Tolerability and Efficacy of ZYN002 Cannabidiol (CBD) Transdermal Gel in Children and Adolescents With Autism Spectrum Disorder: An Open-Label Phase 2 Study (BRIGHT [ZYN2-CL-030])

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BACKGROUND

- Autism spectrum disorder (ASD) is a complex neurodevelopmental disorder characterized by difficulties with behaviors, communication, and reciprocal social interaction. 1
- Current management options for ASD symptoms are restricted to behavioral, emotional, and social problems (Figures 4 and 5).
- Most caregivers indicated improvements in behavioral, emotional, and social problems (Figure 4).

OBJECTIVE

- BRIGHT (ZYN2-CL-030) is an exploratory, single-center, open-label Phase 2 study evaluating the safety and tolerability and efficacy of ZYN002 in children and adolescents with ASD who are 4 to <18 years old.

METHODS

- The study enrolled patients with Clinical Global Impression (CGI)– Severity score ≥4 (moderate or greater) and Aberrant Behavior Checklist (CBCL) Irritability score ≥18.
- The mean baseline ABC-C Irritability score was 30.3 (Table 1).

RESULTS

BASELINE DEMOGRAPHICS AND DISEASE CHARACTERISTICS

- Of the 37 patients (mean age: 9.2 years) enrolled, 94% had moderate-to-severe symptoms per Autism Diagnostic Observation Schedule 2nd edition criteria (Table 1).
- The mean baseline ABC-C Irritability score was 30.3 (Table 1).

SAFETY RESULTS

- All AEs were mild (7%) or moderate (25%) and reported in 49% of patients (Table 2).

CONCLUSIONS

- Through 14 weeks of treatment, BRIGHT provides initial evidence supporting a positive benefit-risk profile for ZYN002 when administered in addition to stable standard of care in children and adolescents with moderate to severe ASD.
- ZYN002 showed improvement in all ASD measures (ABC-C, AIM, PRAS-ASD, CGI and Qualitative Caregiver Assessments).
- Further controlled studies are warranted in this difficult-to-treat population.

REFERENCES


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