



## **News Release**

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### **ASTELLAS AND MITOKYNE TO PURSUE MITOCHONDRIA-RELATED DISEASE THERAPIES**

*Collaboration Will Focus on Expanding the New Frontier of Mitochondria-related Disease*

**Tokyo and Boston, October 7, 2013** --- Astellas Pharma Inc. (Tokyo Stock Exchange: 4503, President and CEO: Yoshihiko Hatanaka, hereinafter called "Astellas") and Mitokyne, Inc. (President and CEO: Kazumi Shiosaki, hereinafter called "Mitokyne") announced today that the two companies have entered into an exclusive R&D collaboration focused on discovering and developing novel drugs that improve mitochondrial functions. These drug candidates have the potential to treat genetic, metabolic or neurodegenerative disorders as well as conditions of aging. The emerging biological understanding of mitochondria, along with new tools and assays available to better elucidate mitochondrial function and processes, will enable the identification of breakthrough treatments for patients suffering from mitochondria-related diseases and improve their quality of life.

Concurrently, Mitokyne closed a \$45M series A equity financing from Astellas, MPM Capital, and Longwood Fund. In connection with the financing, Ansbert Gadicke, MD, MPM Capital, Rich Aldrich, Longwood Fund, and a designee from Astellas joined the Mitokyne Board of Directors.

Under the collaboration agreement, Astellas and Mitokyne will collaborate to discover and develop compounds that target mitochondrial function. Mitokyne will lead all the research and drug discovery activities and be responsible for delivering IND candidates, whereupon Astellas will assume all clinical development activities as well as commercialization. In addition to generating a pipeline of novel drug candidates, Mitokyne is developing a novel mitochondrial screening platform to enhance ongoing R&D programs and identify new drug targets. Astellas has the exclusive right to acquire Mitokyne during certain periods during the term of the five-year agreement. Including upfront, R&D funding, acquisition and milestone payments, the total value of the partnership agreement could reach up to \$730M. Taking into account Astellas' participation in Mitokyne's equity financing, Astellas' actual payment for the acquisition based on equity in Mitokyne could be over \$500M in accordance with conditions of the agreement.

"This R&D partnership is an excellent opportunity for Astellas to utilize more external capabilities and resources, and to undertake initiatives related to new therapeutic areas, as we announced "Reshaping Research Framework" in May," said Yoshihiko Hatanaka, President & CEO of Astellas. "Through this partnership, we expect to pursue advanced drug discovery and establish a winning model with which it can solidify its position as the leader in the field of mitochondria-related diseases, and eventually develop advanced medical solutions for patients around the world, adding to our portfolio of innovative new drugs."

Mitokyne's scientific founders include world-renowned leaders in biology and mitochondrial function. Johan Auwerx is the Nestle Chair in Energy Metabolism and Professor at the École Polytechnique Fédérale de Lausanne. Andrew Dillin is the Siebel Distinguished Chair of Stem Cell Biology, HHMI Investigator and Professor of Genetic, Genomics and Development at UC Berkeley. Ronald Evans is the March of Dimes Chair in Developmental and Molecular Biology and Professor and Director in the Gene Expression Laboratory at Salk Institute for Biological Studies and was awarded the Lasker Award for his work in nuclear hormone receptors. H.

Robert Horvitz is Professor in the Department of Biology and a member of the McGovern Institute for Brain Research and of the Koch Institute for Integrative Cancer Research at the Massachusetts Institute of Technology. Professor Horvitz received the Nobel Prize in 2002 for his contributions to mechanisms of cell death. The scientific founders will join Mitokyne's Scientific Advisory Board, which also includes founding member Jodi Nunnari, a Professor of Molecular & Cellular Biology at University of California, Davis.

"I am delighted that we were able to bring together a committed corporate partner in Astellas along with great investors and scientific advisors to help shape and build this company in such an exciting area," said Kazumi Shiosaki, CEO and co-founder of Mitokyne. "Clearly Astellas and Mitokyne share a singular vision to become leaders in discovering and developing novel compounds that beneficially modulate mitochondrial function. Both companies will be able to contribute their complementary strengths to forge a robust partnership."

The impact of this collaboration has been accounted in Astellas' forecasts for fiscal year ending March 2014.

#### ***About Astellas***

Astellas Pharma Inc., located in Tokyo, Japan, is a pharmaceutical company dedicated to improving the health of people around the world through the provision of innovative and reliable pharmaceuticals. Astellas has approximately 17,000 employees worldwide. The organization is committed to becoming a global category leader in Urology, Immunology (including Transplantation) and Infectious diseases, Oncology, Neuroscience and Diabetes Mellitus (DM) Complications and Kidney diseases. For more information on Astellas Pharma Inc., please visit the company website at [www.astellas.com/en](http://www.astellas.com/en).

#### ***About Mitokyne***

Mitokyne is a biotech company focused on mitochondria-related drug discovery and development. The company is utilizing its innovative screening platform to identify and develop novel drugs that improve mitochondria function and provide treatments for genetic, metabolic and neurodegenerative disorders as well as diseases and conditions of aging. Mitokyne's scientific founders and advisors include world-renowned leaders in biology and mitochondrial function.

#### ***About MPM Capital***

MPM Capital is one of the world's largest life science-dedicated venture investors. With committed capital under management in excess of \$2.6 billion, MPM Capital is uniquely structured to invest globally in healthcare innovation.

#### ***About Longwood Fund***

Longwood Fund is a healthcare venture capital firm that finds, manages, and builds biotechnology companies. Longwood's mission is to identify technologies and to found companies that will advance new therapeutics that can not only make a difference in the lives of patients worldwide, but also create significant value for investors. This is achieved by leveraging the management team's history of successful biotechnology company formation and operational leadership.

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## ATTACHMENT

### **About Mitochondria**

Mitochondria are present in almost every cell of the human body. The main function of mitochondria is generation of ATP as the energy for humans to function. In addition, mitochondria are involved in numerous other functions including fatty acid metabolism, reactive oxygen species (ROS) production/elimination, cell death regulation, and so on.

### **About Mitochondrial diseases and Mitochondria-related diseases**

Mitochondrial diseases are caused by various mutations, acquired or inherited, in either mitochondrial DNA (mtDNA) or nuclear DNA (nDNA). Dysfunction of mitochondria can affect only certain tissues due to factors not yet fully understood. Affected patients suffer from muscular dysfunction, neurological dysfunction, cardiac dysfunction, ocular/otic dysfunction, metabolic dysfunction and so on. Typical onset in most cases is during early childhood, impairing a patient's quality of life.

Three disorders account for 60~70% of mitochondrial diseases:

- 1) mitochondrial myopathy, encephalopathy, lactic acidosis and stroke-like episodes (MELAS);
- 2) chronic progressive external ophthalmoplegia/ Kearns-Sayre syndrome (CPEO/KSS); and
- 3) myoclonus epilepsy associated with ragged-red fibers (MERRF).

Other mitochondria-related diseases include Leigh's syndrome, mitochondrial neurogastrointestinal encephalopathy (MNGIE), Alpers' disease, Leber's hereditary optic neuropathy (LHON), neuropathy, ataxia, and retinitis pigmentosa (NARP) and fatty acid oxidation disorders (FAOD).

Mitochondrial dysfunctions are suspected of playing roles in seemingly unrelated disorders, such as muscular, metabolic, neurodegenerative, ophthalmic, aural and other disorders that include cancer, heart failure and kidney damage. Specific disorders considered to be linked to mitochondria-related diseases include Alzheimer's disease, Parkinson's disease, amyotrophic lateral sclerosis (ALS), stroke, mood disorders, diabetes, fatty liver disease, osteoporosis, cancer, peripheral arterial disease (PAD), age-related hearing loss (AHL) and others.

Today, the diagnosis of mitochondria-related diseases remains a complicated process due to the wide range of symptoms.

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