Tyra Biosciences Announces FDA Orphan Drug Designation for TYRA-300 for the Treatment of Achondroplasia

CARLSBAD, Calif., Aug. 1, 2023 /PRNewswire/ -- Tyra Biosciences, Inc. (Nasdaq: TYRA), a clinical-stage biotechnology company focused on developing next-generation precision medicines that target large opportunities in Fibroblast Growth Factor Receptor (FGFR) biology, today announced that the U.S. Food and Drug Administration (FDA) has granted Orphan Drug Designation (ODD) to its lead precision medicine program, TYRA-300, for the treatment of achondroplasia.

Achondroplasia is the most common form of dwarfism with limited therapeutic options. People living with achondroplasia may experience severe skeletal complications including cranial and spinal stenosis, hydrocephalus and sleep apnea. A specific mutation in FGFR3 causes over 97% of achondroplasia. TYRA-300 is an oral FGFR3 selective inhibitor whose design may have a meaningful impact on achondroplasia and other skeletal dysplasias.

"People living with achondroplasia can have significant health complications that are not adequately addressed with currently available therapies. Our goals with TYRA-300 in achondroplasia are to address not only height, but the long-term health complications associated with this condition," said Hiroomi Tada, M.D. Ph.D., Chief Medical Officer of TYRA. "The FDA's decision to grant Orphan Drug Designation to TYRA-300 is an important recognition of the potential of our approach to deliver benefit to the achondroplasia community. We remain on track to submit an IND to the FDA to enable a Phase 2 study of TYRA-300 in pediatric achondroplasia in 2024."

The FDA's Office of Orphan Products Development grants orphan designation status to drugs and biologics that are intended for treatment, diagnosis or prevention of rare diseases and conditions that affect fewer than 200,000 people in the U.S. Orphan Drug Designation provides certain benefits, including tax credits for qualified clinical trials and exemption from certain user fees to support clinical development and the potential for up to seven years of market exclusivity in the U.S. upon regulatory approval.

TYRA also announced today the appointment of Michael Bober, M.D. Ph.D., as Vice President, Clinical Development and Medical Affairs, to lead the skeletal dysplasia program. Dr. Bober is a leader in the diagnosis and management of skeletal dysplasia. He served on numerous scientific and medical advisory boards within the skeletal dysplasia community. Dr. Bober joins TYRA following a distinguished career as the Medical Director of the Skeletal Dysplasia Program, Nemours Children's Hospital, Delaware.

Dr. Bober added, "I am excited to join TYRA and contribute to the team working to develop TYRA-300 into a therapy for the patient community which I care so much about. I believe TYRA-300 has the potential to improve function and quality of life in achondroplasia."

About TYRA-300

TYRA-300 is the Company's lead precision medicine program stemming from its in-house SNÅP platform. TYRA-300 is an investigational, oral, FGFR3-selective inhibitor currently in development for the treatment of cancer and skeletal dysplasias including achondroplasia. TYRA-300 is being evaluated in a multi-center, open label Phase 1/2 clinical study, SURF301 (**S**tudy in **U**ntreated and **R**esistant **F**GFR3+ Advanced Solid Tumors). SURF301 (NCT05544552) was designed to determine the optimal and maximum tolerated doses (MTD) and the recommended Phase 2 dose (RP2D) of TYRA-300, as well as to evaluate the preliminary antitumor activity of TYRA-300. SURF301 is currently enrolling adults with advanced urothelial carcinoma and other solid tumors with FGFR3 gene alterations. In skeletal dysplasias, TYRA-300 has demonstrated positive preclinical results and the Company expects to submit an IND for the initiation of a Phase 2 clinical study in pediatric achondroplasia in 2024.

About Tyra Biosciences

Tyra Biosciences, Inc. (Nasdaq: TYRA) is a clinical-stage biotechnology company focused on developing next-generation precision medicines that target large opportunities in FGFR biology. The Company's in-house precision medicine platform, SNÅP, enables rapid and precise drug design through iterative molecular SNÅPshots that help predict genetic alterations most likely to cause acquired resistance to existing therapies. TYRA's initial focus is on applying its accelerated small molecule drug discovery engine to develop therapies in targeted oncology and genetically defined conditions. TYRA is based in Carlsbad, CA. For more information

about our science, pipeline and people, please visit www.tyra.bio and engage with us on LinkedIn.

Forward-Looking Statements

TYRA cautions you that statements contained in this press release regarding matters that are not historical facts are forward-looking statements. The forward-looking statements are based on our current beliefs and expectations and include, but are not limited to: the potential to develop next-generation precision medicines and the potential safety and therapeutic benefits of TYRA-300 and other product candidates, including the potential for TYRA-300 to become a treatment option for achondroplasia; the expected timing and phase of clinical development of TYRA-300, including timing of a submission of an IND for TYRA-300 in pediatric achondroplasia; and the potential for SNÅP to enable rapid and precise drug design. Actual results may differ from those set forth in this press release due to the risks and uncertainties inherent in our business, including, without limitation: we are early in our development efforts, have only recently begun testing our lead product candidate in clinical trials and the approach we are taking to discover and develop drugs based on our SNÅP platform is novel and unproven and it may never lead to product candidates that are successful in clinical development or approved products of commercial value; potential delays in the commencement, enrollment, and completion of preclinical studies and clinical trials; results from preclinical studies or early clinical trials not necessarily being predictive of future results; our dependence on third parties in connection with manufacturing, research and preclinical testing; acceptance by the FDA of INDs or of similar regulatory submissions by comparable foreign regulatory authorities for the conduct of clinical trials of TYRA-300 in pediatric achondroplasia; an accelerated development or approval pathway may not be available for TYRA-300 or other product candidates and any such pathway may not lead to a faster development process; unexpected adverse side effects or inadequate efficacy of our product candidates that may limit their development, regulatory approval, and/or commercialization; the potential for our programs and prospects to be negatively impacted by developments relating to our competitors, including the results of studies or regulatory determinations relating to our competitors; we may not realize the benefits associated with ODD, including that orphan drug exclusivity may not effectively protect a product from competition and that such exclusivity may not be maintained; regulatory developments in the United States and foreign countries; we may use our capital resources sooner than we expect; unstable market and economic conditions and adverse developments with respect to financial institutions and associated liquidity risk may adversely affect our business and financial condition and the broader economy and biotechnology industry; and other risks described in our prior filings with the Securities and Exchange Commission (SEC), including under the heading "Risk Factors" in our annual report on Form 10-K and any subsequent filings with the SEC. You are cautioned not to place undue reliance on these forward-looking statements, which speak only as of the date hereof, and we undertake no obligation to update such statements to reflect events that occur or circumstances that exist after the date hereof. All forward-looking statements are qualified in their entirety by this cautionary statement, which is made under the safe harbor provisions of the Private Securities Litigation Reform Act of 1995.

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