UNITY Biotechnology Announces Positive 48-Week Results from Phase 2 BEHOLD Study of UBX1325 in Patients with Diabetic Macular Edema

April 24, 2023

A single injection of UBX1325 led to a statistically significant and clinically meaningful improvement in Best Corrected Visual Acuity (BCVA) of +6.2 ETDRS letters from baseline at 48 weeks

Approximately 53% of UBX1325-treated patients did not require any additional injections through 48 weeks

Retinal structure, as measured by central subfield thickness (CST), was maintained in UBX1325-treated patients throughout the duration of the study

UBX1325 had a favorable safety and tolerability profile with no evidence of intraocular inflammation

UNITY to host investor call with retinal expert
Robert B. Bhisitkul, M.D., Ph.D., today, April 24 at 8:00 a.m. ET

SOUTH SAN FRANCISCO, Calif., April 24, 2023 (GLOBE NEWSWIRE) -- UNITY Biotechnology, Inc. ("UNITY") [Nasdaq: UBX], a biotechnology company developing therapeutics to slow, halt, or reverse diseases of aging, today announced positive results from the long-term follow-up of the Phase 2 BEHOLD study of UBX1325 in patients with diabetic macular edema (DME). A single injection of UBX1325 treatment led to a statistically significant improvement in vision lasting for the duration of the study (48 weeks), marked by a gain of +6.2 ETDRS letters from baseline, representing a difference of +5.6 ETDRS letters compared to sham-treated patients. In addition, patients treated with UBX1325 maintained stable CST compared to worsening in sham-treated patients.

"This is a defining moment for the senolytic therapeutic hypothesis and is a pivotal moment for UNITY. Achieving sustained improvements in visual acuity and stabilization of retinal structure for almost 1 year after a single injection of UBX1325 is a remarkable result," said Anirvan Ghosh, Ph.D., chief executive officer of UNITY. "UBX1325 is the only treatment candidate in clinical development that targets senescent cells to potentially modify the course of disease, and this therapeutic approach could redefine the standard of care in DME. Based on the strong emerging clinical profile of UBX1325, we are planning to move forward with our Phase 2b DME head-to-head study against aflibercept in the second half of 2023."

The BEHOLD study enrolled patients who, despite being on anti-VEGF treatment for at least 6 months, displayed persistent visual acuity deficits and residual retinal fluid. At baseline, patients in the study had an average visual acuity of 61.4 ETDRS letters and a CST of approximately 439.6 microns. In the 6 months prior to study enrollment, patients received an average of 4 anti-VEGF injections, with the last anti-VEGF injection occurring 3-6 weeks prior to randomization. Fifty patients completed the 48-week study extension.

48-Week Phase 2 BEHOLD Data:

- UBX1325 demonstrated a favorable safety and tolerability profile with no cases of intraocular inflammation, retinal artery occlusion, endophthalmitis, or vasculitis
- Patients treated with UBX1325 had a mean change in BCVA of +6.2 ETDRS letters from baseline to 48 weeks (p=0.0037), representing a difference of +5.6 ETDRS letters compared to sham-treated patients (p=0.1198)
- Based on an analysis of the BCVA change from baseline to last observation before anti-VEGF rescue or end of study participation, UBX1325 showed a +7.6 ETDRS letter advantage over sham (p = 0.0007)
- Approximately 53% of UBX1325-treated patients went 48 weeks without requiring any anti-VEGF rescue treatment compared to only 22% of patients in the sham arm
- Patients treated with UBX1325 had a mean change in CST of -16.6 microns from baseline at 40 weeks, representing an improvement compared to sham of -56.3 microns (p = 0.0479); at 48 weeks, UBX1325 had a mean change of -13.7 microns representing an improvement of -37.9 microns compared to sham (p = NS, in part due to the low number of sham patients remaining rescue-free at 48 weeks).

"DME patients are challenging to treat, often requiring frequent injections to decrease retinal edema and improve or even maintain vision. In this study, UBX1325 achieved visual improvement with a single injection, and maintained this improvement in over 50% of patients for a year," said Jeffrey S. Heier, M.D., Director of Retina Research at Ophthalmic Consultants of Boston. "UBX1325, with a novel mechanism of action, could be an important therapeutic option for patients with such a complex, multifactorial disease."

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. Robert B. Bhisitkul, M.D., Ph.D., of University of California San Francisco School of Medicine, as well as members of the UNITY senior management team will lead the discussion on the 48-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 through 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 had the option of continuing in the long-term extension portion of the study through 48-weeks, in which the majority of patients had 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 the 24-week data of the Phase 2 BEHOLD study in patients with DME, a single injection of UBX1325 led to a statistically significant and clinically meaningful improvement in mean Best Corrected Visual Acuity (BCVA) at 24 weeks compared to sham treatment.

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 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, UNITY's 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 results of the clinical trials in 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 recent Annual Report on Form 10-K for the year ended December 31, 2022, filed with the Securities and Exchange Commission on March 15, 2023, 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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Source: Unity Biotechnology, Inc.