The Long-Term Safety and Sustained Efficacy of ZYN002 Cannabidiol Transdermal Gel in Children and Adolescents With Fragile X Syndrome (ZYN2-CL-017)

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BACKGROUND

• Fragile X syndrome (FXS), a condition driven by a mutation in the FMR1 gene, is the most common single gene cause of autism spectrum disorder (ASD)1
• Disruption in the endocannabinoid system as a result of the FMR1 gene is actively enrolling patients with complete (100%) methylation of their FMR1 gene

- Objective
  - To evaluate the long-term safety and sustained efficacy of ZYN002 cannabidiol transdermal gel in children and adolescents with FXS

- Study design
  - ZYN2-CL-017 is an ongoing, open-label extension trial starting in December 2017
  - ZYN2-CL-033, RECONNECT, is an ongoing Phase 3, placebo-controlled, double-blind, parallel-group clinical trial (NCT03614663)

- Population
  - Ineligible to continue in trial, n=33

- Treatment with ZYN002
  - Placebo or ZYN002

SAFETY RESULTS

- Improvements were seen in ABC-CFXS SA in the full population, with the greatest improvements in patients with complete methylation of their FMR1 gene

EFFICACY RESULTS

- ZYN002 was safe and well tolerated in the ZYN2-CL-017 trial

- Most TEAEs were related to conditions commonly reported in FXS and 22q11.2 deletion syndrome (22q)

- Key outcomes
  - Social Avoidance (SA) subscale on the ABC-CFXS

REFERENCES


Acknowledgements

Disclosures

This presentation is intended to communicate scientific and medical information about medical products and devices to healthcare professionals. The presentation is an investigational treatment. This means that it is not approved for commercial distribution by government regulatory bodies, including the US Food and Drug Administration (FDA). This presentation is intended for healthcare professionals. ZYN2-CL-017 was presented at the 56th Annual Meeting of The Society for Neuroscience, November 4-8, 2016, Washington, DC.

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