19 July 2018
Toray Industries, Inc.
BONAC Corporation

Toray Announces Initiation of Phase 1 Clinical Trial of TRK-250 for Patients with Idiopathic Pulmonary Fibrosis

Toray Industries, Inc. (headquarters: Chuo-ku, Tokyo; President: Akihiro Nikkaku; hereinafter referred to as "Toray") announced today that the U.S. Food and Drug Administration (FDA) had accepted its Investigational New Drug (IND) Application in July 2018 to initiate a Phase 1 clinical trial[*1] for a nucleic acid candidate developed in collaboration with BONAC Corporation (headquarters: Kurume-shi, Fukuoka Prefecture; President: Hirotake Hayashi; hereinafter referred to as "BONAC"), TRK-250 (Toray's development code)/BNC-1021 (BONAC's development code), for the treatment of Idiopathic Pulmonary Fibrosis (IPF)[*2]. Upon permission from the FDA, Toray will promptly initiate the Phase 1 clinical trial of the agent.

IPF is disorder with a poor prognosis and an unpredictable clinical course, in which fibrosis of intestinal pneumonia progresses irreversibly. Thus, the development of a novel drug with new mechanisms is expected to broaden the treatment options in clinical practice.

Toray has launched a bio-pharmaceutical, FERON®, and two low-molecular-weight drugs[*3], DORNER® and REMITCH®, and has been developing various types of drugs, such as antibody drugs[*4] according to disease- and treatment-targeting factors. In collaboration with BONAC, Toray will now initiate the Phase 1 clinical trial of the agent.

Nucleic acid pharmaceuticals are agents that act on disease-causing genes and proteins by utilizing the action of DNA (deoxyribonucleic acid) and RNA (ribonucleic acid). Nucleic acid medicine has been increasingly attracting attention as a next-generation medicine following low-molecular-weight drugs and biopharmaceuticals such as antibody drugs due to its potential to offer a new type of medication with fewer side effects.

TRK-250/BNC-1021 is a nucleic acid medicine that inhibits the progression of pulmonary fibrosis by selectively suppressing the expression of transforming growth factor-beta 1 (TGF-β1) protein, a key growth factor involved in lung fibrosis, at the gene expression level. One of the features of the agent is that it is a single strand long-chain nucleic acid with a unique molecular structure employing BONAC's proprietary nucleic acid platform, and it is expected to be a new nucleic acid medicine that has overcome the issue of stability. Moreover, it is in an aerosol form that can be administered directly to the lung, which is expected to carry the agent efficiently to the target organ.

Toray signed a licensing agreement in December 2015 with BONAC, who is the originator of the agent, and Toray has acquired exclusive rights of development, sales, and manufacturing (excluding those of the active pharmaceutical ingredient) for the agent for IPF treatment in Japan. Toray and BONAC have

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been collaborating in problem-solving and project management of non-clinical studies and development of an inhaler-based administration method, and now Toray has announced the initiation of a Phase 1 clinical trial for IPF patients in the U.S.

Toray will receive financial aid from a support program of the Japan Agency for Medical Research and Development (AMED)[*5] for this Phase 1 clinical trial, aiming to demonstrate the safety, tolerability, and pharmacokinetics of the agent administered by an inhaler to patients with IPF. Moreover, a Phase 2 clinical trial will establish the inhibitory effect on the progression of pulmonary fibrosis, which has been demonstrated in non-clinical studies using animal models. Toray and BONAC have been applying their experience and technologies to develop innovative synthetic methods of nucleic acid medicine and will conduct clinical trials in phases to launch the agent in the late 2020s.

*1 Clinical Trial:

A kind of clinical research designed to evaluate new medications based on their safety and efficacy in humans. Clinical trials are generally conducted in stages from phases 1 to 3, where safety and pharmacokinetics are assessed in phase 1 and efficacy and safety are evaluated (including comparison to placebo) in phases 2 and 3.

*2 Idiopathic pulmonary fibrosis (IPF) :

A disease that is considered to be the most difficult to treat among idiopathic interstitial pneumonia types. It is a chronic intractable disease with an associated mean survival time of 3 to 5 years after diagnosis. Interstitial pneumonia is a disease in which inflammation and injuries due to various causes on the wall of alveoli located at the end of lungs induce fibrosis with the accumulation of collagen fibers, leading to difficulty in breathing. Interstitial pneumonia, whose cause remains unknown, is called idiopathic interstitial pneumonia. IPF occurs in 10 out of 100,000 individuals, with an estimated to be more than 10,000 patients in Japan.

*3 Low-molecular-weight drugs:

General pharmaceuticals that are made by chemical synthesis.

*4 Antibody drugs:

Pharmaceuticals whose main ingredients are antibodies, key players in the immune system of the human body. They are highly specific to the target and have fewer side effects. The issues faced include difficulties in mass production and the fact that the drugs are unsuitable for oral administration.

*5 Support program of AMED:

A Support Program for Orphan drug prior to the Designation for the fiscal year 2018 under the Project Promoting Support for Drug Discovery of Japan Agency for Medical Research and Development (AMED)
Project title: “The development of IPF therapeutic drug (TRK-250/BNC-1021)” (Project No. JP18nk0101213)

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<REFERENCE>Company profiles

Toray Industries, Inc.
1) Business: Manufacturing and sales of the following products, among others: fibers and textiles, performance chemicals, carbon fiber composite materials, environment and engineering and life science.
2) Address: Chuo-ku, Tokyo
3) Established: 1926
4) President and CEO: Akihiro Nikkaku
http://www.toray.co.jp/

BONAC Corporation
1) Business: Manufacture of drug substances (manufacture of nucleic acid medicine drug substance) and pharmaceutical development (development of nucleic acid medicines and development support)
2) Address: Kurume-shi, Fukuoka
3) Established: 2010
4) President and CEO: Hirotake Hayashi
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