November 25,2024

Nobelpharma Co., Ltd.

Launch of "ACENOBEL® Sustained Release Tablet 500 mg"
for Distal Myopathy with Rimmed Vacuoles

Nobelpharma Co., Ltd. (Headquarters: Chuo-ku, Tokyo, Managing Director & CEO: Jin Shiomura) announced that its drug for distal myophathy with rimmed vacuoles, "ACENOBEL® sustained release tablet 500 mg" ("Product") would be launched on Thursday, December 19.

We would like to express our deepest apologies for causing patients, their families and healthcare professionals all the concern and troubles by taking prolonged time to release the product since its approval on March 26.

Upon the development requests from National Center of Neurology and Psychiatry ("NCNP") and the Patient Association for Distal Myopathies ("PADM"), the studies on this drug such as the investigator-initiated Phase II/III trials and Japanese long-term administration study as the programs of NEDO, JST, Ministry of Education, Culture, Sports, Science and Technology, Ministry of Health, Labor and Welfare, and AMEDand the efficacy assessment study with the support from National Institutes of Biomedical Innovation, Health and Nutrition were conducted with the chief investigator, Dr. Masashi Aoki, Professor of Department of Neurology, Tohoku University Graduate School of Medicine, and the medical expert, Dr. Ichizo Nishino, Director of Department of Neuromuscular Research, NCNP.

We are deeply grateful for all the efforts from NCNP and Tohoku University and many other physicians and for the cooperation of PADM and all the patients participated in our studies, which lead to the launch of this Product.

Distal myopathy with rimmed vacuoles is an exceptionally rare disease characterized by progressive atrophy and degeneration of the distal muscles which lie farther from the trunk, and the number of patients is estimated about 400. It has been known the

patients of this disease are unable to internally synthesize sufficient aceneuramic acid (N-acetylneuraminic acid, a typical sialic acid). Accordingly, a low amount of sialic acid in muscles and decreased sialic acid content of certain proteins in the patients have been reported. In animal (mice) testing, the physical capabilities, skeletal muscle contraction, blood test results, and muscle pathologies are demonstrated to be similar to those of the normal mice if aceneuramic acid has been administered since before the disease development, which has lead to an expectation of the improvement or progression control in clinical conditions of the patients.

Based on such outcomes, we had advanced the development in collaboration with NCNP and Tohoku University in order to put aceneuramic acid to practical use as a drug for the treatment of distal myopathy with rimmed vacuoles.

As there have been no fundamental treatment for this disease and rehabilitation therapy is given as a supportive measure for the purpose to prevent contracture, we are gratified that we can be of some help for the treatment of distal myopathy with rimmed vacuoles with the launch of this Product.

We are determined to continue contributing to society by providing critical but neglected pharmaceuticals and medical devices.

[Contact for Inquiries]
Noboru Kudo, Head of Communications
Nobelpharma Co., Ltd.
1-17-24, Shinkawa, Chuo-ku, Tokyo 104-0033
Tel: 03-6670-3800