

**From:** [REDACTED] >

**To:** Jeffrey Epstein <jeevacation@gmail.com>

**Subject:** Fwd: Neuroscience Proposal

**Date:** Tue, 27 Aug 2013 02:12:41 +0000

**Attachments:** LeoneP.CV.8-2013.pdf

**Inline-Images:** signature[2].png

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----- Forwarded message -----

**From:** Leone, Paola <[REDACTED]>

**Date:** Fri, Aug 23, 2013 at 2:21 PM

**Subject:** Neuroscience Proposal

**To:** "[jeffrey@jeffreypstein.org](mailto:jeffrey@jeffreypstein.org)" <[jeffrey@jeffreypstein.org](mailto:jeffrey@jeffreypstein.org)>

Dear Mr. Epstein,

I am an Associate Professor of Cell Biology at Rowan University-School of Osteopathic Medicine, where I also direct the Cell & Gene Therapy Center. I am an internationally recognized pioneer in the field of gene therapy for rare neurogenetic diseases, and spearheaded the first clinical viral gene therapy application for a neurologic disease.

Your Foundation mission focusing on igniting groundbreaking ideas in the neurosciences compelled me to contact you. As a visionary science investor in innovative projects ranging from Cancer, Evolutionary Biology and Late-onset Alzheimer's Disease, you may be interested in evaluating the importance of my current research that holds considerable potential for the actual therapeutic development of Age-related Dementias. The World Health Organization's and Alzheimer's Disease International's report on Dementia (2012) recognizes that the global burden of Alzheimer Disease (AD) is forecast to worsen significantly with prevalence predicted to double every 20 years. Interventions that could provide a delay in progression or improvement would provide a significant reduction in health care burden and cost associated with late or end stage of Alzheimer's and other dementia. Recent well-publicized reports of the failure of immunotherapeutic agents in Phase-III clinical trials have highlighted the inadequacy of the "*Amyloid Cascade Hypothesis for Alzheimer's Disease*" and have prompted further discussion about current foci for novel clinical application. My current research represents a significant departure from current dogma and may have broad applicability across the neurodegenerative spectrum.

I have been a passionate and tireless advocate of Canavan Disease research, dedicating most of the past 15 years of research to develop efficacious and beneficial therapeutic approaches for children afflicted by Canavan Disease, which is a pediatric neurogenetic disease, aggressively progressive, severely debilitating and uniformly fatal. This dedication has resulted in one prominent clinical gene therapy trial, with concurrent potential therapies involving stem cells and dietary supplementation currently being pursued. The Cell and Gene Therapy Center (CGTC) combines clinical and basic research. The CGTC serves as liaison to a large and growing international population of Canavan

patients. The focus of the CGTC's clinical work is to provide state-of-the-art clinical support to patients as well as prenatal screening for rare mutations. Patients undergo neuroradiologic/neurometabolic assessments at CHOP (PA) and recommendations for therapies are currently conducted in collaboration with neurologists at NYU (NY).

My basic/clinical research for Canavan Disease has been funded by the NIH as well as charitable Foundations. Jacob's Cure, NY, stands out as one of the most successful fundraiser and supporter of my Canavan research and the sponsorship of Jacob's Cure donors and board of directors has been well-recognized and visibly honored (<http://jacobscure.org/events.php>).

My current project on Alzheimer's Disease (AD) consists of a novel genetic therapeutic approach that in our proof-of-concept studies has shown to significantly reduce  $\beta$ -amyloid plaques, promote neuronal survival and rescue the cognitive phenotype of a well-characterized AD transgenic animal model, carrying 5 familial mutations associated with human early-onset AD (5XFAD Mouse). I have submitted four grant applications during the past 12 months on the basis of this promising preliminary data, but the project remains currently unfunded. The filing of a PCT patent in support of this intervention solicited some corporate interest that also is yet to be materialized.

I serve as scientific advisor in several Foundations and grant review panels and I have first hand experience in review mechanisms that prioritize innovative ideas that, in part, have been already published. Hence, revolutionary ideas proposed in grant applications are scored poorly in the absence of published data. Therefore, at this stage of preclinical development, I am actively seeking the interest of a creative and daring innovator such as yourself to support crucial interim funding required to complete the preclinical proof-of-principle data required for a scholarly publication and future grant funding.

Last but not least, I would like to highly commend you for choosing to fund compassionate and remarkable projects such as raising awareness on HIV treatments in Africa while also promoting charter schools in NY as well as heralding the first college run radio station in the US Virgin Islands and continuing your amazing support of the Pets and Wings Program which is highly regarded by many pet lovers, like myself.

As a scientific advisory board and grant review panel member I am a strong advocate against the unjustified use of large animals in research and although I am not alone, as the NIH and FDA members are restricting funding in support of large animals in biomedical research, I remain a minority in my field and I plan not to cease the challenge. I am confident that you, as you evidently are a compassionate individual and strive against global injustice, would support the same philosophy.

I hope you are interested in acquainting yourself with the promising AD research that I described above.

I would be delighted to send you more detailed information and present the project to you and/or your Foundation Assistants upon request.

I have enclosed my curriculum vitae.

Thank you in advance for your time and consideration.

I look forward to hearing from you.

Respectfully,

A handwritten signature in black ink, appearing to read "Paola Leone".

Paola Leone, Ph.D.  
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Director of Cell and Gene Therapy Center  
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