Transplant Therapeutics Consortium Receives Acceptance of Letter-of-Intent for iBox Scoring System as a Reasonably Likely Surrogate Endpoint from the Biomarker Qualification Program

Collaboration between the transplant community, industry, and regulatory agencies develops biomarker aimed to streamline the development of novel therapies intended to improve long-term outcomes for kidney transplant recipients.

Cary, NC, June 18, 2020 — Critical Path Institute (C-Path) announced Wednesday, June 17, that its Transplant Therapeutics Consortium (TTC), of which Veloxis Pharmaceuticals is a member, has received a positive response to its Letter of Intent (LOI) from the U.S. Food and Drug Administration (FDA) detailing the decision to accept the iBox Scoring System into the Center for Drug Evaluation and Research (CDER) Biomarker Qualification Program (BQP).

In its LOI, the TTC provided information to support the qualification of the iBox Scoring System for its proposed context of use (COU) as a reasonably likely surrogate endpoint in clinical trials intended to evaluate immunosuppressive drugs (ISDs) for individuals living with a kidney transplant. Qualification as a surrogate or reasonably likely surrogate endpoint would allow drug sponsors to pursue accelerated approval, removing a significant barrier to kidney transplant drug development.

Long-term graft failure rates after kidney transplantation remain unacceptably high, despite improved short-term outcomes, with 10-year all-cause graft failure approaching 50% (Hart et al. 2019). Survival of the transplanted organ has been rated, by patients, as the most important outcome, including the overall survival of the patient (Howell et al. 2012). The iBox Scoring System, developed by the Paris Transplant Group, is the first tool of its kind to seek regulatory qualification for use in kidney transplant clinical trials. To date, no biomarkers have been qualified for use as a surrogate or reasonably likely surrogate endpoint in any therapeutic area.

The iBox Scoring System is a risk prediction tool that combines measurements of kidney function, immunological status, and pathological assessment of kidney biopsy histology to predict the risk of graft-loss up to seven years after the time of risk assessment. The iBox Scoring System has been extensively validated for use in the treatment of individual patients in the clinical care setting. The TTC, in close collaboration with the Paris Transplant Group, is seeking to translate this work into the regulatory setting for use in drug development programs.

Ulf Meier-Kriesche, MD FAST, the Chief Scientific Officer at Veloxis Pharmaceuticals, has acted as the work group Co-chair since the inception of the TTC. Dr. Meier-Kriesche states “The lack of a viable clinical trial endpoint has been the Achilles heel of transplant drug development since transplant outcomes have outgrown our current regulatory endpoint at least 20 years ago. The FDA’s acceptance of the LOI is a significant milestone in coming up with a new endpoint which can revive transplant drug development. Veloxis, like other transplant companies, is very interested in bringing new therapies to the community and the advent of a new endpoint could really be a game changer for industry and the community we are serving.”

As part of the 21st Century Cures Act, passed into law in December 2016, public-private partnerships consisting of government entities, including the FDA, the biopharmaceutical industry, healthcare providers, academic researchers, and patient advocacy organizations are encouraged to work together to foster innovation in drug development through drug development tools that facilitate patient access to life-saving medications.

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About Veloxis
Veloxis Pharmaceuticals A/S, an Asahi Kasei company, is a commercial-stage specialty pharmaceutical company committed to improving the lives of transplant patients. Veloxis Pharmaceuticals A/S operates in the U.S. through Veloxis Pharmaceuticals, Inc., a wholly owned subsidiary headquartered in Cary, North Carolina, USA. Veloxis is focused on the direct commercialization of immunosuppression medications in the US, expansion of partnerships for markets around the world, and acquisition of assets utilized in transplant patients and by adjacent medical specialties. For further information, please visit www.veloxis.com.

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