

Immunotherapy for Treatment of Neuromyelitis Optica (NMO)

Tech ID: 28895 / UC Case 2011-096-0

INVENTION NOVELTY

This invention comprises compositions and methods for treatment of neuromyelitis optica (NMO) spectrum disorders using anti-aquaporin-4 (AQP4) antibody lacking effector function.

VALUE PROPOSITION

Neuromyelitis optica spectrum disorders (NMOSD) is an inflammatory disease of the central nervous system characterized by severe, immune-mediated demyelination and axonal damage predominantly targeting optic nerves and spinal cord. A defining feature in most NMOSD patients is the presence of serum autoantibodies (NMO-IgG) against astrocyte water channel aquaporin 4 (AQP4). At present, the therapeutics options include only immunosuppressants, plasma exchange and B-cell depletion.

The present invention defining a disease-specific NMO treatment comprises the following:

- ▶ A reagent containing anti-aquaporin-4 antibody or an antigen-binding fragment lacking effector functions of an intact antibody.
- ▶ A variety of reagent administration options including intraocular, intra-arterial, subcutaneous or intravenous.
- ▶ The reagent can be administered chronically.
- ▶ Can be used in combination therapy with immunosuppressive or other drugs.
- ▶ The method can prevent or reduce the progression of NMOSD.

TECHNOLOGY DESCRIPTION

Researchers at University of California, San Francisco have generated a specific reagent for treatment of NMOSD comprising AQP4 antibody or an antigen binding fragment, lacking effector function. The effector function of the antibody can be abrogated by mutating or chemically modifying Fc region or Fab fragment lacking effector Fc region. The antibody can be optimized for higher affinity AQP4 binding by mutating the Fab region. Treatment with this novel and disease-specific reagent can reduce NMOSD progression leading to visual and motor deficit.

LOOKING FOR PARTNERS

To develop & commercialize the technology as a specific treatment for neuromyelitis optica spectrum disorders.

STAGE OF DEVELOPMENT

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OTHER INFORMATION

KEYWORDS

NMO, NMOSD, AQP4, Water channel, Neuroinflammation, Autoantibodies, Immunotherapy, Antibody engineering

CATEGORIZED AS

- ▶ **Medical**
 - ▶ Disease: Autoimmune and Inflammation
 - ▶ Disease: Central Nervous System
 - ▶ Therapeutics

RELATED CASES

2011-096-0

RELATED MATERIALS

- ▶ Tradtrantip, L., H. Zhang, S. Saadoun, P. Phuan, C. Lam, M.C. Papdopoulos, J.L. Bennett and A.S. Verkman (2012). Anti-aquaporin-4 monoclonal antibody blocker therapy for neuromyelitis optica. *Ann. Neurol.* 71:314-322.

DATA AVAILABILITY

Mouse Model and ex-vivo data

PATENT STATUS

Country	Type	Number	Dated	Case
United States Of America	Issued Patent	11,390,667	07/19/2022	2011-096
United States Of America	Issued Patent	10,654,916	05/19/2020	2011-096
India	Published Application	16662	12/19/2014	2011-096
Rep Of Korea	Published Application			2011-096

ADDITIONAL TECHNOLOGIES BY THESE INVENTORS

- ▶ CFTR potentiators and correctors and bifunctional (corrector/potentiator) compounds for treatment of Cystic Fibrosis
- ▶ SALT-SPARING UREA TRANSPORT INHIBITOR DIURETICS FOR TREATMENT OF CARDIOVASCULAR AND RENAL DISORDERS
- ▶ Potent TMEM16A Small Molecule Treatment for Inflammatory and Reactive Airway Diseases, Asthma, Hypertension, Pain and Cancer
- ▶ Novel Small Molecule Drug for the Treatment of Constipation and Oxalate Kidney Stones
- ▶ Small Molecule Pendrin Inhibitors for Treatment of Inflammatory Airway Diseases and Diuretic Resistance

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