Ardelyx Announces FDA Acceptance for Filing of its New Drug Application of Tenapanor for the Control of Serum Phosphorus in Adult Patients with CKD on Dialysis

September 15, 2020

NDA Supported by Data from Expansive Clinical Development Program Demonstrating Tenapanor's use as Foundational Therapy

PDUFA Goal Date - April 29, 2021

FREMONT, Calif., Sept. 15, 2020 /PRNewswire/ -- Ardelyx, Inc. (Nasdaq: ARDX), a biopharmaceutical company focused on developing first-in-class medicines to improve treatment for people with kidney and cardiovascular diseases, today announced that the U.S. Food and Drug Administration (FDA) has accepted its New Drug Application (NDA) of tenapanor for the control of serum phosphorus in adult patients with chronic kidney disease (CKD) on dialysis.

"The acceptance of our NDA is extremely exciting as it represents the next critical step towards bringing to market a completely new approach to the management of hyperphosphatemia, an area where a significant unmet need exists," said Mike Raab, president, and chief executive officer of Ardelyx. "With potential approval in the second quarter of 2021, we continue to advance commercial preparations for the launch of tenapanor, a first-in-class, non-binder therapy that targets the primary pathway of phosphorus absorption. This is a special time for the Ardelyx team as we have a clear mission – that we can and should do better for patients. We believe that with tenapanor, we have discovered and developed a therapy that will truly advance care for patients on dialysis."

The FDA has set a Prescription Drug User Fee Act (PDUFA) goal date of April 29, 2021.

"I look forward to the prospect of having a novel approach to treating hyperphosphatemia, a condition known to be associated with higher morbidity and mortality in patients with chronic kidney disease on dialysis," said Dr. Kam Kalantar-Zadeh, Chief, Division of Nephrology and Hypertension and Kidney Transplantation, University of California, Irvine, School of Medicine. "I believe innovations that enable us to block phosphorus via the primary pathway of absorption will help us more consistently and effectively manage phosphorus, so we can do better for our patients."

The NDA is supported by three successful Phase 3 trials involving over 1,000 patients that evaluated the use of tenapanor, which included: two monotherapy trials, including a long-term study, to control serum phosphorus in patients with CKD on dialysis, and one trial using a dual-mechanism approach in dialysis patients who had difficult-to-control hyperphosphatemia (≥5.5 mg/dL) despite phosphate binder therapy.

About Tenapanor for Hyperphosphatemia
Tenapanor, discovered and developed by Ardelyx, is a first-in-class, proprietary, oral medicine for which an NDA is under review by the FDA for the control of serum phosphorus in adult patients with CKD on dialysis. Tenapanor has a unique mechanism of action and acts locally in the gut to inhibit the sodium hydrogen exchanger 3 (NHE3). This results in a conformational change of the epithelial cell junctions, thereby significantly reducing paracellular uptake of phosphate at the primary pathway of phosphate absorption. Ardelyx is conducting NORMALIZE, an ongoing extension study of the PHREEDOM Phase 3 monotherapy study, which is designed to evaluate the ability of tenapanor, as monotherapy or in combination with sevelamer, to achieve serum phosphorus levels in the normal range (2.5 – 4.5 mg/dL) in patients with chronic kidney disease (CKD) on dialysis. Planned analyses have demonstrated that the use of tenapanor as a foundational approach, as monotherapy or in combination with sevelamer carbonate, produces a significant phosphorus-lowering effect. After ~20 months of treatment with tenapanor alone or with low doses of sevelamer, patients exhibited a mean serum phosphorus reduction of 2.33 mg/dL, from a mean baseline phosphorus of 7.27 mg/dL at the beginning of the PHREEDOM trial to a mean of 4.94 mg/dL.

About Hyperphosphatemia
Hyperphosphatemia is a serious condition resulting in an abnormally elevated level of phosphorus in the blood that is estimated to affect more than 745,000 dialysis patients in major developed countries. The kidney is the organ responsible for regulating phosphorus levels, but when kidney function is significantly impaired, phosphorus is not adequately eliminated from the body. As a result, hyperphosphatemia is a nearly universal condition among people with CKD on dialysis. Despite treatment with phosphate binders (the only approved therapy for hyperphosphatemia), approximately 70% of CKD patients on dialysis continue to experience elevated phosphorus levels at any point in time (Spherix Global Insights: RealWorld Dynamix, Dialysis 2018). Phosphorus levels greater than 5.5 mg/dL have been shown to be an independent risk factor for cardiovascular morbidity and mortality in patients requiring dialysis (Block 2004), and internationally recognized treatment guidelines recommend lowering elevated phosphate levels toward the normal range (<4.6mg/dL).

About Ardelyx, Inc.
Ardelyx is a biopharmaceutical company focused on developing innovative first-in-class medicines to improve treatment for people with kidney and cardiovascular diseases. The Ardelyx pipeline includes tenapanor for the control of serum phosphorus in adult patients with CKD on dialysis, for which an NDA is under review by the FDA, and RDX013, a potassium secretagogue program for the potential treatment of high potassium, or hyperkalemia, a problem among certain patients with kidney and/or heart disease. In addition, Ardelyx received FDA approval of IBSRELA® (tenapanor) on September 12, 2019. Ardelyx has established agreements with Kyowa Kirin in Japan, Fosun Pharma in China and Knight Therapeutics in Canada for the development and commercialization of tenapanor in the respective territories.

Forward Looking Statements
To the extent that statements contained in this press release are not descriptions of historical facts regarding Ardelyx, they are forward-looking statements reflecting the current beliefs and expectations of management made pursuant to the safe harbor of the Private Securities Reform Act of 1995, including the potential for tenapanor to be approved for marketing by the FDA for the control of serum phosphorus in chronic kidney disease patients on dialysis, the potential for the use of tenapanor as monotherapy and as part of a dual mechanism approach with tenapanor and phosphate binders for the treatment of hyperphosphatemia, the potential for tenapanor alone or with small doses of phosphate binders to achieve target serum phosphorus levels, and Ardelyx's expected timing of the review of its NDA for tenapanor for the control of serum phosphorus. Such forward-looking statements involve substantial risks and uncertainties that could cause the development of Ardelyx's product candidates or Ardelyx's future results, performance or achievements to differ significantly from those expressed or implied by the forward-looking statements. Such risks and uncertainties include, among others, the uncertainties associated with the regulatory approval process, and uncertainties in the drug commercialization process. Ardelyx undertakes no obligation to update or revise any forward-looking statements. For a further description of the risks and uncertainties that could cause actual results to differ from those expressed in these forward-looking statements, as well as risks relating to Ardelyx's business in general, please refer to Ardelyx's quarterly report on Form 10-Q filed with the Securities and Exchange Commission on August 6, 2020, and its future current and periodic reports to be filed with the Securities and Exchange Commission.

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