

Sanford Stem Cell Clinical Center Project Funding Application Instructions 2014-2015

Updated 3-20-2015

Our goal is to provide administrative and other relevant support to help with the execution of creative development of clinical trials of novel stem cell related therapies for a range of human disorders. The work should be done at UC San Diego in the Sanford Stem Cell Clinical Center, except where this is technically unfeasible or undesirable. In general, we will not support preclinical work with the possible exception, based on strong merit, of support needed to finish up IND-enabling studies. The Sanford Stem Cell Clinical Center offers funding for direct costs specifically related to the project. No overhead costs can be paid.

Strong, fundable projects are in the Late Product Development/Translation stage and include plans to get to Phase I/II clinical trials in two years, July 2015-July 2017. Early-Stage applicants (or Feasibility stage applicants) must provide a strongly persuasive argument for the unique scientific merit or provide the bulk of funding on their end. The Sanford Center will consider funding projects that

- a) perform definitive IND-enabling studies (Late Product Development).
- b) perform FDA requested studies to lift clinical hold of an IND.
- c) rare exceptions, based on strong merit, to overcome hurdles and increase the project's chance for further funding from other sources (Late, and possibly Early Product Development).

Fundable projects will have already identified the correct regulatory path - device, biologic (gene therapy, cell, etc.) or drug, have met early regulatory requirements, have a strategy for meeting all regulatory requirements. Strong projects will have in place appropriate Good Documentation Practices and are in transition to Good Manufacturing Practice. Fundable projects have a strong financial partner.

Fundable projects have

1. Proposed mechanism of action (i.e., relevant therapeutic activity or intended biological effect).
2. Pilot proof of concept studies (to establish the feasibility and rationale for use of your investigational product in the targeted patient population).
3. Evidence that the project IND could be submitted to FDA in two years.

THE APPLICATION

Please provide a word document or PDF in the following format.

Use Times New Roman 12 pt with 1 inch margins.

- I. *Project* – 3 pages total, including the 1-page Target Product Profile (TPP). Please use the template provided for the TPP.

UC San Diego Health

Sanford Stem Cell Clinical Center

Please describe the investigational product that would be submitted for IND approval by FDA before July 1, 2017. Also, describe any related device that would be used to deliver the product.

- Outcomes – What are your primary and secondary outcomes for your proposed Phase I clinical trial, and what are your outcome measures? What are the primary endpoints and secondary safety and tolerability endpoints?
- Locations of patient populations, cell manufacturing, and QA/QC.
- Brief summary of cell manufacturing process

II. *References cited* – 1 page

III. *Specific Workplan and Aims* – 1 page

- *Project timeline* – Please identify your preclinical development timeline using the Product Development Stages identified in the attachment. Please provide Gantt chart. Specify Go/no go milestones.

You must specify:

- Date or proposed date to conclude animal studies
- Date or proposed date of pre-pre-IND meeting(s) with FDA
- Date or proposed date of pre-IND meeting with FDA
- Proposed Date of IND submission
- Proposed Date of IRB submission

IV. *Background & Preclinical Studies* – 2 pages

- Your competitive landscape – shortcomings of current therapies for your target indication, and other work in progress by academics or industry.
- Summary of preclinical studies to date. How do your proof-of-concept studies support translation to a human trial?
- Response to questions specific to your proposal

V. *Biosketches* – Please provide 2-page NIH-style biosketches for the PI and proposed team members.

VI. *Total Budget* – Please provide a total budget for your project, using the budget template provided. Please identify the total cost of the project, recognizing that the Sanford Center intends to provide between \$1M-\$2M of your total budget. Please provide any evidence you have of partnerships that may provide the necessary funding for the clinical trial itself.

DEADLINE: May 1, 2015

Please send complete application materials to Jennifer Braswell, PhD at jbraswell@ucsd.edu and cc Cynthia Kuan at c1kuan@ucsd.edu.