May 20th, 12:30 PM

Sulforaphane Treatment of Children with Autism Spectrum Disorder

Eileen Diggins
University of Massachusetts Medical School

Let us know how access to this document benefits you.
Follow this and additional works at: https://escholarship.umassmed.edu/cts_retreat

Part of the Chemicals and Drugs Commons, Pediatrics Commons, and the Psychiatry and Psychology Commons

Repository Citation

Creative Commons License

This work is licensed under a Creative Commons Attribution-Noncommercial-Share Alike 3.0 License. This material is brought to you by eScholarship@UMassChan. It has been accepted for inclusion in UMass Center for Clinical and Translational Science Research Retreat by an authorized administrator of eScholarship@UMassChan. For more information, please contact Lisa.Palmer@umassmed.edu.
**Sulforaphane Treatment of Children with Autism Spectrum Disorder**
Eileen Diggins, BA, Andrew Zimmerman, MD, Kanwaljit Singh, MD, Susan Connors, MD
Division of Neurology, Department of Pediatrics, University of Massachusetts Medical School

**Abstract**

This clinical trial in autism spectrum disorder (ASD) tests a nontoxic approach to therapy of ASD.

**Background:** Direct treatment of underlying mechanisms in ASD is limited. Cellular dysfunction in ASD may involve a number of related metabolic pathways. A clinical clue may be found in the “fever effect” in ASD, in which febrile illness dramatically but temporarily ameliorates disordered behavior. Fever stimulates heat shock proteins (HSP) and cellular stress responses that may ultimately lead to improved synaptic function and increased long-range connectivity. The expression of gene transcription by NFE2L2 (Nrf2), which is reduced in ASD, may also increase during fever. **Sulforaphane (SF),** an isothiocyanate obtained from 3-day-old broccoli sprouts, induces HSP and Nrf2 as well as “cell-protective” responses. SF has several possible modes of action that may benefit ASD through common cellular mechanisms underlying heterogeneous phenotypes. SF crosses the blood brain barrier and is bioavailable orally.

**Preliminary data:** In a randomized, double-blind, placebo-controlled pilot trial in 44 male adolescents and adults (13-30 years), results showed SF was well tolerated without significant side effects. On average, participants on SF (particularly those with a history of fever effect) showed significantly more improvements in ASD symptoms than placebo participants. Significant improvements for SF participants included social interaction, aberrant/abnormal behavior, repetitive/stereotypical behavior, and verbal communication.

**Current study:** Our randomized, double-blind, placebo-controlled phase-2 clinical trial at UMass has three aims: To determine: (1) if orally administered SF has measurable effects in children (ages 3-12 years) with ASD; (2) if treatment with sulforaphane is safe and well tolerated; (3) To elucidate cellular biomarkers that support the mechanisms of action of SF in ASD. We hypothesize that SF will have positive effects, and that these effects will be more marked and lasting in the developing – compared to the mature – brain.

**Contact Information**
Name: Eileen Diggins
Email: Eileen.Diggins@umassmed.edu
Phone: 508-856-4107