



# Corporate Presentation

May 2020

# Forward Looking Statements

This presentation contains forward-looking statements of Lumos Pharma, Inc. that involve substantial risks and uncertainties. All statements, other than statements of historical facts, contained in this presentation are forward-looking statements, within the meaning of The Private Securities Litigation Reform Act of 1995. The words "anticipate," "believe," "estimate," "expect," "intend," "may," "plan," "target," "potential," "will," "could," "should," "seek" or the negative of these terms or other similar expressions are intended to identify forward-looking statements, although not all forward-looking statements contain these identifying words.

These forward-looking statements include, among others, statements regarding the expected initiation of a Phase 2b clinical trial, the sufficiency of funding for such trial, the potential of an orally administered treatment regimen for PGHD and other indications, projected cash position and its sufficiency to fund the company's operations through data read-out for the Phase 2b trial of LUM-201 in PGHD; impact of regulatory feedback to clinical timelines and costs, results of its clinical trials for product candidates; its timing of release of data from ongoing clinical studies; its plans related to execution of clinical trials; plans related to moving additional indications into clinical development; future priority review voucher (PRV) monetization, anticipated funds from monetization of the PRV, milestones or other economic interests, Lumos Pharma's financial guidance for 2020 and beyond; and any other statements other than statements of historical fact.

Actual results or events could differ materially from the plans, intentions and expectations disclosed in the forward-looking statements that Lumos Pharma makes due to a number of important factors, including the effects of pandemics of other widespread health problems such as the ongoing COVID-19 pandemic and those risks discussed in "Risk Factors" and elsewhere in Lumos Pharma's Annual Report on Form 10-K for the year ended December 31, 2019, the proxy statement on Form DEF14A filed on February 13, 2020, and other reports filed with the U.S. Securities and Exchange Commission (SEC). The forward-looking statements in this presentation represent Lumos Pharma's views as of the date of this presentation. Lumos Pharma anticipates that subsequent events and developments will cause its views to change. However, while it may elect to update these forward-looking statements at some point in the future, it specifically disclaims any obligation to do so. You should, therefore, not rely on these forward-looking statements as representing Lumos Pharma's views as of any date subsequent to the date of this presentation.



**Passionately  
focused on  
developing  
therapeutics for  
rare diseases**

# Overview of Company

- Late-stage novel therapeutic asset, LUM-201, with validating Phase 2b trial in Pediatric Growth Hormone Deficiency (PGHD) anticipated to begin prior to the end of 2020
- Established and sizable overall market targeted of over \$1B\*, with potential to disrupt current treatment regimen for significant subset of patients
- Experienced management team with ability to expand pipeline through addition of other rare disease assets
- Current cash position sufficient to support LUM-201 program through Phase 2b trial read-out
- Additional non-dilutive funds from 60% PRV ownership available to expand portfolio

\* USA, Germany, France, Italy, Spain, UK, Japan (Global Data Opportunity Analyzer: Growth Hormone Deficiency Opportunity Analysis and Forecasts to GDHC069POA, May 2017)

# Significant High-Quality Investors

- Well-known healthcare venture investors
- Large healthcare investors that participate in public and private markets
- Large pharma representatives
- Significant long-term investor



Notable investors in Lumos Pharma, Inc.

# Experienced Management



Richard Hawkins  
Chairman, CEO & President



John McKew, PhD  
COO & CSO



Carl Langren  
CFO



Eugene Kennedy, MD  
CMO



Aaron Schuchart  
CBO

## Experienced management team with significant clinical development and commercial experience




- **Richard Hawkins** – Chairman, CEO & President of Lumos Pharma, developer of Growth Hormone (GH) Receptor Antagonist for Acromegaly at Sensus (sold to Pfizer). Built one of the first contract recombinant protein manufacturing facilities (Covance Biotechnology). Co-founded Pharmaco, a contract research organization (merged with PPD).
- **John McKew** – COO & CSO of Lumos Pharma, former Scientific Dir, NIH - National Center for Advancing Translational Science (NCATS) and Therapeutics for Rare and Neglected Diseases (TRND). Director level, Wyeth Research Genetics Institute.
- **Carl Langren** – CFO of Lumos Pharma, former CFO of BioProtection Systems, Housby Mixer Group, Equity Dynamics, Inc., and Tax Manager with McGladrey Pullen & Co.
- **Eugene Kennedy** - CMO of Lumos Pharma, former Associate Professor of Surgery and Chief of the Section of Pancreaticobiliary Surgery Thomas Jefferson University (Philadelphia), former faculty Johns Hopkins Hospital.
- **Aaron Schuchart** - CBO of Lumos Pharma, former CBO of Aeglea BioTherapeutics, former leadership roles in business development and licensing at Coherus Biosciences, Novartis Diagnostics/Grifols, and Amgen.

# Strategic Priorities

- Initial focus will be on initiation of Phase 2b trial of LUM-201 (ibutamoren) for pediatric growth hormone deficiency (PGHD)
  - Build pipeline through strategic acquisitions of assets focusing on rare diseases
- LUM-201: oral secretagogue candidate for PGHD
    - Established regulatory path – planned Phase 2b trial expected to start prior to the end of 2020
    - Significant market opportunity, well-proven value through industry peers



# LUM-201 Program Pipeline

Product Candidate	Orphan Indication	Pre-Clinical	Phase 1	Phase 2	Phase 3	Status
LUM-201 (Ibutamoren)	Pediatric Growth Hormone Deficiency (PGHD)*					Phase 2b expected to initiate prior to the end of 2020
	Turner Syndrome*					Ongoing clinical planning for Phase 2 trial, timing dependent on PGHD data
	Children Born Small for Gestational Age (SGA)*					Ongoing clinical planning for Phase 2 trial, timing dependent on PGHD data

Company plans to look for acquisitions and collaborations to expand pipeline beyond LUM-201



# PGHD and Standard of Care

- PGHD occurs due to inadequate secretion of growth hormone by the pituitary gland during childhood
- PGHD can be either hereditary or acquired, although the majority of cases have unknown causes (idiopathic)
  - Lack of physical growth is the most obvious manifestation; but numerous metabolic processes are also affected
- PGHD incidence in U.S. approximately 1 in 3500 children<sup>1</sup>
- Standard of care consists of daily, subcutaneous injections of recombinant human growth hormone (rhGH)
  - Can be painful, potentially leading to missed doses and sub-optimal growth<sup>2,3</sup>
  - ~2500 injections over years of treatment



Robust, established market primed for an oral alternative

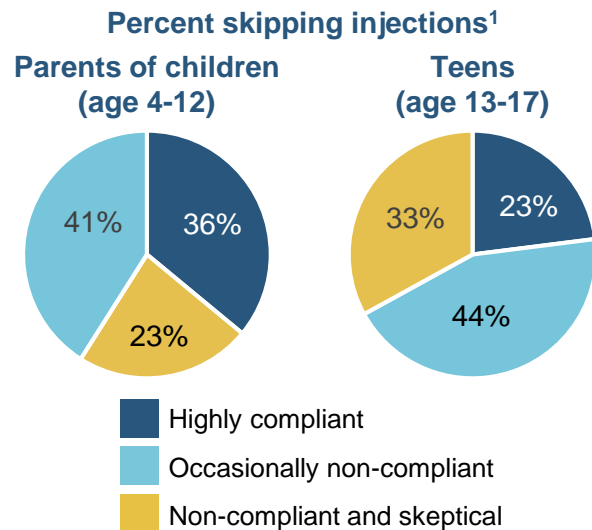
<sup>1</sup> GlobalData EpiCast Report for Growth Hormone Deficiency Epidemiology forecast to 2026

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

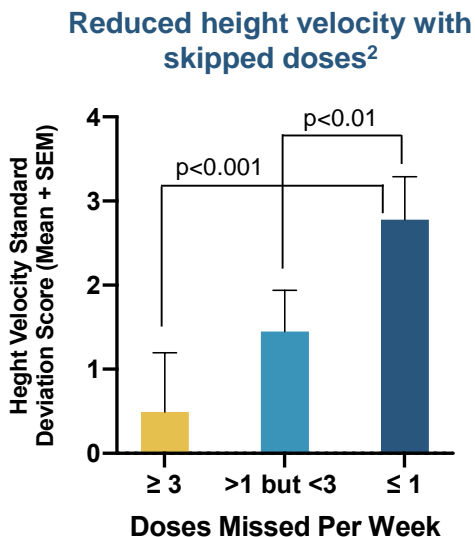
<sup>3</sup> Cutfield 2011 PLOS ONE

# Compliance Issues and Poor Outcomes with Injectables

## Poor Adherence



## Sub-Optimal Outcomes

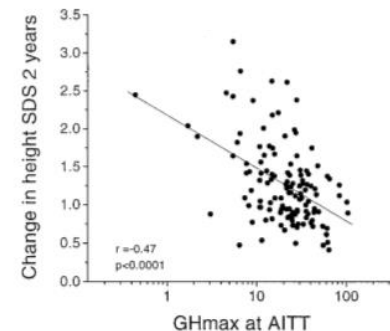
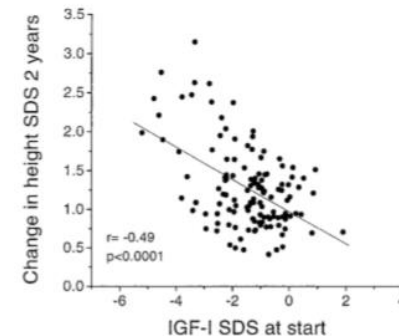


Poor compliance with daily injections of rhGH leads to sub-optimal growth

# GH Deficiency is a Continuum, Not Binary

- It has been long described that:
  - There is a variable range of severity in GHD<sup>1</sup>
  - This contributes to variability in responses to GH therapy: more severely deficient patients respond better than partially deficient<sup>1</sup>
- Several prediction models attempt to explain variability and optimize GH treatment<sup>2</sup>
  - Multiple factors may contribute
  - GH response to standard stimulation tests was most important predictor of first year growth response to rhGH in PGHD in one analysis<sup>3</sup>
  - Inclusion of baseline IGF-1 strengthened model (*right*)<sup>4</sup>

Growth on rhGH and pre-treatment values in PGHD and short children without GHD



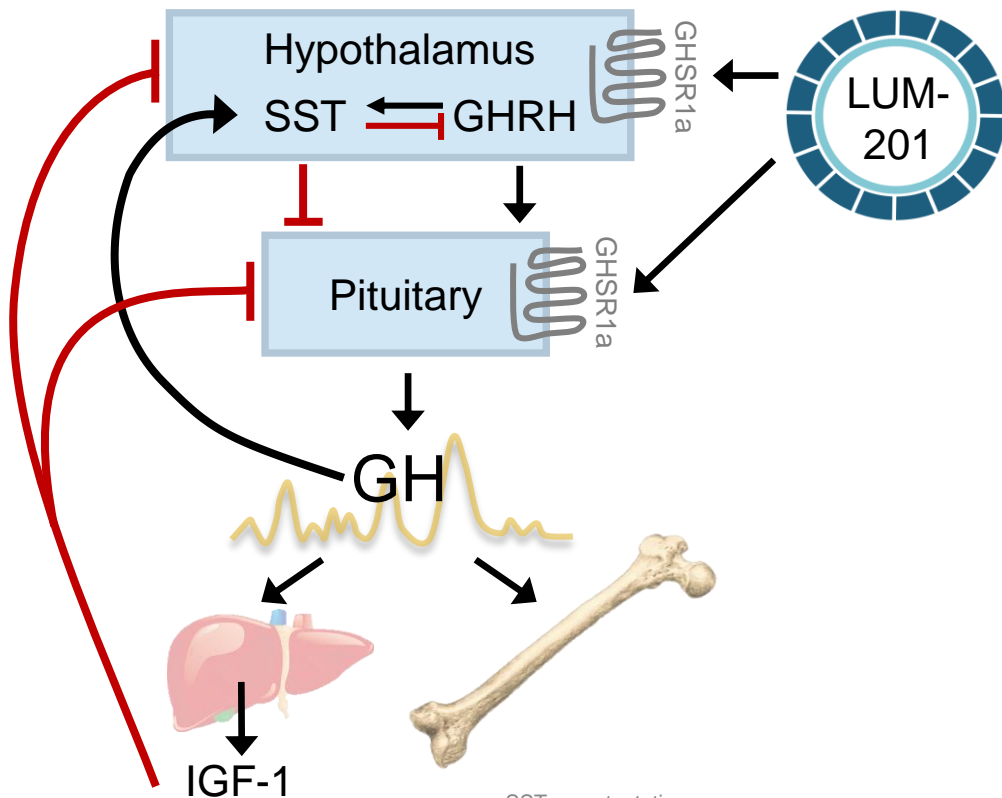
1 Tanner 1971 Arch Dis Childhood

3 Ranke 1999 JCEM

2 Wit 2013 Hormone Res Paed

4 Kristrom 1997 JCEM

# LUM-201 Mechanism of Action



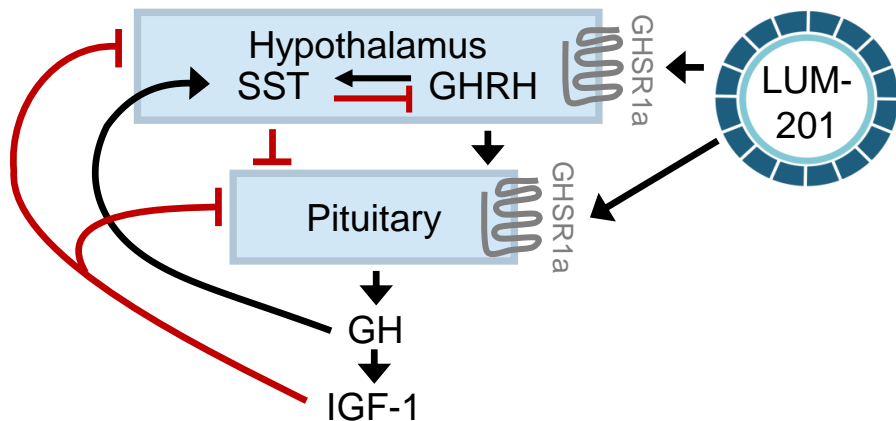
- Oral LUM-201 is a growth hormone (GH) secretagogue
- Acts as an agonist of GH Secretagogue Receptor (GHSR1a) to stimulate GH release<sup>1</sup>
- LUM-201 has been observed to increase the amplitude of endogenous pulsatile GH secretion<sup>2,3</sup>
- LUM-201's stimulatory effect is regulated by GH/IGF-1 feedback

SST somatostatin  
GHRH growth hormone-releasing hormone  
IGF-1 insulin-like growth factor-1  
GHSR1a GH secretagogue receptor 1a

1 Howard 1996 Science  
2 Nass 2008 Ann Intern Med  
3 Chapman 1997 J Clin Endocrinol Metab

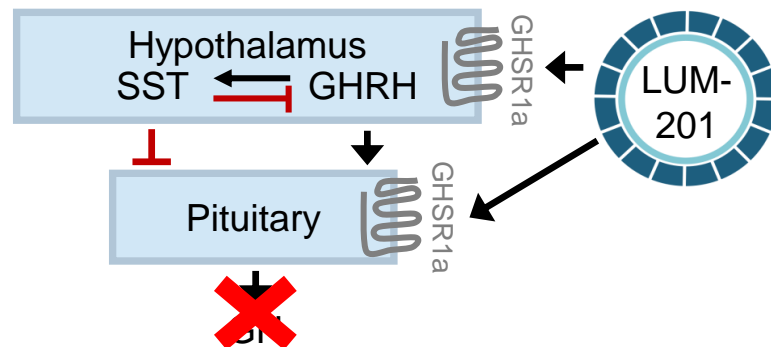
# Targeted PGHD Population

## PEM-Positive: Included



- Functional but reduced HP-GH axis
  - Able to secrete some, but insufficient, GH
  - Expected to respond to LUM-201
  - Represents 50-60% of PGHD patients<sup>1</sup>

## PEM-Negative: Excluded



- Non-functional HP-GH axis
  - Unable to secrete GH
  - Not expected to respond to LUM-201
  - Represents 40-50% of PGHD patients

Predictive Enrichment Markers (PEMs): GH response to single LUM-201 dose and baseline IGF-1 have potential to distinguish these populations

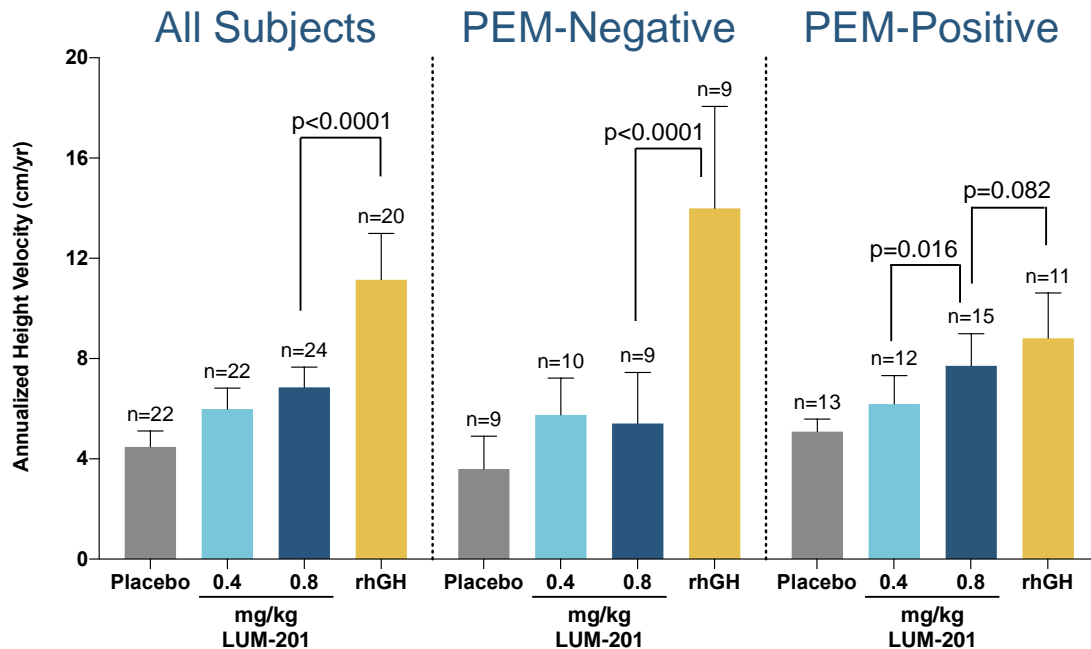
# Prior Clinical Experience in PGHD with LUM-201

- Prior PGHD trials
  - Conducted prior to Lumos acquisition of LUM-201 in July 2018
  - 3 clinical trials in pediatric population explored safety and efficacy
    - Phase 1 Study 019 – PK, Phase 2 Study 020 – Naïve, Phase 2 Study 024 – Previously rhGH treated
  - No significant safety concerns were identified
  - Formulation change midway through Study 020 reduced bioavailability of drug and confounded data
  - Phase 2 trials were discontinued after interim analysis of Study 024
- Scientifically-driven post-hoc analysis enabled (Study 020)
  - Definition of PEM-positive patients, with PEM status planned to be used as an inclusion criteria in future trials

Growth response in prior trials (highest dose tested) suggests potential improved efficacy at higher doses

# Post-Hoc: Predictive Enrichment Markers at Work

- Naïve PGHD, Study 020
  - Data from first 6 months prior to formulation change<sup>1</sup>
- In PEM-positive subset
  - LUM-201 0.8 mg/kg not statistically different from rhGH
  - Dose response observed: LUM-201 0.8 and 0.4 mg/kg are statistically different
- Lumos expects prospective application of PEMs and higher doses to improve response



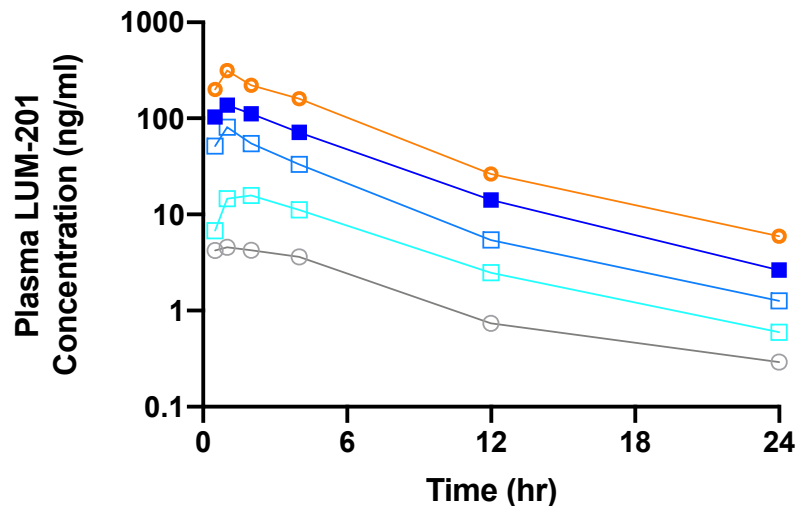
<sup>1</sup> A formulation change occurred 6 months into dosing of this trial and was also used for subsequent PGHD trials, resulting in substantially lower exposure of LUM-201. Data on file.



# PK/PD Response Supports Proposed Doses in PGHD

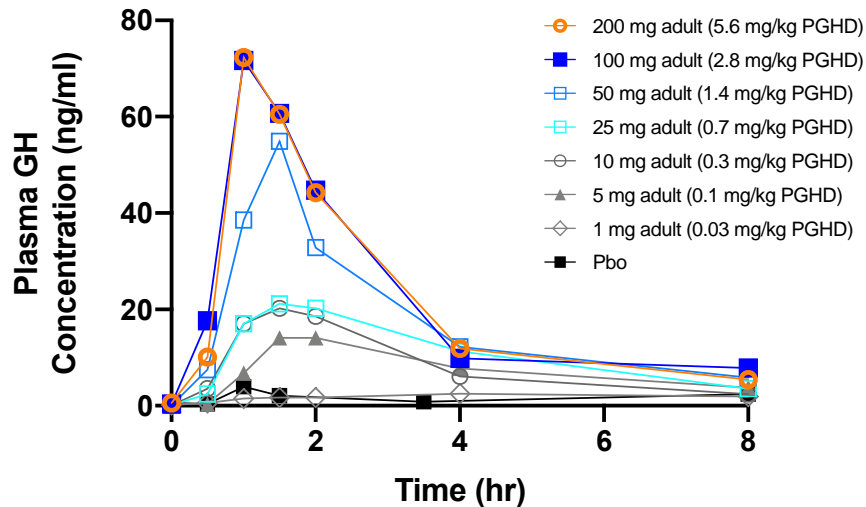
## Pharmacokinetics

- Dose response to 5.6 mg/kg PGHD dose equivalent\*



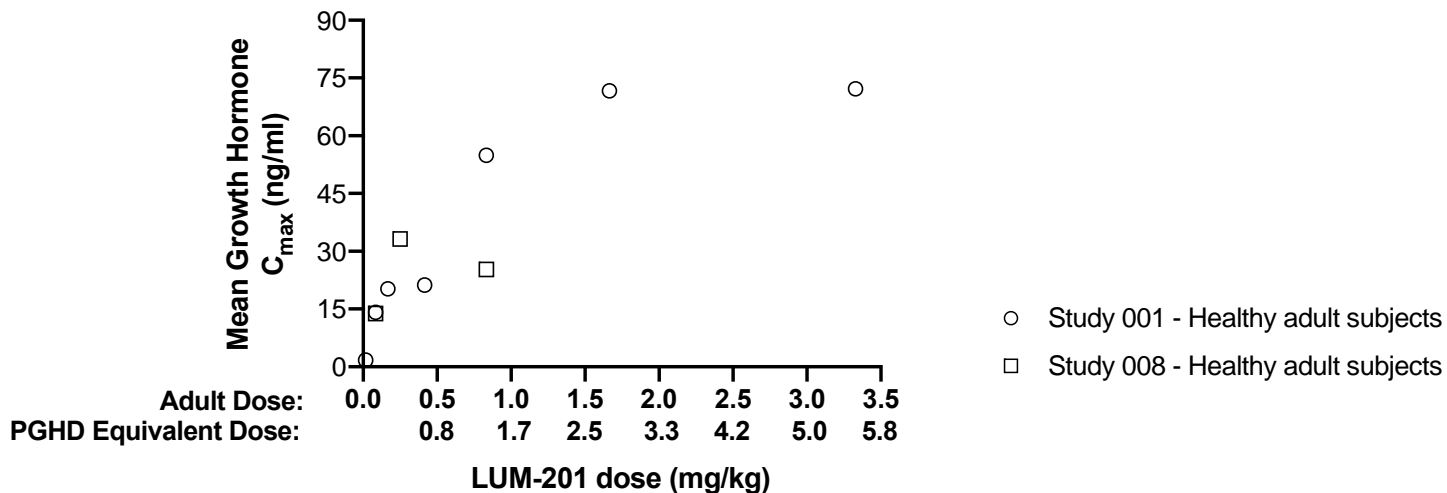
## Pharmacodynamics

- PD plateau possible  $\geq 2.8$  mg/kg PGHD dose equivalent\*



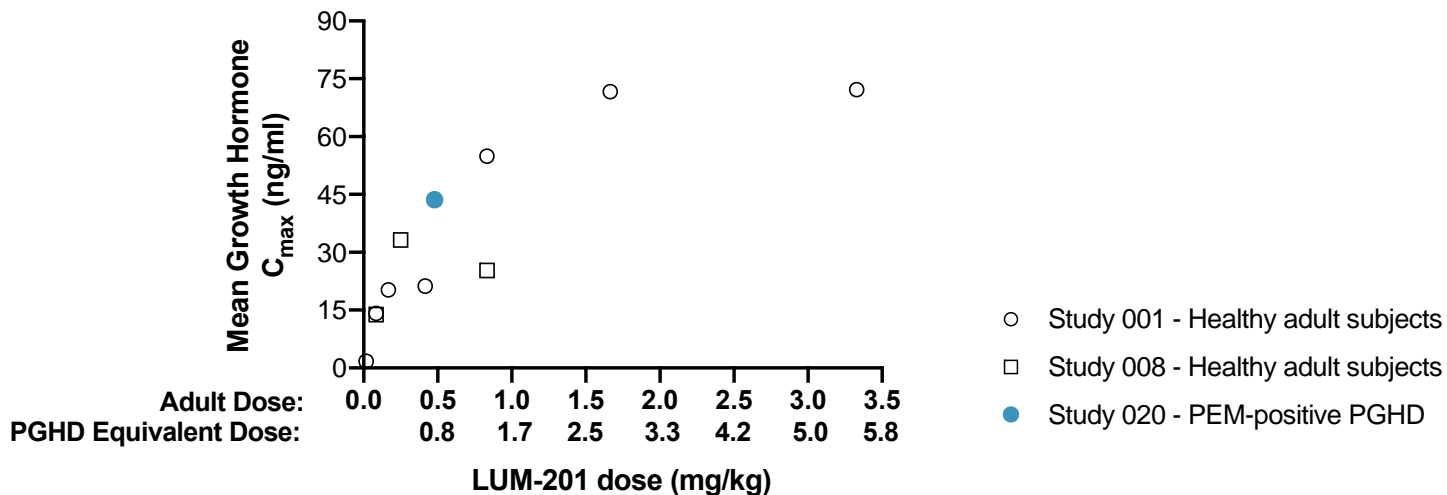
# GH Response to LUM-201 in NHV

- Growth hormone  $C_{\max}$  in response to single doses of LUM-201 in healthy adults can serve as a benchmark



# GH Response to LUM-201 in NHV and PGHD

- Doses >0.8 mg/kg likely needed to maximize primary PD response, GH release, to LUM-201 in PGHD



Anticipate 3.2 mg/kg to produce maximal pharmacodynamic effect

# Clinical Development Outline for PGHD

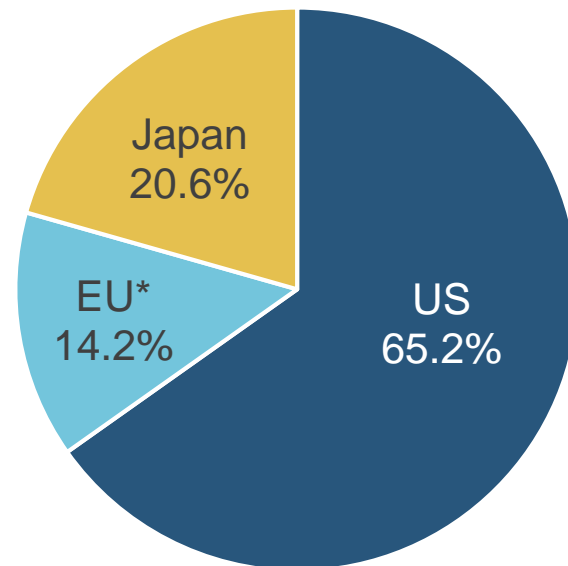
- Proposed Phase 2b PGHD clinical trial design
  - PEM-enabled patient selection
  - Three dose levels of LUM-201 (0.8, 1.6, 3.2 mg/kg)
  - Positive control arm of daily rhGH injections
  - 6-month dosing
  - 20 subjects per arm
  - Treatment-naïve, age-matched cohorts
  - Primary outcome measure: annualized growth height velocity
- Anticipate initiation of Phase 2b trial prior to the end of 2020

Optimal dose to be determined for Phase 3 study

# Pediatric Growth Hormone Deficiency Market Analysis

- Global rhGH sales for pediatric patients with growth hormone deficiency (PGHD) reached \$1.12 Billion in 2016 in major markets<sup>1</sup>
  - Expected CAGR for global PGHD sales is 3.5% leading to a projected market size of \$1.58 Billion<sup>1</sup> in 2026
  - US accounted for 65.2% of global sales of rhGH for PGHD in 2016

Global Sales Distribution of rhGH for PGHD in 2016 in the major markets<sup>1</sup>

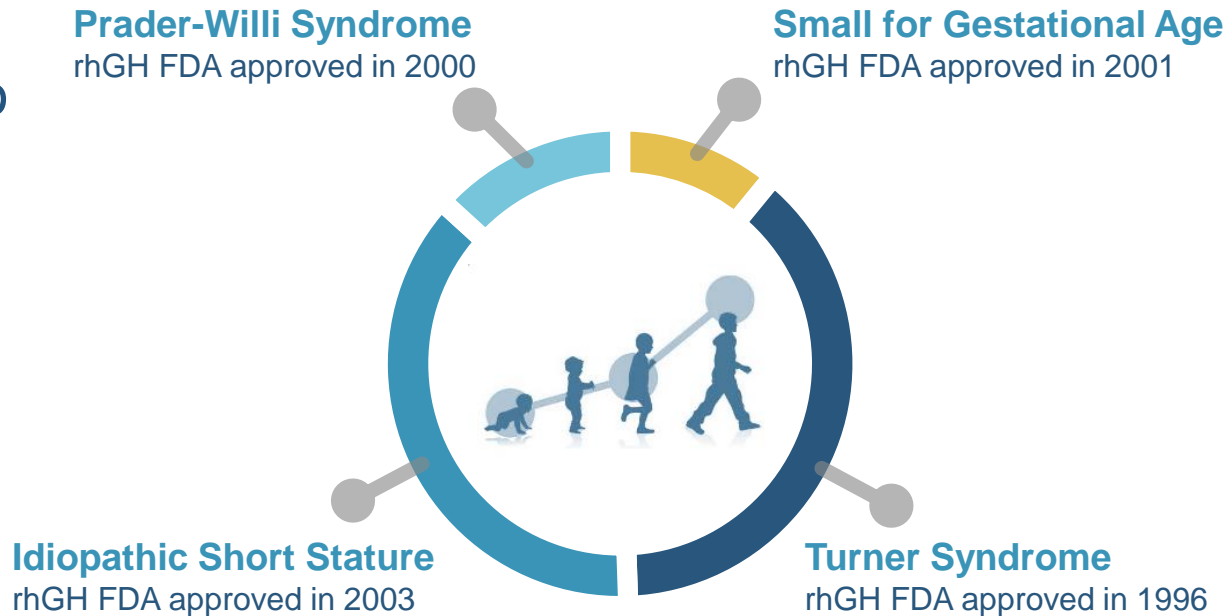


\* Germany, France, Italy, Spain, and UK

<sup>1</sup> Global Data Opportunity Analyzer: Growth Hormone Deficiency Opportunity Analysis and Forecasts to GDHC069POA, May 2017

# LUM-201: Other Potential Rare Endocrine Disorders

- Beyond PGHD, Lumos Pharma also plans to investigate LUM-201 for other rare endocrine disorders, for which rhGH has been approved



Significant opportunities with established regulatory pathways

# Orphan Designation and IP

- Orphan Drug Designation received in US and EU for GHD in 2017
  - With potential pediatric extensions, eligible for 12 years exclusivity in EU and 7.5 years in US.
  - Plan to seek designation in Japan
- Intellectual Property
  - “Detecting and Treating Growth Hormone Deficiency”
    - Use of LUM-201 in PGHD
    - US Patent issued with expiration in 2036
    - Patent applications filed in multiple other countries



# Secure Projected Cash Position

Metric	Position
Projected cash balance on March 31, 2020	In excess of \$80 million
Additional non-dilutive resources expected	Funds from monetization of 60% interest in value of PRV
Projected cash use per quarter through 2020	~ \$6.5 to \$7.5 million
Shares outstanding as of April 27, 2020 <sup>1</sup>	~ 8.29 million

Projected March 31, 2020 cash balance expected to be sufficient to fund the company through Phase 2b trial data read-out

# Lumos Pharma: Summary of Investment Thesis



- Lead program, LUM-201, with potential to be the first oral growth hormone secretagogue therapy for PGHD
- Opportunity to disrupt established and sizable market
- Management team with extensive experience in the clinical advancement of rare disease therapeutics
- Cash position sufficient to support LUM-201 program through Phase 2b trial read-out, with additional non-dilutive PRV funding available to expand portfolio

Potential to significantly increase shareholder value

# Supplemental Materials



# 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

# Importance of Pulsatility in Growth Hormone Release

- Physiological release of GH is **pulsatile**
- The unique basis of LUM-201 therapeutic intervention is to ***increase the amplitude of pulsatile GH secretion***

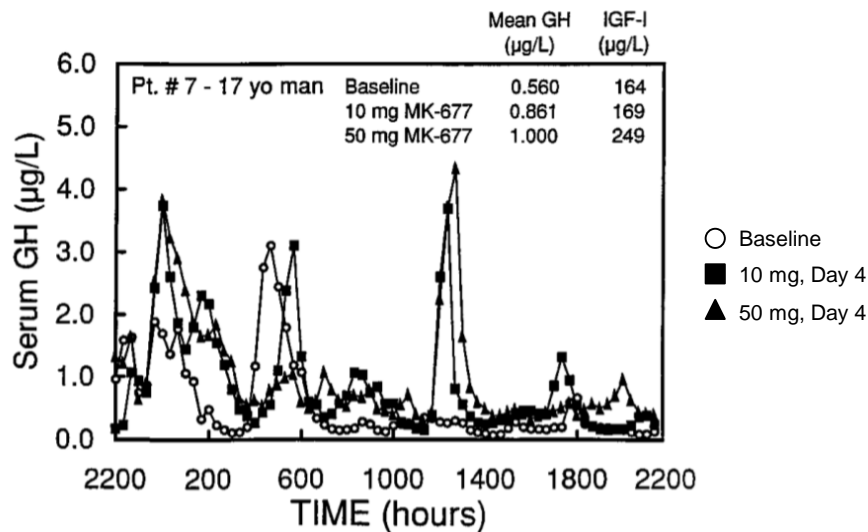
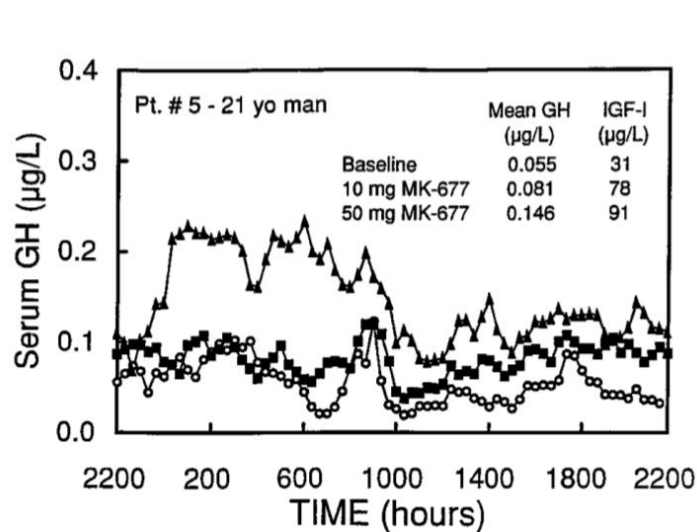


# GH Pulsatility and Connection to Growth

- Challenging to answer
  - Higher doses of rhGH do increase growth in PGHD patients
  - Difficult to directly compare continuous vs pulsatile dosing in patients
- In adults with GHD and healthy elderly, treatment with LUM-201 for up to one year increases the amplitude of GH released in endogenous pulses
- Evidence supports potential for improved growth with pulsatile GH
  - Experiments in rats show pulsatile dosing of GH promotes more growth than continuous dosing<sup>1</sup>
  - Higher frequency of rhGH has been shown to promote more growth than less frequent dosing in PGHD<sup>2</sup>

# LUM-201 Augments Pulsatility

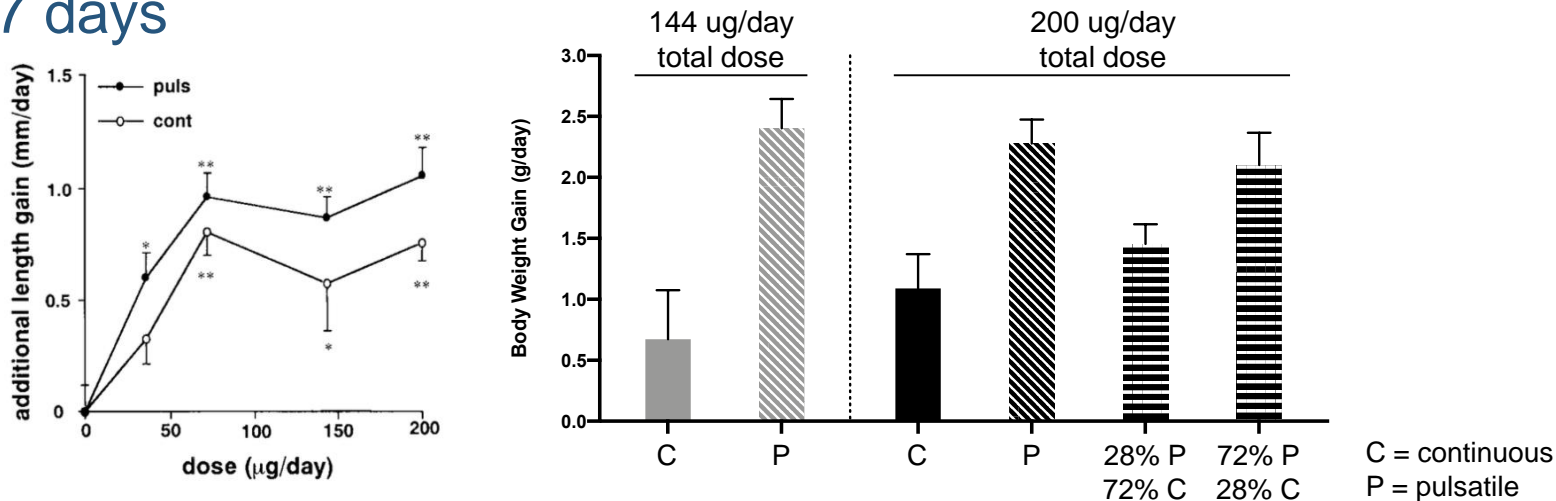
- Adults with GH deficiency
- Individual subjects
- Representative 24-hour GH profiles on Day 4 of treatment





# Pulsatility of GH Release is Important to Growth in Dwarf Rats

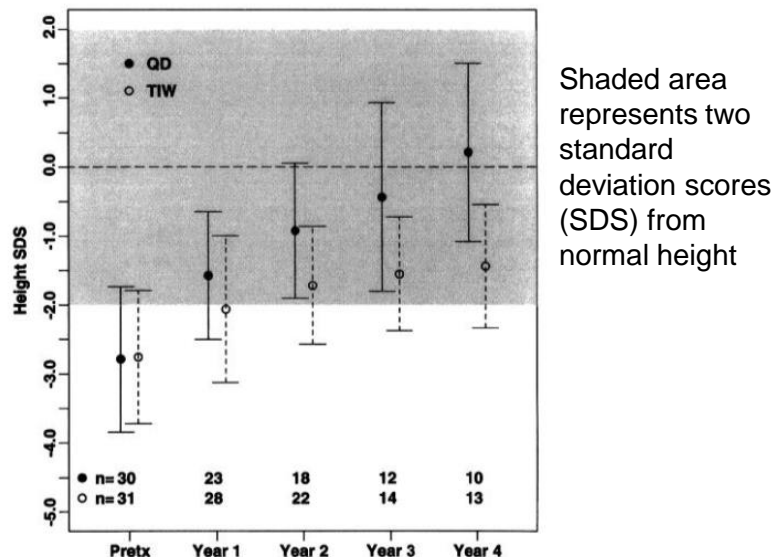
- Dwarf rats treated with continuous, pulsatile or combination rhGH for 7 days



Pulsatile administration of rhGH that mimics natural secretion of GH is more effective in stimulating growth than continuous administration

# Increasing Frequency of rhGH Administration Improves Growth

- PGHD
- Same total weekly dose of rhGH: 0.3 mg/kg/week

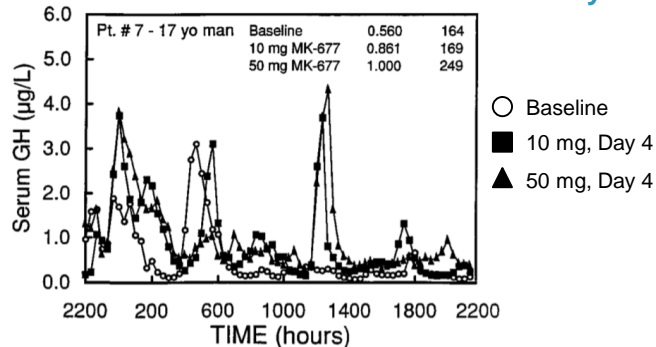


Higher frequency of rhGH administration in PGHD is more effective in stimulating growth than less frequent administration

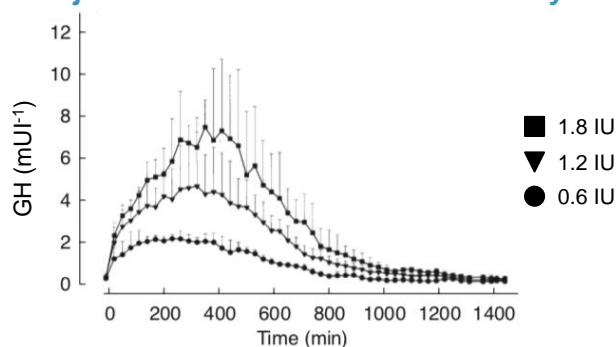
# LUM-201 Augments Pulsatility in GHD Adults

- LUM-201 augments endogenous GH pulses
- rhGH is administered as single, daily bolus doses

24h GH profile following oral LUM-201 administration in an adult with GH deficiency<sup>1</sup>

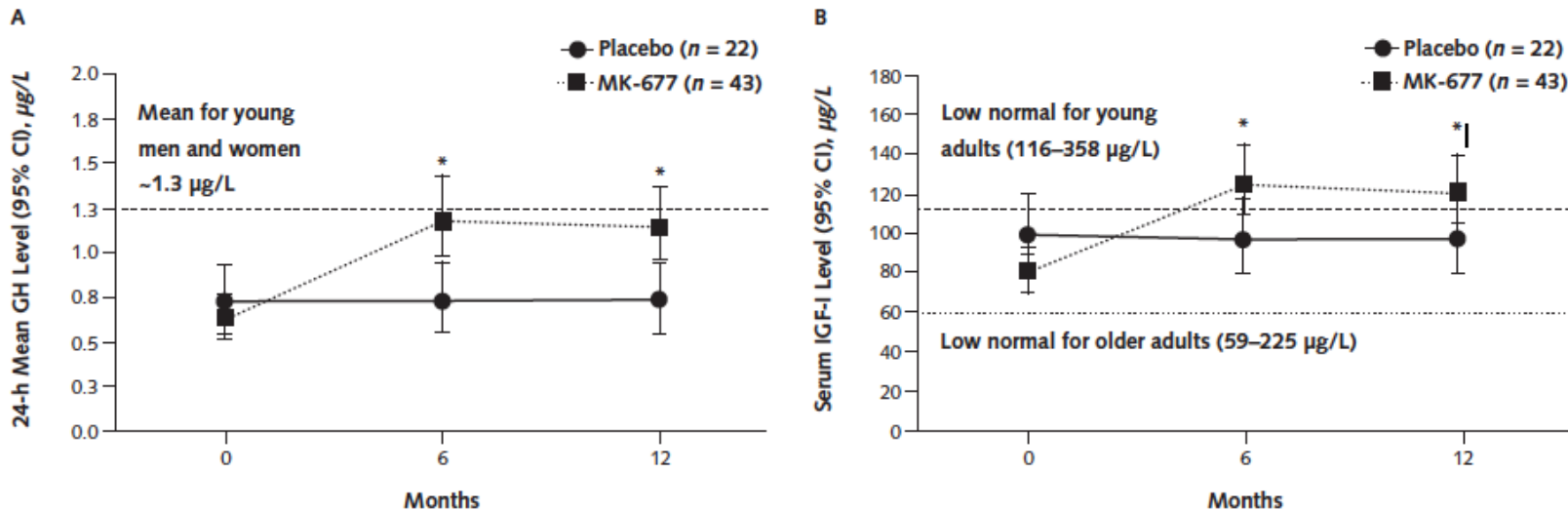


24h PK profile following subcutaneous rhGH injection in adults with GH deficiency<sup>2</sup>



Unlike bolus exposure through rhGH injection, GH exposure stimulated by LUM-201 mimics physiological pulsatility

# LUM-201 Effects Are Durable In Healthy Elderly



LUM-201-mediated increases in serum GH and IGF-1 are sustained over one year of treatment