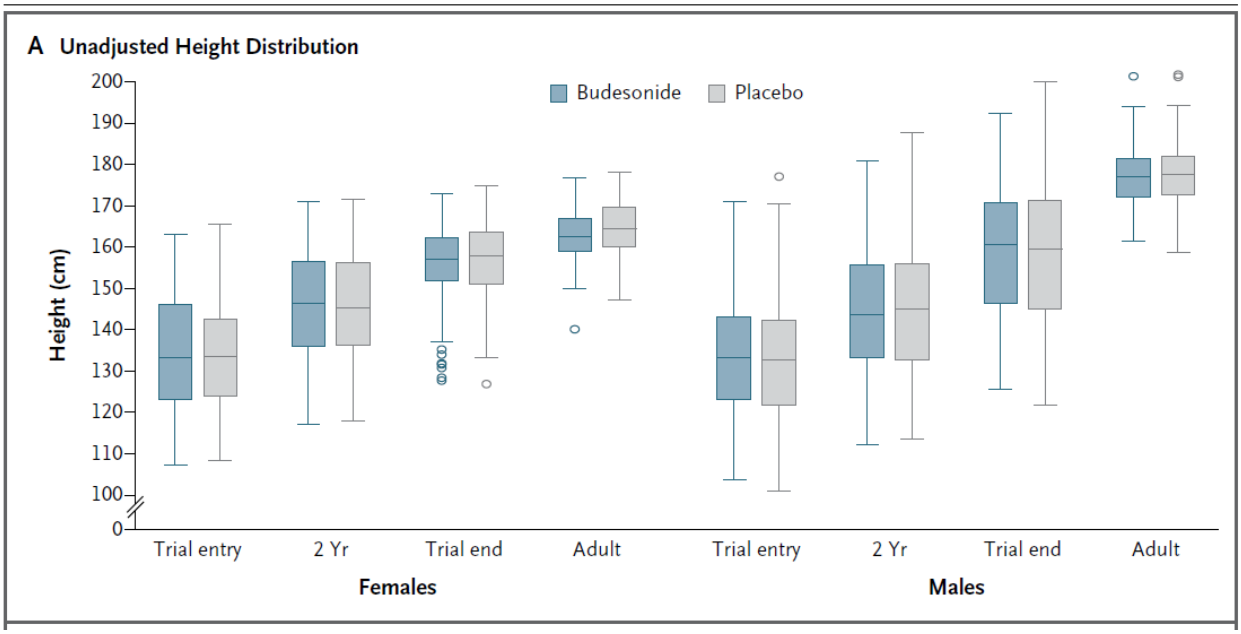
Course Project Quiz Solutions

The following information is relevant to questions 1-6.

A 2012 article in the New England Journal[1] reports the results of a randomized trial to assess the association between regular usage of asthma medication prior to turning 18 years old, and height at age 18 years. As per the authors,

> "From December 1993 through September 1995,we randomly assigned 1041 children between the ages of 5 and 13 years with mild-to-moderate asthma to one of three study groups in the double-blind, placebo-controlled CAMP trial."

The random assignment groups were Budesonide, Nedocromil and placebo. The following graphic shows the distribution of female and male participant heights at different times in the study follow-up, presented for the Budesonide and placebo samples.



1. Based on the graphic, what is the approximate range of individual height values at trial entry for females in the Budesonide group?

   a. 124 cm to 146 cm
   b. 108 cm to 163 cm
   c. 108 cm to 133 cm
   d. 133 cm to 163 cm
   e. This cannot be answered without access to a standard normal table.

1 Kelly H, et al. Effect of Inhaled Glucocorticoids in Childhood on Adult Height. *New England Journal of Medicine* (2012). 367 (10)

The smallest height value in the Budesonide sample is approximately 108 cm, and the largest value is approximately 163 cm.

2. Which of the following statements best summarizes the resulting height distributions for **females** in the Budesonide and placebo groups **as adults** (using only the information in the graphic)?

   a. Most women (≈ 75%) in the Budesonide group were shorter than the 25th percentile of height for women in the placebo group.
   b. The two height distributions are statistically significantly different.
   c. While there is substantial crossover in the height distributions, the 25th, 50th, and 75th percentiles were lower in the Budesonide group when compared to same percentiles in the placebo group.
   d. Both height distributions are extremely skewed.

   C is the best answers among the choice. A is false, B cannot be determined by a visual comparison. While there is a little discordance in the distances of the 25th and 75th percentiles from the median height for the Budesonide sample, the height distribution is otherwise generally symmetric, and the placebo sample values even more so.

3. Which of the following statements best summarizes the resulting height distributions for females in the Budesonide and placebo groups as adults (using only the information in the graphic)?

   a. Most women (≈ 75%) in the Budesonide group were shorter than the 25 percentile of height for women in the placebo group.
   b. The two height distributions are substantially different.
   c. While there is substantial crossover in the height distributions, the 25, 50, and 75th percentiles were lower in the Budesonide group when compared to same percentiles in the placebo group.
   d. Both height distributions are extremely left skewed.

4. The mean height of the 281 adult females in the Budenoside group is 163 cm, and the sample standard deviation is 10 cm. Which of the following is true about the sample standard deviation ?

   a. 95% of the sample values for any sample of data are always within +/- 2 sample standard deviations from the sample mean.
   b. This is the variability in the 281 individual heights for the 281 adult females in this Budenoside sample.
   c. If the researchers had randomized 500 women to receive Budenoside, instead of 281 women, the resulting sample standard deviation of these 500 values would be smaller than 10 cm.
   d. If the researchers had randomized 500 women to receive Budenoside, instead of 281 women, the resulting sample standard deviation of these 500 values would be larger

than 10 cm.

5. What is the likely reason that the height distributions for the Budesonide and Placebo groups are similar at "Trial entry" (time of randomization) for both males and females?

   a. Budesonide is not associated with growth in subjects with asthma.
   b. Subjects were randomized to the Budesonide and placebo groups.
   c. Subjects chose whether to enroll in the Budesonide or placebo groups.
   d. This is just a coincidence.

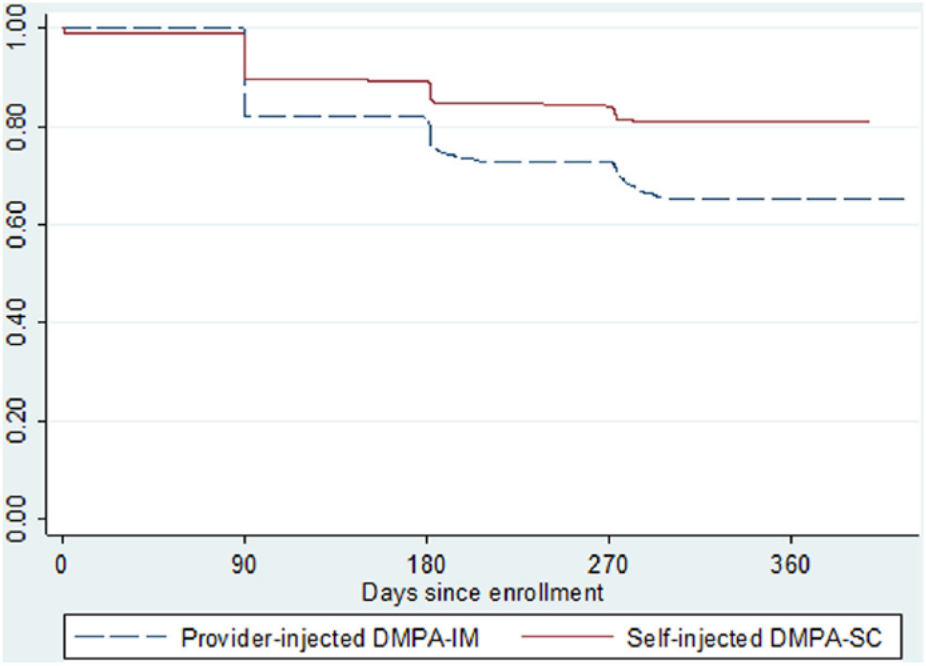
6. Why do both curves start at 1 (100%) at 0 days of follow-up?

   a. Because all study participants in both exposure groups (self-injection and provider injection) are using contraception at the start of the study (start of the follow-up period).
   b. Because none of the study participants in both exposure groups (self-injection and provider injection) are using contraception at the start of the study (start of the follow-up period).
   c. Because there is no censoring in the data.
   d. Because all of the study participants stopped using contraception by the end of the follow-up period.

The following abstract portion is taken from a 2018 article published in *Contraception.*

> Objective: The purpose of this study was to compare 12-month continuation rates for subcutaneous depot medroxyprogesterone acetate (DMPA-SC) administered via self-injection and DMPA-IM administered by a health worker in Uganda.
> Study design: Women seeking injectable contraception at participating health facilities were offered the choice of self-injecting DMPA-SC or receiving an injection of DMPA-IM from a health worker. Those opting for self-injection were trained one-on-one. They self-injected under supervision and took home three units, a client instruction guide and a re-injection calendar. Those opting for DMPA-IM received an injection and an appointment card for the next facility visit in 3 months. We interviewed participants at baseline (first injection) and after 3 (second injection), 6 (third injection) and 9 (fourth injection) months, or upon discontinuation. We used Kaplan–Meier methods to estimate continuation probabilities.

The following graphic shows the estimated Kaplan-Meier curves tracking contraception discontinuation for both groups *(these curves track the proportion of subjects who have not discontinued contraception usage over the follow-up period).*



7. Approximately what percentage of subjects in the "Provider-injected DMPA-IM" group were still using contraceptives at 360 days of follow-up?

    a. 100%
    b. 0%
    c. 64%
    d. 36%

8. Approximately what percentage of subjects in the "Self-injected DMPA-SC" group had stopped using contraceptives at 360 days of follow-up?

    a. 100%
    b. 30%
    c. 20%
    d. 10%

    The Kaplan-Meier curve estimate of the proportion of subjects in the "Self-injected DMPA-SC" who had not stopped using contraceptives at 360 days of follow-up is approximately 80%. As such, approximately 20% of the subjects in this group had stopped using by 360 days.

9. Based on the information in the Kaplan-Meier curves, what can be said about the estimated incidence rate ratio ($\widehat{IRR}$) of discontinuing contraception for the "Self-Injected DMPA-SC" group compared to the "Provider Injected DMPA-IM" group?

    a. $\widehat{IRR} = 1$
    b. $\widehat{IRR} > 1$
    c. $\widehat{IRR} < 1$

    The K-M curves for the two samples shows that the “survival” for the "Self-Injected DMPA-SC" is higher than that of the "Provider-injected DMPA-IM" across the entire follow-up period. As such, a lesser percentage of subjects in the "Self-Injected DMPA-SC" discontinued contraception use of the follow-up period, i.e. the incidence of discontinuing contraception usage was lower in this group.

10. Participants in the study were able to choose between having a provider inject the contraceptive or self-injecting the contraceptive. What implication does this have for basing a conclusion about the differences, if any, with contraceptive continuation between these two groups?

    a. None. When given a choice of two options, people tend to pick one of the two at random.
    b. It is possible that some of the observed difference in contraceptive continuation between the two groups is because of other factors relative to both contraceptive continuation and choosing the type of delivery method (self-injection vs. provider injection).
    c. Researchers are less likely to measure the outcome of interest correctly in non-randomized studies.