# UNITY Biotechnology Announces Positive 24-Week Data from Phase 2 BEHOLD Study of UBX1325 in Patients with Diabetic Macular Edema

November 1, 2022

*A single injection of UBX1325 led to a statistically significant and clinically relevant improvement in Best Corrected Visual Acuity (BCVA) of 7.6 ETDRS letters at 24 weeks compared to sham treatment*

*UBX1325 maintained stabilization of retinal structure, as measured by central subfield thickness (CST) at 24 weeks, as compared to worsening from baseline in sham-treated patients*

*The proportion of rescue-free patients in the UBX1325-treated arm was 59.4% at 6 months after a single injection, as compared to only 37.5% in the sham-treated arm*

*UNITY to host investor call with retinal expert Arshad M. Khanani, M.D., M.A., FASRS, today, November 1, at 8:00 a.m. ET*

SOUTH SAN FRANCISCO, Calif., Nov. 01, 2022 (GLOBE NEWSWIRE) -- UNITY Biotechnology, Inc. ("UNITY") [Nasdaq: UBX], a biotechnology company developing therapeutics to slow, halt, or reverse diseases of aging, today announced that the key safety and efficacy endpoints were met at 24 weeks in the Phase 2 BEHOLD study of UBX1325 in patients with diabetic macular edema (DME). The Company intends to initiate a pivotal study in DME in the second half of 2023.

At 24 weeks after a single dose of UBX1325, the mean change in BCVA of UBX1325-treated subjects was an increase of +6.2 ETDRS letters, representing an improvement of +7.6 ETDRS letters compared to sham-treated subjects from baseline ($p = 0.0084$). In addition, patients treated with UBX1325 maintained CST compared to sham-treated patients who demonstrated worsening of CST (i.e., increased retinal thickness) through 24 weeks. Of patients treated with UBX1325, 59.4% did not require anti-VEGF standard of care through 6 months, as compared to only 37.5% of sham-treated patients.

"It is remarkable to see such clinically meaningful and sustained improvements in vision after a single injection of UBX1325 in patients who had reached a therapeutic plateau with anti-VEGF treatment," said Anirvan Ghosh, Ph.D., chief executive officer of UNITY. "In today's treatment paradigm for DME, most patients require frequent injections – and still a large proportion of patients with the current standard of care have residual visual deficits. Based on the results of this study we believe UBX1325 could lead to significant vision gain while reducing treatment burden for patients. The durable effect we've now observed through 6 months following just a single injection of UBX1325 suggests it could represent a longer-lasting, disease-modifying treatment option for patients."

Patients enrolled in BEHOLD had been on anti-VEGF treatment for at least 6 months prior to enrollment into the study (mean injection frequency of 4 in the preceding 6 months), with the last anti-VEGF injection occurring 3 – 6 weeks prior to randomization.

**Evidence of favorable safety, visual acuity improvement, and structural stability in a difficult-to-treat patient population at 24 weeks:**

- UBX1325 demonstrated a favorable safety and tolerability profile with no cases of intraocular inflammation, retinal artery occlusion, endophthalmitis, or vasculitis
- Patients treated with a single injection of UBX1325 had a mean improvement in BCVA of +7.6 letters compared to sham ($p=0.0084$). UBX1325-treated patients gained +6.2 ETDRS letters from baseline compared to a loss of -1.4 ETDRS letters in sham-treated patients
- Patients treated with UBX1325 had a mean change in CST of -5.4 microns from baseline compared to an increase (worsening) of +34.6 microns in sham-treated patients for a total difference of 40.0 microns ($p=0.1244$)
- 59.4% of UBX1325-treated patients went 6 months without receiving any anti-VEGF rescue compared to 37.5% of sham-treated patients

"A 7.6-letter gain in BCVA from baseline in UBX1325-treated patients who had visual deficits and retinal fluid despite being on anti-VEGF treatment is a clinically meaningful and impressive outcome," said Arshad M. Khanani, M.D., M.A., Director of Clinical Research at Sierra Eye Associates. "A potential treatment with a novel mechanism of action that provides significant and durable gain in vision would be of great value to patients with DME."

Jamie Dananberg, M.D., chief medical officer of UNITY added, "We believe we have altered the disease trajectory of patients treated with UBX1325, as evidenced by the 24-week results. Observing significantly greater letter gains and stabilization of retinal structure compared to the sham arm after a single injection of UBX1325 is encouraging and speaks to the potential of disease modification with a senolytic treatment. We look forward to further evaluating the durability of treatment effect through 48 weeks with our long-term extension of the BEHOLD study."

**Upcoming Clinical Milestones for UBX1325**

- 48-week long-term safety and efficacy data from Ph2 BEHOLD study in DME expected in Q2 2023
- 16-week safety and efficacy data from Ph2 ENVISION study in wet age-related macular degeneration (AMD) expected in Q1 2023, and 24-week safety and efficacy data expected in Q2 2023

**Conference Call at 8:00 a.m. ET Today**
UNITY will host a video conference call and webcast for investors and analysts today at 8:00 a.m. ET to discuss the most recent UBX1325 clinical data. Dr. Arshad M. Khanani, M.D., M.A., FASRS, Director of Clinical Research at Sierra Eye Associates, as well as members of the UNITY senior management team, will lead the discussion on the 24-week BEHOLD study results. The live webcast can be accessed in the "Investors and Media" section of our website, www.unitybiotechnology.com, under "Events & Presentations" or by clicking here. A replay will be available two hours after the completion of the call and can be accessed in the "Investors & Media" section of our website, under "Events and Presentations."

**About the BEHOLD Study**
The proof-of-concept Phase 2 BEHOLD study is a multi-center, randomized, double-masked, sham-controlled study designed to evaluate the safety, tolerability, efficacy and durability of a single 10 mcg dose of UBX1325 in patients with DME evaluated though 24 weeks. The study enrolled 65 patients being actively treated with anti-VEGF who had a visual acuity deficit (73 ETDRS letters, approximately 20/40, or worse) and residual retinal fluid (CST ≥300 microns). Patients have the option of continuing in the long-term extension portion of the study through 48-weeks. To date, a majority of patients have opted to remain in the study. More information about the study is available here (NCT04857996).

**About UBX1325**
UBX1325 is an investigational compound being studied for age-related diseases of the eye, including diabetic macular edema (DME), age-related macular degeneration (AMD), and diabetic retinopathy (DR) that is not approved for any use in any country. UBX1325 is a potent small molecule inhibitor of Bcl-xL, a member of the Bcl-2 family of apoptosis regulating proteins. UBX1325 is designed to inhibit the function of proteins that senescent cells rely on for survival. In a Phase 1 clinical study in advanced wet AMD and DME, UBX1325 showed a favorable safety profile and improvements in visual acuity sustained through 24 weeks following a single intravitreal injection. In preclinical studies, UNITY has demonstrated that targeting Bcl-xL with UBX1325 preferentially eliminated senescent cells from diseased tissue while sparing cells in healthy tissue. UNITY's goal with UBX1325 is to transformationally improve real-world outcomes for patients with DME, AMD, and DR.

**About UNITY**
UNITY is developing a new class of therapeutics to slow, halt, or reverse diseases of aging. UNITY's current focus is on creating medicines to selectively eliminate or modulate senescent cells and thereby provide transformative benefit in age-related ophthalmologic and neurologic diseases. More information is available at www.unitybiotechnology.com or follow us on Twitter and LinkedIn.

**Forward-Looking Statements**
This press release contains forward-looking statements including statements related to UNITY's understanding of cellular senescence and the role it plays in diseases of aging, the potential for UNITY to develop therapeutics to slow, halt, or reverse diseases of aging, including for ophthalmologic and neurologic diseases, our expectations regarding potential benefits, activity, effectiveness, and safety of UBX1325, the potential for UNITY to successfully commence and complete clinical studies of UBX1325 for DME, AMD, and other ophthalmologic diseases, the expected timing and nature of results of our studies of UBX1325 including BEHOLD and ENVISION, including the risk that interim results of our clinical studies may not be indicative of future results, the timing of the expected commencement, progression, and conclusion of our studies including those of UBX1325, and UNITY's expectations regarding the sufficiency of its cash runway. These statements involve substantial known and unknown risks, uncertainties, and other factors that may cause our actual results, levels of activity, performance, or achievements to be materially different from the information expressed or implied by these forward-looking statements, including the risk that the COVID-19 worldwide pandemic may continue to negatively impact the development of preclinical and clinical drug candidates, including delaying or disrupting the enrollment of patients in clinical trials, risks relating to the uncertainties inherent in the drug development process, and risks relating to UNITY's understanding of senescence biology. We may not actually achieve the plans, intentions, or expectations disclosed in our forward-looking statements, and you should not place undue reliance on our forward-looking statements. Actual results or events could differ materially from the plans, intentions, and expectations disclosed in the forward-looking statements we make. The forward-looking statements in this press release represent our views as of the date of this release. We anticipate that subsequent events and developments will cause our views to change. However, while we may elect to update these forward-looking statements at some point in the future, we have no current intention of doing so except to the extent required by applicable law. You should, therefore, not rely on these forward-looking statements as representing our views as of any date subsequent to the date of this release. 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 UNITY in general, see UNITY's most recently filed Quarterly Report on Form 10-Q for the quarter ended June 30, 2022, filed with the Securities and Exchange Commission on August 12, 2022, as well as other documents that may be filed by UNITY from time to time with the Securities and Exchange Commission.

**Media Contact**
Evoke Canale
Katherine Smith
Katherine.Smith@evokegroup.com

**Investor Contact**
LifeSci Advisors, LLC
Joyce Allaire
jallaire@lifesciadvisors.com


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