




A service of the U.S. National Institutes of Health

Outcome Measures Module

Results Database Train-the-Trainer Workshop
September 2015



<http://ClinicalTrials.gov>

FDAAA 801 - Outcomes

“...a table of values for each of the primary and secondary outcome measures for each arm of the clinical trial...including the results of scientifically appropriate tests of the statistical significance of such outcome measures.”

[Sec. 282(j)(3)(C)(ii)]

FDAAA 801 = Section 801 of the Food and Drug Administration Amendments Act of 2007

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Results: Outcome Measures

Publication

“At week 52, no difference was noted in major clinical responses or partial clinical responses between the placebo group (15.9% had a major clinical response ...) and the rituximab group (12.4% had a major clinical response ...)”

Response Category	Placebo (n)	Rituximab (n)	Placebo (%)	Rituximab (%)
No Clinical Response	63	119	71.6	70.4
Partial Clinical Response	11	29	12.5	17.2
Major Clinical Response	14	21	15.9	12.4
MCR+PCR	25	50	28.4	29.6

Figure 2A. Proportion of patients experiencing a major clinical response (MCR) ... at 52 weeks

ClinicalTrials.gov

Primary Outcome

Measure Title	Participants Achieving Either a Major Clinical Response (MCR) or Partial Clinical Response (PCR) Defined by British Isles Lupus Assessment Group (BILAG) Scores Over the 52-week Treatment Period
Measure Description	The BILAG Index is used for measuring clinical disease activity in Systemic Lupus ...
Time Frame	Baseline to 52 weeks

Measured Values

	Placebo + Prednisone	Rituximab + Prednisone
Number of Participants Analyzed	88	169
[units: participants]		
MCR (excluding PCR)	14	21
PCR	11	29
Nonclinical Response	63	119

Adapted from Merrill JT et al. *Arthritis Rheum* 2010 and NCT00137969

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Outcome Measure Template

http://prsinfo.clinicaltrials.gov/results_table_layout/ResultSimpleForms.html

* Outcome Measure Type	(Circle One) Primary Secondary Other Pre-specified Post-Hoc	Safety Issue?	(Circle One) Yes No
* Outcome Measure Title			
Outcome Measure Description			
* Outcome Measure Time Frame			
* Arm/Group Title			
Arm/Group Description ①			
* Number of Participants Analyzed			
Analysis Population Description			
* Measure Type	* Measure of Dispersion/Precision		
(Circle One) Number Mean Median Least Squares Mean Geometric Mean Log Mean	(Circle One) Not Applicable ② Standard Deviation Standard Error Inter-Quartile Range Full Range ____ % Confidence Interval Geometric Coefficient of Variation		
[*] Category Title ④		③	③
[*] Category Title ④		③	③
* Unit of Measure			

* Required by ClinicalTrials.gov

[*] Conditionally required by ClinicalTrials.gov

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Outcome Measures Checklist

http://prsinfo.clinicaltrials.gov/results_table_layout/ResultSimpleForms.html

Outcome Measure Data Preparation Checklist

Overview: A tabular summary of outcome measure results by comparison group. You must report tables for each pre-specified primary and secondary outcome and any appropriate statistical analyses. The outcomes that were pre-specified in the Protocol Section of the record will be available to use and edit during results data entry. You may also include other pre-specified and post hoc outcomes. Use this checklist with the [Outcome Measure Simple Results Template](#) and [Results Data Element Definitions](#).

	Information to have available for each Outcome Measure	Term
<input type="checkbox"/>	<ul style="list-style-type: none">Label the measure as Primary, Secondary, Other Pre-specified, or Post hoc.	¹ Outcome Measure Type
<input type="checkbox"/>	<ul style="list-style-type: none">Title—Describe specifically what was measured and will be reported as data<ul style="list-style-type: none">For example, "Change from baseline in systolic blood pressure at 6 months" specifically describes what was measured and how the outcome data will be reported; "Principle Vital Signs" does not.Description—Any elaboration needed to understand the measure and the reported data. Information should be written for a public audience (i.e., not specialists in your field, but general readers of the medical literature).<ul style="list-style-type: none">For example, a description of how the measure was taken, relevant definitions (e.g., explain "response"), any methods of assessment, and/or calculations that were performed to summarize the dataIf the measure was based on a scale, explain any numerical categories or provide the range and direction of possible scores (0=no pain; 10=worst possible pain) to allow a reader to properly interpret any reported values.	¹ Outcome Measure Title ¹ Outcome Measure Description
<input type="checkbox"/>	<ul style="list-style-type: none">The time point(s) or duration over which a participant was assessed for the measure, and for which data are being reported<ul style="list-style-type: none">For a time-to-event measure—A definition of the stopping rule and the longest duration over which a participant was observed (e.g., from randomization until death, up to 3 years)	¹ Outcome Measure Time Frame
<input type="checkbox"/>	<ul style="list-style-type: none">The number of separate groups for which summary data will be providedTip: Generally equal to the number of intervention strategies or groups compared	Arm/Groups
<input type="checkbox"/>	<ul style="list-style-type: none">For each group:<ul style="list-style-type: none">Title—A descriptive label for the group (header for table column). Use informative labels (e.g., "Placebo"), not generic labels (e.g., "Group 1").Description—A detailed explanation of the participants included in the group and the interventions received. This may include a description of how groups of participants were recombined for analysis purposes.	² Arm/Group Title ² Arm/Group Description
<input type="checkbox"/>	<ul style="list-style-type: none">Number of participants, in each group, from whom data were collected and summarized.<ul style="list-style-type: none">If the unit of analysis is not participants, also provide the name of the unit (e.g., eyes, lesions) and the number of units [Type/Number Units Analyzed].	² Number of Participants Analyzed

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Outcome Measures Conceptual Framework

Four Levels of Specification in Reporting Outcome Measures

Time Frame: Baseline and Time X

Level 1
Domain:

Anxiety

Depression

Schizophrenia

Etc.

Level 2
Specific Measurement:

Beck Anxiety Inventory

Hamilton Anxiety Rating Scale

Fear Questionnaire

Level 3
Specific Metric:

End Value

Change from Baseline

Time to Event

Level 4
Method of Aggregation:

Continuous

Categorical

Mean

Median

Proportion with Decrease ≥ 50%

Proportion with Decrease ≥ 8 points

Zarin DA, Tse T, Williams RJ, Califf RM, Ide NC. *N Engl J Med* 2011; 364:852-860.

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Specification of Outcome Measures in Protocol

Level	Primary OMs (% Total) n=100
1 – Domain (only)	36%
2 – Specific Measurement	25%
3 – Specific Metric	26%
4 – Method of Aggregation	13%
Included Specific Timeframe	63%

Zarin DA, Tse T, Williams RJ, Califf RM, Ide NC. *N Engl J Med* 2011; 364:852-860.

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Best Practices

- If the Number of Participants Analyzed is not the same as a “row” in Participant Flow (Started, Completed, Other Milestone), describe the population in the Analysis Population Description
- Use multiple Outcome Measures to report results for the same measure at different time points
- If the reporting groups are different from Participant Flow, use Outcome Measure Arm/Group Title/Descriptions to explain and relate to Participant Flow Arm/Group Title/Descriptions

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Outcome Measures

Tutorial

Results Section

ID: Parallel 2015Parallel Study Design Example 2015[NCT ID not yet assigned]

Results Section

Record Summary

Preview Results

Download Results XML

Delete Results

Help

Open

Participant Flow

Pre-assignment Details

Trial Period:Overall StudyTotal Started: 200[Protocol Enrollment: 200]

Open

Baseline Characteristics

Overall Number of Baseline Participants: 200

Age, Continuous

Gender, Male/Female

Race/Ethnicity, Customized

Region of Enrollment

Quebec Task Force Classification of Spinal Disorders [Study-Specific Measure]

Body Mass Index [Study-Specific Measure]

Short Pain Scale (SPS-11) Score [Study-Specific Measure]

Duration of Condition A [Study-Specific Measure]

Height [Study-Specific Measure]

Weight [Study-Specific Measure]

Open

Outcome Measures

Information is required

Edit

Adverse Events

Information is required

Edit

Limitations and Caveats

[Not Specified]

Outcome Measures Overview

ID: Parallel 2015Parallel Study Design Example 2015[NCT ID not yet assigned]

Outcome Measures Overview

[Results Section](#)[Add Outcome Measure](#)[Reorder Outcome Measures](#)[Help](#)[Definitions](#)[Show All](#)

Outcome Measures copied from Protocol Section

Outcome Measure Data is required for at least one primary outcome measure.

1. Primary Outcome

Edit

Delete

Title:Change From Baseline in Pain on the 11-point Short Pain Scale (SPS-11) at Week 24

Description:[Not specified]

Time Frame:Week 24

Safety Issue?No

Outcome Measure Data Not Reported

2. Secondary Outcome

Edit

Delete

Title:Number of Patients With a 50 Percent or Greater Reduction in Pain as Determined by SPS-11 at Week 12

Description:[Not specified]

Time Frame:Week 12

Safety Issue?No

Outcome Measure Data Not Reported

3. Secondary Outcome

Edit

Delete

Title:Number of Patients With a 50 Percent or Greater Reduction in Pain as Determined by SPS-11 at Week 24

Description:[Not specified]

Time Frame:Week 24

Safety Issue?No

Outcome Measure Data Not Reported

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Edit Outcome Measure Title Fields

ID: Parallel 2015Parallel Study Design Example 2015[NCT ID not yet assigned]

Edit Outcome Measure Title Fields

[Help](#)[Definitions](#)

* Outcome Measure Type:Primary

* Outcome Measure Title:Change from baseline in pain on the 11-point Short Pain Scale (SPS-11) at week 24

Characters remaining: 174

* Outcome Measure Time Frame:Week 24

Outcome Measure Description:SPS-11 is a validated, self-reported instrument assessing average pain intensity over the past 24 hour period. Possible scores range from 0 (no pain) to 10 (worst possible pain). Change = (Week 24 Score - Baseline score)

Characters remaining: 779

(t) Safety Issue?Is this outcome measure assessing a safety issue?

No

Save

Cancel

Enter Outcome Measure Data

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Select Outcome Measure Arms/Groups

ID: Parallel 2015
Parallel Study Design Example 2015
[NCT ID not yet assigned]

Select Outcome Measure Arms/Groups

Outcome Measure Title: Change from baseline in pain on the 11-point Short Pain Scale (SPS-11) at week 24

Time Frame: Week 24

Description: SPS-11 is a validated, self-reported instrument assessing average pain intensity over the past 24 hour period. Possible scores range from 0 (no pain) to 10 (worst possible pain). Change = (Week 24 Score - Baseline score)

Before entering Outcome Measure data, use a Select button to define the Arms/Groups in your study. You can edit the information on the next screens.

[Help](#)
[Definitions](#)

Copy from: Protocol Section	Arm/Group	Arm/Group
<div> <div>Title</div> <div>Description</div> </div>	<div>Remuverol</div> <div>Participants received Remuverol 15 mg tablet orally twice daily for 24 weeks....</div>	<div>Placebo</div> <div>Participants received Remuverol placebo tablet matching Remuverol orally twice daily for 24 weeks....</div>

Copy from: Participant Flow	Arm/Group	Arm/Group
<div> <div>Title</div> <div>Description</div> </div>	<div>Remuverol</div> <div>Participants received Remuverol 15 mg tablet orally twice daily for 24 weeks.</div>	<div>Placebo</div> <div>Participants received Remuverol placebo tablet matching Remuverol orally twice daily for 24 weeks.</div>

Create: New	Define New Arms/Groups
<div> <div>Select</div> </div>	

Edit Outcome Measure Arms/Groups

ID: Parallel 2015 Parallel Study Design Example 2015 [NCT ID not yet assigned]

Edit Outcome Measure Arms/Groups

Arms/Groups copied from: Participant Flow

[+ Add Arm/Group](#) [Help](#) [Definitions](#)

* Arm/Group Title:			
	Remuverol	Characters remaining: 922	Placebo
Arm/Group Description:	Participants received Remuverol 15 mg tablet orally twice daily for 24 weeks.		Participants received Remuverol placebo tablet matching Remuverol orally twice daily for 24 weeks.
	< Delete	Move ►	< Delete ◀ Move

Save
Cancel

Outcome Measure Data

ID: Parallel 2015Parallel Study Design Example 2015[NCT ID not yet assigned]

HelpDefinitions

* Outcome Measure Type:Primary

Characters remaining: 174

* Outcome Measure Title:Change from baseline in pain on the 11-point Short Pain Scale (SPS-11) at week 24

Characters remaining: 779

Outcome Measure Description:SPS-11 is a validated, self-reported instrument assessing average pain intensity over the past 24 hour period. Possible scores range from 0 (no pain) to 10 (worst possible pain). Change = (Week 24 Score - Baseline score)

Characters remaining: 779

* Outcome Measure Time Frame:Week 24

(t) Safety Issue?No

Arms/Groups (2)Add Arm/Group

Arm/Group Title:Remuverol

Arm/Group Description:Participants received Remuverol 15 mg tablet orally twice daily for 24 weeks.

Number of Participants Analyzed:101

Arm/Group Title:Placebo

Arm/Group Description:Participants received Remuverol placebo tablet matching Remuverol orally twice d...

Number of Participants Analyzed:99

Add Units Analyzed

(Optional) Use only if analysis is based on units other than participants (e.g., eyes, lesions, implants).

Analysis Population Description:Intent to treat population (all participants who received at least one dose of intervention). Last observation carried forward (LOCF) imputation method.

Characters remaining: 198

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Outcome Measure Data (cont.)

Outcome Measure Data Table

* Measure Type:Mean

* Measure of Dispersion/Precision:Standard Error

	Remuverol	Placebo
Mean	-3.84	-2.08
Standard Error	0.61	0.51

Add Category

* Unit of Measure:units on a scale

Commonly reported units: participants years units on a scale percentage of <something>

Save

Validate

Cancel

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Outcome Measures Overview

ID: Parallel 2015Parallel Study Design Example 2015[NCT ID not yet assigned]

Outcome Measures Overview

Results Section

Add Outcome Measure

Reorder Outcome Measures

Help

Definitions

Show All

1. Primary Outcome

Edit

Delete

Copy

Title:Change From Baseline in Pain on the 11-point Short Pain Scale (SPS-11) at Week 24

Description:SPS-11 is a validated, self-reported instrument assessing average pain...

Time Frame:Week 24

Safety Issue?No

▼ Outcome Measure Data

▼ Analysis Population Description

Arm/Group Title	Remuverol	Placebo
Arm/Group Description: Participants received Remuverol 15 ...	Participants received Remuverol 15 ...	Participants received Remuverol pla...
Number of Participants Analyzed	101	99
Mean (Standard Error)	-3.84 (0.61)	-2.08 (0.51)
Units: units on a scale		

Add Statistical Analysis 1

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Add Outcome Statistical Analysis

ID: Parallel 2015Parallel Study Design Example 2015[NCT ID not yet assigned]

Add Outcome Statistical Analysis

Primary Outcome

Title:Change from baseline in pain on the 11-point Short Pain Scale (SPS-11) at week 24

Time Frame:Week 24

Unit of Measure:units on a scale

Tip: Many of the data elements are optional and may be left blank. The minimum requirements are to enter either a P-Value OR an Estimation Parameter (e.g., Mean Difference, Odds Ratio). A Confidence Interval for the Estimation Parameter may also be entered.

Statistical Analysis Overview

Help

Definitions

* Comparison Group Selection:

Select the Outcome Measure Arms/Groups involved in the statistical analysis.
☒ Remuverol ☐ Placebo

Comments:

(Optional) Additional details about the statistical analysis, such as null hypothesis and description of power calculation.

Characters remaining: 102

It was calculated that 200 participants randomized in a 1:1 fashion between the 2 arms would have at least 85% power to detect a difference of 0.56 points in mean SPS-11 pain score between Remuverol and placebo from baseline to week 24. Sample size was determined using a 2-sided 2-sample t test (alpha = 0.05). Assumptions included a common standard deviation of 1.14 and a discontinuation rate of 7%.

* Non-inferiority or Equivalence Analysis?

No

Comments:

If "Yes" (non-inferiority or equivalence analysis), describe details of the power calculation (if not previously provided), definition of non-inferiority margin, and other key parameters.

Characters remaining: 500

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Add Outcome Statistical Analysis (cont.)

Statistical Test of Hypothesis

[Help](#)

[Definitions](#)

P-Value:

(If applicable)

0.002

 (e.g. <0.01)

Comments:

(Optional) Additional information, such as whether or not the p-value is adjusted for multiple comparisons and the *a priori* threshold for statistical significance.

Characters remaining: 250

Method:

(Required if a P-Value is entered)

Mixed Models Analysis

 If other, please specify:

Comments:

(Optional) Any other relevant information, such as adjustments or degrees of freedom.

Characters remaining: 150

Save

Validate

Cancel

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Outcome Measures Overview

ID: Parallel 2015

Parallel Study Design Example 2015

[NCT ID not yet assigned]

Results Section

Add Outcome Measure

Reorder Outcome Measures

[Help](#)

[Definitions](#)

Show All

1. Primary Outcome

Edit

Details

Copy

Title:

Change From Baseline in Pain on the 11-point Short Pain Scale (SPS-11) at Week 24

Description:

SPS-11 is a validated, self-reported instrument assessing average pain...

Time Frame:

Week 24

Safety Issue?

No

Outcome Measure Data

Analysis Population Description

Arm/Group Title	Remuverol	Placebo
Arm/Group Description: Participants received Remuverol 15 ...		Participants received Remuverol pla...
Number of Participants Analyzed	101	99
Mean (Standard Error)	-3.84 (0.61)	-2.08 (0.51)
Units: units on a scale		

Statistical Analysis 1

Details

Statistical Analysis Overview

Comparison Groups	Remuverol, Placebo
Comments	It was calculated that 200 participants randomized in a 1:1 fashion between the 2 arms would have at least 85% power to detect a difference of 0.56 points in mean SPS-11 pain score between Remuverol and placebo from baseline to week 24. Sample size was determined using a 2-sided 2-sample t test ($\alpha = 0.05$). Assumptions included a common standard deviation of 1.14 and a discontinuation rate of 7%.
Non-Inferiority or Equivalence Analysis?	No
Comments	[Not specified]

Statistical Test of Hypothesis

P-Value

0.002

Comments

[Not specified]

Method

Mixed Models Analysis

Comments

[Not specified]

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Enter Outcome Measures

- Example Study Designs
 - Factorial
 - Crossover
 - Cluster Randomized
 - Dose Escalation
 - Multiple Period

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