



# Interim Analysis OraGrowthH210 & OraGrowthH212

November 14, 2022



# Forward Looking Statements

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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, 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 trails 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, 2021, 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.

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# Investment Highlights

## *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
- **Potential to disrupt** significant subset of sizable **injectable market** for GHD



### Pipeline in a Product

- Worldwide injectable market for GHD disorders is **\$3.4 billion\***
- Market for initial oral LUM-201 indication, Pediatric GHD (PGHD), is **\$1.2 billion\***
- Prior data support potential efficacy of LUM-201 in multiple GHD disorders



### Late-stage Trials in PGHD

- Phase 2 OraGrowthH210 Trial & PK/PD OraGrowthH212 Trials ongoing
- Interim data obtained | Primary outcome data expected 2H 2023
- **Approximately 80% enrolled in Phase 2 OraGrowthH210 Trial**



### Solid Financial Position

- Cash balance of **\$73.7 million** as of close of **3Q 2022**
- Cash runway **into 2Q 2024**, beyond OraGrowthH210 & OraGrowthH212 primary outcome data



PGHD = Pediatric Growth Hormone Deficiency

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

# 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 conversation to oral therapy

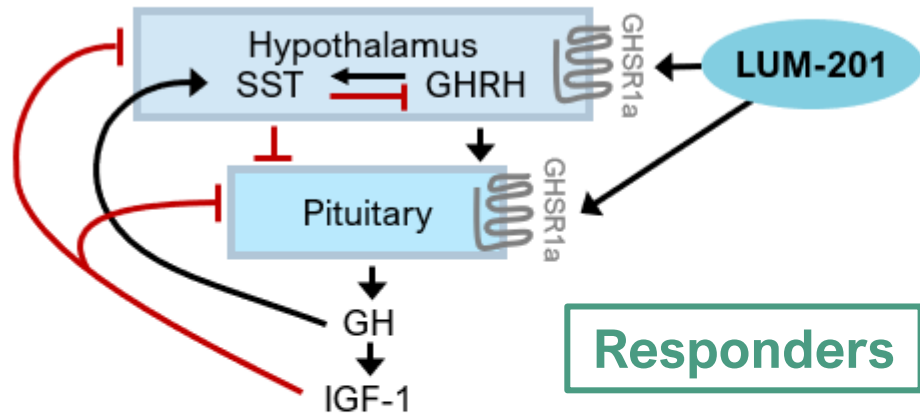
# OraGrowthH210 Trial

Phase 2 Trial Evaluating Oral LUM-201 in Moderate PGHD

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

**~ PEMs = Predictive Enrichment Markers ~  
A single dose of LUM-201 can identify likely responders**

## Moderate (PEM+): Included in Clinical Trials

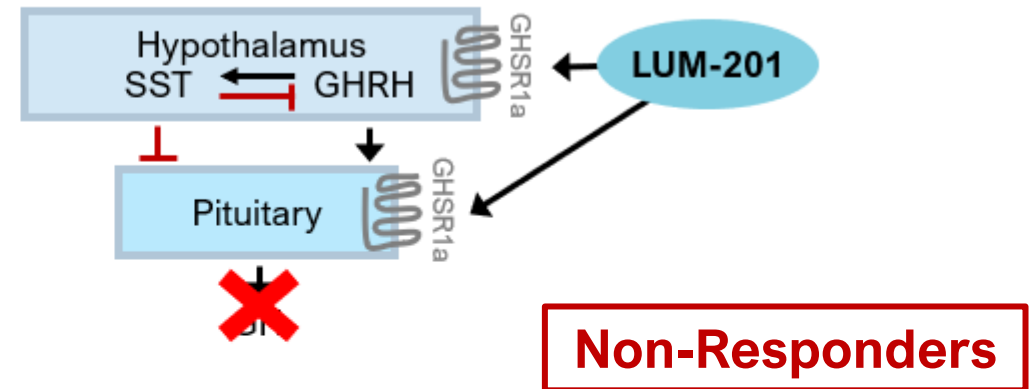


### Moderate (PEM+) = Idiopathic PGHD

#### Functional but reduced HP-GH axis

- Able to secrete some, but insufficient, GH
- Expected to respond to LUM-201<sup>1</sup>
- Represents ~60% of PGHD patients<sup>2</sup>

## Severe (PEM-): Excluded from Clinical Trials



### Severe (PEM-) = Organic PGHD

#### Non-functional HP-GH axis

- Unable to secrete GH
- Not expected to respond to LUM-201
- Represents ~40% of PGHD patients<sup>2</sup>

(PEM+) PEM-positive = PGHD patients with baseline IGF-1 > 30 ng/ml & stimulated GH ≥ 5 ng/ml  
HP-GH axis – hypothalamic pituitary growth hormone axis

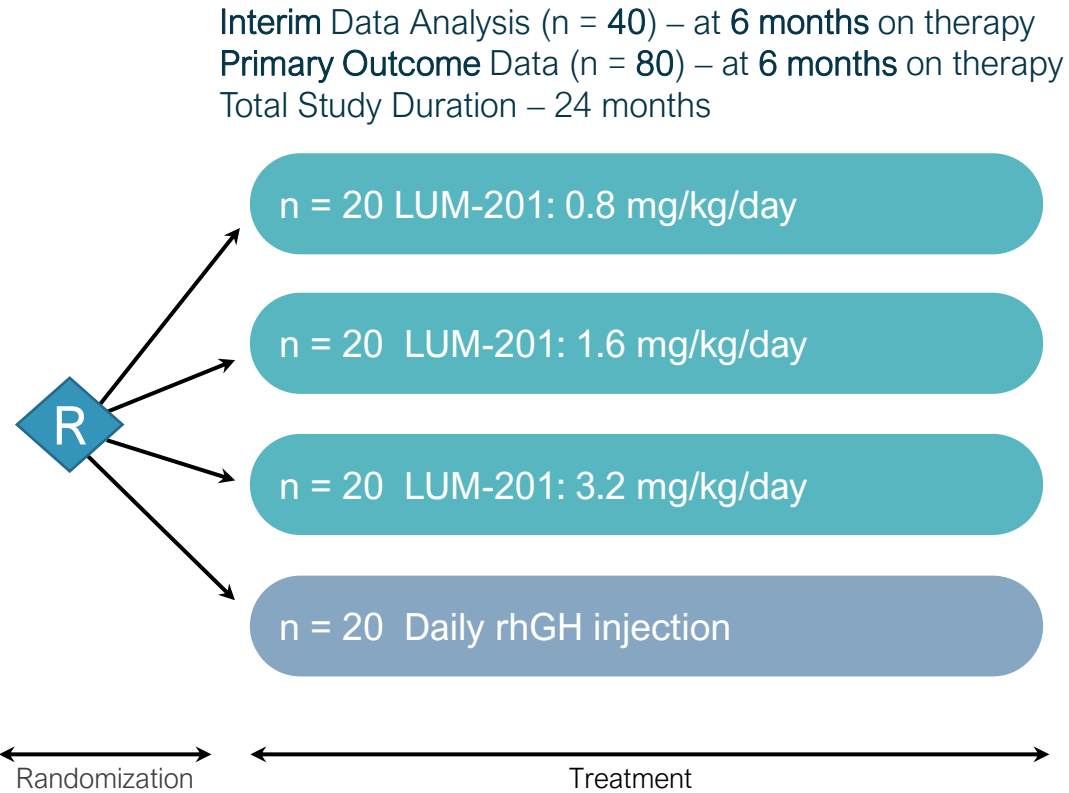
<sup>1</sup> Bright 2021 JES

<sup>2</sup> Blum 2021 JES

# OraGrowthH210 Trial: Phase 2 Trial in PGHD

## OraGrowthH210 TRIAL

- n = 80
- PEM(+) PGHD subjects
- Inclusion: stim GH  $\geq$  5 ng/ml and baseline IGF-1  $>$ 30 ng/ml
- rhGH treatment naïve
- ~40 trial sites US & International
- Trial opened Q4 2020



## Objectives

### Primary Endpoint:

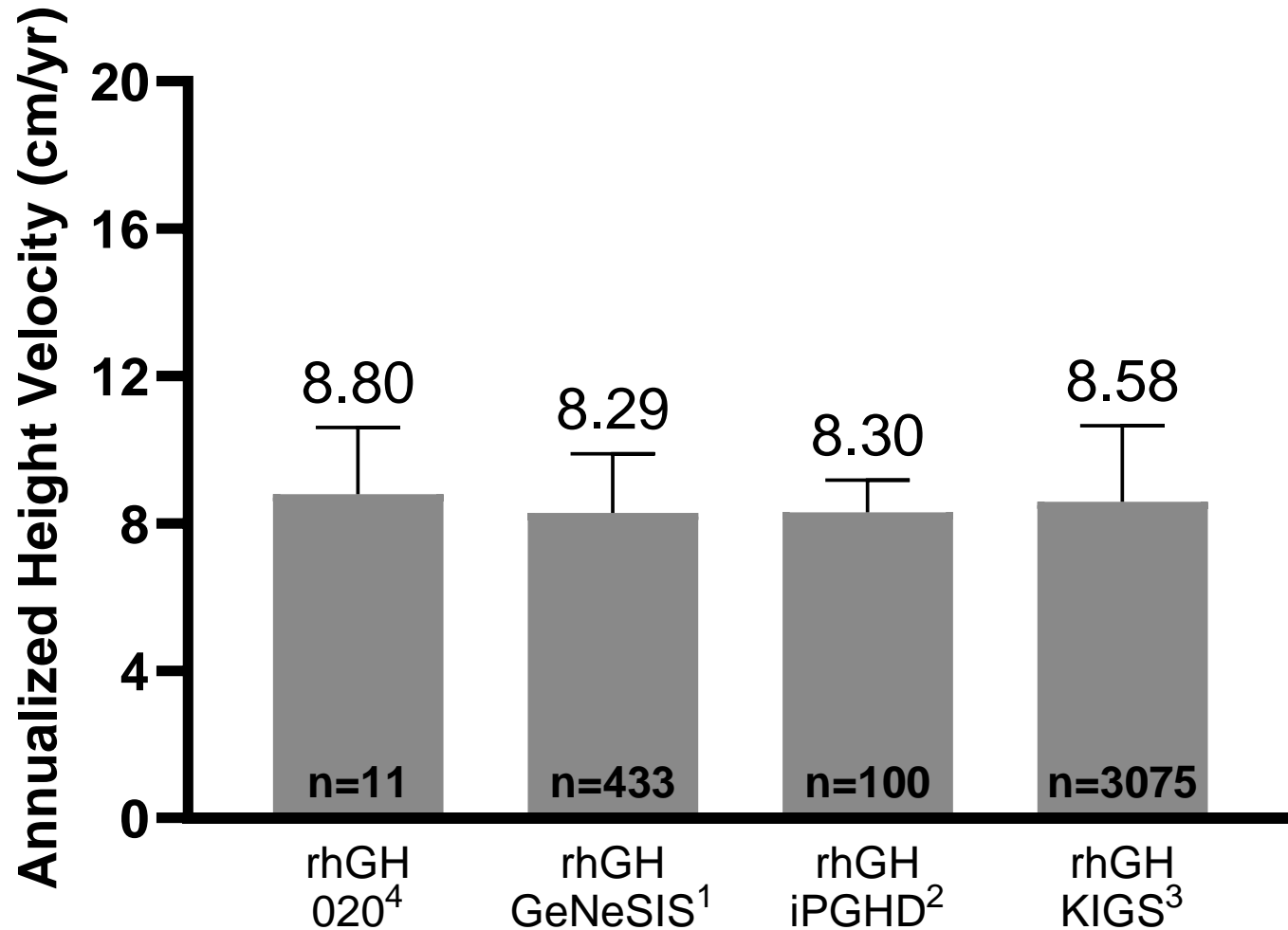
- Annualized Height Velocity (AHV)

### Goals:

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

**Interim AHV and safety data on 40 subjects at 6 months on therapy announced Nov 14, 2022**  
**Primary outcome data for OraGrowthH210 Trial on 80 subjects anticipated 2H2023**

# Historical Data for rhGH Growth Rates in Moderate PGHD Patients



## Historical Datasets

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

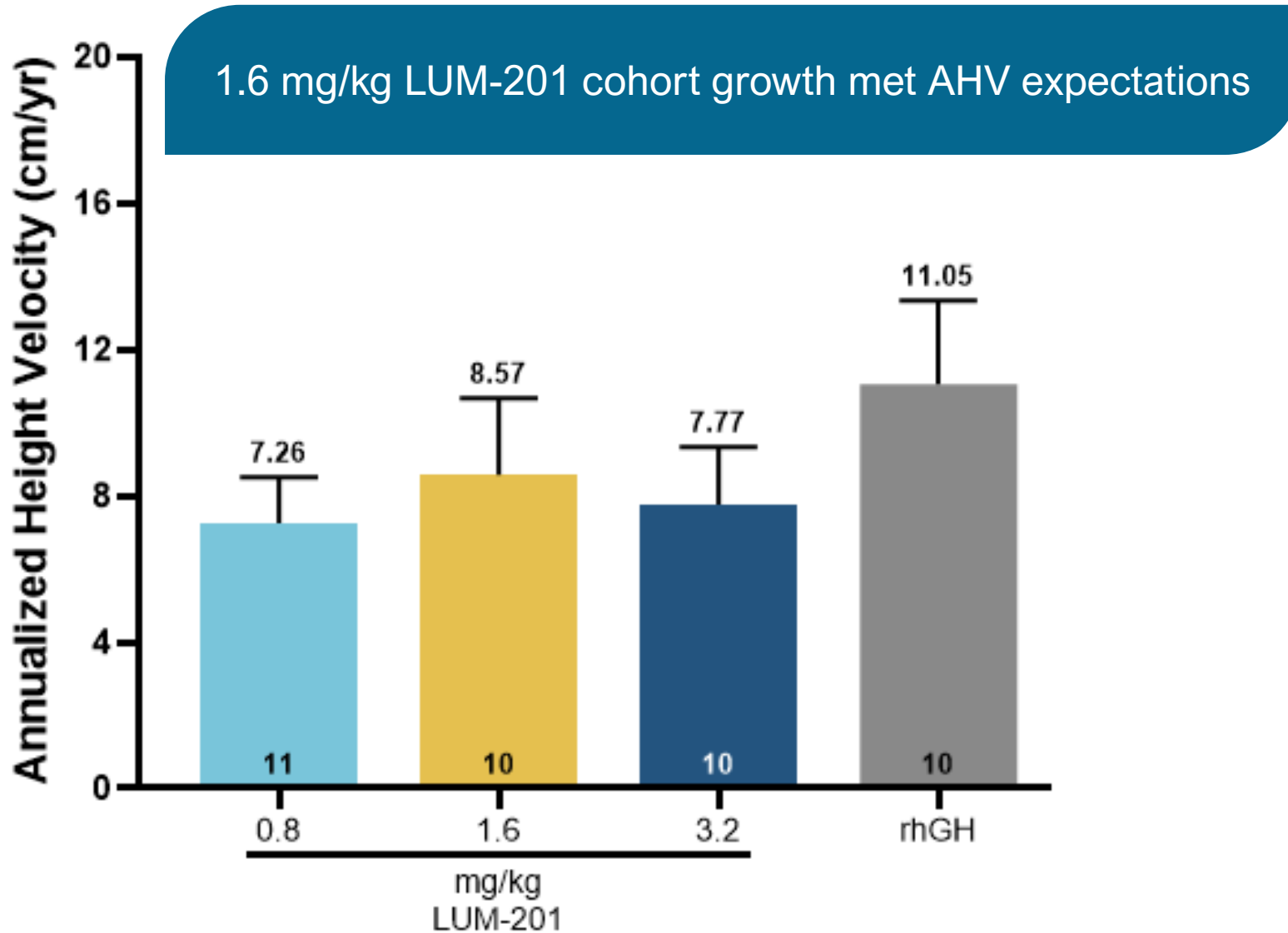
## Prediction

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



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



## Results

- LUM-201 1.6 mg/kg/day cohort grew 8.6 cm/yr, in line with the expected rate of 8.3 cm/yr based on prior data
- rhGH cohort grew at a much faster rate than expected or previously reported in moderate PGHD population
- Cohort baseline differences predict faster first-year growth in the rhGH arm<sup>1,2</sup>
- The balance between cohorts should continue to improve with further enrollment

# OraGrowthH210 Trial Baseline Characteristics – at Interim Data (N=41)

*Imbalance in baseline characteristics between rhGH and LUM-201 arms*

	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 <math>\Delta</math></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)

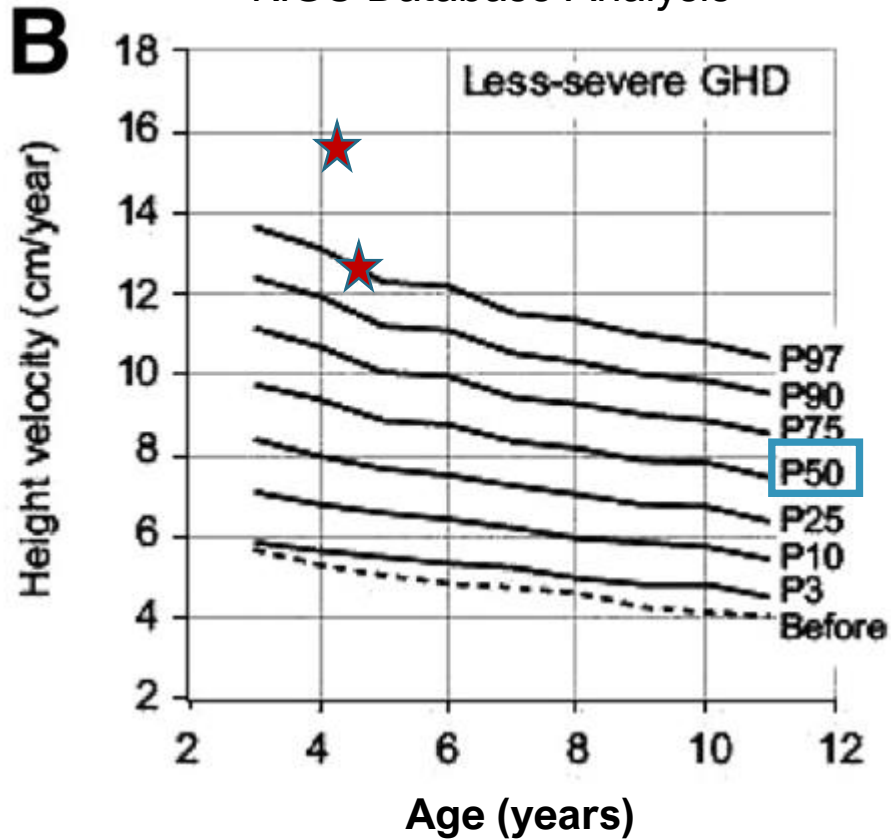
Baseline characteristics for the rhGH arm predict this cohort will show a faster first-year growth rate on treatment than the LUM-201 cohorts <sup>2,3</sup>

<sup>1</sup> Yang, et al. Nature Sci Rep 2019, 9(1); 16181 <sup>2</sup> Blum et al JES 2021, <sup>3</sup> Ranke et al JCEM 2010

KEY: 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: 2/3 Subjects under 5 Randomized to rhGH

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



★ OraGrowth210 youngest subjects in rhGH cohort at 6-months AHV

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

<sup>1</sup> Ranke, et al 2010 JCEM

# OraGrowthH210 Trial Baseline Characteristics – at ~75% Enrollment

*Balance improves at ~75% enrollment*

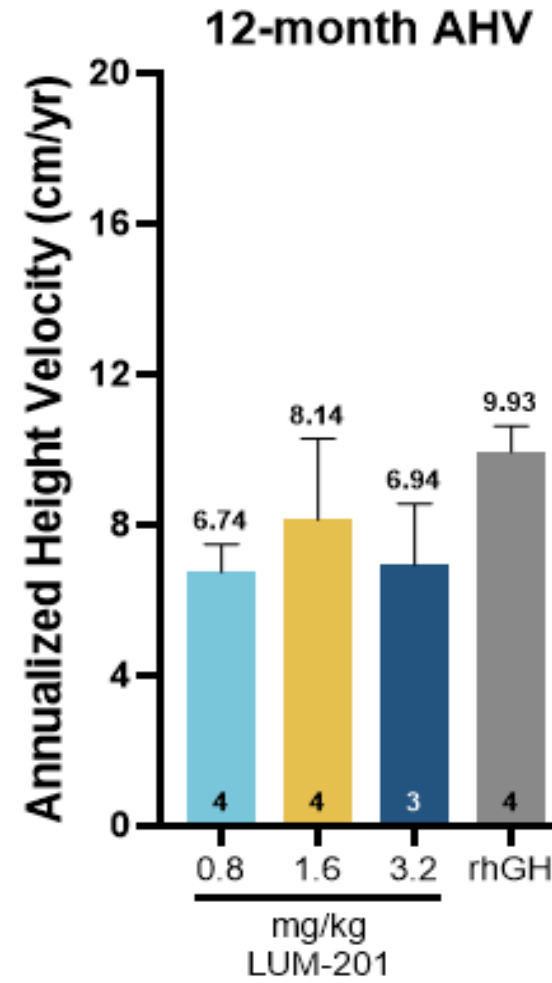
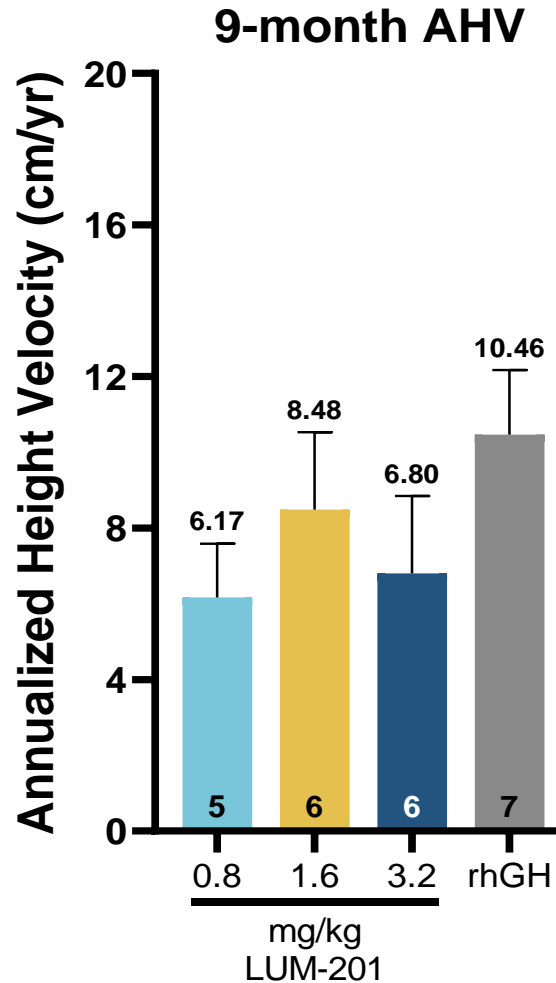
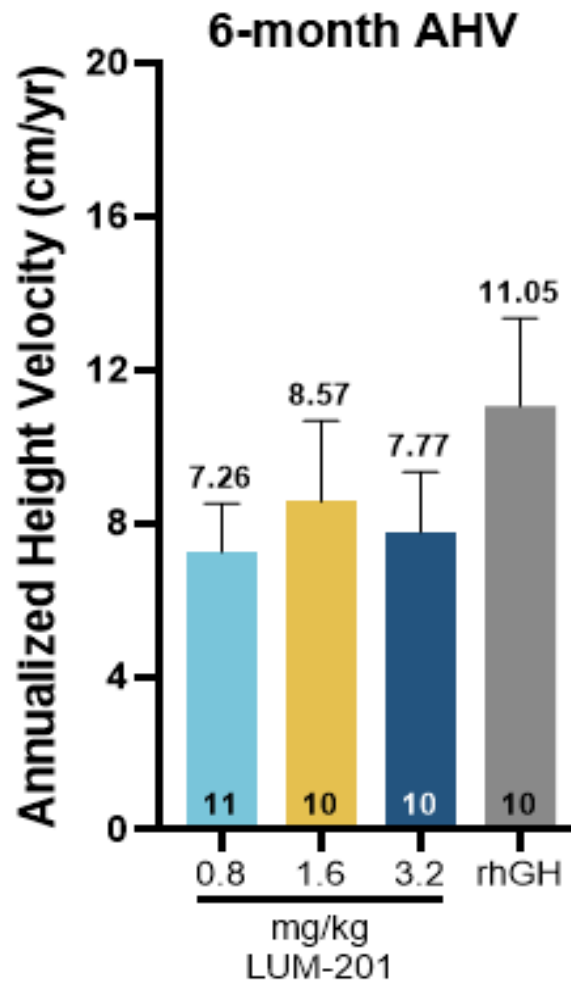
	LUM-201 0.8 mg Mean (SD) N=14	LUM-201 1.6 mg Mean (SD) N=15	LUM-201 3.2 mg Mean (SD) N=14	rhGH Mean (SD) N=15
<b>Age (months)</b>	99.1 (28.3)	98.4 (28.6)	92.9 (22.6)	94.1 (23.7)
<b>Height (cm)</b>	115.1 (12.5)	114.6 (11.2)	112.4 (9.2)	113.4 (10.6)
<b>Height SDS</b>	-2.32 (0.3)	-2.31 (0.5)	-2.32 (0.4)	-2.25 (0.4)
<b>Max Height SDS</b>	-1.76	-1.66	-1.57	-1.73
<b>IGF-1 SDS</b>	-1.43 (0.67)	-1.30 (0.67)	-1.35 (0.57)	-1.32 (0.46)
<b>Max IGF-1 SDS</b>	-0.3	-0.3	-0.6	-0.7
<b>MPH (cm)</b>	165.5 (7.1)	164.3 (7.2)	166.1 (7.0)	168.5 (7.9)
<b>MPH SDS <math>\Delta</math></b>	1.43 (0.66)	1.70 (0.54)	1.92 (0.73)	1.75 (0.63)
<b>BA Delay (yrs)</b>	1.89 (1.02)	1.91 (0.53)	2.20 (0.86)	1.68 (0.9)
<b>BMI SDS<sup>1</sup></b>	-0.47 (1.09)	-0.38 (0.91)	-0.55 (0.79)	+0.14 (1.08)

At ~75% enrollment balance between arms is very good, effect of outliers should be diminished

<sup>1</sup> Yang, et al. Nature Sci Rep 2019, 9(1); 16181 <sup>2</sup> Blum et al JES 2021, <sup>3</sup> Ranke et al JCEM 2010

KEY: 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

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



## Conclusions

- LUM-201 growth rates consistent from 6 to 12 months
- rhGH growth rates decline more from 6 to 12 months, narrowing the AHV  $\Delta$  between the arms at 12 months
- A Phase 3 non-inferiority trial is expected to be a 12-month study in a much larger population
- Historically, non-inferiority margin for AHV's in Phase 3 trials was ~2 cm

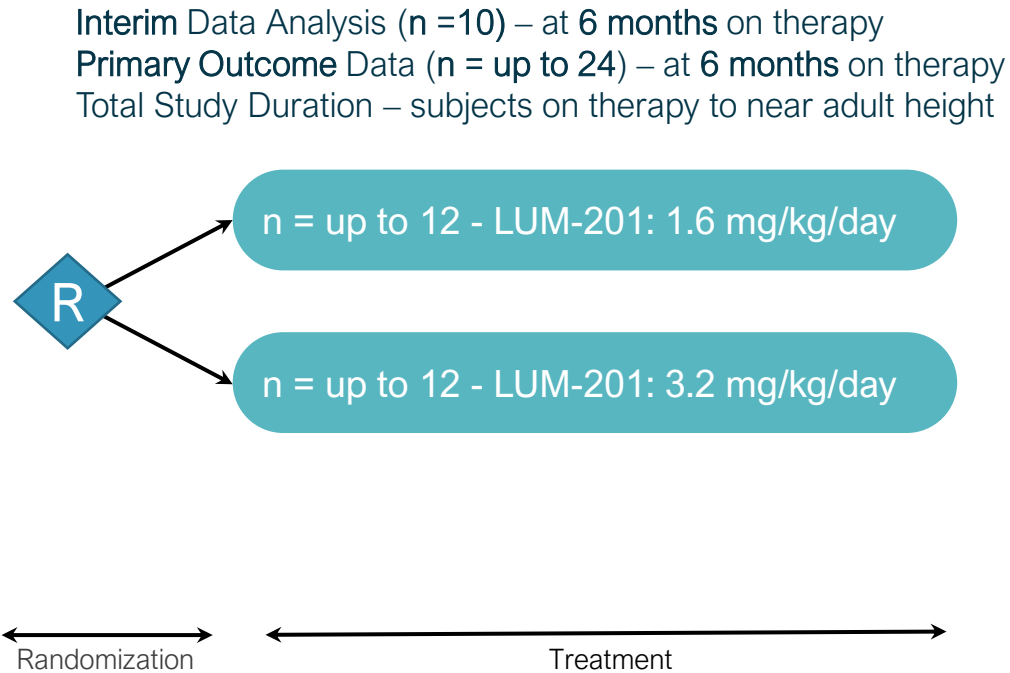
# OraGrowthH212 Trial

PK/PD Trial Evaluating Oral LUM-201 in PGHD

# OraGrowthH212 Trial: Pharmacokinetic / Pharmacodynamic Trial in PGHD

## OraGrowthH212 TRIAL

- n = up to 24
- Open-label study
- PGHD patients
- rhGH-treatment naïve
- Dosing to near-adult height
- Single, specialized clinical site
- Q10 minute GH sampling for 12 hours



### Objectives

#### Primary Endpoints:

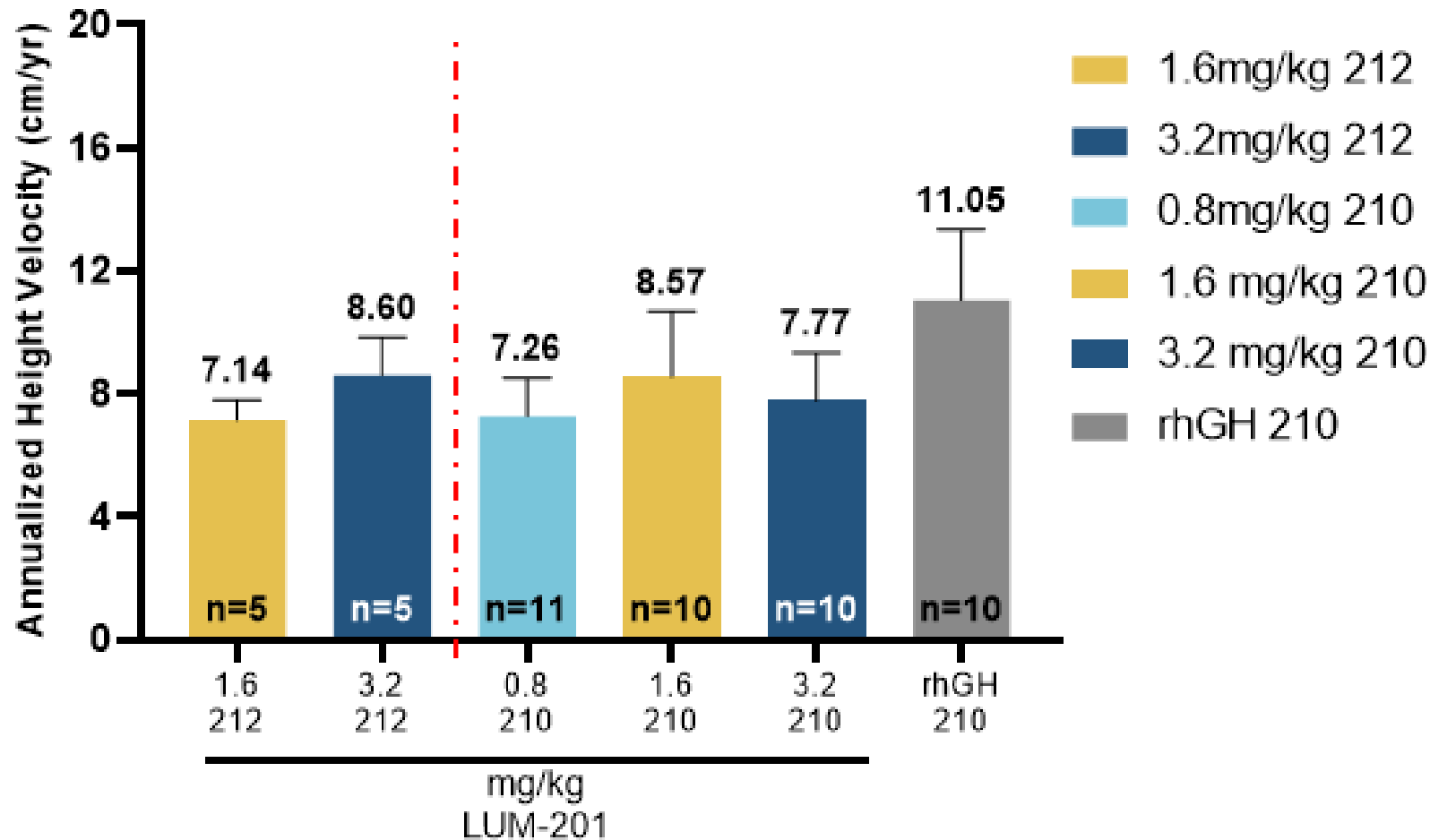
- Assess LUM-201 effect on endogenous GH pulsatility and Annualized Height Velocity (AHV)
- Evaluate PK/PD in children

#### Goals:

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

**Interim AHV and safety data on 10 subjects announced Nov. 14, 2022**  
**Primary outcome data on up to 24 patients anticipated 2H 2023**

# OraGrowthH212 & OraGrowthH210 Comparative AHVs at 6 Months

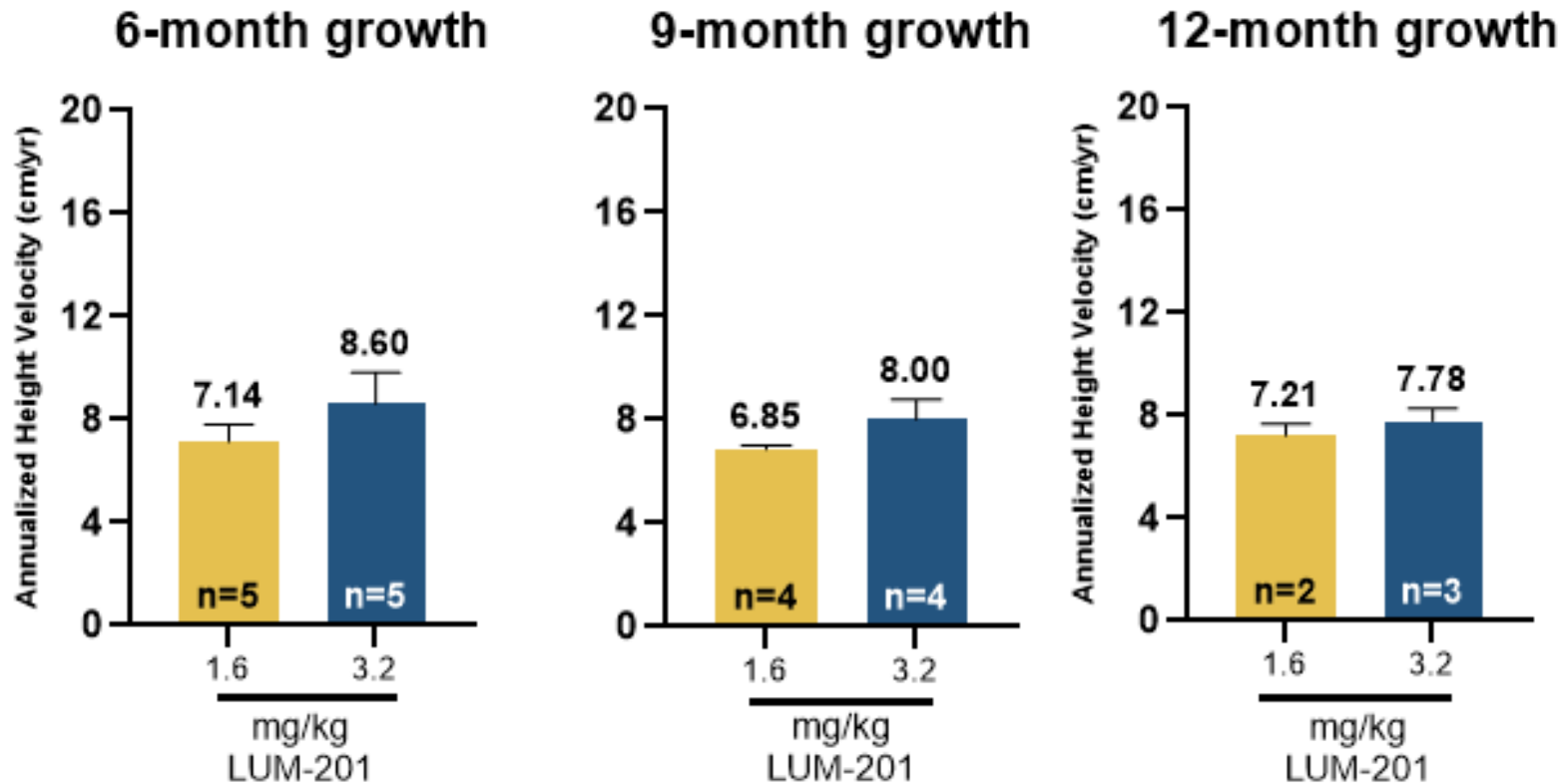


## Conclusions

- OraGrowthH212 Trial results showed a similar growth rate to 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 growth rate seen in OraGrowthH210 and OraGrowthH212



# OraGrowthH212 Data Demonstrate Durable Response

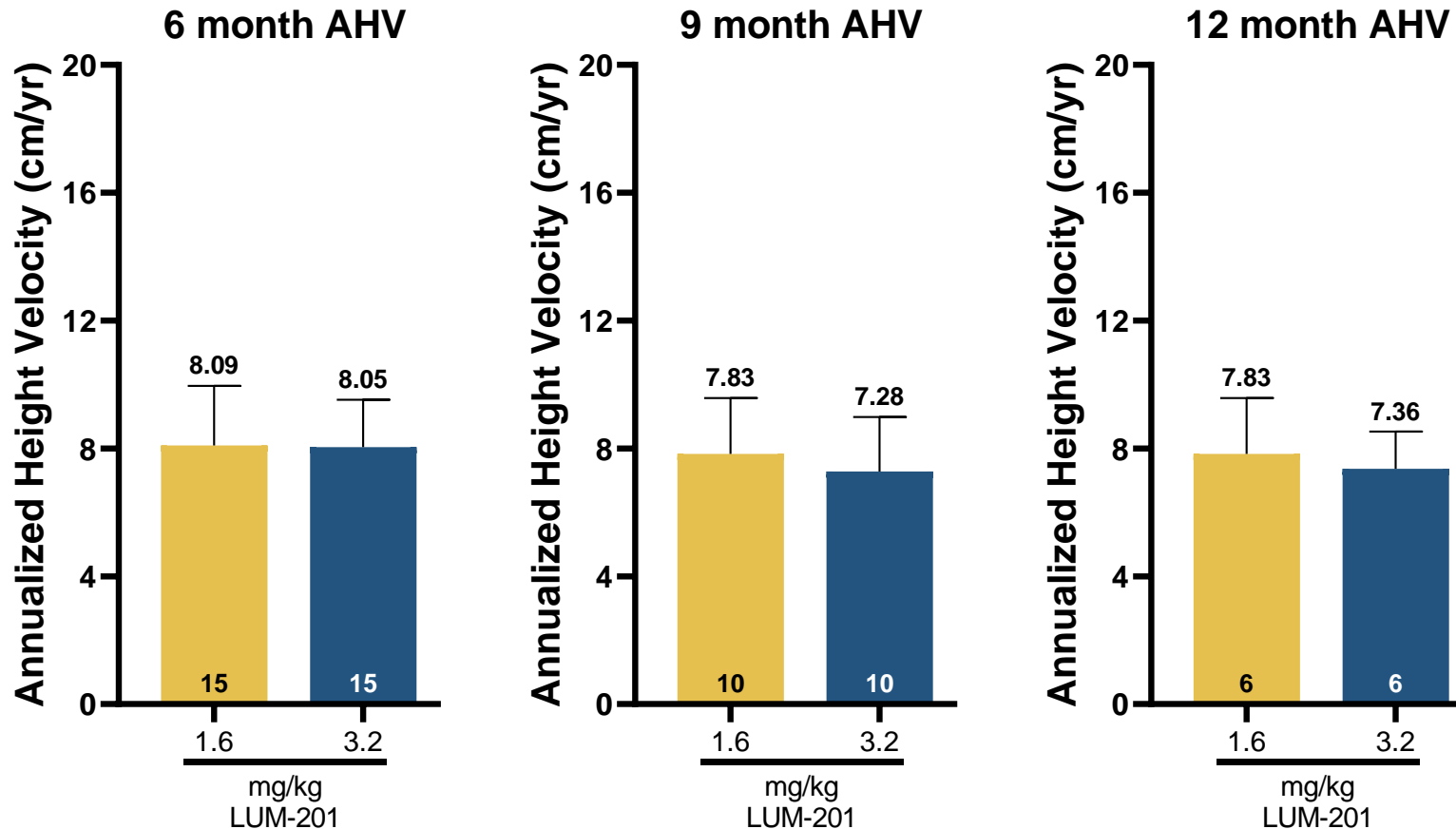


## Conclusions

- OraGrowth212 data also demonstrate growth is durable out 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

# OraGrowtH210 & OraGrowtH212 Interim Data Combined

## Annualized Height Velocity for LUM-201 Combined Data from OraGrowtH210 & OraGrowtH212 Trials



### 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

# Safety and Tolerability

# Interim Safety and Tolerability Profile

- We believe LUM-201 will demonstrate a favorable safety profile as data from both OraGrowth trials to date show comparable safety and tolerability to the rhGH subjects in the trials.
- **No meaningful safety signals to date**
  - In laboratory values
  - In Adverse Event (AEs) data
  - In ECGs values

# Financials

# Lumos Pharma Financial Information as of September 30, 2022

Values in USD

Cash	\$73.7M
Debt	\$0
Shares Outstanding	8.4M
Cash Use in Q4 2022	\$8.5-\$9.5M
Fiscal Year End	December 31



**Cash balance to support current operations into 2Q 2024,  
Beyond primary outcome data readouts for OraGrowth210 and OraGrowth212 Trials 2H 2023**

# Conclusions

# 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 conversation to oral therapy



# Interim Analysis Supplemental Materials

# Safety Profile at Interim Analysis for OraGrowthH210 Trial

66 subjects randomized to date with safety data available for 58 subjects at interim analysis

	<b>PEM Dose*</b>	<b>0.8 mg/kg</b>	<b>1.6 mg/kg</b>	<b>3.2 mg/kg</b>	<b>ALL LUM-201</b>	<b>rhGH 34 mcg/kg</b>
<b>N =</b>	<b>86</b>	<b>14</b>	<b>15</b>	<b>14</b>	<b><u>43</u></b>	<b>15</b>
<b>Number of AEs</b>	29	31	45	38	114	21
<b>Subjects with AE (%)</b>	17 (19.8%)	8 (57.1%)	13 (86.7%)	9 (64.3%)	30 (69.8%)	9 (60.0%)
<b>Treatment Related AEs (N)</b>	7	2	1	3	6	3
<b>Subjects with Treatment Related AEs (%)</b>	4 (4.7%)	1 (7.1%)	1 (6.7%)	2 (14.3%)	4 (9.3%)	2 (13.3%)
<b>Subjects with SAEs (%)**</b>	1 (1.2%)**	0 (0.0%)	0 (0.0%)	0 (0.0%)	0 (0.0%)	0 (0.0%)

\*Subjects that received a single PEM dose during screening and prior to randomization are included in the safety database, not included in ALL LUM-201

\*\*PEM dose SAE deemed not treatment related: Dehydration related to Rotavirus infection acquired between PEM dose & randomization

# Specific AEs – No meaningful signal

66 subjects randomized to date with safety data available for 58 subjects at interim analysis

	PEM Dose* N=86	0.8 N=14	1.6 N=15	3.2 N=14	ALL N=43	rhGH N=15
<b>Arthralgia</b>	1 (1.2%)	1 (7.1%)	2 (13.3%)	2 (14.3%)	5 (11.6%)	1 (6.7%)
<b>Myalgia</b>	0 (0.0%)	0 (0.0%)	0 (0.0%)	0 (0.0%)	0 (0.0%)	3 (20.0%)
<b>Headache</b>	2 (2.3%)	2 (14.3%)	3 (20.0%)	2 (14.3%)	7 (16.3%)	2 (13.3%)
<b>Lethargy</b>	2 (2.3%)	0 (0.0%)	0 (0.0%)	0 (0.0%)	0 (0.0%)	0 (0.0%)
<b>Abd. pain</b>	1 (1.2%)	0 (0.0%)	0 (0.0%)	2 (14.3%)	2 (4.7%)	0 (0.0%)
<b>Emesis</b>	2 (2.3%)	1 (7.1%)	1 (6.7%)	1 (7.1%)	3 (7.0%)	1 (6.7%)
<b>Inc. appetite</b>	2 (2.3%)	1 (7.1%)	1 (6.7%)	0 (0.0%)	2 (4.7%)	2 (13.3%)
<b>Hypoglycemia</b>	1 (1.2%)	0 (0.0%)	0 (0.0%)	0 (0.0%)	0 (0.0%)	0 (0.0%)
<b>Orophary. pain</b>	0 (0.0%)	1 (7.1%)	1 (6.7%)	0 (0.0%)	2 (4.7%)	1 (6.7%)

\*Subjects that received a single PEM dose during screening and prior to randomization are included in the safety database, not included in ALL LUM-201

# Specific AEs - No meaningful signal

66 subjects randomized to date with safety data available for 58 subjects at interim analysis

	PEM Dose N=86	0.8 N=14	1.6 N=15	3.2 N=14	ALL N=43	rhGH N=15
<b>Asthma</b>	0 (0.0%)	0 (0.0%)	0 (0.0%)	1 (7.1%)	1 (2.3%)	0 (0.0%)
<b>Hyperhydrosis</b>	2 (2.3%)	0 (0.0%)	0 (0.0%)	0 (0.0%)	0 (0.0%)	0 (0.0%)
<b>Rash</b>	0 (0.0%)	0 (0.0%)	1 (6.7%)	1 (7.1%)	2 (4.7%)	0 (0.0%)
<b>Urticaria</b>	0 (0.0%)	0 (0.0%)	0 (0.0%)	0 (0.0%)	0 (0.0%)	1 (6.7%)
<b>Inj. Site bruising</b>	0 (0.0%)	0 (0.0%)	0 (0.0%)	0 (0.0%)	0 (0.0%)	1 (6.7%)
<b>Hematuria</b>	0 (0.0%)	0 (0.0%)	0 (0.0%)	0 (0.0%)	0 (0.0%)	1 (6.7%)
<b>FT4 decrease</b>	0 (0.0%)	0 (0.0%)	0 (0.0%)	0 (0.0%)	0 (0.0%)	1 (6.7%)
<b>Urine ketones</b>	1 (1.2%)	0 (0.0%)	0 (0.0%)	0 (0.0%)	0 (0.0%)	0 (0.0%)
<b>Hypotension</b>	1 (1.2%)	0 (0.0%)	0 (0.0%)	0 (0.0%)	0 (0.0%)	0 (0.0%)

\*Subjects that received a single PEM dose during screening and prior to randomization are included in the safety database, not included in ALL LUM-201

# Laboratory Shifts: No meaningful signal

66 subjects randomized to date with safety data available for 58 subjects at interim analysis\*

	0.8 mg/kg N=14	1.6 mg/kg N=15	3.2 mg/kg N=14	ALL N=43	rhGH N=15
<b>ALT NI to high</b>	2/12 (16.7%)	1/15 (6.7%)	2/12 (16.7%)	5/39 (12.8%)	5/12 (41.7%)
<b>TAP NI to high</b>	1/12 (8.3%)	0/15 (0.0%)	1/12 (8.3%)	2/39 (5.1%)	5/12 (41.7%)
<b>Bili NI to high</b>	0/13 (0.0%)	0/15 (0.0%)	0/13 (0.0%)	0/41 (0.0%)	0/15 (0%)
<b>Creat. NI to high</b>	0/13 (0.0%)	0/15 (0.0%)	0/13 (0.0%)	0/43 (0.0%)	0/12 (0%)
<b>Gluc NI to high</b>	0/13 (0.0%)	3/15 (20.0%)	1/13 (7.7%)	4/41 (9.8%)	1/12 (8.3%)
<b>Phos. NI to high</b>	3/13 (23.1%)	2/15 (13.3%)	3/13 (23.1%)	8/41 (19.5%)	5/12 (41.7%)
<b>Eos NI to high</b>	2/11 (18.2%)	3/15 (20.0 %)	2/13 (15.4%)	7/39 (17.9%)	3/12 (25.0%)
<b>Gran. NI to low</b>	1/11 (9.1%)	3/15 (20.0%)	4/13 (30.8%)**	8/39 (20.5%)	1/12 (8.3%)
<b>Gran. NI to high</b>	0/11 (0.0%)	1/15 (6.7%)	2/13 (15.4%)**	3/39 (7.7%)	0/12 (0%)

\* Percentages calculated based on subjects with both baseline and post-baseline assay data

\*\* Bidirectional shifts diminish any concern

# Baseline Characteristics for Top Dose Cohorts in 210 and 212 Studies

	210 1.6 mg/kg N=10	210 3.2 mg/kg N=10	212 1.6 mg/kg N=5	212 3.2 mg/kg N=5
Age (Mos)	99.3	96.1	93.6	91.0
Height SDS	-2.35	-2.30	-1.99	-2.26
IGF-1 SDS	-1.17	-1.39	-1.11	-0.83
Delta MPH	1.76	1.96	0.57	0.70
BA delay yr	1.91	2.19	1.59	1.96
BMI SDS	-0.35	-0.70	0.05	0.66
AHV @ 6 Mos	8.57	7.77	7.14	8.60

# Additional Supplemental Materials



# LUM-201 Program Pipeline

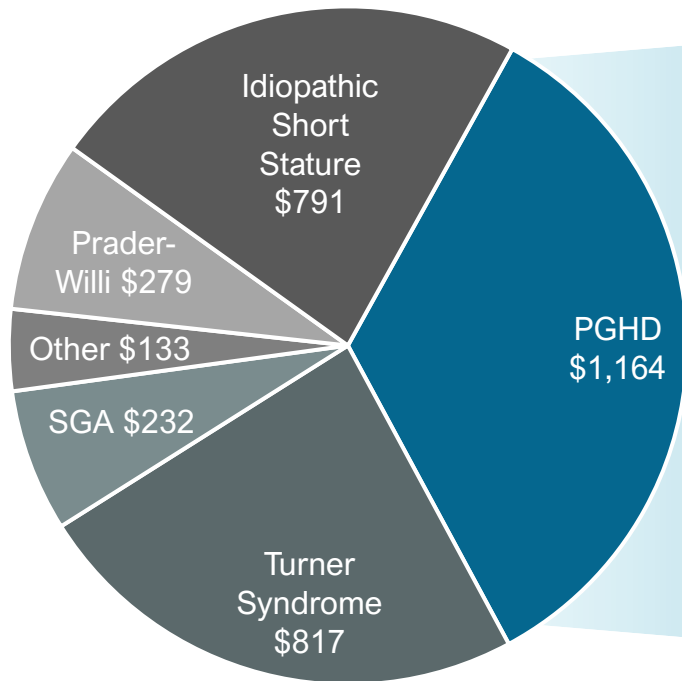
	Study	Pre-Clinical	Phase 1	Phase 2	Phase 3	Status
LUM-201 (Ibutamoren) in PGHD	Phase 2					Ongoing Phase 2 trial: Interim analysis obtained Primary outcome data 2H 2023
	Long-term extension					Proposed long-term extension study for OraGrowth Trials
	PK/PD trial					Ongoing PK/PD trial: Interim analysis obtained Primary outcome data 2H 2023
	Switch trial					Switch trial evaluating LUM-201 in subjects from rhGH arm of OraGrowthH210 Trial: Ongoing
LUM-201 in NAFLD	Phase 2 pilot trial					Pilot trial initiated by Mass Gen Hospital (MGH) evaluating LUM-201 in NAFLD: Enrolling

**Lumos Pharma is evaluating additional indications for LUM-201 for Phase 2 studies**

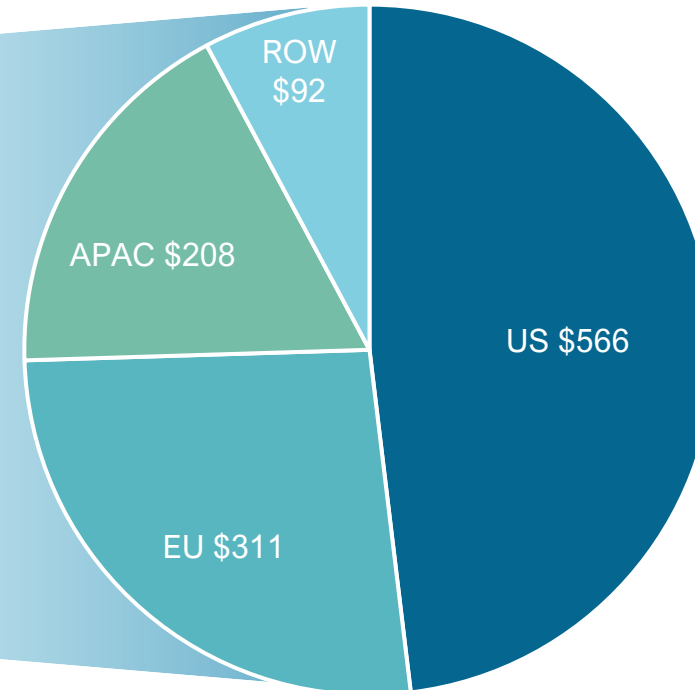


# PGHD is ~35% of the \$3.4B Pediatric Recombinant Growth Hormone Market

2018 Global rhGH Sales \$3.4B\*  
(Values below in \$millions)



2018 Sales of rhGH for PGHD \$1.2B\*  
(Values below in \$millions)



- Pediatric rhGH market projected to grow ~8% per year\*
- Well characterized market with established reimbursement mechanisms
- Current SOC consists of daily injectables; expected to convert to weekly injectables
- **Pediatric rhGH market appears primed for conversion to oral therapy**

\*Grandview Research, hGH Market, 2018, excludes Adult Growth Hormone Deficiency

# Market Research: Daily Oral Therapeutic Preferred Over Weekly Injectable

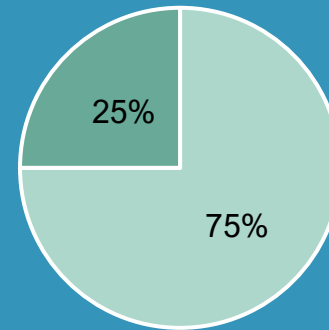
Consideration	Market Research Findings <sup>1</sup>
Unmet Need	Non-injectable (oral) therapy; Less frequent administration of injectable therapy
Preference	Vast majority of physicians & caregivers surveyed prefer daily oral tablet over weekly injectable
MOA	Favorable impression regarding LUM-201 affecting natural physiology vs bolus rhGH treatment
Treatment Decisions	Collaborative between physicians and caregivers
Payer Decisions	Price policies in place for category – small molecule COGS should provide attractive margins



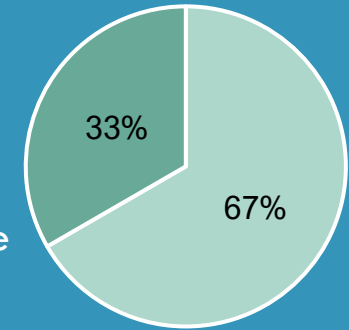
### Interview Question:

If a daily oral secretagogue and a weekly rhGH injectable product were both FDA-approved and available for use, which product would you prefer?

### Physicians



### Caregivers



■ Daily Oral  
■ Weekly Injectable

<sup>1</sup> Initial Primary Research of PGHD Market conducted for Lumos by Triangle Insights. Physicians N = 20. Caregivers N = 9.

# Pediatric Growth Hormone Deficiency (PGHD) – Conversion from Injection to Oral

## PGHD

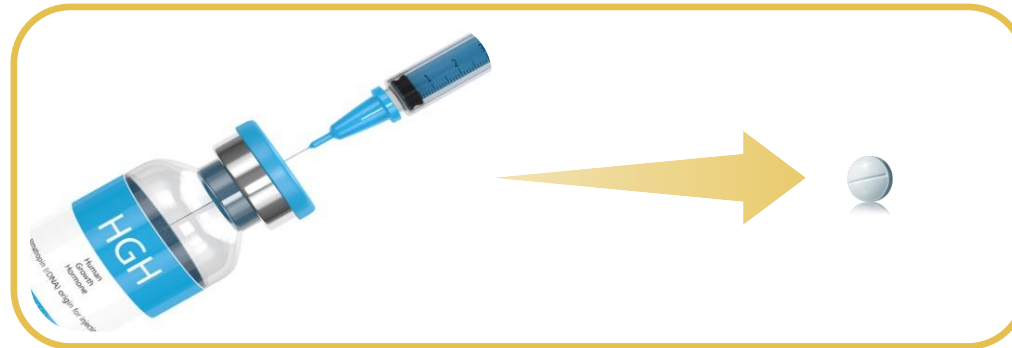
- **Inadequate secretion of growth hormone during childhood**
- Majority of cases are idiopathic
- Slower physical growth
- Negative effect on metabolic processes
- Incidence  $\approx$  1:3500<sup>1</sup>

## Current Treatment

- **Injectable therapies are only options**
- Daily, subcutaneous injections of recombinant human growth hormone (rhGH) represent standard of care
- Weekly rhGH injections are entering the market

## Unmet Need

- **Standard treatment is ~2,500 daily injections over multi-year period**
- Injections can be painful and burdensome
- Missed doses lead to suboptimal growth<sup>2,3</sup>
- Initial market research supports oral therapy vs weekly injections



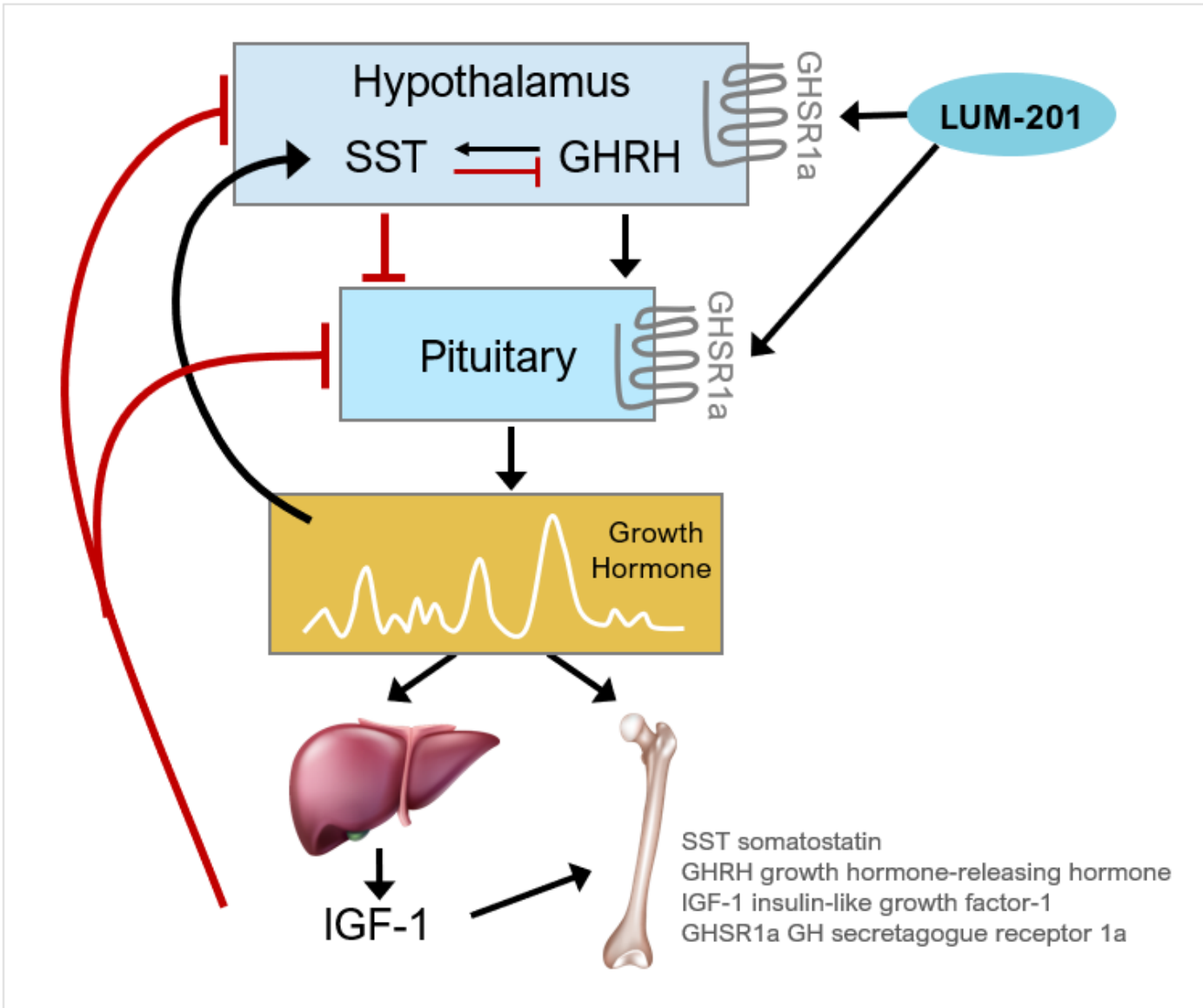
**An established market is now primed for the first oral alternative**

<sup>1</sup> GlobalData EpiCast Report for Growth Hormone Deficiency Epidemiology forecast to 2026

<sup>2</sup> Rosenfeld 2008 Endocrine Practice

<sup>3</sup> Cutfield 2011 PLOS ONE

# LUM-201 Stimulates Natural Growth Hormone Secretion



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

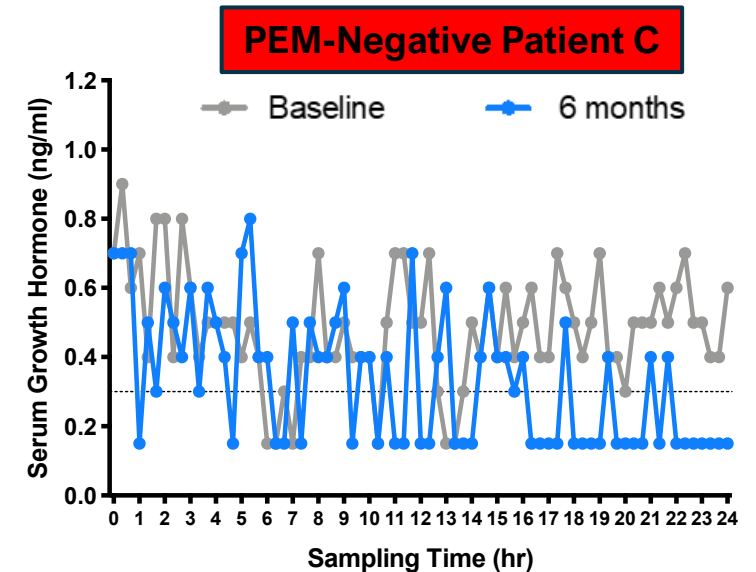
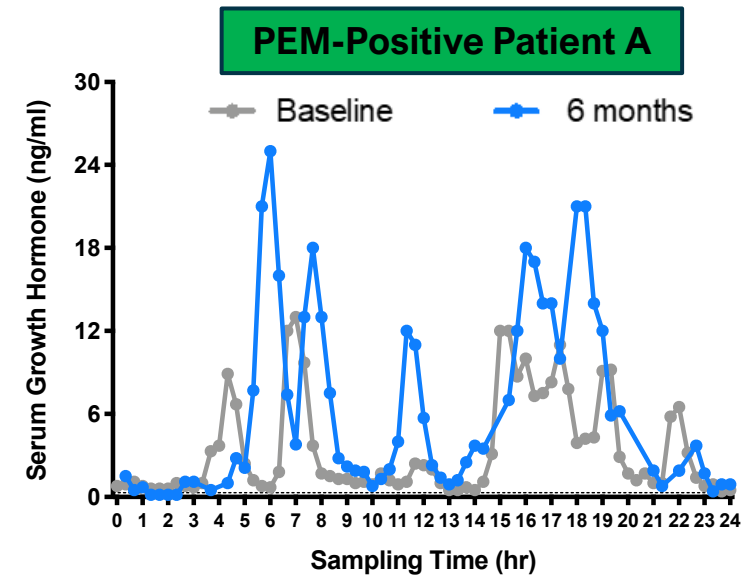
- LUM-201 is an oral GH secretagogue\*
- Acts on specific receptors in hypothalamus and pituitary to stimulate release of GH<sup>1</sup>
- Increases the amplitude of natural pulsatile GH secretion<sup>2,3</sup>
- LUM-201 stimulated GH release regulated by natural GH/IGF-1 feedback mechanisms
- Differentiated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

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

**LUM-201 substantially increased GH secretion & height velocity in PEM+ PGHD patients at 6 months on therapy**

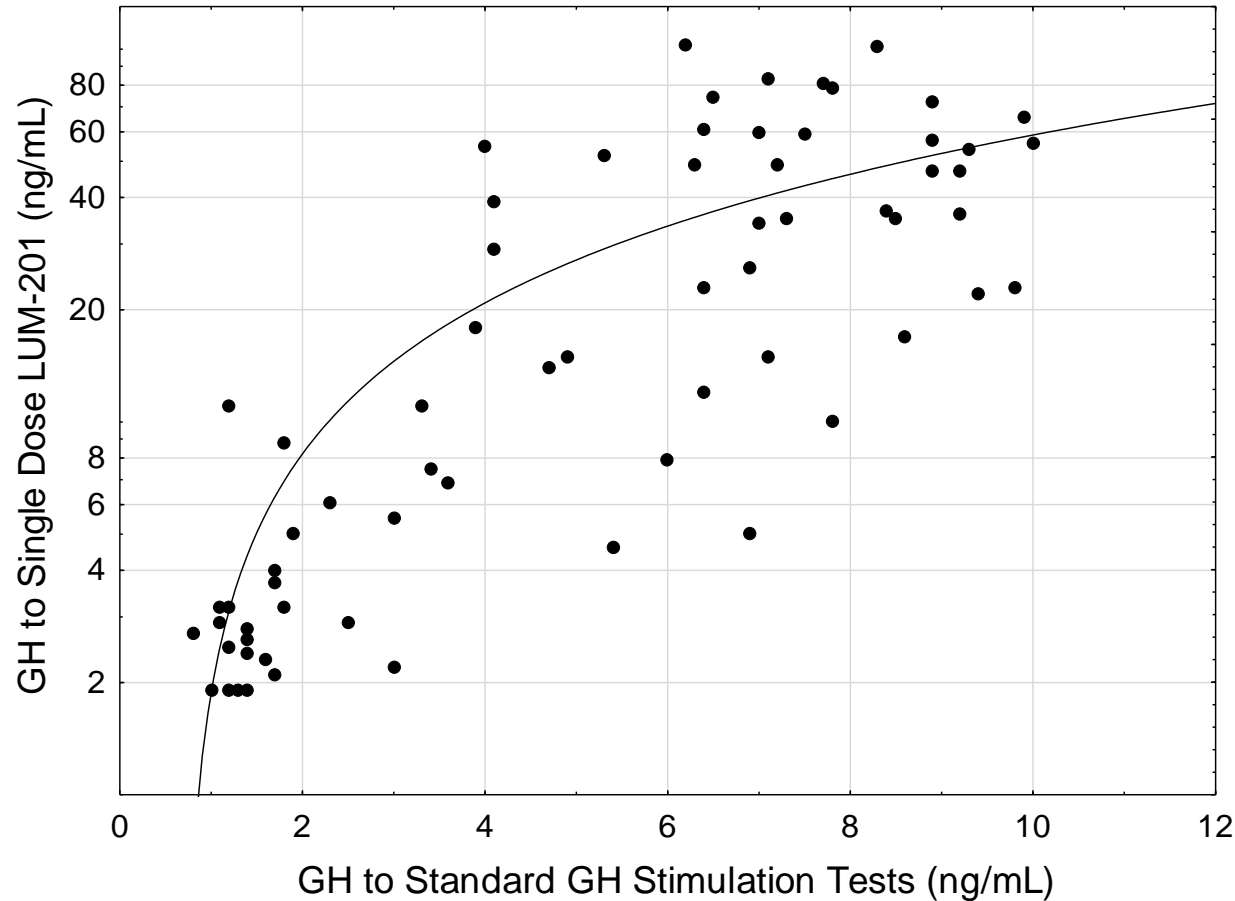
		PEM Positive				PEM Negative	
		Patient A		Patient B		Patient C	
		Baseline	6months	Baseline	6months	Baseline	6months
	IGF-1 (ng/ml)	182	231	53	72	17	15
Q20m 24h GH	Mean (ng/ml)	3.4	6.3	1.0	1.3	0.5	0.3
	AUC (ng*hr/ml)	75.5	137.3	17.6	25.0	4.9	3.4
	Height Velocity (cm/yr)	3.7	7.9	3.5	8.9	1.1	1.8

PGHD patients administered 0.8 mg/kg/day LUM-201 for 6 months\*



\* Merck Study 020 patient subset. Cassorla, F.

# More GH Released from LUM-201 Stim than from Standard Stim Test Agents



68 children with growth hormone deficiency

All had 2 standard GH stimulation tests

- Standard test agents: arginine, clonidine, l-dopa, glucagon, insulin

All had a single dose of LUM-201 stim test

*Data presented at the 2021 Annual Meeting of The Endocrine Society and published online in the journal, Hormone Research in Paediatrics, March 2022*

## Exclusivity and Barriers with Orphan Designation and IP

Orphan Drug Designation received in US and EU for GHD in 2017

With potential pediatric extensions, LUM-201 is eligible for:

12 years market exclusivity in EU \*

7.5 years market exclusivity in USA \*

Plan to seek Orphan Drug Designation in Japan

Intellectual Property – Patent granted for “Detecting & Treating GHD”

Use of LUM-201 in PGHD and other GHD indications based on PEM strategy

Patents for LUM-201 in GHD with protection through 2036

- Patents granted in US, Australia, EU, Israel, Japan, S. Korea, Hong Kong and Ukraine
- Additional applications pending in multiple jurisdictions

Applications for LUM-201 in NAFLD being prosecuted in multiple jurisdictions

# Study of Oral LUM-201 in Non-Alcoholic Fatty Liver Disease (NAFLD) Mass General Investigator-Initiated Phase 2 Pilot Trial

## MGH Initiated Phase 2 Pilot Trial<sup>#</sup>

- n = 10
- Adult NAFLD subjects with relative GH/IGF-1 deficiency
- Open-label
- Single-site pilot study
- 6-month dosing

Currently prescreening subjects<sup>##</sup>

Study Duration – 6 months

n = 10 – LUM-201 at dose level of 25 mg/day

## Objectives

### Primary Objective:

- Determine changes in intra-hepatic lipid content, inflammation, and potentially fibrosis resulting from LUM-201 induced GH augmentation compared to historical placebo-treated controls

**Massachusetts General Hospital (MGH) initiated pilot study of oral LUM-201 in NAFLD  
Prescreening subjects**

<sup>#</sup> Principal Investigator: Laura Dichtel, MD, Assistant Professor, Massachusetts General Hospital

<sup>##</sup> As of August 9, 2022



# LUM-201 Deal Terms

Partner	Upfront Payment	Development Milestones*	Sales Milestones* Worldwide	Sales Royalties, Combined
Ammonett	\$3.5M	\$17M first indication \$14M second indication	\$55M	10% to 12%, subject to standard generic erosion reductions
Merck	N/A	\$14M first indication \$8.5M second indication	\$80M	

\*Milestone figures are maximum, may be less depending on development stage achieved and total net sales up to \$1B