Roxadustat Granted Orphan Drug Designation for the Treatment of Myelodysplastic Syndromes by the U.S. Food and Drug Administration

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- *Company is on track to file the Phase 3 protocol in the fourth quarter of 2025*

SAN FRANCISCO, Dec. 15, 2025 (GLOBE NEWSWIRE) -- FibroGen, Inc. (NASDAQ: FGEN) today announced that the Office of Orphan Products Development of the U.S. Food and Drug Administration (FDA) has granted roxadustat Orphan Drug Designation for the treatment of myelodysplastic syndromes (MDS).

"The Orphan Drug Designation granted to roxadustat for MDS underscores the significant treatment gap in this indication, and highlights patients' need for additional convenient treatments that can provide durable response," said Thane Wettig, Chief Executive Officer of FibroGen. "Roxadustat showed an improvement in transfusion-independence in a subset of patients with high transfusion burden in a post-hoc analysis from the Phase 3 MATTHERHORN trial, which along with its favorable tolerability profile and oral route of administration has the ability to set it apart from current second-line treatments. Our team is finalizing the Phase 3 protocol in this patient population for submission to the FDA in the fourth quarter of 2025."

There are approximately 58,000 patients diagnosed with LR-MDS in the U.S. with 85% of them suffering from anemia. Anemia in patients with MDS is associated with increased risk of cardiovascular complications and the need for blood transfusions. Transfusion-dependent patients suffer higher rates of complications and decreased quality of life. Current first-line treatments lead to transfusion independence in less than 50% of patients and relief is often temporary with limited options for second line and beyond treatments. In a post-hoc analysis from the Phase 3 MATTERHORN trial, roxadustat demonstrated transfusion independence benefits compared to placebo in patients with high transfusion burden.

The FDA Orphan Drug Designation is granted to drugs intended for the treatment, diagnosis, or prevention of rare diseases that affect fewer than 200,000 people in the U.S. Benefits of the designation may include exemption from certain FDA fees, financial incentives for qualified clinical development, and seven years of market exclusivity in the U.S. following drug approval.

About Myelodysplastic Syndromes Anemia
Myelodysplastic syndromes (MDS) are a group of disorders characterized by dysfunctional progenitor blood cells and stem cells, resulting in chronic anemia in most patients. Annual incidence rates of MDS are estimated to be 4.9/100,000 adults in the U.S, of which 77% are considered lower-risk MDS. Approximately 80% of patients with MDS have anemia at the time of diagnosis, and around 60% of patients with MDS will experience severe anemia (hemoglobin <8 g/dL) at some point during the course of their disease. Anemia in patients with MDS is associated with increased risk of cardiovascular complications and the need for blood transfusion. Approximately 50% of patients with MDS require regular red blood cell transfusions. Transfusion-dependent MDS patients suffer higher rates of cardiac events, infections, and iron overload with the related complications. In addition, anemia frequently leads to significant fatigue, cognitive dysfunction, and decreased quality of life. Today, patients are routinely treated with erythropoiesis-stimulating agents (ESAs), luspatercept, imetelstat, or lenalidomide in lower-risk MDS with isolated del(5q), and hypomethylating agents (HMAs) in higher-risk disease. Only 35-40% of patients respond to current treatments and the durability of response is short. Moreover, these treatments are challenging to dose-calibrate and can only be administered via subcutaneous injection or through IV infusion. There remains a high unmet need for the treatment of anemia associated with MDS, and new strategies that provide durable response and the convenience of oral administration are highly desired in managing patients with MDS.

About Roxadustat
Roxadustat, an oral medication, is the first in a new class of medicines comprising HIF-PH inhibitors that promote erythropoiesis, or red blood cell production, through increased endogenous production of erythropoietin, improved iron absorption and mobilization, and downregulation of hepcidin.

Roxadustat is approved in Europe, Japan, and numerous other countries for the treatment of anemia of CKD in adult patients on dialysis (DD) and not on dialysis (NDD). FibroGen has the sole rights to roxadustat in the United States, Canada, Mexico, and in all markets not held by AstraZeneca or licensed to Astellas. Astellas and FibroGen are collaborating on the commercialization of roxadustat for the treatment of anemia in territories including Japan, Europe, Turkey, Russia, and the Commonwealth of Independent States, the Middle East, and South Africa.

About FibroGen
FibroGen, Inc. is a biopharmaceutical company focused on development of novel therapies at the frontiers of cancer biology and anemia. Roxadustat (爱瑞卓®, EVRENZO™) is currently approved in Europe, Japan, and numerous other countries for the treatment of anemia in chronic kidney disease (CKD) patients on dialysis and not on dialysis. The Company continues to evaluate

the development plan for the Phase 3 trial of roxadustat in anemia associated with lower-risk myelodysplastic syndrome (LR-MDS) in the U.S. FG-3246 (also known as FOR46), a first-in-class antibody-drug conjugate (ADC) targeting CD46, is in Phase 2 development for the treatment of metastatic castration-resistant prostate cancer. This program also includes the development of FG-3180, an associated CD46-targeted PET biomarker. For more information, please visit www.fibrogen.com.

Forward-Looking Statements

This release contains forward-looking statements regarding FibroGen's strategy, future plans and prospects, including statements regarding its commercial products and clinical programs and those of it and its collaboration partners Fortis and UCSF. These forward-looking statements include, but are not limited to, statements regarding the efficacy, safety, convenience, and potential clinical or commercial success of FibroGen products and product candidates, statements about regulatory interactions, and statements about FibroGen's plans and objectives. These forward-looking statements are typically identified by use of terms such as "may," "will", "should," "on track," "could," "expect," "plan," "anticipate," "believe," "estimate," "predict," "potential," "continue" and similar words, although some forward-looking statements are expressed differently. FibroGen's actual results may differ materially from those indicated in these forward-looking statements due to risks and uncertainties related to the continued progress and timing of its various programs, including the enrollment and results from ongoing and potential future clinical trials, and other matters that are described in FibroGen's most recent Annual Report on Form 10-K and Quarterly Report on Form 10-Q, each as filed with the Securities and Exchange Commission (SEC), including the risk factors set forth therein. Investors are cautioned not to place undue reliance on these forward-looking statements, which speak only as of the date of this release, and FibroGen undertakes no obligation to update any forward-looking statement in this press release, except as required by law.

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