



# Corporate Presentation

April 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 combined 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-10 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 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 mid-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, and UK

\* Global Data Opportunity Analyzer: Growth Hormone Deficiency Opportunity Analysis and Forecasts to GDHC069POA Published: May 2017

# SIGNIFICANT HIGH-QUALITY INVESTORS

Notable investors in Lumos Pharma, Inc.

- ▶ Well-known healthcare venture investors:

NEA®



- ▶ Large healthcare investors that participate in public and private markets:

**Blackstone**

**LION POINT**  
CAPITAL

**DEERFIELD**

- ▶ Large pharma representatives:



- ▶ Significant long-term investor:





# EXPERIENCED MANAGEMENT



Richard Hawkins  
Chairman, CEO & President



John McKew, PhD  
COO & CSO



Carl Langren  
CFO



Eugene Kennedy, MD  
CMO

## 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.

# STRATEGIC PRIORITIES

Initial focus will be on initiation of Phase 2b trial of LUM-201 (ibutamoren) for pediatric growth hormone deficiency (PGHD)

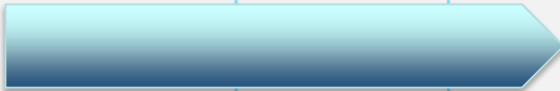


Build pipeline through strategic acquisitions of assets focusing on rare diseases

## **LUM-201: Oral secretagogue candidate for PGHD**

- ▶ Established regulatory path – planned Phase 2b expected to start mid-2020
- ▶ Potential to address 50-60%<sup>1</sup> of total PGHD patients
- ▶ Significant market opportunity, well-proven value through industry peers

1. Lumos Pharma data on file

# LUM-201 PROGRAM PIPELINE

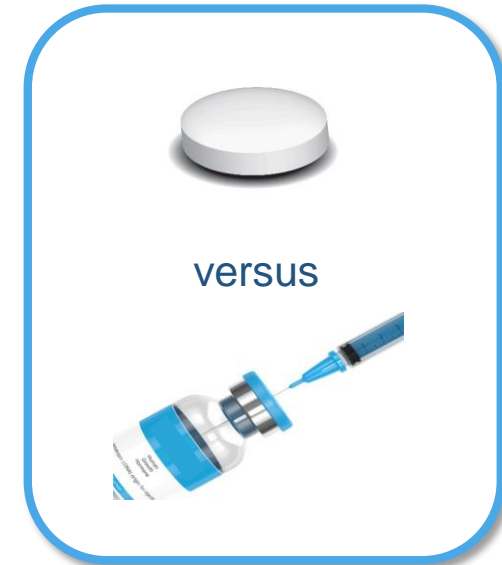
Product Candidate	Orphan Indication	Preclinical	Phase I	Phase II	Phase III	Status
<b>Ibutamoren (LUM-201)</b>	Pediatric Growth Hormone Deficiency (PGHD)					Phase 2b – expected to initiate mid-2020
	Turner Syndrome					Ongoing clinical planning for Phase 2 trial, timing dependent on PGHD data
	Children 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 the 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

1. GlobalData EpiCast Report for Growth Hormone Deficiency Epidemiology forecast to 2026

2. Rosenfeld, R., et al *Compliance and Persistence in Pediatric and Adult Patients Receiving Growth Hormone Therapy*, Endocrine Practice, 2008, 14(2), 143-154

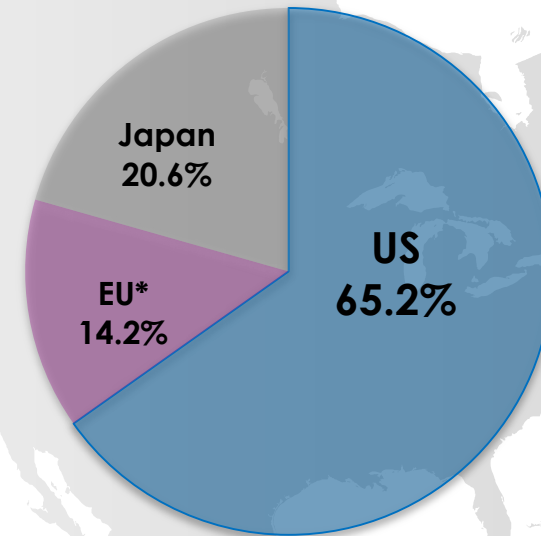
3. Cutfield, W. S. et al *Non-Compliance with Growth Hormone Treatment in Children Is Common and Impairs Linear Growth*, PLOS ONE 6(1): e16223. <https://doi.org/10.1371/journal.pone.0016223>

# 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

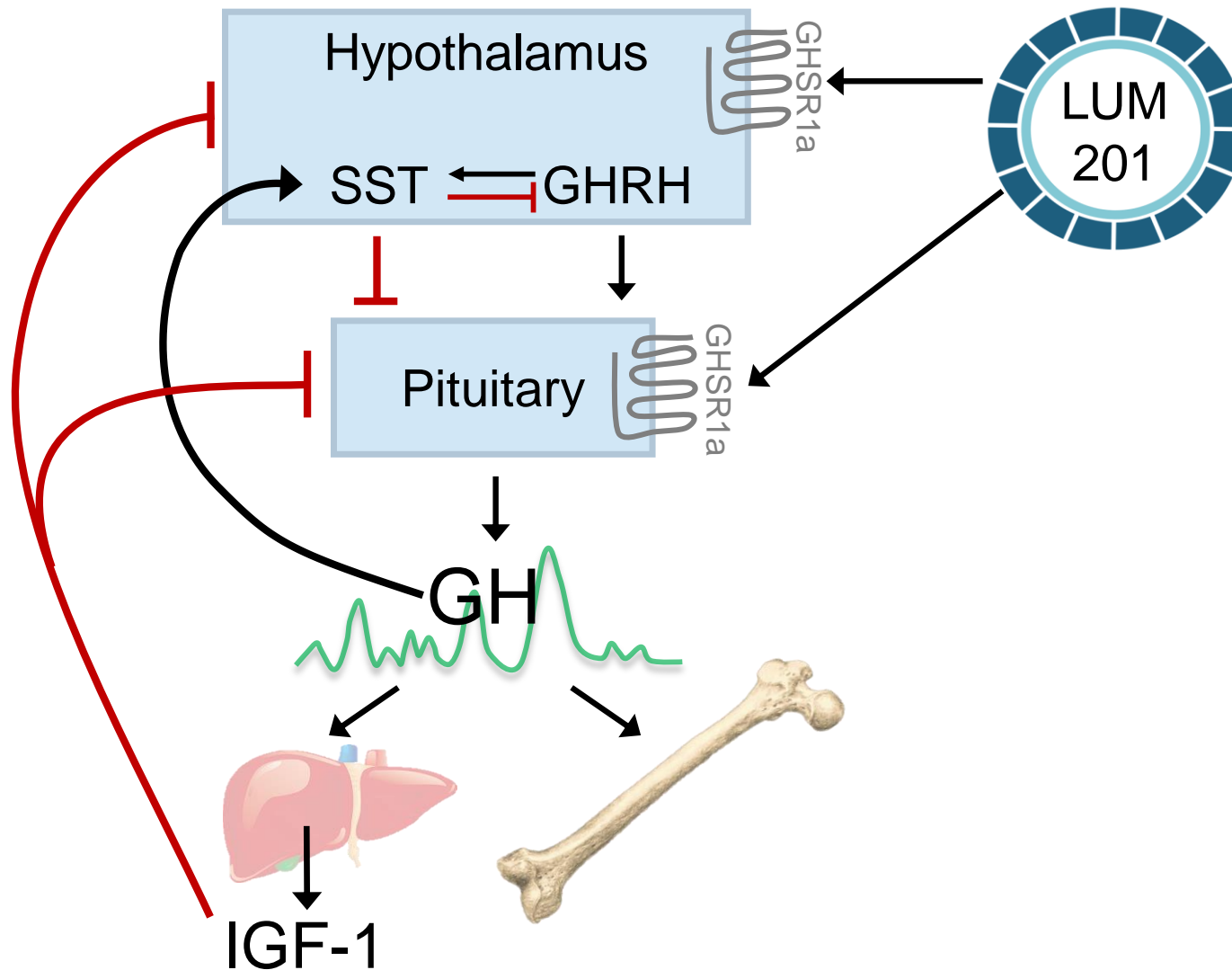
**Global Sales Distribution of rhGH for PGHD in 2016 in the major markets<sup>1</sup>**



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

1. Global Data Opportunity Analyzer: Growth Hormone Deficiency Opportunity Analysis and Forecasts to GDHC069POA Published: May 2017

# 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

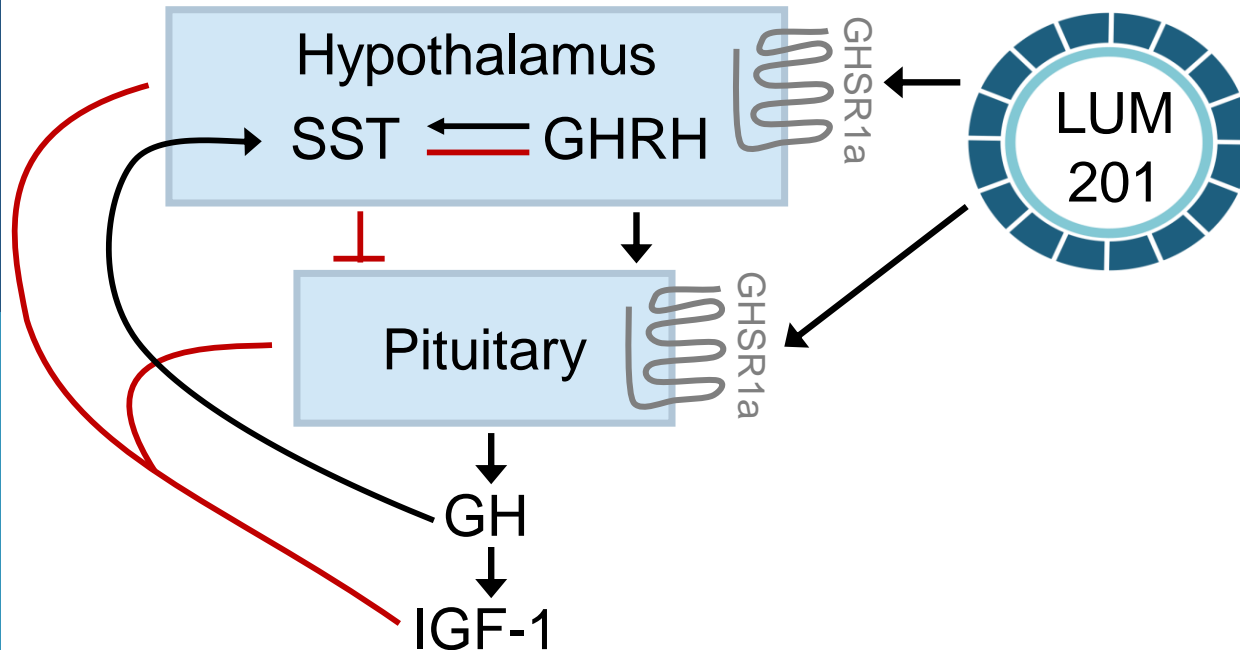
1. Howard 1996 Science  
2. Nass 2008 Ann Intern Med  
3. Chapman 1997 J Clin Endocrinol Metab

SST = somatostatin  
IGF-1 = insulin-like growth factor-1

GHRH = growth hormone-releasing hormone  
GHSR1a = GH secretagogue receptor 1a

# TARGETED PGHD POPULATION

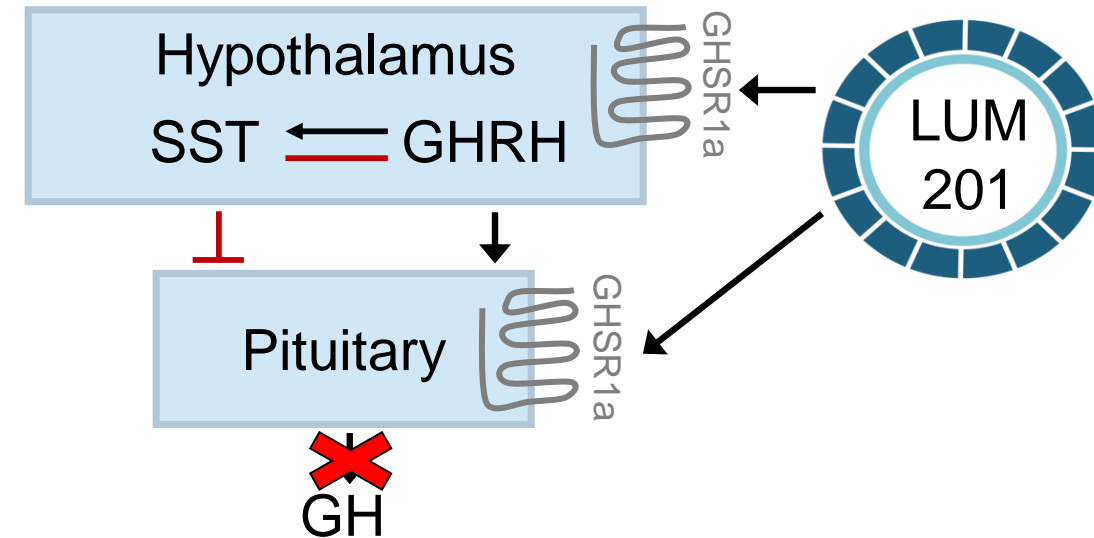
## PEM-Positive: Included



### 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 (PEM)

- ▶ GH response to a single LUM-201 dose and baseline IGF-1 has the potential to distinguish these two populations

<sup>1</sup>HP-GH = hypothalamic-pituitary-growth hormone

1. Lumos Pharma data on file

# PRIOR CLINICAL EXPERIENCE IN PGHD WITH LUM-201

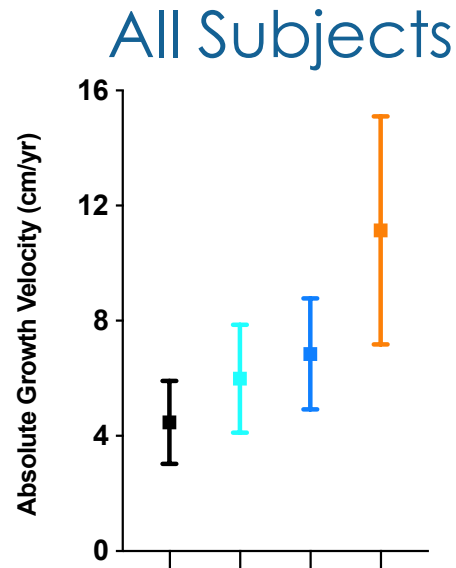
- ▶ Prior PGHD trials
    - ▶ Conducted prior to Lumos acquisition of LUM-201 in July 2018
    - ▶ 3 clinical trials in pediatric population explored safety and efficacy
      - ▶ Phase 1 Trial 019 – PK, Phase 2 Trial 020 – Naïve, Phase 2 Trial 024 – Previously rhGH treated
    - ▶ No significant safety concerns were identified
    - ▶ Formulation change midway through Trial 020 reduced bioavailability of the drug and confounded data
    - ▶ Phase 2 trials were discontinued after interim analysis of Trial 024
  - ▶ Scientifically-driven post-hoc analysis enabled (Trial 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 a higher dose



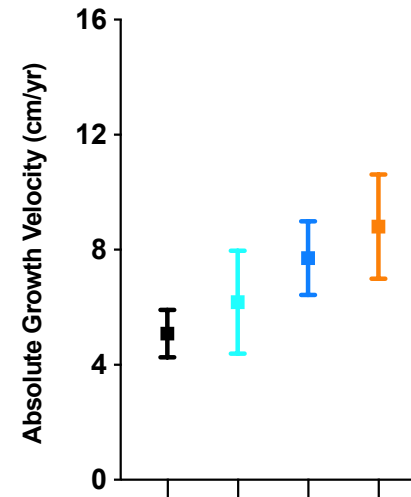
# POST-HOC: PREDICTIVE ENRICHMENT MARKERS AT WORK

## Absolute Growth

means and SD



## PEM-Positive

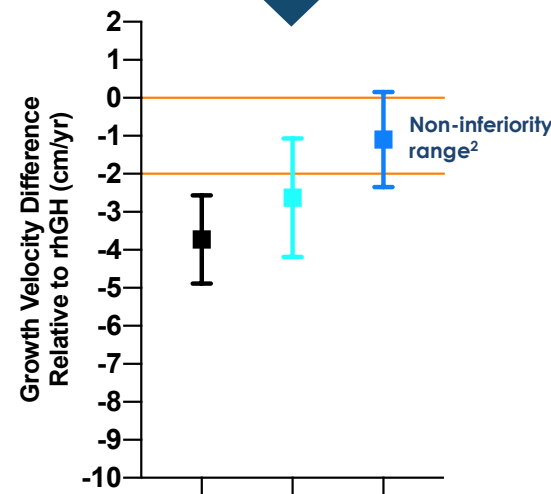
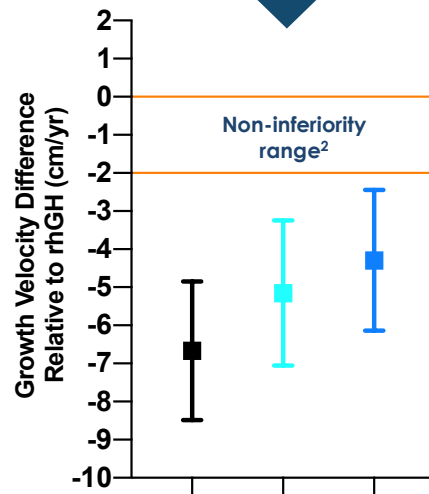


- ▶ Naïve PGHD patients, 020 trial
  - ▶ Data from first 6 months prior to formulation change<sup>1</sup>
- ▶ LUM-201 0.8 mg/kg not statistically different from rhGH but insufficient to meet FDA requirements for non-inferiority<sup>2</sup>
- ▶ Lumos expects **prospective application of PEMs** and **higher doses** to improve response and meet statistical non-inferiority margin

## Relative Growth: non-inferiority analysis

[growth velocity in rhGH minus growth velocity in test group]

means of the difference and 95% CI



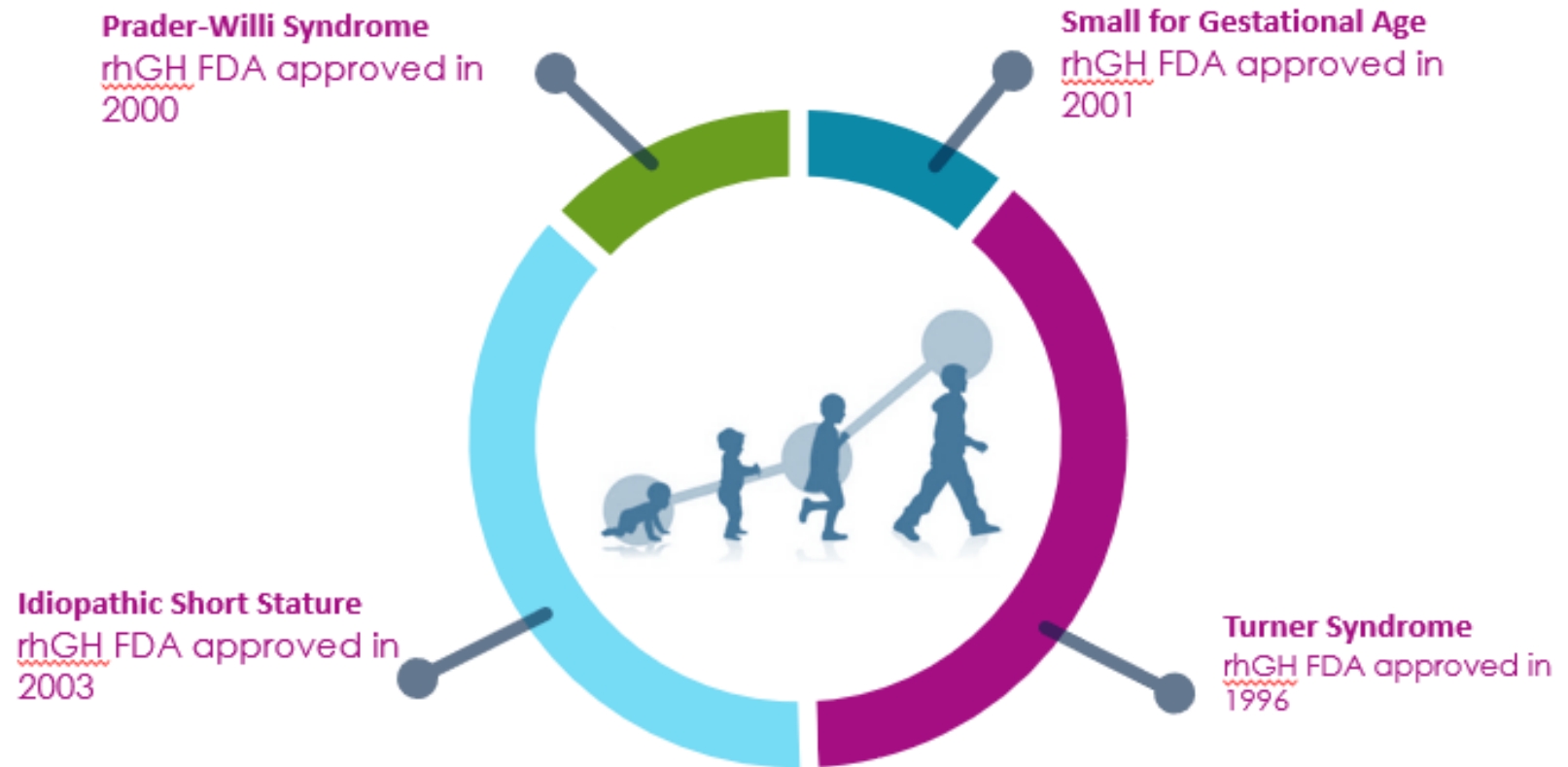
- Placebo
- LUM-201 0.4 mg/kg/d
- LUM-201 0.8 mg/kg/d
- rhGH 42 mcg/kg/d

# 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 mid-2020
- ▶ Optimal dose to be determined for Phase 3 study

# 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

<b>Projected cash balance on March 31, 2020</b>	<b>In excess of \$80 million</b>
<b>Additional non-dilutive resources expected</b>	<b>Funds from monetization of 60% interest in value of PRV</b>
<b>Projected cash use per quarter through 2020</b>	<b>~ \$6.5 to \$7.5 million</b>
<b>Shares outstanding as of March 18, 2020</b>	<b>~ 8.26 million</b>

- ▶ 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