ASX ANNOUNCEMENT

FDA grants Actinogen Rare Paediatric Disease Designation in FXS

Sydney, 5 February 2021. Actinogen Medical ASX: ACW ('ACW 'or 'the Company') is pleased to announce that the **United States Food and Drug Administration (FDA)** has granted Actinogen's drug Xanamem **Rare Paediatric Disease Designation (RPDD)** for the treatment of Fragile X syndrome (FXS). Fragile X syndrome is a rare and serious genetic disorder, typically diagnosed in children but with life-long symptoms, that is characterised by a range of development problems including behavioural problems, autism features, severe anxiety, cognitive impairment and disordered sleep .

Key highlights:

- **FDA granted Actinogen RPDD for Xanamem in FXS, providing for priority review of Xanamem for FXS and a second, transferable RPDD priority review voucher, if Xanamem is approved for FXS**
- **FXS is a rare and serious genetic disorder characterised by a range of neurological developmental problems**
- **XanaFX, a phase II proof-of-concept clinical trial, will evaluate Xanamem's effect on core symptoms associated with FXS: behavioural problems, severe anxiety and sleep, and is expected to commence in CY21**

Dr. Bill Ketelbey, CEO and MD of Actinogen Medical, commented:

"We are delighted to receive the Rare Paediatric Disease Designation from the FDA. The significant strategic advantages from this approval include commercial, development and regulatory benefits for the development of Xanamem in FXS, with priority review designed to increase overall speed to market. Additionally, RPDD designation includes the potential for the Company to receive a second, transferable priority review voucher that holds substantial additional value in it's own right

Actinogen has devoted increased resources to broadening Xanamem's clinical development pipeline, with Fragile X syndrome selected as the second indication to pursue alongside Alzheimer's disease. FXS is a rare genetic and often debilitating disease that is usually diagnosed in children at around 3 to 5 years of age. Management of FXS is often complex, with life-long treatment required for patients. FXS presents as a significant unmet medical need as there are currently no approved drugs to treat the condition. We will be investigating Xanamem as a potential treatment for the severe anxiety, sleep and behavioural problems associated with FXS, in our XanaFX clinical trial, which is expected to commence later this year."

The US FDA has granted Actinogen's drug Xanamem, RPDD for the treatment of FXS in patients under 18 years of age. The FDA's RPDD program is designed to incentivise the development of drugs for rare childhood illnesses.

Actinogen will benefit from the RPDD with increased speed to market from priority review of Xanamem in FXS. In the USA, drugs that qualify for RPDD may also receive a **Priority Review Voucher (PRV)** from the FDA upon marketing authorisation. A second voucher could be used for a different indication, and be tradeable, hence coming with its own substantial commercial value. In recent years, biopharma companies have on-sold PRVs to other pharmaceutical companies for an average over US$100m.

XanaFX, Actinogen's planned phase II proof-of-concept study, will assess Xanamem's effect on anxiety, sleep, and behavioural problems in ~40 adolescents with Fragile X syndrome. The trial is expected to commence in CY21 and will be conducted in partnership with the Murdoch Children's Research Institute (MCRI). The primary endpoints will evaluate the safety and tolerability of Xanamem in this population, with secondary endpoints assessing the improvement in anxiety, sleep, behavioural problems, communication, socialisation and daily living skills. The trial is fully funded and expected to complete within 12 months of commencement.

Clinical trials for rare diseases typically enroll fewer patients across a smaller number of clinical trials, potentially decreasing costs and shortening development timelines. Importantly, the rapid data generated for FXS could be leveraged across other paediatric conditions, potentially decreasing the time to market for these additional indications, presenting a significant potential upside with FXS-related conditions, such as Autism Spectrum Disorder.

ENDS

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Announcement authorised by the Board of Directors of Actinogen Medical

About Actinogen Medical

Actinogen Medical (ASX:ACW) is an ASX-listed biotechnology company developing novel therapies for neurological, psychiatric and metabolic diseases associated with chronically elevated cortisol. The company is currently developing its lead compound, Xanamem, as a promising new therapy for Alzheimer's disease, Fragile X syndrome, schizophrenia and diabetes. The cognitive dysfunction, behavioural abnormalities, and neuropsychological burden associated with these conditions is significantly debilitating for patients, and there is a substantial unmet medical need for new and improved treatments.

About Xanamem™

Xanamem's novel mechanism of action works by blocking the production of intracellular cortisol – the stress hormone – through the inhibition of the 11β-HSD1 enzyme in the brain. There is a strong association between persistent stress and the production of excess cortisol that leads to detrimental changes in the brain, affecting memory, cognitive function and behaviour and neuropsychological symptoms. The 11β-HSD1 enzyme is particularly highly concentrated in the hippocampus and frontal cortex, areas of the brain impacted by a number of diseases and disorders, including Alzheimer's disease, Fragile X syndrome, schizophrenia, diabetes and other conditions associated with chronically raised cortisol.

The Company's XanaHES Phase I trial exploring the safety and tolerability of Xanamem 20mg once daily in healthy elderly volunteers, confirmed the drug's safety profile with no treatment-related serious adverse events. Additionally, the trial demonstrated that Xanamem produced a statistically significant improvement in cognition over placebo, which, along with other recently generated data, confirms 11β-HSD1 inhibition by Xanamem as a promising potential treatment for cognitive impairment associated with raised cortisol.

The Company plans to initiate Phase II studies of Xanamem in various disease areas in 2021, including MCI due to Alzheimer's disease, and Fragile X syndrome.

Xanamem is an investigational product and is not approved for use outside of a clinical trial by the FDA or by any global regulatory authority.

Xanamem™ is a trademark of Actinogen Medical.

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