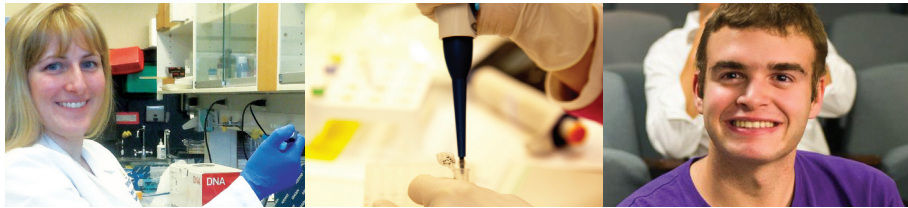# AUTISM SPEAKS®

CONSENSUS CONFERENCE:

IDENTIFYING NEXT STEPS IN RESEARCH ON CANNABIS AND AUTISM

NOVEMBER 29 & 30, 2018

NEW YORK CITY

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.

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?*

PARTICIPANTS

Greg Barnes, MD | Conference Co-Chair
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Autism Speaks staff:
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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

SCHEDULE

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

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 (TWO 15-MIN TALKS; 30-MIN PANEL WITH BRIEF STATEMENT)

Heike Newman
Dominic Chiapperino
Ryan Vandrey
Julie Johnson
Britte McBride
Doris Trauner

3:00 pm
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)

DAY 2 | MORNING

8:00 am
BREAKFAST

9:00 am
BREAKOUT GROUPS

ROUND 2 (45 MIN)

A. Safety/tolerability issues and their measurement

B. Biomarkers and measurement of outcomes (specific measures)

RECONVENE AND REPORT (30 MIN)

ROUND 3 (45 MIN)

1. Next steps in CBD-only studies

2. Next steps in CBD-THC and other formulations

RECONVENE AND REPORT (30 MIN)

ROUND 4 (45 MIN)

1. Next steps in observational studies

2. Next steps in clinical trials

3. Alternative clinical trial designs for maximizing treatment response, especially for challenging sub-populations

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

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