PCORI INTERIM PROGRESS REPORT

Use continuation pages as needed. Limit 20 pages, not including the Certification page.

Updated on Tuesday, July 7, 2015

Date: 07/31/2015

Title of Project: Sensitivity Analysis Tools for Clinical Trials with Missing Data

Type of Progress Report: ☐ 6-month ☐ 12-month ☒ 18-month ☐ 24-month ☐ 30-month ☐ Other (specify in the box below)

Period Covered by this Report: 02/02/2015 to 07/31/2015

Principal Investigator & Institution Updated Contact Information:

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Key Patient and Other Stakeholder Partner Contact Information (up to three): N/A

Name:		
Telephone:		
Email:		
Name:		
Telephone:		
Email:		

Name:	
Telephone:	
Email:	

MILESTONES UPDATE

Expand on milestone status in the Accomplishments and Challenges section. Add additional rows if necessary.

Record each milestone label, name, description, and projected completion date, as shown in Attachment B (Milestone Schedule) of your Contract. All milestones in Attachment B should be listed in Columns A – D.

Column E: Check appropriate box indicating milestone completion status during reporting period.

Column F: Select actual date of milestone completion.

Column G: Select appropriate reason for delay/non-completion of projected milestone during the specified reporting period.

Note: Retain information regarding milestone completion from all previously submitted reports (e.g., Milestone Updates submitted on the 18-month report should contain information from the 12-month report, and so on).

Column A	Column B	Column C	Column D	Column E	Column F	Column G
Milestone Label *(e.g., B-1, etc.)*	**Milestone Name**	**Description**	**Projected Completion Date**	**Completed? (Yes/No)**	**Date Completed**	**If Not Completed, Reason for Delay**
B-1	Website	Expand registration on website to include PCO researchers	7/31/2014	☒Yes☐No	10/31/2014	Choose an item.
B-2	Advisory Board	Convene Meeting	7/31/2014	☒Yes☐No	7/21/2014	Choose an item.
C	Submit Interim Progress Report	Interim Progress Report	7/31/2014	☒Yes☐No	7/31/2014	Choose an item.
D-1	Case studies/training materials	Create PCO-centered case study and training materials	1/31/2015	☐Yes☒No	10/30/2015	Choose an item.
D-2	Short courses	Facilitate two short courses	1/31/2015	☒Yes☐No	1/12/2015	Choose an item.
D-3	Adobe connect session	Adobe connect session with users	1/31/2015	☒Yes☐No	1/12/2015	Choose an item.

D-4	Manuscript for monotone missing data	Submit case study to PCOR focused journal	1/31/2015	☐Yes☒No	10/30/2015	Choose an item.
E	Submit Interim Progress Report	Interim Progress Report	1/31/2015	☒Yes☐No	2/2/2015	Choose an item.
F	Advisory Board	Convene Meeting	7/31/2015	☐Yes☒No	11/31/2015	Choose an item.
G	Submit Interim Progress Report	Interim Progress Report	7/31/2015	☒Yes☐No	8/21/2015	Choose an item.
			Click here to enter a date.	☐Yes☐No	Click here to enter a date.	Choose an item.
			Click here to enter a date.	☐Yes☐No	Click here to enter a date.	Choose an item.
			Click here to enter a date.	☐Yes☐No	Click here to enter a date.	Choose an item.
			Click here to enter a date.	☐Yes☐No	Click here to enter a date.	Choose an item.
			Click here to enter a date.	☐Yes☐No	Click here to enter a date.	Choose an item.
			Click here to enter a date.	☐Yes☐No	Click here to enter a date.	Choose an item.
			Click here to enter a date.	☐Yes☐No	Click here to enter a date.	Choose an item.
			Click here to enter a date.	☐Yes☐No	Click here to enter a date.	Choose an item.
			Click here to enter a date.	☐Yes☐No	Click here to enter a date.	Choose an item.
			Click here to enter a date.	☐Yes☐No	Click here to enter a date.	Choose an item.
			Click here to enter a date.	☐Yes☐No	Click here to enter a date.	Choose an item.

RECRUITMENT UPDATE

Projects enrolling participants in a trial **must** complete this section. Leave blank if not applicable

Record the target recruitment number for your project: _____

Complete Table 1 to assist in recruitment tracking over the duration of the project. Retain information regarding recruitment from all previously submitted reports. Add additional rows as necessary.

Column A: record the date of each interim report.

Column B: record the number of participants screened for enrollment to date.

Column C: record the number of eligible participants of those screened.

Column D: record the total number of participants enrolled in the study to date.

Column E: record the number of participants projected to be enrolled to date.

Column F: record the total number of participants retained to date. (If the number of participants retained is less than the number enrolled in the study, please explain in the Accomplishments and Challenges section.)

Table 1 Recruitment

Column A	Column B	Column C	Column D	Column E	Column F
Report Date	**Screened Participants (N)**	**Eligible Participants of those Screened (N)**	**Recruited Participants to Date (N)**	**Participants Projected to be Recruited to Date, Per Project Milestone Schedule (N)**	**Retained Participants to Date (N)**
Example 6/15/2014	*1000*	*800*	*650*	*750*	*650*

RECRUITMENT UPDATE (*Continued)*

Complete Table 2 by listing the Racial/Ethnic and Gender breakdown of the participants enrolled in your study to date. Ensure totals are calculated and appropriately recorded. If you have not collected these data, please explain why.

Table 2 Racial/Ethnic and Gender Enrollment Table*

Race	**Male (N)**	**Female (N)**	**Total (N)**
American Indian/ Alaska Native			
Asian			
Black/ African American			
Hawaiian/ Pacific Islander			
White			
Multi-race			
Ethnicity	**Male (N)**	**Female (N)**	**Total (N)**
Hispanic (Latino/Latina)			
Non-Hispanic			

*If more detailed information is available regarding racial/ethnic subgroups for the participants in your study, please provide that information in a separate table.

ENGAGEMENT REPORT

(Annual report only)

Descriptive information on engagement of patients and/or other stakeholders should be reported in each **annual** report (i.e., at year 1, year 2, etc. but not at 6 months or 18 months).

This report is intended to capture the perspective of the researcher(s) on your team. You will be given the opportunity to nominate patient and/or stakeholder partners to provide additional engagement information to PCORI.

Use the link below to complete this report. Your Username is your PCORI contract number *(no letters, dashes, or spaces)*.

Provide your engagement report here: https://live.datstathost.com/PCORI-Collector/Survey.ashx?Name=Engagement_Report_Login

When you have completed the questions, record your confirmation code: _____

ACCOMPLISHMENTS AND CHALLENGES

Use continuation pages as needed. Limit three pages.

Discuss and document study progress and all significant events. **In particular, please discuss**:

- Any significant change from the funded proposal, including changes in the study protocol, engagement plan, endpoints, sample size, etc. Include reasons for these changes. Please note that you should discuss changes with PCORI program staff prior to implementation.

  No significant change.

- Progress and accomplishments achieved during the reporting period, with reference to planned project activities, milestones, and planning for dissemination.

  Solved methodological issues regarding confidence interval construction and intermittent missing data. Presented poster at the FDA ORSI Symposium and gave short course at the Society of Clinical Trials Annual Meeting in Arlington, VA. Posted a new R version of SAMON. Completed manuscript about the methodology underlying SAMON, which will be submitted to the *Journal of the American Statistical Association* by 8/31/2015. Recorded lecture for PCORI on methods for analyzing studies with missing data.

- Progress on your engagement plan during the reporting period, relative to the activities outlined in your application.

  We interacted informally with some members of our advisory board regarding technical issues.

- Challenges with **project progress** (e.g., delays in IRB approval, delays in recruitment of sites, participant retention issues). Have you overcome these challenges? If yes, please describe. If not, what resources or guidance do you need?

  The greatest challenge was solving methodological issues regarding confidence interval construction and intermittent missing data. So far, these solutions appear adequate.

  - Describe any barriers that you are expecting to occur in your project in the next six months and ways you plan to overcome them.

    Access to PCO-oriented datasets. Identification of venues for short courses. Dissemination of methods and software. We plan to work with our advisory board to address these issues.

- Summarize any significant actions taken by the IRB, DSMB or other regulatory oversight body during the reporting period.

  Not applicable.

- Challenges with **patient and stakeholder engagement**. Have you overcome these challenges? If yes, please describe. If not, what resources or guidance do you need?

  We could use the assistance of PCORI to help disseminate our methods and software, for example, through the PCORI listserv, social media, newsfeeds, and methods meetings/seminars.

- PCORI is interested in monitoring project adherence to **PCORI's Methodology Standards.** Your Program Officer will review compliance with Methodology Standards and work with you to develop a plan for follow-up on all important methodological issues. As part of this report, you may provide documentation of adherence to Standards that apply to your ongoing research. You may include additional documents with this report, for example, a final study protocol.

  Not Applicable

PROGRESS STATEMENT FOR PUBLIC USE

Limit 250 words.

Describe notable progress to date, preliminary results, or engagement/stakeholder experiences using nontechnical language that is ready for public use. (Note: This information may be publicly disseminated by PCORI.)

We posted a new version of the R software package SAMON on the www.missingdatamatters.org website.

FINANCIAL STATUS UPDATE

Limit two pages.

Describe any significant deviations in costs and budget, how those deviations affected the study progress (e.g., staffing and cost estimates), and any anticipated need for budget modifications. Significant deviations are considered any adjustment that exceeds 25% of a budget category.

There have not been any significant deviations in costs and budget.

KEY PERSONNEL EFFORT UPDATE

Use continuation pages as needed. Limit one page.

Key Personnel changes in excess of 25% of contracted level of effort, must be reported annually. Report the individual's role, change in percentage effort, and an explanation for changes. If you have more than five changes to report, please include additional information under "Explanation of Changes." Send PCORI a biosketch for all new key personnel working on the project.

[X] No changes in key personnel

Name (First, Last)	Title	Contracted Percentage Effort	Actual Percentage Effort
		%	%
		%	%
		%	%
		%	%
		%	%

Explanation of Changes:

Note any **proposed** changes to key personnel in the next 6 months, if applicable, and provide an explanation for changes, below. Add rows as necessary. If you have more than five proposed changes to report, please include additional information under "Explanation of Changes." Send PCORI a biosketch for all new key personnel working on the project.

[X] Not Applicable

Name (First, Last)	Title	Contracted Percentage Effort	Actual Percentage Effort
		%	%
		%	%
		%	%
		%	%
		%	%

Explanation of changes:

PUBLICATIONS UPDATE

REMINDER: Please make sure that all publications/communication/media pieces contain the following acknowledgement of PCORI funding: "Research reported in this [work, publication, article, report, presentation, etc.] was [partially] funded through a Patient-Centered Outcomes Research Institute (PCORI) Award (##-###-####)."

Please make sure your publications that present research findings, conclusions or other editorial content also include this disclaimer: "The [views, statements, opinions] in this [work, publication, article, report] are solely the responsibility of the authors and do not necessarily represent the views of the Patient-Centered Outcomes Research Institute (PCORI), its Board of Governors or Methodology Committee."

In the tables below, record information regarding publications and presentations (scientific and non-scientific) related to your PCORI-funded research that occurred as of the reporting date. Retain information submitted in previous reports.

Publications and/or presentations by any member of the research team, including patient and stakeholder partners, should include those:

- **In preparation to be submitted.**
- **That have been submitted to a publication.**
- **That have been accepted to a publication.**
- **That are in-press.**
- **That have been published.**

a) Peer and Non Peer-Reviewed Publications (e.g., scientific publications and non-scientific publications such as, organizational journals, magazines, newsletters, newspapers, blogs, other lay press)

Author(s)	Date	Title	Type (e.g., manuscript, abstract, poster, newspaper or magazine article, blog, other)	Name of publication	URL, if applicable	Status (e.g., in preparation, submitted, accepted, in press, published)
PEER REVIEWED						
Scharfstein, Rotnitzky, Abraham, McDermott, Chaisson, Geiter	7/26/2015	On the Analysis of Tuberculosis Studies with Intermittent Missing Data	Manuscript	Annals of Applied Statistics	www.missingdatamatters.org	In Press
Wang, Scharfstein,Colantuoni, Girard, Yan	6/25/2015	Inference in Randomized Trials with Death and Missingness	Manuscript	Biometrics	www.missingdatamatters.org	Under Revision
Scharfstein, McDermott, Diaz, Carone, Lunardon, Turkoz	8/31/2015	Global Sensitivity Analysis of Repeated Measures Studies with Informative Drop-out: A Semi-	Manuscript	Journal of the American Statistical Association		Submitted

		Parametric Approach				
Colantuoni, Scharfstein, Wang, Needham, Girard	8/21/2015	Accounting for Mortality and Missing Data when Comparing Outcomes Across Treatment Groups in Randomized Trials	Manuscript	Annals of Internal Medicine		In Preparation
Scharfstein, McDermott	8/21/2015	Global Sensitivity Analysis of Clinical Trials with Missing Patient Reported Outcomes	Manuscript	Clinical Trials		In Preparation
NON-PEER REVIEWED						
Kharazzi, Wang, Scharfstein	4/18/2014	Prospective HER-Based Clinical Trials: The Challenge of Missing Data	Editorial	Journal of General Internal Medicine	www.missingdatamatters.org	Published

b) Presentations (e.g., invited talk, local provider meeting, webinar, YouTube video)

Presenter(s)	Date	Title	Type (e.g., oral, poster, YouTube video, webinar, other)	Name and Location (e.g., conference/meeting name and location, recorded webinar with URL, local meeting name and location)	Audience, if known (e.g., researchers, patients, clinicians, policy makers, students, community organizations)	Status (e.g., in preparation, submitted, accepted, completed)
PEER REVIEWED						
NON-PEER REVIEWED						
McDermott	8/3/2014	Global Sensitivity Analysis of Repeated Measures Studies with Informative Dropout: A Semi-Parametric Approach	Oral	Joint Statistical Meetings, Boston, MA	Researchers	Completed
Scharfstein	9/18/2014	Global Sensitivity Analysis of Repeated Measures Studies with Informative Dropout: A Semi-Parametric Approach	Oral	Andrei Yakovlev Colloquium, University of Rochester, Rochester, NY	Researchers	Completed
Wang	9/24/2014	Inference in Randomized Trials with Death and Missingness	Oral	ASA Biopharmaceutical Section FDA-Industry Workshop, Rockville, MD	Researchers, Practitioners	Completed

Scharfstein	12/8/2014	Global Sensitivity Analysis of Randomized Trials with Missing Data: Recent Advances	Oral	Short course at Deming Conference, Atlantic City, NJ	Researchers, Practitioners	Completed
Scharfstein, Wang, McDermott	1/12/2015	Analysis of Randomized Trials with Missing Data	Oral, Adobe Connect	Short course at Johns Hopkins University, Baltimore, MD	Researchers, Practitioners	Completed
Scharfstein	4/27/2015	Global Sensitivity Analysis of Randomized Trials with Missing Data	Poster	FDA ORSI Symposium	Researchers, Practitioners, Policy Makers	Completed
Scharfstein	5/17/2015	Global Sensitivity Analysis of Randomized Trials with Missing Data	Oral	Short course at Society for Clinical Trials, Arlington, VA	Researchers, Practitioners	Completed
Scharfstein	7/31/15	Analysis of Prospective Studies with Missing Data	Online Lecture	Johns Hopkins University, Baltimore, MD	Researchers, Practitioners, Policy Makers	Under Editorial Review

PUBLICATIONS UPDATE (*Continued*)

c) Peer Review Rejection Notifications

If applicable, provide information regarding scientific article(s) submitted for publication and rejected, during the reporting period.

Author(s)	Date Rejected	Title	Type (e.g., manuscript, abstract, poster, other)	Name of publication	URL, if applicable

ADDITIONAL DOCUMENTS

All attachments should be combined with this document and submitted to PCORI as one PDF.
Additionally, please complete the "Other Publications" section below, if necessary.

Any documents that you feel are relevant and noteworthy can be shared, such as:

- Copies of drafts of instruments, data dictionaries, educational materials, manuals, or other project deliverables, if not already delivered
- Abstracts from presentations made to professional groups or associations
- Summaries of preliminary data
- Bibliographies
- Summaries from DSMB meetings
- Final study protocol
- Other communications efforts
- Copies of reports from any consultants or advisors, where applicable
- Other documents or materials, as appropriate

CERTIFICATION

This document must be certified by the Principal Investigator and the designated Administrative Official (AO).

Principal Investigator:

☒ I certify that I, as the Principal Investigator, have reviewed and approved this document (and any associated attachments, if applicable).

PI First Name: Daniel
PI Last Name: Scharfstein
Date: 8/21/2015

Administrative Official:

☐ I certify that I, as the designated Administrative Official, have reviewed and approved this document (and any associated attachments, if applicable).

AO First Name:
AO Last Name:
Date: