### Outcome Measures and Statistical Analyses: Study Design Examples

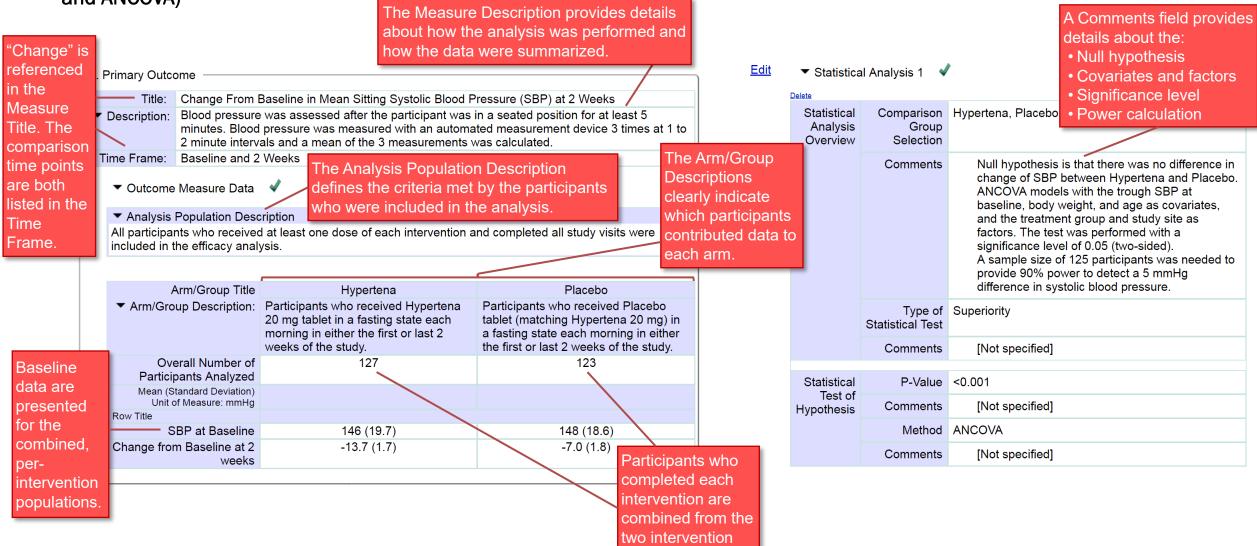
Results Database Train-the-Trainer Workshop

August 2021



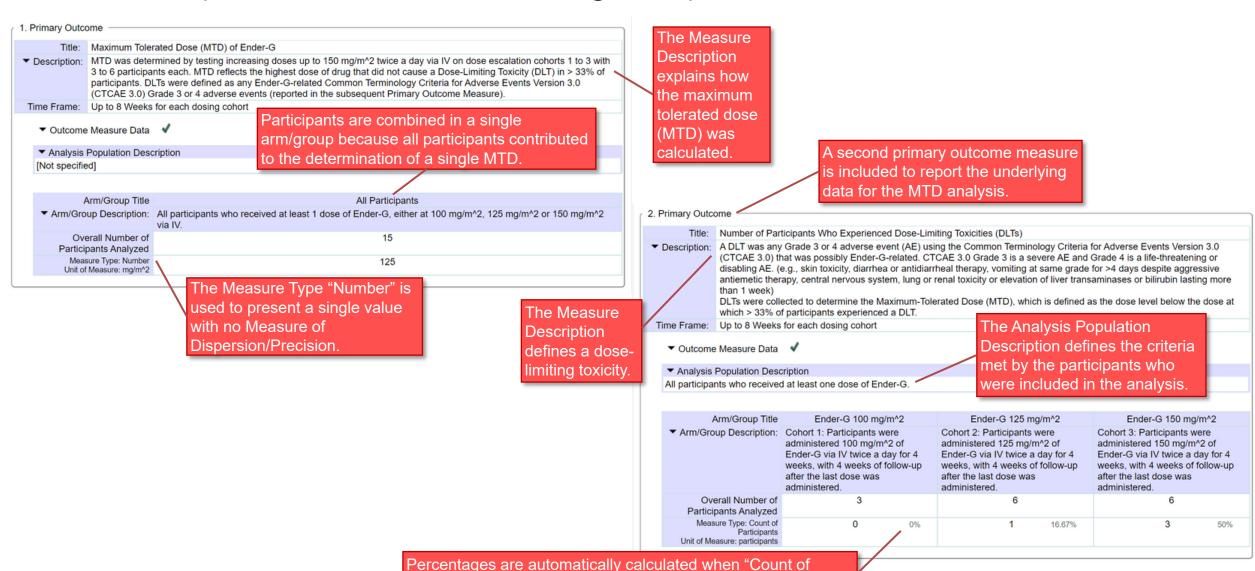


### Crossover (Change from Baseline and ANCOVA)



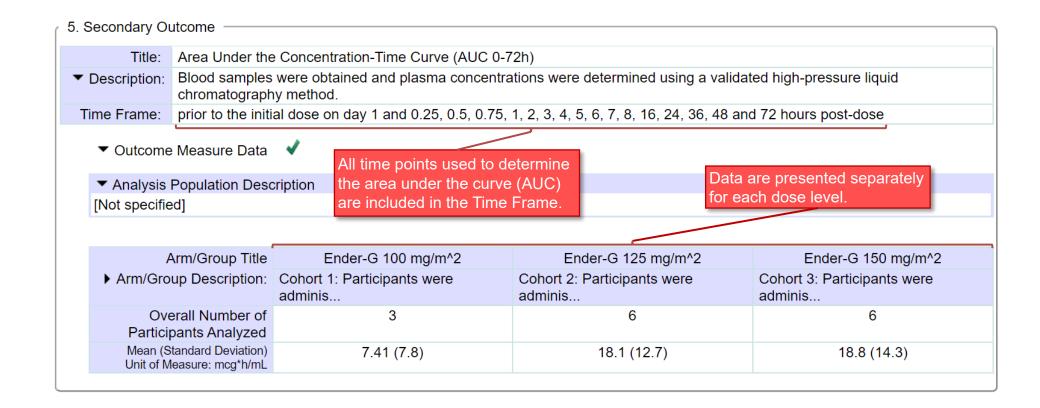
periods.

#### Dose Escalation (Maximum Tolerated Dose and Dose-Limiting Toxicities)



Participants" is the Measure Type. Displaying them is optional.

#### **Dose Escalation (Pharmacokinetics)**



#### **Units Other Than Participants (Count of Units)**

The Measure Description explains how bleeding on 2. Secondary Outcome probing (BOP) was assessed. Title: Number (%) of Implant Sites With Bleeding on Probing Bleeding on probing (BOP) is a measure of gingival inflammation and tissue destruction. Bleeding sites were identified by Description: gently probing the base of the implant site and assigning a score of 0 (no bleeding) or 1 (bleeding). Percentage of BOP = 100% \* (total implant sites that bled) / (total number of implants). If reporting a score on a scale, please include the unabbreviated scale title, the minimum and maximum values, and whether higher scores mean a better or worse outcome Time Frame: 12 months The Analysis Population Description defines the criteria Outcome Measure Data met by the participants who ▼ Analysis Population Description were included in the analysis. Per Protocol population, defined as participants completing the 12-month follow-up visit Arm/Group Title Ghostsply® Implants Crestene® Implants Arm/Group Description: Titanium Ghostsply® implants were randomly Ceramic Crestene® implants were randomly assigned to the left or the right mandible side in a assigned to the left or the right mandible side in a split-mouth randomized design. split-mouth randomized design. Overall Number of 24 Participants Analyzed Overall Number of Units 45 39 Analyzed

24.44%

11

When "Count of Units" is selected as the Measure Type, the Unit of Measure is automatically set to "implants."

Type of Units Analyzed: implants

Measure Type: Count of Units

Unit of Measure: implants

Percentage of BOP is represented by percentages that are automatically calculated when "Count of Units" is the Measure Type.

12

30.77%

A row is added to the Outcome Measure table to:

The Overall

Participants

arm/group.

Analyzed must be

included for each

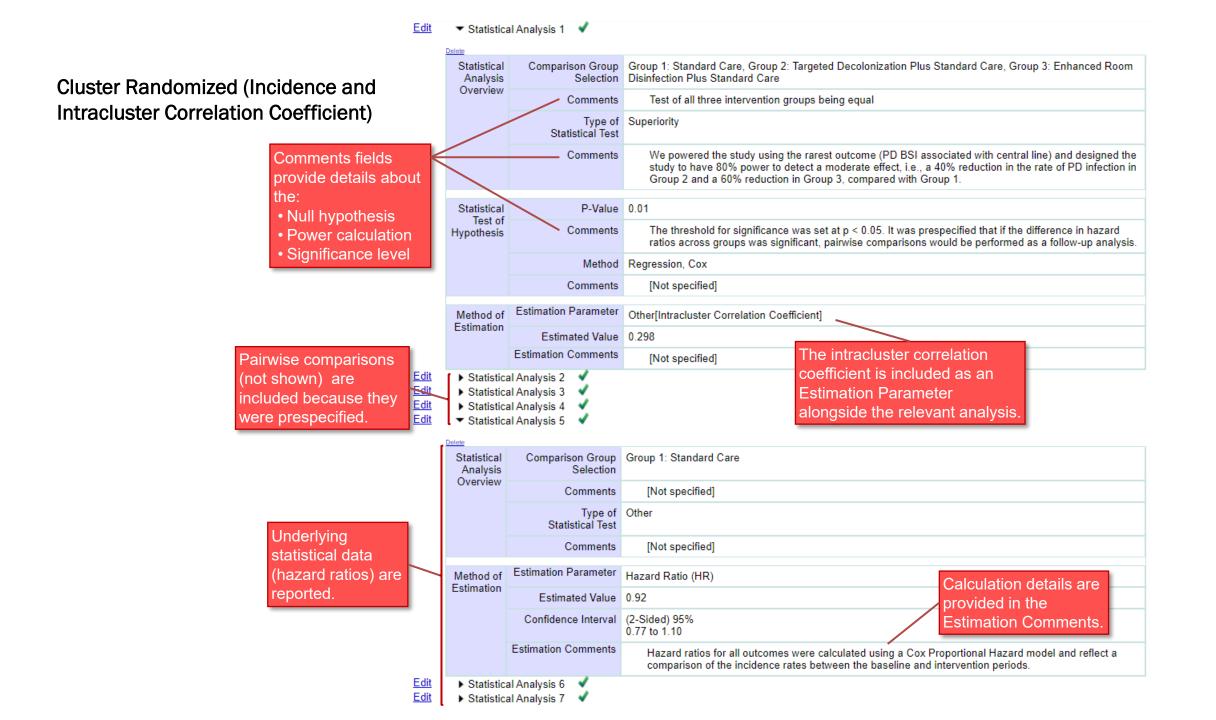
Number of

- Display the Type of Units Analyzed and the Overall Number of Units Analyzed per arm/group
- Allow the selection of "Count of Units" as the Measure Type



Cluster Randomized (Incidence and Intracluster Correlation Coefficient)

'Incidence" is used precisely as defined (that is, as the number of The Measure Description new cases over a specified period) defines a "confirmed" infection 1. Primary Outcome Title: Incidence of Confirmed ICU-Attributable PD Infection ▼ Description: Intensive care unit (ICU)-attributable Poissonosis davrilarum (PD) infection is defined as a clinical culture that tests positive at any point from the third day after ICU admission through two days after discharge. Confirmed infections included any positive cultures collected from skin or mucosal surfaces and polymerase chain reaction (PCR)-verified bloodstream infections (BSIs) Time Frame: Assessed from 3 days after ICU admission to 2 days post discharge for each participant during the baseline (12 months) and intervention (12 months) periods, a total of 123,272 days for Group 1, 119,872 days for Group 2, and 136,922 days for Group 3 ▼ Outcome Measure Data The Analysis Population Description ▼ Analysis Population Description defines the criteria met by the participants Participants assessed for ICU-attributable PD-positive culture in the baseline and intervention periods who were included in the analysis. Arm/Group Title Group 1: Standard Care Group 2: Targeted Decolonization Plus | Group 3: Enhanced Room Disinfection Standard Care Plus Standard Care ▼ Arm/Group Description: Patients were screened for As in Group 1, patients were screened As in Groups 1 and 2, patients were Poissonosis davrilarum (PD) infection screened for PD infection on ICU for PD infection on ICU admission and on intensive care unit (ICU) admission each enrolled ICU took transmissionadmission and each enrolled ICU took Each enrolled ICU took transmissionbased precautions, based on guidance transmission-based precautions, based from the CDC. In addition, PD-positive on guidance from the CDC. In addition. based precautions, based on guidance from the Centers for Disease Control patients received a 5-day rooms from which PD patients were decolonization regimen of twice-daily discharged were disinfected with a and Prevention (CDC). Incidence values, which have intranasal 2% No-Bug cream and daily solution containing hypochlorite bathing with 4% No-Scrub sanitizing (bleach) plus a disinfecting ultraviolet no Measure of Dispersion/ light (UV-C) device. cloths. Precision, are presented using Overall Number of Participants Analyzed 78,653 80,685 77,593 the Measure Type "Number." Measure Type: Number Unit of Measure: Infections per 1,000 Patient-Days Row Title **Baseline Period** Number Analyzed 39,530 participants 41,229 participants 38,804 participants 3.3 4.1 3.5 Intervention Period Number Analyzed 39,123 participants 39,456 participants 38,789 participants 3.0 3.2 2.2



#### Fractional Factorial (Scaled Assessment and ANOVA)

Full scale information is provided, including the:

- Range and directionality of scores for each item
- Calculation to produce a total score
- Range and directionality of the total score

1. Primary Outcome Title: Pre- and Post-intervention Scores on the Center for Epidemiological Studies Depression Scale (CES-D) ▼ Description: The CES-D is a 20-item measure that rates how often patients experience symptoms associated with depression. Responses are scored 0 (none of the time) to 3 (most or all of the time) for each item. Responses are summed for a final score ranging from 0 to 60, with higher scores indicating worse outcomes, i.e., higher levels of depression. If reporting a score on a scale, please include the unabbreviated scale title, the minimum and maximum values, and whether higher scores mean a better or worse outcome Time Frame: Pre-intervention (during the first counseling session) and post-intervention (at 7 months, during the last counseling session)

▼ Outcome Measure Data

▼ Analysis Population Description

All participants who received the noted level of each factor and completed both the pre- and post-intervention assessments were combined for this analysis.

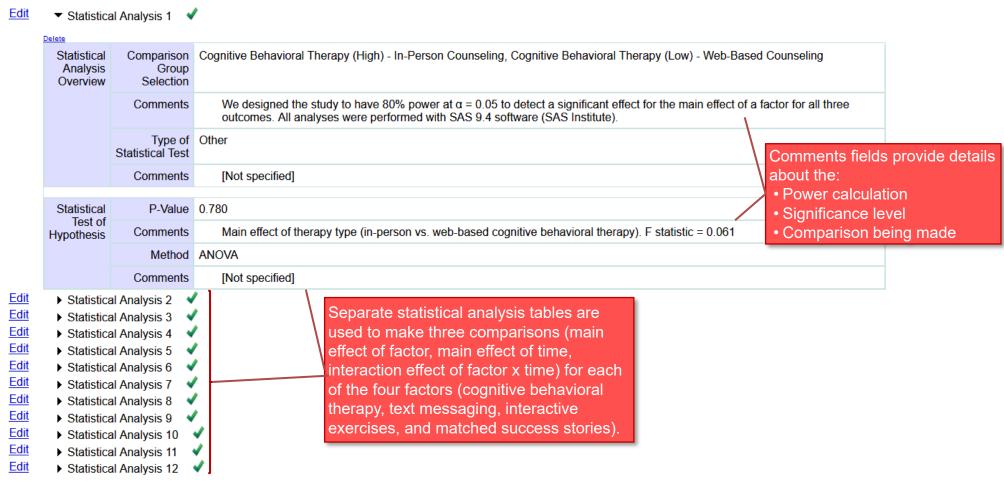
Participants are combined according to the factor level received.

The Analysis Population Description defines the criteria met by the participants who were included in the analysis.

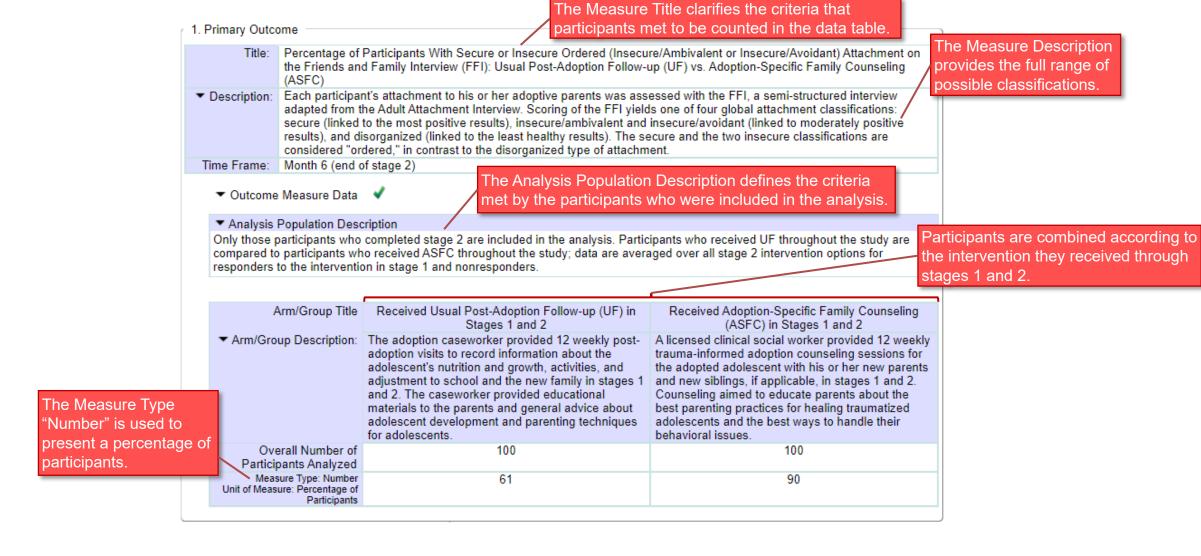
Scores from two time points (preand postintervention) are included in a primary outcome measure because they are compared in the statistical analyses.

S	Arm/Group Title	Cognitive Behavioral Therapy (High) - In-Person Counseling	Cognitive Behavioral Therapy (Low) - Web-Based Counseling	Text Messaging (Yes)	Text Messaging (No)	Web-Based Interactive Exercises (Yes)	Web-Based Interactive Exercises (No)	Web-Based Matched Success Stories (High)	Web-Based Matched Success Stories (Low)
d	▼ Arm/Group Description:	Participants received school- based, in-person cognitive behavioral therapy. Counseling sessions occurred weekly for 7 months, except during school holidays. During breaks, participants were granted access to counseling on an as-needed basis, up to once a week.	Participants received web-based cognitive behavioral therapy. Counseling sessions occurred weekly for 7 months, except during school holidays. During breaks, participants were granted access to counseling on an as-needed basis, up to once a week.	Participants received short text messages to support their therapy. Texts were sent daily during the 7-month intervention period.	Participants in the "no" text message factor level received no text messages.	Participants were given online access to short videos and interactive exercises such as quizzes. New interactive sessions were available each week during the 7-month intervention period.	Web-based interactive exercises were not available to participants in the "no" interactive factor level.	Participants were given online access to a new story every 2 weeks about another adolescent who had overcome depression. Stories for the "high" matched factor level were tailored to the participant's sex, age, grade, and ethnicity. New stories were available biweekly for the 7-month intervention period.	Participants were given online access to a new story every 2 weeks about another adolescent who had overcome depression. Stories for the "low" matched factor level were matched only to the participant's sex. New stories were available biweekly for the 7-month intervention period.
O	verall Number of Participants Analyzed	160	160	160	160	160	160	160	160
	Mean (Standard Deviation) Unit of Measure: units on a scale								
Rov	v Title								
	Pre-intervention	25.62 (6.81)	24.33 (7.11)	25.01 (6.97)	25.59 (5.99)	23.31 (7.09)	25.61 (6.59)	25.61 (6.79)	24.99 (6.91)
	Post-intervention	18.99 (7.32)	20.38 (7.98)	19.65 (7.65)	22.45 (6.01)	17.57 (8.09)	23.55 (5.89)	18.53 (7.31)	18.06 (8.11)

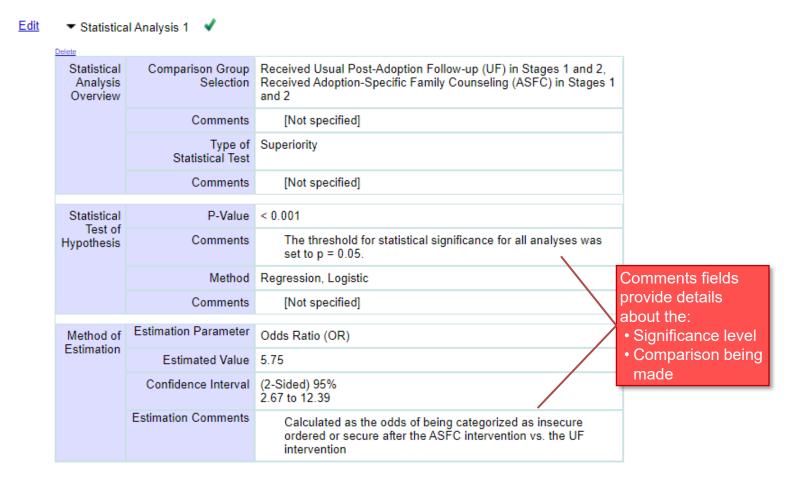
# Fractional Factorial (Scaled Assessment and ANOVA)



## Sequential, Multiple Assignment Randomized Trial (SMART) (Odds Ratios)



### Sequential, Multiple Assignment Randomized Trial (SMART) (Odds Ratios)



SMART (Effect Sizes: Cohen's d)

5. Secondary Outcome

Title: Externalizing Behavior and Internalizing Behavior Subscale Scores on the Child Behavior Checklist/6-18 (CBCL): Individual Child Education vs. Individual Child Therapy

The Time Frame defines time points in the context of stage 2.

▼ Description: Adolescent behavior based on the CBCL. The school-age CBCL is designed for children and adolescents ages 6-18 and consists of 120 questions, 113 of which are scored on a three-point Likert scale (0 = not true (as far as you know), 1 = somewhat or sometimes true, 2 = very true or often true). The scored questions are organized into eight syndrome scales; three of these, Anxious/Depressed, Withdrawn/Depressed, and Somatic Complaints, consist of a total of 32 questions and are summed to produce an Internalizing Behavior subscale score ranging from 0 to 64, while two others, Rule Breaking Behavior and Aggressive Behavior, consist of a total of 35 questions and are summed to produce an Externalizing Behavior subscale score ranging from 0 to 70. Higher scores on both subscales indicate more numerous and frequent behavioral problems.

> If reporting a score on a scale, please include the unabbreviated scale title, the minimum and maximum values, and whether higher scores mean a better or worse outcome

Time Frame: Month 3 (baseline for stage 2) and Month 6 (end of stage 2)

▼ Outcome Measure Data

▼ Analysis Population Description



The Analysis Population Description defines the criteria met by the participants who were included in the analysis.

Only those participants who completed stage 2 are included in the analysis. Nonresponders who received individual child education in stage 2 are compared to nonresponders who received individual child therapy; data are averaged over both stage 1 interventions.

Arm/Group Title | Stage 2: Individual Child Education About Adoption Stage 2: Individual Child Therapy Sessions Arm/Group Description: Individual child education about adoption consisted. A licensed clinical social worker provided weekly. of 12 weeks of access to online training about individual therapy to each adolescent for 12 weeks, adoption and books about the experiences of other with emphasis on the adoption experience and how adopted adolescents. This intervention was a stage the adolescent could handle difficult feelings. 2 add-on intervention for stage 1 nonresponders. school challenges, and integration into the new family. This intervention was a stage 2 add-on intervention for stage 1 nonresponders. Overall Number of 65 65 Participants Analyzed Mean (Standard Deviation) Unit of Measure: units on a 9.63 (5.61) 11.40 (5.93) Month 3 Externalizing Behavior Month 6 Externalizing 10.31 (5.70) 9.40 (6.46) Behavior Month 3 Internalizing 11.39 (5.21) 10.86 (4.61) Behavior 10.39 (5.13) 8.89 (4.52) Month 6 Internalizing Behavior

Full scale information is provided for each subscale and includes the:

- Range and directionality of scores for each subscale item
- Calculation to produce each subscale score
- Range and directionality of each subscale score

Participants are combined according to the additional intervention they received in stage 2.

#### SMART (Effect Sizes: Cohen's d)

