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Syros Receives FDA Orphan Drug Designation for Tamibarotene for the Treatment of MDS

CAMBRIDGE, Mass.--(BUSINESS WIRE)-- Syros Pharmaceuticals (NASDAQ:SYRS), a leader in the development of medicines that control the expression of genes, today announced that the U.S. Food and Drug Administration (FDA) has granted orphan drug designation (ODD) to tamibarotene for the treatment of myelodysplastic syndrome (MDS). Tamibarotene, an oral first-in-class selective retinoic acid receptor alpha (RAR α) agonist, is currently being evaluated in combination with azacitidine in the SELECT-MDS-1 Phase 3 trial for RARA-positive patients with newly diagnosed higher-risk MDS (HR-MDS).

“The FDA’s orphan drug designation is an important milestone in the development of tamibarotene as a treatment for MDS,” said David A. Roth, M.D., Syros’ Chief Medical Officer. “We believe tamibarotene’s novel mechanism of action, promising clinical activity data, oral delivery, and favorable tolerability profile supports a potential new option for the approximately 30% of HR-MDS patients who are RARA-positive. We are focused on developing the first potential therapy for a targeted population in HR-MDS as we continue to advance our ongoing SELECT-MDS-1 pivotal trial.”

The FDA's Office of Orphan Drug Products grants orphan status to support development of medicines for the treatment of rare diseases that affect fewer than 200,000 people in the United States. Orphan drug designation may provide certain benefits, including a seven-year period of market exclusivity if the drug is approved, tax credits for qualified clinical trials and an exemption from FDA application fees.

The ongoing SELECT-MDS-1 Phase 3 clinical trial is evaluating the safety and efficacy of tamibarotene in combination with azacitidine for RARA-positive patients with newly diagnosed HR-MDS. Data from the pivotal trial are expected in the fourth quarter of 2023 or the first quarter of 2024, with a potential new drug application filing expected in 2024.

Syros is also evaluating tamibarotene in combination with azacitidine and venetoclax for RARA-positive patients with newly diagnosed unfit acute myeloid leukemia (AML), for which tamibarotene had previously received orphan drug designation. Safety lead-in data from the ongoing SELECT-AML-1 Phase 2 trial is expected in the second half of this year.

About Syros Pharmaceuticals

Syros is redefining the power of small molecules to control the expression of genes. Based on its unique ability to elucidate regulatory regions of the genome, Syros aims to develop medicines that provide a profound benefit for patients with diseases that have eluded other genomics-based approaches. Syros is advancing a robust clinical-stage pipeline, including: tamibarotene, a first-in-class oral selective RAR α agonist in RARA-positive patients with

higher-risk myelodysplastic syndrome and acute myeloid leukemia; SY-2101, a novel oral form of arsenic trioxide in patients with acute promyelocytic leukemia; and SY-5609, a highly selective and potent oral CDK7 inhibitor in patients with select solid tumors and blood cancers. Syros also has multiple preclinical and discovery programs in oncology and monogenic diseases. For more information, visit www.syros.com and follow us on Twitter ([@SyrosPharma](https://twitter.com/SyrosPharma)) and [LinkedIn](https://www.linkedin.com/company/syros-pharma).

Cautionary Note Regarding Forward-Looking Statements

This press release contains forward-looking statements within the meaning of The Private Securities Litigation Reform Act of 1995, including without limitation statements regarding Syros' clinical development plans with respect to tamibarotene, the potential of tamibarotene to benefit RARA-positive HR-MDS patients and to become the first approved therapy in a targeted population in HR-MDS, the timing of anticipated data readouts and potential regulatory submissions from Syros' clinical trials, and the potential for Syros's product candidates to obtain regulatory approval. The words "anticipate," "believe," "continue," "could," "estimate," "expect," "hope," "intend," "may," "plan," "potential," "predict," "project," "target," "should," "would," and similar expressions are intended to identify forward-looking statements, although not all forward-looking statements contain these identifying words. Actual results or events could differ materially from the plans, intentions and expectations disclosed in these forward-looking statements as a result of various important factors, including Syros' ability to: advance the development of its programs, including tamibarotene, under the timelines it projects in current and future clinical trials; demonstrate in any current and future clinical trials the requisite safety, efficacy and combinability of its drug candidates; sustain the response rates and durability of response seen to date with its drug candidates; successfully develop a companion diagnostic test to identify patients with the RARA biomarker; obtain and maintain patent protection for its drug candidates and the freedom to operate under third party intellectual property; obtain and maintain necessary regulatory approvals; identify, enter into and maintain collaboration agreements with third parties; manage competition; manage expenses; raise the substantial additional capital needed to achieve its business objectives; attract and retain qualified personnel; and successfully execute on its business strategies; risks described under the caption "Risk Factors" in Syros' Annual Report on Form 10-K for the year ended December 31, 2020 and Quarterly Report on Form 10-Q for the quarter ended September 30, 2021, each of which is on file with the Securities and Exchange Commission; and risks described in other filings that Syros makes with the Securities and Exchange Commission in the future. In addition, the extent to which the COVID-19 pandemic continues to impact Syros' workforce and its clinical trial operations activities, and the operations of the third parties on which Syros relies, will depend on future developments, which are highly uncertain and cannot be predicted with confidence, including the duration and severity of the pandemic, additional or modified government actions, and the actions that may be required to contain the virus or treat its impact. Any forward-looking statements contained in this press release speak only as of the date hereof, and Syros expressly disclaims any obligation to update any forward-looking statements, whether because of new information, future events or otherwise.

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Media Contact

Courtney Solberg
Syros Pharmaceuticals
917-698-9253
csolberg@syros.com

Investor Contact

Hannah Deresiewicz
Stern Investor Relations, Inc.
212-362-1200
hannah.deresiewicz@sternir.com

Source: Syros Pharmaceuticals