## IN-STK-5000, Medical Project

#### Christos Dimitrakakis chridim@ifi.no

October 22, 2019

Before you start, make sure you have

- Joined one of the project groups in Piazza.
- Forked the code in https://github.com/olethrosdc/ml-society-science
- All questions should go through the QA platform in Piazza.

### 1 Historical data (Nov 6)

First, we shall take a look at historical data present in Matlab/Octave format in data/medical/historical.dat For each patient, we observe the attributes  $\boldsymbol{x}$ , with

- $x_1 \in \{0, 1\}$ , sex.
- $x_2 \in \{0, 1\}$ , smoker.
- $x_{3:128} \in \{0,1\}^{125}$ , gene expression data. These variables can have missing values, but here they are all included in the historical data.
- $x_{129:130} \in \{0,1\}^2$ , symptoms. These can be taken to be akin to labels in supervised learning. There may be missing data here too, but for now we can assume they are included.

We also observe a therapeutic intervention  $a \in \mathcal{A}$ , which is followed by an outcome  $y_t \in \{0, 1\}$ . Consequently, historical data can be described by  $(x_t, a_t, y_t)$ 

Discovering structure in the data. It is uncertain if the symptoms present are all due to the same disease, or if they are different conditions with similar symptoms. (a) looking at only the attributes, estimate whether a single-cluster model is more likely than a multiple-cluster model. You can use anything, starting from a simple clustering algorithm like k-means to a hierarchical Bayesian model. (b) Try and determine whether some particular factors are important for disease epidemiology and may require further investigations.

You need to be able to validate your findings either through a holdout-set methodology, appropriately used statistical tests, or Bayesian model comparison.

Measuring the effect of actions. We also observe the effects of two different therapeutic interventions, one of which is placebo, and the other is an experimental drug. Try and measure the effectiveness of the placebo versus the active treatment. Are there perhaps cases where the active treatment is never effective, or should it always be recommended?

### 2 Improved policies (Nov 20)

The data we have observed comes from some policy  $\pi_0$ , taking actions in  $\{0,1\}$ . Now we have to be more specific about what the meaning of each treatment and outcome variable is. In this case,  $a_t = 0$  is a placebo, and  $a_t = 1$  is an experimental drug, with  $y_t = 0$  meaning no effect, and  $y_t = 1$  meaning a measurable effect. For the purposes of this exercise, you can assume the utility function is:

$$U = \sum_{t} r_t, \tag{2.1}$$

$$r_t \triangleq -0.1a_t + y_t. \tag{2.2}$$

The -0.1 factor implies that the active treatment must be at least 10% more effective than the placebo.

Use the skeleton random\_recommender.py to implement your code.

**Assumption 2.1.** The observed policy  $\pi_0$  and all other policies  $\pi$  can be represented as conditional distributions  $\pi(a \mid x)$ .

EXERCISE 1 (Measuring utility). In this exercise, you should implement the estimate\_utility() method to estimate the utility of policies on the historical data (data, actions, outcome) passed to the function

- 1. Measure the utility of the historical policy  $\pi_0$  on the historical data.
- 2. Provide error bounds on the expected utility and explain how those were obtained.

EXERCISE 2 (Improved policies). 1. Find an improved policy  $\hat{\pi}$ , taking actions in  $\{0,1\}$ . Hint: This can be done by simply selecting, for each  $x_t$ , the action  $a_t$  maximising expected reward according to your model, as the utility is a sum of rewards. Of course, you should first build a model.

2. Calculate the expected utility of the improved policy  $\hat{\pi}$  from the historical data. Implement this in estimate\_utility()

# 3 Adaptive experiment design (Dec 6)

For this part of the exercise, make sure that you have implemented everything within the API defined in the random\_recommender.py skeleton. All the methods you have implemented for feature selection, dimensionality reduction, policy evaluation, etc. must be self-contained within the recommender. The test bench will call your recommender's fit\_data() and fit\_treatment\_outcome() with possibly new datasets. This will be done mainly in order to test how your algorithm behaves with varying amounts of data.

EXERCISE 3 (Online policy testing). Here, make sure that you recommenders are based on random\_recommender.py.

- 1. Create a HistoricalRecommender recommender class that estimates the historical policy  $\pi_0$  when fit\_treatment\_outcome() is called. Measure the expected utility of the original policy  $\pi_0$  using the Test Bench.
- 2. Create a ImprovedRecommender class that implements your creates an improved policy  $\hat{\pi}$  when fit\_treatment\_outcome() is called, and uses this policy when recommend() is invoked. Measure the expected utility of your improved policy  $\hat{\pi}$  using the Test Bench.
- 3. How do those differ from the results you obtained on historical data?

EXERCISE 4 (Adaptive experiments). In this setting you should contrast your ImprovedRecommender with the HistoricalRecommender and an AdaptiveRecommender, that you should implement, which should change its recommendations as it obtains new data through observe(). We will consider two cases:

- 1. The same set of treatments are available.
- 2. There is an additional set of experimental treatments. Action 2 is a potentially improved treatment for the general population, while the remaining target specific genes. Hence, you might expect these to be effective against only a small portion of the patients.

In either case, you can have two alternative goals: (a) discover the most effective treatment policy at the end of the trial (b) maximise the expected number of people to be treated.

For simplicity, use goal (b) to plan the experiments. However, at the end, you should report which treatment policy appears to be the best.

Since the data will be dynamically generated, implement the final analysis in your function. This can show

- 1. Recommending a specific fixed treatment policy
- 2. Suggesting looking at specific genes more closely
- 3. Showing whether or not the new treatment might be better than the old, and by how much.
- 4. Outputting an estimate of the advantage of gene-targeting treatments versus the best fixed treatment.