



ASX Release

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100th Research Participant Recruited in Autism Observational Study

Preliminary analysis of patient data provides unique insights into impact of medicinal cannabis on autism

- **Analysis identified doses and formulations of cannabis that improve behaviour, anxiety, sleep, irritability and hyperactivity**
- **First study to identify marked differences in CBD and THC usage profiles between low, med, and high severity groups**
- **Outcomes informing design of first-in-class randomised autism clinical trial**

Zelda Therapeutics Limited (ASX: ZLD, OTCQB: ZLDAF) reports that its observational autism study has achieved a major milestone with the enrolment of its 100th participant and has generated important information regarding utilisation of medicinal cannabis that is associated with improved behavioural outcomes in autistic children. The U.S.-based study is being conducted at Children’s Hospital of Philadelphia (CHOP).

This observational study is seeking to use parent-reported data to better understand the efficacy of a broad range of medicinal cannabis products in children diagnosed with autism. This study is the first to combine a comprehensive panel of key efficacy, pharmacological and safety read-outs with a community-based real-world assessment of cannabis usage in children with autism. Currently, there are 103 participants enrolled in the study, with the final target of 150 expected by early 2020.

Some key outcomes from the preliminary statistical analysis are:

- The average age of children enrolled in the study was 10 years old.
- The 103 participants enrolled in the study were using 76 different medicinal cannabis products, with over 42% using multiple products at the same time. The extensive range of medicinal cannabis products used to treat autism in children reflects the lack of clinically-validated data to help inform clinicians and parents regarding the best treatment options.
- Medicinal cannabis treatments were associated with parent-reported improvements in key autism-related behaviours and other associated features, including anxiety,

sleep, irritability and hyperactivity as reported by parents/caregivers. The extent of improvement was impacted by the presence of different cannabinoids (THC-only, CBD-only or combinations of both) at different doses.

- Study identified marked differences in CBD and THC dosages between autism severity groups (low, med, high) as reported by parents/caregivers.
- Participants could be classified as 'Responders' and 'Non-Responders' based on the level of improvement in behavioural scores that were reported by parents following treatment with medicinal cannabis. Analysis revealed key differences between these groups in terms of the doses and formulations of medicinal cannabis that were used.

Zelda's Managing Director, Dr Richard Hopkins, commented, "*We're very encouraged by the preliminary analysis of the data from this observational study. Results show that treatments with a range of cannabis medicines were associated with improvements in several key behavioural markers that are prominent in children diagnosed with autism. This highlights the potential for medicinal cannabis to improve outcomes for autistic children.*

To our knowledge, this is the first study to report differences in autism severity based on responses to medicinal cannabis treatments. The ability to stratify patients according to various clinical criteria could help us better predict whether patients will be more likely to benefit from medicinal cannabis treatments.

While these initial findings are encouraging, we need to be cautious interpreting the results from observational studies such as ours, where parent-reported data is used and where there is limited baseline information available prior to treatment, which can result in significant bias.

This situation highlights the need for well-designed clinical trials to underpin and inform treatment options. Zelda is seeking to address this by using the outcomes from this study to inform the design of a randomised blinded clinical trial for autistic children, which is being planned for later this year.

Zelda has established an early mover advantage with its clinical validation strategy for medicinal cannabis. The company is now well-positioned as the sector becomes increasingly regulated and the clinical requirements to support prescription cannabis products become much more demanding."

Tim Slate
Company Secretary

About Zelda Therapeutics (www.zeldatherapeutics.com)

Zelda Therapeutics Ltd is an Australian-based bio-pharmaceutical company developing a range of cannabinoid-based formulations for the treatment of a variety of medical conditions. The Company is undertaking:

- A **human clinical trial program** focused on insomnia, autism and opioid reduction with activities in Australia and the USA.
- A **pre-clinical research program** examining the effect of cannabinoids in breast, brain and pancreatic cancer as well as research examining the potential for cannabinoids to treat diabetes-associated cognitive decline.

The company conducts this work in partnership with world-leading researchers and organisations including Complutense University in Madrid, Spain; Curtin University in Perth, Western Australia; the Telethon Kids Institute in Perth; the University of Western Australia, in Perth; St. Vincent's Hospital in Melbourne, Australia; and the Children's Hospital of Philadelphia (CHOP) in the United States.

Zelda has also formed a strategic partnership with European medicinal cannabis group HAPA Medical BV, to access HAPA's EU- GMP grade manufacturing capabilities and accessing its German distribution network providing a credible and rapid path to commercialisation for successful clinically validated formulations.

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