

Consortia to Accelerate Therapeutic Applications of Stem Cells
RFA # 1212030210
QUESTIONS AND ANSWERS #1
Received November 15 through December 17, 2013
(Including an applicant conference held on December 13, 2013)

Eligibility and Collaborations

1. Could you please email me a list of names for the other individuals from my institution who are participating in this call so we don't make the mistake of submitting multiple applications from our institution?
 - A. No. Registration in and participation on the call were not mandatory, so the list could be incomplete. The best way for you to determine competing interest from within your organization is to contact appropriate offices within your institution such as the VP of Research and the Office of Sponsored Programs. They will need to coordinate efforts so that only one application is received from your institution. Otherwise all applications from your institution will be disqualified.

2. Would a consortium of clinical and research PIs within our own institution (and our affiliated clinical center) satisfy the requirement for collaborative proposal?
 - A. Yes, it could. Provided that the team is multi-disciplinary.

3. Is there a minimum number of subcontractors and/or PIs expected?
 - A. No. The team must be multi-disciplinary.

4. Does it matter if a collaborator on this project is out of the country, or out of the state?
 - A. No, as long as they are able to comply with all parts of the contract.

5. Can a subcontractor be for-profit?
 - A. Yes.

6. Does the industrial partner have to be in New York State?
 - A. No, none of your partners need to be in New York State. The only geographic eligibility requirement is that the applicant needs to be either a not-for-profit or government organization (e.g., SUNY, CUNY) in New York State.

7. If we need to outsource a part of this project, is there a limit on the project's percentage to allocate for this?
 - A. No. The key is to reach the clinical goal set out in your application within the budget and timeframe established. The amount of funding that goes out of state or to a for-profit contracted research organization (CRO) is not

likely to be a significant factor for the Funding Committee when making an award recommendation.

General Expectations (Section III of the RFA)

8. Is the goal of the RFA to have a GMP cell product that will be ready to go in the clinic at the end of the four year period? If so, will money be provided for the actual clinical trial?
 - A. Yes. If clinical trials are included in the budget and timeline of the four year award, those expenses can be supported.
9. Given that clinical trials are the intended aim of the RFA, is partnership with industry in anyway evaluated as part of the score?
 - A. Yes. See page 15, Evaluation Criteria (Investigators and Management) in Section VI.D. of the RFA.
10. Is there an expectation that the application include large animal models?
 - A. Not specifically. The application must include data developed by the participating investigators that demonstrate proof-of-principle in an appropriate pre-clinical model. The choice of animal model(s) is a decision you will have to make, possibly in consultation with the FDA, but could include large animals.
11. What categories or types of stem cells would qualify (e.g., adult, iPS, human embryonic, cancer stem cells)?
 - A. Any type of stem cell can be used that is appropriate to get to clinical application. Consideration should be given to potential pitfalls in using that cell type, particularly from a regulatory standpoint.
12. Is the use of stem cells as a drug screening tool eligible?
 - A. No. The use of stem cells as a platform or a tool (e.g., for drug screening) is not eligible under this RFA. The emphasis is as follows: "The ESSCB wishes to accelerate development of **new clinical applications** of stem cells for prevention and/or treatment of disease."
13. If the intended application is a clinical trial that has already been approved by the FDA, is the project eligible?
 - A. The answer likely depends on which pieces of the clinical trial you would be asking NYSTEM to support and whether the Funding Committee will view them as "novel." Assuming the application scores well, that is up to their discretion.
14. Would a dual-approach (one that includes drug screening and a cellular-based therapy) be responsive to the RFA?

- A. Such an application would be eligible but is likely to present concerns regarding the feasibility of accomplishing the goal within the budget and timeline. Assuming the application scores well, the Funding Committee would determine whether it wishes to fund such a project.
15. Would funding for a Phase II trial, testing efficacy of the stem cell treatment compared to controls, be eligible under this RFA?
- A. It could. Assuming the application scores well, the Funding Committee would determine whether it wishes to fund such a project.
16. Preliminary data suggest that our stem cell treatment would be more effective if combined with an “add-on” treatment. Would preclinical testing and a move into Phase I trials within the period of the award be considered “novel” under this RFA?
- A. It could. Assuming the application scores well, the Funding Committee would determine whether it wishes to fund such a project.
17. Does the RFA allow construction of a stem cell facility to support the needs of our institution to scale up manufacturing of cells moving from Phase I to Phase II?
- A. No. Construction costs are not eligible under this RFA. Collaboration with existing facilities would be appropriate.
18. Page 2 of the RFA states a responsive application “...will present a strong explanation of the capability to achieve a significant measurable advance toward clinical application within the period of the award.” Is there any pre-determined benchmark that must be met to qualify as “a significant measurable advance toward clinical application”? Will responsive applications be expected to include an IND application, for example?
- A. Essentially, yes. The purpose of the funds is “to achieve a significant measurable advance toward clinical applications.” If your consortium can define an alternative “measurable advance” to an IND, and the application scores well, the Funding Committee would determine whether it wishes to fund such a project.

Staffing and Leadership

19. Do you recommend that the Project Manager be identified at the time of application?
- A. If you have a commitment from a highly qualified, specially trained scientific project manager who has done work in preparing for clinical studies similar to yours, then name them in the application. 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.

20. Can the full time 100% effort Project Manager position be shared by two or more individuals?
- A. No. A single, full time Project Manager who puts 100% professional effort toward the project is required. However, additional staff may contribute additional professional effort as project managers if well-justified. NOTE: This position is much more specialized than a lab manager position.
21. Does the Project Manager need to be an employee?
- A. No. But the position does need to be full-time, with 100% professional effort to the project.
22. If a Project Manager is named during the application process but accepts another job during the contract, does the replacement need to be justified and would it be hard to provide that justification?
- A. Replacement of all Key Personnel listed in the application requires advanced approval from NYSTEM. You would submit the CV of your preferred candidate, which should clearly indicate that the level of expertise, experience and skill is sufficient to complete the work assigned to that individual. NYSTEM response time to those types of requests is generally three to five working days.
23. What happens if a PI or Co-PI is named in Part One of the application process and changes for Part Two of the application process?
- A. Except in extraordinary circumstances (e.g., death or disability), the application will be disqualified (see page 30 of the RFA).
24. If someone is currently funded on another project, can we list them as giving one hundred percent effort on this project?
- A. Yes, as long as the adjustment of professional effort (commitment overlap) is resolved prior to their start on the consortium project (see page 38 of the RFA).
25. The final bullet point of Part One Form 4 Self-assessment checklist states “No scientific, budgetary, or commitment overlap exists between the application and any other funded project.” Does this encompass any project, even ones not funded by NYSTEM? If a person is currently 100% as a non-key person on an NIH grant, and we plan to put them on at 50% on this NYSTEM application, with the plan to reduce their effort on the NIH award, must we still select “NO” for this question on the checklist?
- A. Yes, this requirement encompasses all projects regardless of funding source. This is a self-assessment checklist; there are no correct or incorrect responses. You could select “YES” because the overlap will have been resolved prior to the anticipated start of the contract term. Or

you could select “NO” so you remember that you need to resolve the conflict.

26. Page 5 of RFA states that “Scientific, budgetary and commitment overlap with another funded project cannot be resolved by amending a NYSTEM-funded award.” Does this mean that if a person is currently on a NYSTEM funded contract at 100% effort, we are prohibited from including them on this application, even if we include a plan to reduce effort on the current NYSTEM award?

A. No. If the plan is to reduce effort on the current NYSTEM award, that would resolve the commitment overlap (see #26). What that statement does mean is that if NYSTEM holds a contract for all or a portion of the work being proposed in the consortium application, we cannot “fold in” the existing contract to the consortium application from either a scientific or budgetary perspective. Nor can we allow the staff commitment on a funded project to “fold into” the consortia application.

Reporting Obligations

27. What are the financial, progress and intellectual property reporting requirements?

A. Financial reports are provided with each quarterly voucher. Progress report schedules and the requirement for other data, information or updates will be established by the Oversight Panel. Semi-annually the contractor will provide an organization-wide report on any intellectual property activity associated with all NYSDOH-funded research projects that might have occurred during the reporting period.

Review and Award Process

28. How will conflict of interests among peer reviewers be managed? Do we need to note those in the application?

A. NYSTEM doesn’t accept input from applicants on peer reviewer selection. Our peer review process is run independently and externally. Our contractor assesses conflict of interest and bias and enforces strict confidentiality standards. If members on the panel have a conflict of interest with your application, they are not given access to it and are not present for discussion and scoring of your application. Likewise, Funding Committee members who have conflicts of interest do not participate in discussion and award recommendation proceedings. NOTE: Input will be accepted from applicants regarding Oversight Panel composition, with a subsequent round after awards are made.

Budget and Justifications

29. The budget forms have changed. Will the Space/Property and Utility categories of the budget be left blank?

- A. If the expenditures for a budget category are included in the indirect cost (F&A) rate calculation, then leave that budget category blank. NOTE: F&A costs are to be listed as a line item under Other Costs.
30. Are consortia F&A considered part of the direct costs? And if so, that means the maximum F&A listed is for the applicant organization's F&A?
A. Yes. And yes.
31. How do we complete the Match and Other Funds columns on the new budget forms? What about the six columns in the Salary section?
A. Leave the Match and Other Funds columns blank. Complete all six columns in the Salary section.

Other Application Forms

32. Is there a page limit for the biographical sketches?
A. Yes, do not exceed three pages for each. Substituting your NIH biosketch is not acceptable; use the NYSTEM form only.

Oversight Panel Operations

33. Will there be an appeals process in case the Oversight Panel reaches an unfavorable decision?
A. No. The Oversight Panel will make recommendations to NYSTEM. NYSTEM will make the final decision.
34. What if the Oversight Panel asks for clarification or more information?
A. NYSTEM is likely to require it of the consortium.
35. Has any of the three funded consortium contracts been terminated by the Oversight Panel recommendations or NYSTEM determinations?
A. Not as of this date.
36. Have any of the three funded consortium contracts been adversely affected by the Oversight Panel recommendations or NYSTEM determinations?
A. NYSTEM thinks that all recommendations and determinations have benefitted the current consortia.

THERE ARE NO MODIFICATIONS TO THE RFA AS RESULT OF THIS QUESTION AND ANSWER PERIOD