STEM CELLS IN NEUROSCIENCE DISCOVERY

ROBERT MAYS, Vice President, Regenerative Medicine and Neuroscience Programme, **Athersys**

Dr. Mays is the Head of Neurosciences and Vice President of Regenerative Medicine at Athersys, Inc. He is focused on the company's novel adult human stem cell product, MultiStem®, and its applications in Regenerative Medicine and drug discovery, with a specific focus on injuries and diseases affecting the central nervous system. Dr. Mays was the Principal Investigator of the MASTERS (MultiStem Administration for Stroke Treatment and Enhanced Recovery Study) clinical trial assessing the safety and efficacy of MultiStem in treatment of ischemic stroke. He is also the Principal Investigator of the pivotal Phase III MASTERS-2 study, which has received RMAT, Fast Track and Special Protocol Assessment designations from the FDA. Dr. Mays is a member of the National Center for Regenerative Medicine, Center for Stem Cell and Regenerative Medicine, the American Heart Association and is an Adjunct Professor at the Case Western Reserve University School of Medicine. He has authored or co-authored >30 peer reviewed scientific papers or reviews and is the inventor of more than 10 patents relating to the use of stem cells for treating disease. Dr. Mays is on the Commercialization Committee and Co-chair of the Neuroscience Committee for the International Society for Cellular Therapy, and previously the Board of Directors for the United Cerebral Palsy Foundation of Cleveland and the scientific advisory board for the Children's Neurobiological Solutions Network in Los Angeles. He graduated from Carnegie Mellon in 1987 with a B.S. in Cell and Developmental Biology. In 1994, he received his Ph.D. in Molecular and Cellular Physiology at Stanford University. After doing Post-doctoral research at the University of Utrecht in the Netherlands, the Weizmann Institute in Rehovot, Israel and the University of California, San Francisco, Dr. Mays co-founded Athersys Inc., which focuses on developing novel and proprietary best-in-class therapies designed to extend and enhance the quality of human life.

What is your current focus within neuroscience drug discovery?

At Athersys, Inc., we have spent the last 15 years doing translationally relevant pre-clinical research, in vivo and in vitro, with our proprietary adult adherent cell product called MultiStem®. We have been focused on understanding the molecular mechanisms of action through which MultiStem administration improves outcomes across multiple animal models of CNS injury and disease.

Based on the data and information we obtained across multiple acute injury models of CNS injury (ischemic stroke, traumatic brain injury, spinal cord injury, hypoxic ischemic injury) as well as additional data derived from animal models of chronic neurological diseases (multiple sclerosis, ALS, Parkinson's Disease) we have moved in Phase 2 and Phase 3 clinical studies in ischemic stroke, with other clinical programs ready to initiate.

What are the advantages of utilising stem cells in neuroscience discovery? What are your main challenges in this area that you are working to overcome?

There are multiple advantages to using stem cells/cellular products to treat injury and disease when compared to single small molecule agents. Cells are dynamic therapies, with the ability to respond to different inflammatory/injury cues in different ways, depending on where and when you place them into the organism following initiation of the insult. Small molecule drugs and antibodies work through modulating one targeted pathway or physiological target. Cells can work through multiple mechanisms simultaneously.

Describe the main priorities for the neuroscience industry in stem cell research over the next year.

Many researchers believe that cellular therapeutics must be delivered directly into the CNS for the cells to provide meaningful benefit. The industry needs to understand the importance of the peripheral immune system and its involvement in exacerbating pathology of the CNS, and how cellular therapies can contribute to modulating this pathophysiology.

What keeps the life sciences industry so optimistic when it comes to drug approvals in neuroscience?

Recent advancements in understanding the initiators of disease and inflammation in many CNS disease models have provided optimism for new therapies. IPSC derived brain and tissue organoids and the development of CRISPR mediated gene editing has opened the door to more rapidly address many neuroscience related pathophysiology.

What is the biggest recent neuroscience discovery advancement that has affected your work?

Patient derived IPSC research. Allowing researchers the ability to take cells from patients with known diseases, and then recapitulate tissues and organs and look for dysfunction has been a huge step forward for neuroscience research.