A Method to Prevent the Myelin Abnormalities Associated with Arginase Deficiency

Tech ID: 30363 / UC Case 2019-550-0

SUMMARY

UCLA researchers in the Department of Surgery have developed a gene therapy to prevent dysmyelination (and other CNS abnormalities) as a result of arginase deficiency.

BACKGROUND

Arginase deficiency (ARG1 deficiency, argininemia) is an inherited autosomal recessive disorder caused by deficient/defective enzyme arginase 1 in the final step of urea cycle, catalyzing the hydrolysis of arginine to urea and ornithine. Failure to remove nitrogen from arginine, urea cannot be produced normally, and excess nitrogen accumulates in the blood in the form of ammonia. The accumulation of ammonia,arginine and its related metabolits (i.e. guanidino compounds) is toxic to the central nervous system, and is believed to cause the neurological problems associated with ARG1 deficiency. ARG1 deficiency usually becomes evident at early childhood. Untreated individuals exhibit slower than normal growth, development of spasticity, delays in cognitive development, and subsequent loss of developmental milestones. Currently, there is no treatment available for ARG1 deficiency, and disease is generally managed through dietary restriction and ammonia scavenger medications that promote ammonia excretion.

INNOVATION

Researchers at UCLA have determined that the origin of neurological dysfunction found in ARG1 deficiency is in part due to dysmyelination that occurs in the central nervous system. Based on this finding, researchers have developed a gene therapy that can prevent dysmyelination, improve compaction of myelin in the central nervous system, and use for treating ARG1 deficiency.

APPLICATIONS

- Gene therapy for arginase deficiency

ADVANTAGES

- Prevent neurological dysfunction associated with ARG1 deficiency

STATE OF DEVELOPMENT

The described gene therapy has been shown to restore myelination, proper neurological function, and normal behavior in ARG1 deficient mouse model, if administered shortly after birth.

PATENT STATUS

Country	Type	Number	Dated	Case
United States Of America	Published Application	2022-022050	07/14/2022	2019-550

ADDITIONAL TECHNOLOGIES BY THESE INVENTORS

- Treatment for Restoring Ureagenesis in Carbamoyl Phosphate Synthetase 1 Deficiency

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OTHER INFORMATION

KEYWORDS

Arginase deficiency, argininemia, ARG1 deficiency, hyperargininemia, urea cycle disorder, myelination, gene therapy

CATEGORIZED AS

- **Medical**
  - Disease: Central Nervous System
  - Disease: Genetic Diseases and Dysmorphic Syndromes
  - Disease: Metabolic/Endocrinology
  - Gene Therapy
  - Therapeutics

RELATED CASES

2019-550-0

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