"REGULATION OF THE *RAI1* EXPRESSION AS A NEW THERAPY FOR SMITH-MAGENIS SYNDROME" VCU #12-060

Applications

- Smith-Magenis syndrome (SMS)
- Child obesity
- Sleep disturbance
- Behavioral problems

Advantages

- Improvement of cognitive and behavioral functions
- Addressing multiple symptoms at the same time

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Market Need

Smith-Magenis syndrome (SMS) is a developmental disorder that affects approximately 1 in 15,000 to 1 in 25,000 individuals. SMS therapy relies mostly on managing its symptoms and often requires several forms of support, including physical therapy, occupational therapy, and speech therapy. Support is often required throughout an affected person's lifetime. Currently available medications help managing only some of the symptoms, and each of them address only one specific aspect of the disease. There is no available treatment that would manage multiple symptoms at once, improving patient's cognitive and behavioral functions.

Technology Summary

Patients with Smith-Magenis syndrome are characterized by the loss or mutation of the *RAI1* gene, which is associated with developmental and circadian deficits, obesity, sleep disturbance and behavioral problems. This technology focuses on stimulating the expression of the *RAI1* gene with 9-cis and trans forms of retinoic acid (RA) and can be used as a potential therapy for SMS and related disorders. It targets therapy to alleviate the sleep disturbance and to improve cognitive and behavioral functions. This opens the door for potential new molecules that can stimulate *RAI1* expression, and we are currently seeking partners for collaboration on screening drug libraries.

Technology Status

Patent pending: U.S. and foreign rights are available.

Currently seeking partners for a drug library screening.

This technology is available for licensing to industry for further development and commercialization.