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Rigel Announces R289 Granted Orphan Drug Designation by the FDA for MDS

SOUTH SAN FRANCISCO, Calif., Jan. 9, 2025 /PRNewswire/ -- Rigel Pharmaceuticals, Inc. (Nasdaq: RIGL), a commercial stage biotechnology company focused on hematologic disorders and cancer, today announced that the U.S. Food and Drug Administration (FDA) has granted Orphan Drug designation to R289 for the treatment of myelodysplastic syndromes (MDS). R289[1], Rigel's potent and selective dual inhibitor of IRAK1 and IRAK4, is being studied in an ongoing Phase 1b study evaluating the safety, tolerability, pharmacokinetics and preliminary activity in patients with LR-MDS who are relapsed or refractory to prior therapies.

"Receiving Orphan Drug designation for R289 supports the development of this therapeutic candidate for the treatment of MDS and highlights the significant unmet medical need that exists for these patients," said Raul Rodriguez, Rigel's president and CEO. "Orphan Drug and Fast Track designations, along with encouraging initial data from our ongoing Phase 1b study in patients with lower-risk MDS, represent significant milestones in the advancement of R289 as a potential new treatment option."

The FDA Office of Orphan Products Development grants orphan drug designation to support the development of medicines for rare disorders that affect fewer than 200,000 people in the U.S. Under the Orphan Drug Act, orphan drug designation qualifies a company for incentives including tax credits, exemptions from certain FDA fees for clinical trials and the potential for seven years of market exclusivity following drug approval.

R289 was previously granted Fast Track designation by the FDA for the treatment of patients with previously-treated transfusion dependent lower-risk MDS.

About R289

R289 is a prodrug of R835, an IRAK1/4 dual inhibitor, which has been shown in preclinical studies to block inflammatory cytokine production in response to toll-like receptor (TLR) and interleukin-1 receptor (IL-1R) family signaling. TLRs and IL-1Rs play a critical role in the innate immune response and dysregulation of these pathways can lead to various inflammatory conditions. Chronic stimulation of both these receptor systems is thought to cause the pro-inflammatory environment in the bone marrow responsible for persistent cytopenias in lower-risk MDS patients.[2]

About Rigel

Rigel Pharmaceuticals, Inc. (Nasdaq: RIGL) is a biotechnology company dedicated to discovering, developing and providing novel therapies that significantly improve the lives of patients with hematologic disorders and cancer. Founded in 1996, Rigel is based in South San Francisco, California. For more information on Rigel, the Company's marketed products and pipeline of potential products, visit www.rigel.com/.

1. R289 is an investigational compound not approved by the FDA.
2. Sallman DA et al. *Unraveling the Pathogenesis of MDS: The NLRP3 Inflammasome and Pyroptosis Drive the MDS Phenotype*. Front Oncol. June 16, 2016. DOI: https://doi.org/10.3389/fonc.2016.00151

Forward Looking Statements

This press release contains forward-looking statements relating to, among other things, the possible advantages of Orphan Drug and Fast Track designations, the potential benefits of R289 as a therapeutic for MDS and LR-MDS, the existence of patients with an unmet medical need for such therapy, certain potential benefits associated with orphan drug designation, and Rigel's ability to further develop its clinical stage product candidates, including the encouragement of initial data from, and progress of, current clinical trials of R289, and the potential for future trials. Any statements contained in this press release that are not statements of historical fact may be deemed to be forward-looking statements and as such are intended to be covered by the safe harbor for "forward-looking statements" provided by the PSLRA. Forward-looking statements can be identified by words such as "plan", "potential", "may", "look to", "expects", "will" and similar expressions in reference to future periods. Forward-looking statements are neither historical facts nor assurances of future performance. Instead, they are based on Rigel's current beliefs, expectations, and assumptions and hence they inherently involve significant risks, uncertainties and changes in circumstances that are difficult to predict and many of which are outside of Rigel's control. Therefore, you should not rely on any of these forward-looking statements. Actual results and the timing of events could differ materially from those anticipated in such forward-looking statements as a result of these risks and uncertainties, which include, without limitation, Fast Track and Orphan Drug designations may not result in a more expedited development or regulatory review process, and such a designation does not increase the likelihood that R289 will receive marketing approval in the United States; Fast Track and Orphan Drug designations do not change the standards for regulatory approval; the FDA may later decide that R289 no longer meets the conditions for qualification or decide that the time period for FDA review or approval will not be shortened; risks and uncertainties associated with the commercialization and marketing of R289; risks that the FDA or other regulatory authorities may make adverse decisions regarding R289; risks that clinical trials may not be predictive of real-world results or of results in subsequent clinical trials; risks that R289 may have unintended side effects, adverse reactions or incidents of misuses; the availability of resources to develop Rigel's product candidates; market competition; as well as other risks detailed from time to time in Rigel's reports filed with the Securities and Exchange Commission, including its Quarterly Report on Form 10-Q for the quarter ended September 30, 2024 and subsequent filings. Any forward-looking statement made by us in this press release is based only on information currently available to us and speaks only as of the date on which it is made. Rigel does not undertake any obligation to update forward-looking statements, whether written or oral, that may be made from time to time, whether as a result of new information, future developments or otherwise, and expressly disclaims any obligation or undertaking to release publicly any updates or revisions to any forward-looking

statements contained herein, except as required by law.

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