. Tetsuo Ashizawa, Houston Methodist Research Institute
2. Khalaf Bushara, University of Minnesota
3. Michael D Geschwind, University of California, San Francisco
4. Christopher M. Gomez, University of Chicago
5. Sheng-Han Kuo, Columbia University
6. Puneet Opal, Northwestern University
7. Henry Paulson and Vikram Shakkottai, University of Michigan
8. Susan Perlman, University of California, Los Angeles
9. Stefan M. Pulst and Karla P Figueroa, University of Utah
10. Liana Rosenthal and Chiadi Onyike, Johns Hopkins University
11. Jeremy Schmahmann, Harvard University
12. Lauren Seeberger, University of Colorado
13. S.H. Subramony, University of Florida, Gainesville
14. George Wilmot, Emory University
15. Theresa Zesiewicz, University of South Florida

U01 Study 2018-

1. Tetsuo Ashizawa and Leif Petersen, Houston Methodist Research Institute
2. Erika Augustine, University of Rochester
3. Khalaf Bushara, University of Minnesota
4. Michael D Geschwind, University of California, San Francisco
5. Christopher M. Gomez, University of Chicago
6. Sheng-Han Kuo, Columbia University
7. Puneet Opal, Northwestern University
8. Henry Paulson and Vikram Shakkottai, University of Michigan
9. Susan Perlman, Jeanette Papp and Brent Fogel, University of California Los Angeles
10. Stefan M. Pulst and Karla P Figueroa, University of Utah
11. Liana Rosenthal and Chiadi Onyike, Johns Hopkins University
12. Jeremy Schmahmann, Harvard University
13. Lauren Seeberger, University of Colorado
14. Sharon Sha, Stanford University
15. S.H. Subramony, University of Florida, Gainesville
16. George Wilmot, Emory University
17. Talene Yacoubian, University of Alabama Birmingham
18. Theresa Zesiewicz, University of South Florida
19. Thomas Klockgether, University of Bonn (ASG/DZNE)
20. Alexandra Durr and Sophie Tezenas du Montcel, ICM (SPATAX)

If MR data are included

21. Thomas Mareci, University of Florida
22. Gulin Oz, University of Minnesota
23. Eva-Maria Ratal, Harvard Medical School
24. Harris Sair, Johns Hopkins University

Guidelines for Manuscripts

1. Investigators must submit a Project Proposal Form (Appendix 1) to the CRC-SCA Publication Subcommittee prior to embarking on a project.
2. Approval of a research project by the Publication Subcommittee does not guarantee unrestricted approval of resulting manuscripts.
3. On completion of the research project, investigators are required to submit the manuscript of any work arising from the approved project to the CRC-SCA Publication Subcommittee for approval prior to the submission to a journal or conference. Review of submitted manuscripts and abstracts is guaranteed within 15 working days of submission.
4. The submission of a manuscript to the CRC-SCA Publication Subcommittee does not guarantee the approval of the submission. After review, significant modification

of the manuscript or abstract may be required. Therefore, it is the responsibility of the investigator make their submission with ample time before the journal or conference deadline.

5. All publications using CRC-SCA natural history data (old: 2009-2012; or new: 2012-) or the U01 dataset may be required to list CRC-SCA members as co-authors, subject to the decision of the Publication Subcommittee. If not listed as co-authors, CRC-SCA members may be listed as collaborators with “CRC-SCA” listed as co-authors, and detailed collaborator information below the author list in the manuscript.
6. Upon approval by the Publication Subcommittee, the manuscript must be sent to all CRC-SCA members listed as coauthors, for comments prior to submission. The comments must be returned within two weeks. A coauthor may request to be removed from the publication, but may not delay the publication of a manuscript approved by the Publication Subcommittee. If the primary author feels that a coauthor is not responding in a timely manner they may appeal to the Publication Subcommittee for the removal of that coauthor, and the lead investigator of the manuscript or abstract may submit the manuscript.
7. If a CRC-SCA member does not wish to be part of a manuscript or abstract, either as a co-author or a collaborator, s/he will notify the lead investigator and the Publication Subcommittee by email.
8. If authorship copyright forms or conflict of interest statements are required for each author on the manuscript, CRC-SCA member co-authors must fulfill the required form within two weeks of the notification. If this requirement is not met, the lead investigator may change credit from a co-author to a collaborator, who is not required to fill out these forms.
9. All publications using CRC-SCA natural history data should include the following acknowledgement: NIH funding the Rare Disease Clinical Research Network (RDCRN) (RC1NS068897), U01 NS104326 and/or the National Ataxia Foundation as appropriate.
10. A PubMed Central reference number (PMCID) must be obtained submitted to the Publication Subcommittee within 3 months for each publication using CRC-SCA natural history data.
11. The decision of the Publication Subcommittee on publication of a manuscript that contains any part of U01 study data must also be approved by the U01 Steering Committee.

Unusual situations

1. **Egregiously Poor Manuscripts.** If a manuscript is found to be poor in terms of scientific substance or style, the Publication Subcommittee may recommend to the authors that it not be submitted without significant revision. If the authors choose to submit the manuscript against the recommendation, the Publication Subcommittee may make any or all of the following responses: 1) request that the group authorship credit be withheld; 2) request that the authors publish a statement

to the effect that, while CRC-SCA data were used, the CRC-SCA consortium did not find this manuscript of sufficient merit to warrant submission for publication; 3) submit a disclaimer directly to the journal or conference to the effect that the publication was not approved by the CRC-SCA; 4) revoke privileges of the investigator to use CRC-SCA data in the future.

2. **Journal Requirements.** In the event that journal editors do not allow the group authorship format for CRC-SCA member coauthors who have gathered data but not participated in the analysis and writing of the paper, the primary author of the paper should inform the CRC-SCA Publication Subcommittee of the issue. The CRC-SCA Publication Subcommittee will work with the author to resolve this problem in a way that is acceptable to the CRC-SCA. This may involve requiring submission of the manuscript to a different journal.
3. **Fraudulent obtaining of Data or Specimens.** Should the Publication Subcommittee discover an attempt to publish using CRC-SCA data or samples obtained fraudulently or without approval, the user will be sanctioned through Publication Subcommittee by communication with them and their academic supervisors. In cases of egregious fraud, the violator's institution and federal agencies may be notified. Depending on the seriousness of the case, sanctions may include revocation of their access to CRC-SCA data, an lodging of formal complaints with their institution and relevant federal agencies.
4. The decision of the Publication Subcommittee regarding above situations involving any part of U01 study data must also be approved by the U01 Steering Committee.

Request for the Natural history dataset

1. Requests for the natural history dataset must be approved by the Publication Subcommittee. Investigators who wish to perform data mining on the natural history dataset must submit a Project Proposal according to the guidelines above, using the Project Proposal Form (Appendix 1).
2. For the investigators from academic centers, CRC-SCA Publication Subcommittee will make a decision within 15 working days. The decision of the Publication Subcommittee involving any part of U01 study data must also be approved by the U01 Steering Committee. Once approved, CRC-SCA Publication Subcommittee will send the investigators the requested dataset(s) within 2 weeks of the approval.
3. For the investigators from the pharmaceutical industry, CRC-SCA Publication Subcommittee will consult all the CRC-SCA and/or U01 study members and will make a decision within 3 weeks. The decision on a study that involves any part of U01 study data must also be approved by the U01 Steering Committee. Once approved, CRC-SCA Publication Subcommittee will send the investigators the requested dataset(s) within 2 weeks.
4. Investigators cannot distribute the CRC-SCA natural history dataset or the U01 dataset to other investigators or other institutions without the approval of the CRC-SCA Publication Subcommittee and/or the U01 Steering Committee. All the data distribution will be done by the CRC-SCA Publication Subcommittee and/or the

U01 Steering Committee. The dataset must be destroyed upon completion of the proposed study.

Requests for Biospecimen

1. The request for biospecimens from the CRC-SCA natural history study or U01 clinical trial readiness study will first be made by filling out the initial Project Proposal Form (Appendix 1) and submitting the form to the CRC-SCA Biospecimen Subcommittee, who will review and make a decision within 15 working business days. For requests regarding biospecimens from U01, the U01 Steering Subcommittee will make the final decision; therefore, 2 additional weeks are required. The scientific background/rationale and the technical detail of the assays will be necessary to be included in the initial Project Proposal request. In some cases, the CRC-SCA Biospecimen Subcommittee may request for additional information with extended review time.
2. Upon the receipt of the requested form, the CRC-SCA Biospecimen Subcommittee will hold a phone conference within the deadline to discuss the scientific merit of the proposal. The requesting investigators may be invited to attend by phone to explain the proposal.
3. The CRC-SCA Biospecimen Subcommittee will circulate the full proposal to all the CRC-SCA members who contributed to biospecimen collection for further advice. If CRC-SCA members who do not respond to the Email sent out by the CRC-SCA Biospecimen Subcommittee within 15 working days, it will be deemed that the CRC-SCA member will honor the decision of the CRC-SCA Biospecimen Subcommittee.
4. The CRC-SCA Biospecimen Subcommittee will make a final decision regarding the distribution of the biospecimen. However, if an individual CRC-SCA site/ member wishes not to be part of the study, he/she can opt out of the particular study and the biospecimen from the particular site will not be distributed to the requesting investigators. The CRC-SCA member who chooses not to participate still reserves the right be listed as a co-author based on the contribution to the data collection in the CRC-SCA natural history study.
5. Once approved, the biospecimen will be distributed within 2 months and the requesting investigators will cover the cost of labor, shipping, and handling of the biospecimen incurred at University of Utah.
6. The CRC-SCA Biospecimen Subcommittee can also decide to distribute part of the requested biospecimen for the testing of the assay. If the the preliminary testing shows the validation of the assay, then all requested samples will be sent to the requesting investigators.
7. No MTA is required for University of Utah to distribute the biospecimen. However, handling charges will apply.
    a. The 2017 rates are:
        i. $2.50/tube (consumables)
        ii. Labor $35.00/hour with one hour minimum (sample labeling and aliquots), labor is billed at real time.
        iii. Shipping container $30

iv. Dry Ice $7/lb.

DNA concentration is determined via Nanodrop ND-100. Qubit is available at an additional charge. The 2017 rate per the University of Utah Genomics core (http://cores.utah.edu/genomics/) is $2.00/tube and $50.00/hour for labor with 1 hour minimum. Requesting institution will pay for shipping costs.

8. The decision of the Biospecimen Subcommittee that contains any part of U01 study data must also be approved by the U01 Steering Committee.

CRC-SCA data and publication and biospecimen request document

CRC-SCA study requested document should include Project Proposal Form (Appendix 1) with project summary, which should contain the following sections: Background, Hypothesis, Outcome measures, Significance (impact), Analysis plans (including statistical analysis), Expected outcomes and Time line, which will be written in a non-tabular form not exceeding 3 pages (Arial 11pt font, single spaced, with 0.5 in margins all around).

Please send this form to Dr. Sheng-Han Kuo, sk3295@columbia.edu and Dr. Tetsuo Ashizawa, tashizawa@houstonmethodist.org. If biospecimen is also requested, please include Dr. Puneet Opal: p-opal@northwestern.edu and Stefan Pulst: stefan.pulst@hsc.utah.edu, and Karla P. Figueroa: karlaf@genetics.utah.edu.

APPENDIX 1. PROJECT PROPOSAL FORM

Type of submission:

- ❑ Data Analysis
- ❑ Manuscript
- ❑ Biospecimen Analysis

Project primary investigator contact information:

Name_____

Institution_____

Phone_____

Email_____

Project co-investigators:

Name_____ Institution_____

Name_____ Institution_____

Name_____ Institution_____

Name_____ Institution_____

CRC-SCA Natural History Study Affiliation. Please describe your connection with the CRC-SCA Natural History Study. Are you a current member? An external affiliate? What is your funding source?

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Project Title_____

Expected Completion Date_____

Project Summary. Please provide a summary abstract of your proposed project (three pages or less), which includes Background, Hypothesis, Outcome measures, Significance (impact), Analysis plans (including statistical analysis), Expected outcomes and Time line If biospecimen is requested, please also include the sample size, amount of samples, the required DNA concentration, and shipping details.