Outcome Measures and Statistical Analyses: Study Design Examples

Results Database Train-the-Trainer Workshop

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Crossover (Change from Baseline and ANCOVA)

"Change" is referenced in the Measure Title. The comparison time points are both listed in the Time Frame.

The Measure Description provides details about how the analysis was performed and how the data were summarized.

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

The Arm/Group Descriptions clearly indicate which participants contributed data to each arm.

Baseline data are presented for the combined, per-intervention populations.

Participants who completed each intervention are combined from the two intervention periods.

A Comments field provides details about the:
- Null hypothesis
- Covariates and factors
- Significance level
- Power calculation

. Primary Outcome

Title:	Change From Baseline in Mean Sitting Systolic Blood Pressure (SBP) at 2 Weeks
Description:	Blood pressure was assessed after the participant was in a seated position for at least 5 minutes. Blood pressure was measured with an automated measurement device 3 times at 1 to 2 minute intervals and a mean of the 3 measurements was calculated.
Time Frame:	Baseline and 2 Weeks

Outcome Measure Data

Analysis Population Description

All participants who received at least one dose of each intervention and completed all study visits were included in the efficacy analysis.

Arm/Group Title	Hypertena	Placebo
Arm/Group Description:	Participants who received Hypertena 20 mg tablet in a fasting state each morning in either the first or last 2 weeks of the study.	Participants who received Placebo tablet (matching Hypertena 20 mg) in a fasting state each morning in either the first or last 2 weeks of the study.
Overall Number of Participants Analyzed	127	123
Mean (Standard Deviation) Unit of Measure: mmHg		
Row Title		
SBP at Baseline	146 (19.7)	148 (18.6)
Change from Baseline at 2 weeks	-13.7 (1.7)	-7.0 (1.8)

Edit

Statistical Analysis 1

Delete

Statistical Analysis Overview	Comparison Group Selection	Hypertena, Placebo
	Comments	Null hypothesis is that there was no difference in change of SBP between Hypertena and Placebo. ANCOVA models with the trough SBP at baseline, body weight, and age as covariates, and the treatment group and study site as factors. The test was performed with a significance level of 0.05 (two-sided). A sample size of 125 participants was needed to provide 90% power to detect a 5 mmHg difference in systolic blood pressure.
	Type of Statistical Test	Superiority
	Comments	[Not specified]
Statistical Test of Hypothesis	P-Value	<0.001
	Comments	[Not specified]
	Method	ANCOVA
	Comments	[Not specified]

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

1. Primary Outcome

Title:	Maximum Tolerated Dose (MTD) of Ender-G
▼ Description:	MTD was determined by testing increasing doses up to 150 mg/m^2 twice a day via IV on dose escalation cohorts 1 to 3 with 3 to 6 participants each. MTD reflects the highest dose of drug that did not cause a Dose-Limiting Toxicity (DLT) in > 33% of participants. DLTs were defined as any Ender-G-related Common Terminology Criteria for Adverse Events Version 3.0 (CTCAE 3.0) Grade 3 or 4 adverse events (reported in the subsequent Primary Outcome Measure).
Time Frame:	Up to 8 Weeks for each dosing cohort

▼ Outcome Measure Data ✓

▼ Analysis Population Description

[Not specified]

Arm/Group Title	All Participants
▼ Arm/Group Description:	All participants who received at least 1 dose of Ender-G, either at 100 mg/m^2, 125 mg/m^2 or 150 mg/m^2 via IV.
Overall Number of Participants Analyzed	15
Measure Type: Number Unit of Measure: mg/m^2	125

Participants are combined in a single arm/group because all participants contributed to the determination of a single MTD.

The Measure Description explains how the maximum tolerated dose (MTD) was calculated.

The Measure Type "Number" is used to present a single value with no Measure of Dispersion/Precision.

A second primary outcome measure is included to report the underlying data for the MTD analysis.

2. Primary Outcome

Title:	Number of Participants Who Experienced Dose-Limiting Toxicities (DLTs)
▼ Description:	A DLT was any Grade 3 or 4 adverse event (AE) using the Common Terminology Criteria for Adverse Events Version 3.0 (CTCAE 3.0) that was possibly Ender-G-related. CTCAE 3.0 Grade 3 is a severe AE and Grade 4 is a life-threatening or disabling AE. (e.g., skin toxicity, diarrhea or antidiarrheal therapy, vomiting at same grade for >4 days despite aggressive antiemetic therapy, central nervous system, lung or renal toxicity or elevation of liver transaminases or bilirubin lasting more than 1 week) DLTs were collected to determine the Maximum-Tolerated Dose (MTD), which is defined as the dose level below the dose at which > 33% of participants experienced a DLT.
Time Frame:	Up to 8 Weeks for each dosing cohort

The Measure Description defines a dose-limiting toxicity.

▼ Outcome Measure Data ✓

▼ Analysis Population Description

All participants who received at least one dose of Ender-G.

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

Arm/Group Title	Ender-G 100 mg/m^2	Ender-G 125 mg/m^2	Ender-G 150 mg/m^2
▼ Arm/Group Description:	Cohort 1: Participants were administered 100 mg/m^2 of Ender-G via IV twice a day for 4 weeks, with 4 weeks of follow-up after the last dose was administered.	Cohort 2: Participants were administered 125 mg/m^2 of Ender-G via IV twice a day for 4 weeks, with 4 weeks of follow-up after the last dose was administered.	Cohort 3: Participants were administered 150 mg/m^2 of Ender-G via IV twice a day for 4 weeks, with 4 weeks of follow-up after the last dose was administered.
Overall Number of Participants Analyzed	3	6	6
Measure Type: Count of Participants Unit of Measure: participants	0 0%	1 16.67%	3 50%

Percentages are automatically calculated when "Count of Participants" is the Measure Type. Displaying them is optional.

Dose Escalation (Pharmacokinetics)

5. Secondary Outcome

Title:	Area Under the Concentration-Time Curve (AUC 0-72h)
▼ Description:	Blood samples were obtained and plasma concentrations were determined using a validated high-pressure liquid chromatography method.
Time Frame:	prior to the initial dose on day 1 and 0.25, 0.5, 0.75, 1, 2, 3, 4, 5, 6, 7, 8, 16, 24, 36, 48 and 72 hours post-dose

▼ Outcome Measure Data ✓

All time points used to determine the area under the curve (AUC) are included in the Time Frame.

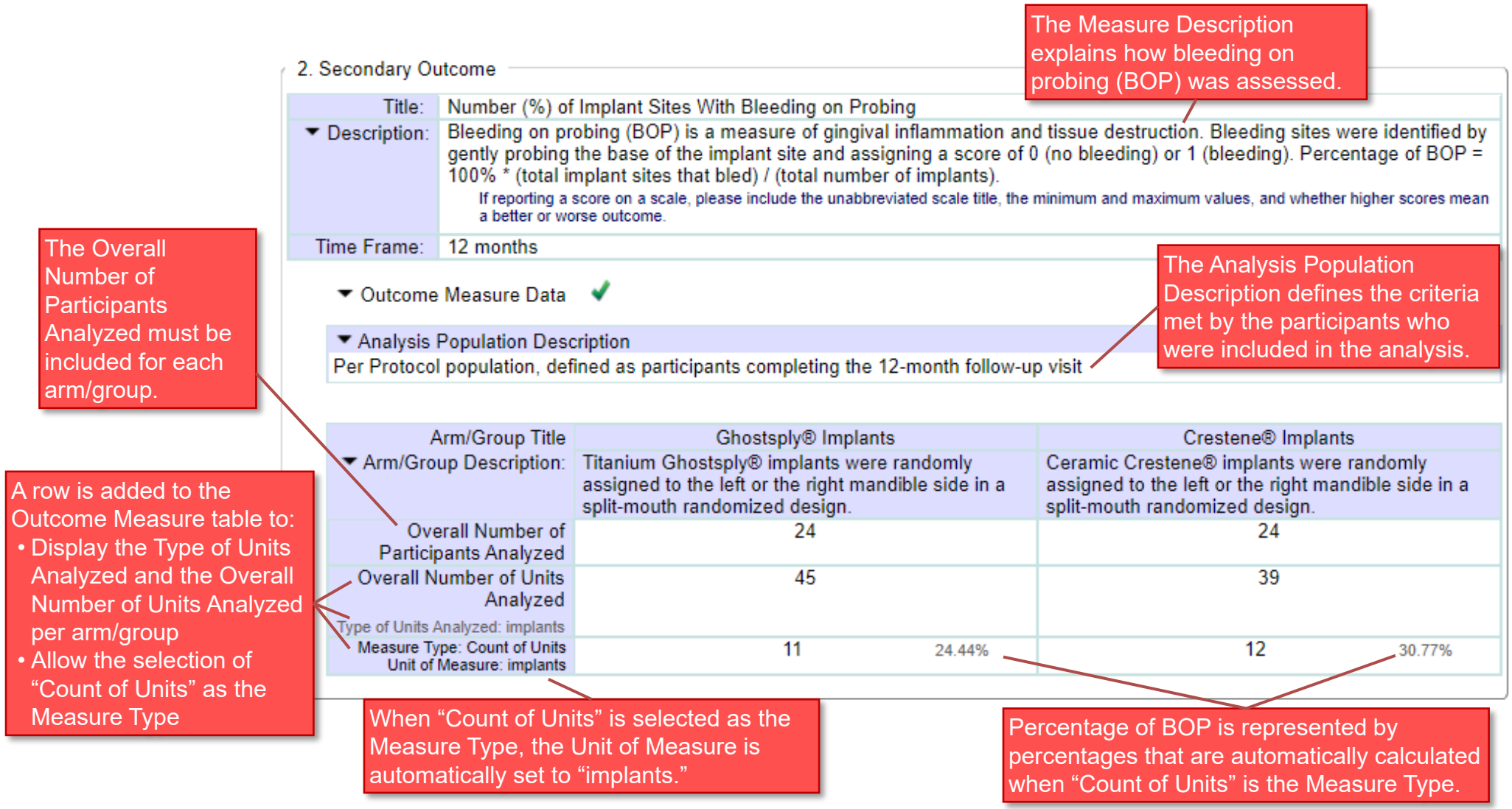
▼ Analysis Population Description

[Not specified]

Data are presented separately for each dose level.

Arm/Group Title	Ender-G 100 mg/m^2	Ender-G 125 mg/m^2	Ender-G 150 mg/m^2
▶ Arm/Group Description:	Cohort 1: Participants were adminis...	Cohort 2: Participants were adminis...	Cohort 3: Participants were adminis...
Overall Number of Participants Analyzed	3	6	6
Mean (Standard Deviation) Unit of Measure: mcg*h/mL	7.41 (7.8)	18.1 (12.7)	18.8 (14.3)

Units Other Than Participants (Count of Units)



Cluster Randomized (Incidence and Intracluster Correlation Coefficient)

"Incidence" is used precisely as defined (that is, as the number of new cases over a specified period).

The Measure Description 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 ✓

▼ Analysis Population Description

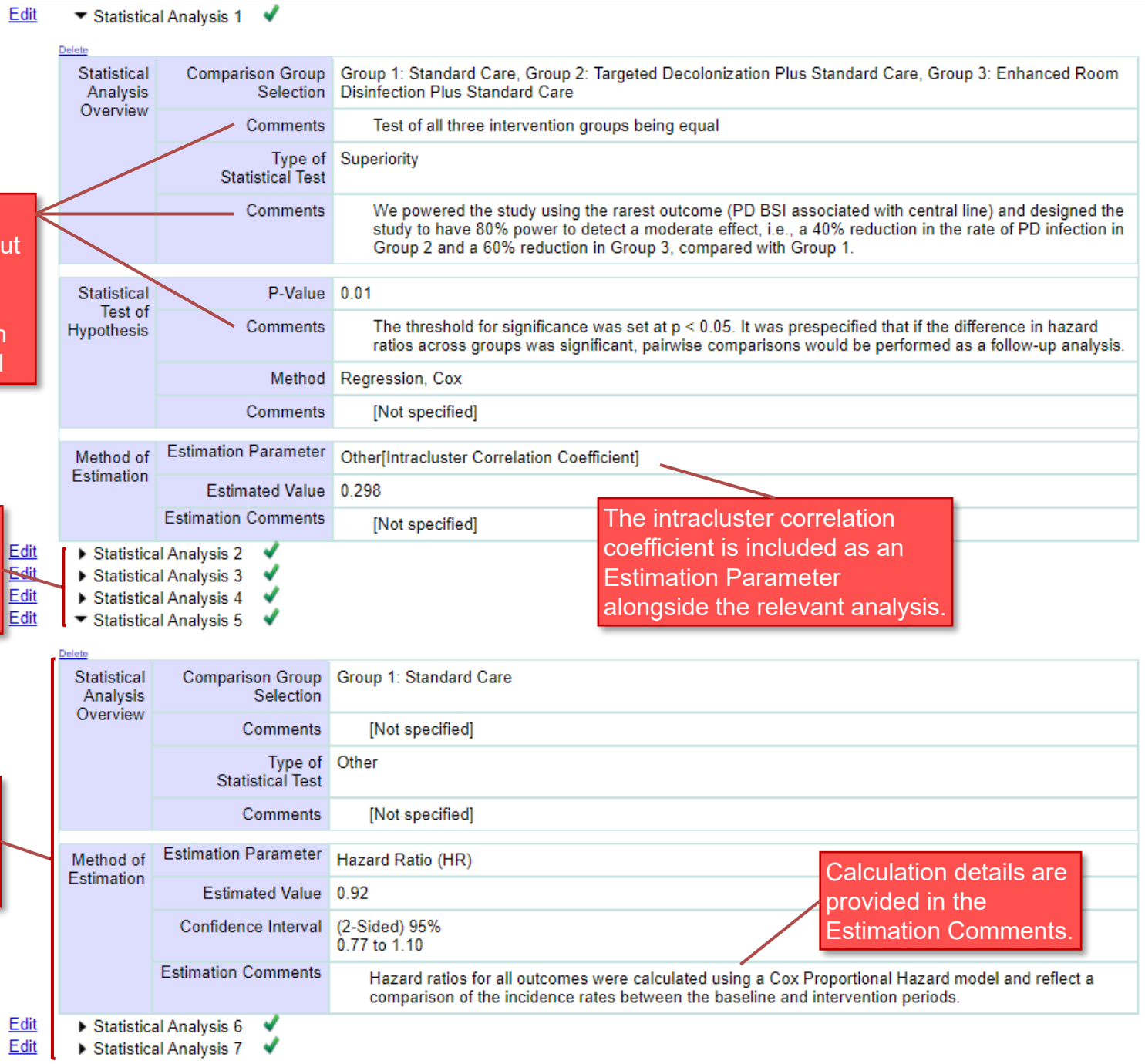
Participants assessed for ICU-attributable PD-positive culture in the baseline and intervention periods

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

Arm/Group Title		Group 1: Standard Care	Group 2: Targeted Decolonization Plus Standard Care	Group 3: Enhanced Room Disinfection Plus Standard Care
▼ Arm/Group Description:		Patients were screened for Poissonosis davrilarum (PD) infection on intensive care unit (ICU) admission. Each enrolled ICU took transmission-based precautions, based on guidance from the Centers for Disease Control and Prevention (CDC).	As in Group 1, patients were screened for PD infection on ICU admission and each enrolled ICU took transmission-based precautions, based on guidance from the CDC. In addition, PD-positive patients received a 5-day decolonization regimen of twice-daily intranasal 2% No-Bug cream and daily bathing with 4% No-Scrub sanitizing cloths.	As in Groups 1 and 2, patients were screened for PD infection on ICU admission and each enrolled ICU took transmission-based precautions, based on guidance from the CDC. In addition, rooms from which PD patients were discharged were disinfected with a solution containing hypochlorite (bleach) plus a disinfecting ultraviolet light (UV-C) device.
Overall Number of Participants Analyzed		78,653	80,685	77,593
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

Incidence values, which have no Measure of Dispersion/ Precision, are presented using the Measure Type "Number."

Cluster Randomized (Incidence and Intracluster Correlation Coefficient)



Comments fields provide details about the:
- Null hypothesis
- Power calculation
- Significance level

Pairwise comparisons (not shown) are included because they were prespecified.

The intracluster correlation coefficient is included as an Estimation Parameter alongside the relevant analysis.

Underlying statistical data (hazard ratios) are reported.

Calculation details are provided in the Estimation Comments.

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.

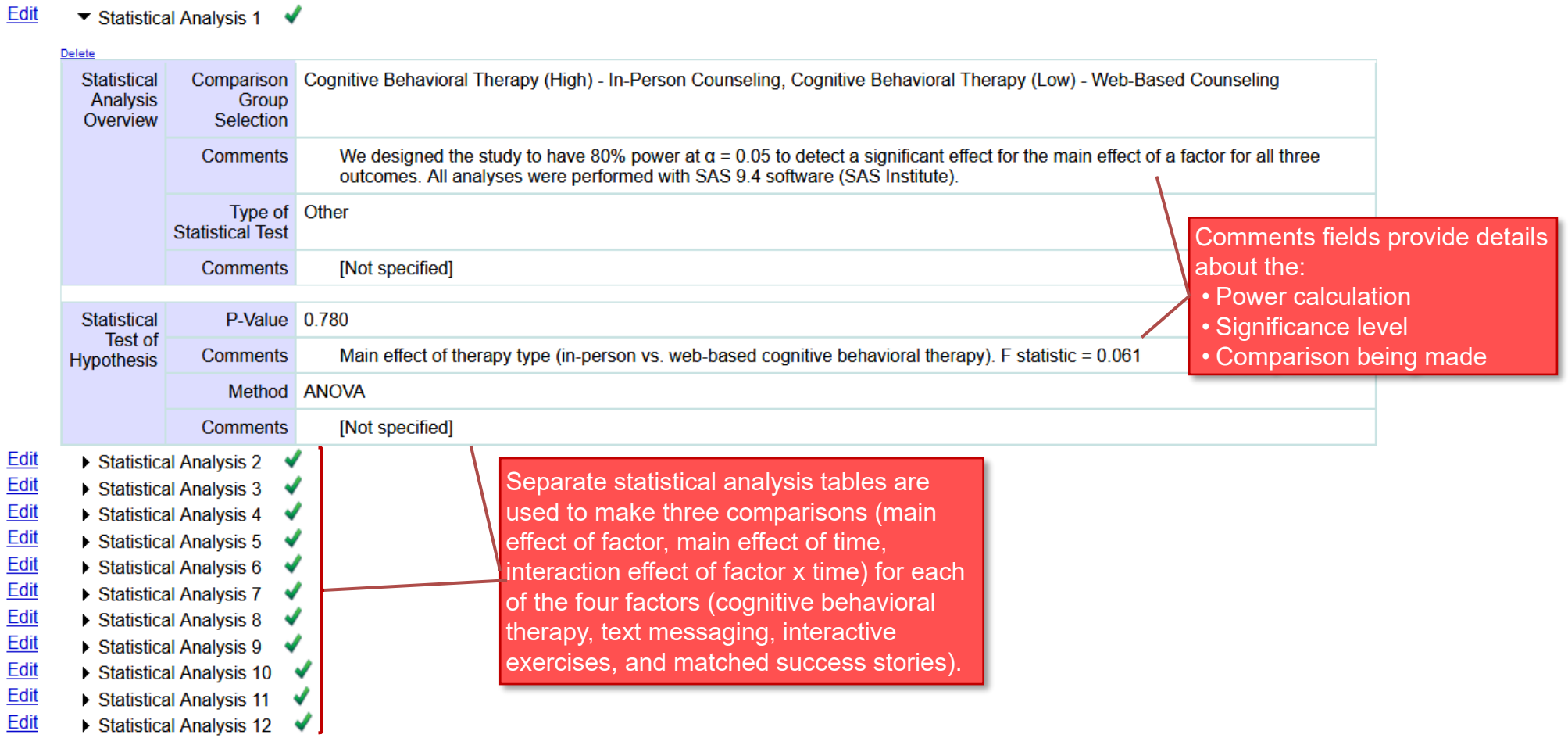
Participants are combined according to the factor level received.

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

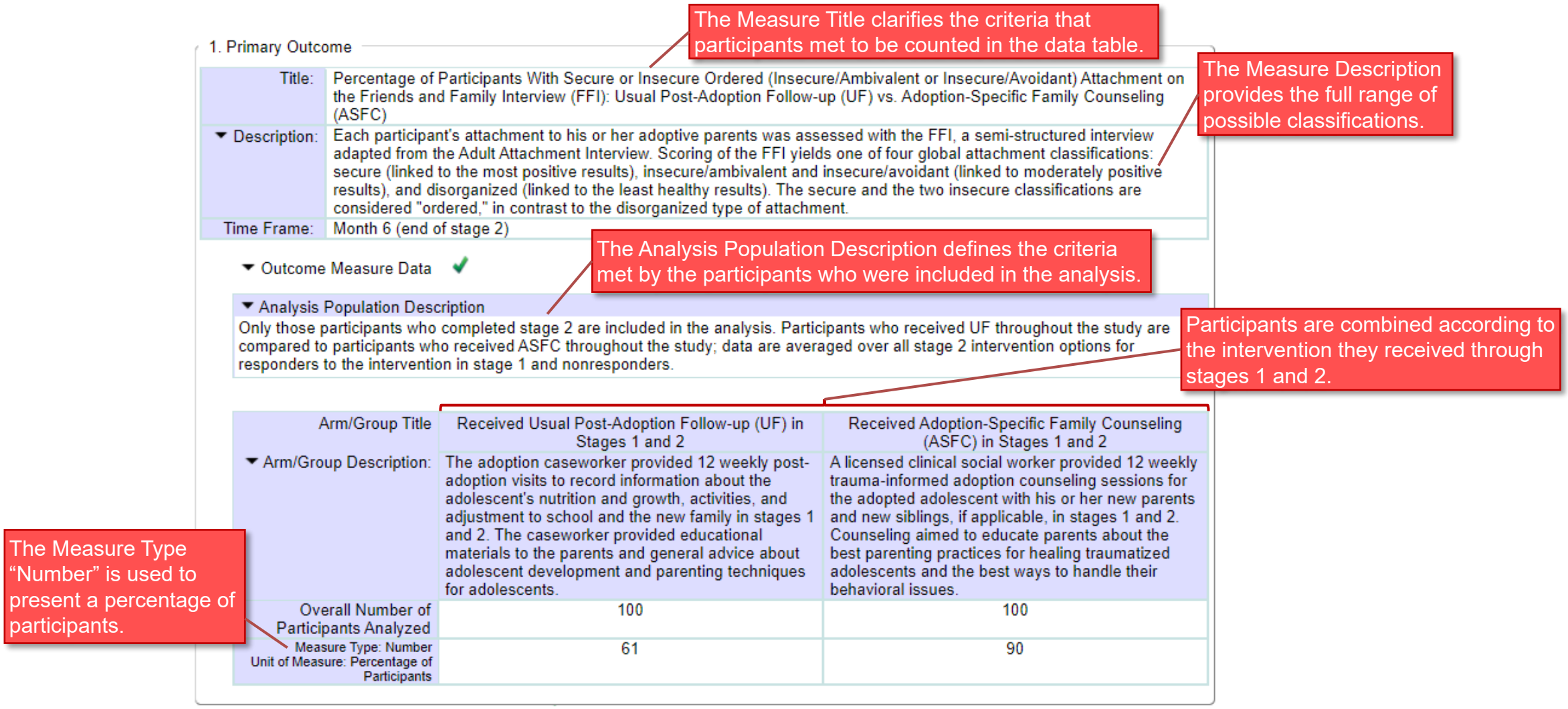
Arm/Group Title	Cognitive Behavioral Therapy (High) - In-Person Counseling	Cognitive Behavioral Therapy (Low) - Web-Based Counseling	Text Messaging (Yes)	Text Messaging (No)	Web-Based Interactive Exercises (Yes)	Web-Based Interactive Exercises (No)	Web-Based Matched Success Stories (High)	Web-Based Matched Success Stories (Low)
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.
Overall Number of Participants Analyzed	160	160	160	160	160	160	160	160
Mean (Standard Deviation) Unit of Measure: units on a scale								
Row 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)

Scores from two time points (pre- and post-intervention) are included in a primary outcome measure because they are compared in the statistical analyses.

Fractional Factorial (Scaled Assessment and ANOVA)



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



1. Primary Outcome

Title:	Percentage of Participants With Secure or Insecure Ordered (Insecure/Ambivalent or Insecure/Avoidant) Attachment on the Friends and Family Interview (FFI): Usual Post-Adoption Follow-up (UF) vs. Adoption-Specific Family Counseling (ASFC)
▼ Description:	Each participant's attachment to his or her adoptive parents was assessed with the FFI, a semi-structured interview adapted from the Adult Attachment Interview. Scoring of the FFI yields one of four global attachment classifications: secure (linked to the most positive results), insecure/ambivalent and insecure/avoidant (linked to moderately positive results), and disorganized (linked to the least healthy results). The secure and the two insecure classifications are considered "ordered," in contrast to the disorganized type of attachment.
Time Frame:	Month 6 (end of stage 2)

▼ Outcome Measure Data ✔

▼ Analysis Population Description

Only those participants who completed stage 2 are included in the analysis. Participants who received UF throughout the study are compared to participants who received ASFC throughout the study; data are averaged over all stage 2 intervention options for responders to the intervention in stage 1 and nonresponders.

Arm/Group Title	Received Usual Post-Adoption Follow-up (UF) in Stages 1 and 2	Received Adoption-Specific Family Counseling (ASFC) in Stages 1 and 2
▼ Arm/Group Description:	The adoption caseworker provided 12 weekly post-adoption visits to record information about the adolescent's nutrition and growth, activities, and adjustment to school and the new family in stages 1 and 2. The caseworker provided educational materials to the parents and general advice about adolescent development and parenting techniques for adolescents.	A licensed clinical social worker provided 12 weekly trauma-informed adoption counseling sessions for the adopted adolescent with his or her new parents and new siblings, if applicable, in stages 1 and 2. Counseling aimed to educate parents about the best parenting practices for healing traumatized adolescents and the best ways to handle their behavioral issues.
Overall Number of Participants Analyzed	100	100
Measure Type: Number Unit of Measure: Percentage of Participants	61	90

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

Edit ▼ Statistical Analysis 1 ✔

Delete

Statistical Analysis Overview	Comparison Group Selection	Received Usual Post-Adoption Follow-up (UF) in Stages 1 and 2, Received Adoption-Specific Family Counseling (ASFC) in Stages 1 and 2
	Comments	[Not specified]
	Type of Statistical Test	Superiority
	Comments	[Not specified]
Statistical Test of Hypothesis	P-Value	< 0.001
	Comments	The threshold for statistical significance for all analyses was set to p = 0.05.
	Method	Regression, Logistic
	Comments	[Not specified]
Method of Estimation	Estimation Parameter	Odds Ratio (OR)
	Estimated Value	5.75
	Confidence Interval	(2-Sided) 95% 2.67 to 12.39
	Estimation Comments	Calculated as the odds of being categorized as insecure ordered or secure after the ASFC intervention vs. the UF intervention

Comments fields provide details about the:
- Significance level
- Comparison being made

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
▼ 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

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

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

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

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

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

SMART (Effect Sizes: Cohen's d)