

News release

Kyowa Kirin initiated Phase 3 Clinical Study of Bardoxolone Methyl (RTA 402) for Autosomal Dominant Polycystic Kidney Disease (ADPKD) in Japan

Tokyo, Japan, January 6, 2021 -- Kyowa Kirin Co., Ltd. (President and CEO: Masashi Miyamoto, "Kyowa Kirin", TSE: 4151) today announced the initiation of a Phase 3 study (study name: FALCON) in Japan for bardoxolone methyl (Code name: RTA 402), a small-molecular compound licensed from Reata Pharmaceuticals, Inc. (Plano, Texas, USA; CEO and President: Warren Huff, "Reata").

The FALCON study is an international, multi-center, randomized, placebo-controlled, double-blind clinical trial for patients with autosomal dominant polycystic kidney disease (ADPKD), where efficacy and safety of RTA 402 are to be evaluated by oral administration of the drug or placebo once a day. This study was initiated by Reata on May 29, 2019 and is currently ongoing in North America, the EU, and Oceania. Japan is included to the study at this time, where Kyowa Kirin is the ICC (In-Country Clinical Care-taker).

Yoshifumi Torii, Ph.D., Executive Officer, Vice President, Head of R&D Division of Kyowa Kirin commented, "We are profoundly delighted to mark a new step toward delivering the drug to patients with ADPKD. RTA 402 has the potential to bring new benefits and smiles to patients with ADPKD."

Under the license agreement signed on December 24, 2009 with Reata, Kyowa Kirin owns exclusive rights to develop and commercialize bardoxolone methyl in kidney disease and certain other indications in Japan, China, Taiwan, South Korea and Southeast Asia.

The Kyowa Kirin Group companies strive to contribute to the health and well-being of people around the world by creating new value through the pursuit of advances in life sciences and technologies.

【Study overview】

Study Population	Patients with ADPKD
Primary Efficacy Endpoint	Off-treatment change from baseline in eGFR at Week 52 or following a 4-week drug treatment withdrawal period in the first year of treatment
Key Secondary Endpoint	Off-treatment change from baseline in eGFR at Week 104 or following a 4-week drug treatment withdrawal period in the second year of treatment
Estimated Enrollment	300 participants

Location	North America, EU, Oceania, Japan
Estimated Study Completion	August 2023

About Bardoxolone Methyl

Bardoxolone methyl is a small-molecular compound that activates nuclear factor erythroid 2-related factor 2 (Nrf2), a transcription factor that has a key role in the body's protective response to stress. A wide range of anti-oxidative and anti-inflammatory effects of this compound could improve kidney function. In the phase 2 clinical study conducted in Japan (TSUBAKI), administration of bardoxolone methyl resulted in a clear improvement in glomerular filtration rate (kidney function) measured using the inulin clearance method. In Japan, Kyowa Kirin is conducting the phase 3 study for Diabetic Kidney Disease (AYAME) and preparing the marketing authorization application for Alport Syndrome based on the results of the international Phase 2/3 study (CARDINAL), which was led by Reata.

About autosomal dominant polycystic kidney disease (ADPKD)

ADPKD is the most common hereditary cystic kidney disease that causes the progressive development and enlargement of numerous cysts on both sides of the kidneys, as well as liver cysts and other complications in addition to the kidneys. It is estimated that about half of ADPKD patients will develop end-stage kidney disease by the time they are in their 60s. This disease is certified as a designated intractable disease in Japan. As with other chronic kidney diseases, oxidative stress and inflammation are thought to be involved in the progression of ADPKD. The number of patients in Japan is estimated to be approximately 31,000.

About In-Country Clinical Care-taker (ICCC)

In case that companies which do not have their domicile in Japan conduct clinical trials in Japan, they need to select an ICCC among companies which has their domicile in Japan. ICCC is responsible for all operations related to clinical trials in Japan on behalf of those companies.

About eGFR

Abbreviation of estimated glomerular filtration rate. Although the inulin-clearance method, the gold standard for measuring GFR, is used in case of need for an accurate evaluation of kidney function for such as kidney transplant donor, eGFR based on the serum creatinine level is widely used to assess kidney function in clinical practice.