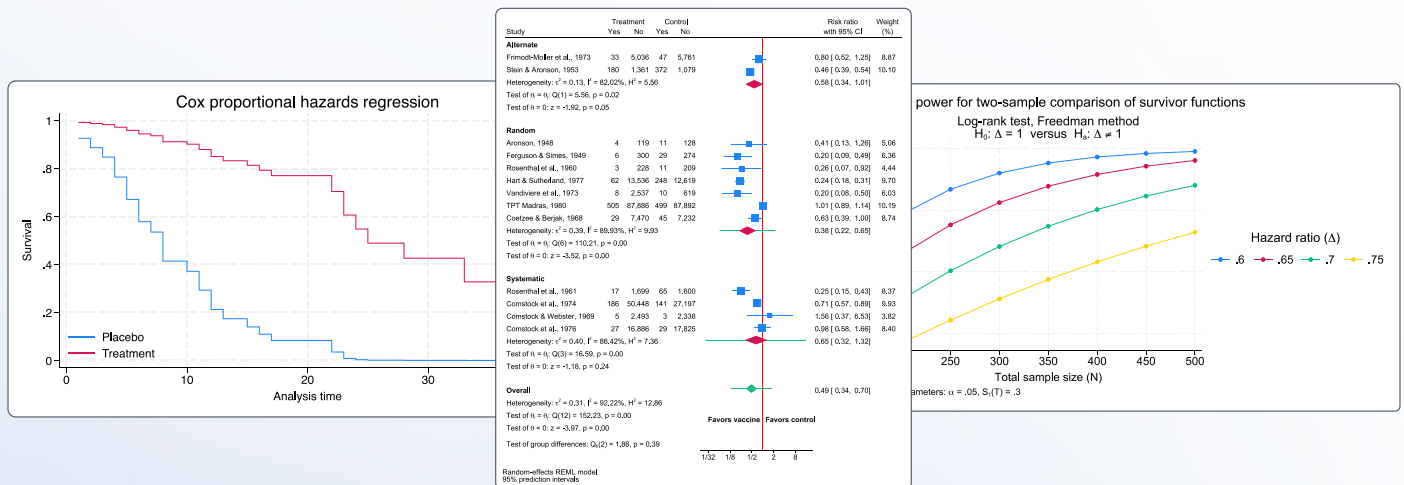


Clinical trials

Design and analysis

Stata provides a wide range of features to design and analyze clinical trials: power and sample-size determination using the **power** command, group sequential designs using the **gsdesign** command, evaluation of drugs' safety using the pharmacokinetic **pk** suite, analysis of survival-time outcomes by fitting a Cox model with the **stcox** command, combining results of multiple trials using the meta-analysis **meta** suite, and much more.



Create and automate reports

Stata offers powerful tools for clearly communicating your results.

From power curves to survivor functions to forest plots, Stata makes it easy to create publication-quality visualizations.

Customize tables reporting baseline characteristics, adverse events, regression results, and more.

With a single script, you can automate the creation of a reproducible report, complete with formatted text, tables, and graphs.

```

20 version 18
21
22 // Begin Word document
23 putdocx begin
24
25 // Add header
26 putdocx paragraph, style(Heading1)
27 putdocx text ("Follow-up measures")
28
29 // Create the table of measures and tests
30
31 dtable age hgt hwt iron albumin vitaminc zinc copper lead height weight ///
32 bmi sbp dbp chol trig hdl, by(treated, nototal tests) nosample ///
33 continuous(, stat(mean)) style(myStyle2, override) label(MyLabel2)
34
35
36 collect style putdocx, layout(autofitcontents)
37 collect title ///
38
39 "Table 2: Demographic, anthropometric, and lab measures"
40
41 putdocx collect
42
43 graph box chol, over(treated, des) title("Cholesterol")
44 name(chol, replace) ytitle("")
45 graph box sbp, over(treated, des) title("Systolic blood")
46 name(sbp, replace) ytitle("")
47 graph box dbp, over(treated, des) title("Diastolic blood")
48 name(dbp, replace) ytitle("")
49 graph combine chol sbp dbp, cols(3) imargin(medlarge)
50 graph export followup.png, replace
51
52 putdocx paragraph, halign(center)
53 putdocx image followup.png
54 putdocx save report.docx, replace
  
```

	Control	Treated	Test
Age (years)	54.9781	42.3505	<0.001
Hemoglobin (g/dl)	14.42456	14.14038	<0.001
Hematocrit (%)	42.44271	41.85235	<0.001
Serum iron (mg/dL)	96.17436	101.842	<0.001
Serum albumin (g/dL)	4.654088	4.680395	<0.001
Serum vitamin C (mg/dL)	1.015469	1.048238	0.007
Serum zinc (mcg/dL)	85.74782	87.06462	<0.001
Serum copper (mcg/dL)	126.3356	125.0756	0.067
Lead (mcg/dL)	14.93369	13.87513	<0.001
Height (cm)	167.5506	167.7243	0.366
Weight (kg)	76.85565	68.35026	<0.001
Body mass index (BMI)	27.36081	24.20231	<0.001
Systolic blood pressure (mmHg)	150.5388	116.485	<0.001
Diastolic blood pressure (mmHg)	92.01394	74.17222	<0.001
Serum cholesterol (mg/dL)	229.8738	208.7272	<0.001
Serum triglycerides (mg/dL)	166.0427	129.2284	<0.001
High-density lipids (mg/dL)	49.23794	49.84449	0.019

Community-contributed features

In addition to built-in features, Stata has a large and active community of researchers who are continuously adding new methods to Stata and often publish them in the *Stata Journal*. Finding and installing such commands is easy!

For instance, in Stata you can type

```
. search multi-stage design
```

You will see the **nstage** package in the list of other suitable commands. You can click on it to read more about it, install it, and then use it like any other Stata command:

```

> . nstage, accrue(100 100 100)      ///
>   arms(3 3 2)                   ///
>   alpha(0.4 0.2 0.025)          ///
>   omega(0.95 0.95 0.90)         ///
>   hr0(1 1) hr1(0.75 0.75)       ///
>   t(2 2) s(0.5 0.5)             ///
>   aratio(1) nstage(3) tunit(1)  ///
>   esb(0.025)

```

n-stage trial design version 4.0.1, 2 Nov 2018

Sample size for a 3-arm 3-stage trial with time-to-event outcome based on Royston et al. (2011) *Trials* 12:81 and Blenkinsop et al. (2019) *Clinical Trials*

Note: I outcome and D outcome are identical
Median survival time: 2 time units

Operating characteristics

Stage	Alpha (LOB)*	Alpha (ESB)*	Power	HR H0	HR H1	Crit.HR (LOB)	Crit.HR (ESB)	Length**	Time**
1	0.4000	0.0002	0.950	1.000	0.750	0.964	0.447	5.235	5.235
2	0.2000	0.0039	0.950	1.000	0.750	0.909	0.556	2.136	7.371
3	0.0250	.	0.900	1.000	0.750	0.842	.	2.742	10.113

Pairwise Error Rate 0.0233 Pairwise Power 0.8640
Familywise Error Rate (SE) 0.0425 (0.0002)

* All alphas are one-sided
** Length (duration of each stage) is expressed in periods and assumes survival times are exponentially distributed. Time is expressed in cumulative periods.

Sample size and number of events

	Stage 1		
	Overall	Control	Exper.
Arms	3	1	2
Acc. rate	100	33	67
Patients*	524	175	349
Events**	254	94	160

	Stage 2		
	Overall	Control	Exper.
Arms	3	1	2
Acc. rate	100	33	67
Patients*	737	246	491
Events**	431	157	274

	Stage 3		
	Overall	Control	Exper.
Arms	2	1	1
Acc. rate	100	50	50
Patients*	1011	383	628
Events**	492	260	232

* Patients are cumulative across stages
** Events are cumulative across stages, but are only displayed for those arms to which patients are still being recruited
** Events are for the same outcome at all 3 stages

Viewer - search clinical trials

search for **clinical trials** (manual: [R] search)

Search of official help files, FAQs, Examples, and Stata Journals

[ADAPT] gs Introduction to commands for group sequential design (help gs)

[PSS-2] power Power and sample-size analysis for hypothesis tests (help power)

[PSS-3] ciwidth Precision and sample-size analysis for CIs (help ciwidth)

[R] pk Pharmacokinetic (biopharmaceutical) data (help pk)

[R] ttest t tests (mean-comparison tests) (help ttest)

[ME] menl Nonlinear mixed-effects regression (help menl)

Video Group sequential designs

4/23 <http://www.youtube.com/watch?v=h02qW1NLrMk>
Demonstration of Stata 18's new gs suite of commands for designing group sequential trials. Compute efficacy and futility boundaries and sample sizes at each look for mean tests, tests of proportions, log-rank tests, or any user-defined test.

SJ-23-1 **st0700** . . . Sample-size calculation for an ordered categorical outcome White, Marley-Zagar, Morris, Parmar, Royston, and Babiker (help artcat if installed) M. J. Crowther Q1/23 SJ 23(1):3--23
calculates sample size or power for a randomized controlled trial or similar experiment with an ordered categorical outcome, where analysis is by the proportional-odds model

SJ-22-1 **st0275_1** Simulating time-to-event data (help survsim if installed) M. J. Crowther Q1/22 SJ 22(1):3--24
describes some substantial extensions to the survsim command for simulating survival (time-to-event) data from parametric distributions, custom distributions, competing-risks models, and general multistate models

SJ-18-4 **st0510_1** Power analysis for the Royston-Parmar combined test: Update (help power_ct, power_plot_survival if installed) P. Royston Q4/18 SJ 18(4):995--996
notes change for the Royston (2018, *Stata Journal* 18: 3-21) article

CAP NUM INS

Easily access your data

Use ODBC or JDBC to access your data from password-protected databases, including Oracle, MySQL, Amazon Redshift, Snowflake, Microsoft SQL Server, and more.

```
. odbc sqlfile("query.sql"), dsn("TrialData") user(myid) password(mypass)
```

Stata and FDA regulatory compliance

Learn how Stata satisfies FDA requirements, including installation qualification, documentation, certification, and more, at stata.com/stata-fda-compliance.

Multi-Arm Multi-Stage Trial Designs

Design parameters Operating characteristics Intermediate outcome Primary outcome

Total number of stages 3

E:C Allocation ratio 1 :1

Time unit (= 1 period) Year

Time of stopping accrual _____ periods

Show probabilities for number of arms in each stage

Calculate familywise error rate (FWER)

Control the FWER at level: 0.025

Assume non-binding stopping boundaries for lack-of-benefit

? OK Cancel Submit