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The Stata Journal Editors' Prize 2016: Patrick Royston

1 Prize announcement

The editors of the *Stata Journal* are delighted to announce the award of the Editors' Prize for 2016 to **Patrick Royston**.

The aim of the prize is to reward contributions to the Stata community in respect of one or more outstanding papers published in the *Journal* in the previous three calendar years. For the original announcement of the prize and its precise terms of reference, see Newton and Cox (2012), which is accessible at the following website: http://www.stata-journal.com/sjpdf.html?articlenum=gn0052.

John Patrick Royston was born in 1948 in Sutton Coldfield in the West Midlands of England. Although christened John Patrick and originally known as John, after three weeks, his parents decided Patrick was nicer, and that name has stuck. Patrick grew up in the West Midlands and also in Buckinghamshire and Dorset, where he attended Bryanston School (1961–1966). He received bachelor's and master's degrees in mathematics from the Universities of Essex and Warwick in 1971 and 1972, respectively. He earned a doctorate of science for his publications from the University of London in 1993.

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Having moved to the capital in 1973, Patrick has worked in London throughout his career, spending extended periods at the Medical Research Council (MRC) Clinical Research Centre, the Royal Postgraduate Medical School, and Imperial College. Since 2000, he has worked for the MRC Clinical Trials Unit and lately for University College London (UCL), following the merging of the MRC unit with UCL in 2013. He is a senior scientist at the unit and a professor of statistics at UCL.

Patrick's research interests center on statistical modeling and its medical applications, modeling continuous predictors, survival analysis, methodology of clinical trial design and analysis, and imputation of missing covariate data. He has always given a high priority to good-quality software implementations of novel statistical methods. Since the late 1980s, his preferred platform for such activity has been Stata.

Outside his day job, Patrick enjoys listening to classical music (excluding opera), particularly instrumental and chamber music, and duplicate bridge. He also enjoys walking.

We single out two areas arbitrarily that have been matched by research texts.

First, fractional polynomials as a modeling tool. Here Patrick has been the prime mover within statistical science, with original collaboration with Douglas G. Altman and continuing collaboration with Willi Sauerbrei and others. The method of fractional polynomials cleverly but simply takes many of the advantages of polynomials while protecting against some of their disadvantages and further extending their flexibility. The definitive survey is by Royston and Sauerbrei (2008). Here we echo the remarks in a review by Hosmer (2009, 990):

> . . . I think the book does an excellent job presenting what fractional polynomials are and how to use them to model continuous covariates in regression models. The authors are to be commended for giving a thorough treatment of factors that can have an adverse effect on their application. As I mentioned earlier, the book is more than its title, it really is a treatise on how to model data by two experienced and competent analysts. The book could be used as a text in an intermediate to advanced applied course on regression modeling and I highly recommend it to applied statisticians who want to learn about fractional polynomials and how to use them.

Second, work on survival analysis "beyond the Cox model". The text by Royston and Lambert (2011) traverses the middle ground between the Cox model and parametric models such as that based on the Weibull distribution. Researchers need both flexibility of fit and smoothness and versatility in prediction. Tools such as Royston–Parmar models, restricted cubic splines, and fractional polynomials help greatly. Survival analysis is vitally important territory, not only for its evident medical and industrial applications but also for other fields, including various social sciences.

The award specifically recognizes three outstanding papers by Royston (2014d, 2015b,a):

- Tools for checking calibration of a Cox model in external validation: Approach based on individual event probabilities (*Stata Journal* 14: 738–755)
- Tools for checking calibration of a Cox model in external validation: Prediction of population-averaged survival curves based on risk groups (*Stata Journal* 15: 275–291)
- Estimating the treatment effect in a clinical trial using difference in restricted mean survival time (*Stata Journal* 15: 1098–1117)

The award generally recognizes his enormous contributions to Stata and the Stata community over the last quarter-century.

The first and second papers focus on the Cox proportional hazards model, which has been used extensively in medicine over the last 40 years. A popular application is to develop a multivariable prediction model, often a prognostic model to predict the clinical outcome of patients with a particular disorder from "baseline" factors measured at some initial time point. For such a model to be useful in practice, it must be validated; that is, it must perform satisfactorily in an external sample of patients independent of the sample on which the model was originally developed. One key aspect of performance is calibration, which is the accuracy of prediction, particularly of survival (or equivalently, failure or event) probabilities at any time after the time origin. However, systematic evaluation of the calibration of a Cox model has been largely ignored in the literature.

The first paper suggests an approach to assessing calibration using individual event probabilities estimated at different time points. The method is exemplified by detailed analysis of two datasets in the disease primary biliary cirrhosis, a derivation and a validation dataset. A new command, `stcoxcal`, performs the necessary calculations. Results can be displayed graphically, which makes it easier for users to picture calibration (or lack thereof) according to follow-up time.

The second paper closely follows the first. It suggests an approach to assess calibration by comparing observed (Kaplan–Meier) and predicted survival probabilities in several prognostic groups derived by placing cutpoints on the prognostic index. Patrick distinguishes between full validation, where all relevant quantities are estimated on the derivation dataset and predicted on the validation dataset, and partial validation, where the prognostic index and prognostic groups are derived from published information and the baseline distribution function is estimated in the validation dataset. Partial validation is more feasible in practice because it is uncommon to have access to individual patient values in both datasets. The same datasets are used for examples. A new command, `stcoxgrp`, performs the necessary calculations and (as with `stcoxcal`) allows a strongly graphical approach.

The third paper discusses assessment of the causal effect of a new medical treatment compared with a standard regimen in a randomized controlled trial setting. When the main outcome is time to some event of interest, such as death, studies often use the hazard ratio to describe the treatment effect. Typically, proportional hazards are assumed. However, there are several major disadvantages to using the hazard ratio, including its vulnerability to the proportionality assumption, its relative nature, and its lack of relationship with time-to-event or survival probabilities. Restricted mean survival time is an alternative outcome measure in time-to-event trials. With this method, the treatment effect is defined as the difference in restricted mean between the trial arms. Royston and Parmar's class of flexible parametric models can be used to estimate the required quantities. With this approach, proportional hazards are not assumed. A new command, `strmst`, implements these calculations. This method supports "direct" adjustment for covariates by using marginalization over their observed distribution, and it supports estimation of treatment effects conditional on fixed values of covariates. One of the examples demonstrates the importance of understanding the relationship between the treatment effect, the prognosis of the disease outcome, and the often-neglected time domain.

These articles focus on areas where current statistical practice is inadequate. They explain in intuitive terms how researchers can do better and provide Stata commands and code that make the execution of the methods convenient to users. This convenience and intuition is very important to applied statisticians and characteristic of Patrick's work.

We now turn to Patrick's wider contributions. In the *Stata Technical Bulletin* (*STB*) and *Stata Journal* alone, Patrick published 82 papers between 1991 and 2016, many of outstanding Stata and statistical import. He has served as an associate editor of the *STB* and the *Stata Journal* since 1994. Patrick has been an organizer of 10 Stata Users Group meetings in London between 1996 and 2016 and has frequently given Stata presentations in London and elsewhere.

Patrick is credited as an original author of several official Stata commands, including `centile` ([R] **centile**), `cusum` ([R] **cusum**), `dotplot` ([R] **dotplot**), `lnskew0` and `bcskew0` ([R] **lnskew0**), `mfp` ([R] **mfp**), `nl` ([R] **nl**), and `swilk` and `sfrancia` ([R] **swilk**). His original code or design advice lies behind several more, including `boxcox` ([R] **boxcox**), `dydx` and `integ` ([R] **dydx**), `fp` ([R] **fp**), `glm` ([R] **glm**), `lowess` ([R] **lowess**), `sktest` ([R] **sktest**), and the entire `mi` suite.

The lengthy bibliography here is by no means a complete record of Patrick's work, because it omits his many papers (more than 200) in other journals.

Beyond his evident international and national reputation, Patrick's influence is also strong in his own workplace. We have ample testimony that about 60 statistical and epidemiological colleagues frequently use and much appreciate his Stata programs to design and analyze clinical trials, to develop and validate prognostic models, to model survival data, and to handle missing data. He has supervised and mentored several Ph.D. students and postdocs, who have in many cases developed their own substantial Stata programs, and patiently helped many people who stumble trying to use his methods, face to face or via email.

In sum, we salute Patrick for outstanding contributions to the Stata community and specifically through his recent publications in the *Stata Journal*.

As editors, we are indebted to the awardee for biographical material and to a necessarily anonymous nominator for a most helpful appreciation. We include below a full bibliography of Patrick's publications in the *Stata Technical Bulletin* and *Stata Journal*.

H. Joseph Newton and Nicholas J. Cox
Editors, *Stata Journal*

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