

A Cross-sectional Survey on Crinecerfont and Quality of Life of Adults with Classic Congenital Adrenal Hyperplasia in the United States Participating in CAHtalyst® Adult Open-Label Extension Study

Sonal Vaid¹, Jessica Abramowitz², Irina Bancos³, Henry Kim Cheng⁴, Jean L. Chan⁴, Robert H. Farber⁴, Nandini Hadker⁵, Sanjay Keswani⁴, Hyunwoo Kim⁴, Vivian H. Lin⁴, Natalie Nokoff⁶, Eiry Roberts⁴, Maamoun Salam⁷, Brittany Smith⁵, Oksana Hamidi²

¹National Institutes of Health Clinical Center, Bethesda, MD, USA; ²University of Texas Southwestern, Dallas, TX, USA; ³Mayo Clinic, Rochester, MN, USA; ⁴Neurocrine Biosciences, Inc., San Diego, CA, USA; ⁵Trinity Life Sciences, Waltham, MA, USA; ⁶University of Colorado Anschutz, Aurora, CO, USA; ⁷Washington University School of Medicine, St. Louis, MO, USA

INTRODUCTION

- Classic congenital adrenal hyperplasia (CAH) is a rare inherited condition characterized by cortisol deficiency and increased adrenocorticotropic hormone (ACTH) secretion, usually leading to excess adrenal androgen production^{1,2}
- While glucocorticoids (GCs) are necessary to replace endogenous cortisol deficiency, doses higher than needed for cortisol replacement (supraphysiologic) have historically been used to reduce ACTH and androgens^{2,3}
- Achieving the optimal balance of androgen control and GC exposure is critical to reduce impacts of excess ACTH and androgens (e.g., advanced bone age, reduced fertility) and supraphysiologic GC exposure (e.g., cardiometabolic comorbidities, osteoporosis)¹⁻³
- Moreover, CAH is associated with reduced quality-of-life (QoL), such as poor sleep, fatigue, lack of confidence, anxiety, feelings of hopelessness, and difficulties in daily functioning^{4,5}
- Crinecerfont is a first-in-class CRF type 1 receptor (CRF₁) antagonist that is FDA-approved as an adjunct to GC replacement to control androgens in patients with classic CAH (4 years and older), including salt-wasting and simple virilizing forms, enabling GC dose reduction⁶
- In the CAHtalyst® Adult and CAHtalyst Pediatric studies, sustained reductions in GC doses while maintaining androgen control were observed in participants who received ≥18 months of crinecerfont treatment⁷⁻¹⁰
- Based on survey responses from participants who received ≥18 months of crinecerfont in these clinical trials, treatment satisfaction and QoL changes associated with long-term crinecerfont use were assessed

METHODS

- Participants from US sites in CAHtalyst Adult who received ≥18 months of crinecerfont treatment were invited to take the survey on their final visit of the trial's open-label extension (OLE) period
- This custom, quantitative survey assessed overall treatment satisfaction and patient-reported outcomes across multiple aspects of their overall health and daily life
 - Respondents were asked to compare their experience in the most recent month on crinecerfont with their experience prior to starting the trial
 - Respondents who indicated improvement were asked if it was meaningful
- The first 9 participants who completed the survey and expressed interest were included in structured follow-up interviews
- Preliminary findings from the analogous, ongoing CAHtalyst Pediatric survey are also presented

RESULTS

- Responses from 48 participants in CAHtalyst Adult who completed the survey were included for analysis (Figure 1)
 - Preliminary parent/caregiver responses for 29 participants in CAHtalyst Pediatric were also analyzed
- Among participants who completed the OLE and responded to the survey, overall satisfaction with crinecerfont was high, with no participants reporting dissatisfaction with treatment (Figure 2)
- Most participants reported meaningful improvements across multiple aspects of their daily lives (Figure 3)
- Nine adult participants were interviewed; six themes were identified through thematic analysis; representative quotes are presented in Figure 4

LIMITATIONS

- Survey participation was limited to participants who chose to continue on open-label crinecerfont for ≥18 months, which may have introduced selection bias toward those with more favorable treatment experiences
- In addition, these were self-reported outcomes assessed retrospectively, which may have resulted in recall bias
- This study captured patient experiences at a single time point rather than longitudinally, limiting the ability to infer causal relationships between treatment and outcomes

Figure 1. Selection of Adult Survey Sample

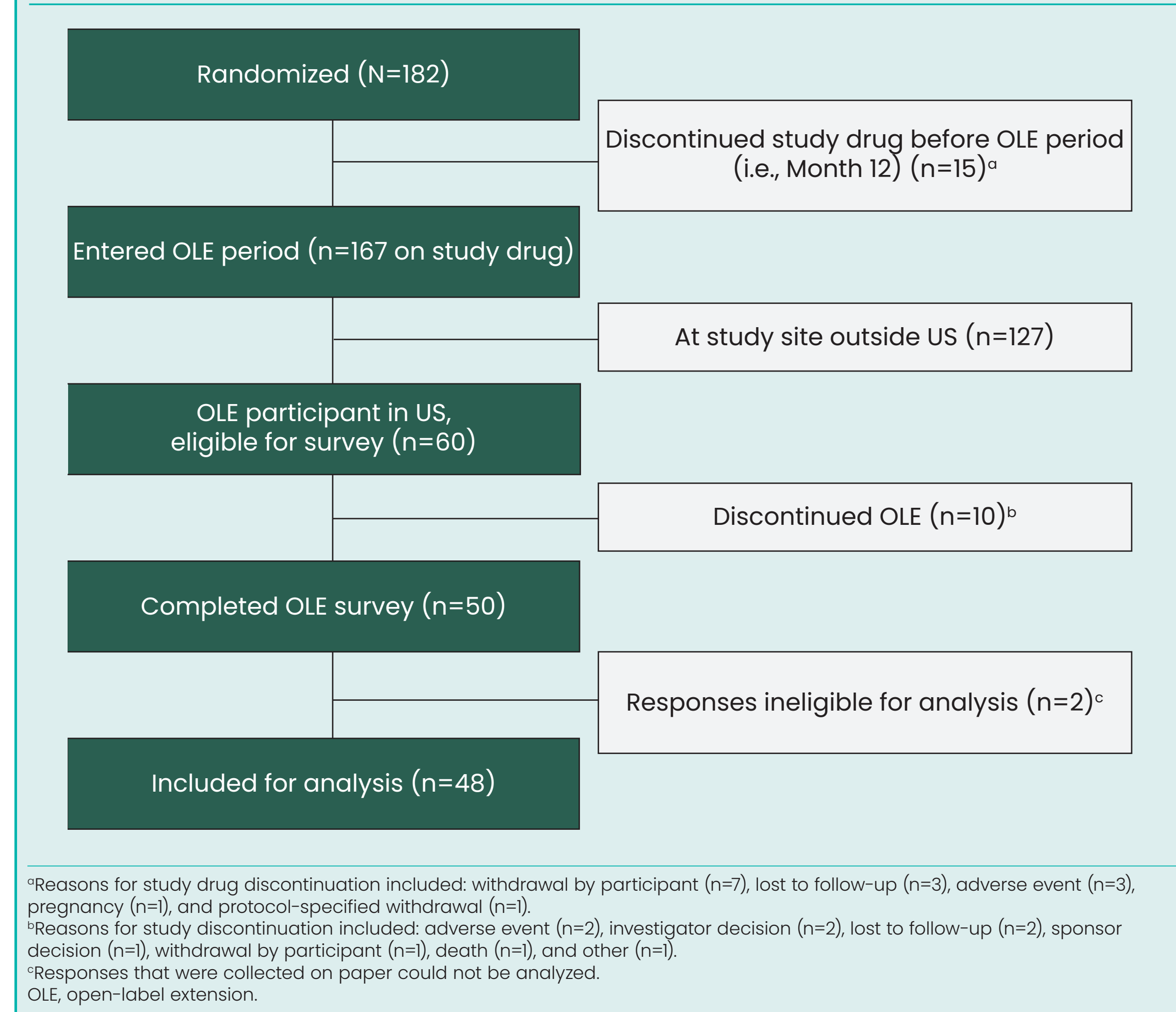


Figure 2. Overall Satisfaction With Crinecerfont

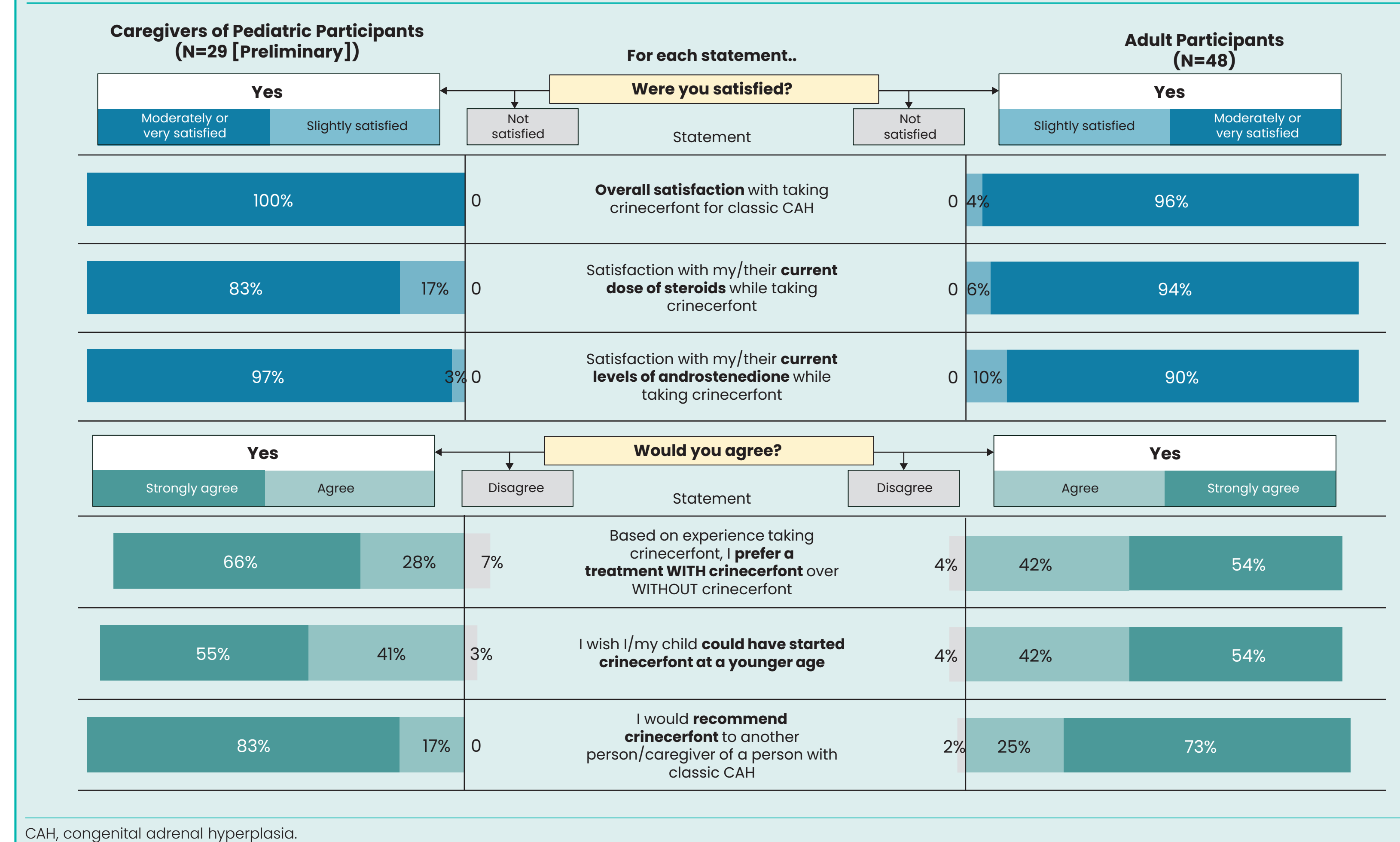


Figure 3. Survey Responses

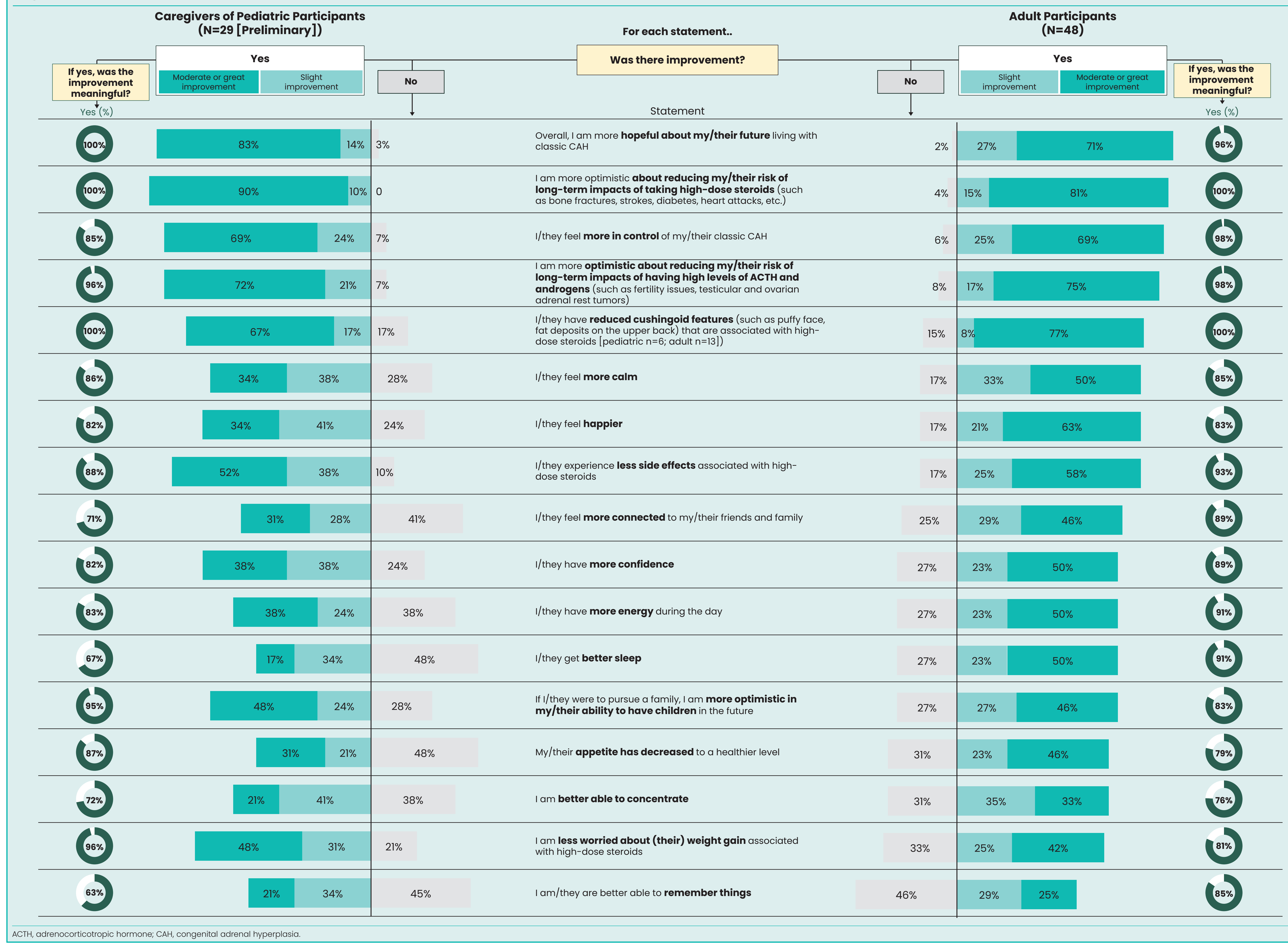


Figure 4. Themes and Quotes from Adult Patient Interviews (n=9)

Crinecerfont Redefined What "Normal" Feels Like

Tangible Improvement and Optimism in Reproductive Health

Improved Overall Sleep and Less Fatigue

Positive Body Image and Appearance

Improved Mental Health Status

Positive Impact of Bone Health on Daily Life and Functioning

Quotes:

- "All of these girls that are getting on this drug before puberty are [...] going to have a world of difference." - Female participant, 40s
- "I never ever got my period until I started crinecerfont." - Female participant, 40s
- "This is the first time in my life where I feel like I can actually feel, like, physically strong and resilient." - Male participant, 20s
- "Now I have like a whole lot of mental and physical energy to do things that are, like, way more worth spending your life thinking about and doing." - Male participant, 20s
- "The sleeping better at night is a huge deal." - Female participant, 40s
- "I feel better about myself with the weight loss afforded with the lower steroid dose thanks to taking this new drug." - Female participant, 40s
- "I think mentally it chilled me out a lot. It just calmed me down." - Male participant, 50s
- "It helped with a confidence component... like I could stay in my own skin and not feel like I had to run away." - Female participant, 40s
- "This morning, I played pickleball for the first time ever with my son. And I don't know that I would have done that before because I would have worried about hurting myself." - Female participant, 50s

CONCLUSIONS

- All adult participants and caregivers of pediatric participants who received ≥18 months of crinecerfont were satisfied with their treatment
- In addition, >90% reported wishing that they had started crinecerfont sooner, feeling more hopeful about their future, and feeling more in control of their CAH
- Most participants reported experiencing fewer GC-related side effects (e.g., puffy face) and less worry about the risk of GC-related consequences such as weight gain and osteoporosis
- In addition, many participants reported improvements in mental health (e.g., happier, calmer, more confident) and energy levels
- These favorable responses were consistent with favorable clinical outcomes and high rates of study completion, highlighting the potential benefits of crinecerfont on patients' QoL and function

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