Maryland Stem Cell Research (MSCR) Commission Meeting (June 04, 2009)
Public Presentation: Madhusudan V. Peshwa, Ph.D.

Good Afternoon, Madam Chairperson and Commission Members

Thank you for this opportunity to present at the public hearing of the Commission. I am here today to request the Commission to, and outline specific measures on how the Commission can, leverage the State Stem Cell Fund to catalyze early-stage translational development of tangible products and cures arising from innovative stem cell research.

The historical track record of the Fund provides evidence that Maryland is definitely supporting novel, innovative, cutting-edge research in the field of stem cells. The challenge that this leads us to, are to ask the questions:

- What do we need to do next in order to capitalize on this strength, and to encourage further investigations to validate the research outcomes in the context of meaningful applications?
- How do we stimulate investment and development of tangible product and cures based on such innovative hypothesis-driven stem cell research?

There is a clear and present need to provide vision, leadership and targeted focus to catalyze and accelerate further private and commercial efforts, in the state of Maryland, to develop effective products and cures that are uniquely enabled by innovative stem cell research. The key actions, which I will elaborate on further during my presentation, to support this need require:

1. Establishment of a separate Request for Applications (RFA), of a scope similar to the current Investigator-Initiated Grants, that focuses on early-stage development of products and cures based on innovative hypothesis-driven stem cell research
2. Establishment of criterion for objective evaluation of proposals focused on early-stage development of products and cures that specifically address the questions:
    - Does the technical work plan lead to effective proof-of-concept; in other words: is the biology validated, so as to not just be a hypothesis
    - Is there a meaningful path to commercialization defined; including articulation of the specific need, developmental path and timeline, and an initial outline of business and regulatory considerations.

I stand before you today as a scientifically trained business entrepreneur with 20+ year experiences and track record of development of cell therapy and stem cell products. My experiences include leading the development

and manufacture of 8+ cell therapy products in 15+ IND applications through various stages of pre-clinical and clinical development, both in North America and in Asia. The highlights of which are

- development from concept through U.S. FDA approval of a Medical Device for stem cell isolation,
- development from concept through completion of two phase III studies and BLA filing for an autologous cancer vaccine in U.S., and
- development and technology transfer leading to market launch of an autologous oncology therapeutic service in Japan.

Immediately prior to relocating to Maryland in 2005, I participated in birthing a company focused on development of therapies for neurodegenerative diseases employing adult stem cells, which were re-programmed using small molecule compounds. I present you with these credentials as background supporting my qualification to present.

What I want to share with you today is partly driven by my experiences and passion for taking novel research concepts and translating them into tangible products or cures, and partly from the perspective of stimulating and challenging the Commission to set for itself as a goal: To identify and articulate solutions, that will allow the state of Maryland to build a leadership position in not only innovative stem cell research but in catalyzing the early-stage translation of such research efforts down the path toward development of effective products or cures, that demonstrate societal benefits or lead to improvement in human health.

The task of early-stage translational product development is often, not simply innovative research. It requires as its foundation, innovative research driven validation of basic science, coupled with expertise that addresses "rubber-meets-the-road" aspects to ensure delivery of tangible products through development of safe, effective, robust and scalable manufacturing processes. Such efforts need strong collaborative partnership between innovative research and product development expertise. The former, of course, is the strength of academic investigators or stem cell researchers. The latter skill set is usually found mostly within the biotechnology industry or select large academic / clinical translational medicine facilities. Even in instances of strong collaborative program proposals under traditional RFAs, very often these do not lend themselves to being selected for funding because they are typically evaluated by research scientists, who use standard NIH criterion that are primarily geared toward identification of innovative hypothesis-driven research. Thus, funding for translational product development is not as easily available other than within the confines of individual small biotechnology companies. The small biotechnology companies are typically venture backed and resource constrained to the extent that often they are unable to effectively support their primary product programs, let alone take on the additional challenges of investing in early-stage translational development of higher risk novel stem cell research concepts to validate them through the proof-of-concept stage. The current economic environment further works to alleviate this challenge, as

venture funding is being increasing restricted to solely later stage products wherein there has been higher level of risk reduction, and thus making it even more difficult to attract monies for purposes of early-stage translational development. This in essence creates a clear and present need for programs, such as that of this Commission, to take leadership in supporting early-stage translational efforts by creating specific and targeted programs that will stimulate the development of products and cures based on innovative stem cell research.

I would therefore request the Commission to take upon itself the mandate to discuss how within the scope of its charter, the Commission can demonstrate creativity and leadership to identify and institute program(s), with associated relevant and objective evaluation metrics thereof, that will promote strengthened industry-academia partnerships to take on the task of moving beyond innovative stem cell research to demonstrated validation through 'proof-of-concept' stage of the applicability of such innovative research in leading to the development of tangible products that accelerate the drive toward cure of diseases that can only be uniquely addressed by stem cell research.

I believe, that one approach to providing such a solution is what I had outlined at the start of my presentation:

1. To establish a separate RFA, a separate Request for Applications (RFA), of a scope similar to the current Investigator-Initiated Grants, that focuses on early-stage development of products and cures, and
2. To evaluate proposals submitted in response to his RFA using relevant and objective criterion that assess:
    - Does the technical work plan lead to effective proof-of-concept; in other words: is the biology validated, so as to not just be a hypothesis
    - Is there a meaningful path to commercialization defined; including articulation of the specific need, developmental path and timeline, and an initial outline of business and regulatory considerations.

I appreciate the opportunity to present in front of the Commission. Thank you.

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