Phase 2 Topline Results LUM-201 for Pediatric Growth Hormone Deficiency

OraGrowthH210 and OraGrowthH212

November 2023

Forward Looking Statements

This presentation contains forward-looking statements of Lumos Pharma, Inc. 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. This 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 the plan to have an end-of-phase 2 meeting with the FDA in the first half of 2024 and the anticipated initiation of a Phase 3 program in the second half of 2024, our Phase 2 data providing a clear path to Phase 3 in PGHD, that PEMs enrich trials for patients likely to respond to LUM-201, the expected benefits to LUM-201, 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 LUM-201 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 presentation.

11.7.2023

Phase 2 Data Provides Clear Path to Phase 3 in PGHD

OraGrowtH210 – Met All Primary and Secondary Endpoints

- ✓ LUM-201 1.6 mg/kg/day dose AHV at 6 months and 12 months were 8.2 cm/yr and 8.0 cm/yr, respectively
  - → Comparable to historical rhGH AHV data in moderate PGHD population
  - → 6 and 12-month AHV within 1.8 cm of the comparator rhGH arm
  - → 1.8 – 2.0 cm non-inferiority margin has been the historical Phase 3 standard for rhGH approvals
- ✓ Met pre-specified primary endpoint: Preliminary validation of PEM test
- ✓ Met pre-specified secondary endpoint: PEM+ classification 100% reproducible exceeds statistical objective

OraGrowtH212 – Met All Primary and Secondary Endpoints

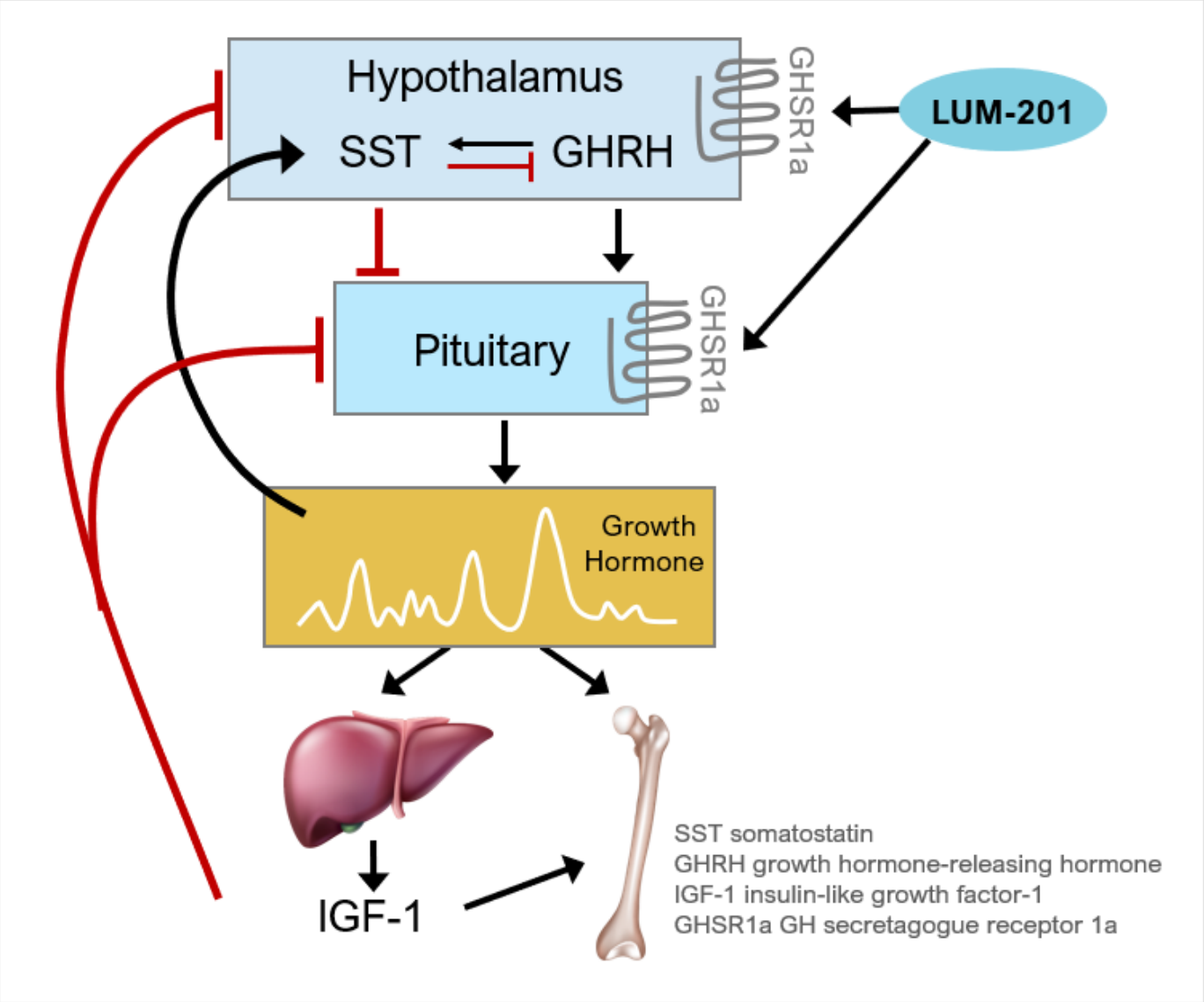
- ✓ LUM-201 restored GH secretion comparable to normal children
- ✓ Increased 6-month AHV materially from baseline
- ✓ LUM-201 normalized IGF-1 SDS values within 6 months of treatment

Secondary Observations – Durable Effect with Favorable Phase 2 Safety

- ✓ Durable effect on AHV at 12 months with 8.0 cm AHV in 1.6 mg/kg/day cohort
- ✓ Initial 24-month data demonstrate more durable effect than rhGH after year-1 treatment
- ✓ Favorable Phase 2 safety profile of LUM-201 in both studies to date

[1] AHV = Least Squares Mean Annualized Height Velocity, throughout this presentation AHV values from the OraGrowth studies are based on ANCOVA model (details provided on subsequent slides)

LUM-201 Stimulates Natural Growth Hormone Secretion



LUM-201 mimics natural release of growth hormone (GH)
Different from injections of synthetic GH

- LUM-201 is an oral GH secretagogue*
- Acts on specific receptors in hypothalamus and pituitary to stimulate release of GH[1]
- Increases the amplitude of natural pulsatile GH secretion[2,3]
- LUM-201 stimulated GH release regulated by natural GH/IGF-1 feedback mechanisms
- Differentiated mechanism versus exogenous injection of recombinant human growth hormone (rhGH) products

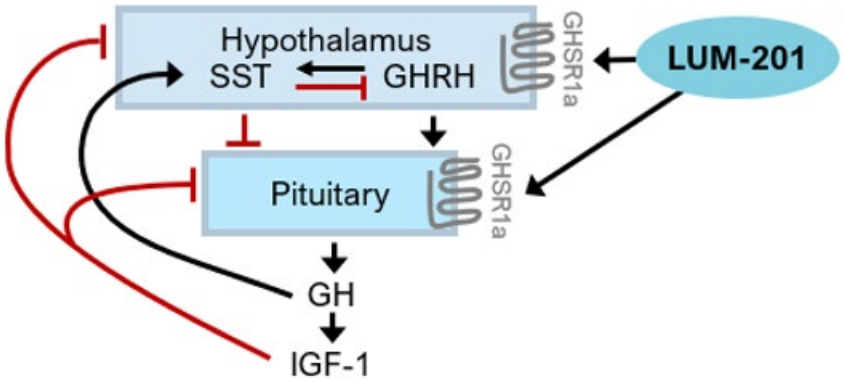
[1] Howard 1996 Science [2] Nass 2008 Ann Intern Med [3] Chapman 1997 J Clin Endocrinol Metab * GH secretagogue = molecule that stimulates the secretion of growth hormone (GH)

PEMs Enrich Trials for Patients Likely to Respond to LUM-201

LUM-201

Single Stimulation Dose Identifies LUM-201 Responders

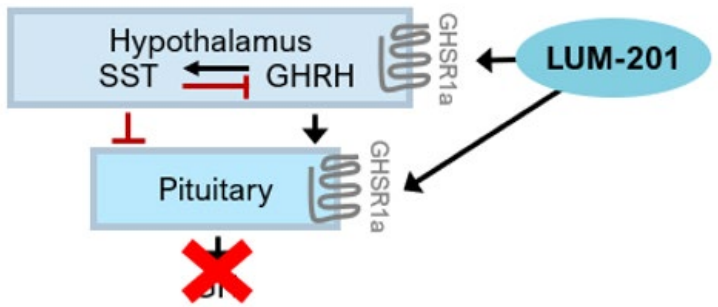
Moderate PGHD
PEM-Positive
~60% of total PGHD population[1]



Responders to LUM-201[2]

Predictive Enrichment Marker Positive (PEM+)
- Baseline IGF-1 **>** 30 ng/ml
- Stimulation LUM-201 peak GH **≥** 5 ng/ml
- Functional but reduced HP-GH axis

Severe PGHD
PEM-Negative
~40% of total PGHD population



Non-Responders to LUM-201

Predictive Enrichment Marker Negative (PEM –)
- Baseline IGF-1 **<** 30 ng/ml
- Stimulation LUM-201 peak GH **<** 5 ng/ml
- Non-functional HP-GH axis

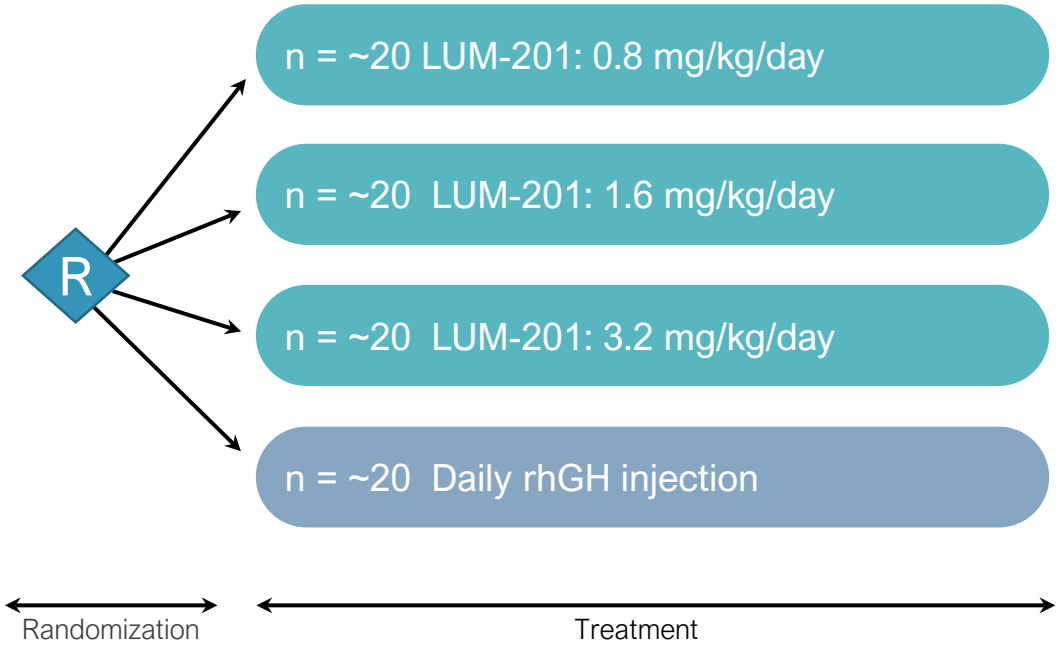
[1] Blum 2021 JES [2] Bright 2021 JES HP-GH axis – hypothalamic pituitary growth hormone axis

OraGrowtH210 Trial: Phase 2 Trial in Naïve Moderate PGHD

OraGrowtH210 TRIAL

- n = 82
- PEM(+) PGHD subjects
- Inclusion: stim GH ≥ 5 ng/ml and baseline IGF-1 >30 ng/ml
- rhGH treatment naïve
- ~45 trial sites US & International

Primary Outcome Data (n = **82**) – at **6 months** on therapy
Total Study Duration – 24 months



Objectives

Study Objectives:

- Prospectively confirm utility of PEM strategy
- Evaluate reproducibility of PEM classification
- Annualized Height Velocity (AHV)

Goals:

- Determine optimal dose for Phase 3

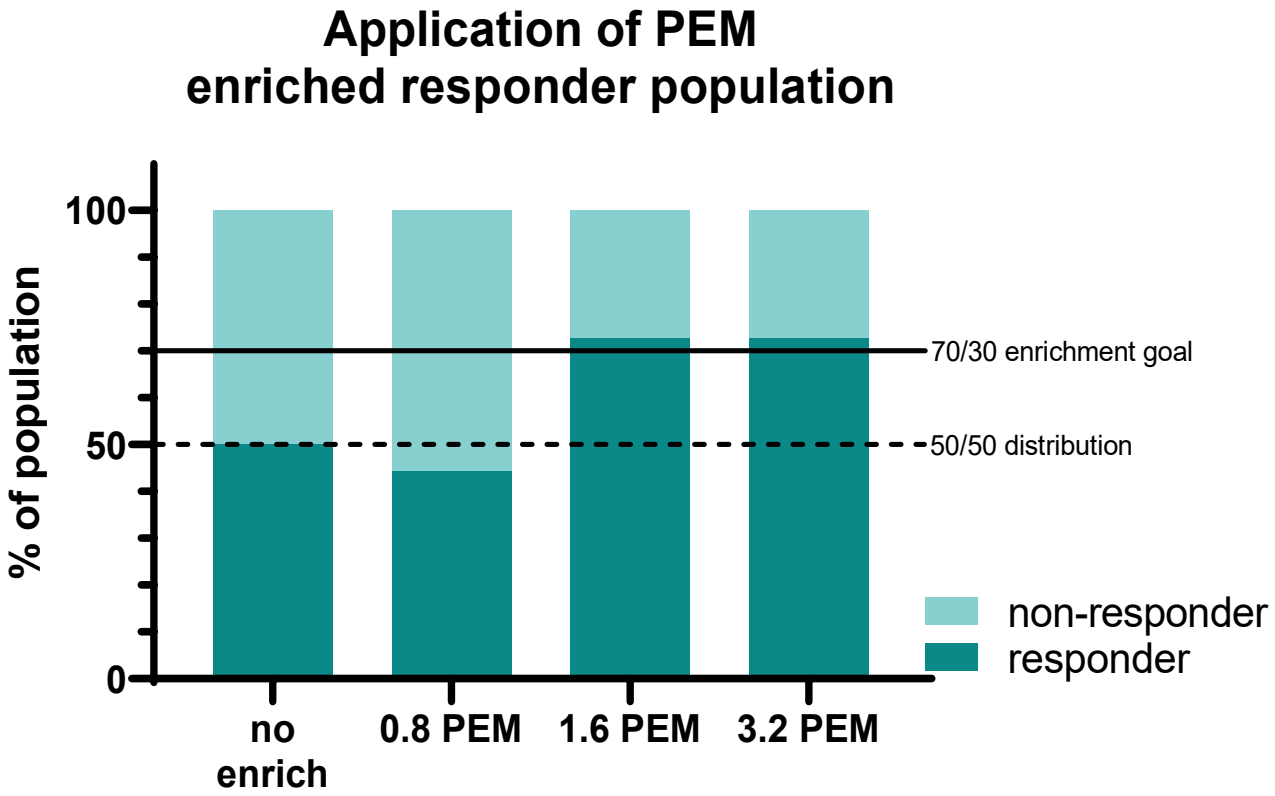
Study not powered to show statistical non-inferiority

OraGrowtH210 Baseline Demographics

	LUM-201 0.8 mg Mean (SD) N=18	LUM-201 1.6 mg Mean (SD) N=22	LUM-201 3.2 mg Mean (SD) N=22	rhGH Mean (SD) N=19
Age (months)	101.3 (29.2)	95.2 (27.3)	94.5 (21.1)	90.7 (23.7)
Height (cm)	116.4 (12.4)	113.6 (11.0)	113.8 (9.2)	112.9 (10.7)
Height SDS	-2.32 (0.30)	-2.33 (0.54)	-2.29 (0.59)	-2.19 (0.41)
IGF-1 SDS	-1.46 (0.62)	-1.38 (0.61)	-1.39 (0.53)	-1.25 (0.49)
MPH (cm)	165.3 (7.1)	164.9 (7.4)	167.4 (7.7)	169.4 (8.7)
MPH SDS Δ	-1.47 (0.67)	-1.61 (0.68)	-1.87 (0.59)	-1.94 (0.62)
BA Delay (yrs)	1.8 (0.9)	1.9 (0.8)	2.0 (0.9)	1.9 (0.9)
BMI SDS	-0.55 (1.10)	-0.18 (0.87)	-0.57 (0.99)	+0.16 (0.88)

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

OraGrowtH210 Met Primary Statistical Objective: PEM enriches the responder population



Highlights

- PEM test ensures patients enrolled in the study are capable of secreting GH in response to a single-dose of LUM-201
- PEM-positive criteria:
  - PGHD patients with baseline IGF-1 >30 ng/ml
  - Peak stimulated GH ≥ 5 ng/ml after a single 0.8 mg/kg dose of LUM-201

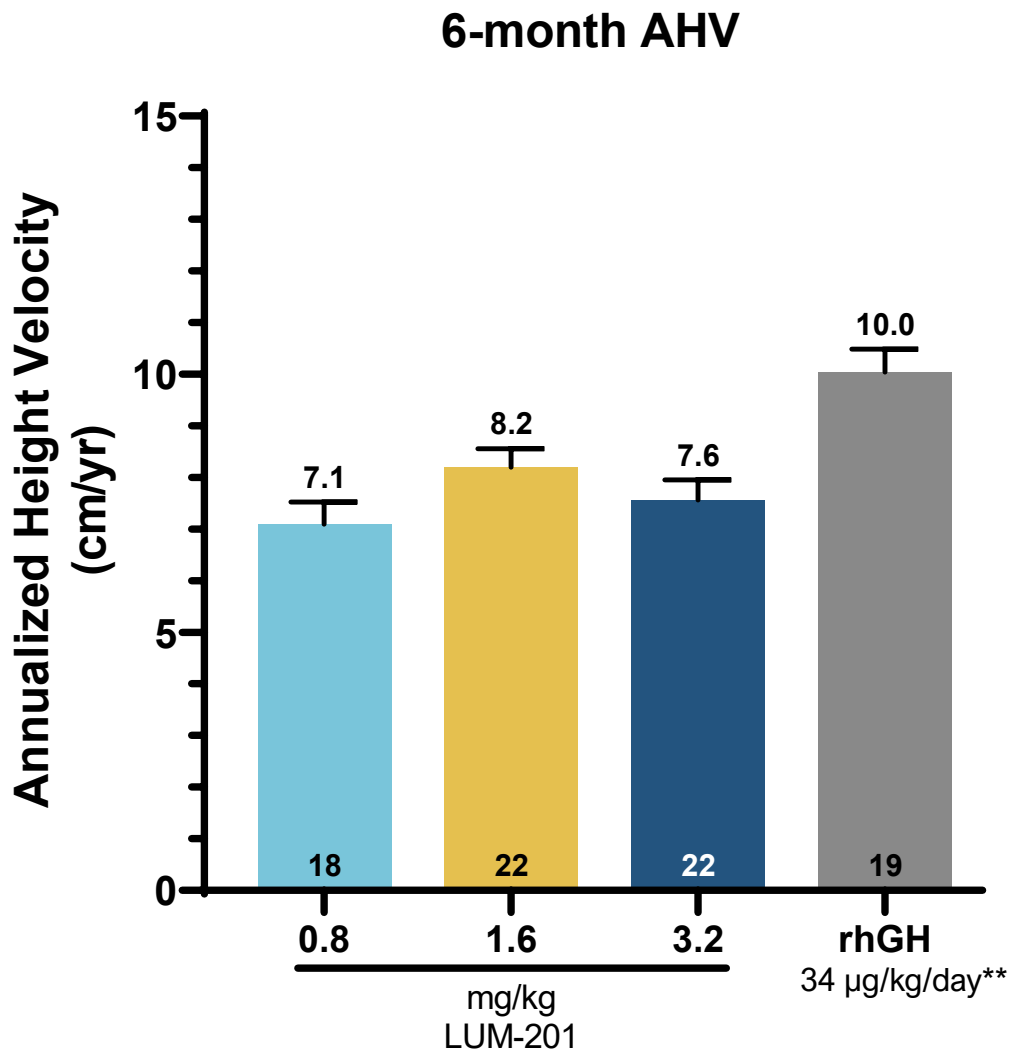
Enrichment strategy demonstrated that >70% of PEM+ subjects met pre-specified target growth in 1.6 and 3.2 mg/kg/day cohorts

OraGrowtH210 Secondary Statistical Objective: PEM test yields highly reproducible results

PEM Test Reproducibility	
Subjects with Positive Agreement on PEM Tests	76/76
Reproducibility Rate	100%
95% Confidence Interval	(95.3%, 100%)

PEM positive classification was 100% reproducible and exceeded pre-specified statistical objective

OraGrowtH210 Met Primary Objective:
6-Month AHV Supports 1.6 mg/kg as Optimal Dose for Phase 3

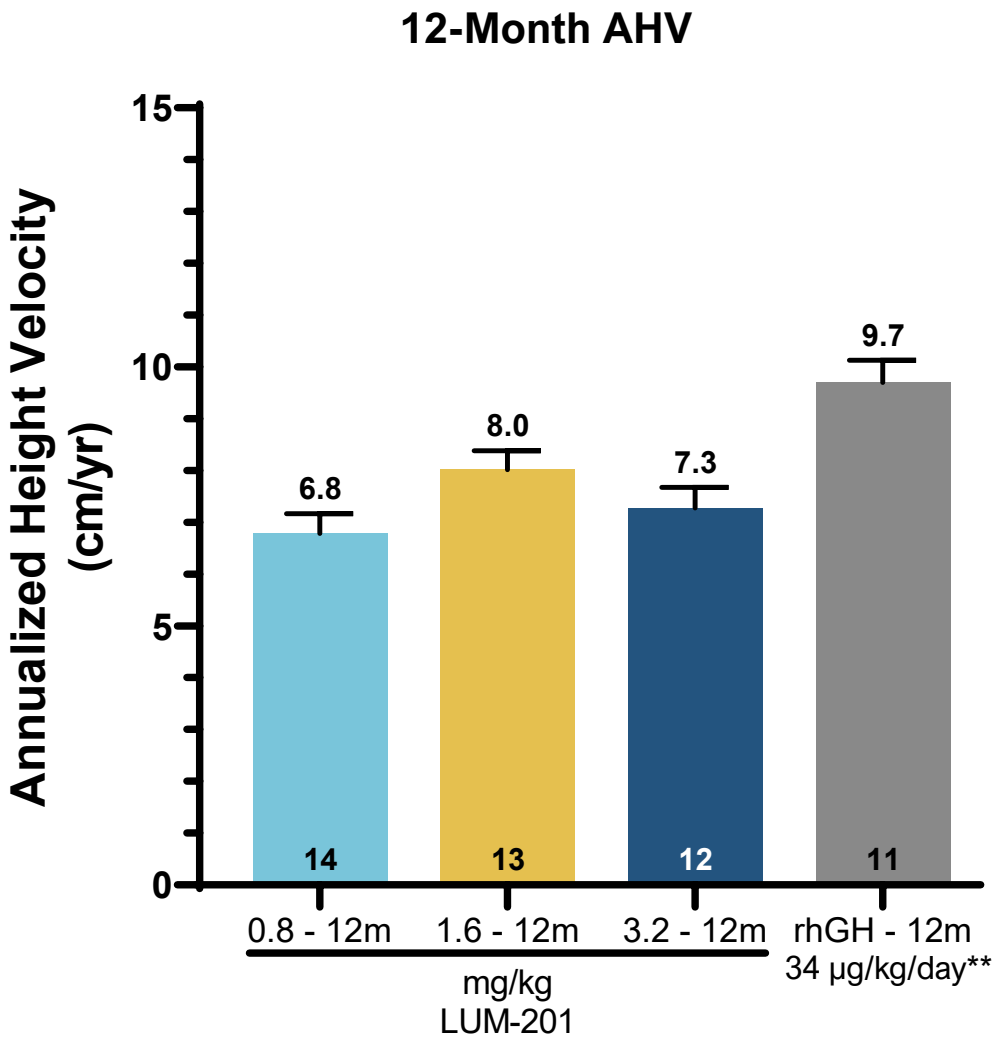


Highlights

- 1.6 mg/kg demonstrates highest LUM-201 AHV at 6 months
- 1.8 cm difference between 1.6 mg/kg LUM-201 dose and rhGH comparator arm

AHV 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
** Equates to 0.24 mg/kg/wk (approved rhGH dose range: 0.17-0.24 mg/kg/wk for Norditropin)

OraGrowtH210: 12-Month AHV Data Available for 50/81 Subjects Growth Rates are Durable at 12 Months



Highlights

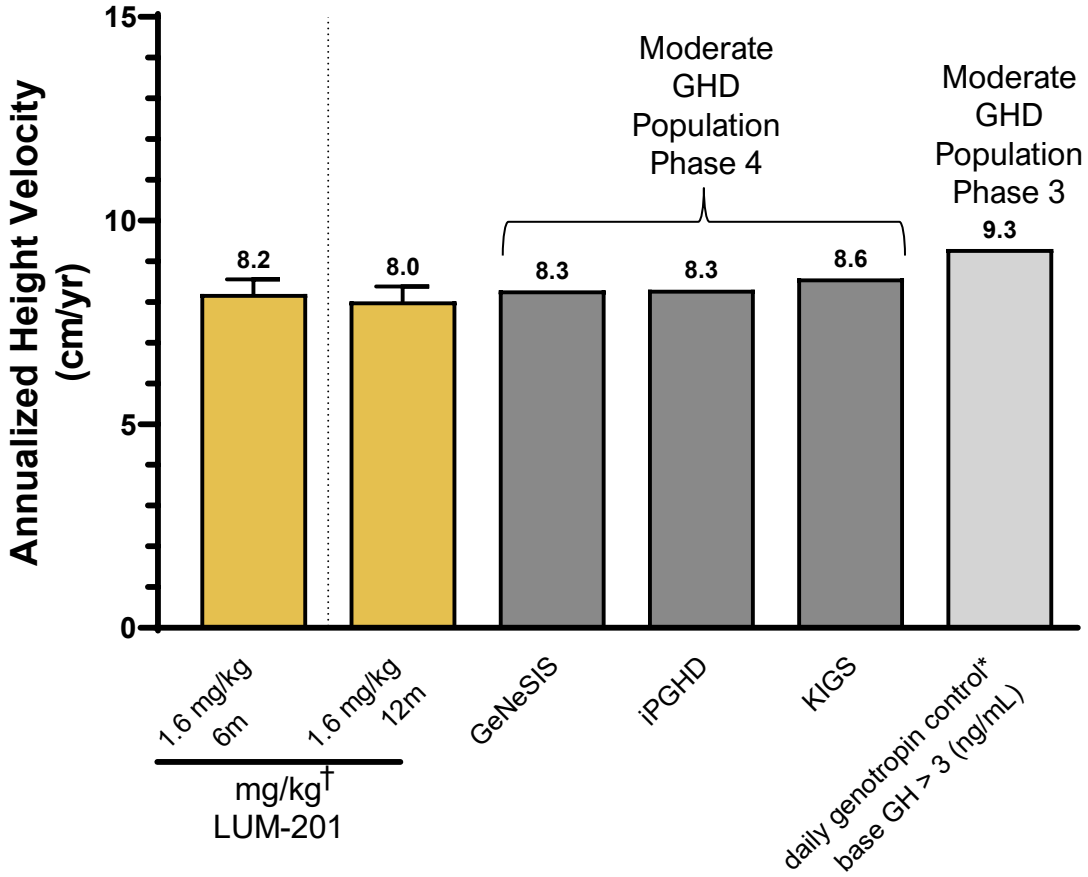
- 1.6 mg/kg best performing LUM-201 cohort
  - Growth of 8.0 cm comparable to historical 12-month AHV for moderate population
- 1.7 cm difference between 1.6mg/kg and rhGH cohorts
  - Differences less than 1.8 – 2.0 cm have been the historical Phase 3 non-inferiority margin for rhGH approvals

AHV 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

** Equates to 0.24 mg/kg/wk (approved rhGH dose range: 0.17-0.24 mg/kg/wk for Norditropin)

The N in each cohort represents the number of subjects who have received 12 months of treatment at the time we read out the 6-month primary readout

LUM-201 Growth Comparable to Multiple 12-Month Historical Datasets

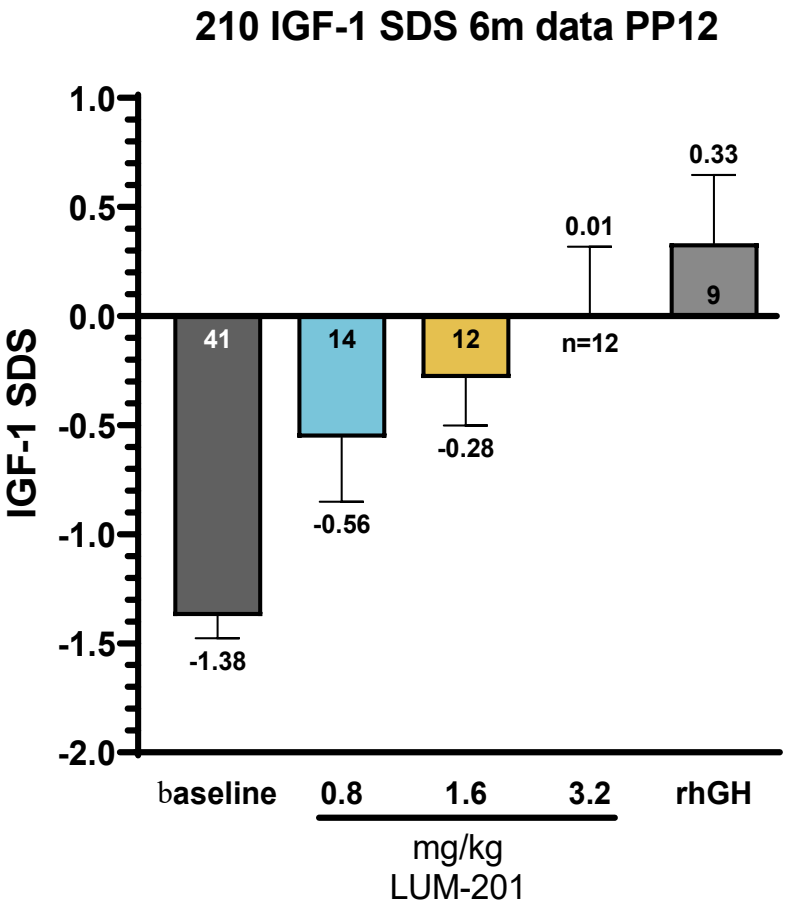


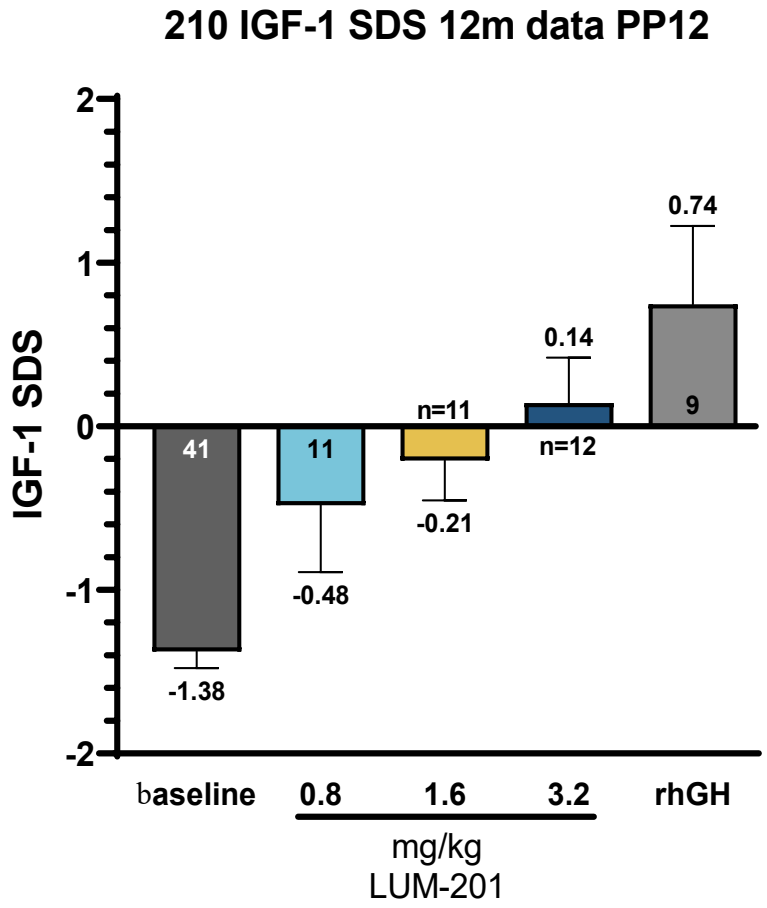
Highlights

- AHVs range from 8.3-9.3 cm/yr in datasets of moderate PGHD patients treated with daily rhGH
- LUM-201 AHVs in line with historical rhGH growth rates in comparable patient populations

†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
*Daily Genotropin control group for Somatrogon Ph3 dosed at 0.034 mg/kg/day (equates to 0.24 mg/kg/wk); subjects were stratified based on GH production during a standard stim test.

OraGrowtH210 Phase 2: IGF-1 Standard Deviation Score (SDS)
LUM-201 Normalizes IGF-1 SDS with Durable Effect out to 12 months





Highlights

- LUM-201 normalizes IGF-1 within 6 months
- Durable effect out to 12 months

Bars represent sample mean, and error bars represent Standard Error of the Mean

OraGrowtH210 Summary

- ✓ All primary and secondary endpoints met
- ✓ LUM-201 AHV's consistent with pre-specified targets from historical benchmarks in moderate PGHD population
- ✓ AHV delta for LUM-201 1.6 mg/kg from comparator daily rhGH arm at 6- and 12-months is within the non-inferiority margin (difference less than 1.8 to 2.0 cm) typically used in Phase 3 pivotal trials for rhGH approvals
- ✓ LUM-201 normalizes IGF-1 SDS within 6 months on treatment
- ✓ Investigational product safety profile remains clean after >1,300 patients treated to date[1]
- ✓ Phase 2 results support advancing to Phase 3 with final design to be confirmed following EOP2 FDA meeting, anticipated in 1H 2024

[1] Includes adult and pediatric subjects from prior Merck studies

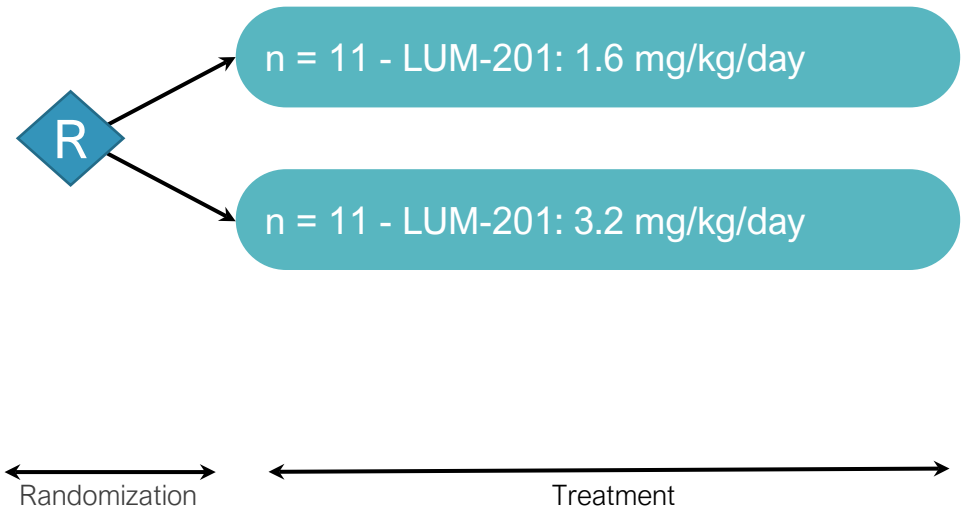
EOP2 = End of Phase 2

OraGrowtH212 Trial: PK/PD Trial in Naïve Moderate PGHD

OraGrowtH212 TRIAL

- n = 22
- Open-label study
- Moderate PGHD patients
- rhGH-treatment naïve
- Dosing to near-adult height
- Single, specialized clinical site in Santiago, Chile
- Q10 minute GH sampling for 12 hours

Primary Outcome Data (**n = 22**) – at **6 months** on therapy
Total Study Duration – Subjects on therapy to near adult height



Objectives

Study 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

OraGrowtH212 was a single-site trial with a more homogenous patient population than larger international OraGrowtH210 Trial

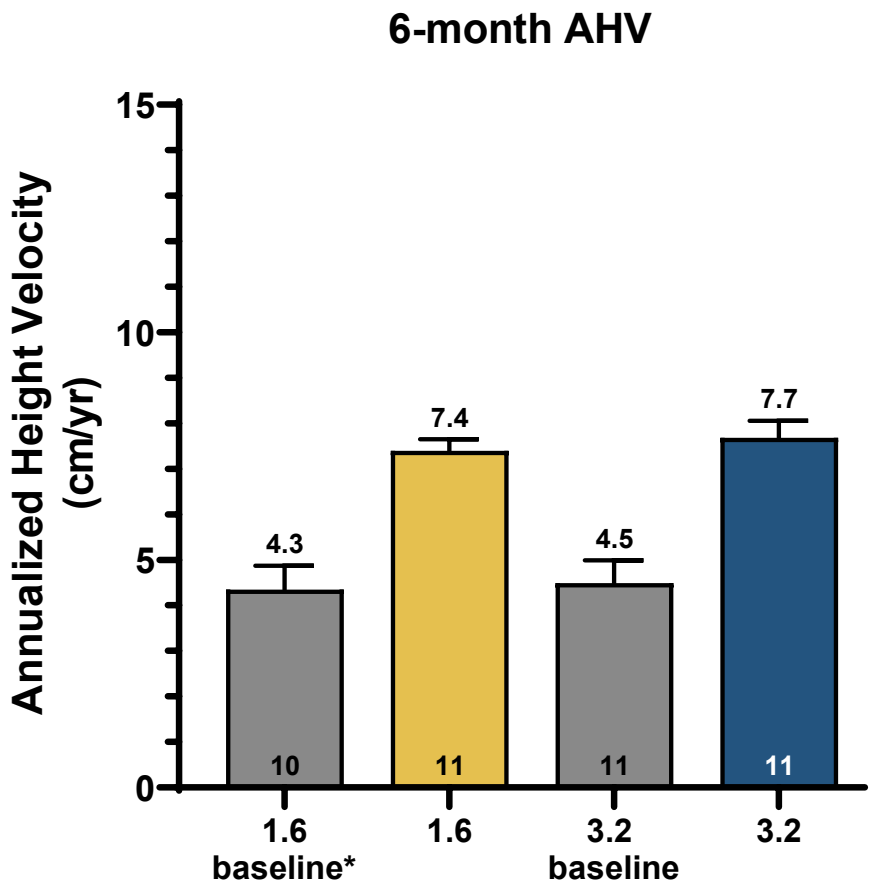
PK/PD = Pharmacokinetic / Pharmacodynamic

OraGrowtH212 Trial Baseline Demographics

	LUM-201 1.6 mg Mean (SD) **N=11**	**LUM-201 3.2 mg** Mean (SD) **N=11**
Age (months)	99.7 (15.2)	100.9 (21.1)
Height (cm)	116.5 (5.5)	116.6 (9.5)
Height SDS	-2.15 (0.28)	-2.26 (0.38)
IGF-1 SDS	-1.01 (0.64)	-0.85 (0.50)
MPH (cm)	162.6 (7.0)	160.3 (8.7)
MPH SDS Δ	-0.85 (0.53)	-0.73 (0.51)
BA Delay (yrs)	1.7 (0.86)	1.8 (0.96)
BMI SDS	-0.07 (0.85)	0.28 (0.97)

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

OraGrowtH212: Significant Increase in Growth from Baseline AHV at 6 Months



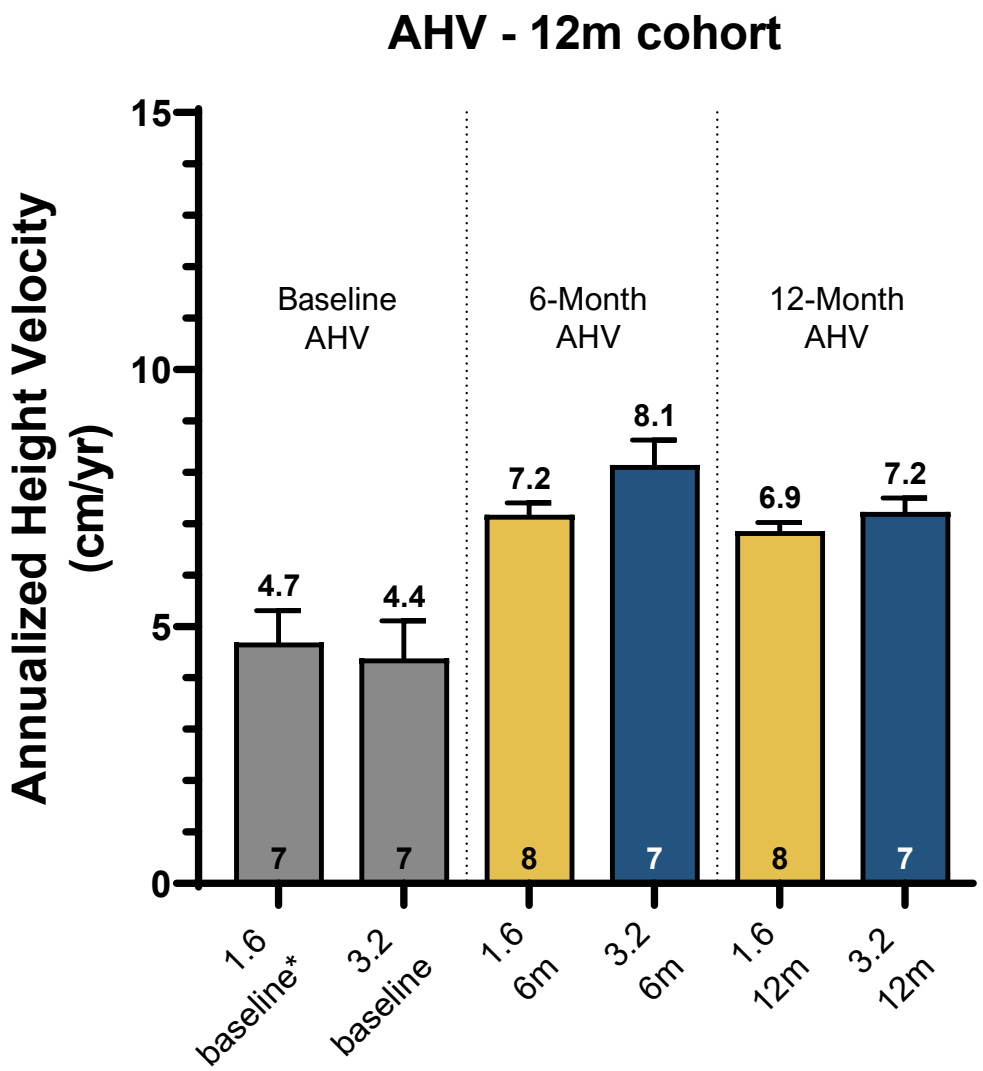
- AHV = Annualized Height Velocity
- Bars represent sample mean
- Error bars represent SEM

Highlights

- AHV at 6 months:
  - 7.4 in the 1.6 mg/kg arm
  - 7.7 in the 3.2 mg/kg arm
- OraGrowtH212 is a single-site study with a seemingly more homogeneous population than those enrolled in the global OraGrowtH210

AHV 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),
*Baseline AHV was not measured for one patient in the 1.6 mg/kg cohort.

OraGrowtH212: Significant Growth from Baseline Per Protocol 12-Month Population: 6 and 12-Month AHVs



- AHV = Annualized Height Velocity
- Bars represent sample mean
- Error bars represent SEM

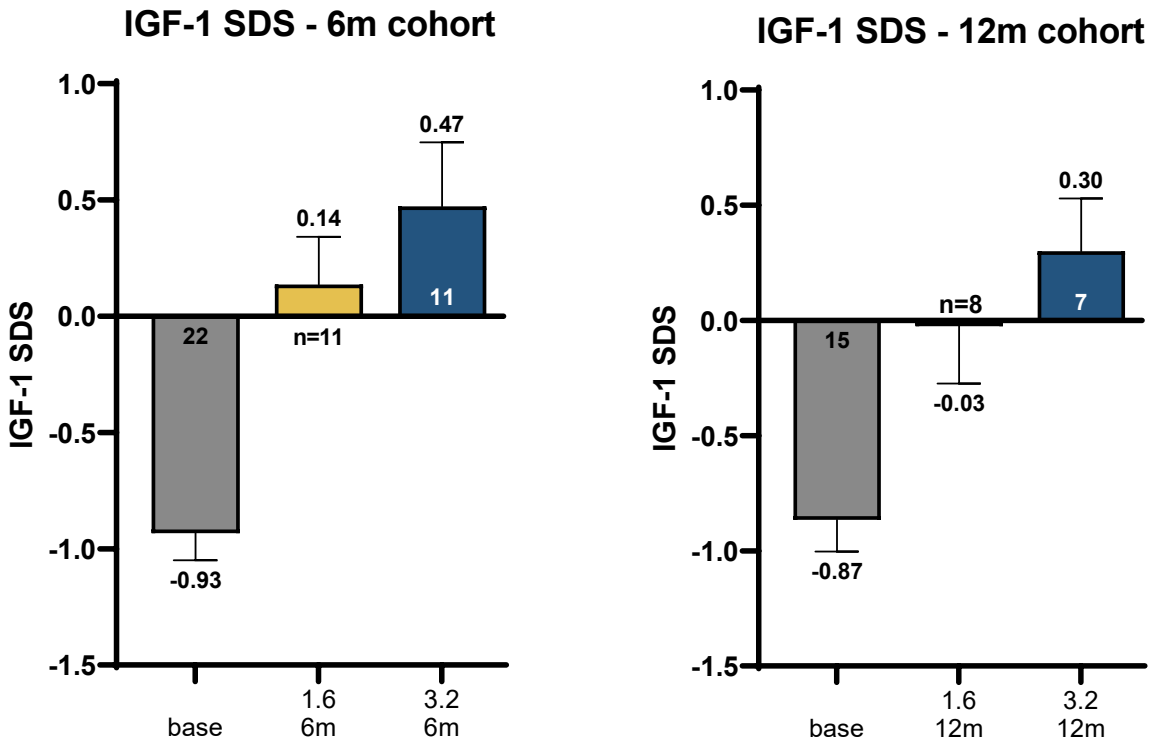
Highlights

- Significant increase in growth from baseline
- Durable effect to 12 months
- Minimal drop off in AHV between 6 and 12 months
- No material difference between 2 dose cohorts at 6 or 12 months
- AHV at 12 months:
  - 6.9 cm in the 1.6 mg/kg arm
  - 7.2 cm in the 3.2 mg/kg arm

AHV 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

*Baseline AHV was not measured for one patient in the 1.6 mg/kg cohort.

OraGrowtH212 Phase 2: IGF-1 SDS
LUM-201 Normalizes IGF-1 Level with Durable Effect out to 12 months



- Bars represent sample mean
- Error bars represent SEM
- Data represent number of patients for whom data was available at each timepoint; not all patients had reached 12 months on treatment at time of data pull.

Highlights

- LUM-201 normalizes IGF-1 within 6 months
- Durable effect on IGF-1 out to 12 months
- 0 Subjects > 2 SDS between 0 and 12 months

OraGrowtH212: LUM-201 Normalizes GH Concentrations in Moderate PGHD

Time period	Normal healthy (IC-GH‡)	Untreated GHD (IC-GH‡)	LUM-201 (baseline GH)*	LUM-201 (treat 6M GH)*
	Zadik†		N = 22	
12h (day) µg/kg.12hr	3.3 ± 1.3	1.1 ± 0.5	1.3	2.6
24h µg/kg/24hr	**5.0 ± 1.3**	1.4 ± 0.5	1.7	**3.3 – 4.0**
Ratio 24:12(day)	1.52	1.27	1.27	1.27-1.52

LUM-201 stimulates an increase in pulsatile secretion of GH approximating normal physiologic levels

‡ IC-GH: integrated concentration of Growth Hormone; data represent mean ± standard deviation

*GH concentrations from the combined 1.6 and 3.2 mg/kg/day cohorts

† Zadik et al Horm Res 1992

Notes for clarification of methods:

- Similar methodology used for the Zadik manuscript and the OraGrowtH212 Trial for calculation of the total AUC and derivation of secretion rate per kg body weight
- Assays for measurement of GH are different between the two studies

OraGrowtH212: LUM-201 Normalizes GH Concentrations in Moderate PGHD

Time period	Normal healthy (IC-GH‡)	Untreated GHD (IC-GH‡)	LUM-201 (baseline GH)*	LUM-201 (treat 6M GH)*	Comparator arm rhGH 34 µg/kg/day
	Zadik†		N = 22		Albertsson-Wikland††
12h (day) µg/kg.12hr	3.3 ± 1.3	1.1 ± 0.5	1.3	2.6	-
24h µg/kg/24hr	**5.0 ± 1.3**	1.4 ± 0.5	1.7	**3.3 – 4.0**	**~20 µg/kg/24hr††**
Ratio 24:12(day)	1.52	1.27	1.27	1.27-1.52	-

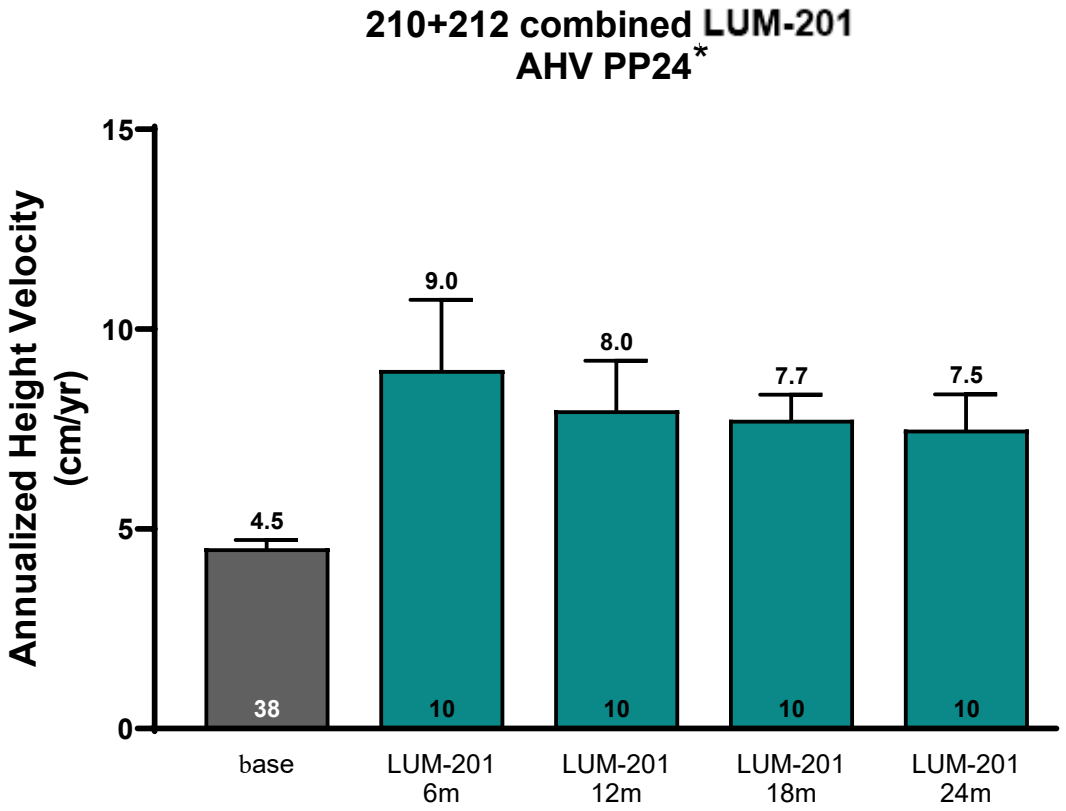
Increasing 24-hour pulsatile secretion, LUM-201 achieves comparable growth to exogenous injectable rhGH, with only 20% of GH concentration levels

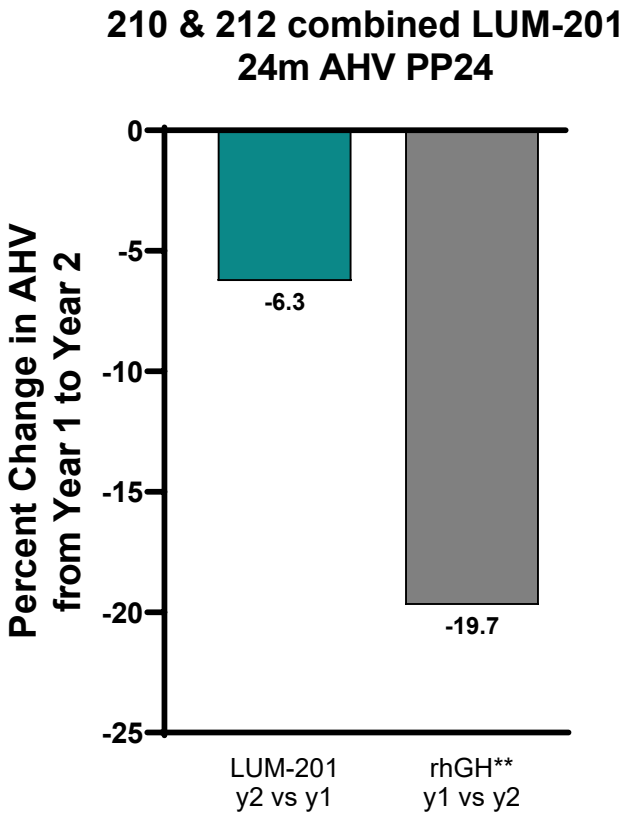
‡ IC-GH: integrated concentration of Growth Hormone; data represent mean ± standard deviation
*GH concentrations from the combined 1.6 and 3.2 mg/kg/day cohorts
† Zadik et al Horm Res 1992
†† Adapted from data in Albertsson-Wikland et al JCEM 1994; 24h exposures listed reflect absorbance/bioavailability of ~60% of the administered dose

OraGrowtH212 Summary

- ✓ All primary and secondary endpoints met
- ✓ Increased 6- and 12-month AHV meaningfully from baseline
- ✓ LUM-201 normalized IGF-1 SDS values within 6 months of treatment with durable effect
- ✓ LUM-201 stimulates an increase in pulsatile secretion of GH approximating normal physiologic levels
- ✓ Increasing 24-hour pulsatile secretion, LUM-201 achieves comparable growth to daily exogenous injectable rhGH, with only 20% of GH concentration levels

LUM-201 Data Suggests Greater Durability of Response than rhGH to 24 Months OraGrowtH210 & OraGrowtH212 Combined (1.6 and 3.2 mg/kg LUM-201)





Highlights

- Preliminary data demonstrate LUM-201 AHV durable to 24 months
- More moderate year 2 AHV decline than rhGH likely due to LUM-201 restoration of GH and IGF-1 to normal levels via pulsatile secretion

AHV values from the OraGrowth studies are based on ANCOVA model (details provided on previous slides)
* At 24 months, data include a subset of subjects from OraGrowtH210 trial who met protocol criteria to continue past 12 months.
** Ranke et.al. 2010 – rhGH treated cohort of moderate GHD children; mean AHV for the moderate GHD cohorts were 8.58 cm/yr in year 1 and 6.89 cm/yr in year 2.

Safety Data from Combined Trials

	PEM	0.8 mg/kg	1.6 mg/kg	3.2 mg/kg	rhGH
	N =129	N =18	N =33	N=33	N =20
Number of AEs	38	59	155	150	54
Subjects with AE (%)	24 (18.6%)	14 (77.8%)	31 (93.9%)	30 (90.9%)	16 (80.0%)
Treatment Related AEs *	7	2	17	20	6
Subjects with Treatment Related AEs (%)	4 (3.1%)	1 (5.6%)	13 (39.4%)	13 (39.4%)	5 (25.0%)
Subjects with SAEs (%)	0 (0%)	#2 (11.1%)	1 (3.0%)	0 (0%)	##1 (5.0%)
Subject with Treatment Related SAEs (%)	0 (0%)	0 (0%)	0 (0%)	0 (0%)	0 (0.0%)

Topline Safety Results

- No meaningful 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 to date
- * Treatment related AEs in 1.6 and 3.2 groups: Increased appetite (23), Pain in extremity (7), Arthralgia (5), Abdominal pain (1), Transaminases Increased (1)

One subject had SAE between PEM dose and randomized dose

Subject had SAE between PEM dose and randomized dose

Lumos Pharma Financial Information as of September 30, 2023

Values in USD

Cash, equivalents & short-term investments	$42.7M
Debt	$0
Shares Outstanding	7.9M
Cash Use for 4Q 2023	~ $9.0-$10.0M
Fiscal Year End	December 31

Cash, cash equivalents, & short-term investments to support operations through 3Q 2024, inclusive of activities related to advancing the PGHD program into Phase 3

Recap Summary and Next Steps

OraGrowtH210 and OraGrowtH212 Phase 2 Clinical Trials

- Met all primary and secondary endpoints
- LUM-201 increases pulsatility, restores GH secretion and normalizes IGF-1
- LUM-201 promotes growth comparable to rhGH with only 20% of GH concentration levels
- Optimal 6-month LUM-201 dose vs rhGH AHV delta (1.8 cm) within historical Phase 3 non-inferiority margins
- Optimal 12-month LUM-201 dose vs historical rhGH AHV delta (~1.3 cm) within historical Phase 3 non-inferiority margins

Considerations for Phase 3 in PGHD

- Plan to request End-of-Phase 2 meeting with FDA and conduct in 1H 2024
- Anticipate initiating Phase 3 program in 2H 2024

AHV = Least Squares Mean Annualized Height Velocity, AHV values from the OraGrowth studies are based on ANCOVA model (details provided on previous slides)

Supplementary Materials

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
N =	**18**	**22**	**22**	**62**	**20**
Number of AEs	59	79	74	212	54
Subjects with AE (%)	14 (77.8%)	20 (90.9%)	19 (86.4%)	53 (85.5%)	16 (80.0%)
Treatment Related AEs (N)	2	2	4	8	6
Subjects with Treatment Related AEs (%)	1 (5.6%)	2 (9.1%)	3 (13.6%)	6 (9.7%)	5 (25.0%)
Subjects with SAEs (%)	#2 (11.1%)	1 (4.5%)	0 (0.0%)	2 (3.2%)	##1 (5.0%)
Subjects with Treatment Related SAEs (%)	0 (0.0%)	0 (0.0%)	0 (0.0%)	0 (0.0%)	0 (0.0%)

One subject had SAE between PEM dose and randomized dose

Subject had SAE between PEM dose and randomized dose

Related 210 AEs –

Preferred Term, N (%)	0.8 N=18	1.6 N=22	3.2 N=22	ALL N= 62	rhGH N=20	Comments		
Contusion	--	--	--	--	1 (5.0)	Grade 1, Recovered by next visit		
Injection Site Bruising	--	--	--	--	2 (10.0)	Grade 1, Recovered by next visit		
Increased Appetite (*All Grade 1*)	1 (5.6)	1 (4.5)	1 (4.5)	3 (4.8)	2 (10.0)	Duration:	0.8	Ongoing
							1.6	1 & 7 months
							3.2	Ongoing
							rhGH	9, 13 & 15 months
Arthralgia	--	1 (4.5)	1 (4.5)	2 (3.2)	--	Both Grade 1, Duration was a few days		
Growing Pains	1 (5.6)	--	--	1 (1.6)	--	Grade 1		
Pain in Extremity	--	--	2 (9.1)	2 (3.2)	1 (5.0)	All Grade 1, Intermittent or short duration		

Serious Adverse Events 210 Trial

Serious Adverse Event	System Organ Class	Gr	Study Treatment	Relatedness	Serious Criteria
Product Administration Error	Injury, Poisoning and Procedural Complications	1	NA *(occurred prior to receiving any study drug)*	**Unrelated**	Hosp
Dehydration	Metabolism and Nutrition Disorders	3	*PEM (single 0.8 mg/kg)	**Unrelated**	Hosp
Glycosuria	Renal and Urinary Disorders	1	**PEM (single 0.8 mg/kg)	**Unrelated**	Hosp
Cartilage Development Disorder	Musculoskeletal and Connective Tissue Disorders	3	0.8 mg/kg/day	**Unrelated**	Hosp
Pain in Extremity	Musculoskeletal and Connective Tissue Disorders	2	1.6 mg/kg/day	**Unrelated**	Hosp

* This subject was later randomized to the 0.8mg/kg study arm
** This subject was later randomized to the rhGH arm

There have been no SAEs in the 212 trial to date

Related 212 AEs –

Preferred Term, N (%)	1.6 N=11	3.2 N=11	ALL N=22	Comments	
Abdominal Pain	1 (9.1)	--	1 (4.5)	Grade 1, Duration: few days	
Transaminases Increased	--	1 (9.1)	1 (4.5)	Grade 1, Duration: <3 months	
Increased Appetite	11 (100.0)	10 (90.9)	21 (95.5)	19 Grade 1	9 ongoing
					10 resolved (duration 1-23, avg 9.7 months)
				2 Grade 2, both ongoing	
Arthralgia	1 (9.1)	2 (18.2)	3 (13.6)	All Grade 1, Duration: < 2 weeks	
Pain in Extremity	2 (18.2)	3 (27.3)	5 (22.7)	All Grade 1, All with duration: < 2 weeks, except one with ongoing intermittent leg pain	

Specific OraGrowtH210 AEs – No meaningful signal

safety data available for 82 subjects at interim analysis

	0.8 N=18	1.6 N=22	3.2 N=22	ALL N=62	rhGH N=20
Arthralgia	2 (11.1%)	3 (13.6%)	2 (9.1%)	7 (11.3%)	2 (10.0%)
Myalgia	0 (0.0%)	0 (0.0%)	0 (0.0%)	0 (0.0%)	3 (15.0%)
Headache	5 (27.8%)	7 (31.8%)	5 (22.7%)	17 (27.4%)	3 (15.0%)
Lethargy	0 (0.0%)	0 (0.0%)	0 (0.0%)	0 (0.0%)	0 (0.0%)
Abd. pain	1 (5.6%)	3 (13.6%)	5 (22.7%)	9 (14.5%)	1 (5.0%)
Emesis	0 (0.0%)	1 (4.5%)	3 (13.6%)	4 (6.5%)	3 (15.0%)
Inc. appetite	1 (5.6%)	1 (4.5%)	1 (4.5%)	3 (4.8%)	2 (10.0%)
Hypoglycemia	0 (0.0%)	0 (0.0%)	0 (0.0%)	0 (0.0%)	0 (0.0%)
Orophary. pain	2 (11.1%)	2 (9.1%)	0 (0.0%)	4 (6.5%)	1 (5.0%)

Laboratory Shifts: No meaningful signal

82 subjects

	0.8 mg/kg N=18	1.6 mg/kg N=22	3.2 mg/kg N=22	ALL N=62	rhGH N=20
ALT NI to high	2/17 (11.8%)	5/22 (22.7%)	4/22 (18.2%)	11/61 (18%)	7/20 (35%)
AST NI to high	3/14 (21.4%)	4/21 (19%)	5/22 (22.7%)	12/57 (21.1%)	6/20 (30%)
Bicarb NI to high	0/18 (0%)	0/22 (0.0%)	1/22 (4.5%)	1/62 (1.6%)	0/20 (0%)
Bicarb NI to low	8/18 (44.4%)	6/22 (27.3%)	8/22 (36.4%)	22/62 (35.5%)	5/20 (25%)
Bilirubin NI to high*	4/18 (22.2%)	4/22 (18.2%)	4/22 (18.2%)	12/62 (19.4%)	2/20 (10%)
Calcium NI to low	1/18 (5.6%)	2/21 (9.5%)	4/22 (18.2%)	7/61 (11.5%)	2/20 (10%)
Calcium NI to high	0/18 (0%)	2/22 (9.1%)	0/22 (0.0%)	2/61 (3.3%)	0/20 (0%)
Creatinine NI to low	2/18 (11.1%)	3/22 (13.6%)	2/22 (9.1%)	7/62 (11.3%)	2/20 (10%)
GGT NI to high	2/17 (11.8%)	6/22 (27.3%)	8/22 (36.4%)	16/61 (26.2%)	1/20 (5%)

For the shift to study visit, the denominator is the number of subjects with a non-missing value for the given parameter at baseline and the visit.
Baseline is defined as the latest results obtained prior to the first dose of study drug.
* Bilirubin Q2 laboratory normal range high values are lower than most laboratories

Laboratory Shifts

	0.8 mg/kg N=18	1.6 mg/kg N=22	3.2 mg/kg N=22	ALL N=62	rhGH N=20
Urea nitro Nl to low	4/18 (22.2%)	4/21 (19%)	7/22 (31.8%)	15/61 (24.6%)	7/20 (35%)
Urea nitro Nl to high	1/18 (5.6%)	0/22 (0%)	1/22 (4.5%)	2/62 (3.2%)	0/20 (0%)
Basophils Nl to high	7/17 (41.2%)	12/22 (54.5%)	10/21 (47.6%)	29/60 (48.3%)	4/20 (20%)
Eosinophils Nl to high	2/17 (11.8%)	4/22 (18.2%)	3/21 (14.3%)	9/60 (15%)	5/20 (25%)
Hematocrit Nl to low	2/18 (11.1%)	0/22 (0.0%)	2/22 (9.1%)	4/61 (6.6%)	0/20 (0%)
Hematocrit Nl to high	1/17 (5.9%)	1/22 (4.5%)	2/22 (9.1%)	4/61(6.6%)	0/20 (0%)
Hemoglob. Nl to low	4/18 (22.2%)	2/22 (9.1%)	5/22 (22.7%)	11/62 (17.7%)	0/20 (0%)
Lymphoc. Nl to low	3/17 (17.6%)	0/21 (0.0%)	1/21 (4.8%)	4/59 (6.8 %)	1/20 (5%)
Lymphoc. Nl to high	0/17 (0.0%)	0/22 (0.0%)	2/21 (9.5%)	2/60 (3.3%)	0/20 (0%)

Laboratory Shifts

	0.8 mg/kg N=18	1.6 mg/kg N=22	3.2 mg/kg N=22	ALL N=62	rhGH N=20
Globulin NI to low	6/18 (33.3%)	4/22 (18.2%)	4/22 (18.2%)	14/62 (22.6%)	5/20 (25%)
Glucose NI to high	0/18 (0%)	5/22 (22.7%)	6/22 (27.3%)	11/61 (18%)	0/20 (0%)
Glucose NI to low	0/18 (0%)	0/22 (0.0%)	1/22 (4.5%)	1/62 (1.6%)	0/20 (0%)
Insulin NI to low	2/17 (11.8%)	2/20 (10%)	1/21 (4.8%)	5/58 (8.6%)	0/20 (0%)
Phosphate NI to low	0/18 (0%)	0/22 (0.0%)	1/22 (4.5%)	1/61 (1.6%)	1/20 (5%)
Phosphate NI to high	6/17 (35.3%)	4/22 (18.2%)	7/22 (31.8%)	17/61 (27.9%)	7/20 (35%)
Protein NI to high	0/18 (0%)	1/22 (4.5%)	5/22 (22.7%)	6/62 (9.7%)	1/20 (5%)
Protein NI to low	0/18 (0%)	2/22 (9.1%)	2/22 (9.1%)	4/62 (6.5%)	3/20 (15%)
Potassium NI to high	4/16 (25%)	9/22 (40.9%)	7/22 (31.8%)	20/60 (33.3%)	1/20 (5%)

Laboratory Shifts

	0.8 mg/kg N=18	1.6 mg/kg N=22	3.2 mg/kg N=22	ALL N=62	rhGH N=20
Ery. crp. Hb Nl to low	2/17 (11.8%)	2/22 (9.1%)	3/22 (13.6%)	7/61 (11.5%)	2/20 (10%)
Ery. crp. vol Nl to low	1/18 (5.6%)	3/21 (14.3%)	3/22 (13.6%)	7/61 (11.5%)	1/20 (5%)
Ery. crp vol Nl to high	0/17 (0.0%)	0/22 (0.0%)	0/22 (0.0%)	0/61 (0%)	0/20 (0%)
Monocytes Nl to low	3/17 (17.6%)	3/21 (14.3%)	1/21(4.8%)	7/59(11.9%)	1/20(5%)
Monocytes Nl to high	3/17 (17.6%)	3/22 (13.6%)	4/21 (19%)	10/60(16.7%)	0/20 (0%)
Neutroph. Nl to high	0/18 (0%)	2/22 (9.1%)	2/21 (9.5%)	4/60 (6.7%)	1/20 (5%)
Neutroph. Nl to low	3/17 (17.6%)	4/21 (19%)	6/21 (28.6%)	13/59 (22%)	3/20 (15%)
Platelets Nl to low	0/18 (0.0%)	0/22 (0%)	1/22 (4.5%)	1/62 (1.6%)	0/20 (0%)
Platelets Nl to high	6/17 (35.3%)	5/22 (22.7%)	6/22 (27.3%)	17/61 (27.9%)	0/20 (0%)

Laboratory Shifts: No meaningful signal

	0.8 mg/kg N=18	1.6 mg/kg N=22	3.2 mg/kg N=22	ALL N=62	rhGH N=20
Eryth. Nl to high	1/17 (5.9%)	2/22 (9.1%)	2/22 (9.1%)	5/61 (8.2%)	1/20 (5%)
Eryth. Nl to low	1/18 (5.6%)	0/22 (0.0%)	0/22 (0.0%)	1/62 (1.6%)	0/20 (0%)
Leukocyt. Nl to high	1/17 (5.9%)	2/22 (9.1 %)	2/22 (9.1%)	5/61 (8.2%)	1/20 (5%)
Leukocyt. Nl to low	4/17 (23.5%)	4/21 (19%)	2/22 (9.1%)	10/60 (16.7%)	2/20 (10%)