May 16th, 1:45 PM

Sulforaphane Treatment of Children with Autism Spectrum Disorder – A Progress Report

Eileen Diggins
*University of Massachusetts Medical School*

Andrew Zimmerman
*University of Massachusetts Medical School*

Kanwaljit Singh
*University of Massachusetts Medical School*

*See next page for additional authors*

Follow this and additional works at: [https://escholarship.umassmed.edu/cts_retreat](https://escholarship.umassmed.edu/cts_retreat)

Part of the [Mental Disorders Commons](https://escholarship.umassmed.edu/cts_retreat), [Pediatrics Commons](https://escholarship.umassmed.edu/cts_retreat), and the [Translational Medical Research Commons](https://escholarship.umassmed.edu/cts_retreat)

---


[https://escholarship.umassmed.edu/cts_retreat/2017/posters/19](https://escholarship.umassmed.edu/cts_retreat/2017/posters/19)

This material is brought to you by eScholarship@UMMS. It has been accepted for inclusion in UMass Center for Clinical and Translational Science Research Retreat by an authorized administrator of eScholarship@UMMS. For more information, please contact [Lisa.Palmer@umassmed.edu](mailto:Lisa.Palmer@umassmed.edu).
Presenter Information
Eileen Diggins, Andrew Zimmerman, Kanwaljit Singh, and Susan Connors

Keywords
autism spectrum disorder, Sulforaphane

Creative Commons License
This work is licensed under a Creative Commons Attribution-Noncommercial-Share Alike 3.0 License.

This poster abstract is available at eScholarship@UMMS: https://escholarship.umassmed.edu/cts_retreat/2017/posters/19
This ongoing 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. The “fever effect” in ASD, in which febrile illness dramatically but temporarily ameliorates disordered behavior, offers a clinical clue. 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 – including social interaction, aberrant/abnormal behavior, repetitive/stereotypical behavior, and verbal communication – than placebo participants.

**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 positive effects of SF will be more marked and lasting in the developing brain. To date, 7 participants have completed the trial, and 22 are actively enrolled. Recruitment is ongoing, with a target sample size of 50.

**Contact:**
Eileen Diggins
University of Massachusetts Medical School
Eileen.Diggins@umassmed.edu