

**MOD-4023: 12 month Safety and efficacy of  
a weekly long-acting GH compared to daily rhGH  
therapy in pre-pubertal GHD children**

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# DISCLOSURE

**Ron Rosenfeld is a member of the Pediatric Clinical Advisory Board and a consultant for OPKO**

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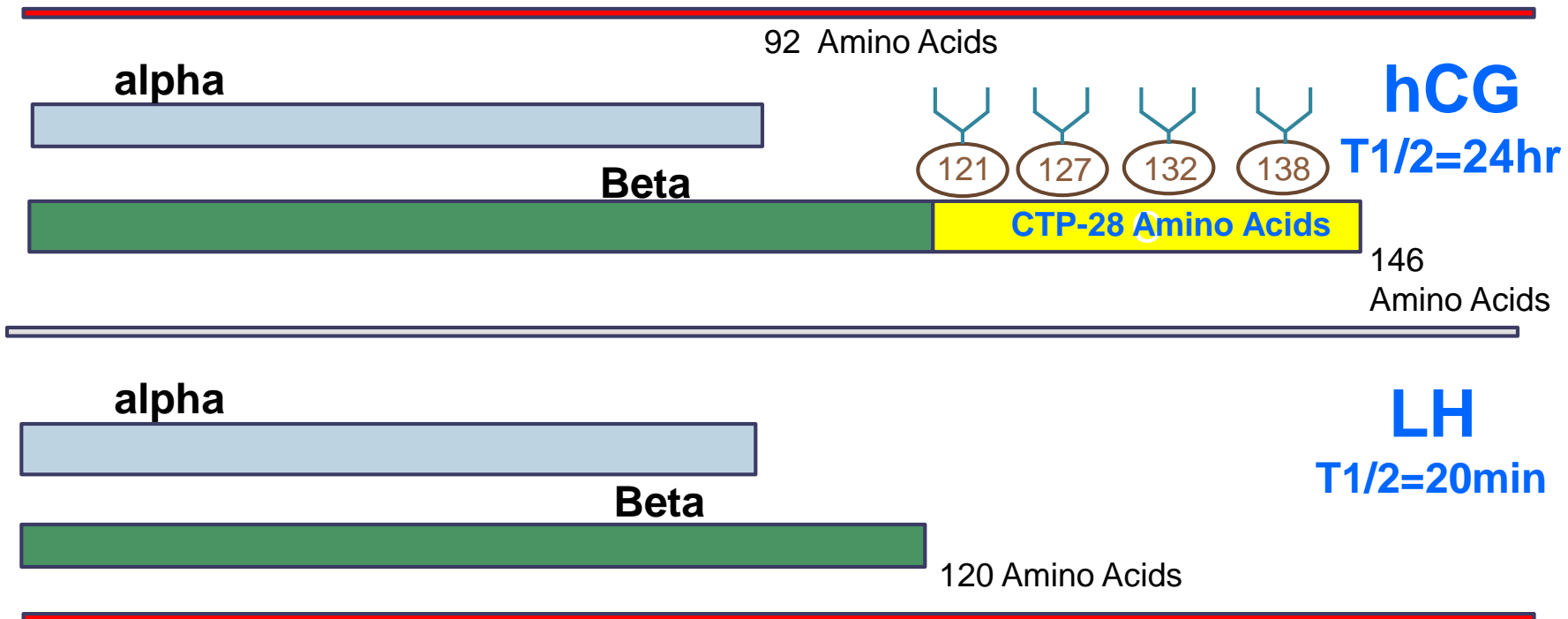
# Principal Investigators

Thank you all !!!

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# The Power Of C-Terminal Peptide (CTP)

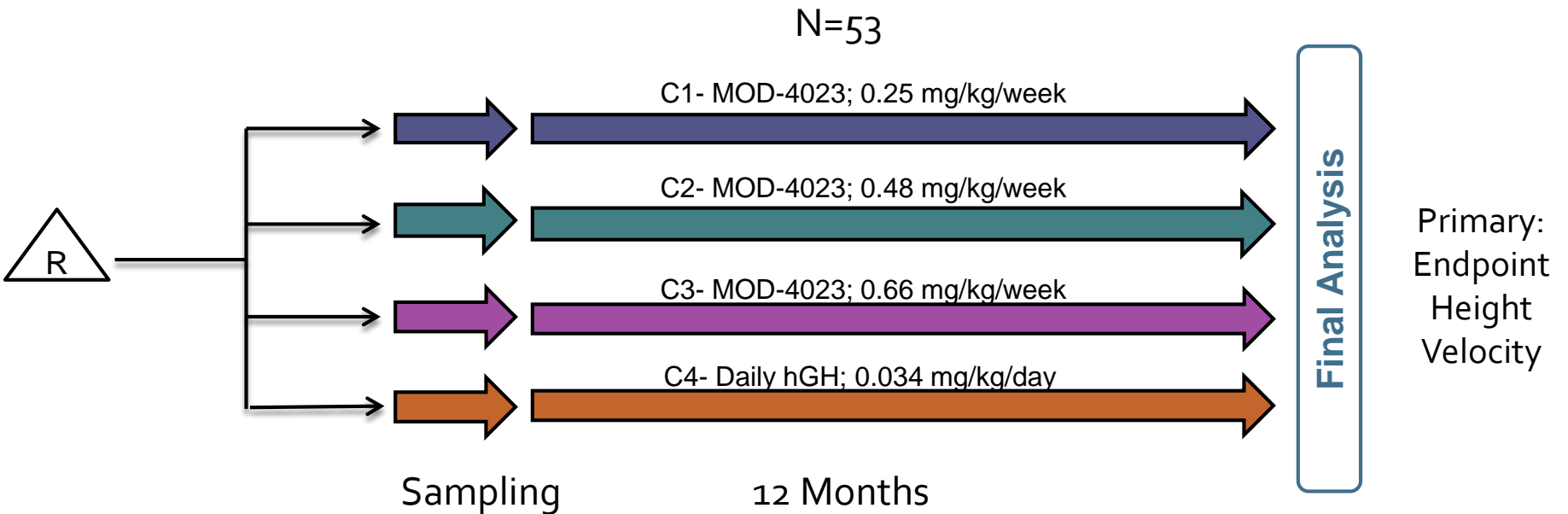
## Created By Nature During Evolution



To Extend Circulation Time of Functional Proteins

# CP-4-004 Global Phase 2 Study in Pediatric Growth Hormone Deficient Patients

# Study Design



PK and PD samples were collected after the 2<sup>nd</sup> dose at the final randomized dose

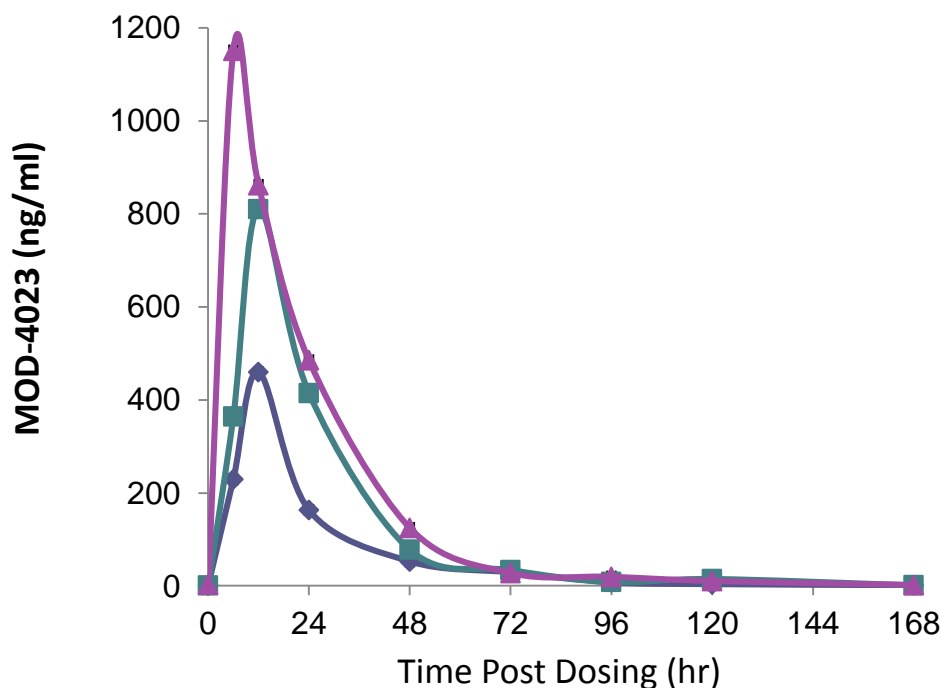
# Baseline Characteristics (N=53)

	<b>MOD-4023</b> 0.25 mg/kg/week Cohort 1 (n=13)	<b>MOD-4023</b> 0.48 mg/kg/week Cohort 2 (n=15)	<b>MOD-4023</b> 0.66 mg/kg/week Cohort 3 (n=13)	<b>Daily hGH</b> 0.034 mg/kg/day Cohort 4 (n=11)
<b>Age (yrs)</b>	<b>6.92</b> ±2.19	<b>6.53</b> ±2.20	<b>7.08</b> ±2.40	<b>6.43</b> ±1.79
<b>Ht SDS</b>	<b>-3.64</b> ±0.97	<b>-3.72</b> ±0.87	<b>-4.21</b> ±1.45	<b>-4.22</b> ±1.58
<b>Ht SDS – TH SDS</b>	<b>-3.22</b> ±0.95	<b>-3.00</b> ±0.70	<b>-3.36</b> ±1.54	<b>-3.68</b> ±1.70
<b>HV SDS</b>	<b>-2.93</b> ±1.42	<b>-2.68</b> ±1.00	<b>-3.01</b> ±1.42	<b>-3.29</b> ±1.91
<b>Peak GH (ng/nl)</b>	<b>3.93</b> ±3.15	<b>4.13</b> ±2.64	<b>3.97</b> ±2.97	<b>3.82</b> ±2.78
<b>IGF-I SDS</b>	<b>-2.13</b> ±0.85	<b>-2.13</b> ±0.77	<b>-1.97</b> ±0.83	<b>-2.15</b> ±0.94

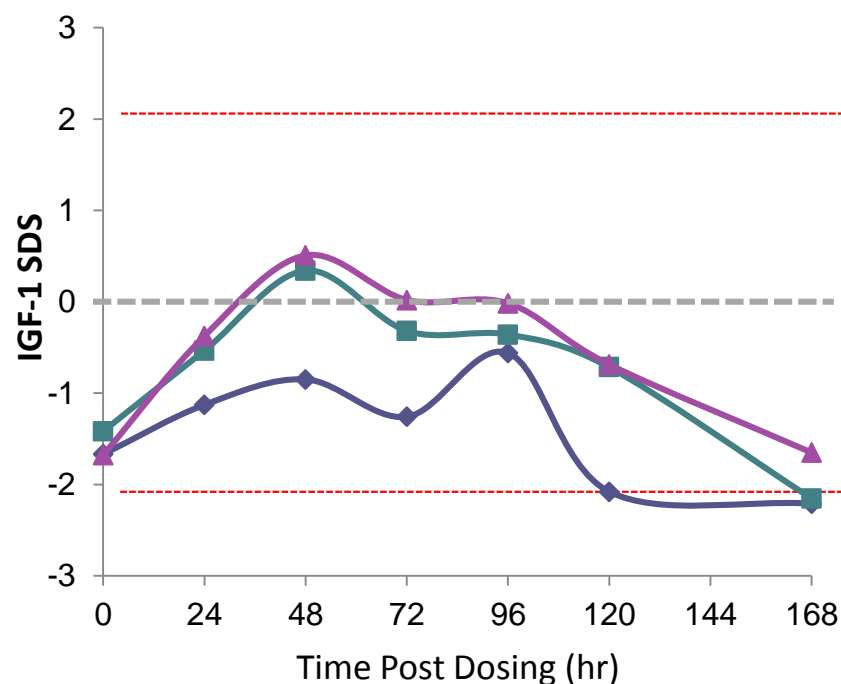


# MOD-4023 Concentrations and IGF-1 SDS Weekly Profile (After 2<sup>nd</sup> Dose )

## Serum MOD-4023 concentration

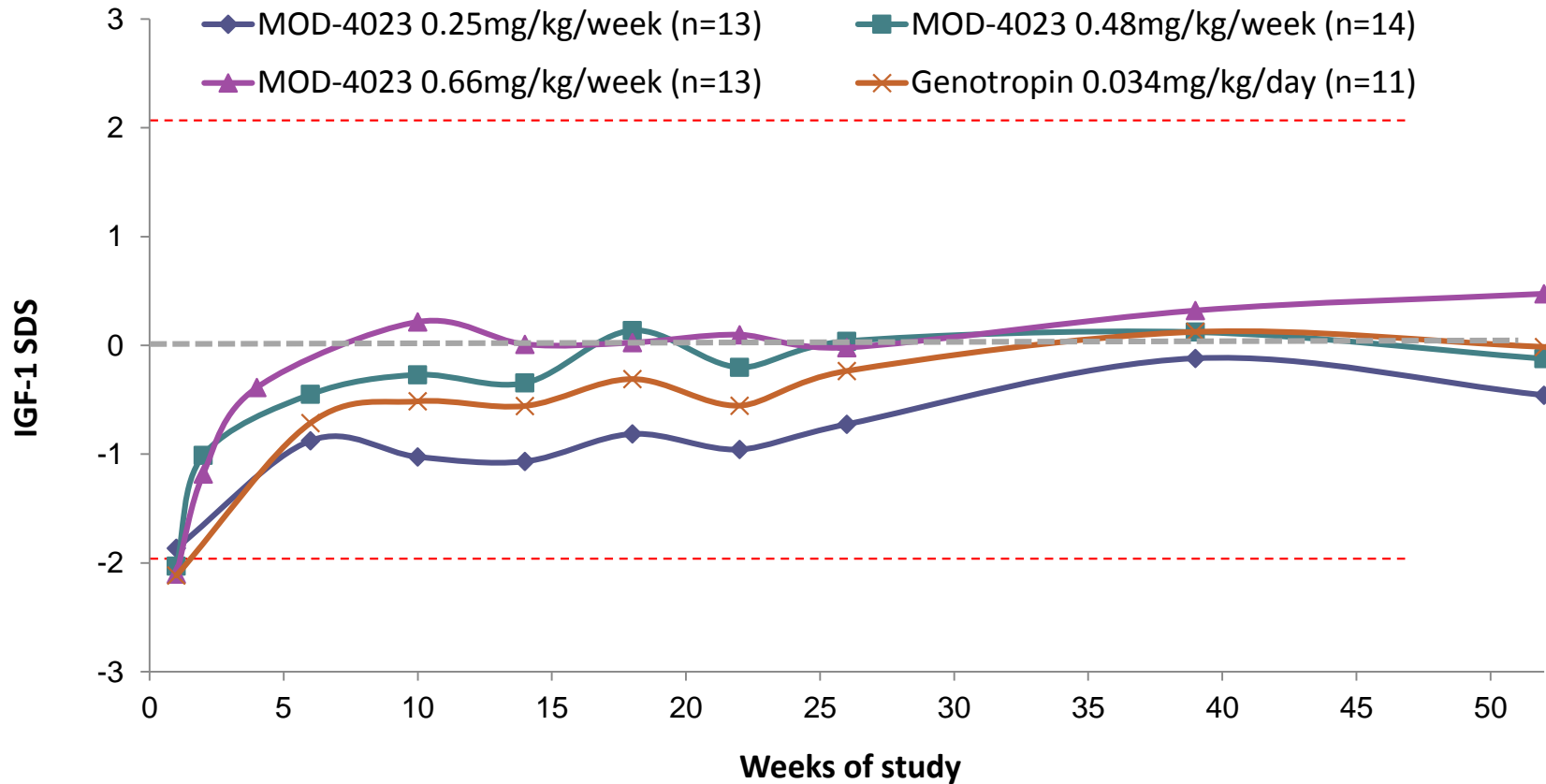


## IGF-1 SDS

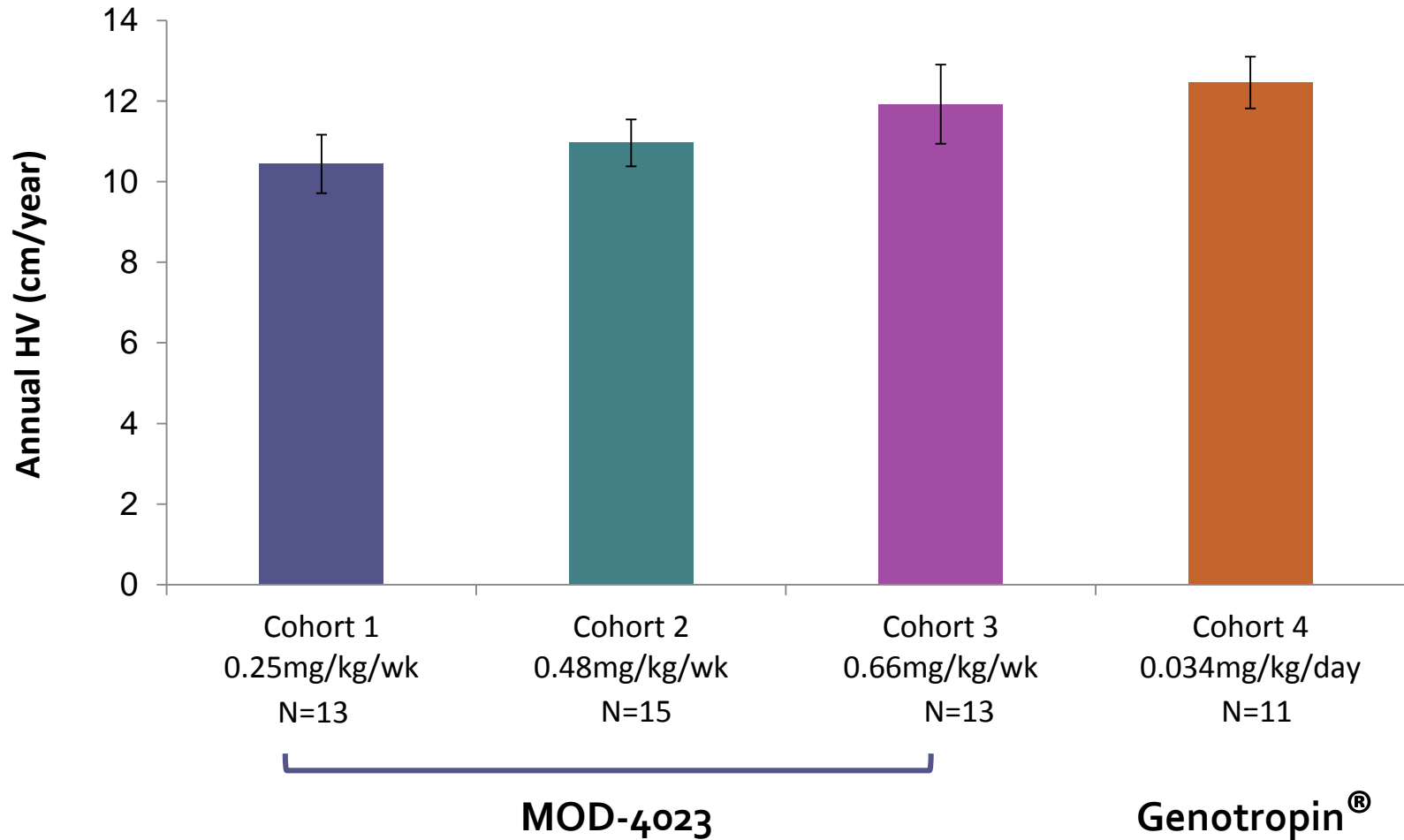


- ◆ MOD-4023 0.25mg/kg/week (n=13)
- MOD-4023 0.48mg/kg/week (n=15)
- ▲ MOD-4023 0.66mg/kg/week (n=13)

# IGF-1 SDS Profile; Day 4 Post-Dose During 12 Months Period.

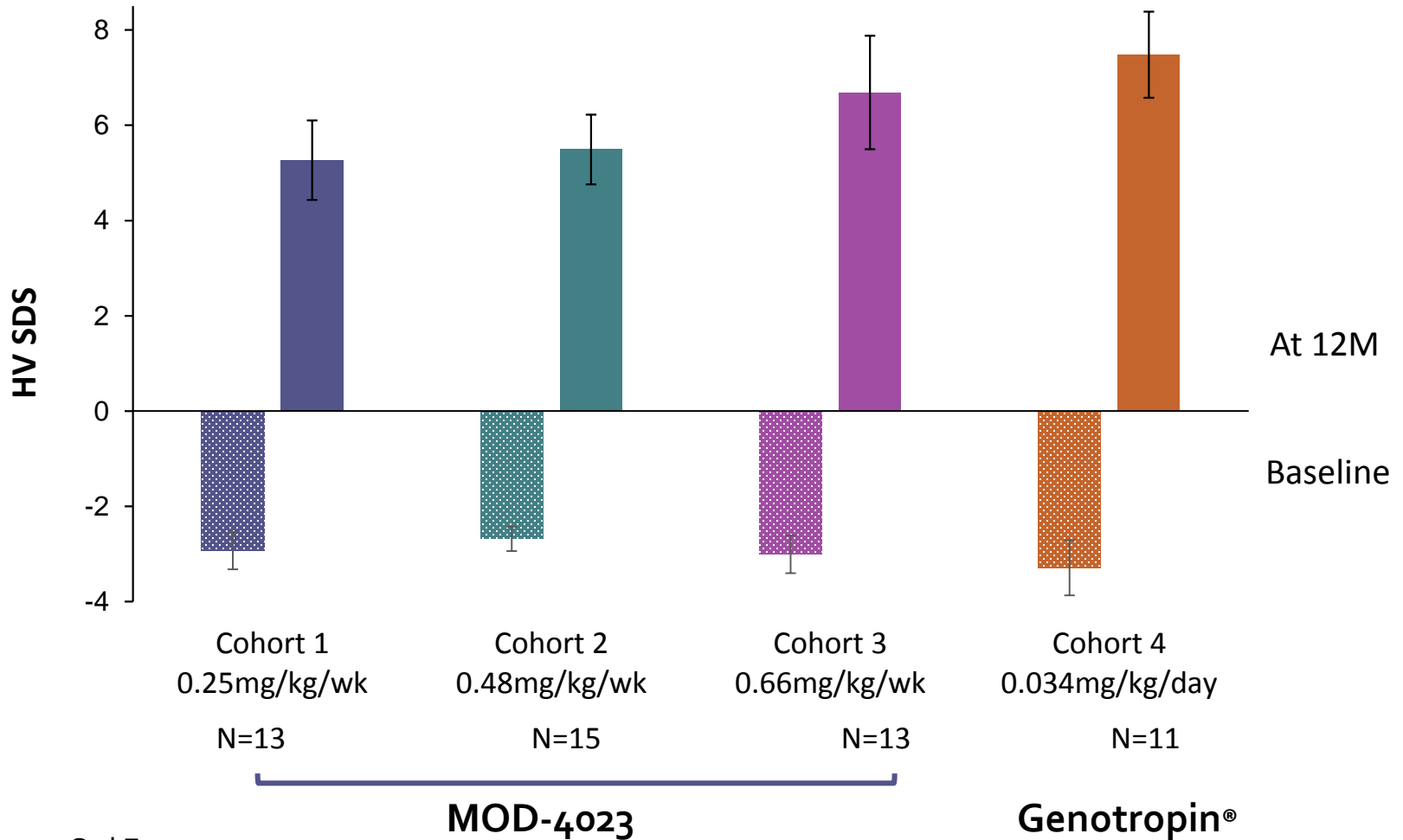


# First-year HV (N=53)



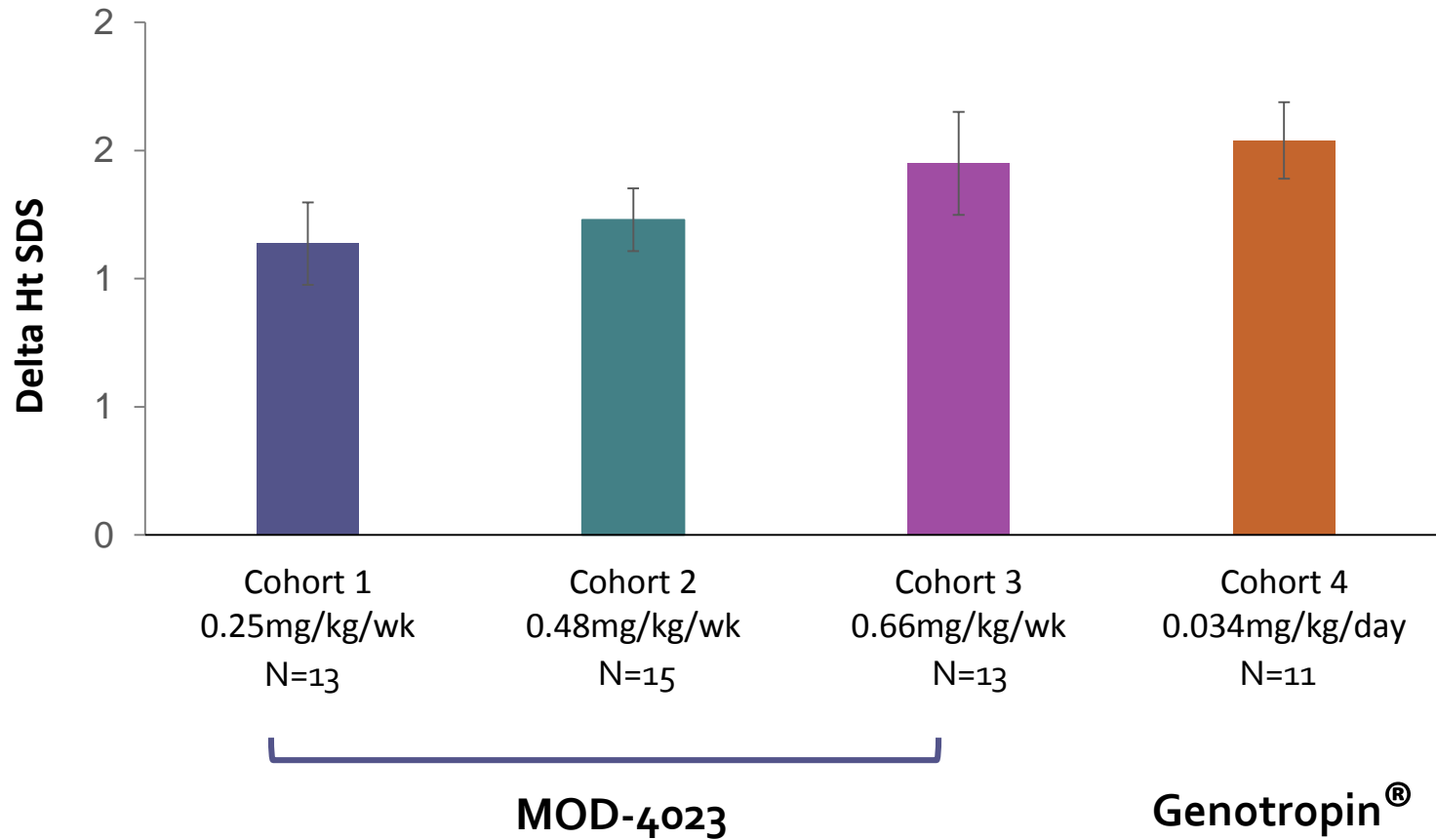
\* Mean  $\pm$  Std Err

# HV SDS at Baseline and 12 Months (N=53)



\* Mean ± Std Err

# Delta Ht SDS at 12 Months (N=53)



\* Mean  $\pm$  Std Err

# Safety Profile

- No severe or serious adverse events (AEs) with daily r-hGH or weekly MOD-4023
- No lipoatrophy
- No clinically significant local tolerability issues
- No neutralizing antibodies or related AEs

# MOD-4023: Summary & Perspective

- Weekly administration of MOD-4023 provides similar growth response as daily GH
- MOD-4023 weekly administration provides comparable safety profile to daily hGH in pediatric GHD

Based on the promising phase 2, the company will initiate a global pivotal phase 3 study in pre-pubertal GHD children



**Thank You**