

Effect of pioglitazone treatment on behavioral symptoms in autistic children

Below is the abstract of a paper I helped co-author on a treatment study for 25 children with autism. In an open study (not placebo-controlled), we found that ACTOS (pioglitazone) helped reduce 4 categories of symptoms (irritability, lethargy, stereotypy, and hyperactivity) in children with autism. ACTOS is an FDA-approved medication that helps regulate the immune system, which is often abnormal in children with autism. This work has also been presented at past DAN! meetings.

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Boris M, Kaiser C, Goldblatt A, Elice MW, Edelson SM, Adams JB, Feinstein DL.
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ABSTRACT: INTRODUCTION:

Autism is complex neuro-developmental disorder which has a symptomatic diagnosis in patients characterized by disorders in language/communication, behavior, and social interactions. The exact causes for autism are largely unknown, but it has been speculated that immune and inflammatory responses, particularly those of Th2 type, may be involved. Thiazolidinediones (TZDs) are agonists of the peroxisome proliferator activated receptor gamma (PPARgamma), a nuclear hormone receptor which modulates insulin sensitivity, and have been shown to induce apoptosis in activated T-lymphocytes and exert anti-inflammatory effects in glial cells. The TZD pioglitazone (Actos) is an FDA-approved PPARgamma agonist used to treat type 2 diabetes, with a good safety profile, currently being tested in clinical trials of other neurological diseases including AD and MS. We therefore tested the safety and therapeutic potential of oral pioglitazone in a small cohort of children with diagnosed autism.

CASE DESCRIPTION:

The rationale and risks of taking pioglitazone were explained to the parents, consent was obtained, and treatment was initiated at either 30 or 60 mg per day p.o. A total of 25 children (average age 7.9 +/- 0.7 year old) were enrolled. Safety was assessed by measurements of metabolic profiles and blood pressure; effects on behavioral symptoms were assessed by the Aberrant Behavior Checklist (ABC), which measures hyperactivity, inappropriate speech, irritability, lethargy, and stereotypy, done at baseline and after 3-4 months of treatment.

DISCUSSION AND EVALUATION: In a small cohort of autistic children, daily treatment with 30 or 60 mg p.o. pioglitazone for 3-4 months induced apparent clinical improvement without adverse events. There were no adverse effects noted and behavioral measurements revealed a significant decrease in 4 out of 5 subcategories (irritability, lethargy, stereotypy, and hyperactivity). Improved behaviors were inversely correlated with patient age, indicating stronger effects on the younger patients.

CONCLUSION:

Pioglitazone should be considered for further testing of therapeutic potential in autistic patients.