ZYN002 (CBD) is a pharmaceutically manufactured CBD transdermal gel in clinical development for the treatment of behavioral symptoms in preclinical models of FXS.8,9 ZYN002 is a cannabinoid receptor agonist that has shown promise in preclinical models of FXS.8,9 Fragile X syndrome (FXS) is a rare genetic condition characterized by a 1 in 4000-6000 (females) to 1 in 2500-3000 (males) lifetime risk of disease.10 FXS involves genetic deletions or less commonly, mutations on the X chromosome that prevent or inhibit proper functioning of the FMR1 gene.11 FXS is characterized by a broad spectrum of behavioral, cognitive, and medical characteristics.12

OBJECTIVE

To evaluate the potential benefit of ZYN002 on ABC-UI in pediatric and adolescent patients with FXS

PATIENTS

Inclusion criteria – a diagnosis of FXS was required for study entry.

PATIENTS

STUDY DESIGN AND TREATMENT

ZYN002 (CBD) is a novel, open-label, phase 2 study to evaluate the efficacy and safety of ZYN002 in pediatric and adolescent patients with FXS (Figure 1.)

RESULTS

IMPROVEMENT IN ABC-UI SCORE WITH ZYN002 TREATMENT

Figure 2. ABC-UI score at screening with reference to literature describing HUI in other childhood conditions

REFERENCES

• The ABC-UI, HUI2, and HUI3 may not be directly comparable, and direct comparisons have not been made.

CONCLUSIONS

• The ABC-UI score in ZYN002-treated pediatric and adolescent patients with FXS was significantly higher than that in historically described children and adolescents in literature describing HUI in other childhood conditions, suggesting a potential broad spectrum of activity for ZYN002 in the treatment of FXS symptoms. This improvement was noted in all domains of the ABC-UI, including anxiety, irritability, depression, hyperactivity, and cognition. These findings suggest that ZYN002 may have therapeutic potential in the treatment of FXS symptoms, highlighting the need for further investigation in this patient population.

LIMITATIONS

• The ABC-UI score in ZYN002-treated pediatric and adolescent patients with FXS was significantly higher than that in historically described children and adolescents in literature describing HUI in other childhood conditions, suggesting a potential broad spectrum of activity for ZYN002 in the treatment of FXS symptoms. This improvement was noted in all domains of the ABC-UI, including anxiety, irritability, depression, hyperactivity, and cognition. These findings suggest that ZYN002 may have therapeutic potential in the treatment of FXS symptoms, highlighting the need for further investigation in this patient population.

Figure 3. Scatter plot of ABC-UI score and CGI-S score at signal timepoint during treatment with ZYN002.