UNITY Biotechnology Announces Positive Data in Phase 2 BEHOLD Study of UBX1325 in Patients with Diabetic Macular Edema

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A single injection of UBX1325 led to a progressive, statistically significant, and clinically meaningful improvement in mean Best Corrected Visual Acuity (BCVA) at 12- and 18-weeks compared to sham treatment

UBX1325 treatment also stabilized retinal structure, as measured by central subfield thickness (CST) at 12- and 18-weeks, as compared to worsening in sham-treated patients

The treatment effect was seen in a patient population with visual acuity deficits and residual retinal fluid despite frequent and recent anti-VEGF therapy

UNITY to host investor call with retinal expert Robert Bhisitkul, M.D., Ph.D., today, August 12, at 8:00 a.m. ET

SOUTH SAN FRANCISCO, Calif., Aug. 12, 2022 (GLOBE NEWSWIRE) -- UNITY Biotechnology, Inc. (“UNITY”) [Nasdaq: UBX], a biotechnology company developing therapeutics to slow, halt, or reverse diseases of aging, today announced 12- and 18-week data from its Phase 2 BEHOLD study of UBX1325, a senolytic Bcl-xL inhibitor, in patients with diabetic macular edema (DME).

At 18 weeks after a single UBX1325 injection, the mean change in BCVA of UBX1325-treated subjects was an increase of 6.1 ETDRS letters, representing an improvement of +5.0 ETDRS letters compared to sham-treated subjects (p = 0.0368). In addition, patients treated with UBX1325 maintained CST compared to sham-treated patients who demonstrated progressive worsening of CST (i.e., increased retinal thickness) through 18 weeks. The separation of UBX1325-treated patients from sham-treated patients at 18 weeks in measures of both visual function and retinal structure following a single UBX1325 injection suggests that one dose could have a durable therapeutic effect. The current standard of care for DME with the leading anti-VEGF therapeutic requires 3-5 monthly loading doses followed by every 8-week dosing, imposing a significant treatment burden on patients.

“The 12- and 18-week BEHOLD results are especially impressive considering that UBX1325 was given as a single injection in a patient population in which anti-VEGF treatment was no longer providing optimal benefit,” said Anirvan Ghosh, Ph.D., chief executive officer of UNITY. “The vision gains observed are greater than what has been previously reported with the standard of care in similar patient populations, and the durability of effect suggests that UBX1325 could address the large unmet need for longer-lasting, disease-modifying treatments for patients with DME. These data represent an important and exciting step in validating the senolytic therapeutic concept that is core to UNITY’s platform.”

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

Overall Safety

UBX1325 demonstrated a favorable safety and tolerability profile with no cases of intraocular inflammation, retinal vein occlusion, endophthalmitis, or vasculitis.

At 12 weeks (primary analysis set of 65 patients)

- Patients enrolled in BEHOLD were receiving regular anti-VEGF treatment prior to enrollment into the study (mean rate of approximately 4 injections in the preceding 6 months) with the last anti-VEGF injection occurring 3 – 6 weeks prior to randomization
- Patients treated with a single injection of UBX1325 had a mean improvement in BCVA of +4.7 ETDRS letters from baseline compared to +1.3 ETDRS letters in sham-treated patients (p=0.1148)
- Patients treated with UBX1325 had a mean change in CST of -1.4 microns from baseline compared to +40.3 microns in sham-treated patients (p=0.0747)

At 18 weeks (primary analysis set of 54 patients)

- Patients treated with UBX1325 had a mean improvement in BCVA of +6.1 ETDRS letters from baseline compared to +1.1 EDTRS letters in sham-treated patients (p=0.0368)
- Patients treated with UBX1325 had a mean change in CST of +3.2 microns from baseline compared to +53.5 microns in sham-treated patients (p=0.0719)

Based on pre-defined proof-of-concept criteria of alpha=0.15, the study demonstrated a statistically significant treatment effect for both BCVA and CST at both 12- and 18-weeks.

“A 6.1-letter gain from baseline in DME patients who had been actively receiving anti-VEGF treatment and had vision loss with persistent fluid is a clinically meaningful and impressive outcome,” said Arshad M. Khanani, M.D., M.A., FASRS, Director of Clinical Research at Sierra Eye Associates, Reno, Nevada. “The results are particularly noteworthy considering that there is a progressive improvement in vision through 18 weeks after a single injection of UBX1325. A treatment based on a new mechanism of action that shows a meaningful and sustained improvement in BCVA and stability of CST while also reducing the frequency of injections would be of huge value for patients with DME.”

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 rolling over to a 48-week long term extension and a majority of patients who have completed their 24-week visit have opted to remain in the study. More information about the study is available here (NCT04857996).

“Bolstered by positive proof-of-concept data, we believe UBX1325 could represent a much-needed alternative to all other currently available treatments for DME, including the standard of care anti-VEGF therapy,” said Jamie Dananberg, M.D., chief medical officer of UNITY. “The 12- and 18-week results indicate that a single injection of UBX1325 resulted in significantly greater letter gains and stabilization of retinal structure than the sham treatment, but also likely altered the disease trajectory of these patients who had been on anti-VEGF treatment. We are greatly encouraged by these findings and look forward to our upcoming 24-week BEHOLD (DME) and 16-week ENVISION (wet AMD) study readouts in the months ahead.”

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. Robert Bhisitkul, M.D., Ph.D., professor of ophthalmology and director of the Retina Fellowship at University of California, San Francisco, as well as members of the UNITY senior management team, will lead the discussion on the 12- and 18-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 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.

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