Press Release

U.S. FDA Grants Fast Track Designation for ASP0367/MA-0211, a Selective PPARδ Modulator Being Developed for the Treatment of Primary Mitochondrial Myopathies

TOKYO, October 20, 2020 - Astellas Pharma Inc. (TSE: 4503, President and CEO: Kenji Yasukawa, Ph.D., "Astellas") today announced that the U.S. Food and Drug Administration (FDA) has granted Fast Track designation for the development of ASP0367/MA-0211 ("ASP0367") as a potential treatment for primary mitochondrial myopathies (PMM).

PMM is a complex mitochondrial disease in which genetic mutations primarily impair the function of mitochondria, resulting in reduced muscle function, reduced endurance to exercise (i.e., exercise intolerance), increased fatigue, and muscle atrophy. In addition, PMM may present serious and life-threatening health conditions due to multiple organ involvement. For example, decreased myocardial function may lead to cardiomyopathy and heart failure. Decreased function of respiratory muscles may cause respiratory failure and pneumonia. In patients with mitochondrial disease due to pathogenic mutations in either the mtDNA or nDNA genome, the minimum point prevalence is estimated at 12.5 in 100,000 (approximately 1 in 8,000) for adults with clinical manifestations, and 23 in 100,000 (approximately 1 in 4,300) for adults with or without clinical manifestations.[1] There is no FDA-approved treatment for PMM, a rare disease with a high unmet medical need.

Preclinical data and results from Astellas' Phase I healthy volunteer study collectively suggest that ASP0367 - an orally administered PPARδ modulator - could improve exercise intolerance and fatigue in PMM patients by increasing the number and enhancing the function of mitochondria in patient's cells. ASP0367 will be entering a Phase II / III study (MOUNTAINSIDE) to validate the efficacy and safety in PMM patients.

Astellas explores mitochondrial biology as a Primary Focus of its R&D strategy and is committed to providing treatments using new modalities. Targeting mitochondrial function is an innovative approach to address diseases with significant unmet medical needs in novel and effective ways. With the acquisition of Mitobridge, Inc. in 2018,

Astellas has gained access to Mitobridge's expertise in mitochondrial biology and a pipeline of innovative programs including ASP0367.

The FDA's Fast Track designation system aims to expedite the development and review of therapeutic agents for serious or potentially life-threatening diseases with high unmet medical need. The Fast Track designation is expected to accelerate the clinical development of ASP0367.

"Primary mitochondrial myopathies are a serious, complex disease with significant unmet need and no approved therapies," said Salim Mujais, M.D., Senior Vice President and Therapeutic Area Head, Medical Specialties, Astellas. "With the development of ASP0367, an oral, once daily modulator of mitochondrial energy production, we are hoping to alleviate the serious burden of this disease on patients, their families and caregivers."

"At Astellas, mitochondrial biology is one of our Primary Focus Areas, driving our commitment to bring new biological concepts into the clinic for rapid proof of concept," said Mike Patane, Ph.D., President of Mitobridge, an Astellas Company, located in Cambridge, Mass. and part of the Astellas Biomedical Innovation Hub. "We are pleased to see this new milestone for ASP0367 as it shows steady progress in our Primary Focus and further demonstrates our focus on turning innovative science into value for patients."

About ASP0367

ASP0367, a selective modulator of PPARδ, has the potential to be a first-in-class treatment for PMM. In a preclinical study, ASP0367 increased the expression of PPARδ target genes and enhanced mitochondrial function in fibroblasts collected from patients with PMMs. A phase I study in healthy adults demonstrated dose-dependent increased expression of PPARδ target genes and ASP0367 doses were found to be safe and well tolerated in this study. ASP0367 is also being developed as a potential treatment for Duchenne muscular dystrophy. Other Phase Ib studies are also under preparation. For more information on clinical trials, visit www.clinicaltrials.gov.

About Mitobridge, an Astellas Company.

Mitobridge, Inc., located at Astellas' site in Cambridge, Mass., is dedicated to delivering therapeutics that improve mitochondrial function. Our team of experienced drug discovery and development scientists is leveraging their exceptional knowledge of mitochondria biology to develop a pipeline of innovative programs for the treatment of kidney, muscle and other diseases with high unmet medical need. Mitobridge was launched in October 2013, and the Company was acquired by Astellas Pharma in January of 2018. For more information about the Company, please visit www.mitobridge.com.

About Astellas

Astellas Pharma Inc. is a pharmaceutical company conducting business in more than 70 countries around the world. We are promoting the Focus Area Approach that is designed to identify opportunities for the continuous creation of new drugs to address diseases with high unmet medical needs by focusing on Biology and Modality. Furthermore, we are also looking beyond our foundational Rx focus to create Rx+® healthcare solutions that combine our expertise and knowledge with cutting-edge technology in different fields of external partners. Through these efforts, Astellas stands on the forefront of healthcare change to turn innovative science into value for patients. For more information, please visit our website at https://www.astellas.com/en.

Cautionary Notes

In this press release, statements made with respect to current plans, estimates, strategies and beliefs and other statements that are not historical facts are forward-looking statements about the future performance of Astellas. These statements are based on management's current assumptions and beliefs in light of the information currently available to it and involve known and unknown risks and uncertainties. A number of factors could cause actual results to differ materially from those discussed in the forward-looking statements. Such factors include, but are not limited to: (i) changes in general economic conditions and in laws and regulations, relating to pharmaceutical markets, (ii) currency exchange rate fluctuations, (iii) delays in new product launches, (iv) the inability of Astellas to market existing and new products effectively, (v) the inability of Astellas to continue to effectively research and develop products accepted by customers in highly competitive markets, and (vi) infringements of Astellas' intellectual property rights by third parties. Information about pharmaceutical products (including products currently in development) which is included in this press release is not intended to constitute an advertisement or medical advice.

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Reference:

1: Gorman GS, et al. Prevalence of nuclear and mitochondrial DNA mutations related to adult mitochondrial disease. Ann Neurol. (2015); 77:753-759.