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Ferrer and Verge Genomics Announce Agreement to Co-Develop Clinical-Stage ALS Therapy VRG50635

Verge Genomics, a leading clinical-stage biotechnology company, and Ferrer, an international B Corp pharmaceutical company with an increasing focus in rare neurological disorders, have announced a strategic collaboration to co-develop VRG50635, Verge's lead drug candidate for the treatment of sporadic and familial forms of amyotrophic lateral sclerosis (ALS), in Europe, Central and South America, Southeast Asia and Japan. VRG50635 is a potential best-in-class, small molecule inhibitor of PIKfyve, a therapeutic target for ALS discovered in diseased human tissues using CONVERGE®, Verge's all-in-human, AI-powered platform.

The collaboration combines Verge's all-in-human technology for target discovery and drug development and their leadership in clinical trial innovation with Ferrer's global expertise in clinical development, manufacturing, and commercialization. Under the terms of the agreement, Ferrer will obtain the exclusive rights to co-develop and commercialize VRG50635 for ALS in multiple regions outside of the United States of America. Verge has retained all rights to development and commercialization for VRG50635 for all uses in the United States and all countries outside the agreement.

"We're thrilled to work with Ferrer to progress VRG50635 through clinical development and towards potential commercialization. Ferrer has extensive experience navigating clinical development and regulatory landscapes across the globe. They also understand the complex and variable payer and reimbursement environments in the territories in which we will collaborate," said **Alice Zhang**, CEO and cofounder of Verge Genomics. "This partnership is another clear recognition of the value of our CONVERGE® platform and its ability to identify novel targets more successfully for complex diseases that can be rapidly translated to the clinic."

"We are pleased to partner with Verge Genomics as this is important news for people living with ALS, their families, and their caregivers. In line with our purpose to use business to fight for social justice, we are reinforcing our commitment to bring transformative therapeutic

solutions for people living with severe and debilitating diseases", said **Mario Rovirosa**, CEO at Ferrer.

"We believe VRG50635 represents a promising new approach to treating this devastating disease, and we look forward to combining our strengths to accelerate the development of this potential treatment. This is a very significant addition to our growing portfolio of treatments for rare neurological disorders while revalidating our strong commitment to the scientific community and the patients suffering from ALS", explained **Oscar Pérez**, Chief Scientific Officer at Ferrer.

VRG50635 is one of the first candidate drugs to enter the clinic that was entirely discovered and developed from an AI-enabled platform. It is a potent, orally bioavailable PIKfyve inhibitor that could improve survival in ALS patient neurons and has shown efficacy in multiple preclinical studies in ALS-relevant models of motor neuron degeneration[1,2]. Currently, VRG50635 is undergoing a Phase 1B Proof-of-Concept (PoC) study in Canada and several European Countries[3]. Verge's PoC study, which has been applauded for its cutting-edge design, incorporates innovative technology that makes it possible to collect dense amounts of unbiased, objective disease-relevant data to properly assess safety, tolerability, pharmacological dose response, and potential efficacy, including for disease modification, early in clinical development.

About Amyotrophic Lateral Sclerosis (ALS)
Amyotrophic Lateral Sclerosis (ALS), the most common motor neuron disease, is a progressive neurodegenerative disease of motor neurons in the brain and spinal cord, resulting in progressive paralysis, with death typically within 2 to 5 years of diagnosis. ALS is a rare disease with multifactorial etiology, and the precise pathogenic mechanism is still unknown. ALS typically occurs in people between 40-70 years old, slightly more men than women. It is caused by a multitude of factors: 10% familiar ALS, 90% sporadic ALS[4].

References

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