Rgenta Therapeutics to Present at the 19th Annual Huntington's Disease Therapeutics Conference

CAMBRIDGE, Mass., February 21, 2024 /PRNewswire/ -- Rgenta Therapeutics ("Rgenta" or the "Company") today announced it will participate at the 19th Annual Huntington's Disease Therapeutics Conference being held February 26-29, 2024, in Palm Springs, California.

As part of the annual conference, Travis Wager, Ph.D., President & CSO of Rgenta will participate in the *Targeting the DNA repair machinery to modulate somatic instability* session on Tuesday, February 27, 2024 at 2:00 PM PT. Dr. Wager's presentation, entitled, "*Developing oral small molecule splice modulators targeting PMS1 to treat repeat expansion diseases: Huntington's Disease (HD) and beyond*," will review the Company's discovery process, highlight the progress of Rgenta's oral small molecule splice modulators, and discuss their potential in treating the underlying causes of repeat expansion diseases.

The PMS1 gene is a key component of the DNA mismatch repair pathway, implicated in the pathological somatic trinucleotide repeat expansion observed in HD and other trinucleotide repeat expansion disorders such as Myotonic Dystrophy Type 1 (DM1), Fragile X Syndrome (FXS), and Friedreich's Ataxia (FRDA). Genome-wide association studies (GWAS) have identified PMS1, along with other genes in the mismatch repair pathway (e.g., MLH1, MSH3, PMS2, and FAN1), as genetic modifiers impacting the age of onset in HD. Functional studies, including the genetic knockout of PMS1 in HD mouse models, which stalled repeat expansion, further reinforce the gene's important role in the progression of somatic repeat expansions.

To date, there has been a lack of successful strategies for directly targeting the PMS1 protein with small molecule inhibitors. Rgenta is developing first-in-class oral small molecules, leveraging its integrative RNA-targeting small molecule discovery platform, which are designed to modulate PMS1 pre-mRNA splicing, curtailing the production of the aberrant PMS1 protein, and thereby potentially modifying the trajectory of somatic repeat expansion diseases. The Company's leading compounds have demonstrated the ability to decrease PMS1 RNA and protein levels in human cell lines and in animal models. Significantly, these molecules exhibit promising pharmacological efficacy in inhibiting repeat expansions in disease models and possess favorable pharmacokinetic profiles that facilitate crossing the blood-brain barrier.

For more information about the event, please visit the conference website.

About Rgenta Therapeutics

Rgenta Therapeutics is developing a pipeline of oral, small-molecule RNA-targeting medicines with an initial focus on oncology and neurological disorders. Our proprietary platform mines the massive genomics data to identify targetable RNA processing events and design small-molecule glues to modulate the interactions among the spliceosome, regulatory proteins, and RNAs. Our lead programs and unique approach are unlocking the therapeutic potential of historically undruggable targets in human diseases. Learn more at www.rgentatx.com.

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