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

LIBERTYVILLE, IL and BOSTON, MA, June 17, 2008 -- Families of Spinal Muscular Atrophy (FSMA) and Paratek Pharmaceuticals, Inc. today announced they have extended and significantly expanded their joint research and development 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.

The partners have agreed to extend their collaboration for a third year and to approximately triple the resources dedicated to the program, with both partners increasing their investment in the effort. The Krainer Laboratory at Cold Spring Harbor Laboratory and the Hastings Laboratory at Rosalind Franklin University of Medicine and Science are also key collaborators in the program.

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 that the early, positive results of this collaborative program justify this new level of commitment,” said Kenneth Hobby, Executive Director at FSMA. “Our drug discovery strategy is to invest funds to enable companies to begin early-stage programs for this orphan disease. As this and other programs progress to later stages, we are looking for funding to transition from non-profit to commercial sources.”

This exciting announcement also coincides with the SMA community bringing its annual conference to Boston, where Paratek is headquartered. This is the largest conference in the world for families affected by SMA and also for the researchers and companies working to discover treatments and a cure for this devastating disease. Mayor Thomas M. Menino has proclaimed Saturday, June 21st as “SMA Awareness Day” for the City of Boston. Mr. Hobby stated, “FSMA is excited to be in Boston for our conference this year – an important hub of the biotech industry in the U.S. During our conference, we hope to increase awareness about SMA to the Boston biotech community.”

Jill Jarecki, Ph.D., FSMA's Research Director added, "The goal of this effort is to develop an SMA drug that safely and effectively restores the proper amount of SMN protein in the body by correcting the splicing of the *SMN2* gene. Compounds generated by Paratek have been shown to accomplish this in a number of tissues in animal models of the disease."

Dr. Stuart B. Levy, M.D., Paratek's Vice Chairman, Chief Scientific Officer and co-founder, stated, "We are extremely pleased with the continuation and expansion of our collaboration with FSMA and equally excited about the progress of our SMA program. Developing a clinical candidate for SMA, an orphan disease, plays an important role in demonstrating new applications of our technology platform based on tetracycline derivatives and in the extension of Paratek's R&D pipeline into genetic disorders."

Paratek reported early preclinical research from the collaboration at the recent annual meeting of the American Academy of Neurology. *In vitro* data showed that tetracycline derivatives could modify defective splicing of mRNA resulting from the gene defect seen in SMA. More research, including *in vivo* preclinical studies, is planned and will be presented in the suitable peer-reviewed forum in the future.

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, PTK 0796, is a broad spectrum antibiotic derived from the tetracycline class with oral and IV formulations that is being developed for the treatment of the most common and serious hospital bacterial infections, including those caused by resistant strains such as MRSA (methicillin-resistant *Staphylococcus aureus*) and MDRSP (multi-drug resistant *Streptococcus pneumoniae*). Oral and IV formulations of PTK 0796 were compared to Zyvox® in a recently completed Phase 2 clinical study in complicated skin and skin structure infections (cSSSI). In addition to PTK 0796, Paratek is also developing other broad- and narrow-spectrum tetracycline antibiotics to treat hospital and community infections based on its novel tetracycline chemistry expertise.

Outside of its tetracycline antibacterial program, Paratek has also identified small molecules that inhibit bacteria-specific transcription factors for Multiple Adaptational Response (*MAR*) genes which control bacterial virulence and resistance development..

Based upon a growing body of clinical research and as part of its effort to exploit its novel tetracycline derivatives and their unique mechanism of action in selected inflammatory and neurodegenerative conditions, 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. In addition, Paratek is encouraged by early evidence of the ability of tetracycline derivatives to affect mRNA splicing, as in SMA, which may also

have activity in related orphan genetic disorders, such as cystic fibrosis (CF), and Duchene Muscular Dystrophy.

Paratek has active collaborations with Merck & Co., MerckSerono, Warner-Chilcott and FSMA to develop tetracycline derived small molecule drugs for bacterial infections, multiple sclerosis (MS), acne & rosacea, and spinal muscular atrophy (SMA), respectively. Paratek is privately held and headquartered in Boston, Massachusetts, USA. For more information about Paratek and its research and development initiatives, visit Paratek's website at <http://www.paratekpharm.com>.

About Families of SMA

FSMA is dedicated to developing a treatment and cure for SMA by promoting and supporting research, 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 50,000 members and supporters. FSMA receives the majority of its funding through volunteer efforts, investing almost \$40 million to date. FSMA-sponsored research has made significant contributions to advancing new therapies towards human clinical testing.

For more information, visit the website www.curesma.org or call 1-800-886-1762.

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