Principles of Statistical Data Analysis
Written Exam, January 5, 2015

The Problem Some guidelines

- This is a written open book exam, which is accompanied by an oral exam. At the oral exam you will present part of the project with your group (maximum 15 minutes for the project) and answer questions along the way.
- The written exam will last until 11:30. The exam papers counts... pages in total.
- You can use books and other written material you wish to bring, but cannot involve any online consulting or other contacts.
- Please write your name on each page of the exam paper. Do write clearly in the lines foreseen. If needed, add sentences on the opposite white page.
- This assignment paper and other used papers should be handed in at the end of the exam.
- Turn off your mobile phone.
- The groups who have their oral exam in the morning should go straight from A1 to Room V1 at their scheduled time for the oral exam. You should do this in silence. You are not allowed to interact with each other (especially about the exam questions) on your way to the oral and back to the written exam. Anyone noticing any infractions should let this be known. Students with an oral exam in the morning will be given the corresponding extra time to finish the oral exam.
- We wish you the best of luck!

Questions

1. A recent study was set up to investigate the potential value of general practicioner-(GP-)led and nurse-led telephone triage in patients requesting same-day consultations at a general practice. Implications for patients, practices, or the wider health-care system were examined.

   20 990 patients were randomly assigned (1:1:1) to GP triage, nurse triage or usual care (n=6695 vs 7012 vs 7283, respectively) of whom 16 211 (77%) patients provided primary outcome data (n=5171 vs 5468 vs 5572). We consider the summary statistics provided per intervention arm in table 3 below.

	GP triage	Nurse triage	Usual care	GP triage *vs* usual care (OR [95% CI])	Nurse triage *vs* usual care (OR [95% CI])	Nurse triage *vs* GP triage (OR [95% CI])
Total (case notes reviewed)	6695 (5171)	7012 (5468)	7283 (5572)	..	..	..
Total deaths (n/1000 patients)	5 (0·7)	2 (0·3)	1 (0·1)	..	..	..
Patients with at least one emergency hospital admission (n [%])	59 (1%)	69 (1%)	52 (<1%)	1·17 (0·75–1·85)	1·31 (0·83–2·07)	1·12 (0·73–1·72)
Number of bed days for patients admitted to hospital (mean [SD])	3·4 (3·7)	4·5 (5·7)	3·8 (6·4)	..	..	..
Patients with at least one accident and emergency attendance within 28 days of index day (n [%])	171 (3%)	156 (3%)	166 (3%)	1·18 (0·87–1·61)	1·09 (0·80–1·49)	0·92 (0·67–1·26)

Mortality, and number and duration of emergency hospital admissions within 7 days, and patients with at least one accident and emergency department attendance within 28 days of index consultation request. GP=general practitioner. OR=odds ratio.

Table 3: **Patient safety**

(a) In line 4 of the table the 'Number of bed days for patients admitted to hospital' are described (mean [SD]). What does it tell you about the shape of its distribution in each group?

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(b) Based on the data underlying this line in the table, how would you construct a 95% prediction interval for patients admitted to hospital after GP triage? You need not actually do the calculation but briefly explain how and why.

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(c) Depending on the use(r) of the result above one or another measure of central tendency is more relevant. Give an example of two such different user groups and explain how the different measures would matter to them.

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(d) Suppose one wishes to test whether the population average 'Number of bed days' is constant over the 3 intervention arms, would an ANOVA test be useful here? Explain why or why not. If not what would be a better alternative?

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(e) Next consider the numbers dead and alive per treatment arm. Explain how a chi-squared test could be considered to test for equal risk of death over the three groups. Give the value of the test statistic.

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(f) What assumptions are made to allow for the (approximate) chi-squared distribution of this test statistic under H_0. Are they fulfilled here? If not, how could one build on this test statistic to arrive at a correct p-value for the comparison across groups ? Explain.

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(g) Should we examine any observed differences between the 3 groups in age distribution, baseline disease severity etc. before interpreting the effect on expected bed days as a causal effect of the type of triage? Explain why or why not.

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(h) Now consider the fact that 77% of patients provided outcome data. How does this influence the above results. Would you change any answers/interpretation in light of this fact? Explain.

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(i) Suppose GP-practices rather than individual patients had been randomised over the three ways of referring patients to the next consultation. What impact is that expected to have on any confidence interval or p-value produced from this data. Explain.

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2. Jao et al. (AIDS 2015, 29:111- 116) compare the growth patterns in the first year of life between children born to perinatally HIV-infected (PHIV) vs. nonperinatally HIV-infected (NPHIV) women in the United States. The figure below shows results of the weight for age z-scores measured on a retrospective cohort study of HIV-infected pregnant women who received care and delivered a live-born at two urban tertiary centers from January 2004 to March 2012.

   (a) Define formally and in words what a simple weight for age z-score is or could be.

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   (b) For any true weight distribution at a given age in the reference population, what properties is a random sample of the corresponding weight for age z-scores supposed to have ?

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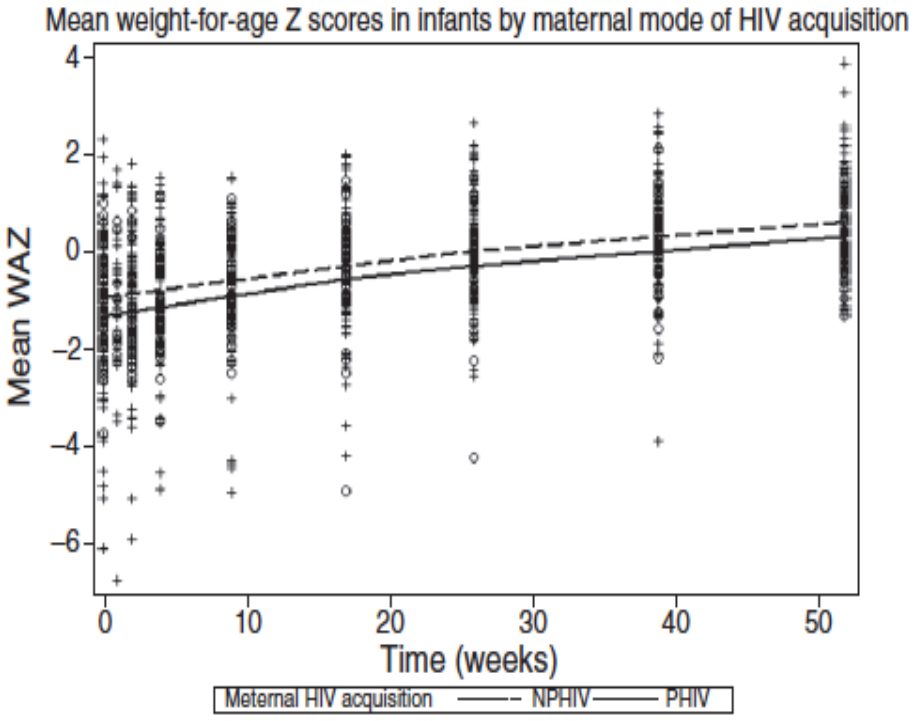

Figure 1: Scatter plot of weight for age z-scores as they evolve over time

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(c) What does the above imply for the boxplot presenting the data of this sample?

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(d) Now turn back to the study depicted in Figure C and interpret its results at baseline (week 0). What do they suggest about the observed distribution for the PHV group? Explain.

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(e) Compare observed results at baseline (week 0) with the results at 52 weeks, in a descriptive manner. What do the data for the NPHV group suggest?

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(f) Suppose we wanted to make a more formal comparison between mean results at baseline (week 0) and at 52 weeks. Do we have enough information to approximate the confidence interval for the difference in population means in the NPHV group? If not, what extra information would you need?

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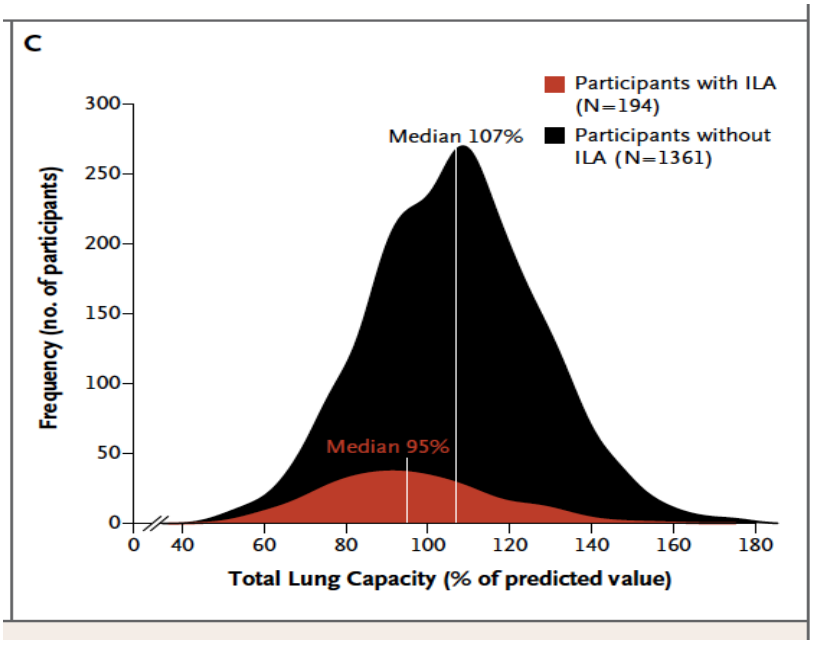
3. In a well defined cohort of smokers, one looked for lung abnormalities on specific scans of the lungs and found 194 patients with versus 1361 without interstitial lung abnormalities (ILA). The observed distribution of percentage of predicted lung capacity is given for both groups in Figure C below. Please refer to this figure to answer the following questions.



(a) Which of the two groups (with or without ILA) has the highest estimated density at 60% of the predicted total lung capacity? Explain.

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(b) Which of the two groups (with or without ILA) has the highest estimated variance based on the figure? Explain.

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(c) Does the distribution for the participants with ILA appear normally distributed? Explain how you expect the value of one or more summary statistics of the observed data will support your point.

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(d) Among the 194 participants with ILA, 97 are current smokers compared to 609 out of the 1361 participants without ILA. Is this difference significant at the 5% significance level?

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(e) In the paper, data on age are given as median and quartiles. For the group without ILA this is 60 (52-67), for the group with ILA: 64 (56- 72). Assuming age is normally distributed within each group, derive whether the difference in mean ages is significant at the 5% significance level.

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(f) What power did the test above have to detect a mean difference of 2 years in age between the two groups?

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