

**UNITED STATES  
SECURITIES AND EXCHANGE COMMISSION  
Washington, D.C. 20549  
FORM 8-K**

**CURRENT REPORT  
Pursuant to Section 13 OR 15(d) of The Securities Exchange Act of 1934**

December 12, 2022  
Date of Report (date of earliest event reported)

**LUMOS PHARMA, INC.**  
(Exact name of registrant as specified in its charter)

**Delaware**  
(State or other jurisdiction of incorporation or organization)

**001-35342**  
(Commission File Number)

**42-1491350**  
(I.R.S. Employer Identification No.)

**4200 Marathon Blvd., Suite 200**  
**Austin, Texas 78756**  
(Address of Principal Executive Offices)  
**(512) 215-2630**  
Registrant's telephone number, including area code

Check the appropriate box below if the Form 8-K filing is intended to simultaneously satisfy the filing obligation of the registrant under any of the following provisions (see General Instruction A.2. below):

- Written communications pursuant to Rule 425 under the Securities Act (17 CFR 230.425)
- Soliciting material pursuant to Rule 14a-12 under the Exchange Act (17 CFR 240.14a-12)
- Pre-commencement communications pursuant to Rule 14d-2(b) under the Exchange Act (17 CFR 240.14d-2(b))
- Pre-commencement communications pursuant to Rule 13e-4(c) under the Exchange Act (17 CFR 240.13e-4(c))

Securities registered pursuant to Section 12(b) of the Act:

Title of each class	Trading Symbol(s)	Name of each exchange on which registered
Common Stock	LUMO	The Nasdaq Stock Market

Indicate by check mark whether the registrant is an emerging growth company as defined in Rule 405 of the Securities Act of 1933 (§230.405 of this chapter) or Rule 12b-2 of the Securities Exchange Act of 1934 (§240.12b-2 of this chapter). Emerging growth company

If an emerging growth company, indicate by check mark if the registrant has elected not to use the extended transition period for complying with any new or revised financial accounting standards provided pursuant to Section 13(a) of the Exchange Act.

**Item 8.01 Other Events.**

On December 12, 2022, Lumos Pharma, Inc. issued a press release titled "KOL Review of Lumos Pharma's Interim Phase 2 Data Supports Potential for New Oral Therapeutic Paradigm for Moderate Idiopathic PGHD Patients."

A copy of the press release and the slide deck used during the KOL event are attached hereto as Exhibit 99.1 and 99.2, respectively, and are incorporated herein by reference.

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**Item 9.01. Financial Statements and Exhibits.**

(d) Exhibits.

Exhibit Number	Description
99.1	Press Release, dated December 12, 2022, entitled " <a href="#">KOL Review of Lumos Pharma's Interim Phase 2 Data Supports Potential for New Oral Therapeutic Paradigm for Moderate Idiopathic PGHD Patients.</a> "
99.2	<a href="#">KOL Slide Deck</a>

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**SIGNATURES**

Pursuant to the requirements of the Securities Exchange Act of 1934, the registrant has duly caused this report to be signed on its behalf by the undersigned hereunto duly authorized.

Dated: December 12, 2022

LUMOS PHARMA, INC.,  
a Delaware corporation

By: /s/ Richard J. Hawkins  
Richard J. Hawkins  
Its: Chief Executive Officer

## KOL Review of Lumos Pharma’s Interim Phase 2 Data Supports Potential for New Oral Therapeutic Paradigm for Moderate Idiopathic PGHD Patients

AUSTIN, TX, December 12, 2022 – [Lumos Pharma, Inc.](https://www.lumospharma.com) (NASDAQ:LUMO), a clinical-stage biopharmaceutical company focused on rare disorders, hosted two key opinion leaders (KOLs) in the field of pediatric endocrinology on December 6<sup>th</sup> to review the Company’s interim data from two Phase 2 trials evaluating oral LUM-201 in moderate idiopathic Pediatric Growth Hormone Deficiency (iPGHD). Andrew Dauber, MD, MMSc, Chief of Endocrinology, Children’s National Hospital, and Fernando Cassorla, MD, Chief of Pediatric Endocrinology, University of Chile, discussed recently released (on November 14, 2022) and new interim data (presented on December 6, 2022) from two ongoing OraGrowth trials.

“Lumos Pharma was delighted to host Drs. Andrew Dauber and Fernando Cassorla, investigators in our OraGrowthH210 and OraGrowthH212 trials, respectively, to share their insights about oral LUM-201 and its potential to treat children with moderate idiopathic PGHD,” said Rick Hawkins, Chairman and CEO of Lumos Pharma. “For the last 40 years, these children have had only injectable growth hormone as a treatment option. In their discussion, both Dr. Dauber and Dr. Cassorla highlighted interim clinical data that supports the potential for LUM-201 as a welcome oral alternative to current therapies that require frequent injections.”

<b>Replay Links:</b>	<a href="#">KOL Webcast</a>
	<a href="#">Lumos Events &amp; Presentations</a>

### Highlights from Dr. Andrew Dauber’s Discussion of Interim Phase 2 OraGrowthH210 Results

- LUM-201, an oral growth hormone secretagogue, has a unique mechanism of action, targeting specific receptors on the pituitary and hypothalamus to stimulate the natural pulsatile secretion of growth hormone for moderate idiopathic PGHD patients with an intact pituitary-hypothalamic axis
- Reviewed the Interim Phase 2 OraGrowthH210 data announced on November 14, 2022. Reiterated that the annualized growth of 8.6 cm/year at 6 months on 1.6 mg/kg/day of LUM-201 met expectations of 8.3-8.5 cm/year annualized growth at 12 months on therapy established across multiple large datasets of moderate idiopathic PGHD patients on daily rhGH<sup>1,2,3,4</sup>
- Outsized growth in the trial’s control arm of 10 subjects was likely due to imbalances of baseline characteristics in that subjects compared to the 31 subjects across the LUM-201 arms and would likely resolve at full enrollment of 80 subjects in the Phase 2 trial and would not likely be repeated in a large Phase 3 trial
- Safety and tolerability for LUM-201 appear comparable to rhGH in this study period

### Highlights from Dr. Fernando Cassorla’s Discussion of Interim PK/PD OraGrowthH212 Results

- New data: A stimulation dose of 0.8 mg/kg LUM-201 produces a substantial growth hormone (GH) response

- New data: LUM-201 at 1.6 and 3.2 mg/kg/day produced a substantial increase in height velocity at 6 months versus baseline for all 10 subjects evaluated in the interim analysis of the OraGrowthH212 Trial
- New data: Substantial increases in growth hormone (GH) area under the curve (AUC) and IGF-1 levels at 6 months on both doses of LUM-201 compared to baseline were also observed
- New baseline data showing age and Height SDS values for OraGrowthH212 was presented which suggest that the 3.2 mg cohort was somewhat more growth hormone deficient than the 1.6 mg cohort, likely explaining the seemingly faster growth seen in the 3.2 mg/kg/day LUM-201 cohort

<sup>1</sup> Blum et al JES 2021, <sup>2</sup> Lechuga-Sancho et al JPEM 2009, <sup>3</sup> Ranke et al JCEM 2010, <sup>4</sup> Bright et al JES 2021.

Drawing from their experience in the clinic, Dr. Dauber and Dr. Cassorla noted the compliance issues with the injectable growth hormone, the only current treatment option for PGHD, supporting the potential for an oral therapeutic like LUM-201 to potentially improve compliance for their moderate idiopathic PGHD patients. Both doctors commented that there were also a number of patients currently not receiving treatment for PGHD because of their aversion to injections. Dr. Dauber and Dr. Cassorla acknowledged that it was likely that an oral therapeutic like LUM-201 would appeal to this latter population, potentially expanding the market for PGHD therapies.

#### **About Lumos Pharma**

Lumos Pharma, Inc. is a clinical stage biopharmaceutical company focused on the development and commercialization of therapeutics for rare diseases. Lumos Pharma was founded and is led by a management team with longstanding experience in rare disease drug development and received early funding from leading healthcare investors, including Deerfield Management, a fund managed by Blackstone Life Sciences, Roche Venture Fund, New Enterprise Associates (NEA), Santé Ventures, and UCB. Lumos Pharma's lead therapeutic candidate is LUM-201, an oral growth hormone stimulating small molecule, currently being evaluated in a Phase 2 clinical trial, the OraGrowthH210 Trial; a PK/PD trial, the OraGrowthH212 Trial; and a switch trial, the OraGrowthH213 Trial for the treatment of Pediatric Growth Hormone Deficiency (PGHD). If approved by the FDA, LUM-201 would provide an orally administered alternative to recombinant growth hormone injections that PGHD patients otherwise endure for many years of treatment. LUM-201 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 progress in our*

*clinical efforts including comments concerning screening and enrollment for our trials, expecting the primary outcome data readout for our trials, the potential to expand our LUM-201 platform into other indications, anticipated market reception to our treatment regimen for PGHD and other indications, plans related to initiation and execution of clinical trials; plans related to moving additional indications into clinical development; future financial performance, results of operations, cash position and sufficiency of capital resources to fund our operating requirements through the primary outcome data readout from the OraGrowtH210 and OraGrowtH212 Trials, 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. Forward-looking statements contained in this announcement are made as of this date and Lumos undertakes no duty to update such information except as required under applicable law. 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 in the "Risk Factors" section and elsewhere in Lumos Pharma's Annual Report on Form 10-K for the year ended December 31, 2021, as well as other reports filed with the SEC including our Quarterly Reports on Form 10-Q. 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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**lumos**  
PHARMA

## **KOL Event**

**December 6, 2022**





## Forward Looking Statements

This presentation contains proprietary and confidential information of Lumos Pharma, Inc. ("Lumos," "we," "us" and "our"), and such content should be considered "Confidential Information" and covered by your confidentiality obligations to Lumos. This presentation is made solely for informational purposes, and no representation or warranty, express or implied, is made by Lumos or any of its representatives as to the information contained in these materials or disclosed during any related presentations or discussions.

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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 presentation.

The data contained herein is derived from various internal and external sources. All of the market data in the presentation involves a number of assumptions and limitations, and there can be no guarantee as to the accuracy or reliability of such assumptions. Further, no representation is made as to the reasonableness of the assumptions made within or the accuracy or completeness of any projections or modeling or any other information contained herein. Any data on past performance or modeling contained herein is not an indication as to future performance. 11.12.2022

## Investment Highlights

Lead asset targeting children with growth disorders

<b>Novel Oral Rare Disease Asset</b>	<ul style="list-style-type: none"><li>• Novel <b>oral</b> therapeutic asset, <b>LUM-201</b>, for growth hormone deficiency (GHD) disorders</li><li>• LUM-201 acts within natural endocrine pathway, differentiated from injectable therapies</li><li>• <b>Potential to disrupt</b> significant subset of sizable <b>injectable market</b> for GHD</li></ul>	
<b>Pipeline in a Product</b>	<ul style="list-style-type: none"><li>• Worldwide injectable market for GHD disorders is <b>\$3.4 billion*</b></li><li>• Market for initial oral LUM-201 indication, Pediatric GHD (PGHD), is <b>\$1.2 billion*</b></li><li>• Prior data support potential efficacy of LUM-201 in multiple GHD disorders</li></ul>	
<b>Late-stage Trials in PGHD</b>	<ul style="list-style-type: none"><li>• Phase 2 OraGrowthH210 Trial &amp; PK/PD OraGrowthH212 Trials ongoing</li><li>• Interim data obtained   Primary outcome data expected 2H 2023</li><li>• <b>Approximately 80% enrolled in Phase 2 OraGrowthH210 Trial</b></li></ul>	
<b>Solid Financial Position</b>	<ul style="list-style-type: none"><li>• Cash balance of <b>\$73.7 million</b> as of close of <b>3Q 2022</b></li><li>• Cash runway <b>into 2Q 2024</b>, beyond OraGrowthH210 &amp; OraGrowthH212 primary outcome data</li></ul>	

PGHD = Pediatric Growth Hormone Deficiency

\* USA, Germany, France, Italy, Spain, UK, Japan (Grandview Research, Growth Hormone Market Forecast, 2019)

## Key Opinion Leaders in the Field of Pediatric Endocrinology



**Andrew Dauber, M.D., M.M.Sc.**, is the Chief of Endocrinology at Children's National Hospital, specializing in growth disorders. Dr. Dauber has served as the program director and director of translational research at the interdisciplinary Cincinnati Center for Growth Disorders at Cincinnati Children's Hospital Medical Center. Additionally, he was the director of their Genomics First for Undiagnosed Diseases Program and guided medical residents and fellows as an associate professor of pediatrics at the University of Cincinnati. He held similar roles as the assistant medical director for the clinical research unit at Boston Children's Hospital and as an assistant professor in pediatrics at Harvard Medical School. Dr. Dauber has authored over 100 publications and is an active member of the Endocrine Society, Pediatric Endocrine Society, European Society of Pediatric Endocrinology and the Society for Pediatric Research, having received several awards and honors from these entities. Dr. Dauber received his M.D. and Master's of Medical Sciences in Clinical Investigation from Harvard Medical School.



**Fernando Cassorla, M.D.** is currently Chief of Pediatric Endocrinology at the Institute of Maternal and Child Research of the University of Chile, a position he has held since 1993. Previously, he served as Senior Investigator at the Developmental Endocrinology Branch of the National Institute of Child Health and Human Development, rising to the position of Clinical Director of this Institute in 1990. Dr. Cassorla has authored numerous chapters in pediatric endocrinology, authored or co-authored over 200 original articles in peer reviewed journals, and has presented over 300 abstracts at scientific meetings. Dr. Cassorla received his MD from the University of Chile. He is Board Certified in both Pediatrics and Pediatric Endocrinology, having completed his pediatric residency at the Albany Medical Center in New York and his fellowship in Pediatric Endocrinology at the Children's Hospital of Philadelphia. Dr. Cassorla has received several international awards for his work, including the ESPE International Research Award, September 2022, and was elected to the Chilean Academy of Medicine for a lifetime position in 2003.

Andrew Dauber, M.D., M.M.Sc.

OraGrowthH210 Trial

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# Review of Interim Data for Phase 2 OraGrowthH210 Trial

## Evaluation of Oral LUM-201 in Moderate Idiopathic Pediatric Growth Hormone Deficiency (PGHD)

**Andrew Dauber, MD MMSc**  
Chief of Endocrinology  
Children's National Hospital



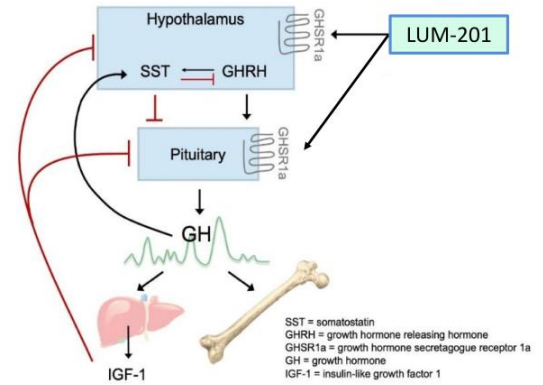
## Disclosures

- Consulting fees or speaker honoraria:
  - Ascendis, OPKO, BridgeBio, Novo Nordisk, Pfizer, Ipsen, Sandoz
- Prior Research Support
  - Novo Nordisk, Ipsen, Pfizer
- Current Research Support
  - BioMarin, NICHD, Pfizer
- Site Investigator in Lumos OraGrowth210 Trial

# LUM-201 – Oral Growth Hormone Secretagogue

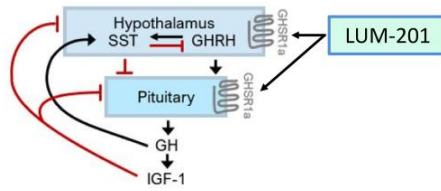
## LUM-201

- Binds to GH Secretagogue (ghrelin) receptor
- Increases amplitude of endogenous GH pulses
- Acts within intact GH/IGF-1 feedback loop
- Phase 2 study ongoing
  - 3 doses of LUM-201 vs daily rhGH
  - 24-month study
  - Pre-pubertal GH deficiency



# Single Stim Dose of LUM-201 Identifies Likely Responders

**Moderate / Idiopathic PGHD**  
**PEM-Positive**  
 ~60% of total PGHD population<sup>1</sup>



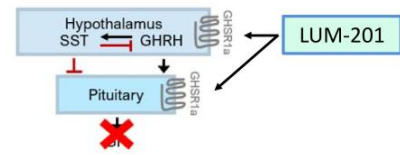
**Responders to LUM-201<sup>2</sup>**

## Predictive Enrichment Marker Positive (PEM+)

- Baseline IGF-1 > 30 ng/ml
- Stim LUM-201 peak GH ≥ 5 ng/ml
- Functional but reduced HP-GH axis

**LUM-201**  
**Stim dose**

**Severe / Organic PGHD**  
**PEM-Negative**  
 ~40% of total PGHD population



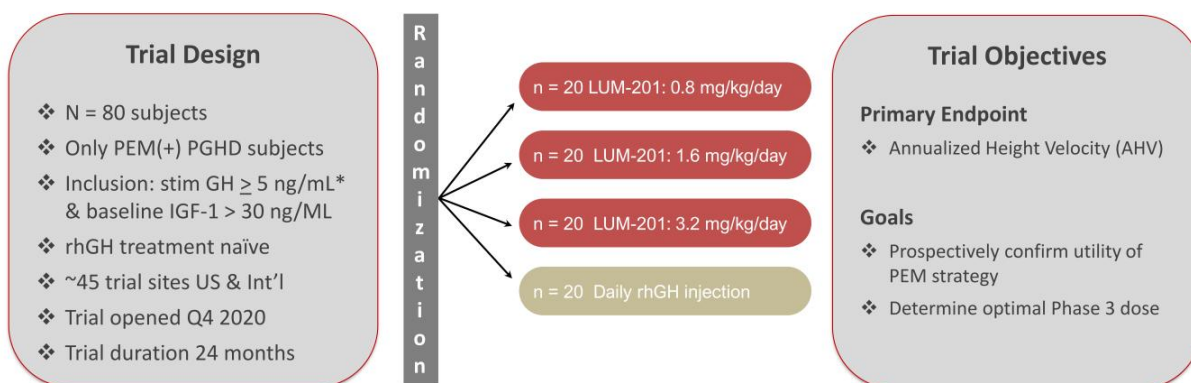
**Non-Responders to LUM-201**

## Predictive Enrichment Marker Negative (PEM-)

- Baseline IGF-1 < 30 ng/ml
- Stim LUM-201 GH < 5 ng/ml
- Non-functional HP-GH axis



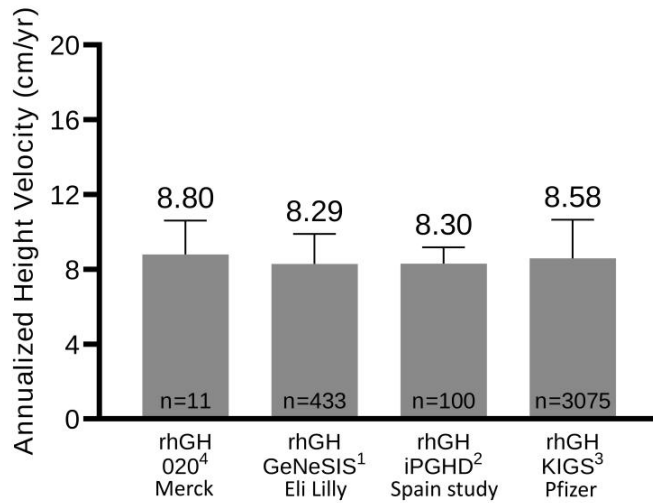
# Phase 2 OraGrowtH210 Trial in Moderate Idiopathic PGHD



Interim Data: 41 subjects @ 6 months on therapy – November 2022

Primary Outcome Data: 80 subjects @ 6 months on therapy – 2H 2023

## Historical Data for rhGH Growth Rates in Moderate PGHD



### Historical Datasets

- GeNeSIS<sup>1</sup>, iPGHD<sup>2</sup>, and KIGS<sup>3</sup> AHV at 12 months on rhGH
- Merck 020<sup>4</sup> AHV at 6 months on rhGH
- These historical trials set precedent for expected growth on rhGH in moderate idiopathic PGHD

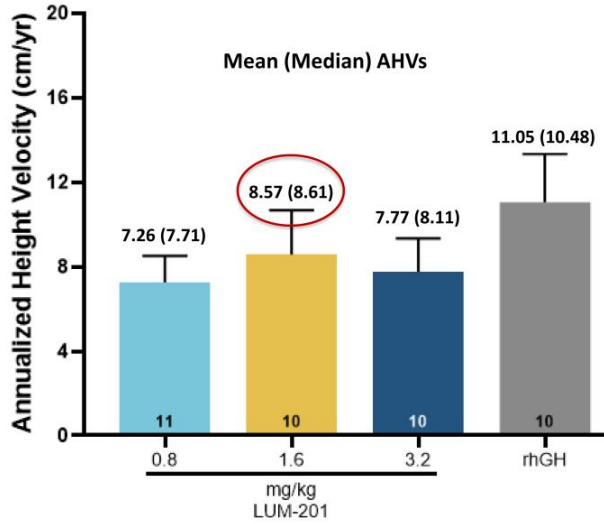
### Predictions

- Prediction for growth in OraGrowthH210 is AHV of ~8.3 cm/yr on both rhGH and LUM-201 based on this historical data



Children's National.

## OraGrowthH210 Interim Analysis: AHV at 6 Months (41 Subjects)



### Interim Results

- 1.6 mg/kg/day LUM-201 cohort growth of 8.6 cm/year was in line with the expected rate of 8.3-8.5 cm/year based on historical data
- rhGH cohort grew at a much faster rate than expected or previously reported in moderate idiopathic PGHD population
- Cohort baseline differences predict faster first-year growth in the rhGH arm<sup>1,2</sup>
- Median AHV values offer more authentic comparison by minimizing impact of outliers
- Further enrollment of trial likely to even out baseline imbalances and produce more similar growth across cohorts



Children's National.

## OraGrowtH210 Baseline Characteristics at Interim (n=41)

	LUM-201 0.8 mg Mean (SD) N=11	LUM-201 1.6 mg Mean (SD) N=10	LUM-201 3.2 mg Mean (SD) N=10	rhGH Mean (SD) N=10
<b>Age (months)</b>	95.5 (28.2)	99.3 (28.3)	96.1 (21.7)	90.3 (26.7)
<b>Height (cm)</b>	113.8 (12.6)	114.6 (9.6)	113.8 (8.8)	111.6 (11.9)
<b>Height SDS</b>	-2.31 (0.32)	-2.35 (0.62)	-2.30 (0.48)	-2.29 (0.43)
<b>Max Height SDS</b>	-1.76	-1.66	-1.57	-1.73
<b>IGF-1 SDS</b>	-1.24 (0.573)	-1.17 (0.72)	-1.39 (0.61)	-1.37 (0.48)
<b>Max IGF-1 SDS</b>	-0.3	-0.3	-0.6	-0.7
<b>MPH (cm)</b>	164.47 (6.44)	166.98 (7.15)	166.20 (8.06)	168.78 (8.85)
<b>MPH SDS Δ</b>	1.29 (0.62)	1.76 (0.60)	1.96 (0.83)	1.76 (0.73)
<b>BA Delay (yrs)</b>	1.89 (1.02)	1.91 (0.53)	2.19 (0.86)	1.78 (0.96)
<b>BMI SDS<sup>1</sup></b>	-0.29 (1.04)	-0.35 (0.79)	-0.70 (0.48)	+0.31 (1.05)

- Imbalances were observed in baseline characteristics between rhGH arm & LUM-201 arms
- Baseline characteristics predict faster 1<sup>st</sup> year growth on therapy for rhGH arm than for LUM-201 arms<sup>2</sup>



Children's National.

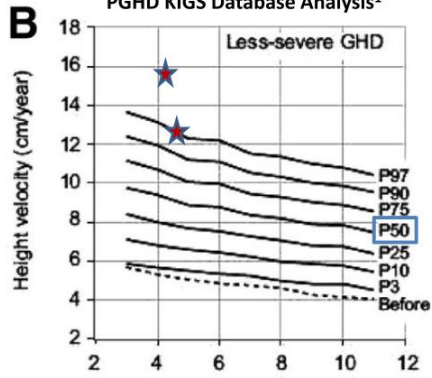
13 <sup>1</sup> Yang, et al. Nature Sci Rep 2019, 9(1); 16181 <sup>2</sup> Ranke et al JCEM 2010

SDS = Standard deviation score | MPH = Mid-parental height (Child's target height) | MPH SDS delta = SD's from target height | BA = Bone age | BMI = Body mass index

# Growth Outliers in the rhGH Cohort:

Two of Three Subjects Under Age 5 Randomized to rhGH

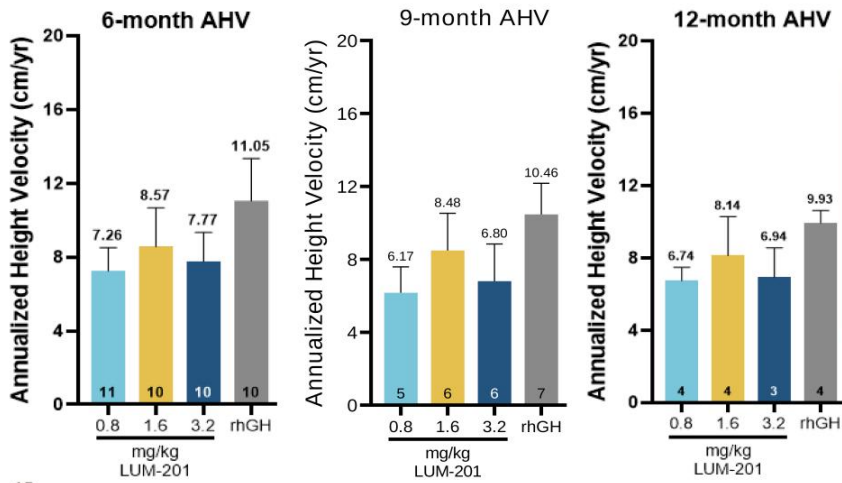
First-year Growth on rhGH for Pfizer's Moderate PGHD KIGS Database Analysis<sup>1</sup>



★ OraGrowthH210 youngest subjects in rhGH cohort at 6-months AHV

P lines = Percentiles  
"Before" line marks height velocity before GH therapy

## Interim OraGrowthH210 Data: LUM-201 Demonstrates Durable Response to 12 Months



### Conclusions

- Growth rates for LUM-201 are consistent from 6 to 12 months
- A Phase 3 non-inferiority trial is expected to be a 12-month study in a significantly larger population
- Historically, non-inferiority margin for AHV's in Phase 3 trials was ~2 cm



**Children's National.**

## Safety Profile at Interim Analysis for OraGrowthH210 Trial

	0.8 mg/kg	1.6 mg/kg	3.2 mg/kg	ALL LUM-201	rhGH 34 mcg/kg
<b>N =</b>	<b>14</b>	<b>15</b>	<b>14</b>	<b>43</b>	<b>15</b>
<b>Number of AEs</b>	31	45	38	114	21
<b>Subjects with AE (%)</b>	8 (57.1%)	13 (86.7%)	9 (64.3%)	30 (69.8%)	9 (60.0%)
<b>Treatment Related AEs (N)</b>	2	1	3	6	3
<b>Subjects with Treatment Related AEs (%)</b>	1 (7.1%)	1 (6.7%)	2 (14.3%)	4 (9.3%)	2 (13.3%)
<b>Subjects with SAEs (%)</b>	0 (0.0%)	0 (0.0%)	0 (0.0%)	0 (0.0%)	0 (0.0%)

### Data Available

- Data available for 58 subjects at time of interim analysis
- 66 subjects total enrolled at time of interim analysis



Children's National.

## Summary of Interim OraGrowthH210 Data

- Growth of 8.6 cm/yr on 1.6 mg LUM-201 in line with expectations
- 9 and 12-month growth data look encouraging for LUM-201, but N is small
- Interim safety data appear comparable to rhGH treatment



Fernando Cassorla, M.D.

OraGrowthH212 Trial

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# Effects of the oral growth hormone (GH) secretagogue LUM-201 (Ibutamoren) on pulsatile GH secretion and linear growth in children with moderate GH deficiency

**Fernando Cassorla MD**

**Chief of Pediatric Endocrinology**

**Institute of Maternal and Child Research**

**University of Chile, Santiago, Chile**



# Disclosure

## Fernando Cassorla M.D.

Dr. Cassorla is an investigator for clinical studies with LUM-201 at the University of Chile and has previously acted as a consultant for Debiopharm, Novo Nordisk, Pfizer, Merck and Sandoz. LUM-201 is an investigational compound and is not approved for use by the FDA or any other regulatory agency. Some of the slides in this presentation are derived or copied from corporate presentations previously given by Lumos Pharma, Inc. These slides are used with permission.



## Questions regarding the effects of LUM-201 in pediatric patients with moderate growth hormone deficiency:

- Does the oral administration of LUM-201 at the doses of 1.6 mg/kg or 3.2 mg/kg for 6 months increase height velocity?
- Is the change in height velocity correlated with changes in GH pulsatility?
- Is the increase in GH pulsatility and height velocity induced by LUM-201 dose dependent?



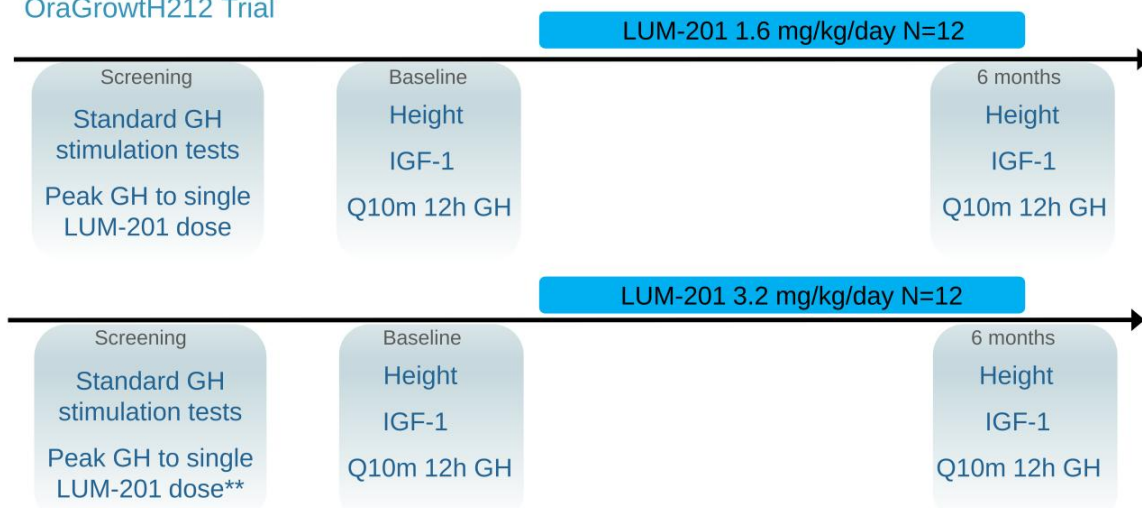
## Design of the OraGrowth212 clinical PK/PD study of LUM-201 in naïve pediatric patients with moderate GH deficiency

- 24 prepubertal patients between the ages of 4 to 8 years (girls) and 10 years (boys) with short stature caused by moderate idiopathic PGHD, and naïve to GH treatment, will be randomized to receive oral LUM-201 at a dose of 1.6 mg/kg/day (n:12) or 3.2 mg/kg/day (n:12)
- Pulsatile GH secretion has been assessed in blood samples obtained every 10 minutes over 12 hours (8 AM to 8 PM), at the beginning and after 6 months of LUM-201 therapy
- Height velocity during the 6 months of LUM-201 administration has been compared with a baseline period prior to intervention



# LUM-201 in naïve prepubertal patients with GH deficiency

OraGrowthH212 Trial



Inclusion Criteria: Height < 2 SD, delayed bone age, serum IGF-1 below the mean for age, and a peak GH response to a clonidine stimulation test between 3 and 10 ng/mL.  
23 \*\* Peak GH response to a single LUM-201 0.8 mg/kg dose was also assessed in screening.



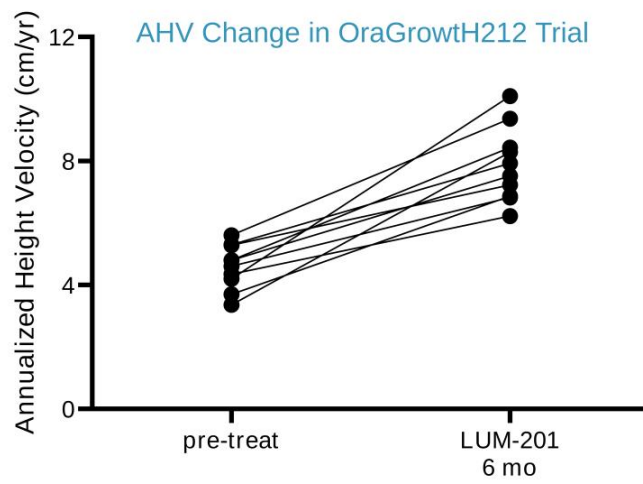
## OraGrowthH212 Patient Baseline Characteristics

Patient	Gender	Age (months)	Dose (mg/kg)	Height (SDS)	Clonidine test (GH ng/mL)		PEM test (GH ng/mL)
				time 0	peak	peak	
801-101	F	91	3.2	-2.39	8.2	32	
801-102	M	74	3.2	-3.12	5.8	22.7	
801-103	M	105	3.2	-1.85	3.1	18.2	
801-104	F	97	1.6	-1.76	5.1	24.3	
801-105	M	97	1.6	-1.85	7.8	23.7	
801-106	F	63	3.2	-1.95	9.6	36.1	
801-107	M	80	1.6	-2.14	9.8	21.5	
801-108	F	89	1.6	-2.32	6.5	21.5	
801-109	M	122	3.2	-2	7.5	19.5	
801-110	M	105	1.6	-1.88	9.9	40.3	

- Mean Height SDS is lower in the 3.2 mg cohort than in the 1.6 mg cohort
- 3.2 mg cohort subjects appear to be more growth hormone deficient than subjects in the 1.6 mg cohort
- Differences between cohorts suggest faster growth on treatment for 3.2 mg subjects

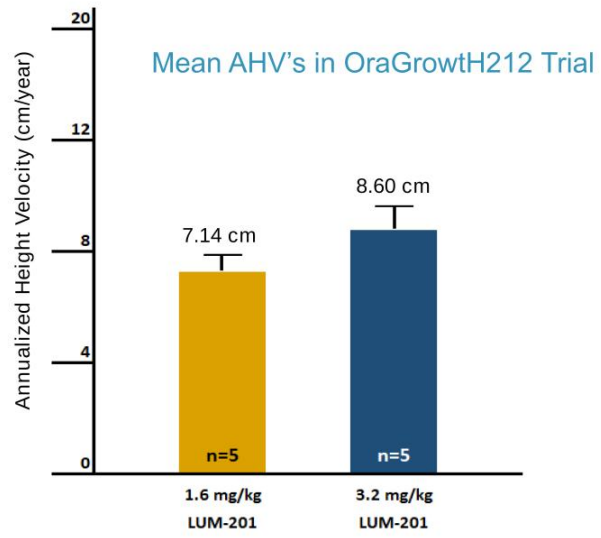


## Height velocity before and after 6 months of LUM-201 administration



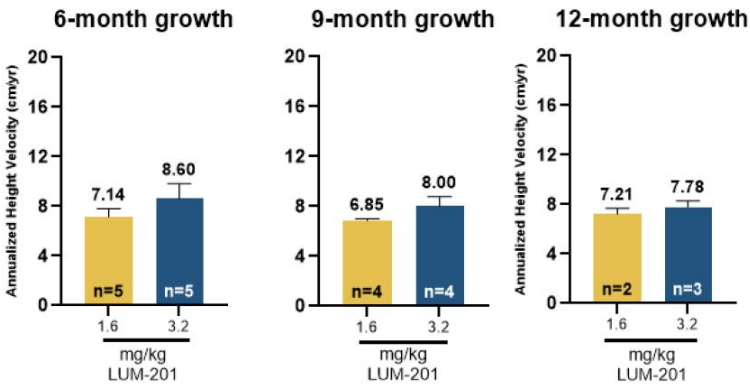


## Height velocity after 6 months of LUM-201 administration



# Durable response after 12 months of LUM-201 administration

Mean AHV's in OraGrowthH212 Trial



- OraGrowthH212 data demonstrate that growth acceleration is durable up to 12 months
- This separate study supports the narrowing of the AHV difference seen in the 210 trial as subjects approach 12 months on treatment
- A Phase 3 non-inferiority trial is expected to be a 12-month study in a much larger population



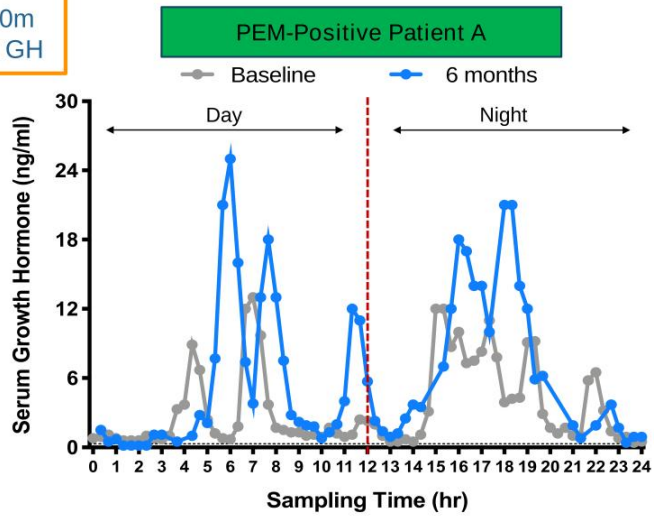
## Questions regarding the effects of LUM-201 in pediatric patients with moderate growth hormone deficiency:

- Does the oral administration of LUM-201 at the doses of 1.6 mg/kg or 3.2 mg/kg over 6 months increase height velocity? ✓
- Is the change in height velocity correlated with changes in GH pulsatility?
- Is the increase in GH pulsatility and height velocity induced by LUM-201 dose dependent?



## Prior PK/PD Data Show LUM-201 Pulsatile MOA & Potential Efficacy in Moderate Idiopathic PGHD

Q20m  
24h GH



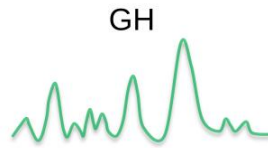
PEM+ PGHD patient administered 0.8 mg/kg/day LUM-201 for 6 months\*

- Prior PK/PD data in PEM+ subjects demonstrate pulsatile MOA of LUM-201
- Data show at 6 months, LUM-201 amplifies baseline GH peaks and increases serum GH AUC
- In prior PK/PD study, blood samples were taken every 20 minutes over 24-hour period inclusive of significant GH secretion known to occur at night
- GH secretion is not symmetric between day and nighttime hours and is not scalable to 24-hour secretion from our data



## Interim Data from OraGrowth212 Trial

Percent change from baseline in GH area under the curve (AUC) after 6-months of LUM-201 administration at doses of 1.6 or 3.2 mg/kg/day



LUM-201 Dose	1.6 mg/kg/day	3.2 mg/kg/day
Subjects (N)	4	5
Mean Change in GH AUC from Baseline	34%	197%



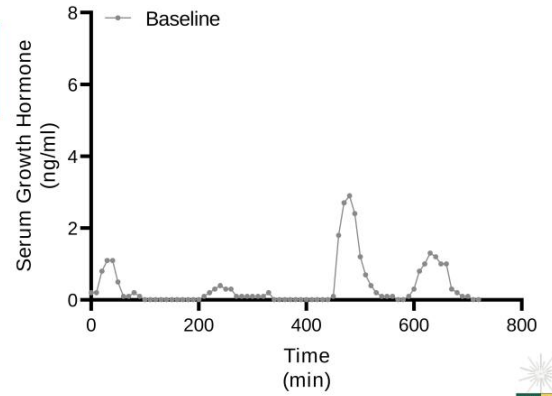
# Interim Data from OraGrowthH212 Trial

Q10m  
12h GH

## Patient 105 – 1.6 mg/kg

Patient 105

	Baseline	6 months LUM-201 1.6 mg/kg/d
IGF-1 (ng/ml)	110	
Q10m 12h GH	AUC <sub>0-12</sub> (ng*hr/ml)	273
Height velocity (cm/yr)	4.6	

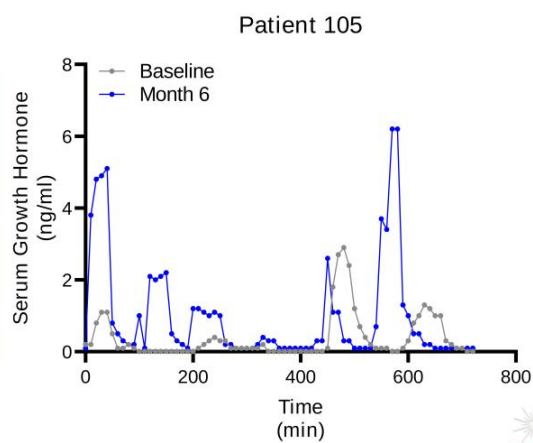


# Interim Data from OraGrowthH212 Trial

Q10m  
12h GH

## Patient 105 – 1.6 mg/kg

	Baseline	6 months LUM-201 1.6 mg/kg/d	
IGF-1 (ng/ml)	110	185	
Q10m 12h GH	AUC <sub>0-12</sub> (ng*hr/ml)	273	724
Height velocity (cm/yr)	4.6	6.8	



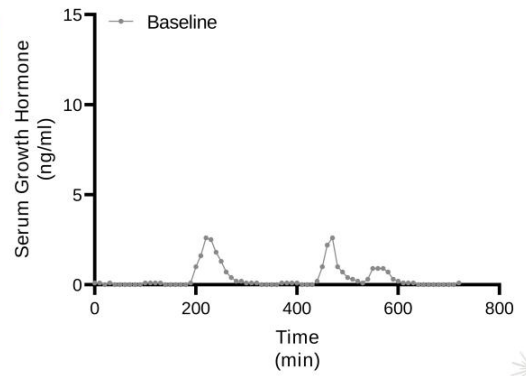
# Interim Data from OraGrowthH212 Trial

Q10m  
12h GH

## Patient 103 – 3.2 mg/kg

Patient 103

	Baseline	6 months LUM-201 3.2 mg/kg/d
IGF-1 (ng/ml)	124	
Q10m 12h GH	AUC <sub>0-12</sub> (ng*hr/ml)	276
Height velocity (cm/yr)	3.4	





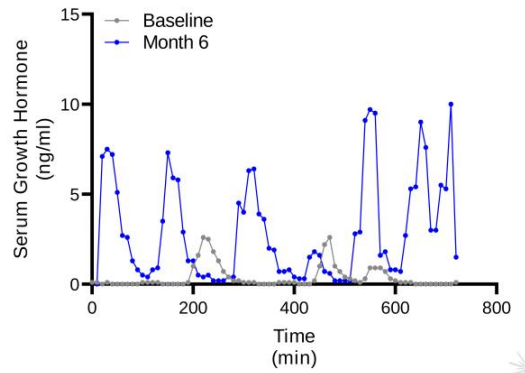
# Interim Data from OraGrowthH212 Trial

Q10m  
12h GH

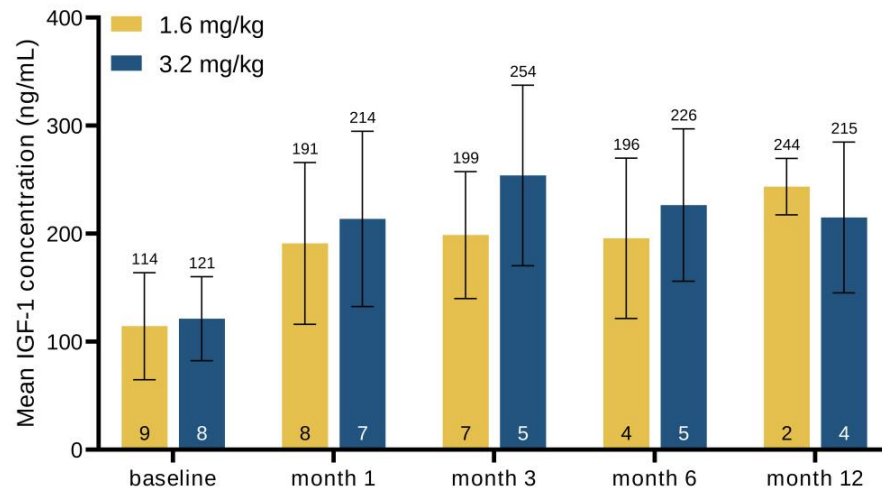
## Patient 103 – 3.2 mg/kg

	Baseline	6 months LUM-201 3.2 mg/kg/d	
IGF-1 (ng/ml)	124	262	
Q10m 12h GH	AUC <sub>0-12</sub> (ng*hr/ml)	276	1959
Height velocity (cm/yr)	3.4	8.3	

Patient 103



## IGF-1 serum concentrations after daily LUM-201 administration in OraGrowthH212 Trial



## OraGrowthH212 Interim results

- We have documented an increase in the height velocity compared to baseline in a group of prepubertal patients with moderate GH deficiency treated with two doses of LUM-201
- The improved height velocity during LUM-201 administration appears to be correlated with an increase in GH pulsatility after 6 months of therapy with this drug
- In this limited sample size, these preliminary findings suggest that the changes in height velocity and GH pulsatility induced by LUM-201 may be dose-dependent



# Institute of Maternal and Child Research Pediatric Team, University of Chile

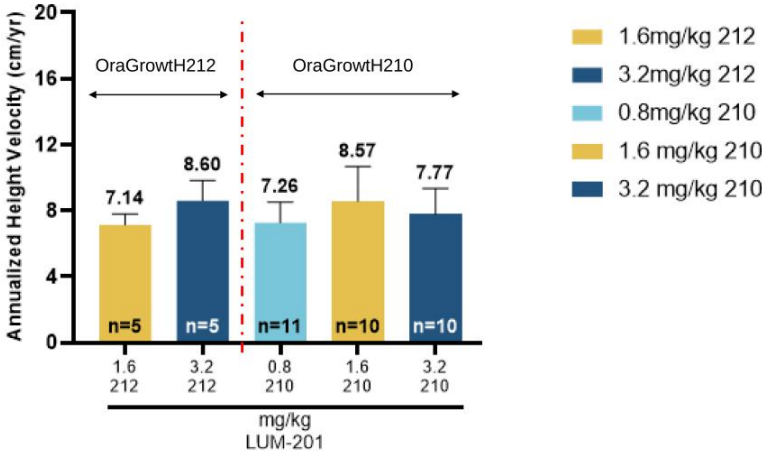


Rick Hawkins, CEO & Chairman

Lumos Pharma, Inc.

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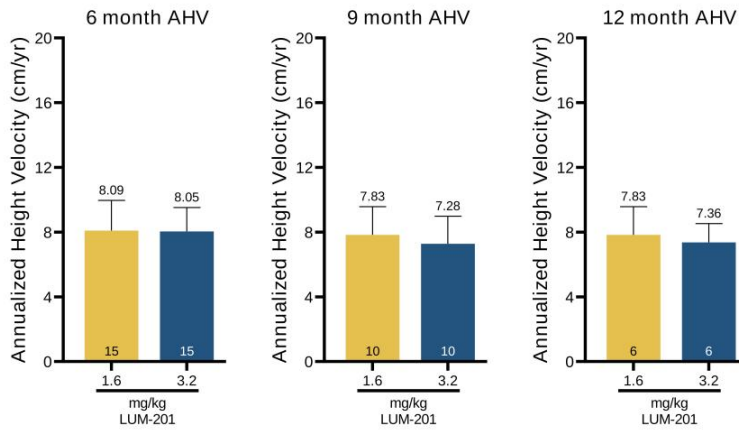
OraGrowthH212 & OraGrowthH210 Comparative AHVs at 6 Months



Conclusions

- OraGrowthH212 Trial results showed a similar growth rate to that seen in the OraGrowthH210 Trial
- Anticipate fully enrolled datasets and larger N from both trials to strengthen these results
- Anticipate larger Phase 3 trial to further support the LUM-201 AHVs seen in both OraGrowthH210 & OraGrowthH212 trials

Annualized Height Velocity for LUM-201  
 Combined Data from OraGrowthH210 & OraGrowthH212 Trials



40

**Conclusions**

- Post-hoc analysis of combined data conducted to determine optimal dose for Phase 3
- Comparable mean AHVs for top 2 LUM-201 doses seen at 6, 9, and 12 months
- Combined interim data supports selection of 1.6 mg/kg/day dose for pivotal Phase 3 trial

## Interim Analysis: LUM-201 Met Expectations in Idiopathic (PEM+) PGHD

### Expected annualized height velocity (AHV) was met

- AHV of 8.6 cm at 6-months on 1.6 mg/kg/day LUM-201, in line with 8.3 cm expected in PEM+ PGHD

### Durability of growth response was observed at 9 and 12 months

- LUM-201 AHVs are sustained & converge with rhGH AHVs at 12-month treatment interval

### Safety and tolerability profile

- No treatment related SAEs, no trial dropouts due to AEs, and no meaningful safety signal

### Evidence of a dose response & Phase 3 dose identified

- Interim safety and efficacy data support selection of 1.6 mg/kg/day for Phase 3

### Data support potential for oral LUM-201 to disrupt injectable PGHD market

- ~\$3.4 billion worldwide GHD market treated by injectable rhGH primed for conversion to oral therapy

SAEs = Serious adverse events   AEs = Adverse events



# Questions & Answers

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