



# Propensity scores: a tool to help quantify treatment effects in observational studies

Cecilia Maria Patino<sup>1,2</sup>, Juliana Carvalho Ferreira<sup>1,3</sup>

## PRACTICAL SCENARIO

To evaluate the effect of early high-frequency oscillatory mechanical ventilation (MV) vs. conventional MV on duration of MV and in-hospital mortality among children with acute respiratory failure, a retrospective cohort study was conducted using data from a randomized controlled trial (RCT).<sup>(1)</sup> Multivariable models, adjusted for confounding factors using a propensity score (PS), showed that the children on high-frequency oscillatory MV, when compared with those on conventional MV, were less likely to discontinue MV (hazard ratio = 0.75; 95% CI: 0.64-0.89; p = 0.001) and not at increased risk of in-hospital mortality (odds ratio = 1.28; 95% CI: 0.92-1.79; p = 0.15).

## BACKGROUND

To evaluate the effect of interventions on health-related outcomes, RCTs are considered the gold standard study design because randomization gives every study participant a pre-established probability of being assigned to either an intervention or a comparison group. The goal is to prevent selection bias and confounding<sup>(2)</sup> at baseline by yielding the two groups with a similar distribution of measured and unmeasured confounders so that study results reflect the effect of the intervention on the outcome.

When conducting an RCT is not a feasible or ethical option, observational studies about interventions using PS to mimic randomization effects may be an alternative. The PS is a new composite variable that is created by combining a set of confounding variables that increase the probability of an individual being assigned to a specific

intervention (treatment A vs. treatment B) and then incorporated into the analysis. In our example, the goal was to evaluate the effect of two MV strategies (intervention) on duration of MV and in-hospital mortality (outcomes). To mimic the effects of randomization and make both groups similar regarding confounding variables, a PS was created, based on variables clinicians utilize to assign the specific MV strategy and included it in the multivariable analysis as a covariate to adjust for confounders.

## PROPENSITY SCORE

**Definition:** a variable that results from calculating the likelihood (propensity) of each participant receiving a treatment conditional on values of variables thought to influence the decision to prescribe treatment A or B.

**Variable selection:** Researchers select variables for PS based on their effect as confounders or predictors of the exposure (the intervention). Typical variables included in PS are demographics (age, gender, and socioeconomic status), disease severity, and characteristics of the treatment environment (characteristics of physicians and their practice). The variables are included as exposure variables in a logistic regression model with the intervention as the outcome. This model calculates a score for each participant representing their estimated likelihood of receiving treatment A or B, conditional on a weighed score of the values of that participant on the set of exposure variables used to create the PS.

**Analytical methods:** Four<sup>(3)</sup> strategies are typically used in observational studies (Table 1), each having advantages and disadvantages. We recommend consulting with a biostatistician to guide all PS processes.

**Table 1.** Methods used in order to include propensity scores in observational studies.

| Method               | Description   |
|----------------------|---|
| Stratification       | Strata are created with the participants that present with equal values in the propensity score. Weighted averages within strata are calculated before the multivariable analysis is conducted. |
| Matching             | Each exposed participant (treatment A) is matched to an unexposed participant (treatment B) with same propensity score value before the multivariable analysis is conducted.                    |
| Inverse weighting    | Two potential samples are created to represent samples that would have been observed if everyone had been exposed to the treatment or no one had been exposed to it.                            |
| Covariate adjustment | A regression model of the intervention on the outcome is fit to the both the intervention group (exposure) and the propensity score (covariate).  |

## REFERENCES

1. Bateman ST, Borasino S, Asaro LA, Cheifetz IM, Diane S, Wypij D, et al. Early High-Frequency Oscillatory Ventilation in Pediatric Acute Respiratory Failure. A Propensity Score Analysis. *Am J Respir Crit Care Med* 2016;193(5):495-503. <https://doi.org/10.1164/rccm.201507-1381OC>
2. Ferreira JC, Patino CM. Randomization: beyond tossing a coin. *J Bras Pneumol.* 2016;42(5):310. <https://doi.org/10.1590/S1806-37562016000000296>
3. Williamson E, Morley R, Lucas A, Carpenter J. Propensity scores: from naïve enthusiasm to intuitive understanding. *Stat Methods Med Res.* 2012;21(3):273-93. <https://doi.org/10.1177/0962280210394483>

1. Methods in Epidemiologic, Clinical and Operations Research-MECOR-program, American Thoracic Society/Asociación Latinoamericana del Tórax, Montevideo, Uruguay.

2. Department of Preventive Medicine, Keck School of Medicine, University of Southern California, Los Angeles, CA, USA.

3. Divisão de Pneumologia, Instituto do Coração – InCor – Hospital das Clínicas, Faculdade de Medicina, Universidade de São Paulo, São Paulo, Brasil.