



*Balancing Life's Tough Times*™



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## Families of Spinal Muscular Atrophy and Paratek Pharmaceuticals Expand Drug Discovery Collaboration for Spinal Muscular Atrophy

Libertyville, IL, and BOSTON, MA, April 3, 2007 — Families of Spinal Muscular Atrophy (FSMA) and Paratek Pharmaceuticals, Inc. today announced they have extended their joint R&D collaboration to develop a drug candidate for the treatment of Spinal Muscular Atrophy (SMA), the leading genetically inherited cause of death of children under the age of two years. The collaboration is focused on optimizing and advancing into the clinic a novel small molecule within Paratek's library derived from the tetracycline class of compounds.

Spinal Muscular Atrophy is an often-fatal genetic disorder resulting from the loss of both copies of the Survival Motor Neuron (SMN1) gene. This causes a chronic deficiency in the production of the SMN protein, which is essential to the proper functioning of the motor neurons in the spinal cord and to the control of muscles in the limbs, neck and chest.

"We are excited by the progress in our program with Paratek, which in the past year has demonstrated positive results in early cell models of this disease," said Kenneth Hobby, Executive Director of FSMA. "The continuation of this collaboration also represents FSMA's broader commitment to expand our relationships with life sciences companies and build a pipeline of drug candidates. As reported recently in the Wall Street Journal, we believe these kinds of interactions and early stage support can provide a critical impetus for companies to innovate new therapies for serious diseases such as SMA that may have been otherwise overlooked."

Dr. Ken Tanaka, Paratek Pharmaceuticals' Vice President of Research and Development, stated, "It is an honor to work with the committed and resourceful team at FSMA. Together with FSMA and with the team of scientists led by Dr. Adrian Krainer at Cold Spring Harbor Laboratory and Dr. Arthur Burghes at Ohio State University, we have come to a greater understanding of the underlying biology of SMA, especially the processes that regulate SMN splicing. Our lead candidate has

been shown in cells in culture and recently in cells in animals to enhance the expression of the SMN2 gene. Our goal is to find a compound that can restore normal levels of SMN protein in nerve tissue and prepare it for human studies to test the ability of this drug to slow or even reverse the disease process."

#### About Paratek Pharmaceuticals, Inc.

Paratek Pharmaceuticals, Inc. is engaged in the discovery and commercialization of new therapeutics that treat serious and life-threatening diseases, with a particular focus on the growing worldwide problem of antibiotic resistance. Paratek is advancing novel compounds that can circumvent or block bacterial resistance. Paratek's lead compound, MK-2764/PTK 0796, being developed in collaboration with Merck, has the potential to be a broad spectrum antibiotic with oral and IV formulations for the treatment of the most common community and hospital bacterial infections, including those caused by resistant strains such as MRSA. Paratek is developing small molecule drugs that can prevent infection by interfering with Multiple Adaptational Response (MAR) mechanisms in bacteria.

Outside the antibacterial therapeutic area, Paratek has also established an effort to exploit its novel tetracycline derivatives and their unique mechanism of action in selected anti-inflammatory and neurodegenerative conditions, including a collaboration to develop novel oral non-antibacterial tetracycline derivatives for multiple sclerosis with Merck-Serono S.A. Paratek has an active chemical synthesis effort to produce novel and diverse small molecules, with the goal of developing non-antibacterial compounds with improved activity in serious inflammatory and neurodegenerative diseases based upon a growing body of clinical and basic research supporting this approach.

Paratek is privately held and headquartered in Boston, Massachusetts, USA. For more information, visit Paratek's website at <http://www.paratekpharm.com/>.

#### About Families of SMA

FSMA is the leading international organization dedicated solely to eradicating SMA by promoting and supporting research in both the private and public sector, helping families cope through informational programs and support, and educating the public and the medical community about SMA. The organization, originally founded in 1984 by a small group of parents, has grown to more than 32 chapters and affiliates worldwide and more than 5,000 member families and is a founding member of the International Alliance for Spinal Muscular Atrophy. FSMA receives the majority of its funding through volunteer efforts, funding over \$25 million to date, and continues to increase its funding commitments each year with \$15 million in new research planned over the next three years. In addition, Families of SMA has funded more than \$3 million in patient support efforts.

Since its founding in 1984, FSMA-sponsored research has made significant contributions to better understanding SMA and advancing new therapies towards human clinical testing. These accomplishments include:

- Identification of a mutation in the SMN1 gene as the cause of the disease. A second copy of the gene called SMN2 produces reduced amounts of SMN protein due to a defect in splicing. In treating the disease, groups

hope to increase synthesis of the SMN2 proteins which can compensate for the loss of the SMN1 gene.

- The establishment of Project CURESMA, a clinical trial network, which is currently testing two medications for their possible impact in increasing SMN protein production from the SMN2 gene. This network will also serve as the conduit for future human drug trials.

For more information visit the website [www.curesma.org](http://www.curesma.org) or call 1-800-886-1762.