Baseline Characteristics: Introduction

Results Database Train-the-Trainer Workshop

August 2021

Results Information Submission

42 CFR Part 11 – Subpart C

§ 11.48 – What constitutes clinical trial results information?

42 CFR 11.48(a) applies to applicable clinical trials required to register and with a Primary Completion Date on or after January 18, 2017 (effective date).

Results information consists of:

- Participant flow
- Demographic and baseline characteristics
- Outcomes and statistical analyses
- Adverse event information
- Protocol and statistical analysis plan
- Administrative information
- Additional clinical trial results information for applicable device clinical trials of unapproved or uncleared device products

What Are Baseline Characteristics?

"A table of the demographic and baseline data collected overall and for each arm of the clinical trial . . ."

From: FDAAA 801, Sec. 282(j)(3)(C)(i)

What Is Included in the Baseline Characteristics?
42 CFR 11.48(a)(2)

- Arm/Group Information (Arm/Group Title and Arm/Group Description)
- Baseline Analysis Population Information
  - Overall Number of Baseline Participants
  - Overall Number of Units Analyzed
    - If the analysis is based on a unit other than participants, a description of the unit of analysis (e.g., eyes, lesions)
  - Baseline Analysis Population Description
    - If the Overall Number of Baseline Participants (or units) differs from the number of human subjects (or units) assigned to the arm

Arm/Group Title	Rituximab 1000 mg + Prednisone	Placebo + Prednisone	Total
▼ Arm/Group Description	Participants received rituximab 1000 mg intravenously (IV) on Days 1, 15, 168, and 182. Participants also received an initial dose of prednisone (0.5, 0.75, or 1.0 mg/kg orally once a day) with tapering beginning at Day 16 for 10 weeks to a dose of ≤ 10 mg/day. Participants also received acetaminophen 1000 mg orally and diphenhydramine 50 mg orally prior to study drug infusion.	Participants received placebo intravenously on Days 1, 15, 168, and 182. Participants also received an initial dose of prednisone (0.5, 0.75, or 1.0 mg/kg orally once a day) with tapering beginning at Day 16 for 10 weeks to a dose of ≤ 10 mg/day. Participants also received acetaminophen 1000 mg orally and diphenhydramine 50 mg orally prior to study drug infusion.	Total of all reporting groups
Overall Number of Baseline Participants	169	88	257
▼ Baseline Analysis Population Description	Intent-to-treat population: All randomized participants who received any amount of study drug.		

What Is Included in the Baseline Characteristics?
42 CFR 11.48(a)(2)

- Baseline Measure Information
  - Age
  - Sex/Gender
  - Race and Ethnicity (if collected under the protocol)
  - Other measure(s) that were assessed at baseline and used in the analysis of the primary outcome measure(s)

Age, Continuous Mean (Standard Deviation) Unit of measure: Years				
	Number Analyzed	169 participants	88 participants	257 participants
		40.2 (11.4)	40.5 (12.8)	40.3 (11.9)
Sex: Female, Male Measure Type: Count of Participants Unit of measure: Participants				
	Number Analyzed	169 participants	88 participants	257 participants
	Female	152 89.9%	82 93.2%	234 91.1%
	Male	17 10.1%	6 6.8%	23 8.9%

Results from: NCT00137969

What Is Included in the Baseline Characteristics?
42 CFR 11.48(a)(2)

- Baseline Measure Information
  - Name and Description of Measure, including any categories used to submit Baseline Measure Data
  - Measure Type and Measure of Dispersion
  - Unit of Measure
- Baseline Measure Data
  - Option for specifying when data are mutually exclusive and exhaustive ("categories")
- Number of Baseline Participants (and Units)
  - If different from the Overall Number of Baseline Participants or Overall Number of Units Analyzed

Age, Continuous Mean (Standard Deviation) Unit of measure: Years				
	Number Analyzed	169 participants	88 participants	257 participants
		40.2 (11.4)	40.5 (12.8)	40.3 (11.9)

Age, Customized Measure Type: Number Unit of measure: Participants	Number Analyzed	169 participants	88 participants	257 participants
< 20 years		2	2	4
20 to 64 years		166	83	249
> 64 years		1	3	4

Where Do Baseline Characteristics Data Come From?

Publication (Table 1)

Table 1. Baseline demographic and disease characteristics of the patients*

Characteristic	Placebo (n = 88)	Rituximab (n = 169)
Female sex	93.2	89.9
Age, mean ± SD years	40.5 ± 12.8	40.2 ± 11.4
Race, %		
White	55.7	56.2
African American	27.3	23.7
Hispanic	9.1	14.2
Asian/Pacific Islander	5.7	3.6
Other	2.2	1.1
Disease duration, mean ± SD years	8.7 ± 7.6	8.5 ± 7.2
Long-term prednisone therapy†	53.4	58.6
Assigned prednisone dosage at screening, mg/kg/day		
0.5	61.4	62.7
0.75	29.5	32.0
1.0	9.1	5.3
Background immunosuppressive drug		
Azathioprine	36.4	32.0
Methotrexate	27.3	27.8
Mycophenolate mofetil	36.4	39.6

ClinicalTrials.gov

Arm/Group Title		Placebo + Prednisone		Rituximab + Prednisone		Total	
▸ Arm/Group Description		Participants received rituximab 100...		Participants received placebo intra...			
Overall Number of Baseline Participants		88		169		257	
▸ Baseline Analysis Population Description							
Age, Continuous Mean (Standard Deviation) Unit of measure: years	Number Analyzed	88 participants		169 participants		257 participants	
		40.5 (12.8)		40.2 (11.4)		40.3 (11.9)	
Sex: Female, Male Measure Type: Count of Participants Unit of measure: participants	Number Analyzed	88 participants		169 participants		257 participants	
	Female	82	93.18%	152	89.94%	234	91.05%
	Male	6	6.82%	17	10.06%	23	8.95%
Race/Ethnicity, Customized Measure Type: Count of Participants Unit of measure: participants	Number Analyzed	88 participants		169 participants		257 participants	
White		49	55.68%	95	56.21%	144	56.03%
African American		24	27.27%	40	23.67%	64	24.9%
Hispanic		8	9.09%	24	14.2%	32	12.45%
Asian/Pacific Islander		5	5.68%	6	3.55%	11	4.28%
Other		2	2.27%	2	1.18%	4	1.56%
Disease duration Mean (Standard Deviation) Unit of measure: years	Number Analyzed	88 participants		169 participants		257 participants	
		8.7 (7.6)		8.5 (7.2)		8.6 (7.3)	

Adapted from: Merrill JT, et al. *Arthrit Rheum*, 2010 and NCT00137969

Best Practices