Pediatric Growth Hormone Deficiency and LUM-201

KOL Event

April 27, 2021

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 potential of an orally administered LUM-201 treatment regimen for PGHD and other indications, the projected cash position and its sufficiency to fund the company's operations through data read-out for the OraGrowtH210 Trial in PGHD; expected initiation of the OraGrowtH212 Trial of LUM-201 in PGHD in Q2 2021; 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; milestones or other economic interests, Lumos Pharma's financial guidance for 2021 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 or 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, 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. 4.14.2021

Agenda

	Lumos Pharma Corporate Overview – *Rick Hawkins, CEO*
	The Future of Therapy for Growth Hormone Deficiency – *Bradley S. Miller, MD, PhD*
	Oral Growth Hormone Secretagogue, LUM-201, Pulsatile Effect – *Fernando Cassorla, MD*
	Questions & Answers

Investment Highlights

Late-stage Rare Disease Asset	• Novel oral therapeutic asset, LUM-201 • Phase 2b in Pediatric Growth Hormone Deficiency (PGHD) – expect data mid-2022 • PK/PD study in PGHD – initiation expected Q2 2021
Large Markets	• Current market for initial indication alone is $1.2 billion* • Potential to disrupt injectable treatment regimen for significant subset of patients • Multiple potential follow-on indications representing up to an additional $2.2 billion
Experienced Management	• Established track record of performance in rare disease drug development • Business development acumen with expertise in licensing pipeline assets
Solid Cash Position	• Cash balance of $98.7M Q4 2020 + $26.0M received in January (PRV proceeds) • Cash runway through P2b OraGrowtH210 Trial readout expected mid-2022 • Balance sheet flexibility to acquire additional assets
Pipeline Expansion	• Multiple follow-on indications under consideration • Actively seeking additional rare disease assets to license or acquire

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

Management – Significant Clinical Development and Commercial Experience

Richard Hawkins Chairman, CEO & President

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). Co-founded Pharmaco, a contract research organization (merged with PPD).

John McKew, PhD COO & CSO

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

Former CFO of BioProtection Systems, Housby Mixer Group, Equity Dynamics, Inc., and Tax Manager with McGladrey Pullen & Co.

Lori Lawley, CPA Senior VP, Finance

Former Senior Manager with Ernst and Young with a focus on public and private companies in the biotech, manufacturing and technology industries.

Aaron Schuchart, MBA CBO

Former CBO of Aeglea BioTherapeutics, former leadership roles in business development and licensing at Coherus Biosciences, Novartis Diagnostics/Grifols, and Amgen.

Bob Davis, PharmD VP, Clinical Operations

Responsible for developing global development strategy and execution at Sensus for GH Receptor Antagonist for Acromegaly (approved in US, Europe, Japan), diabetes, and diabetes retinopathy. Previously, Executive Director at Pharmaco; Senior Director at Searle, Senior Scientist at Parke-Davis.

Key Opinion Leaders

University of Minnesota
Masonic Children's Hospital

Bradley S. Miller, MD, PhD

Dr. Bradley S. Miller[1] is currently Professor, Department of Pediatrics and Faculty Member, Division Director, Division of Pediatric Endocrinology, at the University of Minnesota Medical School. He is a practicing pediatric endocrinologist and published research investigator with an interest in the role of the GH/IGF system on normal and abnormal growth in children. His other area of interest includes the growth and development of children following adversity such as cancer and cancer therapy, fetal alcohol exposure, and international adoption. Dr. Miller received his MD and PhD from the Medical University of South Carolina, Charleston. He completed his residency and fellowship in pediatrics and pediatric endocrinology, respectively, at the Mayo Clinic. Dr. Miller has received numerous awards and recognition throughout his medical training and career and is actively involved with the MAGIC Foundation for Children's Growth, the global leader in endocrine health, advocacy, education, and support.

Institute of Maternal and
Child Research

Fernando Cassorla, MD

Dr. Fernando Cassorla[2] is currently Chief of Pediatric Endocrinology at the Institute of Maternal and Child Research of the University of Chile, a position he has held since 1993. Previously, Dr. Cassorla served as Senior Investigator at the Developmental Endocrinology Branch of the National Institute of Child Health and Human Development, rising to the position of Clinical Director of this Institute in 1990. He has authored numerous chapters in pediatric endocrinology, authored or co-authored over 200 original articles in peer reviewed journals, and has presented over 300 abstracts at scientific meetings. Dr. Cassorla received his MD from the University of Chile. He is Board Certified in both Pediatrics and Pediatric Endocrinology, having completed his pediatric residency at the Albany Medical Center in New York and his fellowship in Pediatric Endocrinology at the Children´s Hospital of Philadelphia. Dr. Cassorla has received several international awards for his work and was elected to the Chilean Academy of Medicine for a lifetime position in 2003.

[1] Dr. Bradley S. Miller is an investigator for the OraGrowthH210 Trial, Phase 2b study of LUM-201 in pediatric growth hormone deficiency (PGHD).
[2] Dr. Fernando Cassorla is the principal investigator for the OraGrowthH212 Trial, a Pharmacokinetic/Pharmacodynamic study of LUM-201 in PGHD.

The Future of Therapy for Growth Hormone Deficiency

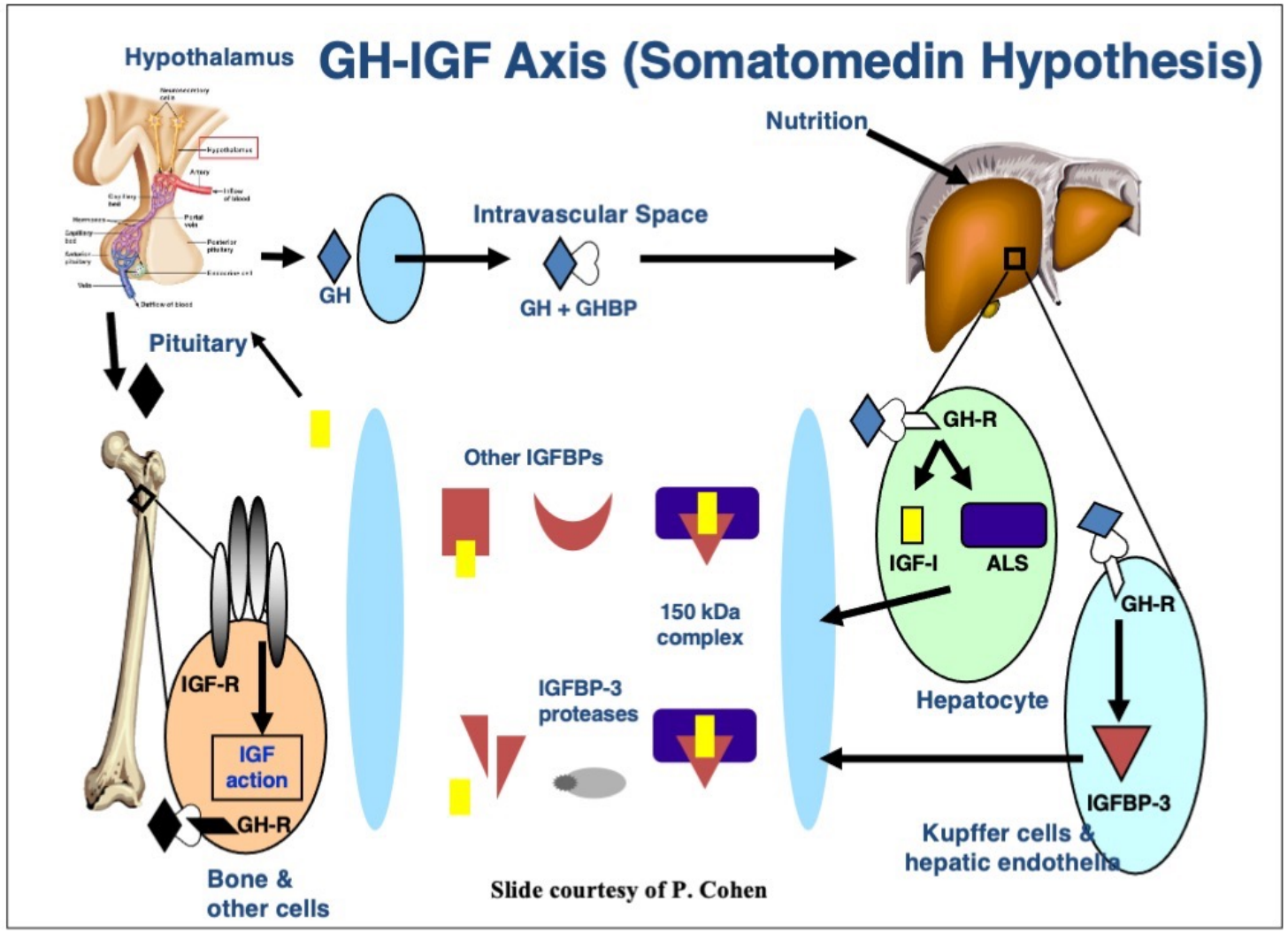
April 27, 2021

Bradley S. Miller, MD, PhD
Professor and Director
Division of Endocrinology
Department of Pediatrics
University of Minnesota Masonic Children's Hospital

University of Minnesota
Masonic Children's Hospital

Disclosure

- Dr. Miller is a consultant for Abbvie, Ascendis, BioMarin, Novo Nordisk, Orchard, Pfizer, Sandoz, Sanofi Genzyme, Tolmar and Vertice and has received research support from Alexion, Abbvie, Amgen, Ascendis, Lumos Pharma, Novo Nordisk, Opko, Pfizer, Sandoz, and Tolmar.
- I will be discussing off-label use of medications since these medications are being developed. LUM-201 is an investigational compound and is not approved by the FDA or any other regulatory agency.
- Some of the slides in this presentation are derived or copied from corporate presentations previously given by Lumos Pharma, Inc. These slides are used with permission.



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Growth Hormone Deficiency

- Growth Failure
- Decreased Pituitary Growth Hormone Production
- Low GH causes Low IGF-1

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Growth Hormone Deficiency

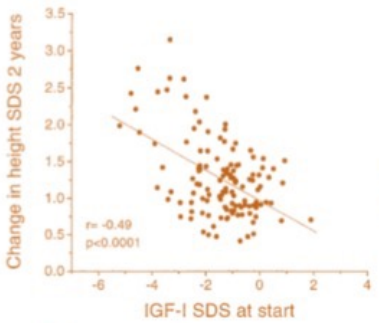
- Incidence: 1 in 3500
- Etiology
  - Idiopathic (majority)
  - Organic (congenital, acquired)

Lindsay R, et al, J Pediatr.125:29, 1994.

Growth Hormone Deficiency Patients Have a Range of Secretion Insufficiency

- Well established in the literature:
  - A wide range of severity in GHD[1]
  - Variability in responses to GH therapy
  - More severely deficient patients respond better than partially deficient[1]
- Several prediction models attempt to explain variability and optimize GH treatment[2]
  - Multiple factors may contribute
  - GH response to standard stimulation tests is most important predictor of first year growth response to rhGH in PGHD in one analysis[3]
  - Inclusion of baseline IGF-1 strengthened model[4]
- Recent publications
  - Baseline IGF-1 and GH response to standard stimulations tests are independent predictors of growth when patients are treated with rhGH[5]
  - Moderate GHD represents ~60% of total PGHD population[5]

Differential rhGH response according to GHD severity



…as defined by baseline IGF-1

…and as defined by GH response to standard stim tests

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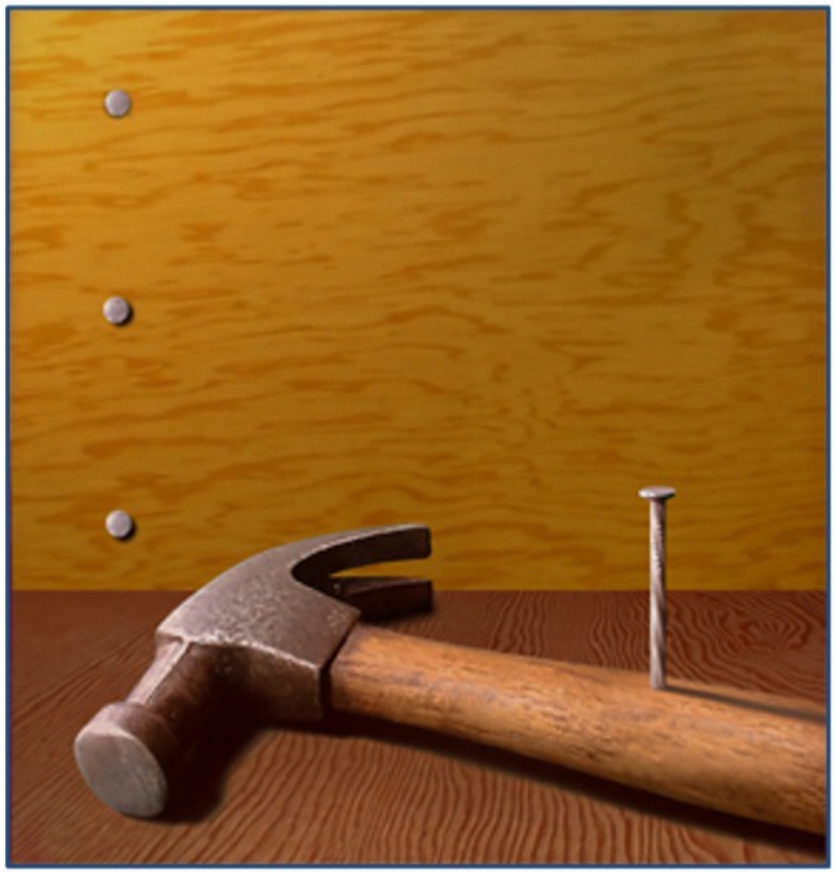
1 Tanner 1971 Arch Dis Childhood
2 Wit 2013 Hormone Res Paed
3 Ranke 1999 JCEM
4 Kristrom 1997 JCEM
5 Blum 2021 JES

Daily Growth Hormone



FDA Approval:

Children:

Growth Hormone Deficiency
Turner Syndrome
Small for Gestational Age
Chronic Renal Insufficiency
Prader Willi Syndrome
Idiopathic Short Stature
SHOX Deficiency
Noonan Syndrome

Adults:

Growth Hormone Deficiency
Aids Wasting
Short Bowel Syndrome

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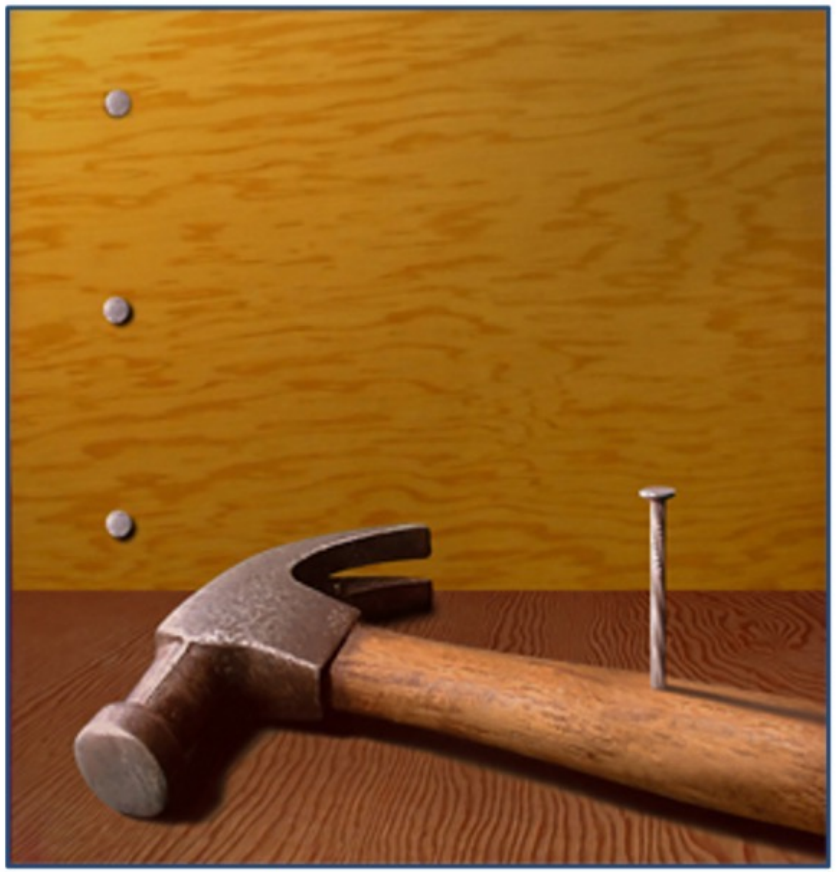
Daily Growth Hormone



Pros:

Overall Safe

- >500,000 patient years safety data (long-term data still needed)

Adult Height near mid-parental target height in children with GHD

Established Short-Term Efficacy in Adults

Cons:

Daily Injection

Cost

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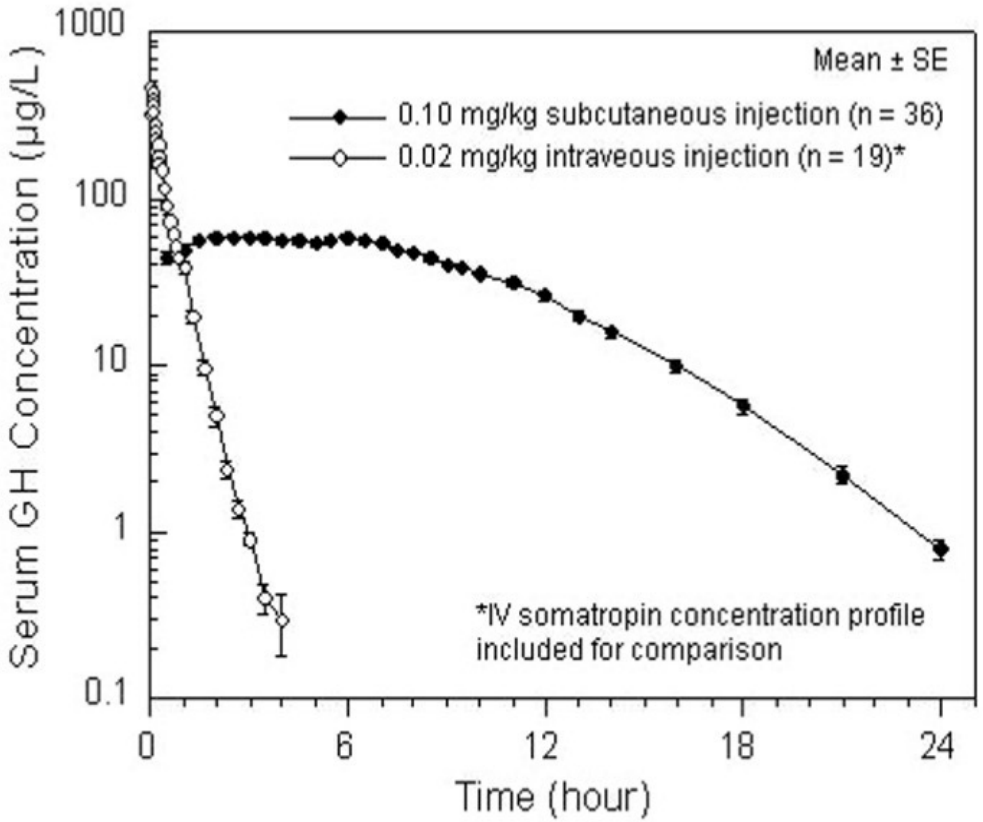
How Long does GH last?

- Clearance from circulation
  - Glomerular filtration/renal metabolism
  - Receptor-mediated uptake
  - Extracellular proteases?
- Half life after IV injection: **14-19 minutes**
- Half life after endogenous pulse: **25 minutes**
- Half life after SQ injection: **2.1 hours**



http://www.drugs.com/pro/nutropin.html
Holl RW, et al. J Clin Endocrinol Metab, 77:216, 1993.

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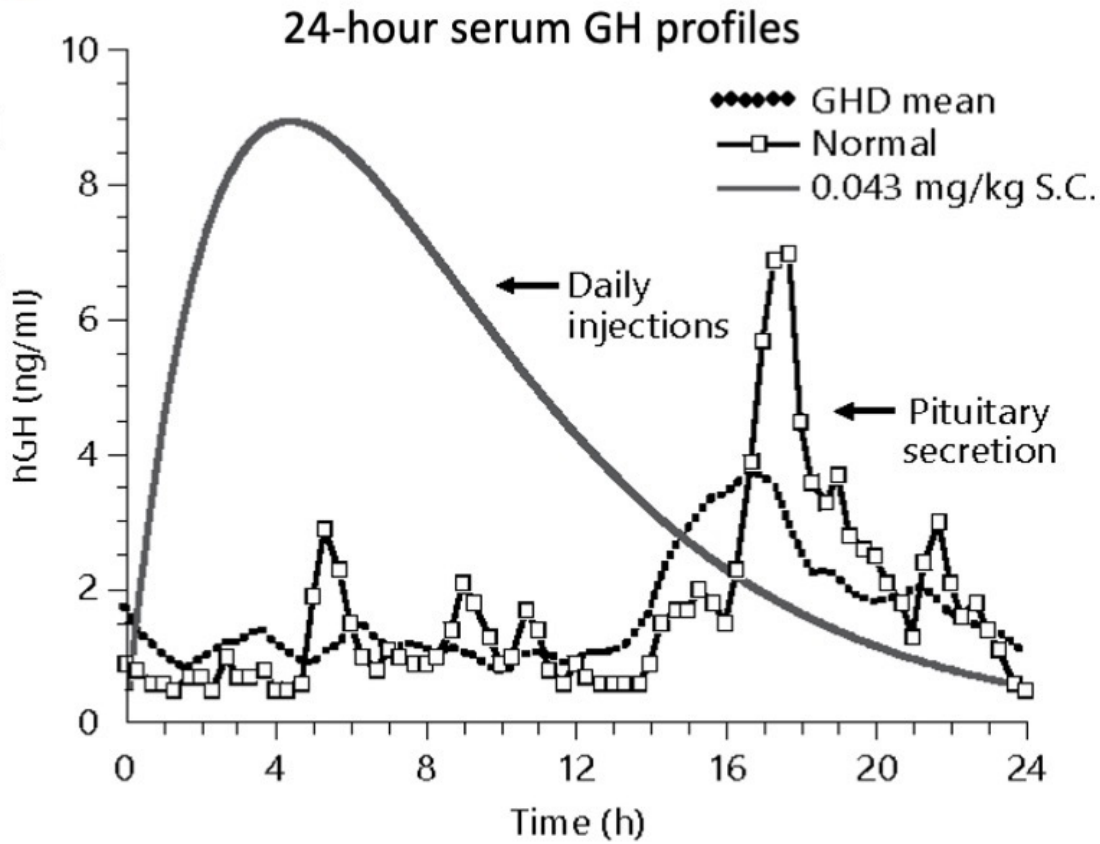
- **Daily GH Non-physiologic**



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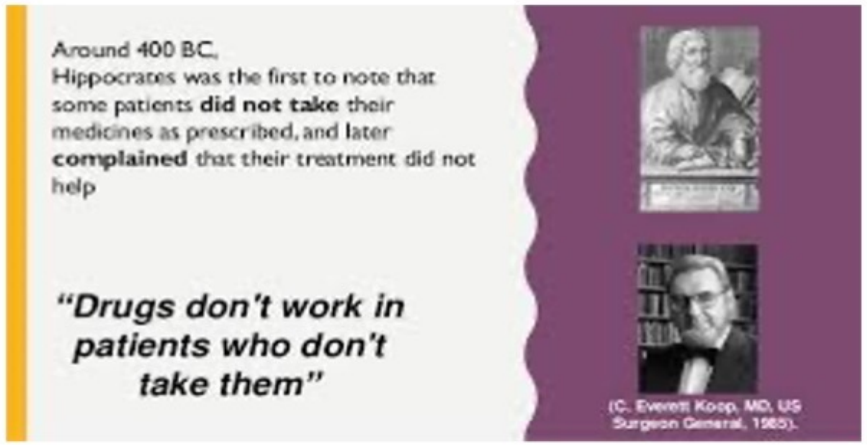
http://www.drugs.com/pro/nutropin.html
Holl RW, et al. J Clin Endocrinol Metab, 77:216, 1993.

Challenges with Daily GH Therapy

- Potential for Improvement
  - Adherence
  - Injection Frequency/Pain



	United States[1]	New Zealand[2]	Spain[3]
Patients, n	609	177	158
Data source	GHMonitor[SM] Registry	National survey	Observation longitudinal study
Noncompliance	10% missed > 15 injections/month (more than half)	66% missed > 1 injection/week	Moderate-to-poor adherence in 33.5% of patients
Effect of noncompliance on growth	HV 67% of that achieved by patients missing fewer injections (p=0.03)	Reduction in HV SDS (p<0.01)	Lower height SDS (p=0.002) Lower IGF-I SDS (p<0.0001)

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1. Desrosiers P, et al. *Ped Endrocrinol Rev* 2:327, 2005.
2. Cutfield WS, et al. *PLoS ONE* 6:e16223, 2011.
3. De Pedro S, et al. *Growth Horm IGF Res* 26:32, 2016.
4. Rosenfeld RG, Bakker B. Endocrine Prac. 14:143, 2008.

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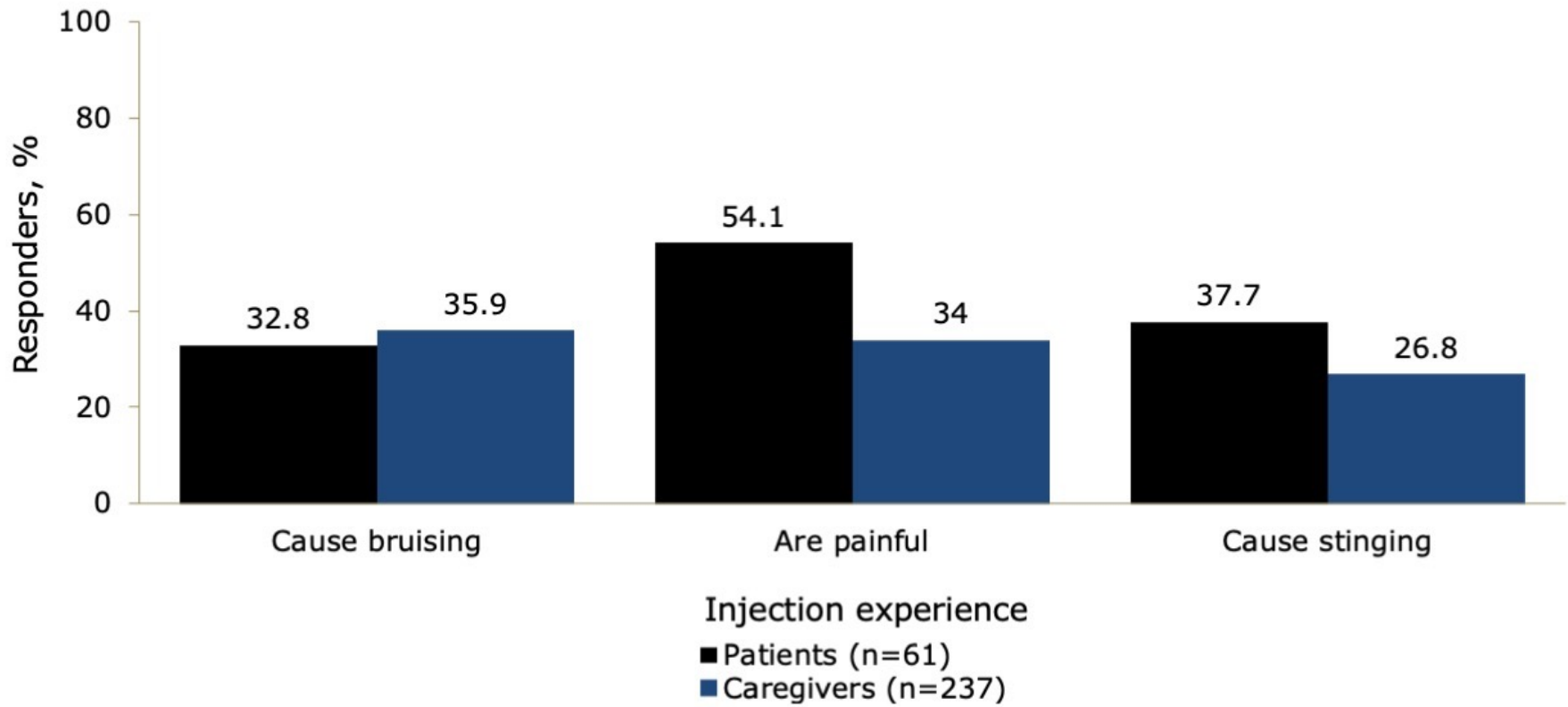
Administration burden of daily GH therapy



Kremidas D, et al. J Ped Nurs 2013;28:55–63

Long-Acting GH Therapies

Will LAGH be Superior to Daily rhGH?

Daily rhGH

Inconvenient, painful and distressing daily injections

Lack of adherence

Reduced efficacy and increased healthcare costs

- **LAGH may increase overall treatment outcomes via *improved adherence, superior serum IGF-1 levels and superior metabolic actions.***
- **Non-physiologic profile may be beneficial *but could have different side effects.***

GenSci 2020 Pediatric Endocrinology Meeting: "Long-Acting Growth Hormone Preparations – Current Status and Future Considerations." Hangzhou, China, 1/20.

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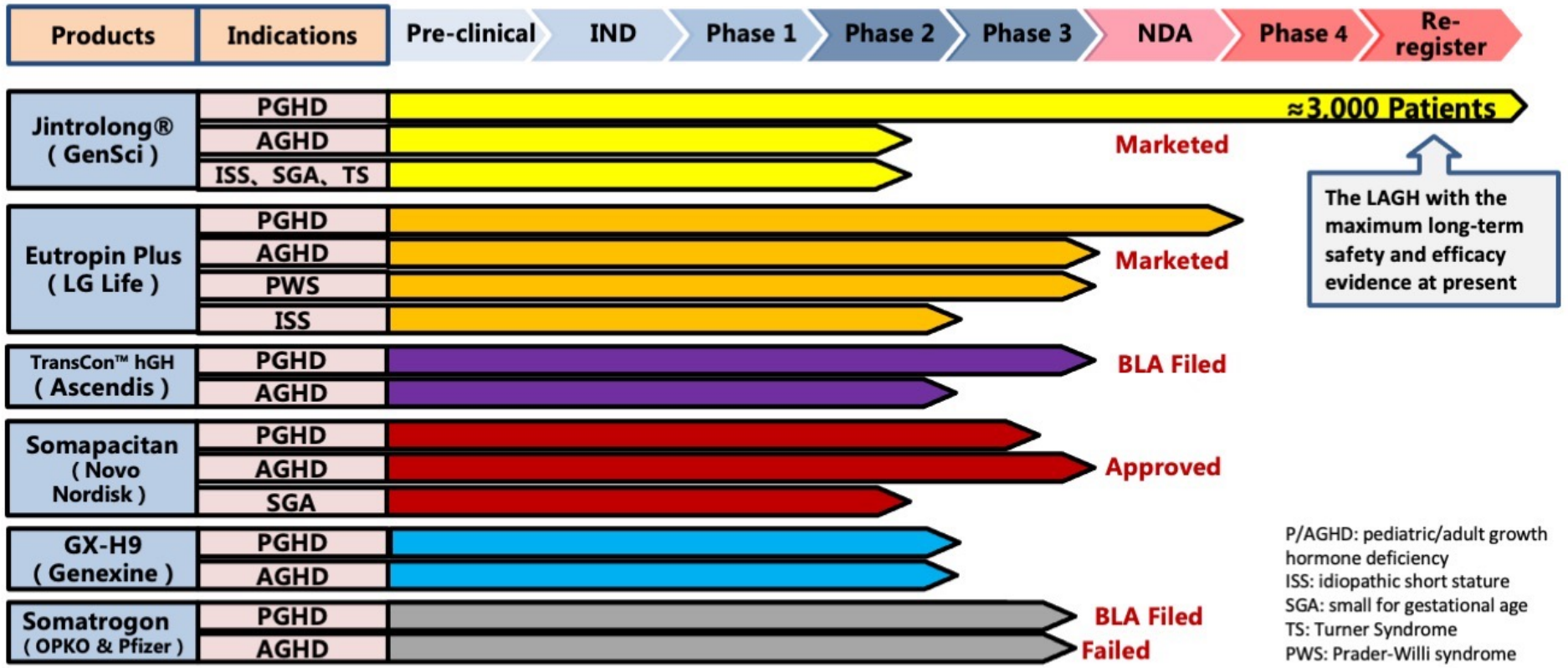
Overview of LAGH Preparations in Development



Adapted from: GenSci 2020 Pediatric Endocrinology Meeting: "Long-Acting Growth Hormone Preparations – Current Status and Future Considerations." Hangzhou, China, 1/20.

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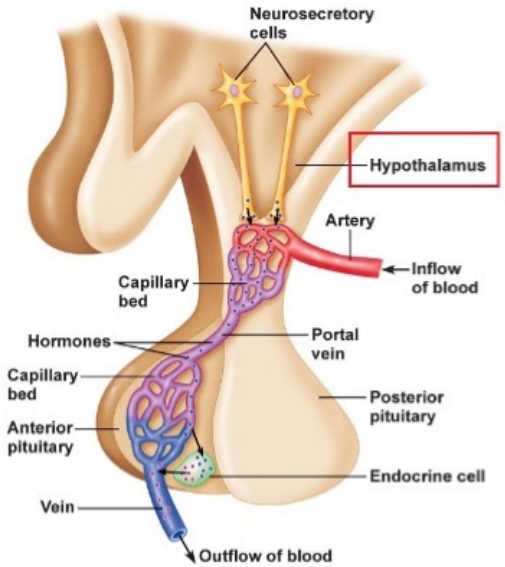
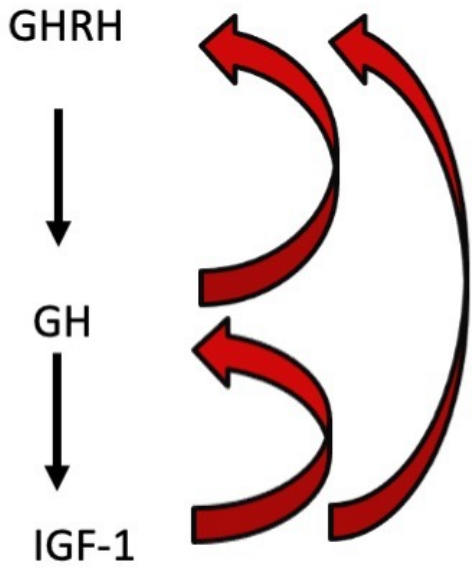
GH Secretagogue

- Molecules that stimulate release of Growth Hormone Releasing Hormone (GHRH), GH or both.





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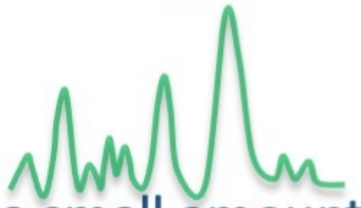
The Spectrum of GH Deficiency and the Importance of Pulsatility

Growth Hormone (GH) is naturally released in the body in a pulsatile fashion



For children who have the ability to secrete a small amount of GH on their own, one potential approach to PGHD therapy is to ***increase the patients' own "waves" of pulsatile GH secretion.***

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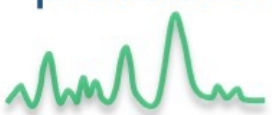
Reference: Lumos Pharma

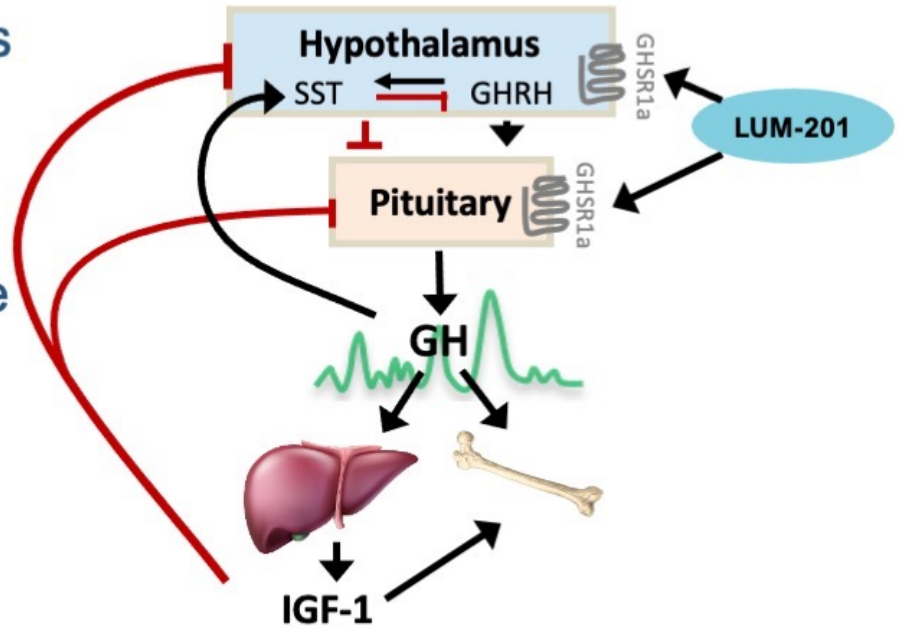
How does LUM-201 work?
And what is a secretagogue?

It works on receptors in the brain

to help you stimulate your own pulsatile production



of natural growth hormone



A secretagogue is a substance that promotes secretion.
LUM-201 is a secretagogue that promotes growth hormone (GH) release.

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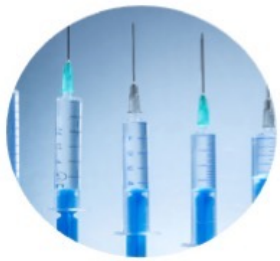
SST somatostatin
GHRH growth hormone-releasing hormone
GPCR G protein-coupled receptor
IGF-1 insulin-like growth factor-1
GHSR1a GH secretagogue receptor 1a

Reference: Lumos Pharma

What is LUM-201 and how is it different from injectable rhGH?



- It is not an injection.
- It is not rhGH in a pill form.

Moving from needles to oral therapy

- ✓ It is a small molecule that stimulates the body's own pulsatile production of growth hormone
- ✓ It is an oral medication
- ✓ Taken once a day
- ✓ Small tablets that are easy to swallow

LUM-201 (ibutamoren) – Phase 2b OraGrowtH210 Trial doses compared in size to baby aspirin.

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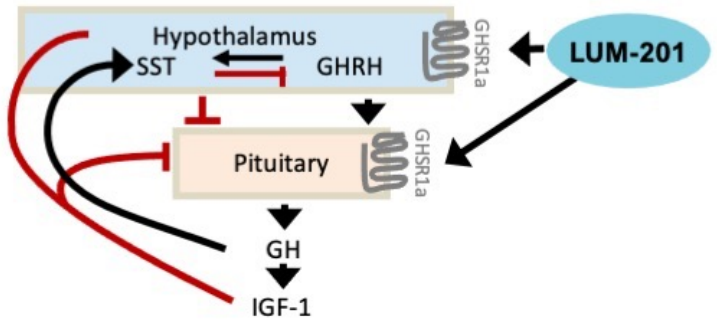
Reference: Lumos Pharma

PEMs Enrich Trials for Patients with Functional But Reduced GH Secretion

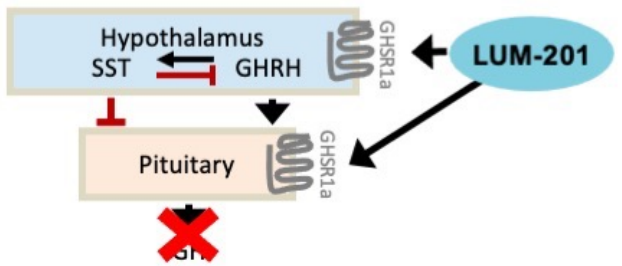
Moderate (PEM +): Included in Clinical Trials



Functional but reduced HP-GH axis

- Able to secrete some, but insufficient, GH
- Expected to respond to LUM-201[1]
- Represents ~60% of PGHD patients[2]

Severe (PEM -): Excluded from Clinical Trials



Non-functional HP-GH axis

- Unable to secrete GH
- Not expected to respond to LUM-201
- Represents ~40% of PGHD patients[2]

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

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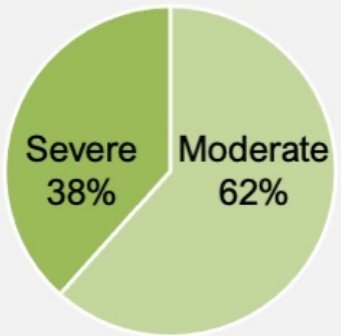
HP-GH – hypothalamic pituitary growth hormone

[1] Bright 2021 JES

[2] Blum 2021 JES

PEM Segmentation Aligns With Patients' Baseline Characteristics

		GeNeSIS[1] 12,315 GHD 514 isolated GHD	
			
Baseline	Chronological age (y)	6.80	7.10
	Height SDS	-3.01	-2.58
rhGH	Height velocity (cm/y)	9.62	8.29
	Height SDS	-2.16	-2.00

Conclusions

Data from large database:

Corroborate PGHD population segmentation by severity

- Segmentation achieved using PEMs (markers) IGF-1 and peak GH to stimulation tests
- Moderate and Severe have distinct characteristics

Indicate that ~60% PGHD population are Moderate

- Likely LUM-201 responders
- Moderate[2]: LUM-201 PEMs baseline IGF > 30 ng/mL & stim GH ≥ 5 ng/mL

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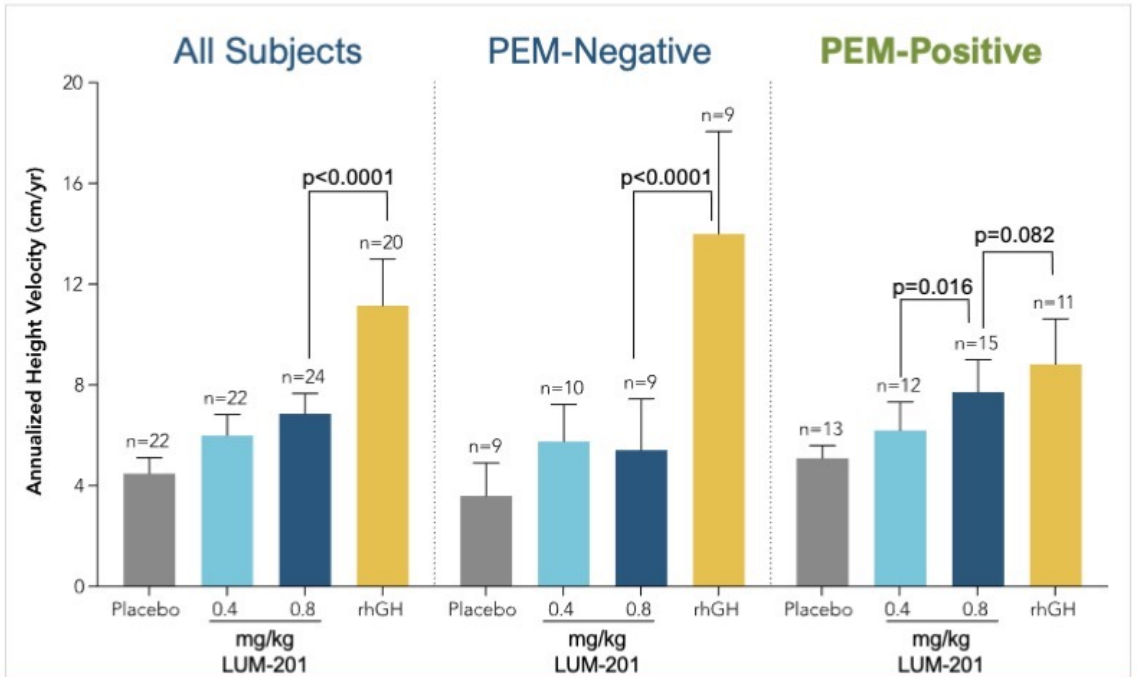
PEM Predictive Enrichment Marker SDS Standard Deviation Score
[1] Blum 2021 JES; annualized growth after 1 year of treatment [2]Bright 2021 JES

Study 020 Post-Hoc Analysis: PEM-Positive Patients Responsive to LUM-201

PEM = Predictive Enrichment Marker

- Naïve PGHD, Merck Study 020[1]
  - N=68; three arms
  - Placebo patients switched to rhGH at 6 months
  - Annualized growth shown for each arm
- PEM-positive subset:
  - LUM-201 0.8 mg/kg not statistically different from rhGH
  - Dose response: 0.8 mg/kg statistically superior to 0.4 mg/kg



Expect prospective inclusion of only PEM(+) patients and higher doses to improve response to LUM-201

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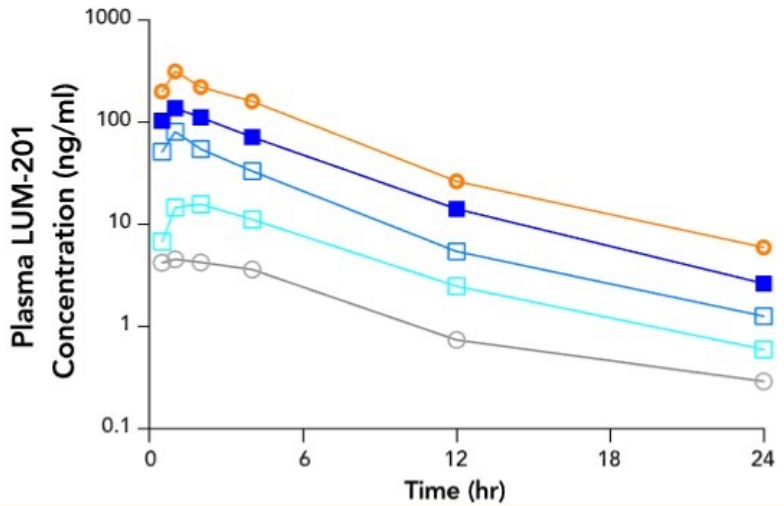
[1] Bright 2021 JES

PK/PD: Evidence of a PK and PD Dose Response in Healthy Volunteers

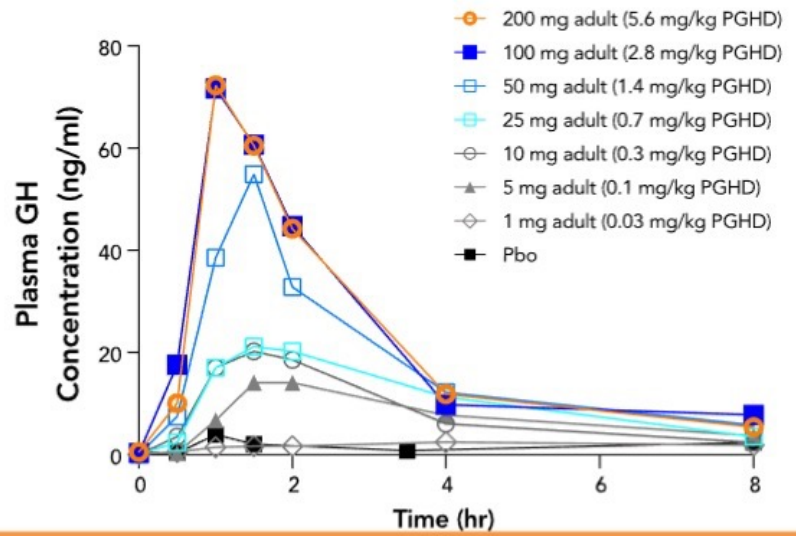
Pharmacokinetics

Dose response to 5.6 mg/kg PGHD dose equivalent*



Pharmacodynamics

PD plateau possible ≥ 2.8 mg/kg PGHD dose equivalent*



Higher LUM-201 doses produce higher plasma concentrations of LUM-201 & GH up to plateau
PD curve shows potential for LUM-201 doses in OraGrowtH210 Trial to produce greater GH response

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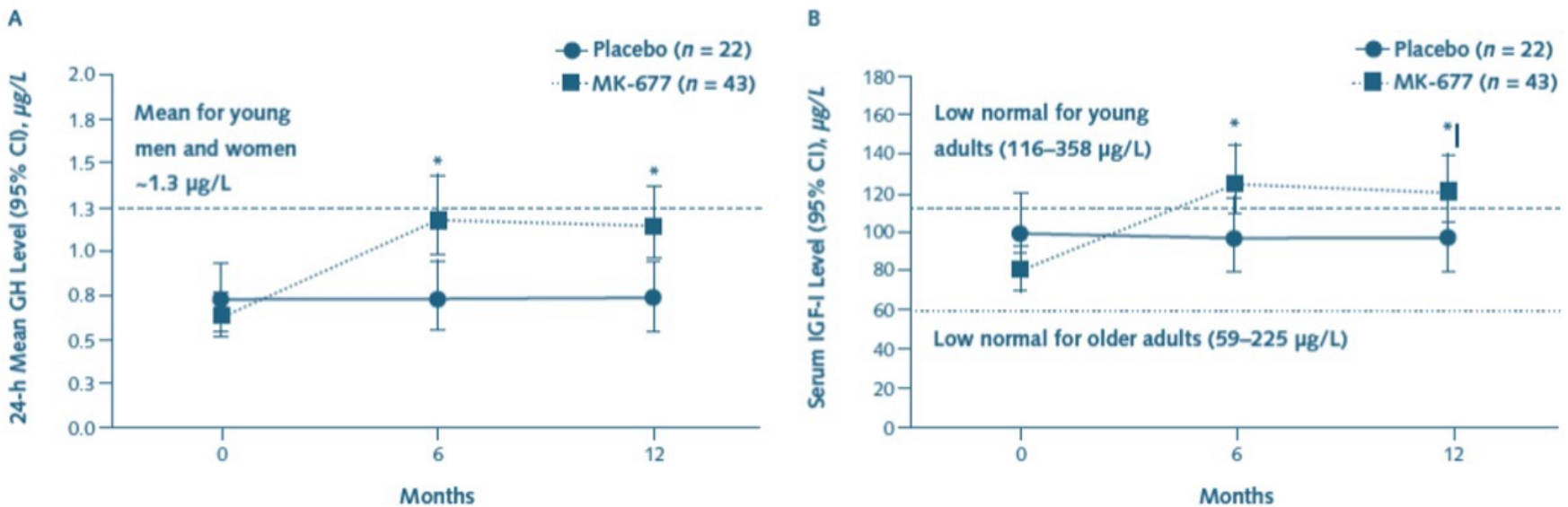
Merck Study 001 in healthy adult subjects
Data provided by Lumos Pharma

***Dose equivalence is based on AUC**

LUM-201 Effects Are Durable In Healthy Elderly



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

Nass 2008 Ann Intern Med

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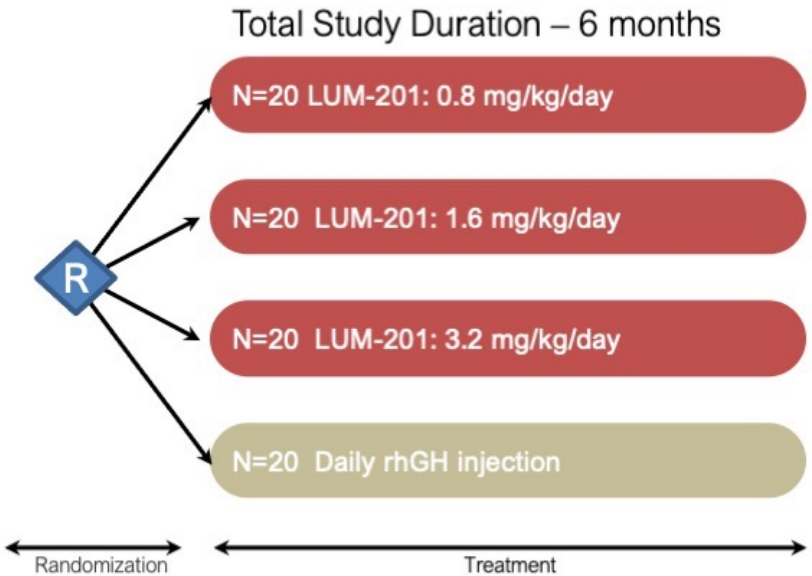
Nass 2008 Ann Intern Med

OraGrowtH210 Trial: Phase 2b Trial in PGHD

OraGrowtH210 TRIAL

- n = 80
- PEM(+) PGHD patients
- Inclusion: stim GH > 5ng/ml and baseline IGF-1 > 30ng/ml
- rhGH treatment naïve
- 40-50 trial sites US & International
- Trial opened Q4 2020



Objectives

Goals:

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

Primary Endpoint:

- Annualized Height Velocity (AHV)

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Reference: Lumos Pharma

Summary

- LUM-201 stimulates pulsatile secretion of endogenous GH and has the potential to improve growth in the majority of children with GHD and non-GHD growth disorders.
- Use of the Predictive Enrichment Marker should identify those children that are able to respond to LUM-201.
- Due to the presence of intact feedback systems in children able to respond to LUM-201, it is unlikely that elevated levels of IGF-1 will occur during LUM-201 therapy.
- Due to the pain of injections, an oral medication for the treatment of GHD and non-GHD growth disorders is a welcome alternative to injectable GH therapy.

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HEALTH FAIRVIEW
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Effects of the oral growth hormone (GH) secretagogue LUM-201 (Ibutamoren) over pulsatile GH secretion and linear growth in children with moderate GH deficiency

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Disclosure

Fernando Cassorla M.D.

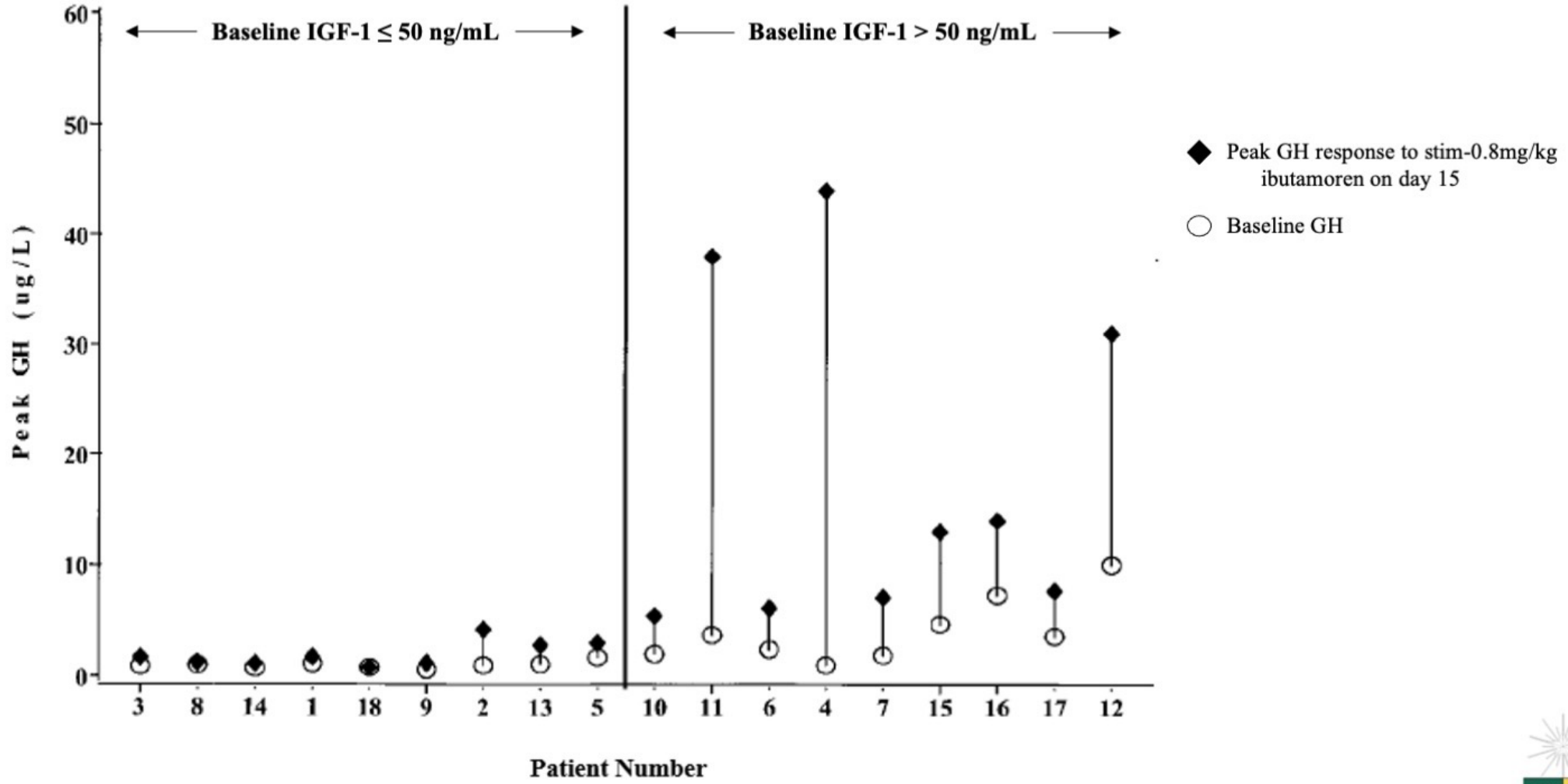
Dr. Cassorla is an investigator for clinical studies with LUM-201 at the University of Chile and has previously acted as a consultant for Debiopharm, Pfizer, Merck and Sandoz. LUM-201 is an investigational compound and is not approved for use by the FDA or any other regulatory agency. Some of the slides in this presentation are derived or copied from corporate presentations previously given by Lumos Pharma, Inc. These slides are used with permission.

Effects of oral administration of ibutamoren mesylate, a nonpeptide growth hormone secretagogue, on the growth hormone–insulin-like growth factor I axis in growth hormone–deficient children

Ethel Codner, MD, Fernando Cassorla, MD, Anatoly N. Tiulpakov, MD, PhD, M. Verónica Mericq, MD, Alejandra Avila, RN, Ora H. Pescovitz, MD, Johan Svensson, MD, Kristine Cerchio, BSN, David Krupa, MS, Barry J. Gertz, MD, PhD, and Gail Murphy, MD *Santiago, Chile, Moscow, Russia, Indianapolis, Ind, Göteborg, Sweden, and Rahway, NJ*

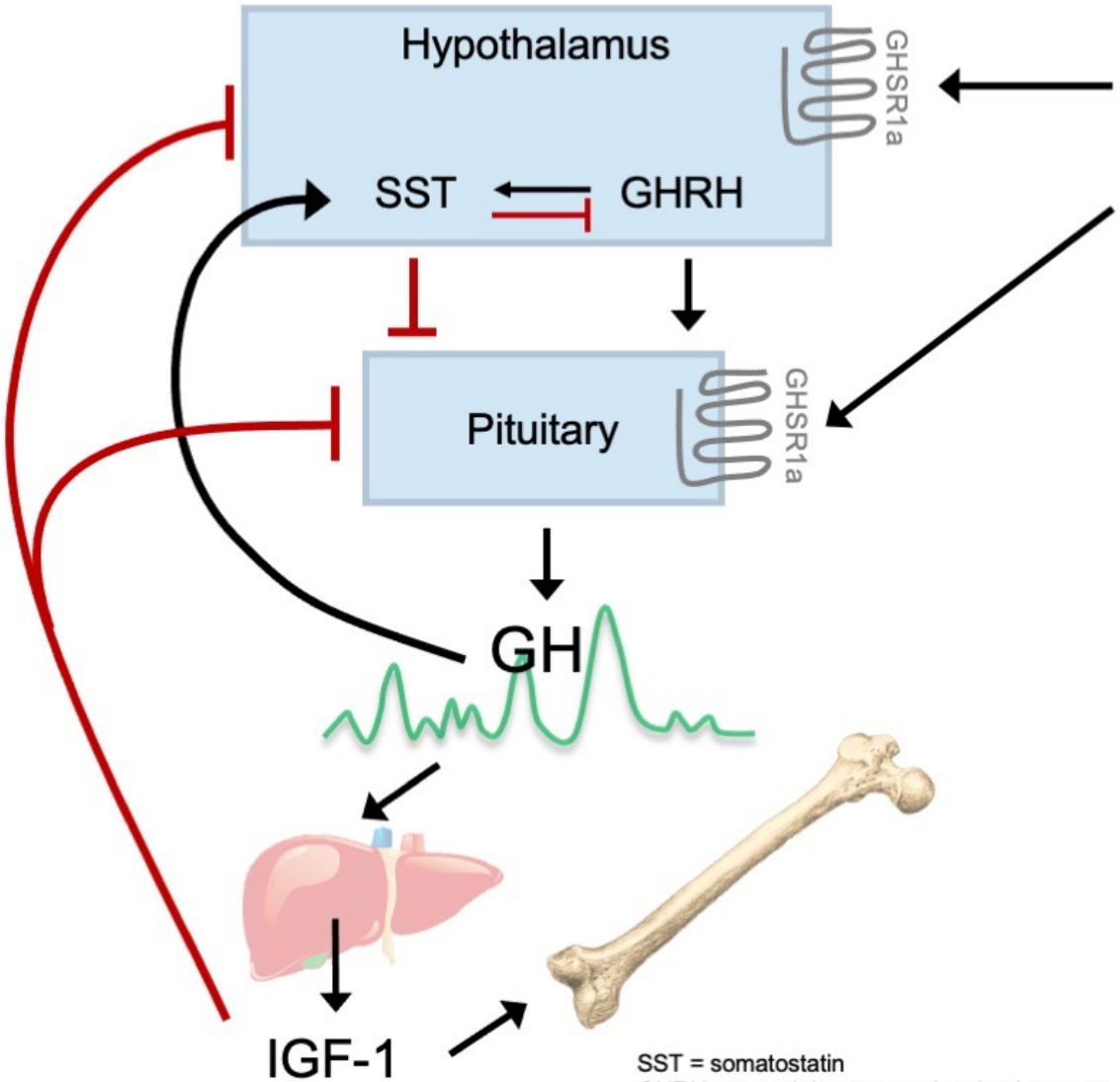
Clin Pharmacol Ther 70:91-98, 2001

Change in peak GH response after oral Ibutamoren administration for 8 days in GH deficient children



Clin Pharmacol Ther 70: 91-98, 2001

LUM-201 (Ibutamoren) Mechanism of Action



LUM-201*

- Oral LUM-201 is a ***growth hormone (GH) secretagogue***
- Acts as an agonist of GH Secretagogue Receptor (GHSR1a) to stimulate GH release[1]
- LUM-201 has been observed to ***increase the amplitude of endogenous, pulsatile GH secretion***[2,3]

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

1. Howard 1996 Science 273:974-977
2. Nass 2008 Ann Intern Med 149:601-611
3. Chapman 1997 J Clin Endocrinol Metab 82:3455-3463

Questions

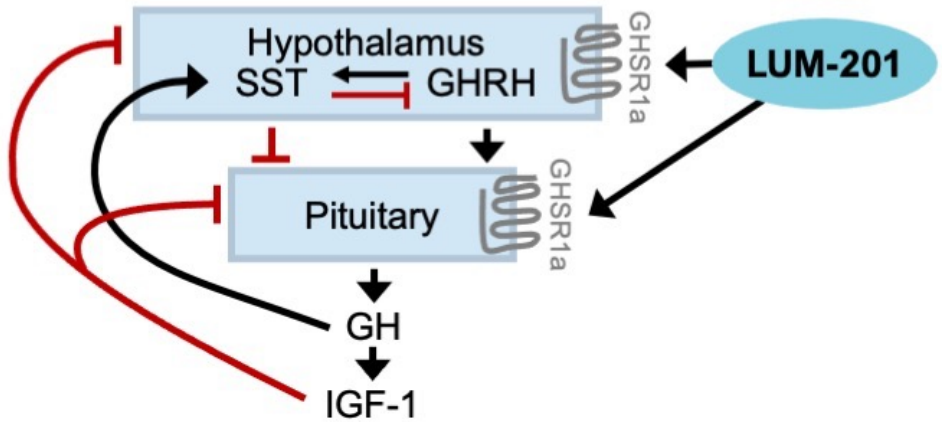
1. Does LUM-201 augment endogenous GH pulses in patients with moderate GH deficiency?

2. Will increased GH pulses improve height velocity?

3. Can these responses be predicted from baseline testing?

Predictive Enrichment Markers for Patients with Functional, But Reduced GH Secretion

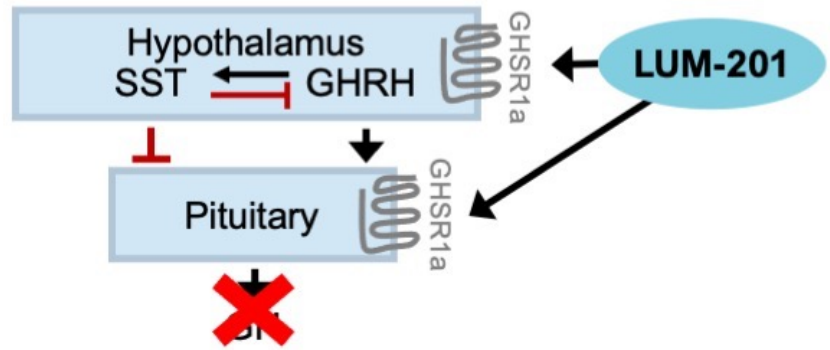
MODERATE GH deficiency: Included in Trials



Functional but reduced pituitary axis

- Able to secrete some, but insufficient, GH
- Expected to respond to LUM-201[1]
- Represents >60% of patients[2]

SEVERE GH deficiency: Excluded from Trials



Non-functional pituitary axis

- Unable to secrete GH
- Not expected to respond to LUM-201
- Represents <40% of patients[2]

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

[1] Bright G and [2] Blum W JES 2021

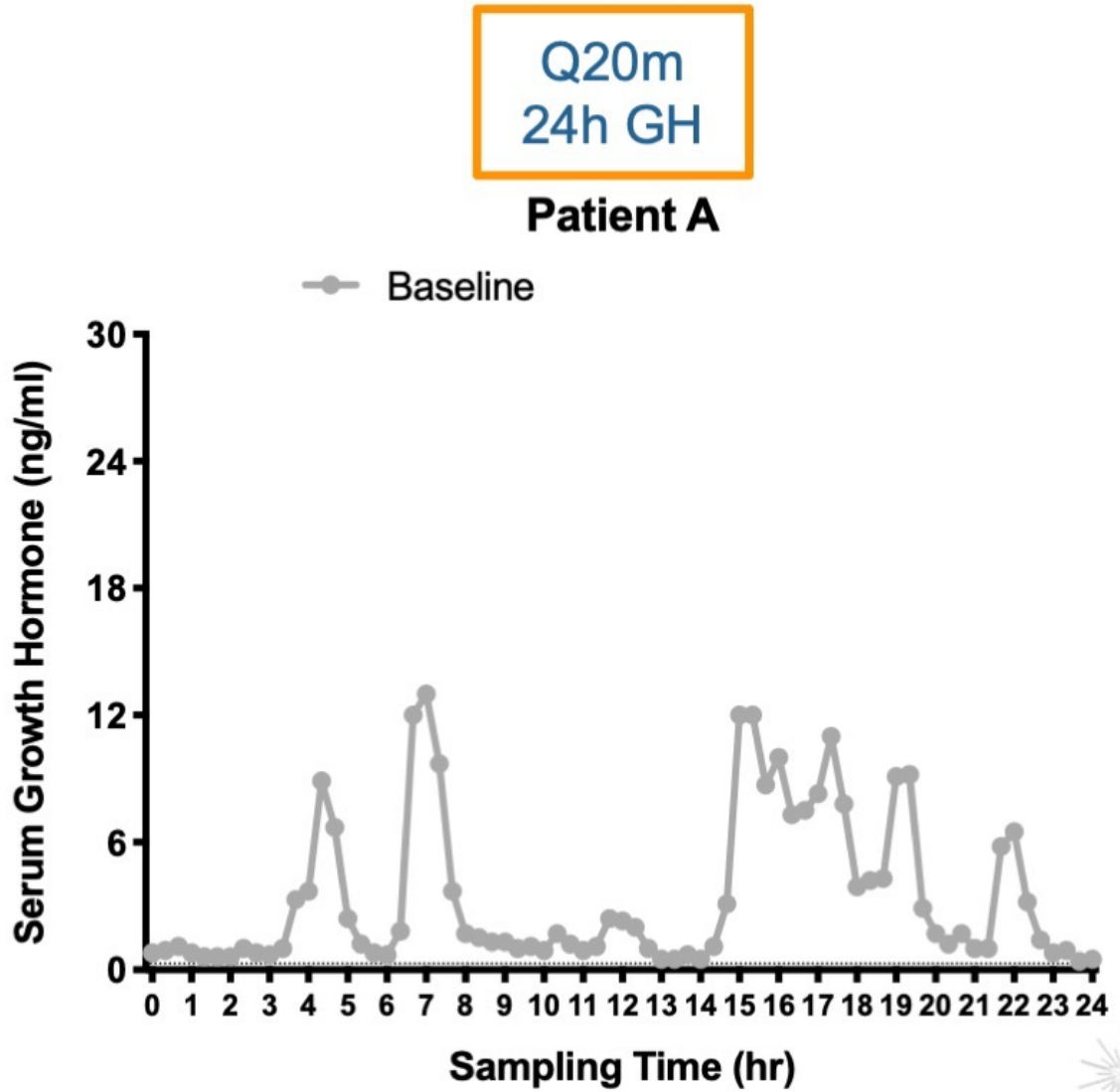
GH Pulsatility in Naïve Prepubertal Patients with GH Deficiency

Subject	Sex	Screening/Baseline				
		Age (yrs)	Height velocity (cm/yr)	Standard GH stimulation tests (ng/mL)	Peak GH to single LUM-201 dose* (ng/mL)	IGF-1 (ng/mL)
A	M	11.9	3.7	8.3	102	182
B	M	9.5	3.5	1.9	5.0	53
C	M	11.8	1.1	1.3	1.9	17

* Highest acute GH response to a single 0.8 mg/kg LUM-201 dose. Merck 020 trial.

Axis **Responsive** to LUM-201: Patient A

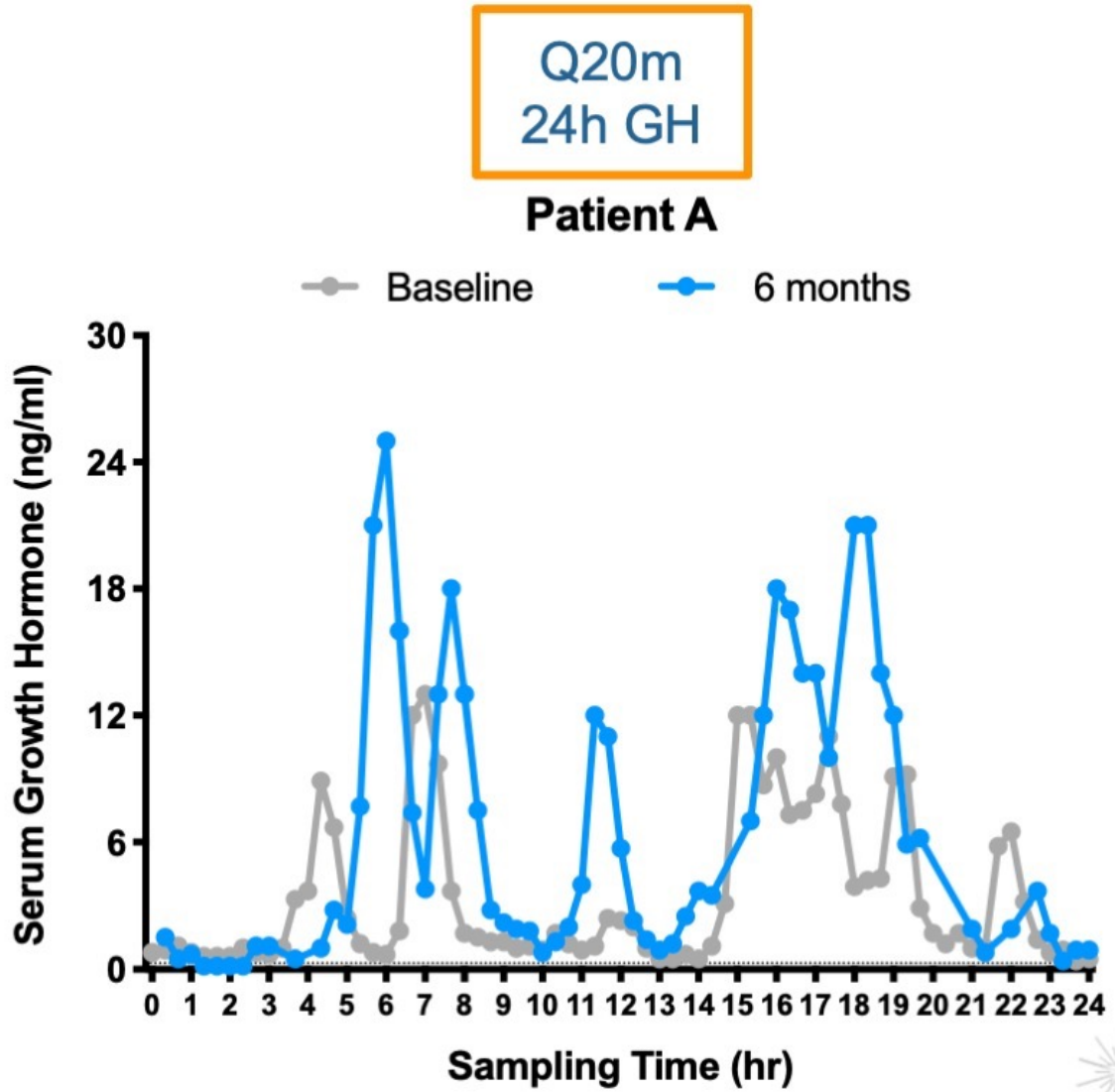
		Baseline	6 months LUM-201 0.8 mg/kg/d
	IGF-1 (ng/ml)	182	
Q20m 24h GH	Mean (ng/ml)	3.4	
	AUC (ng*hr/ml)	75.5	
	Height velocity (cm/yr)	3.7	



Merck 020 trial

Axis **Responsive** to LUM-201: Patient A

		Baseline	6 months LUM-201 0.8 mg/kg/d
	IGF-1 (ng/ml)	182	231
Q20m 24h GH	Mean (ng/ml)	3.4	6.3
	AUC (ng*hr/ml)	75.5	137.3
	Height velocity (cm/yr)	3.7	7.9

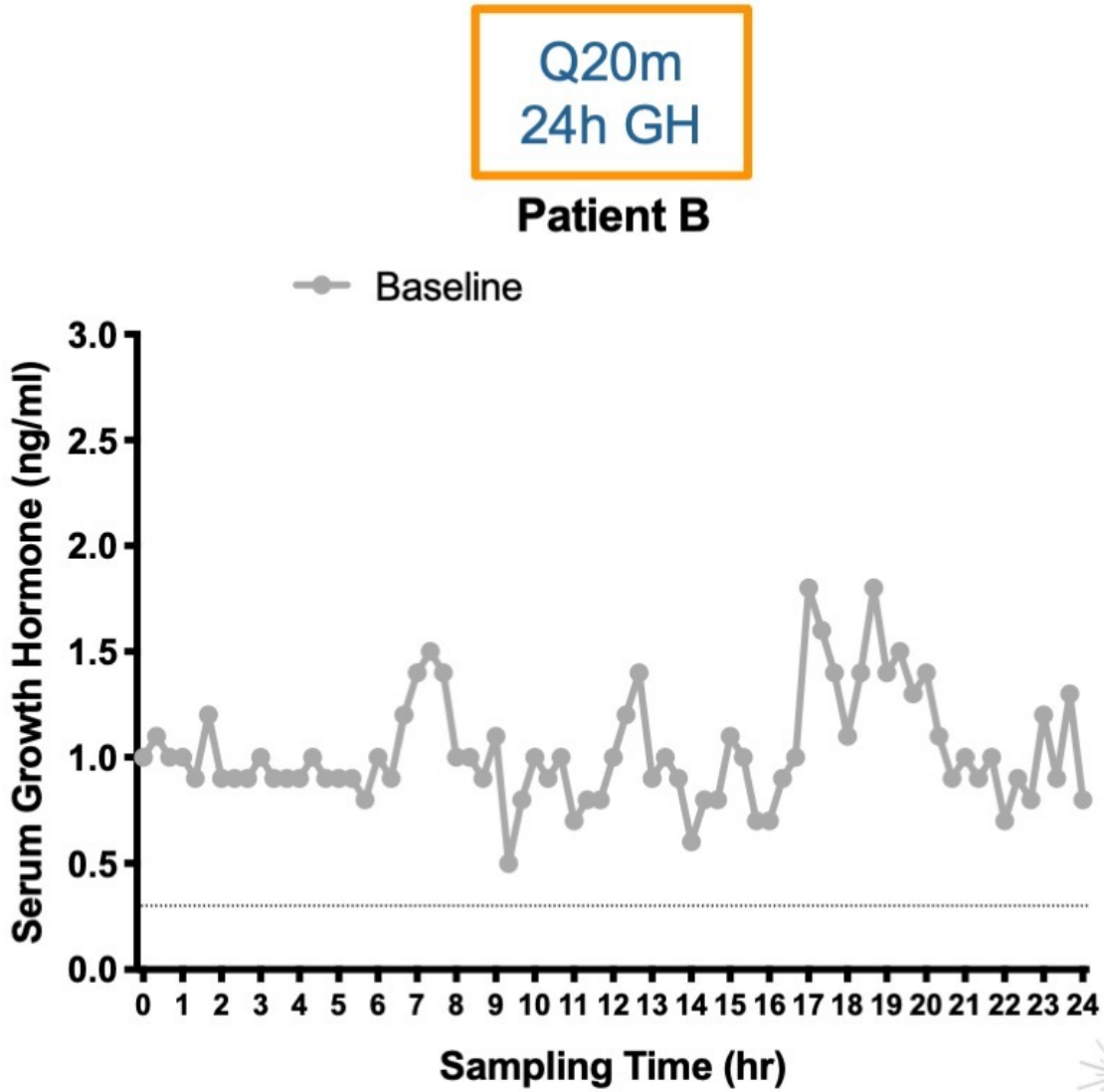


Merck 020 trial

Axis **Responsive** to LUM-201: Patient B

		Baseline	6 months LUM-201 0.8 mg/kg/d
	IGF-1 (ng/ml)	53	
Q20m 24h GH	Mean (ng/ml)	1.0	
	AUC (ng*hr/ml)	17.6	
	Height velocity (cm/yr)	3.5	

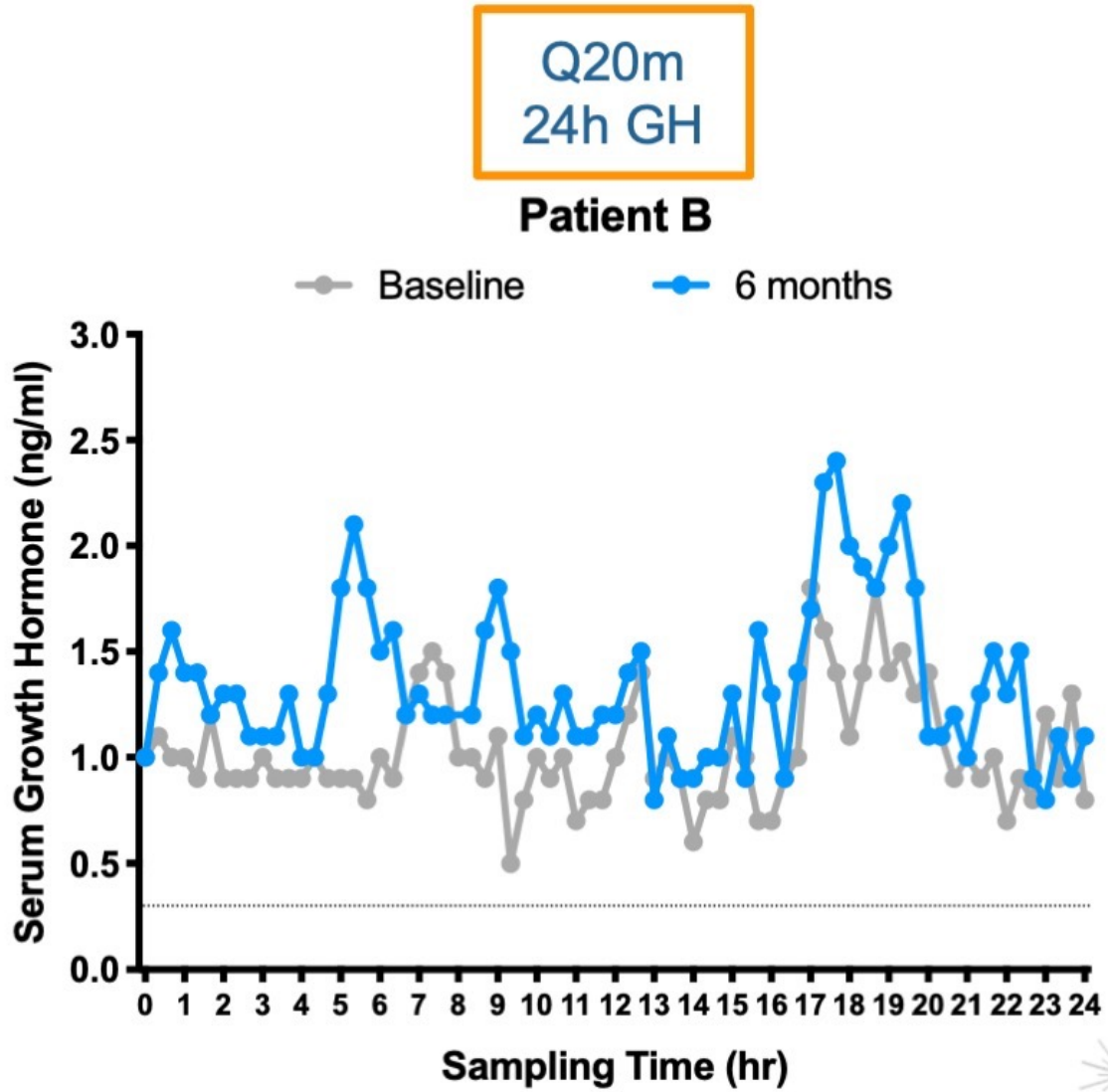
Q20m
24h GH



Merck 020 trial

Axis **Responsive** to LUM-201: Patient B

		Baseline	6 months LUM-201 0.8 mg/kg/d
	IGF-1 (ng/ml)	53	72
Q20m 24h GH	Mean (ng/ml)	1.0	1.3
	AUC (ng*hr/ml)	17.6	25.0
	Height velocity (cm/yr)	3.5	8.9

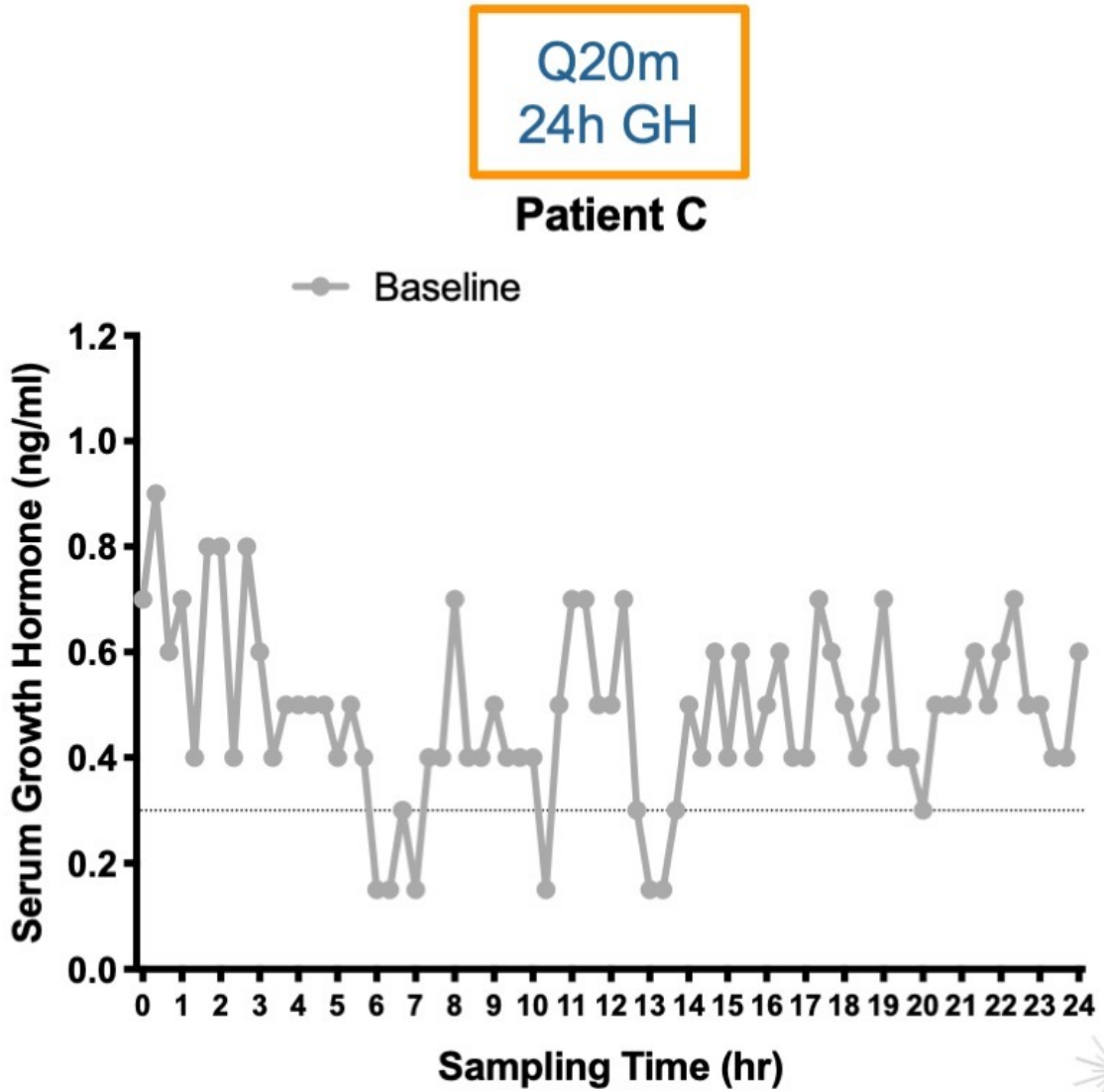


Merck 020 trial

Axis **Not Responsive** to LUM-201: Patient C

		Baseline	6 months LUM-201 0.8 mg/kg/d
	IGF-1 (ng/ml)	17	
Q20m 24h GH	Mean (ng/ml)	0.5	
	AUC (ng*hr/ml)	4.9	
	Height velocity (cm/yr)	1.1	

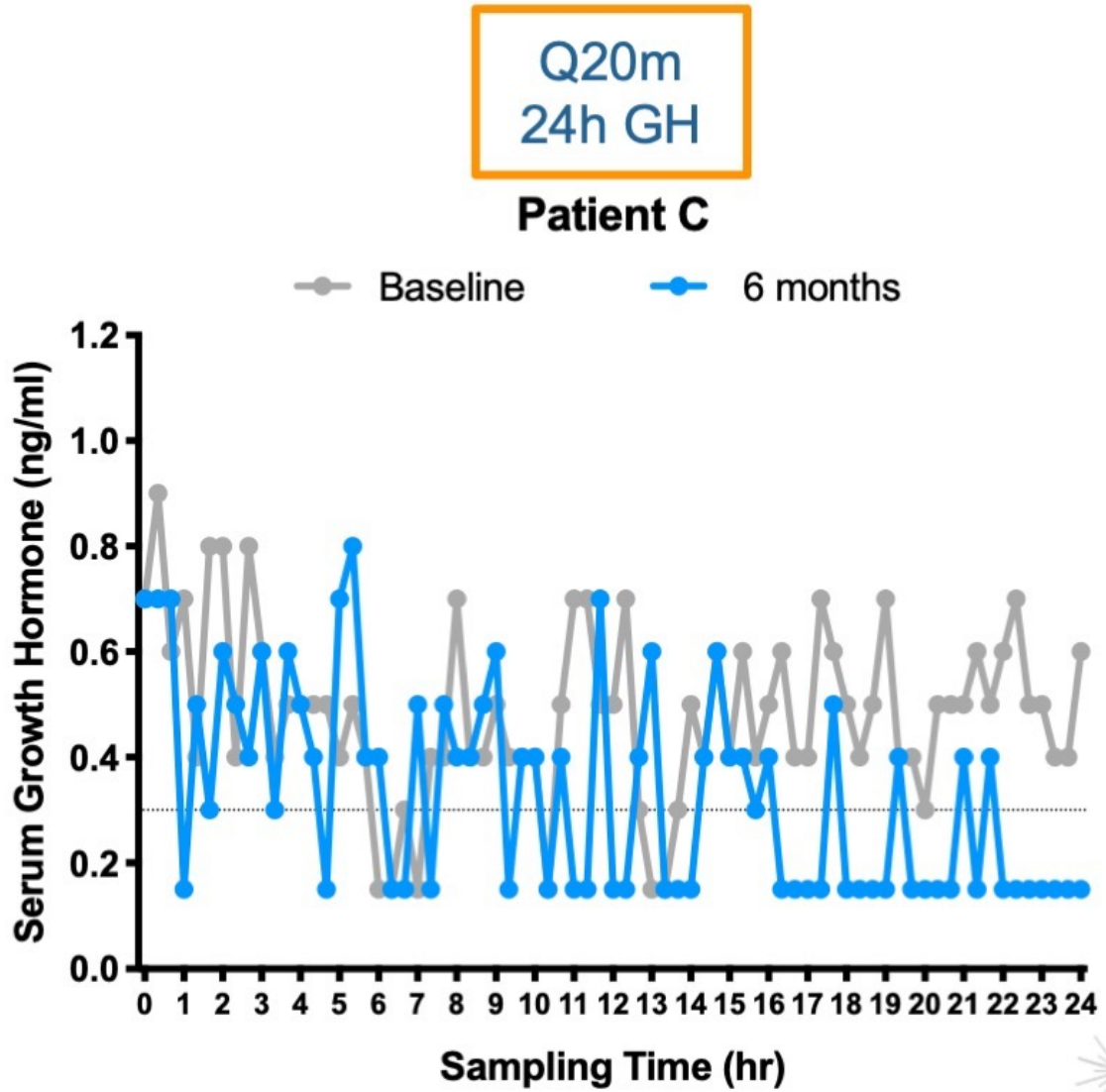
Q20m
24h GH



Merck 020 trial

Axis **Not Responsive** to LUM-201: Patient C

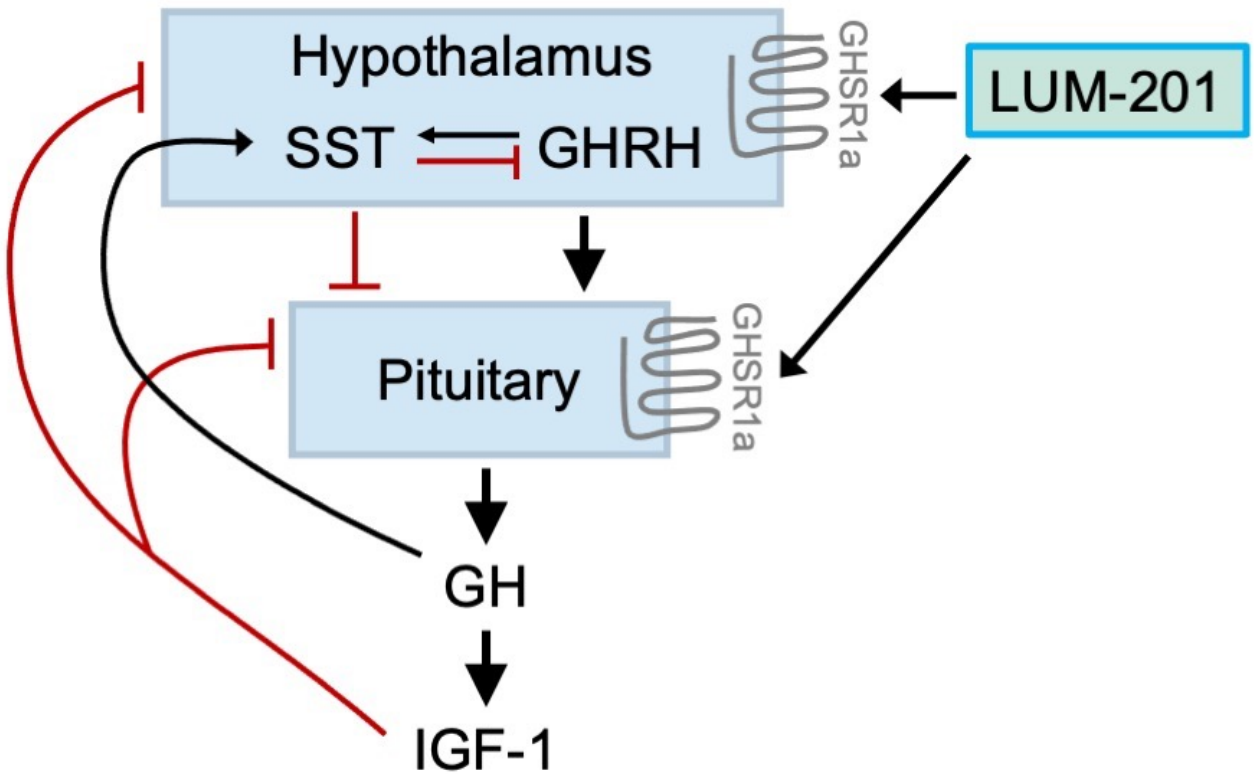
		Baseline	6 months LUM-201 0.8 mg/kg/d
	IGF-1 (ng/ml)	17	15
Q20m 24h GH	Mean (ng/ml)	0.5	0.3
	AUC (ng*hr/ml)	4.9	3.4
	Height velocity (cm/yr)	1.1	1.8



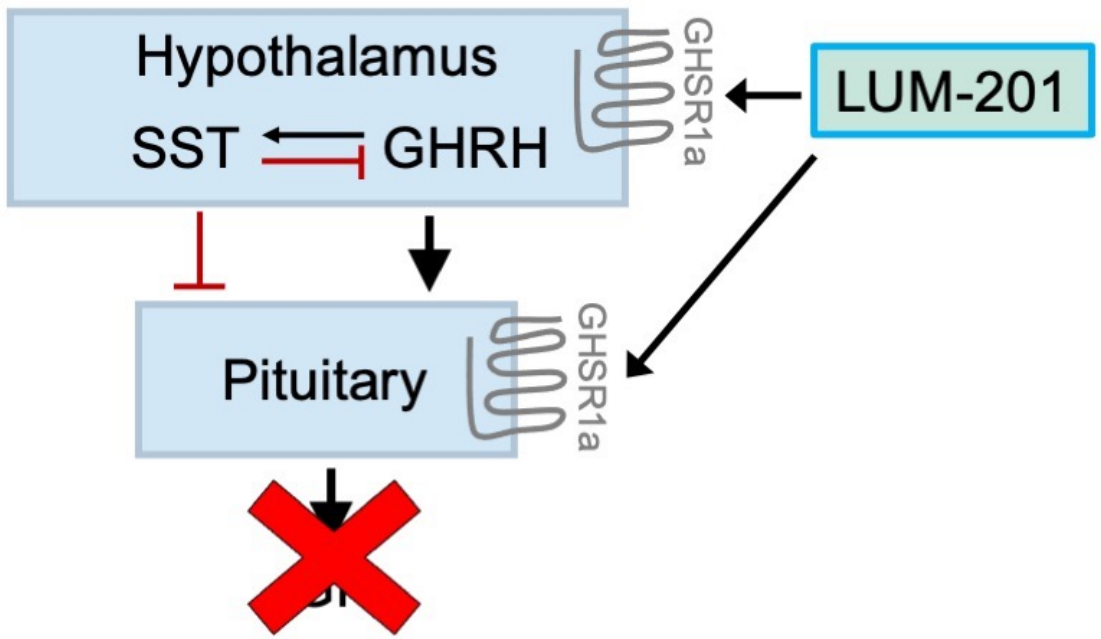
Merck 020 trial

Status of Hypothalamic Pituitary GH Axis

Patients A & B
Axis **Responsive**



Patient C
Axis **Not Responsive**



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

LUM-201 can stimulate pituitary GH secretion and improve height velocity in children with moderate GH deficiency

- Patients A & B
  - Baseline IGF-1 > 30 ng/mL and peak GH to single LUM-201 dose ≥ 5 ng/mL*
  - 24-hour GH is augmented less than two-fold
  - Height velocity is improved
- Patient C
  - Baseline IGF-1 ≤ 30 ng/mL and peak GH to single LUM-201 dose < 5 ng/mL*
  - 24-hour GH not augmented
  - Height velocity not improved

* IGF-I and peak GH cutoffs determined by Receiver Operating Characteristic analyses in 24 PGHD subjects treated with 0.8 mg/kg/day LUM-201; Bright G and Blum W JES 2021

Further studies of LUM-201 in pediatric patients with moderate growth hormone deficiency are required in order to confirm that:

- LUM-201 increases endogenous GH pulsatility
- Augmented GH pulsatility improves height velocity
- Augmentation of GH pulsatility and improvement of height velocity by LUM-201 treatment can be predicted by baseline IGF-1 and peak GH response to a single dose of LUM-201

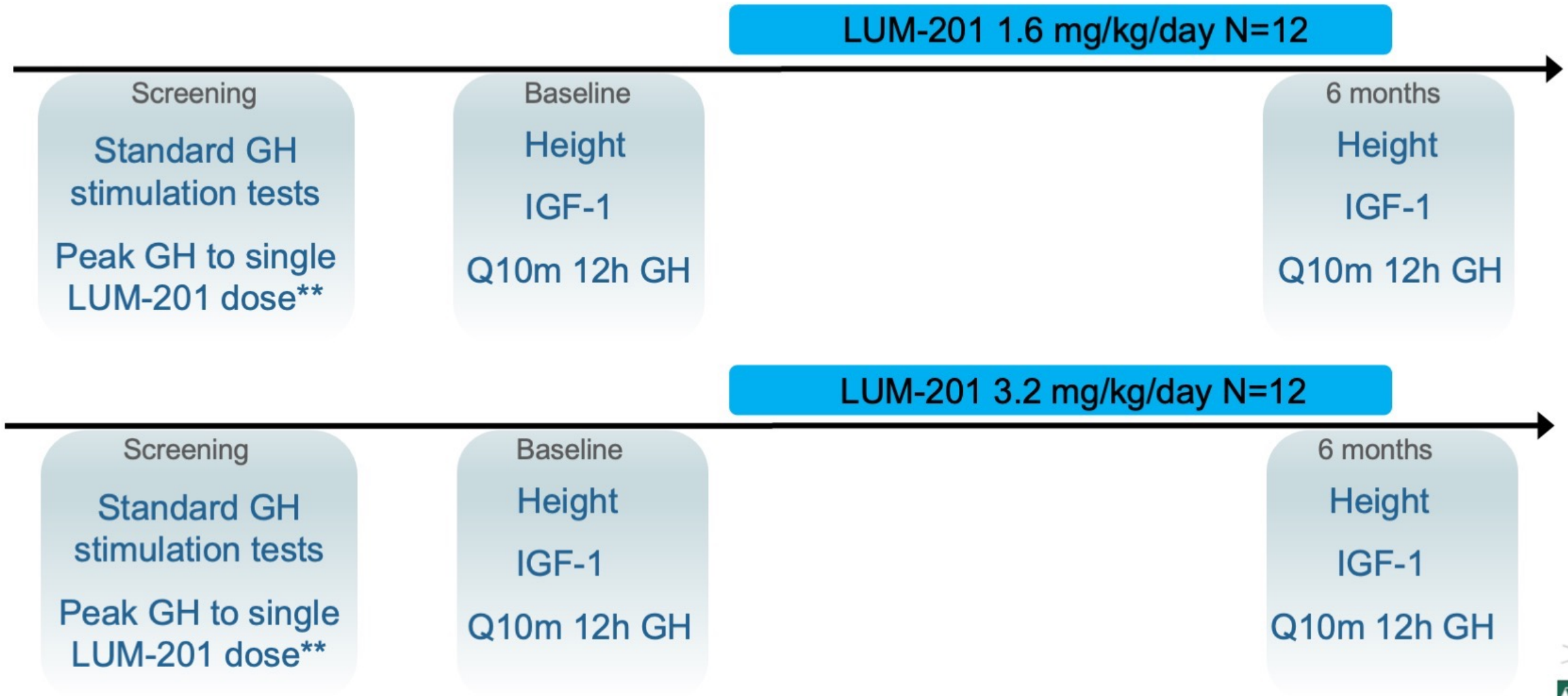
Clinical study of LUM-201 in naïve pediatric patients with moderate GH deficiency at the University of Chile

- 24 prepubertal patients between the ages of 4 to 8 years (girls) and 10 years (boys) with short stature caused by moderate GH deficiency, and naïve to GH treatment, will be assigned randomly to receive oral LUM-201 at a dose of 1.6 mg/kg/day (n:12) or 3.2 mg/kg/day (n:12) for 6 months.

- Pulsatile GH secretion will be assessed in blood samples obtained every 10 minutes during 12 hours (8AM to 8PM), at the beginning and end of the study.

- Height velocity during the 6 months of LUM-201 administration will be compared with a baseline period prior to intervention.

LUM-201 in naïve prepubertal patients with GH deficiency



Inclusion Criteria: Height < 2 SD, delayed bone age, serum IGF-1 below the mean for age, and peak GH response to a clonidine stimulation test between 3 and 10 ng/mL

Expected results

- We will document any increase in the height velocity of these prepubertal patients with moderate GH deficiency treated with LUM-201, which may be dependent on the dose employed.

- The improved height velocity during LUM-201 administration will be correlated with an increase in GH pulsatility after 6 months of therapy with this drug.

- The increase in GH pulsatility and improvement in height velocity induced by LUM-201 can be predicted by the baseline serum IGF-1 concentrations, and the peak GH response to a single dose of LUM-201.

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lumos
PHARMA
Q & A