

Transforming Lives with Rare Focus

## Corporate & Clinical Overview

November 18, 2024



#### 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 our finalization of design details for a Phase 3 clinical trial with the FDA in the fourth quarter of 2024 and our positioning to initiate this trial in the second quarter of 2025, that we believe new analyses provide additional support for our planned approach to a placebo-controlled Phase 3 trial of LUM-201 in moderate PGHD, that we believe the trial design would improve the likelihood of success when compared to a non-inferiority study, that cash on hand is expected to support operations into Q1 2025, the potential for LUM-201 to be the first oral therapeutic for PGHD, 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 timing and ability of Lumos Pharma to structure our Phase 3 trial in an effective and timely manner, the ability to obtain FDA approval of, initiate and advance a pivotal Phase 3 trial, as well as advance our clinical and corporate strategy in general, our ability to obtain the capital needed to fund a Phase 3 trial and other business operations, our ability to project, forecast, and manage future cash utilization and reserves needed for contingent future liabilities and business operations, the ability to successfully develop our product candidate 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 Annual Report on Form 10-K for the year ended December 31, 2023 and Quarterly Report on Form 10-Q for the periods ended March 31, June 30, and September 30, 2024, as well as other subsequent reports filed with the SEC. 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.

11.18.2024

### Overview Oral thora



#### Oral therapeutic candidate targeting \$4.7 billion growth-disorder market

## Attractive Market Opportunity

- Global growth hormone (GH) market of ~\$4.7 billion is primed for conversion to oral therapy
- Lead indication, PGHD, is ~\$1.5 billion global opportunity<sup>1</sup>
  - Market research supports rapid conversion to oral and potential expansion opportunities<sup>2</sup>



## Novel Asset with Unique MOA

- Oral LUM-201 pulsatile GH secretion MOA takes advantage of natural physiology
- Orphan Drug Designation in US/EU and issued patents in major markets
- IP protection through 2042 in the US for novel formulation



## Clear Proof of Concept in PGHD

- PEM strategy de-risks patient selection, identifying likely LUM-201 responders<sup>3</sup>
- Phase 2 trials met all primary and secondary endpoints
- Phase 2 data demonstrated LUM-201 produces significant increase in AHV vs baseline
- Consistent PK/PD and attractive investigational safety profile to date in > 1,300 subjects



## Regulatory Path Clarity

- Positive End-of-Phase 2 meeting with FDA held early Q2 2024 regarding Phase 3 program
- Initiation of Phase 3 trial anticipated Q2 2025



First oral therapeutic represents potential paradigm shift in treatment of GHD

<sup>&</sup>lt;sup>1</sup> Based on gross sales of rhGH worldwide

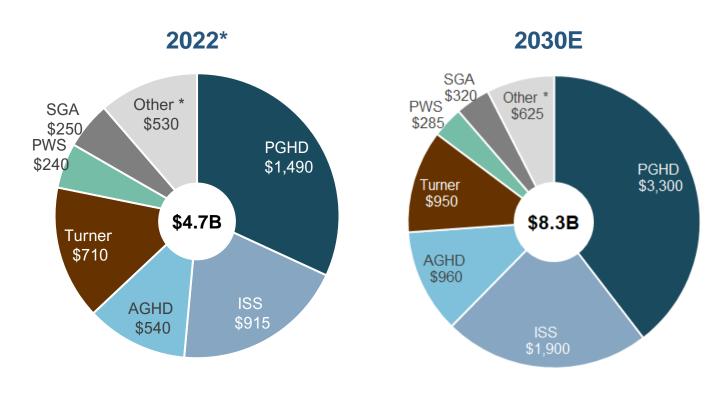
<sup>&</sup>lt;sup>2</sup> Initial Primary Research of PGHD Market conducted for Lumos by Triangle Insights

<sup>3</sup> 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

## Market Opportunity for Oral LUM-201 rhGH Market Projected to Grow through Launch Window



#### rhGH Global Sales by Indication (Gross, US\$ MM)



Global rhGH Market expected to grow by 7.5% CAGR through 2030, reaching \$8.3B



- Long-acting rhGH products addressing limitations of daily rhGH treatment burden
- Growing awareness about GH related diseases
- Increasing healthcare access and spend in developing regions



- Very mature market
- Pricing pressures
- Inconsistent reimbursement policies

<sup>\*</sup> Includes ~\$350M in China sales, indication undisclosed, and ~\$65M in Japan sales, Other / Undetermined; also includes global sales for other short stature syndromes such as Noonan Syndrome, SHOX deficiency, cancer cachexia, etc.

Source: Internal Lumos GH Market Assessment, based on: EvaluatePharma consensus estimates, GlobalData, "GHD Forecast", 2021/04; Grand View Research, "hGH Market Analysis and Segment Forecast", updated 2022 Q1; IQVIA/MIDAS; Japan Pricing Research (Satoru GK, 2023); Regional market participant interviews; Lumos/Akrolyth Analysis



#### LUM-201 Program Pipeline

	Study	Pre-Clinical	Phase 1	Phase 2	Phase 3	Status
LUM-201 (Ibutamoren) in Moderate PGHD	Dose-finding trial	OraGrew TRIAL	tH210			Phase 2 Topline Data met endpoints (Nov 2023) Positive End-of-Phase 2 meeting with FDA
	Long-term extension	OraGrou —— TRIAL	ıtH211			Long-term extension study for OraGrowtH Trials: Ongoing enrollment of patients from Phase 2 trials
	PK/PD trial	OraGrew —— TRIAL	tH212			Phase 2 Topline Data met endpoints (Nov 2023) Data confirms LUM-201's pulsatile MOA
	Switch trial	OraGrew TRIAL	tH213			Switch trial evaluating LUM-201 in subjects from rhGH arm of OraGrowtH210 Trial: Ongoing
LUM-201 in NAFLD	Phase 2 pilot trial	MGH pilot tr	ial			Pilot trial initiated by Mass Gen Hospital (MGH) evaluating LUM-201 in NAFLD: Enrolling

Lumos Pharma is evaluating PWS, ISS, other indications for Phase 2 studies with LUM-201





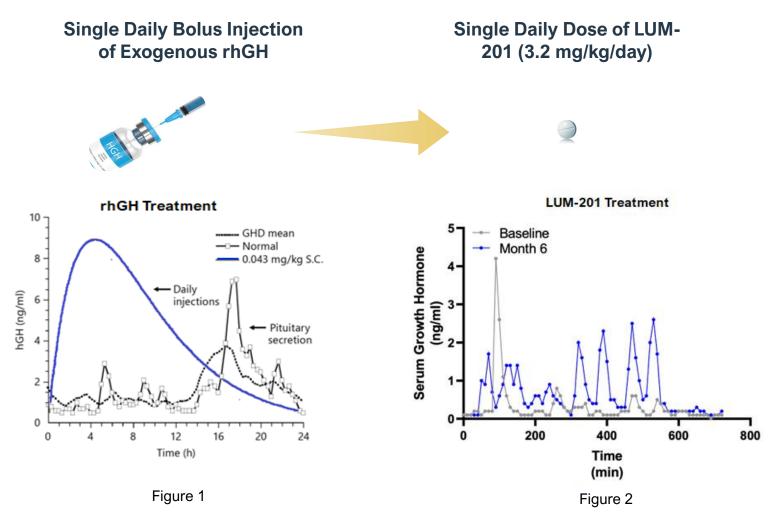




	Current SOC	LUM-201
Route of Administration	Daily injections	Oral
MOA	Synthetic GH	Natural pulsatile GH
Physiology	Exogenous / Supraphysiological	Endogenous / Normal
GH Concentration	4-5X Normal	Normal
IGF-1 Excursions	Often	Rare
Compliance	(-)	+
COGS	High	Low



#### LUM-201 Augments Endogenous Pulsatile Release of Growth Hormone



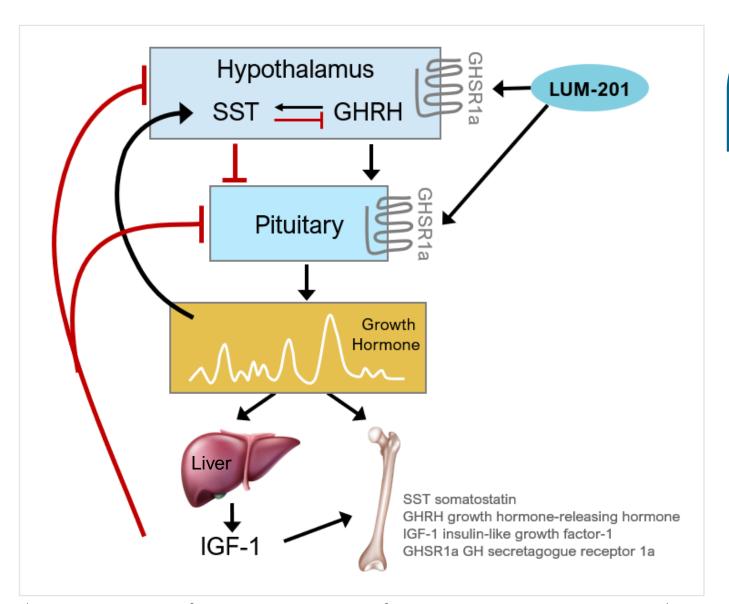
#### **LUM-201 Value Proposition**

- Daily oral therapy
- Normalizes GH and IGF-1 levels through increase in endogenous pulsatile release of Growth Hormone levels
- Consistent PD effect over 24 hours\*
- MOA avoids risk of IGF-1 excursions.
- ➤ Favorable investigational safety profile with >1,300 patients treated to date

Figure 1: Advanced Therapies in Pediatric Endocrinology and Diabetology. Endocr Dev. Basel, Karger, 2016
Figure 2: Cassorla, F, et al. IMPE, March 2023; GH concentrations sampled every 10 minutes for 12-hour period at baseline and after six months of daily oral treatment
\* Merck 020 study



#### LUM-201 Restores Natural Growth Hormone & IGF-1 Secretion



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

- LUM-201 is an <u>oral</u> 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> normalizing GH levels after 6 months on therapy<sup>4</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

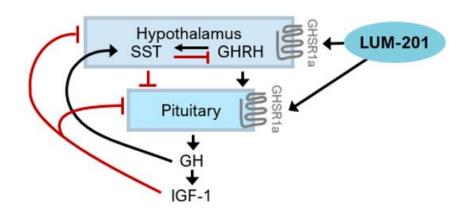
<sup>&</sup>lt;sup>1</sup> Howard 1996 Science <sup>2</sup> Nass 2008 Ann Intern Med <sup>3</sup> Chapman 1997 J Clin Endocrinol Metab <sup>4</sup> Supported by Lumos Pharma Topline Phase 2 Data

<sup>8 \*</sup> GH secretagogue = molecule that stimulates the secretion of growth hormone (GH)



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

## Moderate PGHD (PEM-Positive) Majority of PGHD population<sup>1</sup>



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

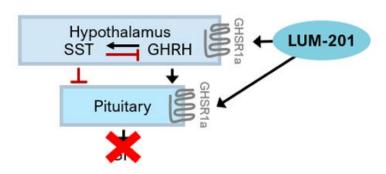
#### **Predictive Enrichment Marker Positive (PEM+)**

- 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
- Functional but reduced HP-GH axis

#### **LUM-201**

Single
Stimulation
Dose
(0.8 mg/kg)
Identifies
LUM-201
Responders

Severe PGHD (PEM-Negative)
Small subset of PGHD population



**Non-Responders to LUM-201** 

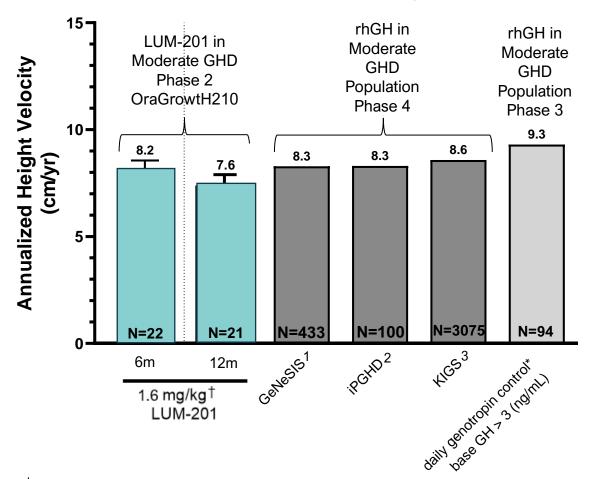
#### **Predictive Enrichment Marker Negative (PEM-)**

- PGHD patients with baseline IGF-1 < 30 ng/ml</li>
- Peak stimulated GH < 5 ng/ml after a single 0.8 mg/kg dose of LUM-201
- Non-functional HP-GH axis



#### OraGrowtH210: LUM-201 Growth Comparable to Multiple 12-Month Historical Datasets

#### 12m ANCOVA vs contemporaries



#### **Highlights**

- AHVs range from 8.3-9.3 cm/yr in historical datasets of moderate PGHD patients treated with daily rhGH
- LUM-201 AHVs of 8.2 and 7.6 cm/yr at 6 and 12 months, respectively, were in line with these historical rhGH growth rates in similar moderate patient populations

<sup>&</sup>lt;sup>T</sup>ANCOVA values represent an analysis of covariates incorporating multiple baseline demographic terms. LUM-201 at 6m PP and 12m PP. Twelve-month LUM-201 AHV updated to include preliminary analysis of full 12-month dataset.

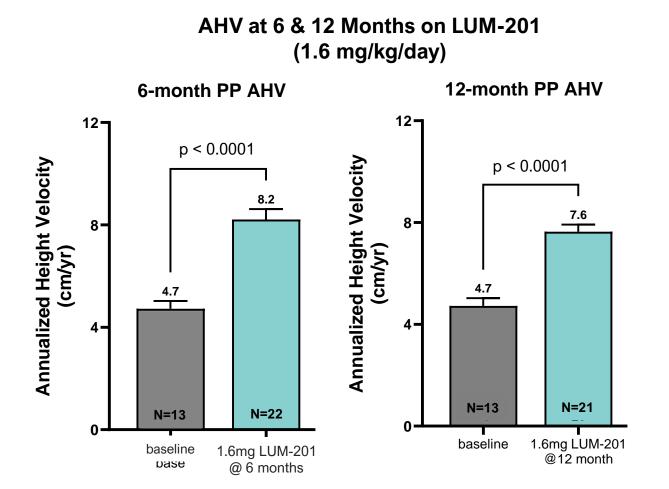
Bars represent Least Squares Mean (LSM); Error bars represent the Standard Error of LSM

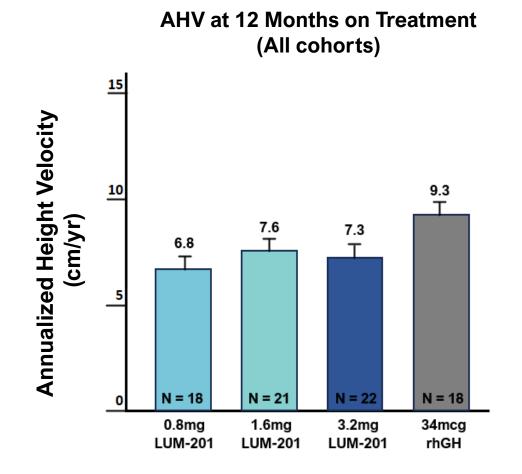
Sources: <sup>1</sup> Blum et al JES 2021, <sup>2</sup> Lechuga-Sancho et al JPEM 2009, <sup>3</sup> Ranke et al JCEM 2010

<sup>\*</sup>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. JCEM Volume 107, Issue 7, July 2022, Pages e2717–e2728.

## Full OraGrowtH210 Data at 12 Months Demonstrate Significant Increase in Growth from Baseline, Durable Effect to 1 year, and Confirm Optimal LUM-201 Dose of 1.6 mg/kg/day



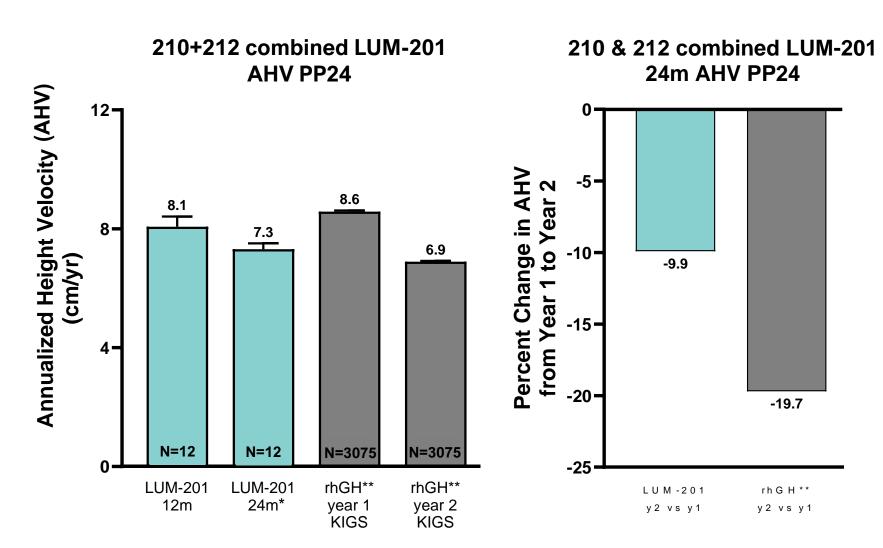




Significant increase in growth on 1.6 mg/kg/day LUM-201 vs baseline suggests this optimal LUM-201 dose is likely to demonstrate superior growth to placebo in Phase 3 trial

#### LUM-201 Data Suggests Sustained Durability of Response vs SOC rhGH OraGrowtH210 & OraGrowtH212 Combined (1.6 and 3.2 mg/kg LUM-201)





#### **Highlights**

- Preliminary data demonstrated 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)

<sup>\*</sup> At 24 months, data include a subset of subjects from OraGrowtH210 trial who met protocol criteria to continue past 12 months.

<sup>\*\*</sup> Ranke et.al. 2010 – Pfizer KIGS database rhGH treated cohort of moderate prepubertal 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.

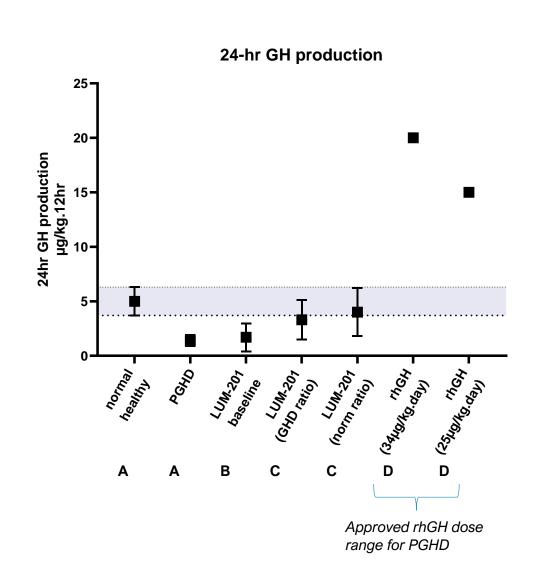


## By Increasing Endogenous 24-hour Pulsatile GH Secretion, LUM-201 Achieved Similar Growth to Exogenous Injectable rhGH, with Only ~20% of GH Concentration Levels

- LUM-201 increased GH to levels similar to a normal growing child
- LUM-201 induced the release of ~20% of the GH from a 34 mcg/kg/day rhGH daily injection, equating to ~26% of GH compared to a 25 mcg/kg/day rhGH dose
- Restoring pulsatility and 24-hr PD effect makes LUM-201 growth more GH efficient as it still captures majority of the growth on rhGH

#### Data Sources/Calculations:

- A Zadik et al Horm Res 1992, 24 hour concentrations calculated based on 12 hour measurement
- B Combined 1.6 and 3.2 mg/kg/day cohorts in '210 and '212 studies
- C 24-hour calculation from 12-hour data using both GHD factor and normal healthy factor
- D Adapted from data in Albertsson-Wikland et al JCEM 1994; 24-hour exposures listed reflect absorbance/bioavailability of  $\sim$ 60% of the administered dose



#### Summary of Topline and Updated Phase 2 Data



#### OraGrowtH210 and OraGrowtH212 Phase 2 Topline Data<sup>1</sup>

- Topline Phase 2 data met all primary and secondary endpoints
- PEM test was reproducible and predicted response to LUM-201
- Oral LUM-201 significantly increased growth rates from baseline
- LUM-201 restored normal GH secretion and IGF-1 levels through increased amplitude of GH pulsatility
- LUM-201 promoted growth similar to injectable rhGH with only 20% of GH concentration levels
- Preliminary 24-month data demonstrated sustained growth on LUM-201
- Favorable investigational safety profile to date

#### **Updated OraGrowtH210 and OraGrowtH212 Data<sup>2</sup>**

- Updated OraGrowtH data corroborate prior data showing durable LUM-201 treatment effect
  - Significant increase in growth from baseline at 6 and 12 months on LUM-201
  - LUM-201 continues to demonstrate durability of response to 12 and 24 months

<sup>&</sup>lt;sup>1</sup> Topline data as announced November 2023.

<sup>&</sup>lt;sup>14</sup> Updated data include full 12-month AHV data for OraGrowtH210 and OraGrowtH212 trials plus additional combined 24-month AHV data announced Q2 2024.



#### Key Milestones Provide Clear Regulatory Pathway

#### **Key Milestones Support Path for Oral LUM-201 to Disrupt Injectable GH Market**

- Positive End-of-Phase 2 meeting with FDA supportive of registrational path forward
  - o FDA recognized LUM-201, a growth hormone secretagogue, as a novel growth promoter
  - FDA acknowledged the use of a placebo-controlled clinical trial design as an appropriate option for a LUM-201 Phase 3 trial
- Phase 3 initiation expected in Q2 2025
  - Proposal of placebo-controlled design should reduce regulatory risk and improve likelihood of success



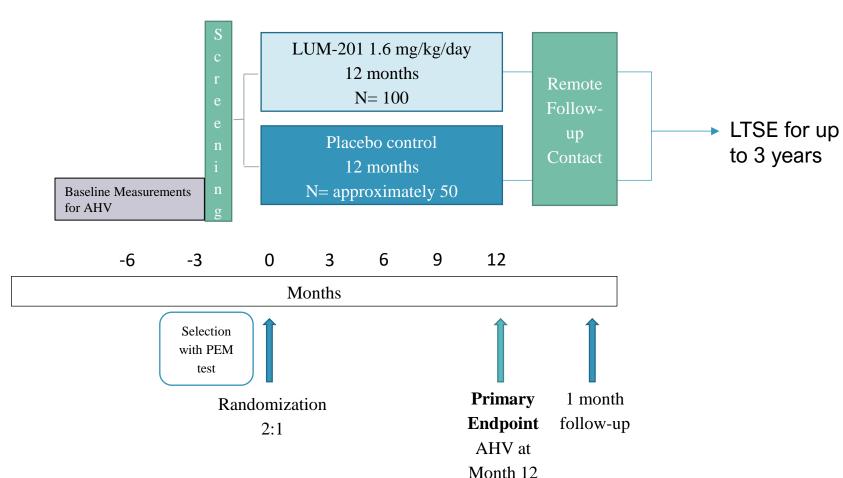
#### Proposed Placebo-Controlled Phase 3 Trial Design



12-month, 2:1 randomization, double-blinded trial of LUM-201 vs placebo with AHV evaluated at 12 months on treatment or placebo.

## Placebo-Controlled Phase 3 Trial

- PEM+ PGHD subjects
- N ~150 total
- 2:1 randomization
- Global trial
- ~80 sites
- AHV primary endpoint at 12 months
- Trial initiation anticipated in Q2 2025



#### **Primary Endpoint:**

Demonstrate superiority of growth on LUM-201 at 12 months vs Placebo at 12 months

LTSE = Long-term Safety Extension AHV = Annualized Height Velocity



#### Why LUM-201 will Win in the Marketplace

LUM-201 Has the Potential to Meet or Beat the Most Important Product Attributes vs. rhGH



## Compliance

- Route of Administration is, by far, the most important product attribute<sup>1</sup>
  - Current compliance for daily GH injections
     50% - 60%<sup>2</sup>
  - 3 or more missed doses per week provides negligible growth benefit<sup>3</sup>
- Overwhelming preference for daily oral over weekly injectable<sup>1</sup>



- 2-year LUM-201 data support more durable growth rates than rhGH through physiologically controlled stimulation of GH axis
- By time of NDA filing, will have 4 – 5 years of data on many subjects from LTSE



# nsurance Coverage

- Currently, physicians are captive to formulary position
- Distinct product category provides opportunity for top tier formulary position for oral LUM-201
- Lowest projected COGS among all competitors

Achieve

Target Height

<sup>&</sup>lt;sup>1</sup>Primary interviews of US Pediatric Endocrinologists and Caregivers, Triangle Insights

<sup>&</sup>lt;sup>2</sup>Kaplowitz, et al, "Economic Burden of Growth Hormone Deficiency in a US Pediatric Population, JMCP, August 2021

<sup>&</sup>lt;sup>3</sup>Cutfield 2011 PLOS ONE



#### LUM-201: Exclusivity and Barriers with Orphan Designation and IP

## Novel Formulation Patent

- Patent allowance granted March 14, 2024, by USPTO for novel LUM-201 formulation
- Formulation enables capsule with mini-tablets of LUM-201 drug product inside
- Extends intellectual property protection through 2042 for covered formulations

## Orphan Drug Designation

- Orphan Drug Designation (ODD) granted in US & EU for GHD in 2017
- LUM-201 eligible for 12 years of exclusivity in EU and 7.5 years of exclusivity in US\*

## Intellectual Property

- Prior 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

## Lumos Pharma Financial Information as of September 30, 2024 *Values in USD*



Cash, Equivalents & Short-term Investments	\$13.5M
Debt	\$0
Shares Outstanding	8.6M
Fiscal Year End	December 31



#### Management – Significant Clinical Development and Commercial Experience





Richard Hawkins Chairman & CEO

Developed Growth Hormone (GH) Receptor Antagonist for Acromegaly at Sensus (sold to Pfizer). Built one of the first contract recombinant protein manufacturing facilities (Covance Biotechnology). Founder of Pharmaco, a pioneer in the contract research organization sector (merged with PPD).



John McKew, PhD
President & Chief Scientific Officer

Prior VP of Research at aTyr Pharma – led team advancing protein-based therapeutics for rare diseases. Former Scientific Director, NIH - National Center for Advancing Translational Science (NCATS) and Therapeutics for Rare and Neglected Diseases (TRND).



Lori Lawley, CPA
Chief Financial Officer

Former SVP, Finance and Controller at Lumos Pharma. Previously, SVP, Finance and Member of the Office of the CEO of NewLink Genetics. Prior to that, Senior Manager in Assurance Services at Ernst and Young.



Pisit "Duke" Pitukcheewanont, MD
Chief Medical Officer

Pediatric endocrinologist and Professor, Clinical Pediatrics, Keck School of Medicine, USC. President, Human Growth Foundation. Former VP Medical Affairs and VP Global Medical Ambassador & Medical Education at Ascendis Pharma; project: long-acting TransCon GH. Former Advisory Board member at Pfizer, Ipsen, Alexion, Ultragenyx, Pharmacia, Serono, others.



Aaron Schuchart, MBA Chief Business Officer

Former Chief Business Officer of Aeglea BioTherapeutics. Former leadership roles in Business Development, Strategy, and Finance at Coherus Biosciences, Novartis Diagnostics/Grifols, and Amgen.