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Codexis Announces FDA Orphan Drug and Rare Pediatric Disease Designations for CDX 6512 for the Treatment of Homocystinuria

REDWOOD CITY, Calif., Jan. 24, 2022 (GLOBE NEWSWIRE) -- Codexis, Inc. (NASDAQ: CDXS), a leading enzyme engineering company enabling the promise of synthetic biology, today announced that the U.S. Food and Drug Administration (FDA) has granted the company orphan drug designation (ODD) for CDX-6512 for the treatment of homocystinuria. CDX-6512 is a gastrointestinal-stable methionine-gamma-lyase, as a potential orally-administered enzyme therapy for homocystinuria (HCU). The FDA also granted the company rare pediatric disease (RPD) designation for CDX-6512. CDX-6512 is currently in pre-IND development and is the most advanced wholly owned program in the Company's biotherapeutics pipeline.

"The orphan drug and rare pediatric disease designations for CDX-6512 further build upon the momentum our engineered enzymes have generated as potential first-in-class oral therapeutics for inborn errors of metabolism," said John Nicols, President and CEO of Codexis. "These designations represent an important step forward in the development of CDX-6512 as a potential treatment of homocystinuria and we look forward to continuing its advancement toward the clinic."

CDX-6512 is a gastrointestinal-stable enzyme specifically engineered to be highly resistant to both the acidic conditions of the stomach and to proteases of the upper intestines, enabling it to effectively degrade methionine that is liberated from protein digestion. Elevated levels of this amino acid and its metabolite homocysteine, leads to the various clinical manifestations of HCU. The Company previously presented pre-clinical data highlighting the CDX-6512 program at the 14th International Congress of Inborn Errors of Metabolism (ICIEM) in November 2021.

ODD is granted by the FDA to drugs and biologics intended for the treatment, diagnosis or prevention of diseases or disorders that affect fewer than 200,000 people in the United States. The designation provides incentives for sponsors to develop products, which may include tax credits toward the cost of clinical trials and prescription drug user fee waivers. The ODD could also entitle Codexis to a seven-year period of marketing exclusivity in the United States for CDX-6512 should the company receive FDA approval for the treatment of homocystinuria for this product candidate.

RPD designation is granted by the FDA to drugs intended to treat serious or life-threatening diseases that primarily affect individuals aged from birth to 18 years and fewer than 200,000 persons in the United States.

About Homocystinuria (HCU)

Homocystinuria is a rare inborn error of metabolism most commonly due to cystathionine beta-synthase (CBS) deficiency and is characterized by elevated levels of homocysteine in blood and urine that when left untreated may lead to learning and intellectual disabilities, cardiovascular disease, osteoporosis, and stroke. Homocysteine is a metabolite derived from methionine, an essential amino acid that enters the body as part of dietary protein. Strict, life-long adherence to a methionine-restricted diet, often paired with vitamin supplementation (e.g., pyridoxine, folate, vitamin B12, betaine), is currently the only available therapy. According to the Genetic and Rare Disease Information Center (GARD), it is thought that world-wide about 1 in 150,000 people has HCU due to either a CBS or an MTHFR gene mutation. HCU is listed on the Recommended Uniform Screening Panel of disorders recommended by the Secretary of the Department of Health and Human Services for states to screen as part of their universal newborn screening programs.

About Codexis

Codexis is a leading enzyme engineering company leveraging its proprietary CodeEvolver® platform to discover and develop novel, high performance enzymes and novel biotherapeutics. Codexis enzymes have applications in the sustainable manufacturing of pharmaceuticals, food, and industrial products; in the creation of the next generation of life science tools; and as gene therapy and biologic therapeutics. The Company's unique performance enzymes drive improvements such as: reduced energy usage, waste generation and capital requirements; higher yields; higher fidelity diagnostics; and more efficacious therapeutics. Codexis enzymes enable the promise of synthetic biology to improve the health of people and the planet. For more information, visit www.codexis.com.

Forward-Looking Statements

To the extent that statements contained in this press release are not descriptions of historical facts regarding Codexis, they are forward-looking statements reflecting the current beliefs and expectations of management made pursuant to the safe harbor provisions of the Private Securities Litigation Reform Act of 1995. You should not place undue reliance on these forward-looking statements because they involve known and unknown risks, uncertainties and other factors that are, in some cases, beyond Codexis' control and that could materially affect actual results. Factors that could materially affect actual results include, among others: our biotherapeutic programs are early stage, highly regulated and expensive; our ability to obtain additional development partners for the programs, to advance our product candidates to clinical trials and to ultimately receive regulatory approvals is highly uncertain; the regulatory approval processes of the U.S. Food and Drug Administration and comparable foreign authorities are lengthy, time consuming and inherently unpredictable, and if we are unable to obtain or maintain regulatory approval for our products and product candidates, our business will be substantially harmed; results of preclinical studies and early clinical trials of product candidates may not be predictive of results of later studies or trials; our product candidates may not have favorable results in later clinical trials, if any, or receive regulatory approval; if any of our product candidates do not work as intended or cause undesirable side effects, it could hinder or prevent receipt of regulatory approval or realization of commercial potential for them or our other product candidates and could substantially harm our business; and even if we obtain regulatory approval for any products that we develop alone or with collaborators, such products will remain subject to ongoing regulatory requirements, which may result in significant additional expense. Additional information about factors that could materially affect actual results can be found in Codexis' Annual Report on Form 10-K filed with the Securities and Exchange Commission ("SEC") on March 1, 2021, and in Codexis' Quarterly Report on Form 10-Q filed with the SEC on

November 5, 2021, including under the caption “Risk Factors,” and in Codexis’ other periodic reports filed with the SEC. Codexis expressly disclaims any intent or obligation to update these forward-looking statements, except as required by law.

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