BIOSTAT 702: Exercise 1.2

Table 1: Describing The Study Participants

Fall 2025

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How to Do This Exercise

We recommend that you read this entire document prior to answering any of the questions. If anything is unclear please ask for help from the instructors or TAs before getting started. You are also allowed to ask for help from the instructors or TAs while you are working on the assignment. You may collaborate with your classmates on this assignment—in fact, we encourage this–and use any technology resources available to you, including Internet searches, generative AI tools, etc. However, if you collaborate with others on this assignment please be aware that *you must submit answers to the questions written in your own words. This means that you should not quote phrases from other sources, including AI tools, even with proper attribution.* Although quoting with proper attribution is good scholarly practice, it will be considered failure to follow the instructions for this assignment and you will be asked to revise and resubmit your answer. In this eventuality, points may be deducted in accordance with the grading rubric for this assignment as described below. Finally, you do not need to cite sources that you used to answer the questions for this assignment.

Grading Rubric

The assignment is worth 20 points (4 points per question). The points for each question are awarded as follows: 3 points for answering all parts of the question and following directions, and 1 point for a correct answer. Partial credit may be awarded at the instructor's discretion.

Resources

The following resources on Canvas / the internet will be helpful for answering the questions for this exercise.

1. The article by Douglas Altman, Comparability of Randomised Groups (The Statistician (1985) 34, pp. 125-136). The PDF is available on Canvas and the article can be viewed online here.
2. The CONSORT 2010 statement available here.
3. The CONSORT 'explanation and elaboration' paper. The PDF is available on Canvas and the article can be viewed online here.
4. The manuscript by Jabbour titled *Ponatinib vs Imatinib in Frontline Philadelphia Chromosome-Positive Acute Lymphoblastic Leukemia: A Randomized Clinical Trial*. The PDF is available on Canvas and the article can also be viewed online here.
5. The dataset for this exercise, simulated_clinical_trial_data.csv.

Question 1 (4 points)

Complete the first 12 items of the CONSORT checklist for the trial reported by Jabbour (you can ignore items 13-25). How closely do the authors adhere to the CONSORT guidelines?

Question 2 (4 points)

1. Write a brief description of the study by Jabbour in your own words, including the following:
   (a) Research objectives
   (b) Patient population recruited for the study
   (c) Study design
   (d) Primary outcome
   (e) Result for the primary outcome
2. Answer the following questions about Table 1 in the article by Jabbour.
   (a) Which variables are continuous?
   (b) Which variables are unordered categorical variables?
   (c) Which variables are ordered categorical variables?
   (d) Why does the table not have an "overall" column that shows descriptive statistics for all 245 patients?
   (e) Why are there no p-values in Table 1?

Question 3 (4 points)

1. Review the CONSORT diagram in Figure 1 from the article by Jabbour and write a brief summary of what you see. Describe reasons why patients were excluded (see notes under Table 1 for additional details) and what, if anything, might cause you to be concerned about generalizing results from those who were analyzed to those who were enrolled and randomized. After doing this, consider what the target population for the study is and list any concerns you have about generalizing the study results to that population.

2. Summarize Altman's argument about not performing statistical testing on Table 1 in a randomized trial. *Note: Your instructors are aware that understanding Altman's argument on a deep level assumes familiarity with material you haven't yet covered in BIOSTAT 701. Don't worry about that for now. Simply do your best to describe Altman's argument in a non-technical fashion using what you currently know about statistics.*

3. Shift your attention back to the ultra-running study for a moment. Recall that this is an observational study instead of a randomized trial, and that our objective (which we are slowly building towards) is to run a simple linear regression predicting best running time based on emotional intelligence. In the last exercise you created a table that compared the 73 participants who would be excluded from the simple linear regression with the 211 participants who would be included. The table had a very similar structure to the typical Table 1 from a randomized trial, i.e., that it compares baseline characteristics between two independent groups. The purpose of the table was different, however. In other words, the exercise with the ultra-running study was intended to identify selection bias, i.e., systematic differences between the analysis set and the entire sample. This is contrasted with the purpose of Table 1 in a randomized trial, which is to assess imbalance in baseline factors that might result in another kind of bias called confounding, which as Altman demonstrates in his paper, can happen even in a randomized trial. This phenomenon occurs specifically when the imbalance is on factor(s) that are also associated with the outcome.

   You already answered a question above about why it doesn't make sense to use p-values to asses baseline differences between randomized groups in a clinical trial. Explain why it also doesn't make sense to use p-values in the ultra-running study context, where our objective was to assess selection bias.

Question 4 (4 points)

Suppose that you were the statistician for this trial and were asked to create Table 1 from the raw data. Generate a Table 1 using the variables described below, stratifying by Group, and including a p-value column as well as a standardized mean difference column. *Note: The table won't exactly match the format of Table 1 in the article, and the summary statistics from the included dataset won't exactly match the numbers from Table 1 in the article either. Also note that we are asking you to put p-values in this table so that you can answer some of the questions we are asking in this exercise, not because p-values are appropriate in a table like this.*

The following is a description of the variables in the data file.

Age –	age of the patient at randomization, in years
Gender –	0=Male, 1=Female
ECOG -	Eastern Cooperative Oncology Group Performance Status
CNS -	Central Nervous System Disease, 0=No, 1=Yes
BCR -	BCR:ABL1 Isoform 0=p210, 1=p190
CV1 -	>=1 Cardiovascular Comorbidity, 0=No, 1=Yes
CV2 -	>=2 Cardiovascular Comorbidity, 0=No, 1=Yes
Hypertension -	0=No, 1=Yes
Diabetes -	0=No, 1=Yes
Obesity -	0=No, 1=Yes
Dyslipidemia -	0=No, 1=Yes
Smoking -	History of Smoking, 0=No, 1=Yes
Treatment –	randomized treatment group

Question 5 (4 points)

Answer the following questions about the Table 1 that you generated.

1. Would you reject the null hypothesis of no difference between the treatment arms for any of the baseline characteristics in Table 1 (using a 5% alpha level)?

2. If you were to interpret the p-value as continuous, rather than using a threshold value like 5%, then are there any factors for which the p-value is suggesting the evidence leans in favor of the alternative hypothesis (i.e., that the distribution of a factor is actually different in patients assigned to Ponatinib vs. Imatinib)? Discuss why this doesn't make any sense in this context. Refer to Altman's paper as a guide as you think about this.

3. Are there any factors that are imbalanced between the groups when you look at the standardized mean differences (use 0.2 as a threshold)? List the factors and describe the imbalance that you see.

4. Why do you think the standardize mean differences are a more satisfactory approach to assessing imbalance than using p-values?

5. Suppose that the factors you identified as being imbalanced were also strongly associated with the primary outcome of the study. Using Altman's argument, what might you consider doing if you were the statistician for the study?

6. Suppose that the factors you identified as being imbalanced had no association with the primary outcome of the study. Again, referring to Altman's paper, is the imbalance a concern and what, if anything, would you consider doing about it?