March 26, 2024

Nobelpharma Co., Ltd.

"ACENOBEL® Sustained Release Tablet 500 mg"
Approved 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") has been approved.

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 AMED[1)] and the efficacy assessment study with the support from National Institutes of Biomedical Innovation, Health and Nutrition[2)] 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.

1) Japanese phase I trial (sialic acid-1 study): 2009 Subsidized Program to Realize Innovation (NEDO) and Adaptable and Seamless Technology Transfer Program through Target Driven R&D/Stage III/NexTEP-A type (JST)
Japanese phase I trial (sialic acid-2 study): Grant for Translational Research Network Program (Ministry of Education, Culture, Sports, Science and Technology) and Health and Labour Sciences Research Grant "Research Program for Overcoming Intractable Diseases" (Ministry of Health, Labor and Welfare)
Japanese Phase II/III trial (sialic acid-3 study) and long-term administration study (sialic acid-4 study): Practical Research Project for Rare/Intractable Diseases (AMED) and Division of Strategic Planning/Support Program for Orphan Drug prior to the Designation (AMED)
2) Efficacy assessment study (NPC-09-1 study): Orphan/Specialized Products Development Support Program (National Institutes of Biomedical Innovation, Health and Nutrition)

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

* This Product received orphan designation in February 2021 ((R3yaku) No. 501).

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

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