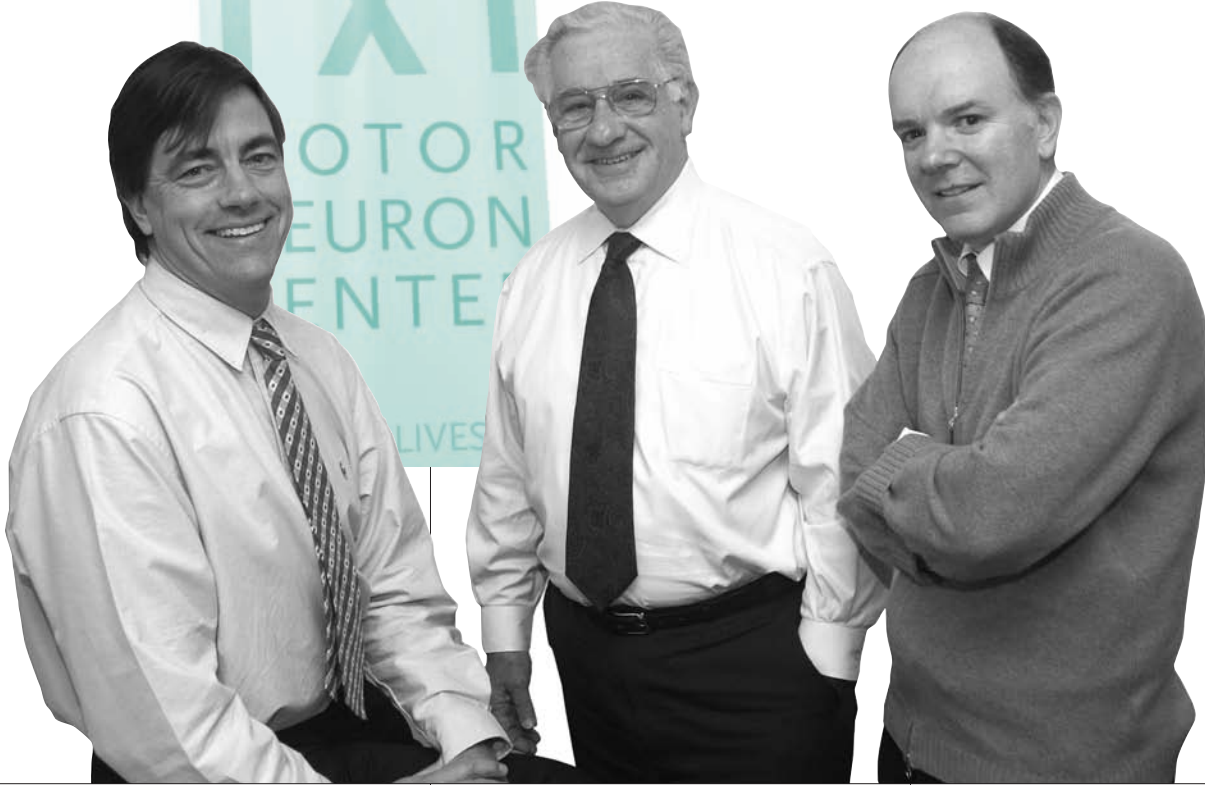


New Motor Neuron Center Will Tackle Neurodegenerative Diseases

INTERDISCIPLINARY APPROACH ADDS UNIQUE PERSPECTIVE



From left: Chris Henderson, Darryl DeVivo and Serge Przedborski, co-directors of the Motor Neuron Center.

The birth of any organization dedicated to reducing human suffering is fueled by hope and propelled by the desire to achieve extraordinary goals; this was certainly true on Nov. 2 when Columbia's Motor Neuron Center was launched.

The gathering of about 40 leading researchers and clinicians from different disciplines at CUMC and Morningside, committed to sharing knowledge, insights, and current problems, will undoubtedly have a direct impact on people who suffer from spinal muscular atrophy (SMA) and amyotrophic lateral sclerosis (ALS). Those who attended the all-day event had the opportunity for the first time to hear in depth about each other's work.

SMA is the most common genetic killer of infants and toddlers, while ALS usually strikes in middle age. Although the diseases may at first seem unrelated, in fact, they both attack motor neurons, the long nerve cells that wire the brain and spinal cord to muscles in the arms, legs, and elsewhere in the body. As the neurons degenerate, the muscles gradually weaken

and waste away. Patients progressively lose – or, in many SMA patients, never gain – the ability to walk, eat, and breathe without mechanical assistance. Both diseases are currently incurable. The NIH, however, has identified SMA as the neurological disease with the greatest potential for a treatment or cure in the near term.

In bringing the leading SMA and ALS researchers and clinicians together with other neuroscientists, new aspects of motor neuron biology will be better understood, which may result in more effective treatments for both diseases.

“Ultimately, we want to cure our patients, but to achieve this goal it is critical not only to study diseased motor neurons, but also to unravel the biology of healthy motor neurons,” says Serge Przedborski, M.D., Ph.D., professor of neurology, pathology and cell biology, who co-directs the Center with Chris Henderson, Ph.D., assistant professor, pathology, and Darryl DeVivo, M.D., the Sidney Carter Professor of Neurology.

Though motor neuron biology and disease have been studied for 150 years, there is limited knowledge about SMA and ALS. “We need to understand everything we can about the motor neuron and use this information to devise new strategies for treatment,” Dr. DeVivo says.

“It is surprising that even though the fundamental feature of ALS is the loss of motor neurons, little is actually known about why and how these neurons die,” says Dr. Henderson. “Even in SMA, which is caused by a mutation in a single gene – called survival of motor neurons, or SMN1 – there are competing hypotheses about what kills motor neurons.”

Many fundamental questions about healthy motor neurons also remain, the answers to which may have important clinical applications in the future. For example, science must identify all the factors that are required to make precursor cells become motor neurons, to guide the

growth of axons during development and to keep the neurons alive.

In addition to bringing a wide variety of researchers together to answer these questions, the Center will also provide the opportunity for clinicians to work closely with researchers. Looking forward, the Center may have its own space where clinicians and researchers will work side by side. An internal grant system will foster new collaborations and projects.

“I think the Center's design is a great model for how an academic medical center can try to develop treatments for a disease,” says Gerald Fischbach, M.D., executive vice president and dean.

Though translational research is typically thought of as a one-way street in which discoveries by basic scientists travel to clinicians, it's equally important that basic scientists learn from clinical discoveries. “There is a continuum from the patient to the scientist that we do not want to break up,” Dr. DeVivo says. Dr. Henderson says that basic scientists need to formulate their questions with the clinician's discoveries in mind, and not simply state that their research will eventually help in the search for new therapies.

“We have researchers in the Center coming from very different horizons. Many have not really thought about motor neurons before,” Dr. Przedborski says. “It was terrific to witness the fact that they all came to talk about the same topic: motor neurons. It went very well, and I think we're off to a great start.”

Work of groups affiliated with the Motor Neuron Center is supported by the SMA Foundation, Claire and Leonard Tow, Project ALS, the Muscular Dystrophy Association, Muscular Dystrophy Association/Wings Over Wall Street, the ALS Association, the New York State Office of Technology and Academic Resources, and the National Institute for Neurological Disorders and Stroke and National Institute on Aging. For more information, visit www.ColumbiaMNC.org



The Motor Neuron Center expects the study of healthy and diseased motor neurons will lead to new therapies for Lou Gehrig's disease and spinal muscular atrophy. Shown above, normal motor neuron growth in a developing chick.