DSMB Report

Project Title: STAAR Study
PI: Marjorie Solomon, PhD & MBA

2nd DSMB Meeting: 04/22/2019

Date of report: 04/18/2019

Data as of: 04/02/2019

Prepared by: Soo Park, BAS
                Junior Specialist
                UC Davis MIND Institute

Section 1. Recruitment and Participant Status (as of 4/2/2019)

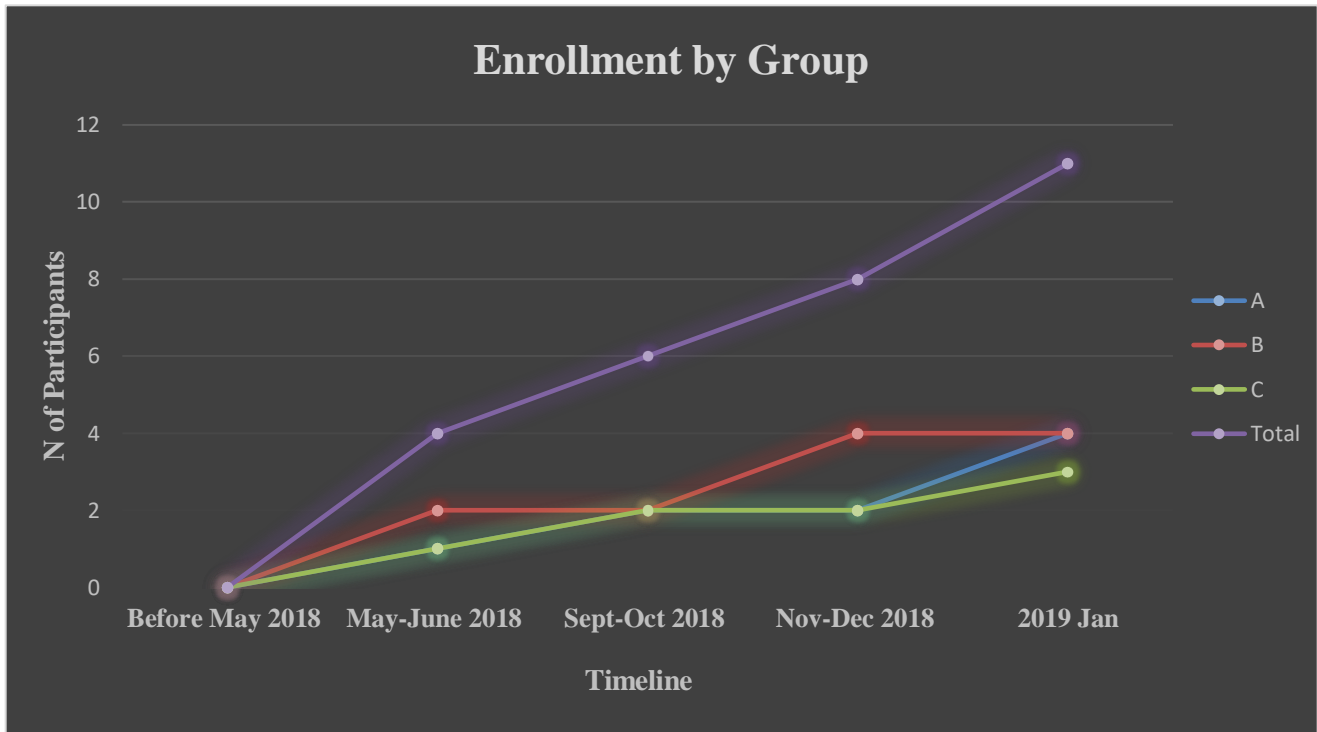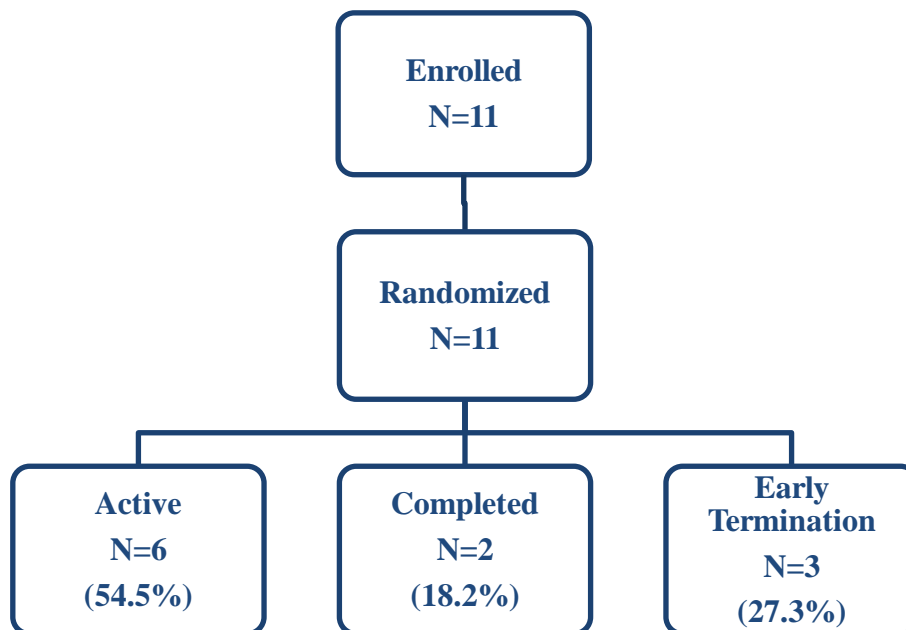## Figure 1. Enrollment and Accrual of Study Participants

Enrollment by Group

Figure 2. Overall Study Status

Enrolled
N=11

Randomized
N=11

Active
N=6
(54.5%)

Completed
N=2
(18.2%)

Early
Termination
N=3
(27.3%)

Section 2. Demographics (as of 4/2/2019)
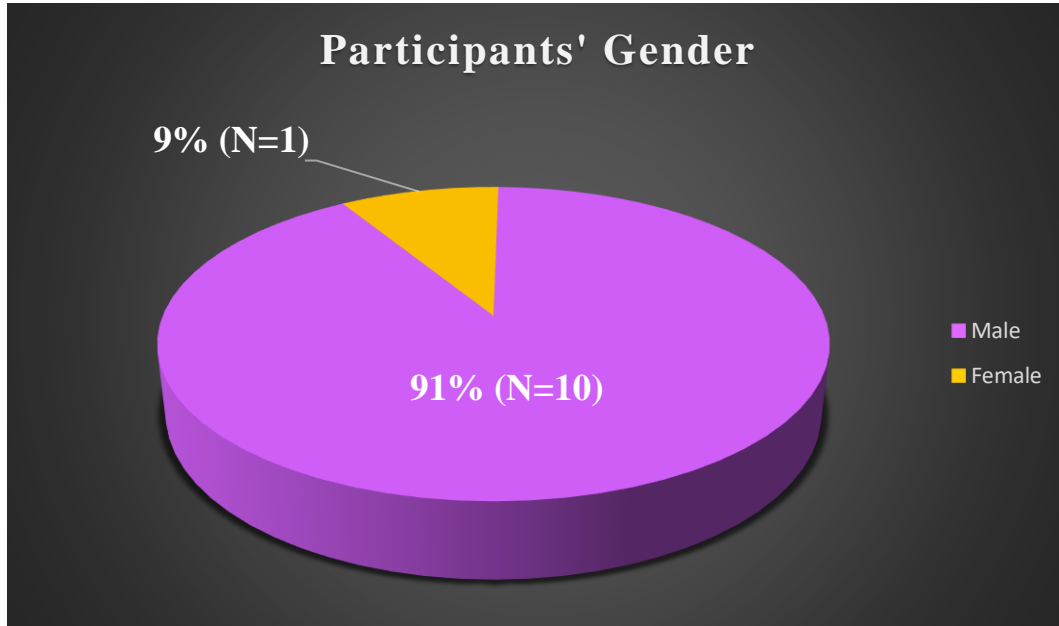
Figure 3. Gender of Study Participants

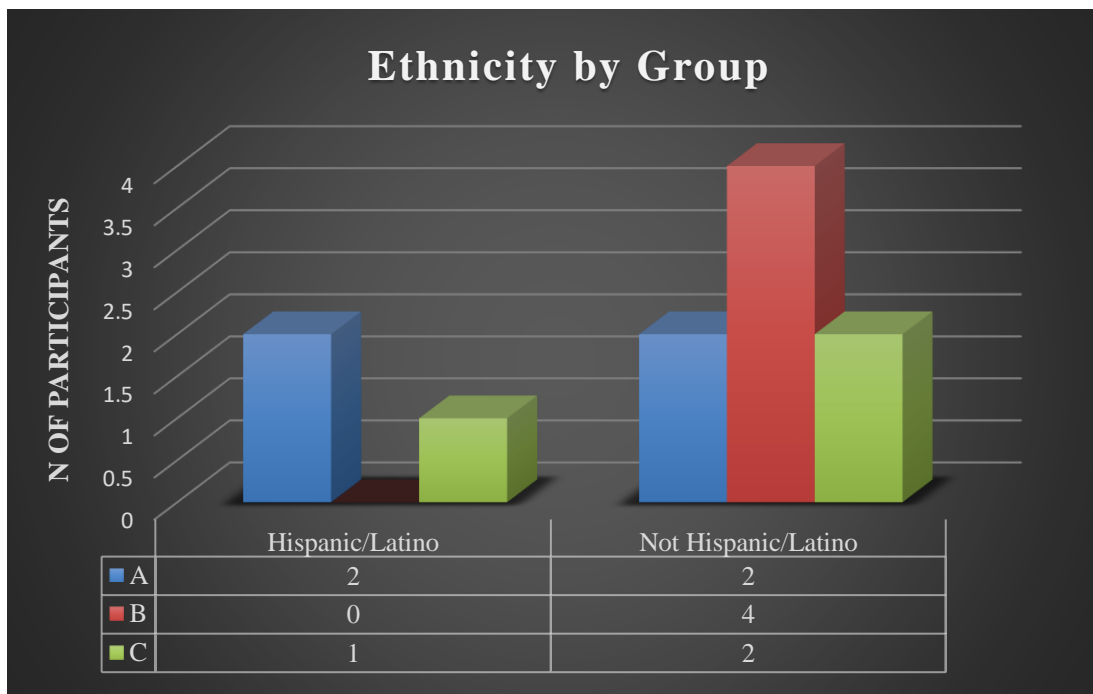**Figure 4. Ethnicity of Study Participants by Study Groups**
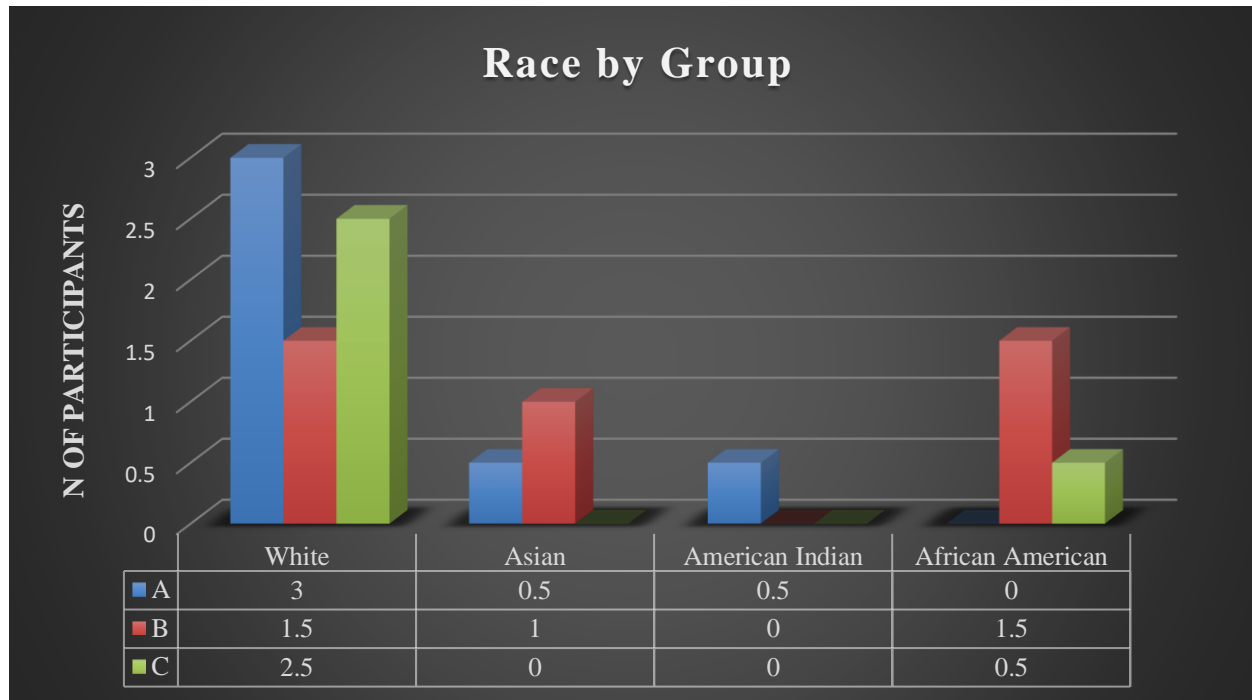
Figure 5. Race of Study Participants by Study Group

Race by Group

N OF PARTICIPANTS

	White	Asian	American Indian	African American
A	3	0.5	0.5	0
B	1.5	1	0	1.5
C	2.5	0	0	0.5

Table 1. Age Descriptive of Study Participants by Study Group

		Group A	Group B	Group C
Age	Mean	10.25	10	10
	Median	10	9.5	10
	SD	1.5	1.41	2
	Minimum	9	9	8
	Maximum	12	12	12

Table 2. Group Difference Test Result

	Gender	Ethnicity	Race	Age
Group Difference P-Value	1	0.42	0.73	0.97

* Group Difference was determined by Fisher's Exact test for categorical variables and ANOVA test for age.

4

Section 2. Safety Assessments for All Participants

The AE data are available for 11 participants as of 4/2/2019. The following statistics were based on the AE data obtained from those 11 participants randomized to treatment groups. Participants had 1.82 events on average with SD= 2.23.

Table 4. Incidence of Adverse Events by Standardized AE Category

Standardized AE Category	Total Participants*	Total %**	Total Events***
Anxiety	2	18.2	4[#]
Diarrhea	1	9.1	2[#]
Disruptive Behavior	1	9.1	2[#]
Dysgeusia	1	9.1	2[#]
Ear Infection	1	9.1	1
Fever	1	9.1	1
listlessness	1	9.1	1
Mouthing	1	9.1	1
Rash	1	9.1	1
Sleep Disturbance	1	9.1	1
Sprain	1	9.1	2[#]
Tics	1	9.1	1
Upper Respiratory Infection	1	9.1	1

*   *Number of participants experiencing an adverse event (participant is to be counted only once for each adverse event)*
**   *% of total number of participants with AE data available to date (total N=11) in the study*
***   *Total number of events overall*
#   *Indicate recurrence of same adverse event*

Table 5. Incidence of Adverse Events by Standardized AE Category, Stratified by Treatment Group

Standardized AE Category	Group A (n=4)			Group B (n=4)			Group C (n=3)		
	Total Participants*	Total %**	Total Events ***	Total Participants*	Total %**	Total Events ***	Total Participants*	Total %**	Total Events ***
Anxiety	1	9.1	2[#]	0	0	0	1	9.1	2[#]
Diarrhea	1	9.1	2[#]	0	0	0	0		0
Disruptive Behavior	1	9.1	2[#]	0	0	0	0		0
Dysgeusia	0	0	0	0	0	0	1	9.1	2[#]
Ear Infection	1	9.1	1	0	0	0	0		0
Fever	1	9.01	1	0	0	0	0		0
Listlessness	1	9.1	1	0	0	0	0		0
Mouthing	1	9.1	1	0	0	0	0		0
Rash	1	9.1	1	0	0	0	0		0
Sleep Disturbance	1	9.1	1	0	0	0	0		0
Sprain	0	0	0	0	0	0	1	9.1	2[#]
Tics	1	9.1	1	0	0	0	0		0
Upper Respiratory Infection	0	0	0	1	9.1	1	0		0

*   *Number of participants experiencing an adverse event (participant is to be counted only once for each adverse event)*

**   *% of total number of participants with AE data available to date (total N=11 per group) in the study*

***   *Total number of events overall*

\#   *Indicate recurrence of same adverse event*

Table 6. Severity of Adverse Events by Treatment Group

Treatment Group	Total Number of Participants * (%**) with				Group Difference p-value
	NO AE	Mild Event	Moderate Event	Serious Event	
Overall	5 (45.5%)	4 (36.4%)	2 (18.2%)	0	0.48
Group A	1	1	2	0	
Group B	3	1	0	0	
Group C	1	2	0	0	

*   Number of **participants** experiencing a certain severity of an adverse event where each participant is counted only once at the highest level of severity for the event
**   % of participants experiencing a certain severity of an adverse event

Treatment Group	Total Number of Adverse Events* (%**)			Group Difference p-value
	Mild	Moderate	Serious	
Overall	16 (80%)	4 (20%)	0	0.41
Group A	9	4	0	
Group B	1	0	0	
Group C	6	0	0	

*   Total number of **events** experiencing a certain severity of an adverse event
**   % of events experiencing a certain severity of an adverse event

Appendix

Listing 1: Adverse Events *

Participant ID	Adverse Event	Severity	Frequency	Action Taken	Outcome**	Relationship to Study Medication***
003X	Diarrhea	1	1	1	1	2
003X	Diarrhea	1	1	1	1	1
005X	Anxiety	1	1	1	1	1
005X	Anxiety	1	1	1	1	1
005X	Sprain	1	3	1	1	1
005X	Sprain	1	3	1	1	2
006X	Upper respiratory infection	1	1	1	1	1
007V	Dysgeusia	1	1	1	1	4
007V	Dysgeusia	1	1	1	1	2
009V	Mouthing	2	1	1	6	2
009V	Sleep disturbance	1	1	1	1	1
009V	Disruptive Behavior	1	1	1	1	1
009V	Fever	2	1	1	3	1
009V	Ear infection	2	1	1	3	1
009V	Disruptive Behavior	1	1	1	1	2
011X	Anxiety	1	1	1	1	1
011X	Tics	1	1	1	3	4
011X	Listlessness	1	1	1	1	2
011X	Anxiety	2	2	4	1	2
011X	Rash	1	3	1	3	1

*   *This listing was sorted by Participant ID.*

Severity	Frequency	Action taken with Study Drug	Outcome	Relationship to study drug
1 = mild 2 = moderate 3 = severe	1 = Intermittent 2 = Continuous 3 = Single	1 = None 2 = Study Drug Temporarily Discontinued 3 = Dose Decrease 4 = Study Drug Permanently Discontinued 5 = Concomitant Medication/ Therapy 6 = Hospitalization/ Hospitalization Prolonged	1 = Recovered 2 = Recovered with sequelae 3 = Recovering 4 = Not Recovered 5 = Fatal 6 = Unknown	1 = Not Related 2 = Unlikely 3 = Possibly related (<50%) 4 = Probably related (>50%) 5 = Definitely related

Listing 2: Demographics

ID	Blinded Group (A, B,or C)	Age at Enrollment (years)	Gender	Race	Ethnicity	Status of Enrollment
001V	B	10	F	Asian	Not Hispanic/Latino	Completed
002X	A	12	M	White	Not Hispanic/Latino	Active
003X	A	9	M	White	Not Hispanic/Latino	Active
004V	B	9	M	White and Black/African American	Not Hispanic/Latino	Early Termination
005X	C	8	M	White	Not Hispanic/Latino	Active
006X	B	12	M	Black/African American	Not Hispanic/Latino	Active
007V	C	10	M	White and Black/African American	Not Hispanic/Latino	Early Termination
008X	B	9	M	White	Not Hispanic/Latino	Active
009V	A	9	M	White and American Indian	Hispanic/Latino	Completed
010X	C	12	M	White	Hispanic/Latino	Active
011X	A	11	M	White and Asian	Hispanic/Latino	Early Termination