IDEAYA Biosciences Announces Successful FDA Type D Meeting on Phase 3 Registrational Trial Design for Darovasertib as Neoadjuvant Therapy for Primary Uveal Melanoma

- Targeting to initiate Phase 3 randomized registrational trial for darovasertib in the neoadjuvant setting in primary UM in H1 2025
- Clinical endpoints supportive of full approval based on FDA Type D Meeting: Eye preservation rate as the primary endpoint for enucleation patients. Proportion of patients with best corrected visual acuity 15-letter loss from time of randomization and time of completion of plaque brachytherapy (PB) as the primary endpoint for PB patients. No detriment to Event-Free-Survival (EFS) is required for both cohorts as a secondary endpoint
- We project registrational trial enrollment for the enucleation cohort will be 120 patients and 400 patients for the plaque brachytherapy cohort with a 2:1 randomization
- Potential to submit the enucleation cohort data for regulatory review earlier than the PB cohort pending the EFS data maturity in both cohorts

SOUTH SAN FRANCISCO, Calif., April 14, 2025 /PRNewswire/ -- IDEAYA Biosciences, Inc. (Nasdaq:IDYA), a precision medicine oncology company committed to the discovery and development of targeted therapeutics, announced a successful FDA Type D meeting on the Phase 3 registrational trial design that will assess the safety and efficacy of darovasertib for potential regulatory approval as neoadjuvant therapy for primary uveal melanoma (UM).

"The successful FDA Type D meeting provides darovasertib a registrational path as neoadjuvant therapy for UM, using primary clinical endpoints of eye preservation and proportion of patients with vision loss, with no detriment to EFS as a secondary endpoint required for both cohorts. Based on the promising clinical efficacy and safety observed with darovasertib in the neoadjuvant setting in over 90 patients and the recent Breakthrough Therapy Designation by the US FDA, we are excited to advance the darovasertib program into our second registrational trial," said Dr. Darrin Beaupre, M.D., Ph.D., Chief Medical Officer, IDEAYA Biosciences.

Darovasertib is a potent and selective protein kinase C (PKC) inhibitor being developed to broadly address primary and metastatic UM (MUM). Darovasertib has received U.S. FDA Breakthrough Therapy Designation as neoadjuvant therapy in enucleation recommended primary UM and Fast Track designation for darovasertib in combination with crizotinib in adult patients with metastatic uveal melanoma (MUM), where a Phase 2/3 registration-enabling trial of the darovasertib and crizotinib combination in 1L HLA-A2-negative MUM is ongoing. Darovasertib has also been designated as an Orphan Drug by the U.S. FDA in UM, including in MUM.

FDA Guidance from the Type D Meeting on the Phase 3 Neoadjuvant Darovasertib Registrational Trial Design for Potential Regulatory Approval in Primary UM

IDEAYA is targeting to initiate the Phase 3 randomized clinical trial evaluating neoadjuvant darovasertib in primary UM in the first half of 2025. The randomized Phase 3 clinical trial design incorporates guidance and feedback from the U.S. FDA following a recent Type D meeting.

In the Phase 3 clinical trial, we currently project approximately 520 patients will be randomized 2:1 to the darovasertib treatment versus control arm. There will be 2 cohorts enrolled: 1) 120 enucleation eligible UM patients, 2) 400 PB eligible UM patients. For the enucleation cohort, the randomization will be with or without darovasertib as neoadjuvant therapy. For the PB cohort, the randomization will be darovasertib followed by PB versus PB alone.

Key highlights of the Phase 3 registrational trial design in neoadjuvant UM, based on FDA guidance:

- Eye preservation rate (exceed lower bound of 10% eye preservation rate with a 95% confidence interval) is the primary endpoint for the enucleation UM cohort
- Proportion of patients with vision loss from the time of randomization and time of completion of PB is the primary endpoint for the plaque brachytherapy cohort. Vision detriment will be measured by the Early Treatment Diabetic Retinopathy Study (ETDRS) Best-Corrected Visual Acuity (BCVA) of ≥15-letters lost
- No detriment to Event-Free-Survival (EFS) is a secondary endpoint for both cohorts and is required for approval. No detriment is defined as overlapping confidence intervals
- Additional secondary endpoints: Overall Response Rate (≥20% ocular tumor shrinkage by product of diameters), proportion of patients with clinically significant macular edema, proportion of subjects with 20/200 vision loss or worse (legal blindness), proportion of subjects with reduction of radiation dose of ≥20% delivered to key eye structures
- Potential to submit the enucleation cohort data for regulatory review earlier than the PB cohort pending the EFS data maturity in both cohorts
- 300mg BID darovasertib will be the move-forward dose for the registrational trial

About IDEAYA Biosciences

IDEAYA is a precision medicine oncology company committed to the discovery and development of targeted therapeutics for patient populations selected using molecular diagnostics. IDEAYA's approach integrates capabilities in identifying and validating translational biomarkers with drug discovery to select patient populations most likely to benefit from its targeted therapies. IDEAYA is applying its research and drug discovery capabilities to synthetic lethality – which represents an emerging class of precision medicine targets.

Forward-Looking Statements

This press release contains forward-looking statements, including, but not limited to, statements related to: (i) the potential regulatory approval of darovasertib as neoadjuvant therapy for primary UM; (ii) the potential therapeutic benefit, including safety and efficacy, of darovasertib; and (iii) the design and timing of the Phase 3 clinical trial of darovasertib for neoadjuvant for primary UM. Such forward-looking statements involve substantial risks and uncertainties that could cause IDEAYA's preclinical and clinical development programs, 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 inherent in the drug development process, including IDEAYA's programs' early stage of development, the process of designing and conducting preclinical and clinical trials, the regulatory approval processes, the timing of regulatory filings, the challenges associated with manufacturing drug products, IDEAYA's ability to successfully establish, protect and defend its intellectual property, and other matters that could affect the sufficiency of existing cash to fund operations. IDEAYA 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 the business of IDEAYA in general, see IDEAYA's Annual Report on Form 10-K dated February 20, 2024 and any current and periodic reports filed with the U.S. Securities and Exchange Commission.

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