March 26, 2024

Nobelpharma Co., Ltd.

"Sargmalin® for inhalation 250 μg" Approved for Autoimmune Pulmonary Alveolar Proteinosis

Nobelpharma Co., Ltd. (Headquarters: Chuo-ku, Tokyo, Managing Director & CEO: Jin Shiomura) announced today that "Sargmalin® for inhalation 250 μg" has been approved for the indication of autoimmune pulmonary alveolar proteinosis (this "Product").

We are deeply grateful for all of the efforts of the principal investigator, Dr. Koh Nakata, University Medical and Dental Hospital Advanced Clinical Research Center Specially Appointed Professor, and many physicians participated in the investigator initiated studies, as well as for the cooperation of the patients in the clinical studies and the assistance from Japan Agency for Medical Research and Development (AMED) and National Institute of Biomedical Innovation, Health and Nutrition, owing to which the Product was approved.

This Product is a "sargramostim (genetically modified)" based inhalation with granulocyte-macrophage colony-stimulating factor (GM-CSF), one of the cytokine having a promoting effect for proliferation and differentiation of myeloid precursor, produced with fermentum. This Product has been in the US market since 1991 as an injectable drug for neutrophil recovery following induction chemotherapy and myeloid cell recovery following peripheral stem-cell transplantation in patients with acute myeloid leukemia on the grounds that it increases and functionally activates neutrophils, eosinophils and monocytes; however, it is not approved for the indication of autoimmune pulmonary alveolar proteinosis nor as an inhalation.

Autoimmune pulmonary alveolar proteinosis is developed as the hyperproduced anti-GM-CSF autoantibody blocks mature alveolar macrophage from decomposing waste products including alveolar surfactant. There have been no drugs approved in any country for autoimmune pulmonary alveolar proteinosis and only established treatment is segmental lung lavage or whole lung lavage that removes accumulated waste products by injecting saline into lung under general anesthesia.

While post-operative rapid symptomatic improvement can be expected with whole lung lavage, there exist clinical challenges of being highly invasive and requiring hospitalization as well as the therapeutic opportunities limited to facilities with experienced specialists.

This Product, as inhaled, acts directly on alveolar macrophage to accelerate its maturation and promotes decomposition of the waste products of alveolar surfactant by such matured macrophage, leading to improvement in lung functions.

The inhalation therapy of this Product is less invasive compared to segmental and whole lung lavage and we are confident this will provide a new treatment option for the clinical practice in addition to the existing treatment.

In Japan, autoimmune pulmonary alveolar proteinosis has become a designated intractable disease since 2015 and the number of patients is expected to be 730 to 770. We are gratified the approval of this Product will assist the treatment of autoimmune pulmonary alveolar proteinosis.

We are committed 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