MS Discovery
Multiple Sclerosis National Research Institute
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# Clemencia Pinilla, Ph.D. continues the search for new compounds to fight MS


*Clemencia Pinilla, Ph.D.*

***"Be who you are and say what you feel, because those who mind don't matter, and those who matter don't mind."*** ***Dr. Seuss***

As a young postdoctoral research student coming from her native Colombia in 1985, Clemencia Pinilla's reception in the United States was a chilly one.

"I had come to Boston to do some work on malaria proteins, and coming from a tropical country, I had never seen snow," Pinilla remembers. "One day I walked outside with wet hair and when I got to the train station, I had icicles in my hair. I remember asking myself, 'What's going on?'"

It was then that she decided she couldn't take the harsh New England winters, and began focusing on making a move to the west coast. Her decision to head west proved to be the right one, not only professionally, but personally as well. She met, fell in love with, and married a fellow scientist, and settled down in beautiful Southern California.

Boston's loss has been the Multiple Sclerosis National Research Institute's gain. Dr. Pinilla has carved out quite a career at MSNRI with her work on synthetic combinatorial libraries and most recently with the study of the commercial drug Copaxone®, a successful treatment for Multiple Sclerosis (MS), during the past three years. This research revolves around understanding how the drug works and by coming up with compounds that are just as good or better than this current MS treatment.

"It's not completely clear how Copaxone® works," Pinilla said about the fastest growing MS treatment. "We are trying to better understand it, and at the same time trying to see if we can identify new compounds that are better than Copaxone®, or that can be better characterized."

MSNRI is currently evaluating 18 different compounds using healthy donors from the local San Diego Blood Bank, and Pinilla's longtime collaborator Dr. Roland Martin of the National Institute of Neurological Disorders and Stroke (a U.S. Government funded medical research group), is carrying out the same testing with MS patients. The hope is that maybe one or more of these compounds will be a better alternative to Copaxone® or can be better evaluated than today's current drug.

***"There's always the hope that the work you're doing will develop into something that will help someone."***

It was her work with peptides (small protein chains) that started Pinilla on her long journey to California from the Instituto de Immunologia Hospital San Juan de Dios, where she worked in microbiology until 1986. The journey began in 1985, when Dr. Pinilla met Dr. Richard Houghten, President of MSNRI, at a research symposium in Colombia.

"The director of the Institute where I worked at the time was the organizer of that meeting and there were 15 to 25 international scientists attending," Pinilla said. "At the time I had been working on malaria and tuberculosis vaccines and identifying proteins."

From that meeting, Houghten offered Pinilla a postdoctoral position in his laboratory at The Scripps Research Institute in San Diego. Pinilla accepted and packed her bags for the United States.

In 1988, Houghten founded a new not-for-profit research institute, called Torrey Pines Institute for Molecular Studies (TPIMS). Houghten and Pinilla left Scripps, and took some of the best and brightest young scientists with them to TPIMS. Within a few years, a portion of the research at TPIMS focused toward MS, and those MS research groups

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## Dr. Clemencia Pinilla

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*Dr. Eva Borras and Dr. Pinilla*

took on the name of the Multiple Sclerosis National Research Institute, to further differentiate their research focus from the rest of the research being conducted at TPIMS.

But it was the early days at Scripps that shaped Pinilla's personal life as she knows it. She eventually met someone that played an important role in her future, fellow researcher, Jon Appel.

"Jon had been hired at Scripps by Richard two weeks before I had arrived," Pinilla said. "We shared benches at Scripps and we were the best of friends."

The two were married in the early part of 1990. They now have eight year-old twin girls, Amanda and Rebeca, and live in Cardiff-by-the-Sea, a community a few miles north of San Diego. The family enjoys outdoor activities like hiking, biking and camping. Annual trips to the Sierra Nevada mountains as well as camping on the beach in San Felipe, Mexico are high on the list of things to do each year.

The eldest of four children, Pinilla takes her own family to Colombia every other year to visit. She recently became an United States citizen.

Did she ever picture herself working and living in the U.S. when she was younger?

"I don't think you can plan those things," she reflects. "I'm a big believer that at each step in life, you are doing the best you can and you have to enjoy what you're doing. That's what I like most about being a scientist.

"There's always the hope that the work you're doing will develop into something that will help someone."❖

# New MS Study Focuses on Regrowth and Repair

A new study beginning November 1, 2000, at the Multiple Sclerosis National Research Institute will focus on repairing the damage to the central nervous system (CNS) caused by Multiple Sclerosis (MS), stroke or trauma.

Roy Riblet, Ph.D. and his research team are working on the identification of genes that regulate the development of stem cells into all neural cell types, and the identification of the mechanisms controlled by these genes to reveal potential targets for therapeutic intervention. The next step in the study will be to screen small molecule libraries for drug candidates that will direct stem cell differentiation towards particular mature cell types needed for tissue restoration.

***Dr. Riblet's study will hopefully lead to regrowth and repair, eventually leading to successful transplant therapy.***

"We want to know what makes these neural stem cells tick," Riblet said of his studies, "We want to know what direction they go in and the different pathways that they take."

MS happens when the body's immune system begins to attack the myelin sheath that protects the CNS. The sheath then becomes impaired and frayed, causing the principal nerves to be interrupted of their normal function. This causes symptoms such as blurred vision, numbness and episodes of temporary paralysis in MS patients.

*Dr. Roy Riblet and Brendan Brinkman*

By studying the developmental pathways from Embryonic Stem (ES) cells through Neural Stem (NS) cells and eventually mature neural cell types (Neurons, Astrocytes and Oligodendrocytes), part of Dr. Riblet's research will focus on the pathway of NS cells to the oligodendrocyte progenitor (OP) pathway.

"By being able to isolate and grow these populations of NS in culture, we can study certain signals they give that make them go to various pathways," Riblet said. "We then can screen the different libraries we have and see what type of effect they have on these stem cells. By trying to understand the genetics of these cells we can find candidates for intervention and novel drugs."

While many of today's MS treatments are focused on suppressing the disease and trying to prevent further damage of myelin, Dr. Riblet's study will hopefully lead to regrowth and repair, eventually leading to successful transplant therapy. Alternatively, the compounds that are being studied could also help the CNS remyelinate without the benefit of transplant. ❖

# MRAA - Medical Research Agencies of America

## Helps MSNRI Raise Funds Through Workplace Contributions

Since 1998, the Multiple Sclerosis National Research Institute (MSNRI) has been a proud member of the Medical Research Agencies of America (MRAA), a federation that assists medical research facilities in raising funds through various workplace campaigns.

Founded in 1991, the MRAA moved from its California offices in 1996 to Washington D.C., where it shares staff and office space with the Christian Service Charities and Human Service Charities of America. Over the last five years, MRAA has raised just under $26 million for its members, through the Combined Federal Campaign (CFC), state, municipal, and corporate campaigns. The MRAA also counts Dr. Richard Houghten, President of MSNRI, as a member of its Board of Directors.

> ***A disease such as MS is of great interest to many workplace donors because it touches the lives of so many people.***

"MRAA considers every member a valuable asset," said Director of Member Services, Sally Cooney. "MSNRI has produced significant results as an MRAA member. A disease such as MS is of great interest to many workplace donors because it touches the lives of so many people."

MRAA offers valuable services to its members. These services primarily focus on the following areas:

### • *Campaign Access*

MRAA assists members in gaining access to new campaigns, in states, counties, cities, and corporations.

### • *Campaign Marketing*

MRAA markets its members as well as training them to market themselves, in order to maximize each member's success.

### • *E-Philanthropy*

MRAA gives members an internet presence in addition to their individual sites. They also work with Internet companies on solutions to provide more options for donors.

MRAA also works in partnership with the US Office of Personnel Management (OPM) in helping to qualify non-profit organizations for the CFC. Since OPM requirements for inclusion are extensive and all-encompassing, MRAA has adopted these requirements for membership as well as the requirement that member charities be engaged in the conduct of funding of medical research. MRAA is also looking to the future to expand its presence to prospective donors.

A MEMBER ORGANIZATION OF
M R A A
MEDICAL RESEARCH
AGENCIES OF AMERICA

"No one can foretell the future of workplace giving," said Cooney. "Some believe that as more and more people use the Internet and work from home, workplace giving will diminish. On the other hand, the CFC's initiative in soliciting federal retirees (which is in the pilot stage) may prove that people who do not go to work are willing to continue to give through a deduction system."

In the meantime, MRAA will continue to focus on increasing member revenue in current campaigns, maximizing results in new campaigns, and pursuing viable revenue-producing programs. ❖

## *Year-End Tax Tips*

Did you know that tax laws intentionally encourage charitable giving? Because of the income tax charitable deduction, anyone who makes a gift to MSNRI by December 31, and itemizes their tax return, can reduce the sting of income taxes for 2000. Just make sure the envelope is postmarked by December 31.

If you would like to give online, just log on to our website at: www.ms-national-research.org. Just click on "Donor Info." Follow the directions given on the link and use your major credit card. Thank you for your generous support of MSNRI. *It's that easy!*

## • Other MS Resources •

### • The Multiple Sclerosis Foundation, Inc.

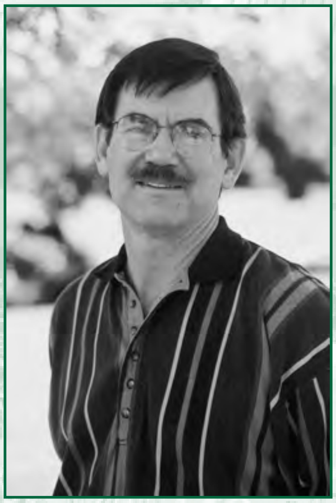
Founded in 1986, the MSF is the oldest national MS support organization that focuses on both complementary and conventional health care options. Log on to *www.msfacts.org*

### • National Institute of Neurological Disorders and Stroke

The nation's leading supporter of biomedical research on disorders of the brain and nervous system. NINDS is an institute within the National Institutes of Health, a United States federal medical research group. Log on to *www.ninds.nih.gov*

# *A Holiday Message of Hope and Gratitude from Richard A. Houghten, Ph.D.*

## *President, Multiple Sclerosis National Research Institute*



On behalf of the staff of the Multiple Sclerosis National Research Institute, I would like to wish everyone a joyous holiday season and a very prosperous New Year.

We thank you for your continued support in our efforts in finding therapeutic treatments for Multiple Sclerosis. As we progress into the millennium, we continue to develop revolutionary methods that have helped shape drug discovery worldwide. Experiments that took months or even years to carry out in the past can now be completed in as little as a single day.

We base our MS studies on the use of novel research methods called, "Combinatorial Libraries". A library is a collection of thousands to billions of potential drugs or vaccines. Studying immune function in MS patients by such methods is faster and more cost-effective compared to traditional research.

The Multiple Sclerosis National Research Institute is part of Torrey Pines Institute for Molecular Studies, a not-for-profit research center recognized internationally as a leader in the design and use of combinatorial libraries and other methods of drug discovery.

The Institute and its achievements have attracted internationally renowned scientists. Our philosophy is that scientific excellence is fostered by providing a stimulating and collaborative research environment, a philosophy that has also helped us to attract and retain our first-rate scientific staff. In the past two years, our research staff has doubled enabling us to expand our focus not only on MS, but many other diseases as well.

Our work is funded by grants from the National Institutes of Health and by support from the Combined Federal Campaign, the United Way, and other workplace campaigns. In addition, individual contributors and the Medical Research Agencies of America also fund MSNRI with generous donations.

Again, we thank you for your support and help in the battle against this crippling disease. ❖

Richard A Houghten, Ph.D.

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