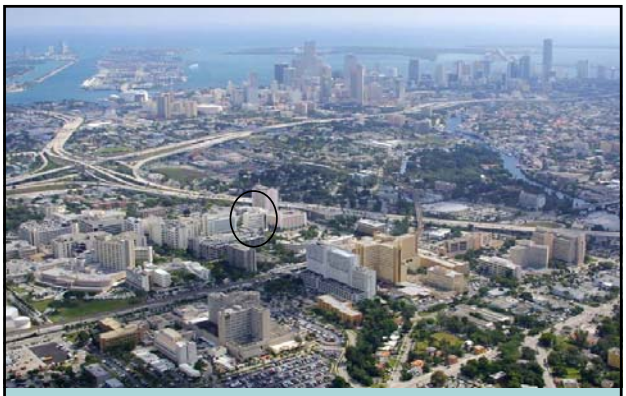
2014
Research Program Update

Marc A. Buoniconti
President
The Miami Project to Cure Paralysis
The Buoniconti Fund

MILLER SCHOOL OF MEDICINE UNIVERSITY OF MIAMI



MILLER | SCHOOL OF MEDICINE UNIVERSITY OF MIAMI

Most Exciting / Productive Year

This has been the most exciting and productive year in the history of The Miami Project with increased extramural grant funding combined with the successful translation of several of our discoveries targeting brain and spinal cord injury patients.

MILLER SCHOOL OF MEDICINE UNIVERSITY OF MIAMI

Scientists and Clinicians

Our educational and mentoring programs continue to help train the next generation of scientists and clinicians in the area of Neuroscience. Our faculty have national prominence in the area of CNS injury and Repair research and serve on numerous national and international scientific boards as well as NIH, DoD, VA and Foundation funding panels.

MILLER SCHOOL OF MEDICINE UNIVERSITY OF MIAMI

2014

Accomplishments and Highlights

MILLER SCHOOL OF MEDICINE UNIVERSITY OF MIAMI

FDA Approval
Sub-acute / Autologous Schwann Cells

We have now transplanted 4 sub-acute spinal cord injured subjects with autologous Schwann cells. We received FDA approval for this Phase I clinical trial that will test the safety of this unique cellular transplantation procedure. This is a major accomplishment for The Miami Project since it represents the first academic institution that has received FDA approval for transplanting cells into spinal cord injured people.

Chronically Injured Autologous Schwann Cells

Last month we received approval from the FDA to transplant autologous Schwann cells into chronically injured subjects. Preclinical data from our rodent and pig SCI studies supports safety and efficiency.

Peripheral Nerve Injury

We are using Schwann cells to target peripheral nerve injury to promote regeneration. This experimental bridging approach was combined with nerve grafts targeting return of leg function.

Deep Brain Stimulation (DBS)

Deep brain stimulation (DBS) is being evaluated for the first time to target neuropathic pain in spinal cord injured subjects. Our first subject was successfully treated.

Adult Mesenchymal Stem Cells

FDA approved trial in the testing of the effect of **adult mesenchymal stem cells transplantation;** the subject has been transplanted and is being monitored

New Floating Catheter

We have submitted an addendum to the FDA to use a new floating catheter for the next series of subjects. This approach is safer and more appropriate for future chronic SCI studies.

Therapeutic Hypothermia

We have initiated therapeutic hypothermia in 35 severe spinal cord injured subjects. One year outcome measures indicate that a 45% conversion from complete to incomplete paralysis which is thus far the best hope for treating early SCI. We have submitted a multi-center trial proposal to NIH to continue these studies.

Therapeutic Hypothermia
TBI Patients

Therapeutic hypothermia is being utilized in severe TBI patients. We have initiated a three site multi-center trial (Miami, Houston, and Pittsburgh) to test the efficacy of therapeutic hypothermia in this patient population (HOPES Trial).

Boot Camp

The Miami Project has initiated a new Boot Camp for chronically injured spinal cord injured patients. We intend to use a very advanced conditioning/rehabilitation strategy to improve the quality of life of people living with paralysis and combine this Boot Camp rehabilitation program with transplantation of Schwann cells in the chronic setting.

Miami Project **Boot Camp**
Preparing People with Chronic SCI for Cellular Therapies

Upper Limb Conditioning

Motor Rehabilitation

Endurance

Balance and Conditioning

Epidural Stimulation

Other combination approaches including epidural stimulation appears to maximize the beneficial effects of Schwann Cell transplantation.

Brain Computer/Interface

We are in the final stages of receiving FDA approval for our Brain/Computer interface work targeting spinal cord injured patients. This investigation has resulted from an alliance with Medtronic, Inc. which will supply state-of-the-art integrators that will allow neuronal impulses from the brain to be transmitted around the injured spinal cord to the muscles of the upper extremity.

SOF
Red Light Camera Bill

State of Florida funding continues to be another source of support for our brain and spinal cord injury studies. We will receive money from the SOF and the Red Light Camera Bill.

Discovery Science Programs

Our discovery science programs are also very successful. We are publishing in high-tier journals on neuroprotection, axonal regeneration and repair. This year, The Miami Project published over 135 peer reviewed articles in many of our leading journals.

State-of-Art Research

- We continue to train many pre-doctoral and postdoctoral fellows to conduct state-of-the-art research.
- Our faculty organizes and teaches in many undergraduate, graduate and other medical school courses.

Neurosurgery and Sylvester Cancer Center

Neurosurgery and the Sylvester Cancer Center have initiated a new Brain Tumor Laboratory in the Lois Pope LIFE Center. This brain tumor laboratory will utilize state-of-the-art cell culture and screening approaches to develop specialized therapeutic interventions targeting brain tumors.

New Rehab Center

The Chrystine E. Lynn Rehabilitation Center

for

The Miami Project to Cure Paralysis.


Thank You
LOIS POPE LIFE CENTER
LOIS POPE LIFE CENTER