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> Clinical Trials

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## 4. Parametric Hypothesis Testing - Clinical Trials

### Clinical Trials

## Clinical trials



Let us go through an example to remind the main notions of hypothesis testing.

- ▶ Pharmaceutical companies use hypothesis testing to test if a new drug is efficient.
- ▶ To do so, they administer a drug to a group of patients (test group) and a placebo to another group (control group).
- ▶ We consider testing a drug that is supposed to lower LDL (low-density lipoprotein), a.k.a "bad cholesterol" among patients with a high level of LDL (above 200 mg/dL)

(Caption will be displayed when you start playing the video.)



### Video

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**Note :** The following are problems we will use to prepare for the upcoming video.

## Clinical Trials: Hypothesis Test

0/1 point (graded)

We consider the clinical trials set-up of the above video. Let  $X_1, \dots, X_n$  be i.i.d. test group samples distributed according to  $\mathcal{N}(\Delta_d, \sigma_d^2)$  and let  $Y_1, \dots, Y_m$  be i.i.d. control group samples distributed according to  $\mathcal{N}(\Delta_c, \sigma_c^2)$ . Assume that  $X_1, \dots, X_n, Y_1, \dots, Y_m$  are independent.

Select from the following all valid hypothesis test formulations to know if the drug has an effect on cholesterol levels (that is, we wish to know whether the drug has a statistically significant effect on the decrease in cholesterol levels when compared to the decrease in the cholesterol levels due to the placebo).

☒  $H_0 : \Delta_d = \Delta_c, H_1 : \Delta_d > \Delta_c$  ✓

☐  $H_0 : \Delta_d \leq \Delta_c, H_1 : \Delta_d > \Delta_c$  ✓

✗

**Solution:**

Both are valid formulations for this specific question that will lead to the same test.

Submit

You have used 1 of 1 attempt

**i** Answers are displayed within the problem

## Clinical Trials: Consequence of Gaussian Samples

1/1 point (graded)

We consider the same set-up as the above problem. Let  $X_1, \dots, X_n$  be i.i.d. test group samples distributed according to  $\mathcal{N}(\Delta_d, \sigma_d^2)$  and let  $Y_1, \dots, Y_m$  be i.i.d. control group samples distributed according to  $\mathcal{N}(\Delta_c, \sigma_c^2)$ . Assume that  $X_1, \dots, X_n, Y_1, \dots, Y_m$  are independent.

Select from the following all statements that are correct or true.

☒  $\bar{X}_n = \frac{X_1 + X_2 + \dots + X_n}{n}$  is a gaussian random variable

☒  $\bar{Y}_m = \frac{Y_1 + Y_2 + \dots + Y_m}{m}$  is a gaussian random variable

☒  $\bar{X}_n - \bar{Y}_m = \frac{X_1 + X_2 + \dots + X_n}{n} - \frac{Y_1 + Y_2 + \dots + Y_m}{m}$  is a gaussian random variable

☒  $X_i + Y_j$  is a gaussian random variable for any  $i = 1, \dots, n$  and  $j = 1, \dots, m$ .

☐ The variance of  $\bar{X}_n - \bar{Y}_m$  is  $\frac{\sigma_d^2 + \sigma_c^2}{n+m}$ .



#### Solution:

The first four choices are true because of a basic principle of gaussian random variables. The linear combination of any two gaussian random variables (independent or not) is a gaussian random variable.

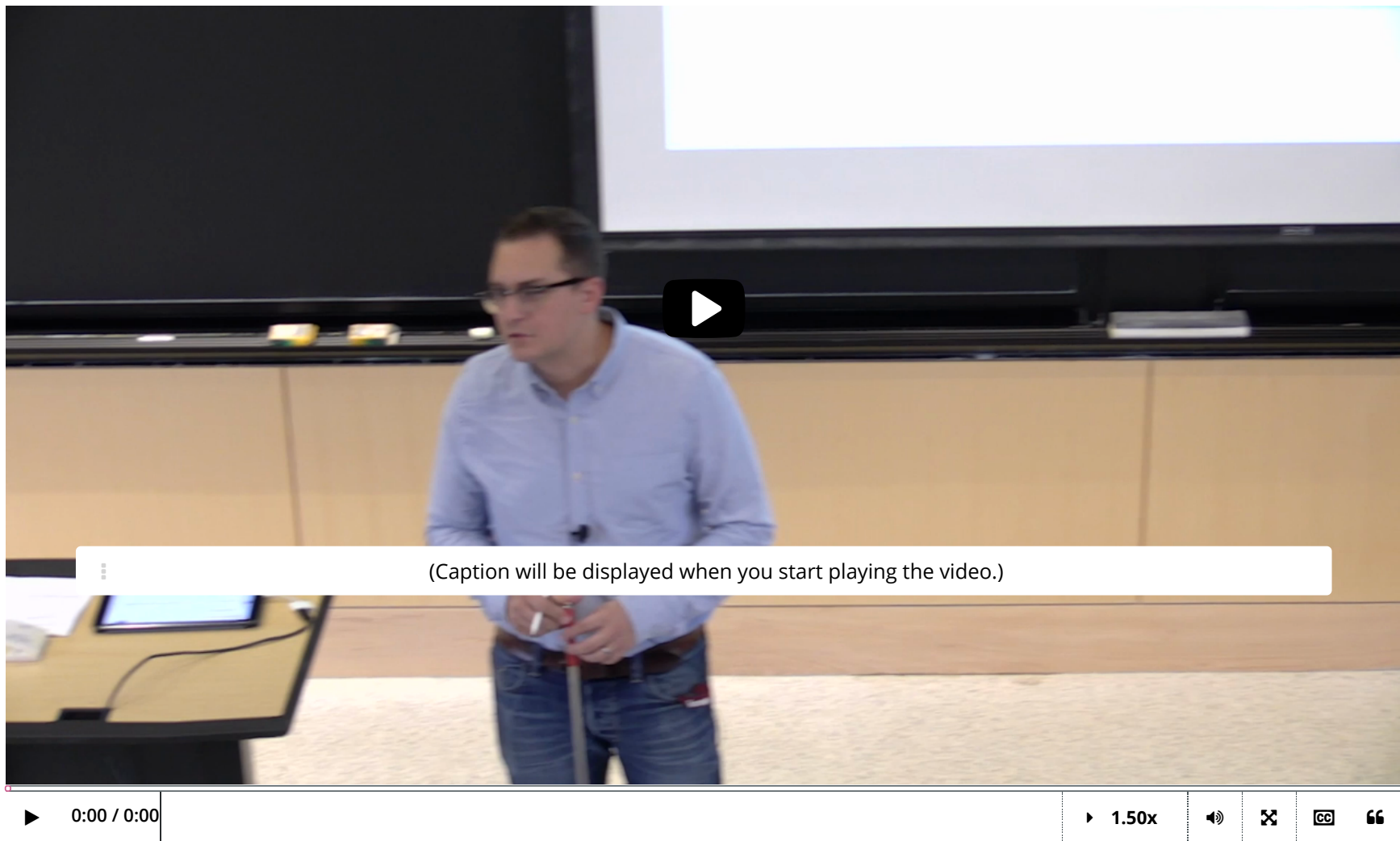
The last choice is not correct because the variance of  $\bar{X}_n - \bar{Y}_m$  is  $\frac{\sigma_d^2}{n} + \frac{\sigma_c^2}{m}$ .

Submit

You have used 2 of 2 attempts

**i** Answers are displayed within the problem

## Clinical Trials - Setting up the Hypothesis Test



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