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

June 21, 2023
Date of Report (date of earliest event reported)

LUMOS PHARMA, INC.

(Exact name of registrant as specified in its charter)

Delaware	**001-35342**	**42-1491350**
(State or other jurisdiction of incorporation or organization)	(Commission File Number)	(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 June 21, 2023, Lumos Pharma, Inc. issued a press release titled "Lumos to Highlight New LUM-201 Data and Analysis Presented at ENDO 2023 in Virtual KOL Webinar."

A copy of the press release and the presentation materials are attached hereto as Exhibit 99.1 and 99.2, respectively, and are incorporated herein by reference.

Item 9.01. Financial Statements and Exhibits.

(d) Exhibits.

Exhibit Number	Description
99.1	Press Release, dated June 21, 2023, entitled "Lumos to Highlight New LUM-201 Data and Analysis Presented at ENDO 2023 in Virtual KOL Webinar."
99.2	Presentation Slide Deck

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: June 21, 2023

LUMOS PHARMA, INC.,
a Delaware corporation

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

Lumos to Highlight New LUM-201 Data and Analysis Presented at ENDO 2023 in Virtual KOL Webinar

Webinar to Review Data Demonstrating Potential Drug Effect and Durable Response

Webinar to be held Today June 21, 2023 at 11:00 AM Eastern Time

AUSTIN, TX, June 21, 2023 – Lumos Pharma, Inc. (NASDAQ:LUMO), a biopharmaceutical company advancing an oral therapeutic candidate for idiopathic Pediatric Growth Hormone Deficiency (iPGHD) through Phase 2 clinical trials, is hosting today a virtual Key Opinion Leader (KOL) Webinar where Drs. Fernando Cassorla and Michael Tansey will highlight the encouraging new data and analysis on oral LUM-201 for idiopathic PGHD from the Phase 2 PK/PD OraGrowtH212 and dose-finding OraGrowtH210 Trials presented at the Endocrine Society (ENDO) Annual Meeting, held in Chicago, Illinois, June 15-18, 2023.

The event will feature presentations by KOLs in the field of pediatric endocrinology, Fernando Cassorla, MD, Chief of Pediatric Endocrinology, University of Chile, and Michael Tansey, MD, Clinical Professor of Pediatrics-Endocrinology and Diabetes, University of Iowa, Carver College of Medicine, who will review interim data from our Phase 2 OraGrowtH210 and OraGrowtH212 Trials presented at ENDO. Drs. Cassorla and Tansey will be available to answer questions following their formal presentations. To register for the virtual KOL Event, please click through the link HERE.

Drs. Cassorla and Tansey gave two oral presentations in the *Update on Growth Disorders* session at the 2023 ENDO Meeting. Presentation slides will be available from the Events and Presentations section of the Lumos website.

Dose Responsiveness of LUM-201 as Measured by Acute GH Response and IGF-1 and Annualized Height Velocity (AHV) Measured at 6 Months in the Interim Analysis of the OraGrowtH212 Study in Idiopathic Pediatric Growth Hormone Deficiency (iPGHD) **(**Fernando Cassorla, MD, Chief of Pediatric Endocrinology, University of Chile)

- New data from OraGrowtH212 trial shows durable response after 12 months of LUM-201 administration
- Clear evidence of potential drug effect observed in consistent improvement in average height velocity over baseline
- Treatment with LUM-201 increased serum IGF-1 concentration and SDS values, which remained within normal range while contributing to meaningful increases in height velocity
- Data support physiologic mechanism of action of LUM-201

Growth Response of Oral LUM-201 in OraGrowtH210 and OraGrowtH212 Trials in Idiopathic Pediatric Growth Hormone Deficiency (iPGHD): Combined Analysis Interim Analysis Data **(**Michael Tansey, MD, Clinical Professor of Pediatrics-Endocrinology and Diabetes, University of Iowa)

- Dr. Tansey presented new analysis of combined interim data from two Phase 2 trials at the 1.6 mg/kg/day and 3.2 mg/kg/day doses, including 15 subjects from the OraGrowtH212 Trial and 20 subjects from the OraGrowtH210 Trial
- Results of the analysis of the additional OraGrowtH212 subjects combined with OraGrowtH210 subjects continue to demonstrate that there is a durable response to LUM-201 from 6 to 12 months
- Pre-treatment baseline AHV data, which was not captured for all of the subjects in our database, was available for 31 of the 35 subjects and showed that LUM-201 at both the 1.6 mg/kg/day and 3.2 mg/kg/day produced clinically meaningful increase in AHV from baseline
- No treatment related Serious Adverse Events (SAEs), no discontinuation due to AEs, and no meaningful safety signals observed

KOL Biographies

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, beginning in 1979 Dr. Cassorla 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. He 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 and was elected to the Chilean Academy of Medicine for a lifetime position in 2003.

Michael Tansey, M.D. is currently Clinical Professor, Department of Pediatrics, Division of Pediatric Endocrinology and Diabetes, University of Iowa, Iowa City, Iowa, a position he has held since 2012, having first served as Clinical Assistant Professor there 2001-2006, then as Clinical Associate Professor 2006-2012. Dr. Tansey also currently serves as Clinical Director for Division of Pediatric Endocrinology and Diabetes, Department of Pediatrics, University of Iowa. He has been a co-investigator for one of the 5 clinical centers for the NIH funded Diabetes Research in Children Network “DirecNet” group since 2001 and has co-authored numerous peer-reviewed scientific publications on brain function and growth in children with Type 1 diabetes. Dr. Tansey received his MD from Loyola Stritch School of Medicine, Maywood, Illinois, and completed his residency in pediatrics and his fellowship in pediatric endocrinology at the University of Iowa Children’s Hospital and University of Iowa Hospitals and Clinics, respectively. He has received several awards including the Riesz Award, University of Iowa, and the Mary Tyler Moore and S. Robert Levine, MD, Excellence in Clinical Research Award.

About Pediatric Growth Hormone Deficiency and LUM-201

Pediatric Growth Hormone (GH) Deficiency is the consequence of inadequate secretion of growth hormone from the pituitary gland in children resulting in low GH in the body, insufficient production of downstream signaling molecules required for growth, and the subsequent lack of growth. LUM-201, also known as ibutamoren, is an orally administered investigational small molecule that promotes the secretion of GH from the pituitary gland and represents an opportunity for moderate idiopathic PGHD patients – the majority of the total PGHD population[1] – to

avoid the daily or weekly injections involved with current or forthcoming therapies. LUM-201 has been observed to increase the amplitude of endogenous pulsatile GH secretion, which mimics the natural pattern of GH secretion.

[1] Blum et al JES 2021

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. Lumos Pharma's lead therapeutic candidate is LUM-201, an oral growth hormone stimulating small molecule, currently being evaluated in several Phase 2 clinical trials for the treatment of idiopathic Pediatric Growth Hormone Deficiency (iPGHD): the dose-finding OraGrowtH210 Trial; the PK/PD mechanistic OraGrowtH212 Trial; and a switch trial, the OraGrowtH213 Trial. If approved by the FDA, LUM-201 would provide an orally administered alternative to recombinant growth hormone injections that PGHD subjects 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/.

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lumos
PHARMA
KOL Event
Response to LUM-201 in Moderate PGHD
Fernando Cassorla, MD and Michael Tansey, MD
June 21, 2023

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.

This presentation contains forward-looking statements of Lumos that involve substantial risks and uncertainties. All such statements contained in this presentation are forward-looking statements within the meaning of The Private Securities Litigation Reform Act of 1995.

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 therapeutics that are safe, efficacious, and offer a 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, momentum building in our LUM-201 program for PGHD, anticipated timing of interim analyses of trials, LUM-201's therapeutic potential when administered to pediatric subjects with idiopathic or moderate growth hormone deficiency, that the interim sample size should be adequate to provide an initial indication of LUM 201's impact, expecting the primary outcome data readout for our trials, market size potential for LUM-201, predictions regarding LUM-201, goals with respect to LUM-201, the potential to expand our LUM-201 platform into other indications, future financial performance, results of operations, cash position, cash use rate and sufficiency of our cash resources to fund our operating requirements through the primary outcome data readout from the OraGrowthH210 and OraGrowthH212 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. Actual results or events could differ materially from the plans, intentions and expectations disclosed in the forward-looking statements that we make due to a number of important factors, including potential material differences between the interim results of our LUM-201 trials and the final results of the trials which are not known at this time, the effects of pandemics (including COVID-19), other widespread health problems, the Ukraine-Russia conflict, the outcome of our future interactions with regulatory authorities, our ability to project future cash utilization and reserves needed for contingent future liabilities and business operations, the ability to obtain the necessary patient enrollment for our product candidate in a timely manner, the ability to successfully develop our product candidate, the timing and ability of Lumos to raise additional equity capital as needed and other risks that could cause actual results to differ materially from those matters expressed in or implied by such forward-looking statements. 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 our Annual Report on Form 10-K for the year ended December 31, 2022, as well as other reports filed with the SEC including our Quarterly Reports on Form 10-Q filed after such Annual Report. 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 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. 5.3.2023

Agenda

Welcome & Overview

- Rick Hawkins, *Chief Executive Officer & Chairman*

Clinical Development Program Outlook

- John McKew, PhD, *President & Chief Scientific Officer*

ENDO Presentation on OraGrowthH212 Interim Data

- Fernando Cassorla, MD, *University of Chile*

ENDO Presentation on Combined OraGrowthH210 & '212 Interim Data

- Michael Tansey, MD, *University of Iowa*

Questions & Answers

Key Opinion Leaders in the Field of Pediatric Endocrinology

Fernando Cassorla, MD 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, Dr. Cassorla 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. He 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 residency in Pediatrics 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 European Society of Pediatric Endocrinology (ESPE) International Research Award, September 2022, and was elected to the Chilean Academy of Medicine for a lifetime position in 2003.

Michael Tansey, MD is currently Clinical Professor, Dept. of Pediatrics, Division of Pediatric Endocrinology and Diabetes, University of Iowa, Iowa City, a position he has held since 2012, having first served as Clinical Assistant Professor 2001-2006, then as Clinical Associate Professor 2006-2012. Dr. Tansey also serves as Clinical Director for Division of Pediatric Endocrinology and Diabetes, Department of Pediatrics, University of Iowa. He has been a co-investigator for one of 5 clinical centers for the NIH-funded Diabetes Research in Children Network "DirecNet" group since 2001 and has co-authored numerous peer-reviewed scientific publications on brain function and growth in children with Type 1 diabetes. Dr. Tansey received his MD from Loyola Stritch School of Medicine, Maywood, Illinois, and completed his residency in Pediatrics at the University of Iowa Children's Hospital and his fellowship in Pediatric Endocrinology at the University of Iowa Hospitals and Clinics. He has received several awards including the Riesz Award, University of Iowa, and the Mary Tyler Moore and S. Robert Levine, MD, Excellence in Clinical Research Award.

Overview
Lead asset targeting children with growth disorders

Novel Oral Rare Disease Asset	• Novel **oral** therapeutic asset, **LUM-201**, for growth hormone deficiency (GHD) disorders • LUM-201 **acts within natural endocrine pathway**, differentiated from injectable therapies
Pipeline in a Product	• **Worldwide *injectable* market** for GHD disorders is **$3.4 billion**, excluding China* • Market for initial oral LUM-201 indication, **Pediatric GHD (PGHD)**, is **$1.2 billion***
Late-stage Trials in PGHD	• **Primary outcome data** for two Phase 2 OraGrowtH Trials expected **4Q 2023** • PEM strategy **de-risks trials** by identifying and enrolling likely LUM-201 responders**
Solid Financial Position	• Cash balance of **$58.0 million** as of close of **1Q 2023** • Cash runway **into 3Q 2024,** beyond Phase 2 OraGrowtH Trials primary outcome data

PGHD = Pediatric Growth Hormone Deficiency
* USA, Germany, France, Italy, Spain, UK, Japan (Grandview Research, Growth Hormone Market Forecast, 2019)
** PEM (Predictive Enrichment Marker) strategy consists of screening for PEM+ PGHD patients = Baseline IGF-1 > 30 ng/ml & Peak stimulation GH ≥ 5 ng/ml from single oral dose of LUM-201

Phase 2 OraGrowtH Trials – Primary Outcome Data Due Q4 2023

OraGrowtH210 TRIAL

- **Dose-finding multi-site study**
- **N = 82** PEM+ PGHD subjects randomized
- 4 treatment arms
  - 0.8 mg/kg/day LUM-201
  - 1.6 mg/kg/day LUM-201
  - 3.2 mg/kg/day LUM-201
  - Standard dose rhGH control arm
- Primary outcome at 6 months on therapy
- On treatment for 24 months
- **To determine optimal Phase 3 dose**

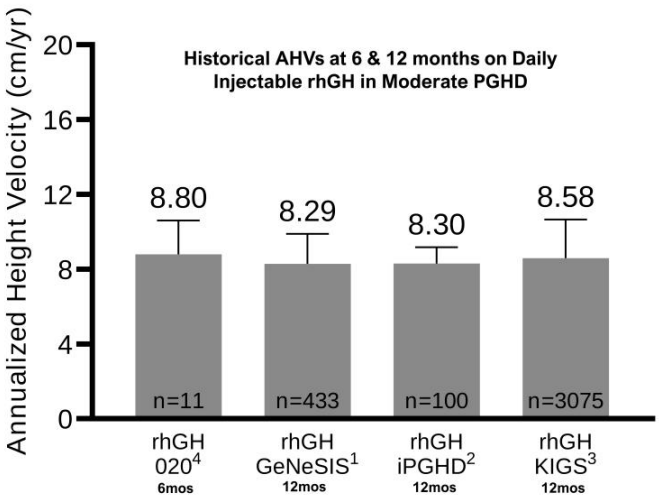
OraGrowtH212 TRIAL

- **Mechanistic single-site PK/PD study**
- **N = 22** PEM+ PGHD subjects randomized
- 2 treatment arms
  - 1.6 mg/kg/day LUM-201
  - 3.2 mg/kg/day LUM-201
- Q10 minute GH sampling for 12 hours
- Primary outcome at 6 months on therapy
- On treatment up to near-adult height
- **To demonstrate pulsatile LUM-201 MOA**

Primary Outcome Readout: 6-month AHV for All Subjects
Additional AHV Data for Subjects at 9, 12, and 18+ Months on Treatment
****Phase 2 trials are NOT powered for efficacy****

* PEM-positive (PEM+) PGHD patients = PGHD patients with baseline IGF-1 > 30 ng/ml & peak GH ≥ 5 ng/ml from single oral 0.8 mg/kg dose of LUM-201

Historical rhGH Data Set Expectations for Growth on Therapy in Moderate PGHD



Historical Datasets for Moderate PGHD

- GeNeSIS[1], iPGHD[2], and KIGS[3] datasets demonstrating AHV at 12 months on rhGH
- Merck 020[4] AHV from 6 months of rhGH
- These trials set precedent for expected growth on rhGH in moderate idiopathic PGHD

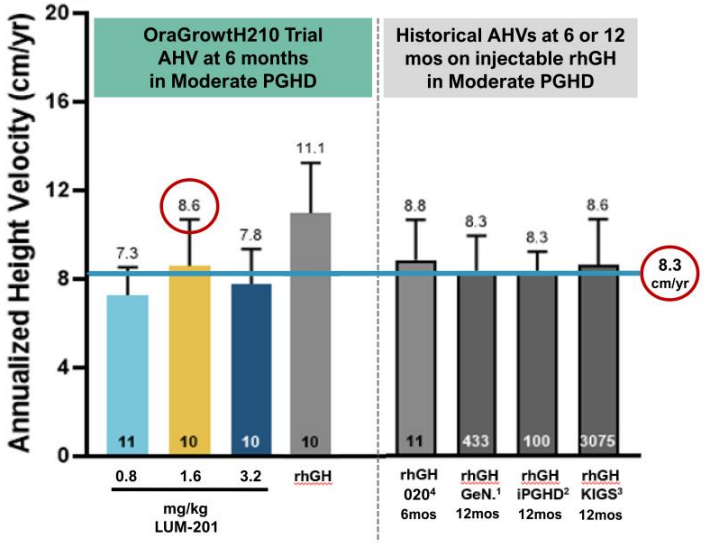
Expected Growth in OraGrowtH210 Trial

- Prediction for growth in OraGrowtH210 is AHV of ~8.3-8.6 cm/yr on both LUM-201 and rhGH based on historical data

Sources: 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.

Interim OraGrowtH210 Data: LUM-201 Growth in Line with Historical Norms

rhGH growth not in line with historical norms for moderate PGHD



OraGrowtH210 Trial Interim Results

- LUM-201 1.6 mg/kg/day growth of **8.6 cm/yr** in line with historical data
- rhGH cohort grew faster than expected due to outliers
- Cohort baseline differences contributed to growth variances[1,3]
- Converging baseline characteristics seen at full enrollment should lead to better AHV balance

AHV disparities should narrow at full data readout

Sources: [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.

OraGrowtH210 Trial Baseline Characteristics at 50% & 100% Enrollment

	At 50% enrollment			At 100% enrollment*	
	LUM-201 1.6 mg Mean (SD) N=10	**rhGH** Mean (SD) N=10		**LUM-201 1.6 mg** Mean (SD) N=22	**rhGH** Mean (SD) N=20
Age (months)	99.3 (28.3)	90.3 (26.7)		95.2 (27.3)	91.4 (23.3)
Height (cm)	114.6 (9.6)	111.6 (11.9)		113.0 (11.0)	112.3 (10.5)
Height SDS	-2.35 (0.62)	-2.29 (0.43)		-2.42 (0.68)	-2.23 (0.41)
IGF-1 SDS	-1.17 (0.72)	-1.37 (0.48)		-1.40 (0.57)	-1.39 (0.47)
MPH (cm)	166.98 (7.15)	168.78 (8.85)		165.4 (7.4)	169.1 (8.26)
MPH SDS Δ	1.76 (0.60)	1.76 (0.73)		1.69 (0.81)	1.91 (0.65)
BA Delay (yrs)	1.9 (0.5)	1.8 (1.0)		1.8 (0.9)	1.9 (0.9)
BMI SDS[1]	-0.35 (0.79)	+0.31 (1.05)		-0.27 (0.90)	+0.01 (0.95)

Imbalance between LUM-201 & rhGH arms narrows at full enrollment, which we expect will diminish the rhGH outlier impact

* Preliminary assessment
[1] Yang, et al. Nature Sci Rep 2019, 9(1); 16181
SDS = Standard deviation score MPH = Mid-parental height (Child's target height) MPH SDS delta = (MPH SDS) – (Height SDS) BA = Bone age BMI = Body mass index

Expectations for a Registrational Phase 3 Trial in Moderate PGHD
Based on recent peer registrational trials in PGHD

Projected Design for Phase 3 Trial	• International multi-center trial • ~200 PEM-positive (PEM+) moderate idiopathic PGHD subjects* • Subjects randomized 2:1 daily oral LUM-201 vs daily injectable rhGH • Stratification by age and 2-3 other factors based on Phase 2 data • 12-month treatment period
Anticipated Endpoints for Phase 3 Trial	• Primary endpoint: AHV at 12 months on treatment • Non-inferiority AHV margin of ~2 cm between LUM-201 & rhGH arms at 12 months

* PEM-positive (PEM+) PGHD patients = PGHD patients with baseline IGF-1 > 30 ng/ml & peak GH ≥ 5 ng/ml from single oral 0.8 mg/kg dose of LUM-201

ENDO 2023 – Session OR21-03

Dose Responsiveness of LUM-201 as Measured by Acute GH Response and IGF-1 and Annualized Height Velocity (AHV) Measured at 6 Months in the Interim Analysis of the OraGrowtH212 Study in Idiopathic Pediatric Growth Hormone Deficiency (iPGHD)

Cassorla F[1], MD; Román R[1], MD; Johnson M[2], PhD; Smith C[2], MS; Avila A[1], RN; Iñiguez G[1], PhD; Baier I[1], MD; Said D[1], RN; Karpf DB[2], MD; McKew JC[2], PhD; Thorner M[2], MB BS, DSc

OraGrowtH212 TRIAL

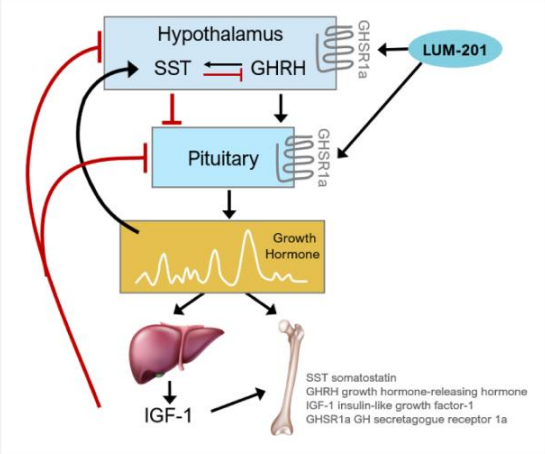
[1]University of Chile, Santiago, Chile
[2]Lumos Pharma, Austin, TX

Disclosure

Dr. Cassorla is an investigator for clinical studies with LUM-201 at the University of Chile (Sponsor - Lumos Pharma, Inc.) and has previously acted as a consultant for Debiopharm, Pfizer, Merck, Novo Nordisk 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.

LUM-201 (ibutamoren) – Mechanism of Action



Oral LUM-201 is a growth hormone (GH) secretagogue

- Acts as a durable agonist of GH Secretagogue Receptor (GHSR1a) to stimulate GH release[1]
- LUM-201 has been observed to increase the amplitude of endogenous, pulsatile GH secretion over 24 hours[2,3]
- Another differentiating feature vs rhGH is the natural negative feedback mechanisms, which limit the potential for hyperstimulation and excessive increases in IGF-1
- LUM-201 promotes pulsatile GH secretion in a selective PGHD Population

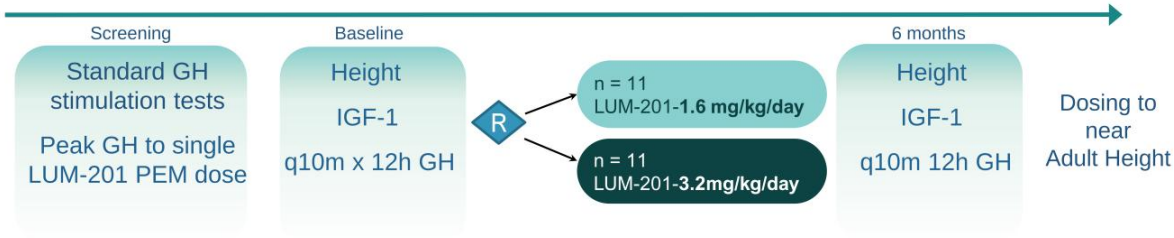
☑ Moderate Idiopathic PGHD - Axis Responsive

1. Howard 1996 Science 273:974-977
2. Nass 2008 Ann Intern Med 149:601-611
3. Chapman 1997 J Clin Endocrinol Metab 82:3455-3463

Phase 2- Pulsatility and PK/PD Study Design

Naive Idiopathic PGHD Patients

OraGrowtH212 TRIAL



Study Information

- Open-label study: N = 22
- PGHD subjects that are rhGH-treatment naïve
- Inclusion: Height < 2 SD, delayed bone age, peak GH response to a clonidine stimulation test between 3 and 10 ng
- Dosing to near-adult height
- Single, specialized clinical site
  University of Chile, Santiago

Primary Endpoints:

- Assess LUM-201 effect on endogenous GH pulsatility and Annualized Height Velocity (AHV)
- Evaluate PK/PD in children

Goals:

- Confirm prior PK/PD data in adults & subset of Merck 020 trial
- Support future regulatory filings & commercialization

Questions

1. Does LUM-201 dose-dependently augment endogenous GH pulses in patients with Idiopathic Pediatric Growth Hormone Deficiency (iPGHD)?

2. Will increased amplitude of GH pulsatility and increase in IGF-1 within normal range improve height velocity?

3. Is the effect on AHV durable out to 12 months?

Baseline Demographics

Subjects N=15	1.6 mg N=8	3.2 mg N=7
	Mean (SD)	
Age (mos)	96.9 (11.9)	95.0 (22.7)
Height (cm)	115.2 (4.57)	113.1(9.97)
Height SDS	-2.12 (0.29)	-2.34 (0.45)
IGF-1 SDS	-1.1 (0.535)	-0.8 (0.377)
MPH (cm)	161.8 (6.98)	160.82 (5.73)
MPH SDS Δ	0.73 (0.47)	0.81 (0.43)
BA Delay (yrs)	1.50 (0.26)	1.83 (0.88)
BMI (SDS)	-0.18 (0.96)	+0.48 (1.02)
Male/Female%	63/37	71/29

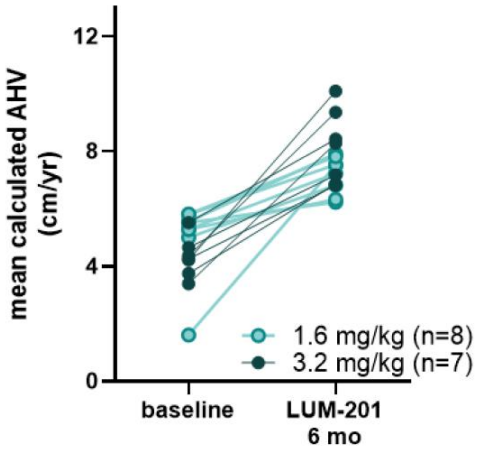
Differences between the two groups:

- Slight imbalance in age and gender
- Slight imbalance in delta below MPH, BMI, and bone age delay

KEY: SDS = Standard deviation score MPH = Mid-parental height (Child's target height) MPH SDS Δ = MPH SDS-Ht SDS BA = Bone age BMI = Body mass index

AHV Before and After 6 months of LUM-201 Treatment

OraGrowth212 TRIAL



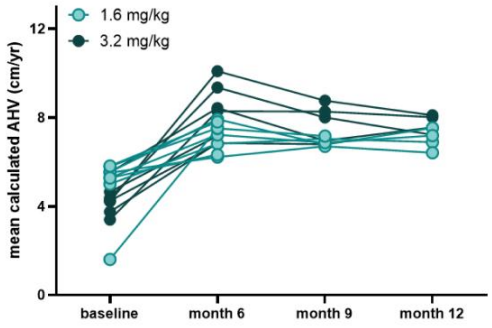
6-month observations:

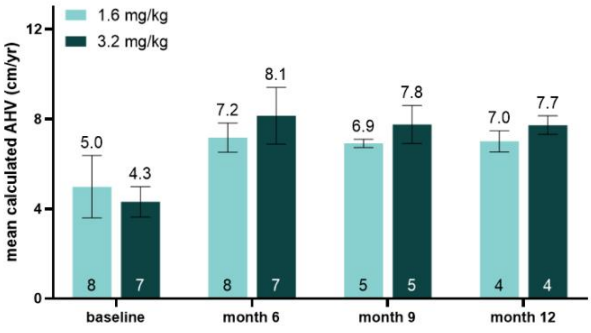
- LUM-201 raised the AHV (growth rate) from baseline after 6 months on therapy for both the 1.6 mg/kg cohort ($p = 0.0006$) and the 3.2 mg/kg cohort ($p < 0.0001$)
- No statistical difference exists between the two cohorts at each timepoint
- As expected, greater growth response was observed in patients with lower baseline height velocity

Durable Response After 12 Months of LUM-201 Administration

OraGrowtH212 TRIAL

Mean AHV's in OraGrowtH212 Trial





[1]Ranke, et.al. 2010 JCEM 95(3):1229-1237.

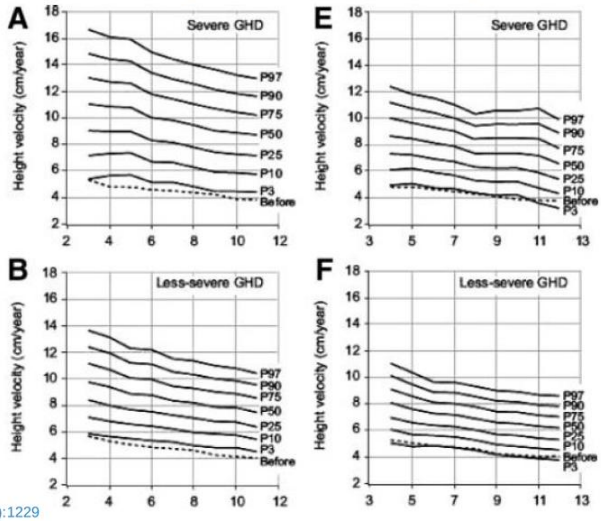
Height Velocity During Daily rhGH Therapy

OraGrowtH212 TRIAL

First year | Second year

Severe PGHD

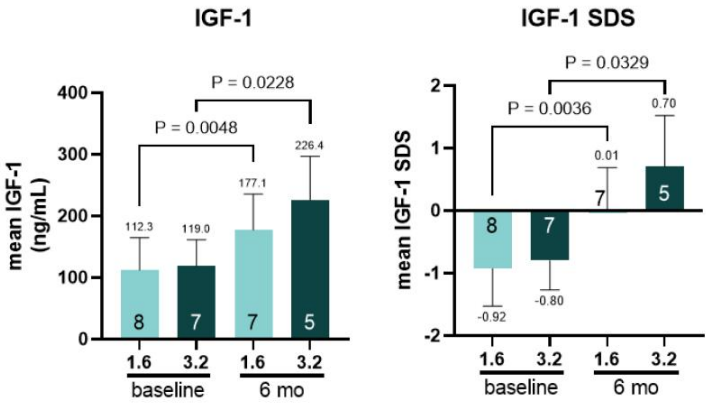
Moderate PGHD



Ranke, et.al. 2010 JCEM 95(3):1229

IGF-1 Values: Treatment with LUM-201 Increased Serum IGF-1 Concentration and IGF-1 SDS Values

OraGrowtH212 TRIAL



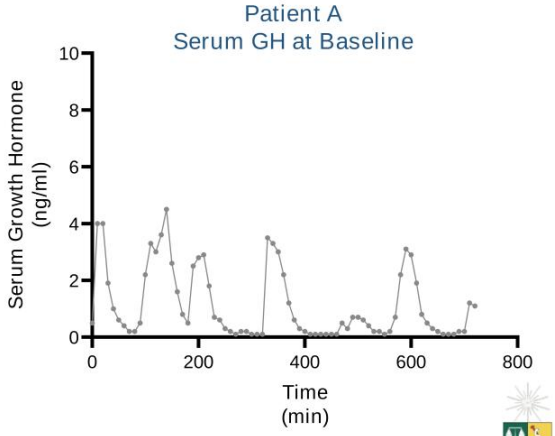
Conclusions :

- There is a significant increase in **IGF-1 levels that remains within the normal range**
- Based on the MOA of LUM-201, these data support the physiological IGF-1 feedback

IGF-1, GH Pulsatility, Height Velocity: Patient A 1.6 mg/kg/day

OraGrowth212 TRIAL

		Baseline	6 months LUM-201 1.6 mg/kg/d
IGF-1 (ng/ml)		179.3	
Q10m 12h GH	AUC_{0-12} (ng*hr/ml)	798.8	
Height velocity (cm/yr)		5.6	



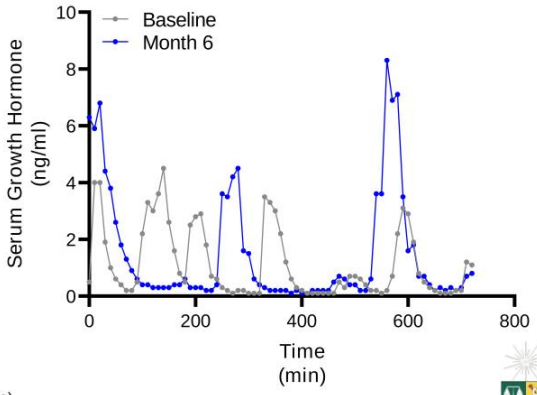
IGF-1, GH Pulsatility, Height Velocity
Patient A 1.6 mg/kg/day

OraGrowtH212 TRIAL

		Baseline	6 months LUM-201 1.6 mg/kg/d
IGF-1 (ng/ml)		179.3	289
		% change from baseline**	61%
Q10m 12h GH	AUC_{0-12} (ng*hr/ml)	798.8	1064.1
		% change from baseline**	33%
Height velocity (cm/yr)		5.6	7.9

**Percent change from baseline calculated as: (6mo value – baseline value) / (baseline value)

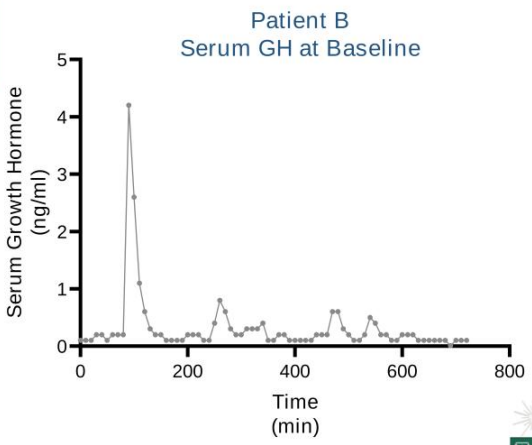
Patient A
Serum GH at Baseline & at 6 months on LUM-201



IGF-1, GH Pulsatility, Height Velocity: Patient B 3.2 mg/kg/day

OraGrowtH212 TRIAL

		Baseline	6 months LUM-201 3.2 mg/kg/d
	IGF-1 (ng/ml)	48	
Q10m 12h GH	AUC_{0-12} (ng*hr/ml)	252.9	
	Height velocity (cm/yr)	4.4	

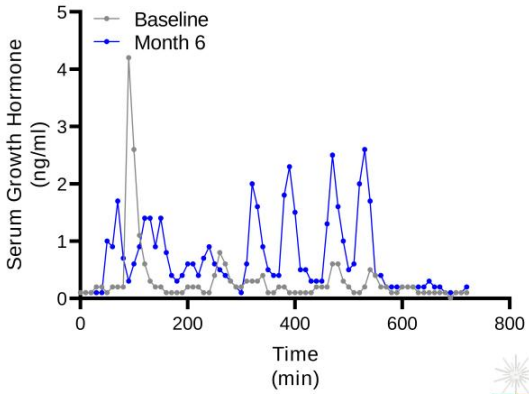


IGF-1, GH Pulsatility, Height Velocity: Patient B 3.2 mg/kg/day

		Baseline	6 months LUM-201 3.2 mg/kg/d
	IGF-1 (ng/ml)	48	111
		% change from baseline**	131%
Q10m 12h GH	AUC_{0-12} (ng*hr/ml)	252.9	481.8
		% change from baseline**	91%
	Height velocity (cm/yr)	4.4	9.4

**Percent change from baseline calculated as: (6mo value – baseline value) / (baseline value)

Patient B
Serum GH at Baseline & at 6 months on LUM-201



Interim Analysis Safety Profile

OraGrowth212 TRIAL

Safety Profile:

- No treatment-related Serious Adverse Events (SAEs) or Severe AEs
- No meaningful safety signals observed in either laboratory values, adverse event data, or in electrocardiogram values.

Most Common AEs (% of subjects) noted are:

- Transient increased appetite (76.5%)
- Pain in extremity (17.6%)
- Arthralgia (11.8%)
- Abdominal pain (5.9%)
- Influenza (5.9%)

Safety Conclusion:

- At time of interim analysis, LUM-201 was well tolerated and showed no significant safety signals

Questions

1. Does LUM-201 dose-dependently augment endogenous GH pulses in patients with idiopathic Pediatric Growth Hormone Deficiency (iPGHD)? ☑

2. Will increased amplitude of GH pulsatility, driving increased IGF-1, improve height velocity? ☑

3. Is the effect on AHV durable out to 12 months? ☑

Conclusions

- Based on Interim Analysis data, OraGrowtH212 data demonstrates that growth acceleration is durable through 12 months in our study population, pre-pubertal, treatment naïve PGHD patients.
- No statistical difference exists between the cohorts at any time point.
- Due to some baseline imbalance, the optimal dose cannot be determined from this data set.
- We plan to continue the OraGrowtH212 Trial until near adult height.
- The observed growth is in line with rhGH historical growth of 8.3-8.6 cm (KIGS [1], GeNeSiS [2]) in this moderate idiopathic PGHD population.

Sources: [1] Blum et al JES 2021, [2] Ranke et al JCEM 2010

University of Chile, Santiago

Institute of Maternal and Child Research Pediatric Team

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ENDO 2023 – Session OR21-06
Growth Response of Oral LUM-201 in OraGrowtH210 and OraGrowtH212 Trials in Idiopathic Pediatric Growth Hormone Deficiency (iPGHD): Combined Analysis Interim Analysis Data

Michael Tansey, MD
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Iowa City, Iowa

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Disclosure

Dr. Tansey is an investigator for clinical studies with LUM-201 at the University of Iowa (Sponsor - Lumos Pharma, Inc.). There are no additional disclosures for this presentation.

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.

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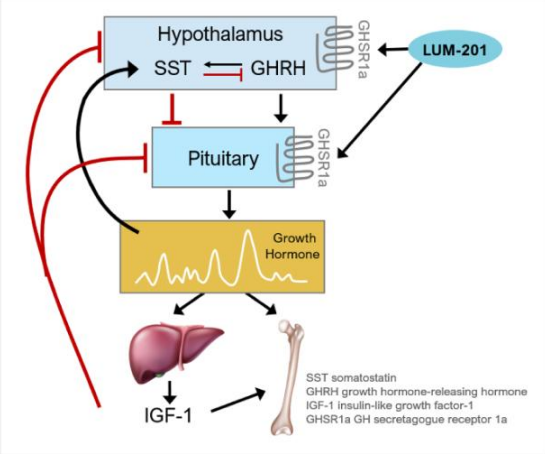
Objective of the Presentation

Report the growth response analyzing the combined interim analysis (IA) data from two Phase 2 trials studying LUM-201 at two different doses (1.6 mg/kg/day or 3.2 mg/kg/day).

IA data from both studies were combined and analyzed for calculated annualized height velocity (AHV). Baseline demographics were analyzed for the two combined cohorts.

LUM-201 (ibutamoren) – Mechanism of Action



- Oral LUM-201 is a growth hormone (GH) secretagogue
- Acts as a durable agonist of GH Secretagogue Receptor (GHSR1a) to stimulate GH release[1]
- LUM-201 has been observed to increase the amplitude of endogenous, pulsatile GH secretion over 24 hours[2,3]
- Another differentiating feature vs rhGH is the natural negative feedback mechanisms, which limit potential for hyperstimulation and excessive increases in IGF-1
- LUM-201 promotes pulsatile GH secretion in a selective PGHD population

1. Howard 1996 Science 273:974-977
2. Nass 2008 Ann Intern Med 149:601-611
3. Chapman 1997 J Clin Endocrinol Metab 82:3455-3463

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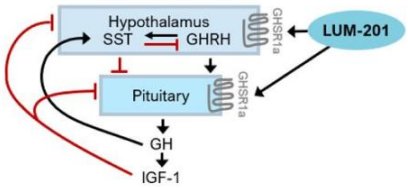
Single Stim Dose of LUM-201 Identifies PEM+ Responders

LUM-201

Single Stimulation Dose

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

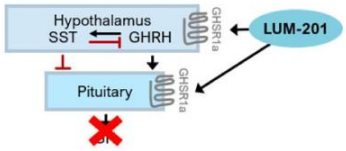


Responders to LUM-201

Moderate / Idiopathic PGHD
PEM Positive
~60% of total PGHD population[1]

Predictive Enrichment Marker Negative (PEM –)

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



Non-Responders to LUM-201

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

1 Blum 2021 JES

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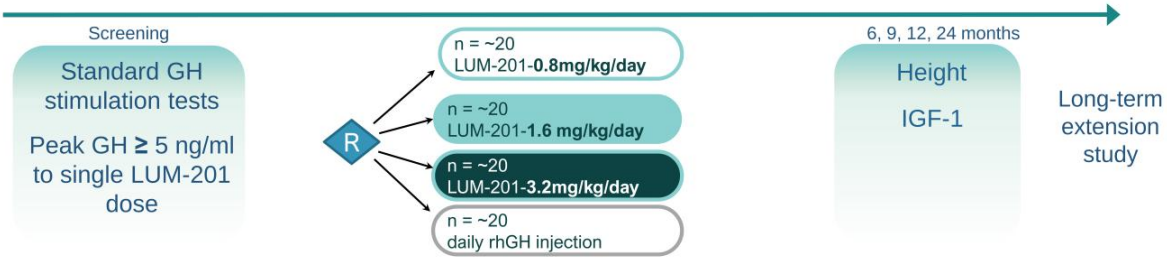
Phase 2 - Dose Finding Study Design

Naive Idiopathic PGHD Patients

OraGrowtH210 TRIAL



Study Information

- N = 82 subjects
- Only PEM (+) pre-pubertal PGHD subjects that are rhGH-treatment naïve
- Inclusion: LUM- 201 stim GH ≥ 5 ng/mL & baseline IGF-1 > 30 ng/mL, Previous iPGHD diagnosis, Height < 2 SD, delayed bone age
- 45 trial sites US and International
- Trial duration: 24 months Feb 28, 2023 enrollment completed
  Topline 6 mos height velocity data expected 4Q 2023

Primary Endpoints:

- Annualized Height Velocity (AHV)

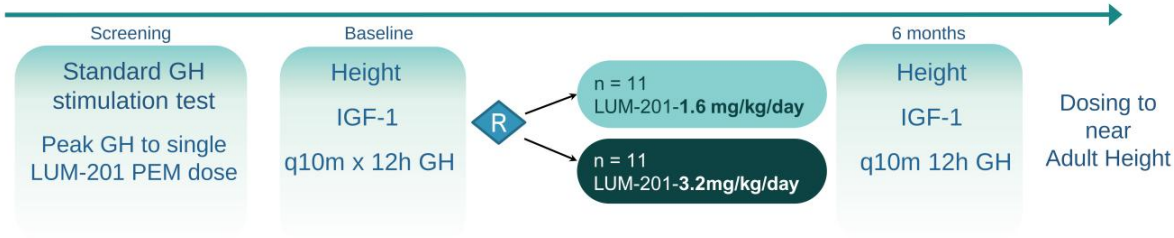
Goals:

- Prospectively confirm utility of PEM strategy
- Determine optimal Phase 3 dose

Phase 2 - Pulsatility and PK/PD Study Design

Naive Idiopathic PGHD Patients

OraGrowtH212 TRIAL



Study Information

- Open-label study: N = 22
- Pre-pubertal PGHD subjects that are rhGH-treatment naïve
- Inclusion: Height < 2 SD, delayed bone age, peak GH response to a clonidine stimulation test between 3 and 10 ng
- Dosing to near-adult height
- Single, specialized clinical site
  University of Chile, Santiago

Primary Endpoints:

- Assess LUM-201 effect on endogenous GH pulsatility and Annualized Height Velocity (AHV)
- Evaluate PK/PD in children

Goals:

- Confirm prior PK/PD data in adults & subset of Merck 020 trial
- Support future regulatory filings & commercialization

Baseline Demographics for OraGrowtH210 and OraGrowtH212

OraGrowtH210 TRIAL

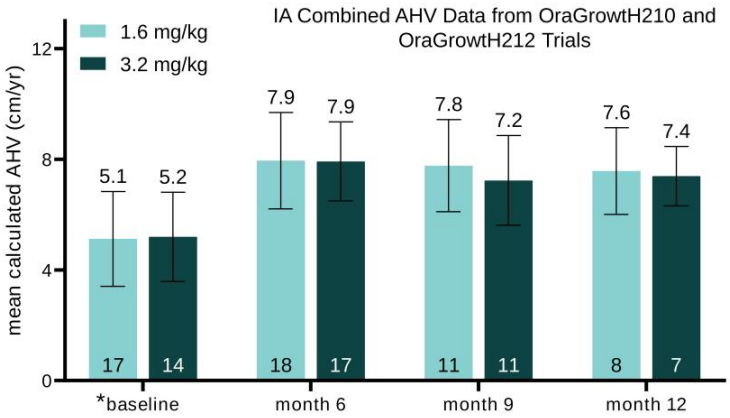
Subjects N=20	1.6 mg N=10	3.2 mg N=10
	Mean (SD)	
Age (mos)	99.3 (28.3)	96.1 (21.7)
Height (cm)	114.6 (9.6)	113.8 (8.8)
Height SDS	-2.35 (0.62)	-2.30 (0.48)
IGF-1 SDS	-1.17 (0.72)	-1.39 (0.61)
MPH (cm)	166.98 (7.15)	166.20 (8.06)
MPH SDS Δ	1.76 (0.60)	1.96 (0.83)
BA Delay (yrs)	1.91 (0.53)	2.19 (0.86)
BMI (SDS)	-0.35 (0.79)	-0.70 (0.48)
Male/Female%	60/40	40/60

OraGrowtH212 TRIAL

Subjects N=15	1.6 mg N=8	3.2 mg N=7
	Mean (SD)	
Age (mos)	96.9 (11.9)	95.0 (22.7)
Height (cm)	115.2 (4.57)	113.1(9.97)
Height SDS	-2.12 (0.29)	-2.34 (0.45)
IGF-1 SDS	-1.1 (0.535)	-0.8 (0.377)
MPH (cm)	161.8 (6.98)	160.82 (5.73)
MPH SDS Δ	0.73 (0.47)	0.81 (0.43)
BA Delay (yrs)	1.50 (0.26)	1.83 (0.88)
BMI (SDS)	-0.18 (0.96)	+0.48 (1.02)
Male/Female%	63/37	71/29

These data represent the patient data that had been collected at time of Interim Analysis calculation.
No statistically significant differences between cohorts in each trial (unpaired t-test comparing baseline mean/SD)
SDS = Standard deviation score MPH = Mid-parental height MPH SDS Δ = MPH SDS-Ht SDS BA = Bone age BMI = Body mass index

Annualized Height Velocity of 2 Doses Show Durable Response from 6-12 Months



*Pre-treatment baseline AHV was not required for this study but available data shown

Interim Analysis (IA) Results

- Interim data demonstrate LUM-201 produces durable AHV response from 6 to 12 months in moderate PGHD
- LUM-201 at both 1.6 mg/kg and 3.2 mg/kg produces a clinically meaningful increase in AHV from baseline

IA Safety Data from Combined Trials

	1.6 mg/kg	3.2 mg/kg
	N =33	N=33
Number of AEs	105	110
Subjects with AE (%)	29 (87.9%)	30 (90.9%)
Treatment Related AEs *	17	19
Subjects with Treatment Related AEs (%)	12 (36.4%)	13 (39.4%)
Subjects with SAEs (%)	0 (0%)	0 (0%)

Interim Analysis (IA) Results

- No treatment-related Serious Adverse Events (SAEs)
- No drop-outs due to SAEs or AEs
- No meaningful safety signals observed in laboratory values, adverse events data, or in EKG values
- * Treatment related AEs in both groups: Increased appetite (21), Arthralgia (6), Pain in extremity (6), Abdominal pain (2), Bone pain (1)

Conclusion

- As the growth velocity was comparable for the two doses of oral LUM-201, this analysis of the combined IA data suggests 1.6 mg/kg/day as the optimal dose for the Phase 3 trial, as doubling the dose appeared to offer no meaningful improvement in efficacy.
- Final dose determination will await final full data set analysis of both studies
- No treatment-related Serious Adverse Events, no discontinuation due to AEs, and no meaningful safety signals observed in either laboratory values, adverse event data, or in electrocardiogram values.

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Questions & Answers