

Empire State Stem Cell Board
Full Board Meeting Minutes
October 31, 2014

The Empire State Stem Cell Board (ESSCB) held a full board meeting on Friday, October 31, 2014, at the offices of the Department of Health, 90 Church Street, New York, New York. Janet Cohn presided as Chair Designee.

Funding Committee Members Present:

Ms. Janet Cohn, Chair Designee
Mr. Robin Elliott
Dr. David Hohn*
Ms. Fonda Kubiak
Dr. Donald Landry
Dr. Norma Nowak
Dr. Allen Spiegel, Vice Chair
Dr. Melissa Wasserstein
Ms. Madelyn Wils
*participated via video-conference

Funding Committee Members Absent:

Dr. Bradford Berk

Ethics Committee Members Present:

Ms. Janet Cohn, Chair Designee
Dr. Richard Dees
Dr. Inmaculada de Melo-Martin
Dr. Samuel Gorovitz
Dr. Robert Klitzman
Dr. Donald Landry
Rev. Hugh Maynard-Reid
Dr. Allen Spiegel, Vice Chair
Dr. Camille Wicher

Ethics Committee Members Absent:

Ms. Jann Armantrout
Ms. Nancy Dubler

Department of Health Staff Present:

Ms. Bonnie Brautigam
Dr. Kathy Chou
Dr. Matthew Kohn

Guests Present:

Dr. Audrey Chapman

Dr. Mahendra Rao

Dr. Michelle Cissell

Welcome and Introductions

Ms. Cohn called the meeting to order, welcomed board members and the public, and introduced four new members: Dr. Richard Dees of the University of Rochester, who would serve on the Ethics Committee; Ms. Fonda Kubiak of the Federal Public Defender's Office for the Western District of New York, for the Funding Committee; Dr. Donald Landry of New York Presbyterian Hospital, who would serve on both committees; and Dr. Norma Nowak of the University at Buffalo, for the Funding Committee. She also announced that this would be Dr. Inmaculada de Melo-Martin's last meeting, and expressed the regret of the board and staff that she would be unable to remain because of her work on a NYSTEM-funded Consortium. Ms. Cohn then asked board members and staff to introduce themselves.

Approval of Minutes of May 21, 2013 Ethics Committee and Full Board Meetings

Ms. Cohn directed Ethics Committee members to the draft minutes of their last meeting on May 21, 2014, and asked for a motion to approve them. Dr. Klitzman so moved and Rev. Maynard-Reid seconded. The motion passed.

Mr. Elliott moved to approve the minutes of the full board meeting held on the same date, a member seconded, and the motion passed unanimously.

Motion to Amend Board Bylaws

Ms. Cohn introduced a proposal to amend the board's bylaws to modify the quorum provisions. She pointed out that although New York State General Construction Law § 41 requires that a quorum for a public board consist of a majority of *authorized* members (i.e., the total number provided for by statute) without regard to vacancies, the Empire State Stem Cell Board legislation, at § 265-a(3), permits the ESSCB to adopt its own quorum rules, "notwithstanding any other provision of law...." Although there are no applicable restrictions on quorum size, Ms. Cohn recommended a modest change, to a majority of currently *appointed* members, in order to maintain the confidence of the public in the board's actions.

Reverend Maynard-Reid moved to approve the proposed amendment, Mr. Elliott seconded, and the motion passed unanimously.

**Presentation by Dr. Audrey Chapman:
Issues Related to Beginning Clinical Trials with Pluripotent Stem Cells**

Ms. Cohn introduced Dr. Audrey Chapman, who holds the Healy Memorial Chair in Medical Ethics and Humanities at the University of Connecticut Health Center, and told members that she has written extensively on ethical and policy issues related to embryonic stem cell research and early clinical trials of pluripotent stem cells.

Dr. Chapman discussed the need for comprehensive guidelines for trials of pluripotent stem cell-based therapeutics, which she considers essential given the current pressure on researchers to develop treatments.

Dr. Chapman identified issues of concern including the lack of suitable animal models, reliance on rodents, the enhanced and prolonged risks of biologics compared to small molecule pharmaceuticals, the tendency for pluripotent cell-based therapies to form tumors, unanswered questions about how long patients should be tracked, all compounded by the fact that Institutional Review Boards often lack the necessary expertise to oversee these issues.

Dr. Chapman raised questions about two of the three clinical trials to date involving pluripotent cells, beginning with the Geron test of a therapy for spinal cord injuries. Dr. Chapman noted that the protocol required that subjects be treated within two weeks of injury and questioned whether anyone was capable of giving informed consent in those circumstances. Dr. Rao countered that two weeks was the earliest that patients could be recruited but that they could go up to one or two years following injury. Dr. Chapman also voiced concern about tumors at the site of injection but Dr. Rao quickly pointed out that they were non-malignant neurocysts.

Further discussion was held about the benefits of non-human primate models compared to rodent models. Dr. Rao suggested that it is not clear, as a general rule, that primates mimic human responses better than rodents do. Furthermore, where size is an issue, as in therapies for knee joints or spinal cords, some work is required to be done on larger animals, but not necessarily the whole study.

After debating the issue, several members of the board, joined by Dr. Rao, urged the group to focus on issues of trial design that are truly unique to pluripotent stem cell therapy. They agreed with Dr. Chapman that many of the issues she raised were of great importance but not unique to pluripotent stem cells, and that the need to provide guidelines specifically for stem cell trials was not established.

The board thanked Dr. Chapman for her excellent presentation.

Presentation and Discussion: Strategic Planning

Ms. Cohn formally introduced Dr. Rao, chair of the Strategic Plan workgroup, and Dr. Michelle Cissell, the author of the board's first Strategic Plan, who would also be drafting the second one.

Dr. Rao described the process of preparing the second five-year plan as part of an effort to create a strategy to extend the program for another term, which he described as a plan for building on the program's success to date. The first part would be to explain what NYSTEM and the board have achieved with the money received so far, and evaluate how well the results have met the goals of the first Strategic Plan. In order to help answer this question, a Scientific Advisory Panel was formed, comprised of the heads of the state's stem cell institutes, to assess the program with respect to its three major components: infrastructure; developing scientific expertise and promoting research; and outreach, education and ethical support.

Dr. Rao explained that a panel of independent experts had been assembled to evaluate the program. These were Dr. Story Landis, until that month Director of the National Institute of Neurological Disorders and Stroke at the National Institutes of Health (NIH) and Chair of the NIH Stem Cell Task Force; Dr. David Scadden, Co-director of the Harvard Stem Cell Institute and Chief of the Center for Regenerative Medicine at the Massachusetts General Hospital; Dr. John Kessler, former Director of the Stem Cell Institute at Northwestern University and Chair of the Department of Neurology; and Dr. Sean Morrison, Director of the Children's Medical Center Research Institute at the University of Texas Southwestern Medical Center and President-elect of the International Society for Stem Cell Research (ISSCR).

Mr. Elliott identified several momentous decisions that had been made early on that he requested be conveyed to the External Review Panel. The first was that NYSTEM money be permitted to go to out-of-state entities, so long as the primary applicant/contractor was a New York State institution. Second was the decision against financing the program through a bond issue, which many had believed was the only hope for funding. The third was the decision that NYSTEM money could go to commercial institutions, so long as it was through a subcontract through a New York contractor. The fourth was a decision not to finance real estate, as California had. Also of importance was the decision to engage an independent contractor to oversee peer review.

Dr. Rao noted that the Scientific Advisory Panel had considered whether, going forward, it made sense to maintain NYSTEM as a separate, distinct program or whether it would benefit from being a part of a broader effort. He reported that a spirited debate was held in which members acknowledged the benefits of both approaches. The strong consensus, however, was to keep NYSTEM a self-contained program.

Dr. Landry questioned whether the addition of experts on the External Review Panel who were not in the stem cell field might not have been wise, particularly in assessing whether to expand NYSTEM to include other areas. Mr. Elliott responded that an equally strong approach

was to view NYSTEM as a model for state funding programs, one that perhaps could be expanded later and certainly followed in other states now.

Dr. Rao added that the Scientific Advisory Panel strongly espoused the view that the current structure has offered a new paradigm for therapy. The Panel further noted that the program should not be seen as limited to stem cell research alone, or to somatic cell nuclear transfer, or embryonic stem cells, but rather as a broad-based way to enhance the capability of New York's great medical institutions in cell-based therapy, with the focus on diseases that cannot be treated in any other way.

Dr. Landry agreed with that characterization, as "stem cells broadly conceived." Dr. Chou pointed out that NYSTEM includes many related fields and that breadth of approach is actually encouraged. For example, the program funds projects that include tissue engineering, biomaterials, bioinformatics and immunology.

Next, Dr. Klitzman briefly listed some of the possible issues for the future focus of the Ethics Committee. He mentioned guidelines for clinical trials, biobanks, stem cell tourism, commercialization and compensation.

Dr. Rao added the issue of "conditional marketing," which was recently adopted in Japan, whereby insurance will be required to reimburse for the use of a therapy that has been approved only for safety, for a period of up to seven years after that limited approval.

Dr. Klitzman then raised the idea of partnering with biotech companies and asked whether the field was ready. Dr. Nowak told members about some of the innovative programs that had come to Buffalo, such as Governor Cuomo's Start-up New York and the Billion for Buffalo programs. She asked whether NYSTEM had ever funded projects similar to the NIH's Small Business Innovation Research (SBIR) and Small Business Technology Transfer (STTR) programs, where an academic group teams up with a company. Ms. Brautigam noted that it would be able to do that in the program's current structure so long as the applicant was a New York State not-for-profit.

Program Updates

Ms. Brautigam provided program updates, noting that the awards approved that day would bring the total of committed funds up to approximately \$346 million.

Motion to Adjourn Full Board Meeting

Dr. Landry moved to adjourn the meeting and Dr. Gorovitz seconded. All members voted affirmatively.

*s/ Janet Cohn
Executive Secretary to the
Empire State Stem Cell Board
Approved: April 28, 2015*

**Empire State Stem Cell Board
Funding Committee Meeting Minutes
October 31, 2014**

The Funding Committee of the Empire State Stem Cell Board held a meeting on Friday, October 31, 2014, at the offices of the Department of Health, 90 Church Street, New York, New York. Janet Cohn presided as Chair Designee.

Funding Committee Members Present:

Ms. Janet Cohn, Chair Designee
Mr. Robin Elliott
Dr. David Hohn*
Ms. Fonda Kubiak
Dr. Donald Landry
Dr. Norma Nowak
Dr. Allen Spiegel, Vice Chair
Dr. Melissa Wasserstein
Dr. Madelyn Wils

Funding Committee Members Absent:

Dr. Bradford Berk

Ethics Committee Members Present:

Ms. Janet Cohn, Chair Designee
Dr. Richard Dees
Dr. Inmaculada de Melo-Martin
Dr. Samuel Gorovitz
Dr. Robert Klitzman
Dr. Donald Landry
Rev. Hugh Maynard-Reid
Dr. Allen Spiegel, Vice Chair
Dr. Camille Wicher

Ethics Committee Members Absent:

Ms. Jann Armantrout
Ms. Nancy Dubler

Department of Health Staff Present:

Ms. Bonnie Brautigam
Dr. Kathy Chou
Dr. Matthew Kohn

*participated via video-conference

Welcome and Introductions

Ms. Cohn called the meeting to order and welcomed members, staff and the public to the meeting of the Funding Committee and invited Ethics Committee members to participate. She then asked board members and staff to introduce themselves.

Approval of Minutes of February 9, 2014, Funding Committee Meeting

Ms. Cohn directed Funding Committee members to the draft minutes of their February 13, 2014, meeting and asked for a motion to approve them. Before the vote took place, Dr. Chou observed that there were important updates to the topic she had discussed at the February meeting, namely, that the new method she had described for creating induced pluripotent stem cells had been challenged, and the papers describing it had been retracted.

Dr. Wasserstein moved to approve the minutes of the February 13, 2014, meeting and Dr. Spiegel seconded. The motion passed.

Executive Session

Ms. Cohn asked committee members for a motion to go to executive session to discuss the evaluations of applications submitted in response to three Requests for Applications (RFAs): Consortia to Accelerate Therapeutic Applications of Stem Cells, Round 2; Stem Cell Research Experience for Pre-College Teachers; and Informal Stem Cell Science Education Programs (Museums). Ms. Kubiak so moved and Dr. Wasserstein seconded. The motion passed unanimously.

Ms. Brautigam reminded members of the evaluation criteria set forth in each RFA and described the peer review evaluation and scoring processes. The Committee was then provided with information relating to individual applications while members with a potential conflict of interest left the room.

During the discussion of the Consortia applications, Funding Committee members reaffirmed the high priority they place on the Consortia-type projects, as well as their duty to protect state funds. The Committee called on NYSTEM staff for assurance that any awarded Consortium's failure to meet early milestones would result in that award's cancellation, and that any funds made available as a result be reallocated to other similar translational and clinical mechanisms, subject to future Funding Committee agreement.

Ms. Cohn then asked for a motion to adjourn the executive session and reconvene in public. Dr. Wasserstein so moved and Ms. Kubiak seconded the motion. The motion passed.

Public Session

Recommendations for Stem Cell Research for Pre-College Teachers

Ms. Cohn advised the Committee that the next order of business would be to vote on applications for Stem Cell Research for Pre-College Teachers. Proceeding in score order, Ms. Cohn announced the application number, the name of the sponsoring institution and the recommended funding amount for each application.

Dr. Landry recused himself from consideration of the first application and left the room. Dr. Klitzman, a member of the Ethics Committee, left the room as well.

Mr. Elliott then moved to recommend approval of Application Number N14T-007, in the amount of \$672,408, from Columbia University. Dr. Spiegel seconded the motion. The motion passed.

The second application was Number N14T-004 from Cornell University in the amount of \$756,000. There were no recusals. Ms. Kubiak moved to recommend approval of the award and Dr. Spiegel seconded. The motion passed.

The Committee then considered the third application, Number N14T-003, in the amount of \$498,420, from Rensselaer Polytechnic Institute. There were no recusals. Mr. Elliott moved to recommend approval. Dr. Nowak seconded the motion. The motion passed.

Recommendations for Informal Stem Cells Science Education Programs

Ms. Cohn asked members to consider the single application received for funding under this RFA, Number N14M-001 from the American Museum of Natural History, with the New York Stem Cell Foundation as sub-applicant. Dr. Wasserstein moved to approve the application in the recommended amount of \$250,000, Mr. Elliott seconded, and it passed unanimously.

Proposal to Amend the Graphic Novel Request for Proposals (RFP)

Ms. Brautigam explained to the Funding Committee that staff had concluded that the RFP as currently drafted would be unworkable. She explained that they had determined that 1,111 schools in the state of New York would be eligible to participate, potentially overwhelming both the ultimate contractor selected and NYSYSTEM staff. She suggested that the school contest component of the project be eliminated and that the contract be limited to creating and disseminating the single graphic novel and teaching guide.

Several board members, including Dr. Gorovitz, Dr. Hohn, Mr. Elliott and Dr. Klitzman, voiced concern about limiting the RFP in this way. They reminded staff and fellow board

members that the initial intent had been to energize and engage the students, something that simply reading the work of the professional contractor might not sufficiently achieve.

Ms. Cohn asked for a motion to amend the plan for the proposal, noting that if it failed to carry the Committee could go on to discuss alternatives. Dr. Landry so moved and Dr. Wasserstein seconded. There were too few votes on either side to constitute an affirmative action of the Committee, however, and the motion was tabled.

Ms. Brautigam summed up the Committee's discussion, saying that staff would develop one or more alternatives and bring it back to the Funding Committee. Dr. Hohn expressed his support for the concept and suggested that any expertise lacking currently be solicited so that the original idea could go forward. Ms. Cohn agreed to work with staff and interested board members to develop further ideas and present them in the future.

Recommendations for Consortia to Accelerate Therapeutic Applications of Stem Cells, Round Two

Ms. Brautigam reminded members that this was the second round of these 4-year milestone-based awards, designed to accelerate development of clinical treatments. She told the members that eight applications had been received and forwarded to the program's external peer review contractor, which convened an expert panel in August 2014. She noted that the Committee had set the cutoff for applications it would consider at a score of 3.9, resulting in five applications to review today.

Ms. Cohn told the board that \$32 million had been set aside to support two awards. Proceeding in score order the first application was N14C-010 from Weill Cornell Medical College. There were no recusals.

Dr. Hohn moved to recommend its approval in the amount recommended, which was \$15,717,575. Dr. Spiegel seconded. The motion passed unanimously.

In response to a request from Mr. Elliott, Ms. Cohn summarized for the record the discussion held in Executive Session. She stated that the board had expressed its strong desire that there be diligent and appropriate oversight to ensure that each Consortium meet its milestones; and that in the event of a failure to do so, the contract be promptly cancelled and the money be redirected to a new RFA for additional consortia or other translational projects.

Ms. Cohn noted that Dr. Hohn and Dr. Nowak had recused themselves from consideration of the next application and asked them to leave the room. Dr. Wicher from the Ethics Committee stepped out as well. Ms. Cohn then asked for a motion to approve the second application, N14C-002 from Roswell Park Cancer Institute, in the amount of \$11,922,885. Ms. Kubiak moved and Dr. Spiegel seconded. The motion passed.

After the recused members returned, Ms. Brautigam noted that the two Consortia recommended so far totaled \$27,640,460, leaving \$4,359,540 uncommitted. Ms. Cohn stated that the next application would require the uncommitted balance plus approximately \$4.5 million to fund. Anticipating a possible request from the Funding Committee, staff had taken the rare step of requesting advance approval for the additional amount. Because that request was pending, she asked for a motion to approve contingently the next application, N14C-003 from the Icahn School of Medicine at Mount Sinai. Dr. Wasserstein had recused herself and she left the room.

Ms. Kubiak moved to approve the application and Dr. Spiegel seconded the motion. It passed unanimously.

Mr. Elliott asked whether the Funding Committee could consider the remaining applications as approved but not funded to take the place of any of the three that were ended early should they fail to meet milestones. Ms. Cohn clarified that a new RFA would be required in that circumstance.

Ms. Cohn next asked for a motion to raise the total funding for the Consortia RFA to \$36.5 million, in the event that approval to do so is given. Dr. Wasserstein so moved and Ms. Kubiak seconded. The motion passed.

Proposal to Fund Previously Awarded Investigator Initiated Research Projects (IIRPs) and Innovative, Developmental or Exploratory Activities (IDEAs)

Ms. Brautigam explained that with the fiscal year coming to a close there was an opportunity to fund seven applications that had been approved but not funded in the fourth round of IIRPs and IDEAs, for a total of \$7.3 million. Again, staff was waiting for approval on its request, but in the interest of expedience she requested the Funding Committee's approval at this time. Ms. Cohn asked for a motion to do so, Dr. Spiegel so moved, and Dr. Nowak seconded, and the motion passed.

Approval to Raise Total Funding on Fifth Round Request for Applications for IDEAs and IIRPs

Ms. Brautigam told members that based on the number and quality of applications for IIRPs and IDEAs received in the last round, staff was requesting that the total funding be raised from \$25 million to \$35 million. Ms. Cohn asked for a motion, reminding members that \$35 million had been the original amount set aside for these RFAs. Dr. Spiegel so moved, Ms. Kubiak seconded, and the motion passed.

Motion to Adjourn Funding Committee Meeting

Mr. Elliott moved to adjourn the meeting. All Funding Committee members voted affirmatively.

*s/ Janet Cohn
Executive Secretary to the
Empire State Stem Cell Board
Approved: January 30, 2015*