Lumos Pharma Announces Topline Data from Phase 2 OraGrowtH210 and OraGrowtH212 Trials of LUM-201 in PGHD Met All Primary and Secondary Endpoints

Nov 7, 2023

Phase 2 Data Provide Supportive Evidence to Advance Oral LUM-201 to Phase 3

- *OraGrowtH210 Results Show LUM-201 Dose of 1.6 mg/kg Achieves Annualized Height Velocities (AHV) of 8.2 cm/yr at 6 Months and 8.0 cm/yr at 12 Months, Comparable to Growth Rates for Moderate PGHD Population*
- *Delta at 6 and 12-month AHV Between Optimal LUM-201 Dose of 1.6 mg/kg and rhGH Comparator Arm is Within the Non-inferiority Margin (< 2 cm/yr) Suggested by FDA for Recent Approvals*
- *Initial 24-month LUM-201 Data from Combined OraGrowtH210 and OraGrowtH212 Trials Demonstrate a Sustained AHV Effect from Year 1 to Year 2*
- *Met Pre-specified Primary Endpoint of Validation of Predictive Enrichment Marker (PEM) Test and Secondary Endpoint Demonstrating 100% Reproducibility of PEM-Positive Classification*
- *OraGrowtH212 Demonstrated That, with Only 20% the GH Concentration of Injectable rhGH, LUM-201 Achieved Expected AHV While Demonstrating the Unique Pulsatile Mechanism of Action of LUM-201*[††]
- *No Safety Signal to Date for LUM-201*

Company to Host Conference Call Tomorrow Morning at 8:30AM ET

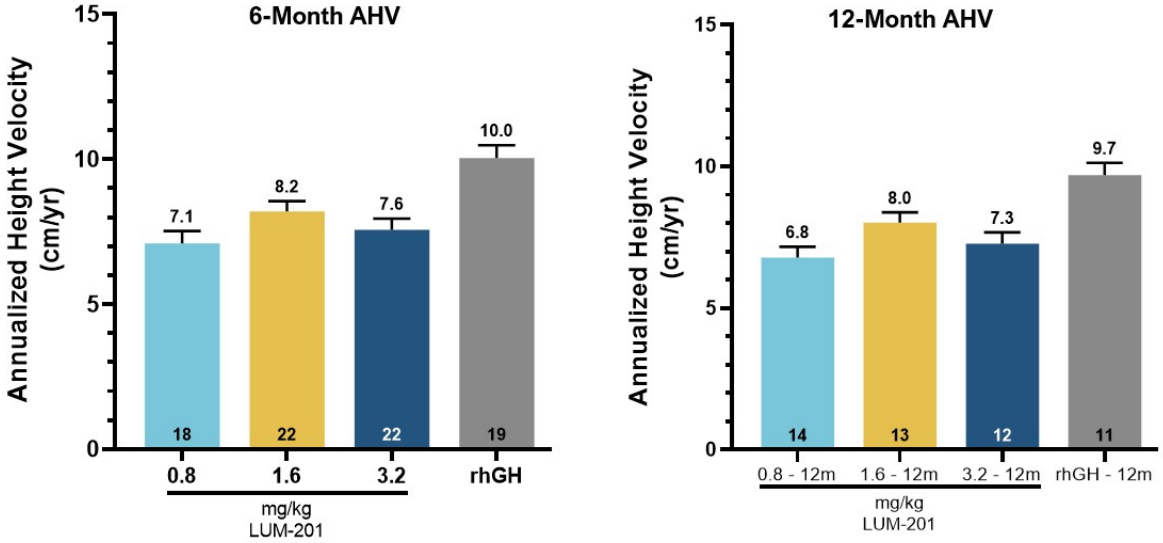
AUSTIN, Texas, Nov. 07, 2023 (GLOBE NEWSWIRE) -- Lumos Pharma, Inc. (NASDAQ:LUMO) today announced that topline results from its Phase 2 OraGrowtH210 dose-finding trial and its Phase 2 OraGrowtH212 Pharmacokinetic/Pharmacodynamic (PK/PD) trial met all primary and secondary endpoints. Data from the OraGrowtH210 Trial demonstrated annualized height velocity (AHV) on the 1.6 mg/kg dose of orally administered LUM-201 of 8.2 cm/yr at six months and 8.0 cm/yr at 12 months on treatment,[*] in line with historical data in moderate pediatric growth hormone deficiency (PGHD) patients and within the targeted 2 cm/yr margin of the comparator injectable recombinant growth hormone (rhGH) arm. Data also provided preliminary validation of the predictive enrichment marker (PEM) strategy, with prespecified primary and secondary outcomes met, de-risking our patient selection for our Phase 3 program. Data from the OraGrowtH212 Trial confirmed that LUM-201's unique pulsatile mechanism produces an increase in growth rates while restoring growth hormone secretion and IGF-1 to within normal ranges [†], with levels substantially below those produced by exogenous injectable rhGH.[††] Additionally, data from a small subset of 10 subjects combined 1.6 and 3.2 mg/kg dosage of LUM-201 in both OraGrowtH210 and OraGrowtH212 trials demonstrated the sustained effectiveness of AHV up to 24 months. Furthermore, the safety profile for LUM-201 remained clean throughout both Phase 2 studies, with no safety concerns identified in either of our Phase 2 trials conducted thus far.

"Results from our OraGrowtH trials have provided us with clear proof of concept that oral LUM-201 has the potential to serve as a viable alternative to injectable therapies in moderately growth hormone deficient patients. Our data indicates that LUM-201 can enhance AHVs in line with established standards for moderate PGHD patients undergoing rhGH therapy, demonstrating a robust and durable response," said Rick Hawkins, Chairman and CEO of Lumos Pharma. "We look forward to discussing these data and finalizing our plans for a Phase 3 pivotal trial with the FDA in our end of Phase 2 meeting anticipated in the first half of 2024."

Renowned pediatric endocrinologist Dr. Ron Rosenfeld, who also serves as the Chairman of our Clinical and Scientific Advisory Board, provided insight on the data, stating, "These findings not only align with historical growth expectations on therapy but also underscore the distinct advantage of LUM-201's unique pulsatile mechanism. Demonstrating the ability to achieve expected growth with oral LUM-201 while exposing patients to only 20% of the growth hormone compared to daily rhGH injections is a significant scientific breakthrough that has the potential to revolutionize the approach to treating children with moderate growth hormone deficiency."

OraGrowtH210 Topline Results Highlights

The OraGrowtH210 trial met its primary objective, with 6-month AHV data of 8.2 cm/yr supporting the 1.6 mg/kg as the optimal dose for a Phase 3 clinical trial.[*] The 6-month and 12-month AHV on 1.6 mg/kg/day met expectations for growth and were within the targeted 2.0 cm/yr margin for non-inferiority against injectable rhGH cohort.



ANCOVA Model Terms: treatment, Age at dose 1, Sex, Baseline HT SDS, Baseline BMI SDS, Baseline IGF-1 SDS, LUM-201 PEM, Baseline BA Delay, HT SDS-MPH
SDS Bars represent Least Squares Mean (LSM), Error bars represent the Standard Error of LSM

- Dosage at 1.6 mg/kg demonstrates highest LUM-201 AHV at six months and 12 months
- 1.7 cm/yr difference between 1.6 mg/kg LUM-201 dose and rhGH comparator arm at 12 months falls within historical non-inferiority Phase 3 margins
- LUM-201 AHVs align with historical growth rates of rhGH in patient populations with similar characteristics.
- 12-month AHV data available for 50/81 subjects: Growth rates durable at 12 months

The mean AHVs at 6 months and 12 months observed in the 1.6 mg/kg dose LUM-201 arm were 8.2 cm/yr and 8.0 cm/yr, respectively. These AHVs were in line with the Company's expectations for 8.3-8.6 cm/yr AHV observed after 12 months of rhGH treatment in a moderate PGHD patient population.[1,2,3]

The higher than anticipated AHV seen in this moderate PGHD population treated in the rhGH control arm of the OraGrowtH210 Trial was inconsistent with multiple historical trials which predicted growth in the 8.3-8.6 cm/yr range for moderate PGHD[1-4]. This distinctive growth pattern observed in the daily GH arm of this study is likely due to a higher dosage and the presence of outliers. We anticipate that in a larger, more statistically robust Phase 3 trial, the AHV associated with rhGH treatment will align more closely with historical values for the moderate patient population.

The OraGrowtH210 Trial met the prespecified percent responder enrichment providing preliminary validation of the PEM strategy. Additionally, we have achieved a 100% success rate in meeting the predetermined outcome for positive PEM specification classification reproducibility.

OraGrowtH212 Topline Results Highlights
The topline results from the OraGrowtH212 Trial reveal that LUM-201 achieved an expected AHV with only 20% of the growth hormone (GH) concentration observed using injectable rhGH. This outcome was achieved through LUM-201's natural pulsatile mechanism, promoting growth in moderate PGHD subjects that align with historical norms. Notably, LUM-201 raised circulating GH to levels closer to normal physiological ranges, whereas treatment with injectable rhGH has been shown to elevate GH levels to four to five times that of typical healthy children. Furthermore, it's important to highlight that during the first 12 months of LUM-201 treatment, no IGF-1 values exceeded 2 standard deviations from the mean.

Combined 24-Month Data from OraGrowtH210 and OraGrowtH212 Trials

- Eighteen and 24-month growth data were available for 10 subjects from the OraGrowtH210 and OraGrowtH212 Trials who met AHV criteria per protocol at 12 months.
- Combined data from the 1.6 mg/kg and 3.2 mg/kg cohorts of both trials demonstrate sustained AHVs from 12 to 24 months without a considerable decline in growth velocity compared to the previously reported ~20% decline in AHV on rhGH from 12 to 24 months observed in the Pfizer Phase 4 KIGS dataset.[3]

Safety & Tolerability Highlights
The topline results from both the OraGrowtH210 and OraGrowtH212 trials have shown a clean safety record, characterized by an absence of treatment-related Serious Adverse Events (SAEs), no instances of participants discontinuing treatment due to adverse events (AEs), and the absence of any significant safety concerns in various parameters such as laboratory values, adverse event data, or in electrocardiogram (ECG) readings.

† Zadik et al Horm Res 1992
†† Adapted from data in Albertsson-Wikland et al JCEM 1994; 24h exposures listed reflect absorbance/bioavailability of ~60% of the administered dose,
** For all OraGrowtH Trial AHV values, ANCOVA Model Terms: treatment, Age at dose 1, Sex, Baseline HT SDS, Baseline BMI SDS, Baseline IGF-1*

SDS, LUM-201 PEM, Baseline BA Delay, for graphs HT SDS-MPH SDS Bars represent Least Squares Mean (LSM), Error bars represent the Standard Error of LSM

[1] Blum et al JES 2021,

[2] Lechuga-Sancho et al JPEM 2009,

[3] Ranke et al JCEM 2010, [4] Bright et al JES 2021

Conference Call and Webcast Details

Date: November 8, 2023
Time: 8:30 AM ET
Dial-in: 1-877-407-9716 or 1-201-493-6779 (international)
Conference ID: 13742617
Or Dial-in registration (Available 15 minutes prior to scheduled start time): https://callme.viavid.com/viavid/?callme=true&passcode=13742617&h=true&info=company-email&r=true&B=6
Webcast link: https://viavid.webcasts.com/starthere.jsp?ei=1642841&tp_key=d9efda8a69

Slides are available on the Lumos Pharma website in the "Investors & Media" section under "Events and Presentations" link: **https://investors.lumos-pharma.com/events-presentations**.

A replay of the call will be available approximately two hours after the completion of the call and can be accessed by using the same numbers as above for two weeks following the call.

Virtual KOL Event Planned

The Company plans to host a virtual KOL Event on December 6th to discuss topline results from OraGrowtH210 and OraGrowtH212 trials in greater detail and provide updates on clinical and corporate strategy. Management will be joined by the following three esteemed thought leaders in the field of endocrinology:

- **Andrew Dauber, MD**, Chief of Endocrinology at Children's National Medical Center, Washington, D.C.
- **Fernando Cassorla, MD**, Chief of Pediatric Endocrinology at the Institute of Maternal and Child Research, University of Chile
- **Leslie A. Soyka, MD**, Chief of Pediatric Endocrinology, UMass Memorial Medical Center; Associate Professor, UMass Chan Medical School, Worcester, MA

Access information regarding the KOL Event will be provided at a later date.

OraGrowtH210 Trial Design

The OraGrowtH210 Trial is a global, multi-site study that assesses the effects of orally administered LUM-201 at three different dose levels (0.8, 1.6, 3.2 mg/kg/day) in comparison to daily injections of recombinant human growth hormone (rhGH) at a dose of 34 µg/kg/day. This trial involves 82 participants diagnosed with moderate Pediatric Growth Hormone Deficiency (PGHD). To enrich the trial population with individuals likely to respond to LUM-201, specific PEM criteria were applied during the screening process. These criteria included having a baseline IGF-1 value above 30 ng/ml and achieving a peak growth hormone value of 5 ng/ml or higher after administering a single 0.8 mg/kg dose of LUM-201 to treatment-naïve PGHD patients. It is important to note that the primary purpose of this study was not to establish efficacy or demonstrate non-inferiority compared to daily GH treatment.

OraGrowtH212 Trial Design

The OraGrowtH212 Trial is a single-site, open-label study designed to assess the pharmacokinetic (PK) and pharmacodynamic (PD) impacts of oral LUM-201. This trial includes up to 24 individuals with no prior treatment for Pediatric Growth Hormone Deficiency (PGHD), who are administered LUM-201 at two different dosage levels, specifically 1.6 and 3.2 mg/kg/day. Every participant in the OraGrowtH212 Trial met the criteria for Patient PEM positivity, ensuring their potential responsiveness to LUM-201.

About Lumos Pharma

Lumos Pharma, Inc. is a clinical stage biopharmaceutical company focused on the development and commercialization of therapeutics for rare diseases. The Company was founded and is led by a management team with longstanding experience in rare disease drug development. Lumos Pharma's lead therapeutic candidate, LUM-201, is a novel, oral growth hormone (GH) secretagogue, seeking to transform the ~$3.4B global GH market from injectable to oral therapy. LUM-201 is currently being evaluated in multiple Phase 2 clinical studies in Pediatric Growth Hormone Deficiency (PGHD) and has received Orphan Drug Designation in both the US and EU. For more information, please visit https://lumos-pharma.com/.

Cautionary Note Regarding Forward-Looking Statements

This press release contains forward-looking statements of Lumos Pharma, Inc. that involve substantial risks and uncertainties. All such statements contained in this press release are forward-looking statements within the meaning of The Private Securities Litigation Reform Act of 1995. A law that, in part, gives us the opportunity to share our outlook for the future without fear of litigation if it turns out our predictions were not correct.

We are passionate about our business - including LUM-201 and the potential it may have to help patients in the clinic. This passion feeds our optimism that our efforts will be successful and bring about meaningful change for patients. Please keep in mind that actual results or events could differ materially from the plans, intentions and expectations disclosed in the forward-looking statements that we make.

We have attempted to identify forward-looking statements by using words such as "projected," "upcoming," "will," "would," "plan," "intend," "anticipate," "approximate," "expect," "potential," "imminent," and similar references to future periods or the negative of these terms. Not all forward-looking statements contain these identifying words. Examples of forward-looking statements include, among others, statements we make regarding our Phase 2 data providing supporting evidence to advance oral LUM-201 to Phase 3, clear proof of concept that oral LUM-201 has the potential to serve as a viable alternative to injectable therapies in moderately growth hormone deficient patients, the potential for LUM-201 to enhance AHVs in line with established standards for moderate PGHD patients undergoing rhGH therapy, looking forward to discussing these data and finalizing our plans for a Phase 3 pivotal trial with the FDA in our end of Phase 2 meeting anticipated in the first half of 2024, that this is a significant scientific breakthrough that

has the potential to revolutionize the approach to treating children with moderate growth hormone deficiency, data from the OraGrowthH210 Trial supporting the 1.6 mg/kg dose for LUM-201 as the optimal dose for a Phase 3 trial, that this distinctive growth pattern observed in the daily GH arm of this study is likely due to a higher dosage and the presence of outliers, that in a larger, more statistically robust Phase 3 trial, the AHV associated with rhGH treatment will align more closely with historical values for the moderate patient population, and any other statements other than statements of historical fact.

We wish we were able to predict the future with 100% accuracy, but that just is not possible. Our forward-looking statements are neither historical facts nor assurances of future performance. You should not rely on any of these forward-looking statements and, to help you make your own risk determinations, we have provided an extensive discussion of risks that could cause actual results to differ materially from our forward-looking statements including risks related to the continued analysis of data from our LUM-201 Trials, the timing and outcome of our future interactions with regulatory authorities including our end of Phase 2 meeting with the FDA, the timing and ability of Lumos to raise additional equity capital as needed to fund our Phase 3 Trial, our ability to project future cash utilization and reserves needed for contingent future liabilities and business operations, the ability to structure our Phase 3 trial in an effective and timely manner, the ability to successfully develop our product candidate, the effects of pandemics, other widespread health problems or military conflicts including the Ukraine-Russia conflict and the Middle East conflict and other risks that could cause actual results to differ materially from those matters expressed in or implied by such forward-looking statements including information in the "Risk Factors" section and elsewhere in Lumos Pharma's Quarterly Report on Form 10-Q for the period ended June 30, 2023, as well as other reports filed with the SEC including our subsequent Quarterly Reports on Form 10-Q and Current Reports on Form 8-K. All of these documents are available on our website. Before making any decisions concerning our stock, you should read and understand those documents.

We anticipate that subsequent events and developments will cause our views to change. We may choose to update these forward-looking statements at some point in the future, however, we disclaim any obligation to do so. As a result, you should not rely on these forward-looking statements as representing our views as of any date subsequent to the date of this press release.

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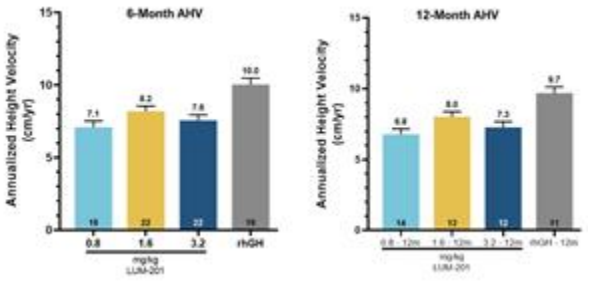
A photo accompanying this announcement is available at https://www.globenewswire.com/NewsRoom/AttachmentNg/e14ed9b0-f190-41e0-8c43-236ed34b5ceb

6 & 12 mo AHV



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Source: Lumos Pharma, Inc.