Dose-Response Assessment in Randomized Controlled Trials

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Annual Meeting of the
Marie Curie International Exchange Program
Hôpital Ste-Justine, Montréal
June 3rd, 2012

Funding: Canadian Institutes of Health Research (CIHR),
Marie Curie International Exchange Program Travel Grant

Dose-Response Assessment

Randomized Controlled Trials (RCT):

Typical evaluation: ***Intent-to-Treat analysis***

Answers the question:
"What are the effects of ***offering*** this program to this population?"

Dose-Response Assessment

Randomized Controlled Trials (RCT):

Important process questions:
"What are the effects of ***participation*** in this program?"
"What are the effects of ***increased participation*** in this program?"

… Are more difficult to assess because of the ***self-selection problem:*** Families who participate more are different from those who participate less on certain characteristics.

How to approach the self-selection problem?

2 Methods to be compared with Preparing for Life data:

-An extension of the ***2-step approach (Heckman)***

1: model the selection process; 2: estimate the effect of program dose net of selection

Orla Doyle and Seong Moon

-An extension of ***propensity-score matching (Hill; Imai & van Dyk; Frangakis & Rubin)***

1: model the selection process; 2: match comparable groups; 3: estimate the effect of program dose for each group

Amélie Petitclerc

What is propensity-score matching (PSM)?

PSM is used when we want to test the effect of a **treatment (or exposure)** with non-experimental data.

It is a statistical method to ***construct a comparison group*** similar to the treatment group with respect to individuals' ***probability of being treated***, given a set of ***observable*** characteristics.

Development and Concepts

Developed by Rosenbaum & Rubin (1983)

An extension of “exact matching” used in psychology

Advantage over exact matching:
Using a large number of variables

What is the Propensity Score?

- Represents the probability for each individual to be exposed to a treatment, given a set of variables.

-The predicted value obtained from a logistic regression (or probit) with the chosen set of variables as predictors and the treatment assignment (0/1) as the outcome.

- Can also be a continuous score for a continuous treatment or dose (Imai & van Dyk, 2004)

Assumption of PSM (for valid causal inference)

"ignorability assumption"

That the propensity score (i.e., the chosen set of predictors) covers all the characteristics that differ between treated and untreated individuals *and* that influence the outcome.

So, conditional on the propensity score, the treatment assignment mechanism is ***independent*** of the outcome.

Suggested approach: **Principal scores stratification**

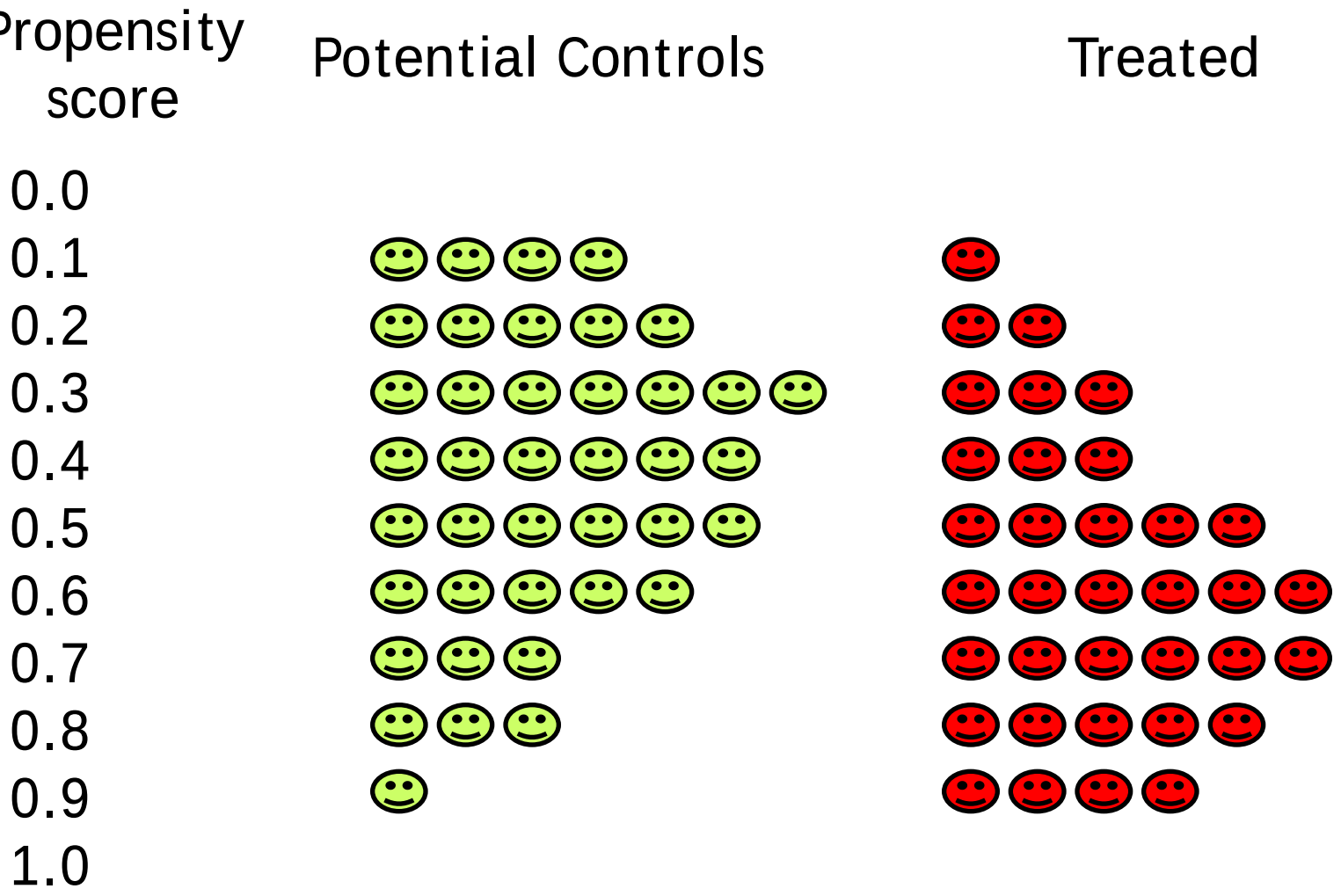
An extension of:

Hill, et al. (2004)
Estimate the effect of a ***high dose*** of treatment (binary) in the context of an RCT

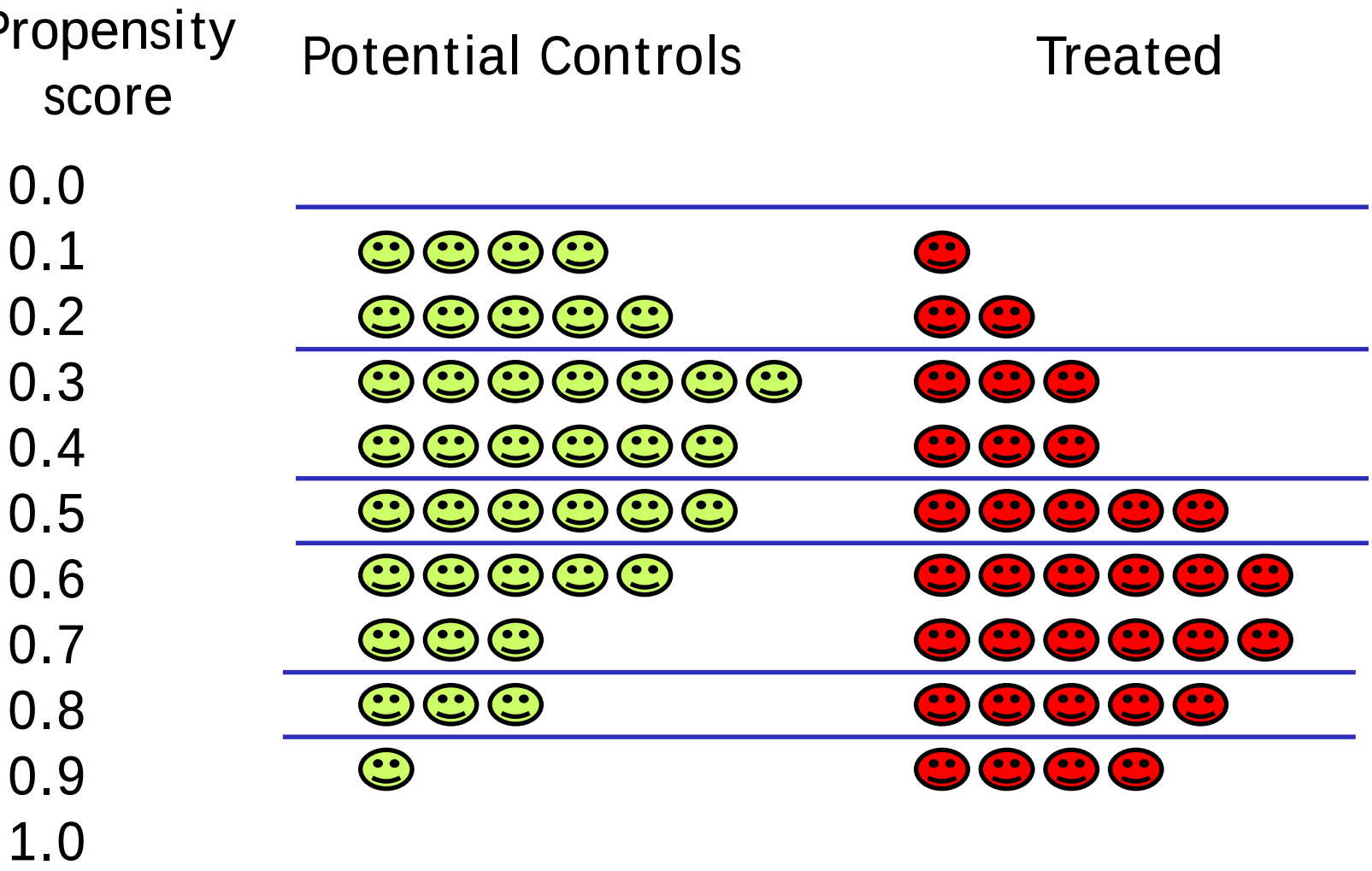
Imai & van Dyk (2004).
Estimate the effect of a continuous dose of treatment in the context of observational data

Goal:
Using a continuous ***predicted dose*** (i.e., number of days in Child Development Centers) to create balanced groups, estimate the effect of the ***actual dose received***.

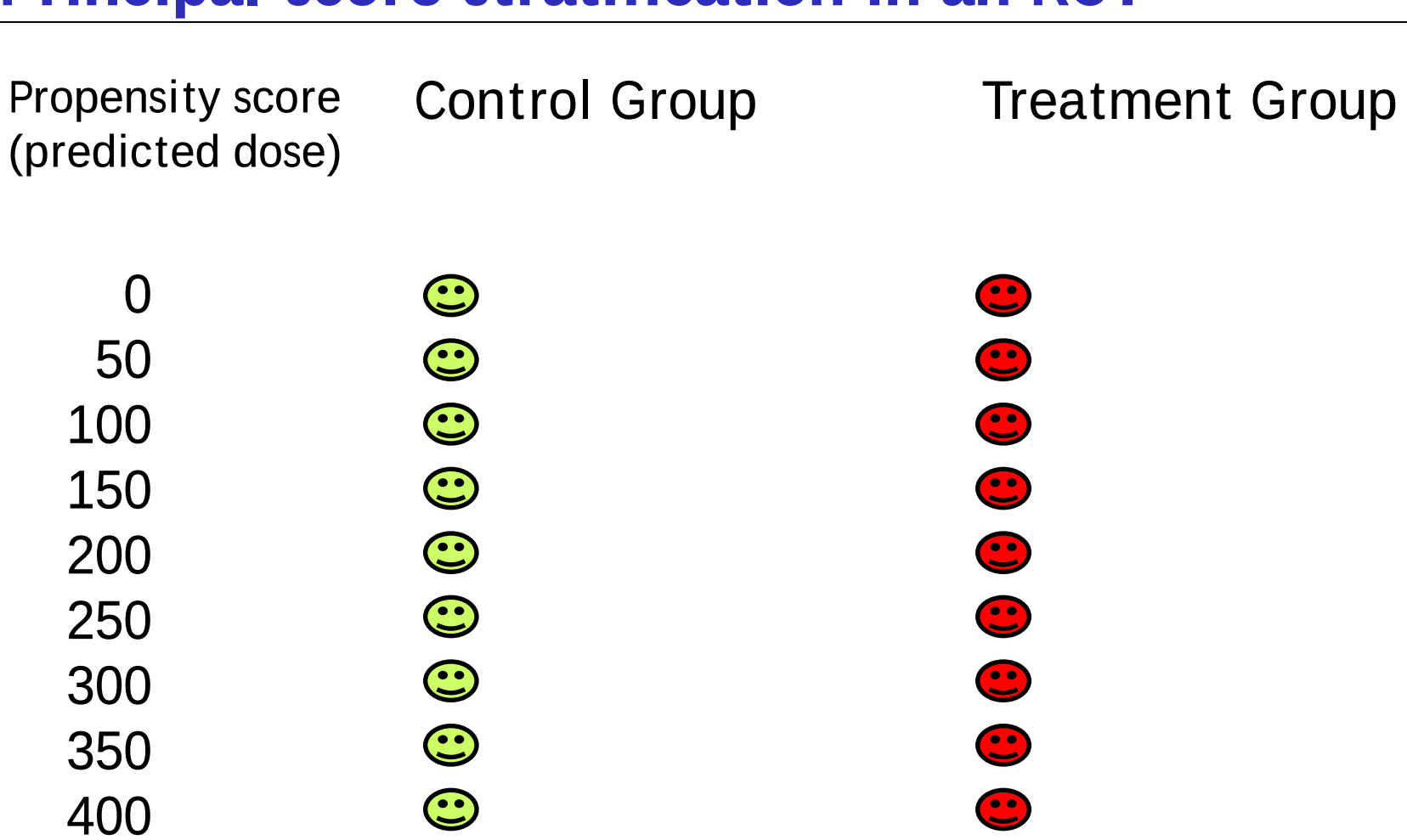
Propensity-score stratification



Propensity-score stratification



Principal-score stratification in an RCT



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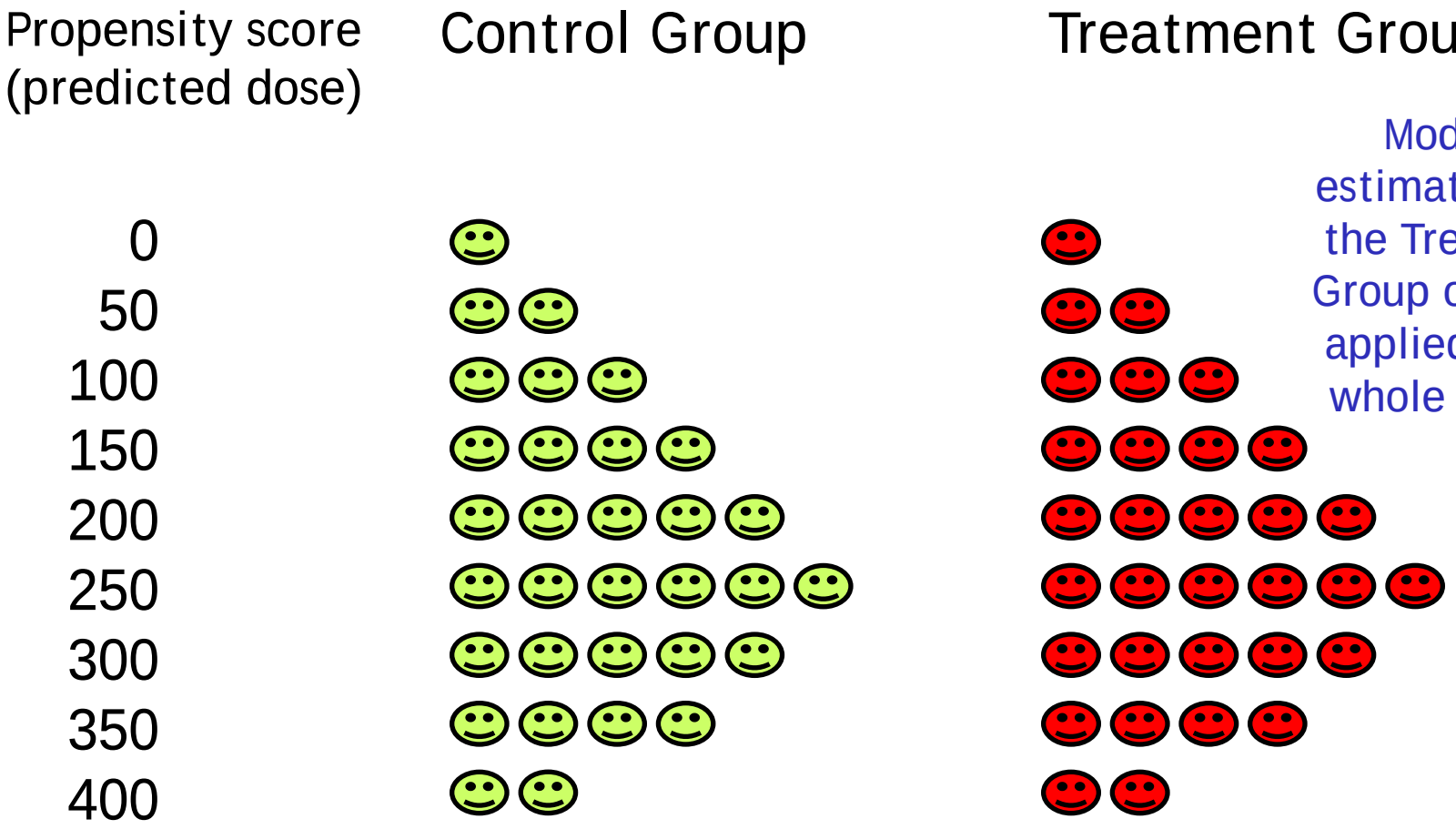

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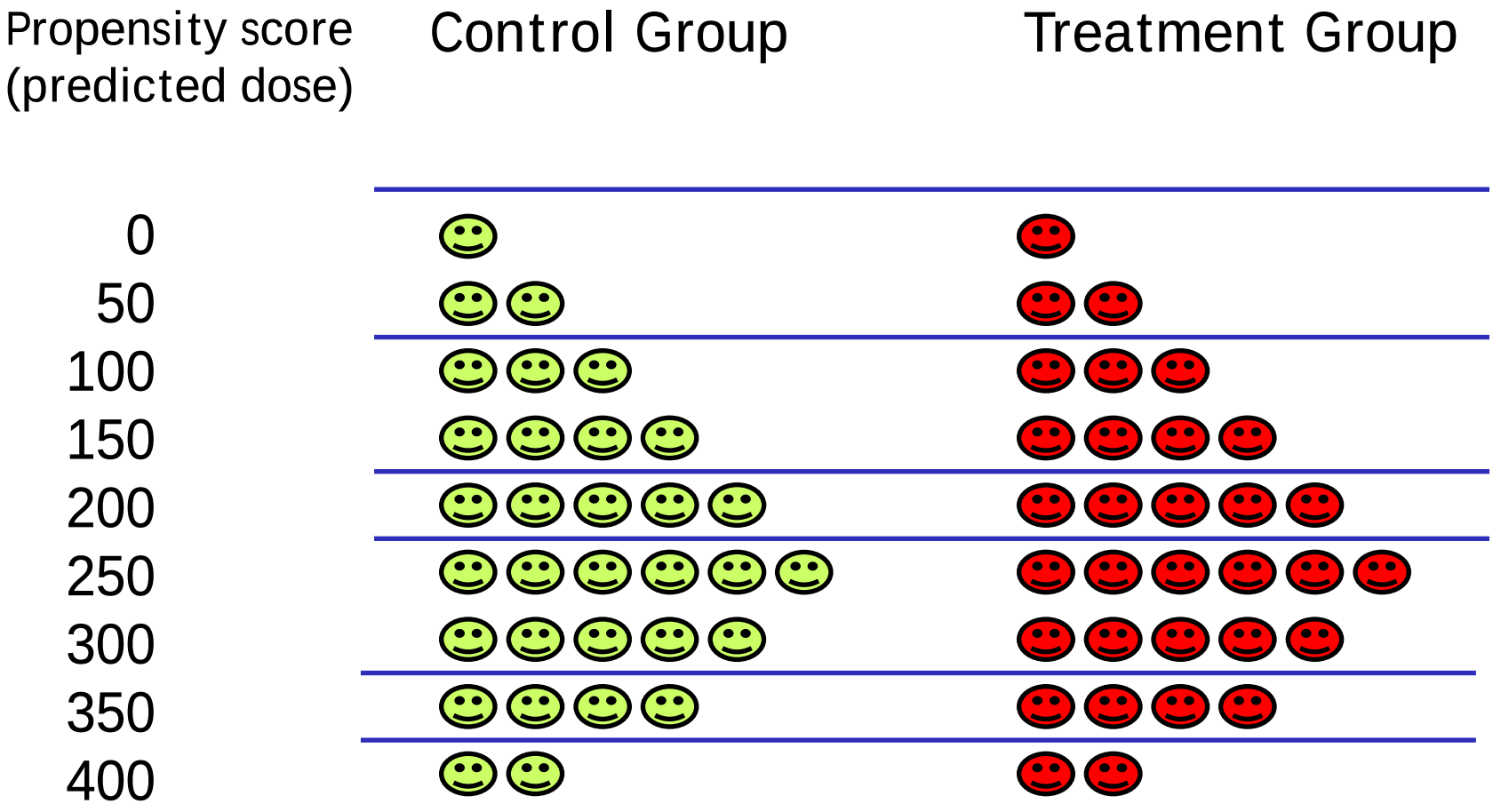


Steps in principal scores stratification analysis

1. Model the probability to participate in more Child Development Center **days** (continuous propensity score).
2. Estimate, for each child in *treatment **and** control groups*, a predicted dose, using the model in #1.
3. Create strata of children who have similar propensity scores, and **evaluate** the balance, within each strata, on all relevant pre-treatment characteristics.
4. Estimate the continuous effect of ***actual dose*** within strata of ***predicted dose***, and pool the results to test the continuous effect of treatment.

Working example

Research Question:

Did the IHDP have a greater effect on children who participated more in the program's development centers?

Data:

Infant Health and Development Program (IHDP)
985 low-birth-weight, preterm infants born in 1985

Home visits: from birth to age 1
Child Development Centers: from age 1 to 3

Dose:

Days in Child Development Centers

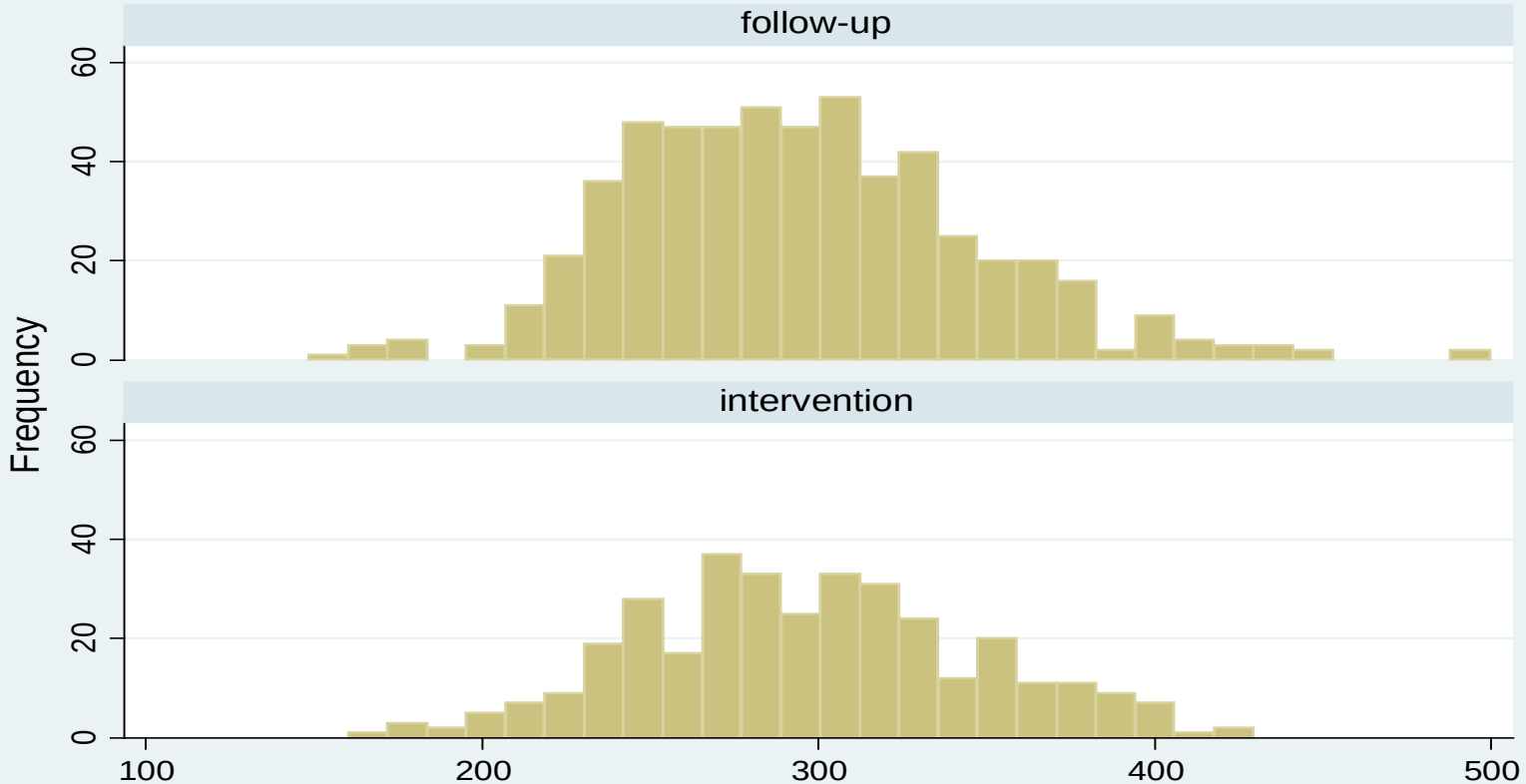
Steps in principal scores stratification analysis

1. Model the probability to participate in more Child Development Center **days** (continuous propensity score).

   Father ethnicity
   Mother working prior to pregnancy
   Mother working during pregnancy
   Neonatal health index
   Head circumference
   Use of cigarette during pregnancy
   Use of drug during pregnancy
   Prenatal care
   Child twin status
   Marital status at 12 months
   Preschool child in family at 12 months
   Family income at 12 months
   Mother employed at 12 months
   Used child care centers before 12 months
   Child's health problems required time off work

Steps in principal scores stratification analysis

2. Estimate, for each child in *treatment **and** control groups*, a predicted dose, using the model in #1.



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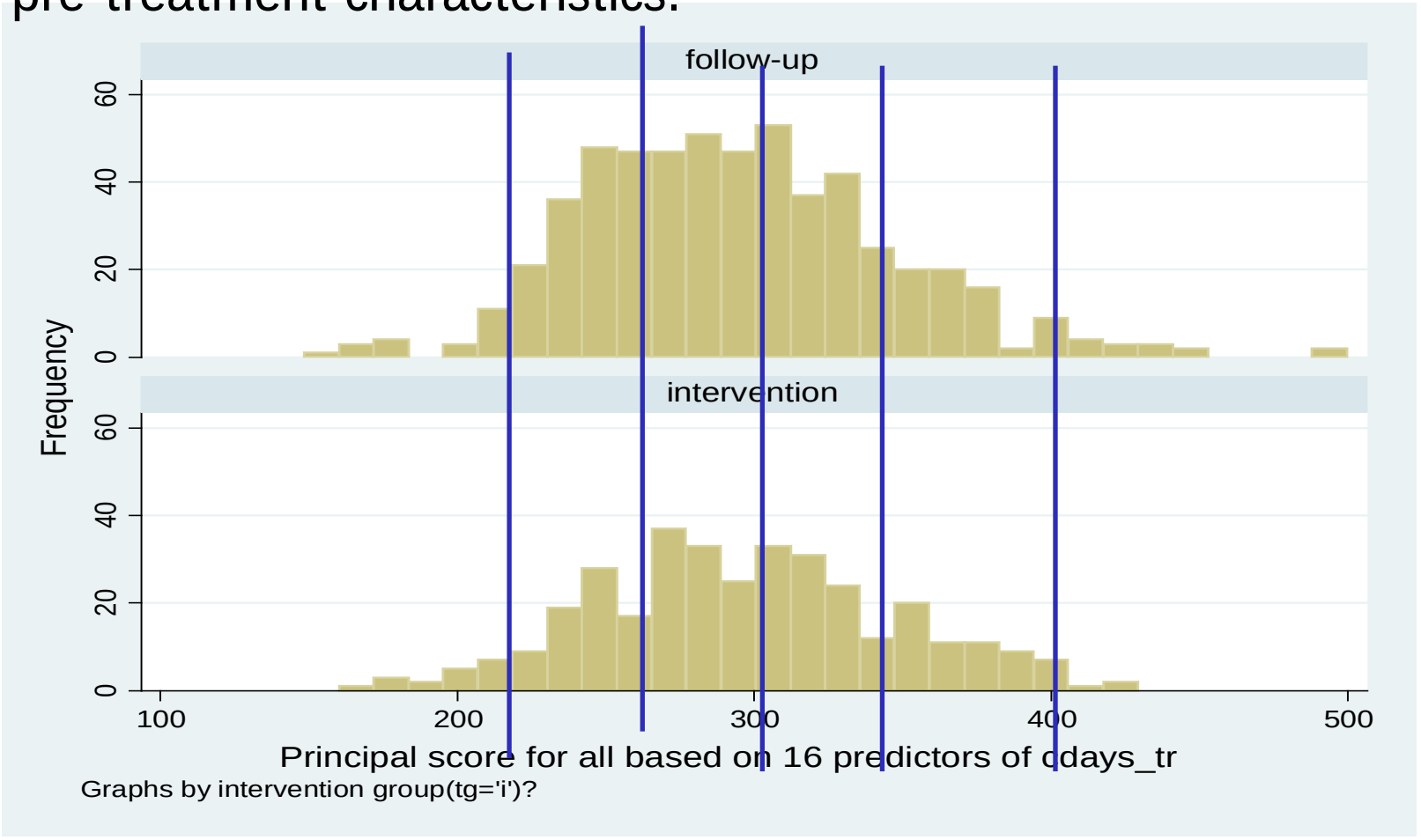

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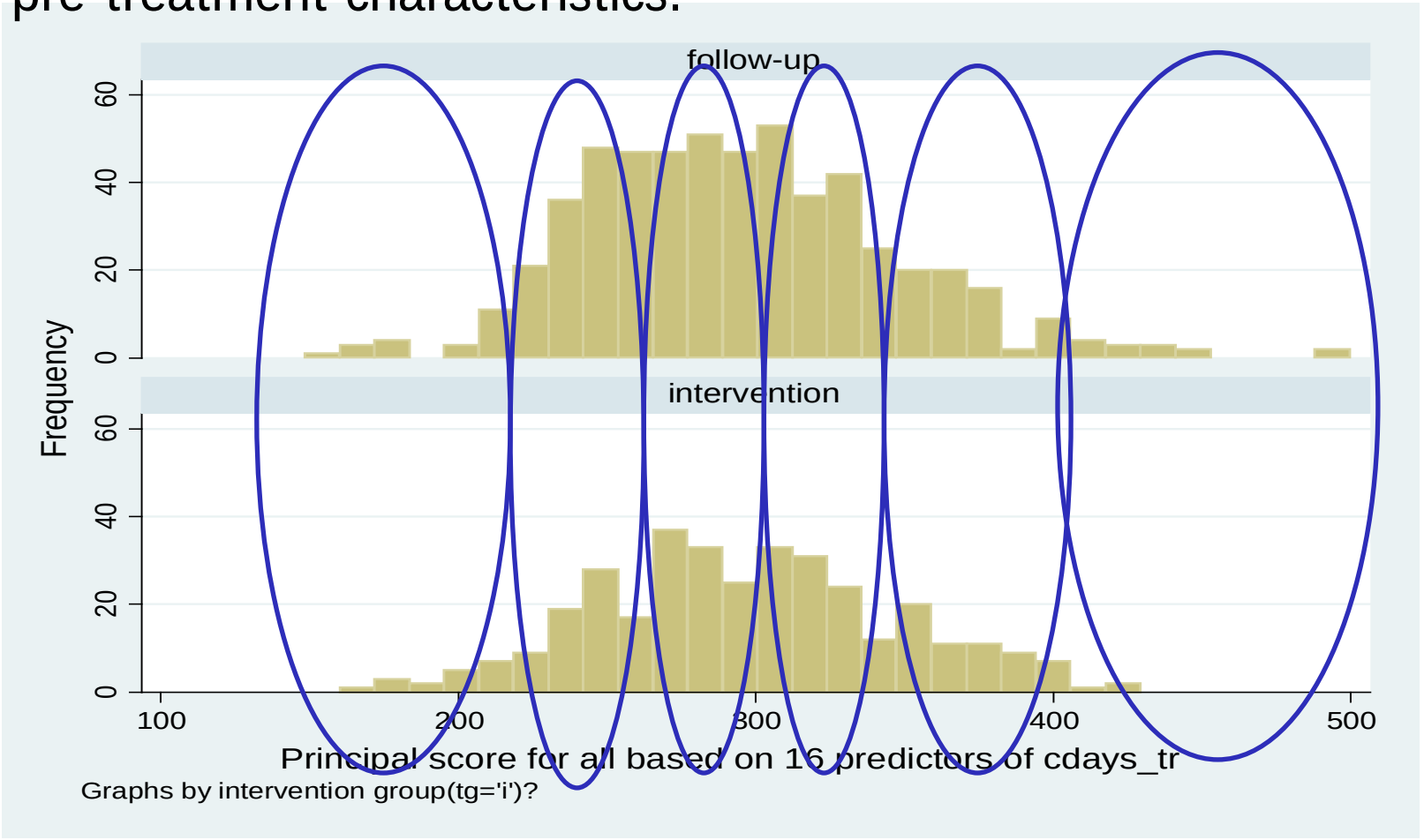

Steps in principal scores stratification analysis

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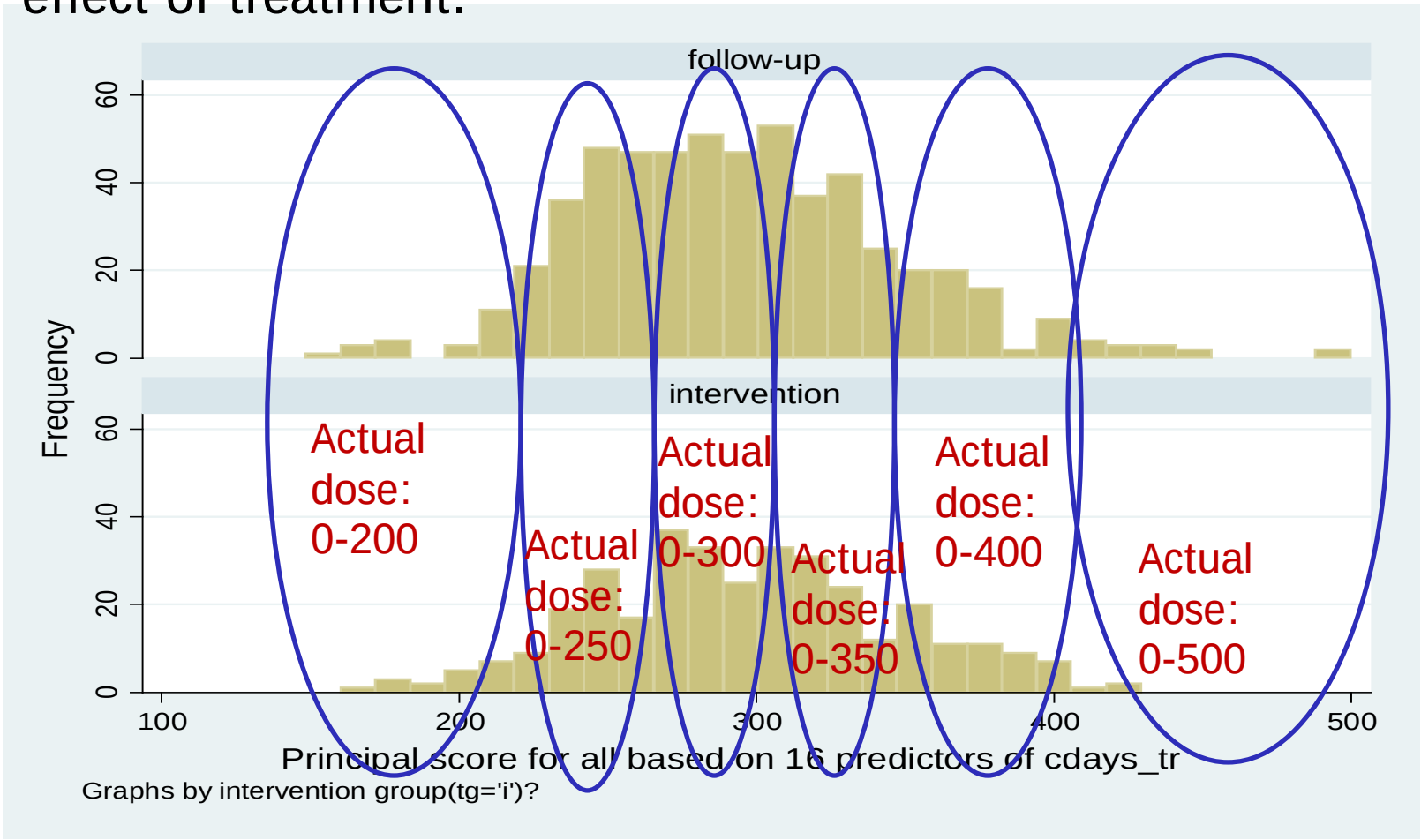

Research questions

Program process questions:

Do effects follow a ***dose response*** ?

Are effects associated with participation in component A or component B?

Method question:

Do we obtain the same estimate of treatment effect with both the principal score stratification approach and the extension of the Heckman 2-step approach?

Thank you

Current Post-doctoral Funding

Canadian Institutes of Health Research (CIHR)

Marie Curie International Exchange Program travel grant – summer 2012

Advantages of PSM over Ordinary Least Squares (OLS) regression

-PSM is not submitted to the linearity assumption

-The possibility to evaluate how comparable the 2 groups are (transparency);

-The possibility to evaluate the quality of the matching, separately from the test of treatment effect;

-The possibility to look at treatment effects on the untreated individuals (ATU)