Consortia to Accelerate Therapeutic Applications of Stem Cells
RFA # 1212030210

QUESTIONS AND ANSWERS AND MODIFICATIONS – SECOND SET
Received January 14, 2014 through January 28, 2014
Including an applicant telephone conference
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MODIFICATIONS TO RFA

The following has been updated/modified in the RFA. Strike-through indicates deleted text; underlined text is new.

1. Due to an error in Section A on Form 16, Human Stem Cells, the form set for Forms 7 & 9-16 has been re-posted to the website. If applicants have already been completing this form set, it is not essential to convert to the revised, re-posted form set. However, compliance to the instructions for Form 16, Section A, #6 (found on page 43 of the RFA) will be required. Thus, #6 on the revised, re-posted Form 16 now reads "SCRO Approval Date (leave blank only if YES to #5)."

2. The instructions for Form 13 – Section d, Milestones and Timeline, have been modified as follows:

   d. Milestones and Timeline
   This section is not counted against page limitations. In the context of the Research and Development Plan, provide a ~~detailed~~ timeline that ~~outlines~~ summarizes specific project activities, key decision points and milestones. Milestones should note specific measurable outcomes of key objectives and tasks (activities) that provide landmarks of progress toward achieving the specific aims of the project, including an estimate of the timing of key decision points. For the purposes of this application, "milestones" are precise, quantifiable study outcomes for key project activities, not simply the work to be conducted or the aims to be met. Also include the outcome of interactions with regulatory bodies as milestones. In addition, outline the critical path to accomplish the goals of the Workplan by the end of the contract term (within four years). It is **highly recommended** that a detailed Gantt chart, decision trees and workflow diagrams be provided in the appendix to supplement this section. **Note:** Milestone achievement will be an important indicator of progress and a major factor in progress reviews; insufficient progress may lead to termination of funding.

3. Instructions for the submission of digital files (RFA page 30) have changed. They now read as follows:

   The CD or DVD should be clearly labeled with the applicant's name. It should contain ~~a single Portable Document Format (PDF) file including~~ the following items:

   - Applicant Forms 1 – 6 in a *single* Microsoft Word (DOC or DOCX) file;
   - Applicant Forms 1 – 6 in a *single* Portable Document Format (PDF) file;
   - Forms 7 and 9 – 16 and all appendix material in a *single* PDF file
   - Form 8s as multiple Microsoft Excel (XLS) files – one workbook file for each budget year for the applicant organization and one workbook file for each budget year for each sub-applicant organization;
   - All Form 8s combined in a single PDF file – include all applicant and sub-applicant budgets for each year;
   - Sub-applicant Form 1-S for each Sub-applicant in Microsoft DOC or DOCX file (Form 1-S may be omitted if there are no Sub-applicants included in the application); and
   - Signed and dated Forms 1 and 1-S (Face Pages for the Applicant and all Sub-applicants) scanned together and saved as a *single* PDF file.

4. Commensurate to Modification #3, the Penalties have changed on the Part Two Application Checklist (RFA page 29). Penalties will be assessed as follows:

APPLICATION PENALTIES:

A total penalty of 0.1 point will be assessed to a Part Two application if:

- Digital submission is password protected
- Forms provided ~~in~~ with this RFA are not used
- Submission does not adhere to page ~~(or word count)~~ limits (Forms ~~5,~~ 6, 9, 10, 12, and 13)
- Submission does not include:
  - Applicant Forms 1 – 6 in a single Microsoft Word (DOC or DOCX) file
  - Applicant Forms 1 – 6 in a single Portable Document Format (PDF) file
  - Forms 7 and 9 – 16 and all appendix material in a single PDF file
  - Form 8s as multiple Microsoft Excel (XLS) files – one workbook file for each budget year for the applicant organization and one workbook file for each budget year for each sub-applicant organization
  - All Form 8s combined in a single PDF file – include all applicant and sub-applicant budgets for each year
  - Sub-applicant Form 1-S for each Sub-applicant in Microsoft DOC or DOCX file (Form 1-S may be omitted if there are no Sub-applicants included in the application) **NOTE:** No other forms may be omitted
  - Signed and dated Forms 1 and 1-S (Face Pages for the Applicant and all Sub-applicants) scanned together as a single PDF file. **NOTE:** Form 1-S may be omitted if there are no sub-applicants. No other forms may be omitted
  - ~~Budget and Justification – Form 8 – one for each year of the applicant and one for each year of the sub-applicant organization~~
  - Biographical Sketch – Form 9 – one for each key personnel ~~listed on each Form 9~~
  - Facilities and Resources – Form 10 – one for the applicant and one for each sub-applicant organization
  - Other Support – Form 11 – one for each key personnel ~~listed on each Form 9~~
  - Introduction – Form 12 – may be left blank or marked "N/A" if application is not a resubmission
  - Workplan – Form 13 – limited to 35 pages for parts a-c
  - Human Subjects – Form 14 – at least one per applicant and sub-applicant; and one for each protocol used for this research project
  - Vertebrate Animals – Form 15 – at least one per applicant and sub-applicant; and one for each protocol used for this research project
  - Human Stem Cells – Form 16 – at least one per applicant and sub-applicant; and one for each protocol used for this research project

5. The following supplemental language is inserted between the first and second paragraphs of the instructions for Form 8 on page 35 of the RFA. Also see Modification #3 & 4 above.

   Submit one full budget form (all six "tabs" within the Excel spreadsheet) for each year for the applicant. Also submit one full budget form (all six "tabs" within the Excel spreadsheet) for each year for each sub-applicant. For example, if the application includes an applicant and two sub-applicants and each of them will have funding in all four years of the contract, you will submit 3 x 4, or 12 budget forms (with 6 tabs each for a total of 72 "tabs"). Each budget form should be submitted as a separate Excel file and must be named appropriately with the application number, institution name, and budget year. Sub-applicant budgets should be labeled with the application number, sub-applicant institution name, and budget year (Ex. "N14C-999 Harvard yr 1 budget", "N14C-999 Harvard yr 2 budget", "N13C-999 sub-Boston College yr1 budget").

   Also submit these budget forms as part of a single PDF file.

6. Due to an error on Part Two Forms 1 and 1-S, these forms have been revised and re-posted to the website.

QUESTIONS AND ANSWERS

General

1. Is this RFA being rereleased because the Board did not like the content of round one applications? Was it the topics or organ systems covered that they didn't like?

   A. No. The Funding Committee authorizes the release of RFAs and makes recommendations for award based on the peer review score and a host of other factors as outlined in Section VI.E. of the RFA. The Funding Committee is not obligated to recommend funding for any application. They do consider the scores for significance and impact, and approach; they have further discretion as well (see RFA VI.E., page 15-16). An additional factor would be peer reviewer feedback that the aims are not likely to be accomplished within the contract term or that the project is not well-developed for such a substantial investment from NYSTEM. NOTE: Page 16 of this RFA clearly states that "The Funding Committee has sole discretion to reject an application that overlaps an existing NYSTEM consortium award under RFA# 0911051012."

2. Will a third round be issued if they don't like the applications from this round?

   A. That determination has not yet been made by the Funding Committee.

3. How many Part One applications were received?

   A. That information cannot be made available at this time.

4. Prior to application submission, should applicants consult with the FDA?

   A. That's up to the Applicant. For instance, the Applicant may need to determine whether portions of the application need to be conducted under GLP or GMP standards so that steps don't need to be repeated prior to submitting an application to the FDA.

5. Is an international subcontractor site allowed?

   A. Yes, as long as the subcontractor will be able to comply with all of the terms and conditions of the contract (see Attachment 4 to the RFA).

Staffing

6. Is there a minimum percentage of effort for the PI?

   A. No. Propose the percentage of effort that you believe is appropriate for that individual to accomplish all that they are responsible for in the award. Explain and justify those decisions (also see questions 20 - 23, below).

7. Will the peer reviewers look to see if there's a specific project manager listed on the application and look into their qualifications? Does it help to list someone or should we say that someone will be hired that fits these qualifications?

    A. If you have a commitment from a highly qualified and trained scientific project manager, it might benefit your application to name him or her. Otherwise, provide a complete explanation of your expectations for the early recruitment of that position (minimum credentials and experience) and make sure the budget reflects those expectations. NOTE: Filling this position at the beginning of the contract term with a highly qualified Project Manager will be critical in putting together the documentation needed for the first meeting with the oversight panel AND any changes to Key Personnel after submission and during the course of the award require pre-approval from NYSTEM. This includes the naming/identification of personnel not named in the application.

8. Can the co-investigators listed change between Part One and Part Two of the application?

    A. Yes, co-investigators can change; PIs and Co-PIs who were named in Part One cannot be changed in Part Two. PLEASE NOTE that changing co-investigators can delay the time it takes to put together the peer review panel. The peer review panel won't be finalized until the contractor screens the Part Two applications and conflicts of interest are identified for the tentative panel review members.

<u>Peer Reviewers and Oversight Panel Member Selection</u>

9. Are the peer review panel members mostly from outside of New York?

    A. Yes, the peer review panel is selected from outside of New York State.

10. The purpose of this application is the production of a product that is going to eventually go into the clinic. So will the peer review group be industry based so that they are better able to evaluate the proposal? This is going to be a little different and not necessarily discovery type of research.

    A. The peer review group is selected based on the expertise needed to review the application with consideration of the project goals (i.e., disease and/or organ system studied, how far along the project is toward clinical application).

11. For Resubmission applications, will peer reviewers have access to the first round peer reviews?

    A. Yes. For Resubmission applications, be sure to complete the Introduction (Form 12) according to the instructions on Page 38 of RFA, do not exceed three pages.

12. For a Resubmission, will the reviewers be the same reviewers used in Round 1 or will selection depend on the information provided within Part One of the application?

    A. We do not have "standing review panels" so the reviewers may or may not have overlap with those used in Round 1. For each round of applications reviewed, the emphasis is to make sure the proper expertise is represented for the applications that are submitted.

13. Do we have input to the composition of the Oversight Panel?

    A. Yes, as per the instructions for Form 3, see "Part Two Independent Oversight Panel – Form 3" on Page 34 of the RFA. The Oversight Panels aren't finalized until after we have made consortia awards and selected the contractor who is going to manage the Oversight Panels for NYSTEM.

Contract Execution Timelines

14. When do you expect to announce awards?

    A. We expect that the Funding Committee will make its recommendations in October. However, recommendations will undergo an internal approval process that can take several weeks.

15. Were the first round Consortia contracts started on time?

    A. The contract term started March 1 and all contracts were executed on or before June 5, 2013. This meant awardees could have been "working at risk" for approximately three months. After contract execution awardees were able to bill NYSTEM for eligible expenditures incurred as of March 1. Because there are statewide changes coming to the contracting process, applicants should plan for the possibility of late contract execution.

Application Content and Forms

16. Are all forms required?

    A. Yes, with only one exception. If your consortium application does not include any sub-applicants, the application will not include Part Two Sub-Applicant Face Page - Form 1-S.

17. Is Form 3 required?

    A. Yes; you must include Form 3 in your application. You may choose not to complete it but do not leave out any of the forms with the possible exception of 1-S (see above # 16) from the application package you submit.

18. How do I complete Form 12 if my application is not a Resubmission?

    A. If your application is not a Resubmission, indicate N/A on Form 12 as per instruction on Page 38 of RFA.

19. How many Forms 14-16 do I need in my application?

    A. The application will have at least one Form 14, 15 and 16 for the applicant organization and a separate Form 14, 15 and 16 for each sub-applicant. For example, if the application includes an applicant and two sub-applicants, there will be at least three of each form, completed according to the instructions provided within the RFA.

       Also, provide a separate form for each research protocol of that type. For example, if the applicant organization's work will require two separate vertebrate animal protocol approvals, the applicant organization will include two Form 15s.

       NOTE: Form 16 has been modified (see **Modifications**, above).

20. How many budget forms will I have to submit?

    A. Submit one full budget form (all six "tabs" within the Excel spreadsheet) for each year for the applicant. Also submit one full budget form (all six "tabs" within the Excel spreadsheet) for each year for each sub-applicant. So if the application includes an applicant and two sub-applicants and each of them will have funding in all four years of the contract, you will submit

3 x 4, or 12 budget forms (with 6 tabs each for a total of 72 "tabs"). Each budget form should be submitted as a separate Excel file and must be named appropriately with the application number, institution name, and budget year. Sub applicant budgets should be labeled with the application number, sub-applicant institution name, and budget year (Ex. "N14C-999 Harvard yr 1 budget", "N14C-999 Harvard yr 2 budget", "N13C-999 sub-Boston College yr1 budget"). All budget forms should also be submitted as a single PDF file. See **Modifications**, above.

21. Since Patient Care is not an allowable expense, where do we list the costs associated with clinical trials?

    A. Ineligible Patient Care expenses are those expenses that would have been incurred even if the research study did not exist**.** Costs associated with clinical trials are not considered to be Patient Care expenditures. For example, a patient enrolls in your clinical trial. Part of the trial is a baseline cardiac fitness screening. The costs for the screening are eligible for reimbursement under this contract. But suppose you detect a significant cardiac function problem in this patient that requires treatment. Contract funds cannot be used to provide that patient with such treatment.

22. For travel budgeting purposes of consortium members, where will meetings of the Oversight Panel take place?

    A. Meetings of the consortium with the Oversight Panel will occur in person at least annually. All other meetings are expected to occur by phone, webinar or videoconferencing. In-person meetings are generally expected to be held within the facilities of the consortium member institution most appropriate to the milestone(s) under review. As you establish your workplan and timeline (Gantt chart) with milestones and key decision points noted, you should be able to anticipate the location where a meeting might be held. PLEASE NOTE that currently funded consortia from "upstate New York" have sometimes met the Oversight Panel in DOH offices in New York City (when proximity to major international airline hubs has allowed scheduling of a timely meeting).

23. How does one make a reasonable estimate of something that can't be described in precise detail such as GMP future pricing?

    A. Provide a carefully-considered estimate and fully describe the rational basis for that estimate.

24. On the first page of the Workplan, Form 13, there is a box where the instructions say to copy and paste our Scientific Abstract. Are we limited to the size of that box?

    A. No. The text box will expand, and if it continues onto additional pages, that is fine. This does not count toward the 35 page limit for the narrative portion of Form 13, Sections a-c. However, your Scientific Abstract should conform to the instructions for Form 6.

25. On the Workplan, Form 13, Detail Tables, can we add rows for additional objectives (aims) and tasks (sub-aims)? Can we delete extra rows?

    A. Yes, you can add or delete rows to that table as necessary.

26. On what page are the instructions for completing the Workplan, Form 13?

    A. Instructions for completing Form 13 begin on page 39 of the RFA.

27. Where should literature citations for publications appear in the application? Are they included in the 35 page limit?

    A. Citations should appear in Section f of the Workplan Narrative (Form 13). They do not count toward page limits.

28. Where are publications or manuscripts to be placed?

    A. The appendix may include up to two highly relevant publications or manuscripts (published or in press) if they are essential to document the investigator's capability to undertake the work proposed (see Part Two Application Checklist, RFA page 29). NOTE: Appendix material may not substitute for any portion of the Workplan Narrative (Form 13) or be used to subvert its 35 page limit.

29. What other items should be placed in the appendix?

    A. RFA page 29 provides a sample list of items which might be included, none of which are required. However, equipment quotes (if equipment is included in the budget), Gantt charts, decision trees, workflow diagrams and other such items should be in the appendix.

30. If the Gantt chart is an appendix, what belongs in Section d of the Workplan Narrative, Form 13?

    A. A detailed Gantt chart should be included in the appendix; a condensed/summary version or table format may be used in Section d. See **Modifications** above.

31. Could we put things like GMP protocols, pre-IND materials and FDA comments in the appendix?

    A. Yes. NOTE: Appendix material may not substitute for any portion of the Workplan Narrative (Form 13) or be used to subvert its 35 page limit.

32. File sizes are limited to 12 megabytes, right?

    A. Yes. Please note that one PDF file will contain Forms 7 and 9-16 and all of your appendix material. The RFA instructions (page 20) include recommendations for reducing file size. All Form 8s will be contained in one additional PDF file. Form 8s will also be submitted as separate Excel files. See **Modifications** above.