Ascendis Pharma A/S

SKYTROFA® FDA Approval

August 25, 2021

Cautionary Note On Forward-Looking Statements

This presentation contains forward-looking statements. All statements other than statements of historical facts contained in this presentation, such as statements regarding our future results of operations and financial position, including our business strategy, expectations regarding the potential benefits of Skytrofa, expectations regarding the projected timing of availability of Skytrofa, expectations regarding a patient support services, availability of funding, clinical trial results, product approvals and regulatory pathways, collaborations, licensing or other arrangements, the potential market size and size of the potential patient populations for Skytrofa and our other product candidates, timing and likelihood of success, plans and objectives of management for future operations, our ability to integrate data informatics to create innovative patient care solutions and enhance patient care, the scope, progress, results and costs of developing our product candidates or any other future product candidates, and future results of current and anticipated products are forward-looking statements. These forward-looking statements are based on our current expectations and beliefs, as well as assumptions concerning future events. These statements involve known and unknown risks, uncertainties and other factors that could cause our actual results to differ materially from the results discussed in the forward-looking statements. These risks, uncertainties and other factors are more fully described in our reports filed with or submitted to the Securities and Exchange Commission, including, without limitation, our most recent Annual Report on Form 20-F filed with the SEC on April 3, 2020 particularly in the sections titled “Risk Factors” and “Management’s Discussion and Analysis of Financial Condition and Results of Operations”. In light of the significant uncertainties in our forward-looking statements, you should not place undue reliance on these statements or regard these statements as a representation or warranty by us or any other person that we will achieve our objectives and plans in any specified timeframe, or at all.

Any forward-looking statement made by us in this presentation speaks only as of the date of this presentation and represents our estimates and assumptions only as of the date of this presentation. Except as required by law, we assume no obligation to update these statements publicly, whether as a result of new information, future events, changed circumstances or otherwise after the date of this presentation.

Skytrofa has been approved by the U.S. Food and Drug Administration for the treatment of pediatric growth hormone deficiency. Skytrofa is and has been under clinical investigation and has not yet been approved for marketing by the European Medicines Agency or other foreign regulatory authorities.

SKYTROFA® Now FDA Approved

- First FDA-approved once-weekly product for pediatric growth hormone deficiency (GHD)
- First FDA-approved product utilizing TransCon™ technology

Skytrofa: Select Highlights of U.S. Prescribing Information

INDICATIONS AND USAGE

Skytrofa is a human growth hormone indicated for the treatment of pediatric patients 1 year and older who weigh at least 11.5 kg and have growth failure due to inadequate secretion of endogenous growth hormone (GH)

DOSAGE AND ADMINISTRATION

Skytrofa should be administered subcutaneously into the abdomen, buttock, or thigh with regular rotation of the injection sites.

The recommended dose is 0.24 mg/kg body weight once-weekly.

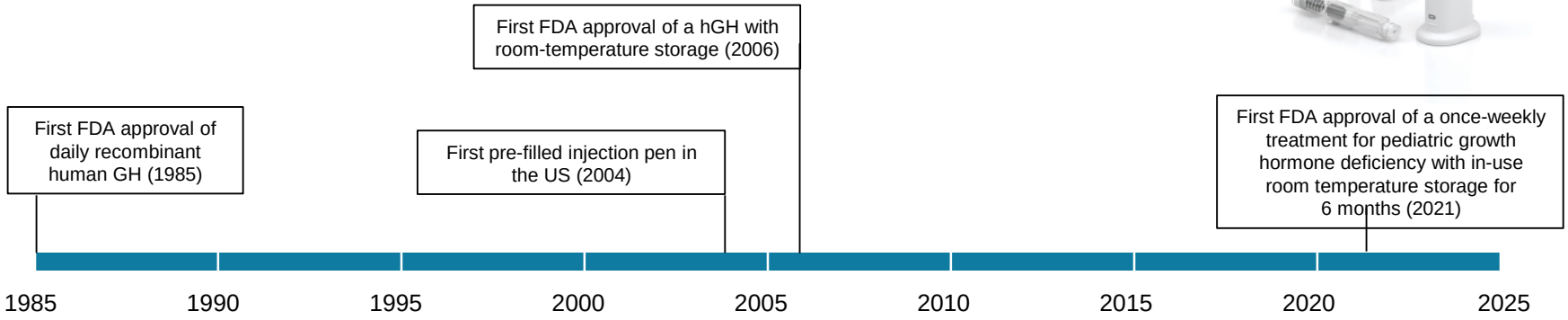
Safety

Skytrofa is contraindicated in patients with:

- **Acute critical illness** after open heart surgery, abdominal surgery or multiple accidental trauma, or those with acute respiratory failure due to the risk of increased mortality with use of pharmacologic doses of somatropin
- **Hypersensitivity** to somatropin or any of the excipients in Skytrofa
- **Children with closed epiphyses**
- **Active malignancy**
- Active proliferative or severe non-proliferative **diabetic retinopathy**
- **Prader-Willi syndrome** who are severely obese, have a history of upper airway obstruction or sleep apnea or have severe respiratory impairment due to the risk of sudden death

Adverse Reactions: Most common adverse reactions (≥5%) were viral infection (15%), pyrexia (15%), cough (11%), nausea and vomiting (11%), hemorrhage (7%), diarrhea (6%), abdominal pain (6%), and arthralgia and arthritis (6%).

Limited Innovation Since rhGH was Introduced >30 Years Ago



Comprehensive Clinical Program

height TRIAL

Clinical evidence provides compelling value proposition and clear pathway for starting **Tx-naïve** patients

fliGHt TRIAL

Data provides support for **switching** patients from daily GH regimens to once weekly Skytrofa

enlIGHten TRIAL

Ongoing study for **long-term** safety assessments

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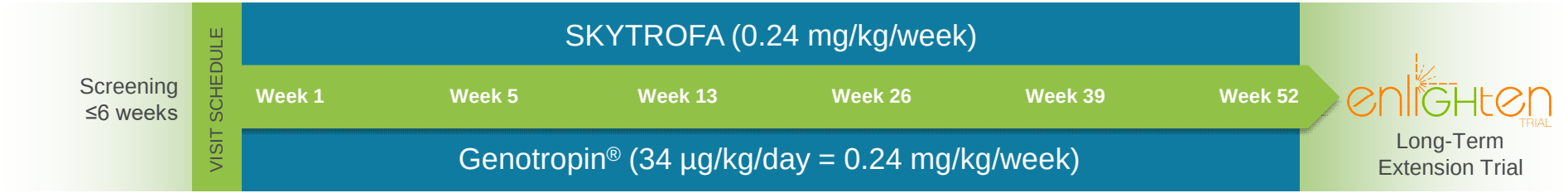
Phase 3 heiGHt Trial

161 treatment-naïve children with GHD dosed (2:1 randomization)



Objective

- Demonstrate non-inferiority

Key Inclusion Criteria

- Prepubertal children with GHD
- Height SDS ≤-2.0
- IGF-1 SDS ≤-1.0
- 2 GH stimulation tests (GH ≤10 ng/mL)
- Bone age ≥6 months behind chronological

Key Endpoints

- Annualized height velocity (HV) at 52 weeks (primary endpoint)
- Annualized HV at earlier time points
- Change in height SDS over 52 weeks
- Change in serum IGF-1/IGFBP-3 levels
- Change in IGF-1 SDS and IGFBP-3 SDS
- Normalization of IGF-1 SDS
- hGH and IGF-1 levels over 168 hours at Week 13 (PK/PD subset)

Thornton PS, J Clin Endocrinol Metab, 2021. In press, https://doi.org/10.1210/clinem/dgab529.

Skytrofa hGH Met Primary Objective of Non-inferiority and Demonstrated Higher AHV at Week 52

	Skytrofa (n = 105)	Genotropin (n = 56)	Estimate of Treatment Difference
LS Mean AHV at Week 52 (cm/year)	11.2	10.3	0.86
Standard Error	0.23	0.30	0.33
95% Confidence Interval (cm/year)	10.71–11.62	9.73–10.89	0.22–1.50

ANCOVA model was applied after missing data were imputed by multiple imputation method.
Thornton PS, J Clin Endocrinol Metab, 2021. In press, https://doi.org/10.1210/clinem/dgab529
Thornton P, et al. Oral presentation at ENDO 2019

Summary of Adverse Events: Safety Population

	Skytrofa (n = 105) n (%)	Genotropin (n = 56) n (%)
Treatment-emergent Adverse Events (TEAEs)	81 (77.1)	39 (69.6)
TEAEs Related to Study Drug	12 (11.4)	10 (17.9)
Serious Adverse Events (AEs)	1 (1.0)	1 (1.8)
Serious AEs Related to Study Drug	0	0
TEAEs Leading to Any Action on Study Drug	2 (1.9)	1 (1.8)
TEAEs Leading to Discontinuation of Study Drug	0	0

- Adverse events for Skytrofa similar with type and frequency observed with Genotropin
- No serious adverse events related to study drug in either arm
- No treatment-emergent adverse event led to discontinuation of study drug in either arm

Thornton PS, J Clin Endocrinol Metab, 2021. In press, https://doi.org/10.1210/clinem/dgab529
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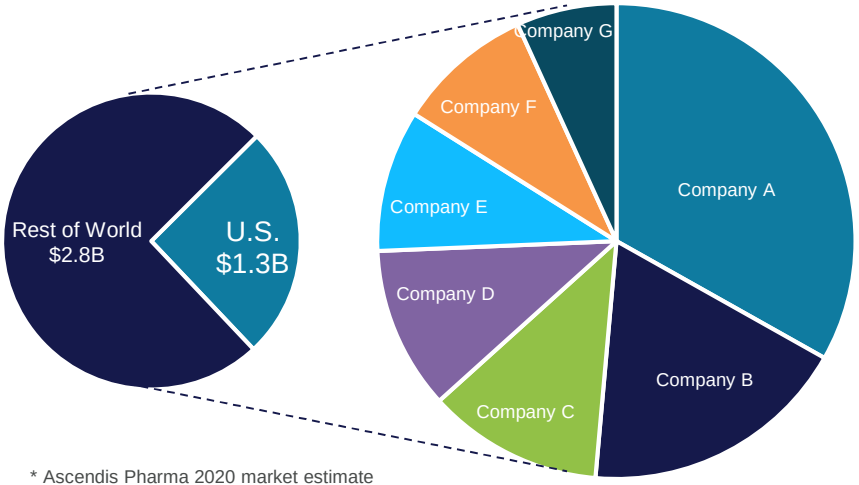
Global Commercial Strategy – Multiple Approaches

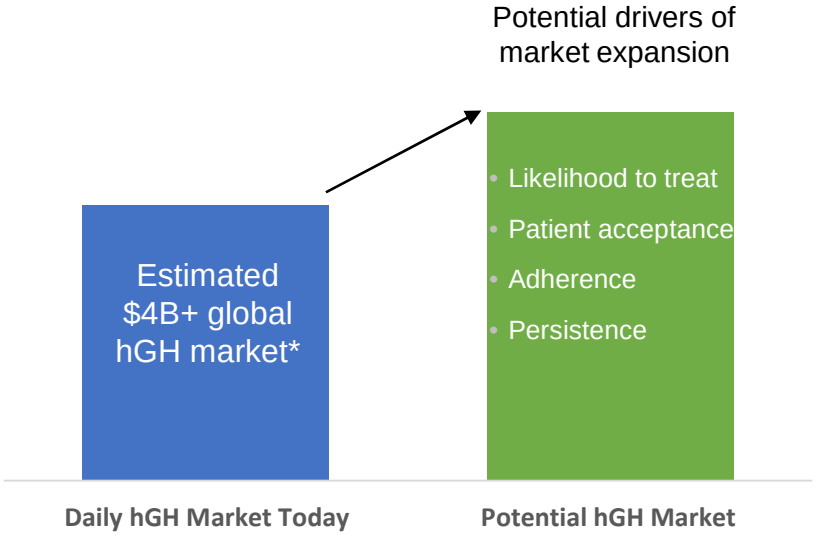
- Establishing global commercial presence to deliver potential best-in-class TransCon product candidates to address patients' unmet medical needs
- Laying groundwork for successful future endocrinology rare disease launches
- US commercial organization in place for upcoming launch of SKYTROFA in pediatric GHD
- Preparing for commercialization in Europe
  - Building integrated organization in select countries for potential lonapegsomatropin MAA approval in Q4 2021
  - Evaluating established distribution channels in other countries
- Establishing global commercial presence through partners with local expertise and infrastructure
  - Collaborating with VISEN Pharmaceuticals for Greater China
  - Partner in Japan and South Korea when appropriate
  - Serve patients in ROW through established sales and distribution systems

Global Human Growth Hormone (hGH) Market Dynamics

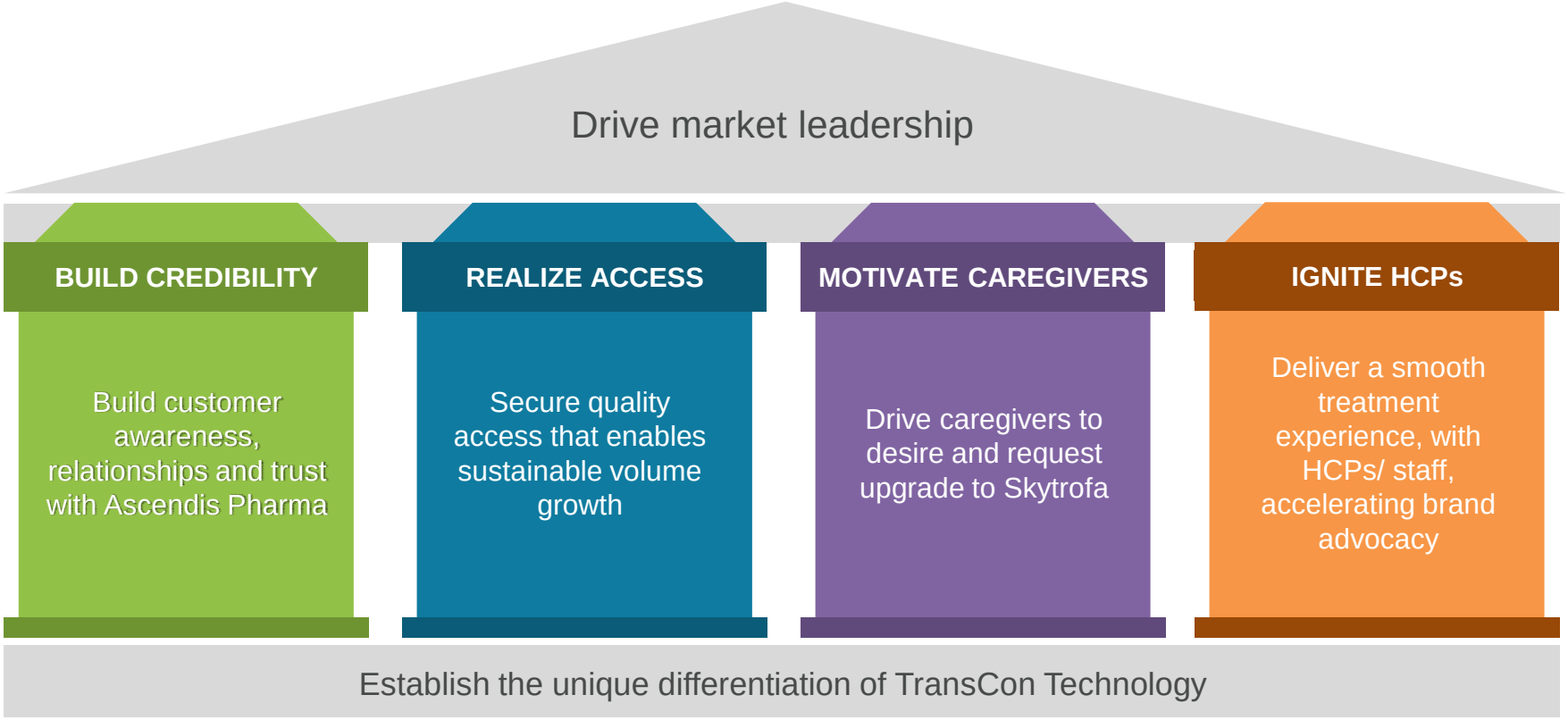


* Ascendis Pharma 2020 market estimate

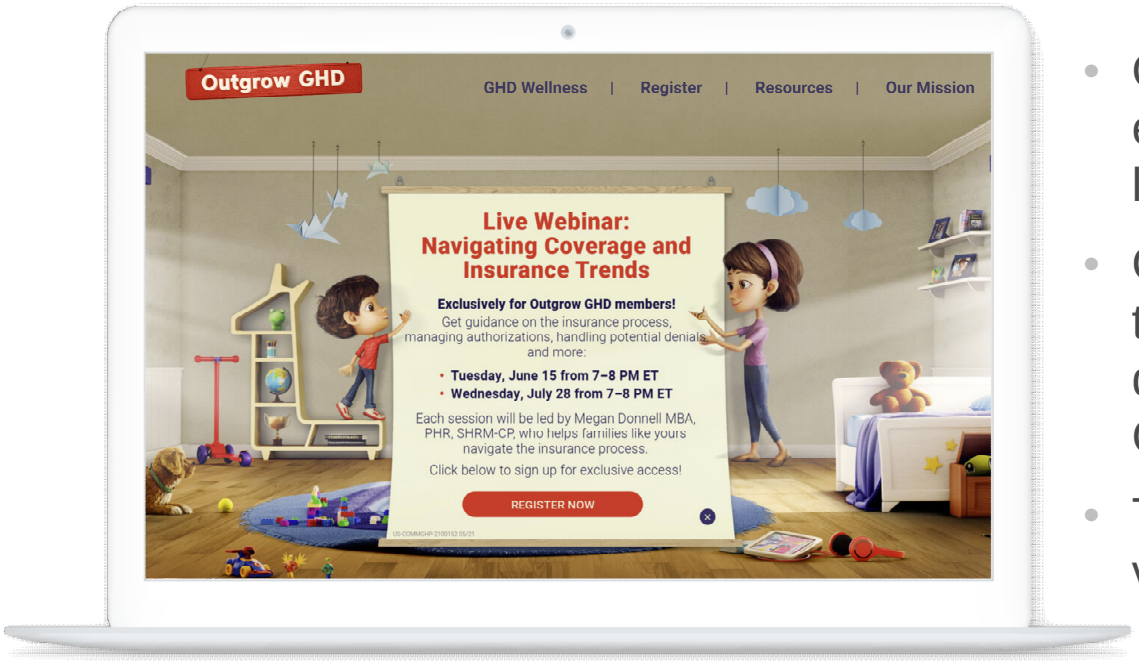
- Global hGH market estimated at >$4 billion*
- U.S. hGH market estimated at $1.3B* for all indications
  - Pediatric GHD estimated ~$700 million
  - Room temp storage driving share



Our First U.S. Commercial Launch Will Pave the Way for Future Success

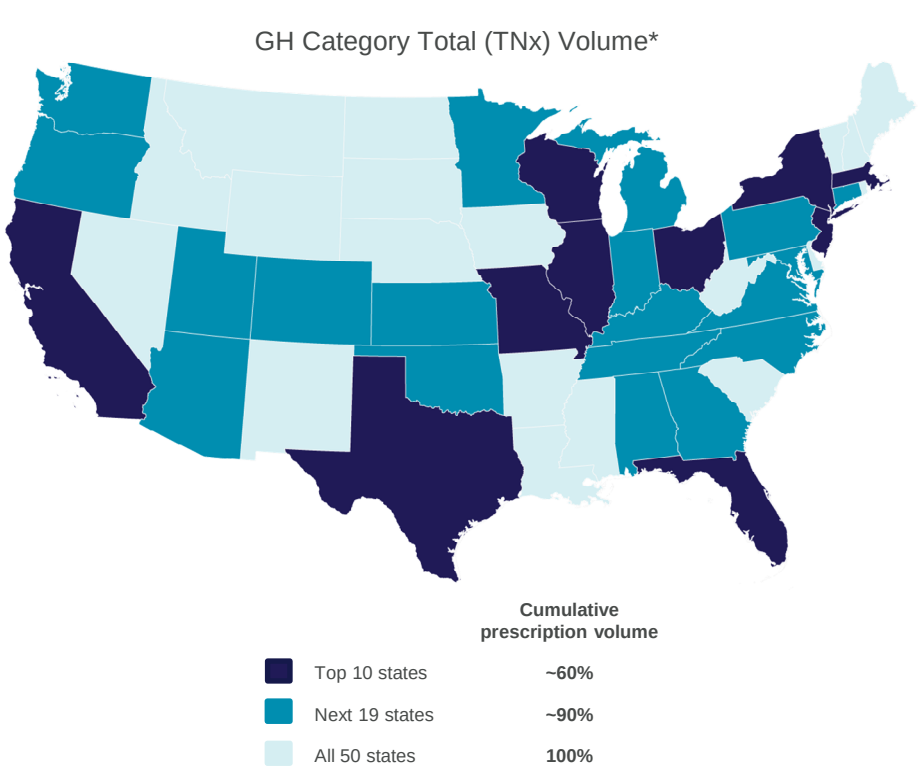


Educational Efforts to Increase Awareness of Pediatric GHD



- Outgrow GHD online educational resource launched in February
- Offers tools and information to help U.S. caregivers face difficult subjects related to GHD
- To date, over 100,000 unique visitors to the site

Targeting Top Pediatric Endocrinology Markets in the U.S.



National commercial organization in place to cover ~1,400 GH prescribers

- Fully staffed/trained endocrine sales team at product launch
- Concentrated market: In the U.S. approximately 20% of prescribers cover ~80% of volume

* 2019 IQVIA Xponent data

Ascendis Signature Access Program (A·S·A·P)

PATIENT ENROLLMENT
- Assigned nurse advocate
- Overall case management
- Insurance verification

PATIENT ACTIVATION
- Fast start fulfillment
- Auto-Injector fulfillment
- Starter kit fulfillment
- Nurse training

PATIENT ACCESS
- Benefits verification
- Prior authorization support
- Appeals support
- Out of pocket assistance

Expanding Global Reach and Indications for Lonapegsomatropin

Geographic Expansion for Pediatric GHD

- Pediatric GHD phase 3 trial in Japan
- Enrollment ongoing

Greater China* VISEN PHARMA

- Pediatric GHD phase 3 trial in China
- Target recruitment reached in Q1 2021

Label Expansion

- Global adult GHD phase 3 trial
- Enrollment ongoing

Future indications

- Additional studies planned

*Conducted by VISEN Pharmaceuticals.

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Changing Patients' Lives: A message from Tiffany & Lyndon

"Thank you doctors for my medicine for my body."

"To get the opportunity to only have to give him a shot once a week, it drastically changed our lives.

We don't have to pin him down…we don't have to take 45 minutes to mix the medication up…

We will forever be thankful for you because you have made our lives so much better."

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