Riaan Research Initiative partners with Rarebase to identify drugs that may assist patients with Cockayne Syndrome, a fatal neurodegenerative genetic disorder

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NEW YORK, NY – Riaan Research Initiative, a 501(c)(3) non-profit organization founded to accelerate and fund the development of treatments for Cockayne Syndrome, announces a new partnership with Rarebase, a public benefit biotech company based in Palo Alto, California, to screen FDA-approved drugs through its platform called Function. The collaborative effort is intended to find drugs that may help overcome the loss of function of CSA/ERCC8, one of the genes implicated in pediatric patients with the disease.

“Developing treatments for deadly genetic diseases and overcoming the glacial pace of drug discovery requires innovative thinking, and a robust, multi-faceted approach. There are many potential therapeutic remedies out there, and we must explore all possible avenues to help find the best ones for our children, especially when time is of the essence and lives are at stake. We’re hopeful that Rarebase will rapidly identify drugs that may alleviate some of the symptoms of Cockayne Syndrome,” said **Jo Kaur, Founder and Chair of Riaan Research Initiative**.

Rarebase will screen their compound library consisting of thousands of FDA-approved drugs and novel drugs to assess whether any small molecule therapies can compensate for the loss of CSA/ERCC8 function in Cockayne Syndrome patients. To date, no drugs or therapies have been approved to treat Cockayne Syndrome, a disease that is almost always fatal in children. If there are any hits on the drug screen, Rarebase will then validate the results on induced pluripotent stem cells, derived from Cockayne Syndrome patient blood samples. The Rarebase team expects to have initial results by Spring 2022, a rapid pace for drug development.

"We're thrilled to be collaborating with Riaan Research Initiative to help find treatments for patients with Cockayne Syndrome. There are thousands of approved drugs, and many of them can have applications beyond their routinely prescribed indication. If such treatments are out there for Cockayne Syndrome, we are committed to finding them using our Function platform," said **Onno Faber, Co-founder and CEO of Rarebase**.

Founded in June 2021, Riaan Research Initiative has helped launch and fund two research programs to develop treatments for Cockayne Syndrome, including CSA gene

replacement therapy at the University of Massachusetts Chan Medical School. For more on the Function platform, please visit rarebase.org or email function@rarebase.org.

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