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The phenomenal story of neuromyelitis optica spectrum disorder: neurological autoimmune mystery to functional cure at the speed of life

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Abstract

Background: Neuromyelitis optica spectrum disorder (NMOSD) is a rare autoimmune disease of the central nervous system that manifests via inflammation and demyelination typically targeting optic nerves & spinal cord. Patients often experience a relapsing course of cumulative neurological debility. Latest epidemiologic data suggest hundreds of thousands of cases worldwide, many of which remain undiagnosed and ineffectively treated. Despite its potential for devastating and irreversible neurologic disabilities, no treatment had been proven safe and effective in prospective, masked and adequately powered clinical trials in over a century.

Key Advances: Integration of innovative molecular and cellular immunology, epidemiology, clinical characterization & treatment effects clearly differentiate NMOSD from multiple sclerosis (MS). Once viewed as solely B-cell driven, autoreactive T cells and loss of immune tolerance to aquaporin-4 or other autoantigens are now recognized as crucial to NMOSD immunopathogenesis. Four phase IIb/III clinical trials have now reported positive findings in evaluating three drugs (eculizumab, inebilizumab, satralizumab) for safety and efficacy to delay or prevent relapses in NMOSD. In 2019, the first of these drugs received regulatory approval having achieved >98% relapse-free efficacy among patients on drug in the trial period. The other agents are now in regulatory review.

Prospectus: In just a single decade, The Guthy-Jackson Charitable Foundation has led a phenomenal story of research innovation, patient advocacy and industry synergy to revolutionize the field and provide a promising future for NMOSD patients. Significant next steps include identifying predictive biomarkers of relapse and restoring immune tolerance for cures to spare patients from cancer and infectious disease risk of lifelong immunosuppressive therapy. Preventative research areas include etiology, genetic or environmental risk factors & epidemiologic correlates of disease course or severity. Scientific and therapeutic breakthroughs in NMOSD will benefit patients with other autoimmune diseases.

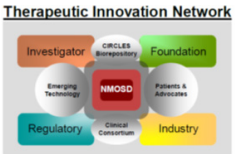

Figure 1. Therapeutic innovation network for NMOSD solutions implemented by the Guthy-Jackson Charitable Foundation. This network catalyzes and sustains the formation of alliances among stakeholders with synergistic interests. Based on the integrative continuum model, this network enables the application of new knowledge in real-time, accelerating quantum-leap advances.

Biography

Michael Yeaman is a tenured Professor of Medicine at the University of California, Los Angeles, Vice Chair of Medicine and Chief of Molecular Medicine at Harbor-UCLA Medical Center. He studies antibiotic-resistant infections & immunology of autoimmune diseases. He is Chair Medical Advisor, Guthy-Jackson Charitable Foundation in its mission to cure neuromyelitis optica. He received the National Research Service Award (NIH), Innovation Award (NIH) and Distinguished Investigator Award (U.S. Department of Defense). He has published >200 scholarly works and holds 27 issued patents. He co-authored the acclaimed book The Power of Rare, a blueprint for solving rare diseases. He founded NovaDigm Therapeutics, Inc. and Metacin, Inc., lectures internationally and serves on editorial boards of premier medical journals. Michael is an accomplished musical composer and performer integrating art, science and medicine for health & wellness. His music appears in films & multimedia, his 15 albums are available on iTunes® and Spotify®, and his Pandora® radio station is part of the music genome

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