IDENTIFYING NEXT STEPS IN RESEARCH ON CANNABIS AND AUTISM

NOVEMBER 29 & 30, 2018

NEW YORK CITY
**OBJECTIVES**

The primary aims of this conference are:

- Greater understanding of the scientific and regulatory landscape for cannabis-related products in autism, and
- Identification of consensus on the research paths best able to advance our understanding of cannabis as a potential intervention for people with autism.

To achieve these aims, the conference will begin by orienting participants with presentations on recent basic research on the endocannabinoid system and discussions on relevant safety and dose/product/delivery issues.

The agenda will continue with presentations on completed and ongoing observational and randomized trials using cannabis-related products and presentations and discussion on regulatory issues related to studies of cannabis in the U.S.

After these presentations, participants will break into workgroups addressing issues related to the following questions:

1. What types of basic research studies could help advance our understanding of the potential for cannabis-related products as autism interventions?
2. What translational studies could help advance cannabis-related products as autism interventions?
3. How do we consider and measure safety and tolerability in trials of cannabis in autism?
   a) in children?
   b) in non-verbal or minimally verbal people of any age?
4. What are the most promising neurological and behavioral targets for cannabis in autism?
   a) how should we measure promising targets?
   b) what biomarkers should we consider? (selection, stratification, mechanistic, target engagement, outcome tracking, etc.)
5. What needs can specific cannabis compounds and products potentially address?
   a) CBD-only products?
   b) CBD-THC formulations?
   c) other formulations?
6. What should future observational studies address?
7. What clinical trial designs should we consider?
8. What delivery mechanisms should we consider in future research?
9. While waiting the results of research, what guidance can the research and medical community provide people considering cannabis products for autism-related symptoms and medical conditions?

---

**PURPOSE**

A growing number of people are using cannabis and cannabis-derived products and compounds (e.g. hemp oil, THC, CBD) for medicinal purposes. This includes many people who have autism. Despite anecdotal reports of benefit, to date, we have little scientific evidence supporting the use of cannabis products to ease autism symptoms or autism’s co-occurring health conditions. As researchers and clinicians, we need to conduct rigorous study of cannabis-related products and their constituent components to assess safety and potential effectiveness in improving daily function and quality of life among people with autism.

The urgency of this consensus conference is driven by many factors, including:

1) The growing, but incomplete, understanding of the human endocannabinoid system,
2) The plethora of biologically active compounds in cannabis plants,
3) Recent evidence of efficacy using cannabidiol to reduce seizures in patients with genetic syndromes closely related to autism,
4) The potential to target core or associated autism symptoms that currently lack adequate pharmacological treatment, and
5) The wide range of possible approaches to study cannabis and its derivatives in people with autism.
PARTICIPANTS

Greg Barnes, MD | Conference Co-Chair
University of Louisville Autism Center

Adi Aran, MD | Shaare Tzedeck Medical Center, Jerusalem

Amanda Bennett, MD, MPH | Center for Autism Research, Children's Hospital of Philadelphia

Sallie Bernard | Ascendigo Autism Services, Aspen, Colorado; Safe Minds Board Member

Tom Bernard | Ascendigo Autism Services, Aspen, Colorado; Autism Speaks Board Member

Marcel Bonn-Miller, PhD | Lambert Center for the Study of Medicinal Cannabis and Hemp, Thomas Jefferson University, Philadelphia, Zynexa Pharmaceuticals and the University of Pennsylvania

Mary Ann DiLiberto, RN | Division of Critical Care Medicine, Children's Hospital of Philadelphia

Ryan Fischer | Parent Project Muscular Dystrophy

Eric Hollander, MD | Albert Einstein College of Medicine and Montefiore Medical Center

Julie Johnson, PhD | Massachusetts Cannabis Control Commission

Debra Shamala Karhson, PhD | Stanford University

Emily Lindley, PhD | Anschutz Medical Campus, University of Colorado, Denver

Britte McBride, JD | Massachusetts Cannabis Control Commission

Ann Neumeyer, MD | Lurie Center for Autism, Mass General Hospital

Heike Newman | Clinical Research Support Center, University of Colorado, Denver

Vera Neggovorova, MD, PhD | Albert Einstein College of Medicine, Montefiore Medical Center, New York

Jessica Roland, PhD | Greenwich Biosciences

J. Cobb Scott, PhD | Neuropsychiatry Section, Perelman School of Medicine, University of Pennsylvania

Tyler Story, PhD | Greenwich Biosciences

Nicole Tartaglia, MD | Denver Fragile X Clinic, Children's Hospital Colorado, University of Colorado School of Medicine

Doris Trauner, MD | Department of Neurosciences, University of California, San Diego

Ryan Vandrey, PhD | Behavioral Pharmacology Research Unit, Johns Hopkins Medicine, Baltimore

Benjamin Yerys, PhD | Center for Autism Research, Children's Hospital of Philadelphia

Athena Zuppa, MD | Center for Clinical Pharmacology, Children's Hospital of Philadelphia

Autism Speaks staff:
Tom Frazier (Conference Co-chair), Chief Science Officer
Andy Shih, Senior VP, Public Health and Inclusion
Stuart Spielman, Senior VP, Advocacy
Dean Hartley, Senior Director, Genetic Discovery
Joan New, Grants Manager
Arun Karpur, Director, Data Science & Evaluation Research
Angie Fedele, Director of Operations, Clinical Programs
Jessica Sachs, Director, Science Communications
DAY 1 | MORNING
8:00 am
BREAKFAST

9:00 am
MOTIVATION & PURPOSE OF CONFERENCE
(10 MIN)
TOM FRAZIER
Attendee Introductions (30 min)
Primary affiliation
Relationship to cannabis and/or autism
Current and planned activities
Possible conflicts of interest

10:00 am
PARTICIPANT PRESENTATIONS
BASIC AND TRANSLATIONAL RESEARCH ON CANNABINOIDS AND ENDOCANNABINOID SIGNALING (20 MIN)
Deb Karhson
SAFETY ISSUES IN CANNABIS RESEARCH
(15 MIN PRESENTATION, 15 MIN PANEL)
J. Cobb Scott, Presenter
Tyler Story
Marcel Bonn-Miller
DOSE, ROUTE, PHARMACOKINETIC/DYNAMIC, LABELING AND OTHER PRODUCT CONSIDERATIONS FOR THE MEDICINAL USE OF CANNABIS (20 MIN)
Ryan Vandrey
TRIALS OF CANNABIS-RELATED PRODUCTS IN OTHER CONDITIONS (15 MIN)
Emily Lindley

SCHEDULE

DAY 2 | MORNING
8:00 am
BREAKFAST

9:00 am
BREAKOUT GROUPS
ROUND 1 (45 MIN)
A. Basic and translational research needed on endocannabinoid system and cannabinoids to advance understanding of potential benefit to autism
B. Most promising neurological targets
C. Most promising behavioral targets
RECONVENE AND REPORT (30 MIN)

12:45 pm
LUNCH

2:00 pm
DELIVERABLES DISCUSSION (30 MIN)
POSSIBLE DELIVERABLES
1. Conference report and/or review article describing research and regulatory landscape, areas of consensus and potential paths forward
2. Generating media related to conference and its outputs
3. IACC reporting
4. Lay abstract of the conference report or other Autism Speaks communications
5. Other deliverables

2:30 pm
CONFERENCE CONCLUDING REMARKS
Greg Barnes
Tom and Sallie Bernard
Tom Frazier

OBSERVATIONAL STUDIES OF CANNABIS-RELATED PRODUCT USE IN AUTISM (20 MIN)
Athena Zuppa
Mary Ann DiLiberto

OPEN-LABEL STUDIES OF CANNABIS-RELATED PRODUCTS IN AUTISM AND RELATED CONDITIONS (20 MIN)
Greg Barnes
Marcel Bonn-Miller

12:30 pm
LUNCH

1:30 pm
RANDOMIZED CONTROLLED TRIALS OF CANNABIS-RELATED PRODUCTS IN AUTISM
(FOUR 15-MIN TALKS, 20 MIN PANEL)
Eric Hollander
Doris Trauner
Nicole Tartaglia
Adi Aran

REGULATORY ISSUES FOR CANNABIS RESEARCH
(FOUR 15-MIN TALKS, 30-MIN PANEL WITH BRIEF STATEMENT)
Heike Newman
Dominic Chiapperino
Ryan Vandrey
Britte McBride
Doris Trauner