Index

Analysis of Means (ANOM)

	•
compared with ANOVA, 194	Phase 1 and Phase 2, 277
experimental design data, 193–194	regression and cause-selecting charts,
proportions, 128–129	293–294
variances, 84	S-chart and S^2 -chart, 284–285
	Shewhart measurement charts, 278
Bioequivalence testing, 215, 251–252	Stage 1 and Stage 2, 277
	\overline{X} -chart, 282–283
Competing probability approach, 32, 254	Cost of sampling, 40
Cohen's effect sizes, 191–192	
Confidence intervals, 2–3	Ethical considerations, 40–42
on sample size and power, 67–68	Equivalence testing, 10–11, 43–45, 229
relationship to hypothesis tests, 5–6	example, 44–45
sample sizes for, 33–36, 105	for one proportion, 109–110
for a proportion	Evidential approach, 32
exact (Clopper–Pearson) interval,	External pilot study, 20
105	
normal approximation approach,	Finite populations, 32–33
110–112	Fisher's exact test, 121–122
with a finite population, 112	
Control charts, 277	Generalized linear models
adaptive, 293	sample size determination, 165
attribute charts, 286–289	
Ryan-Schwertman limits, 286-287	Hypothesis testing, 1–8
average run length (ARL), 280-283, 288,	criticisms, 2
290	limitations, 5
CUSUM and EWMA charts, 289-290	one-sided tests versus two-sided tests, 5,
EWMA for autocorrelated data, 293	41–42
variations, 291	<i>p</i> -value, 3–5
medical applications, 296-297	misinterpretations, 4

multivariate, 295-296

Hypothesis testing (Continued)	retrospective, 9
power of a test, 7	criticism, 9–10
significance level, 5	target, 59–60
Type I error, 8, 21, 75	using the software Design-Expert with
Type II error, 3–5, 8, 69, 75	experimental designs,
probability of, 8	207–208
	Preclinical experimentation, 253
Internal pilot study, 20–22	Probability assessment approach, 31–32
compared with external pilot study,	Probability of underestimating sample size,
20–21	23
for parameter estimation, 20–21	
	QT/QTc studies, 258
Journal of the American Medical	
Association, 253, 263	Reproducibility probability approach, 32
Lancet, 253	Sample size determination
	acceptance sampling, 300–301
Measurement system appraisal, 300	agreement studies, 351
Meta-analyses of clinical trials, 256	agricultural applications, 350-351
Microarray experiments, 219–220	AMOVA (analysis of molecular
	variance), 352
New England Journal of Medicine, 253	Bayesian approaches, 18–19, 24, 30–31
Noninferiority tests, 11, 229	case-control studies, 341
for one proportion, 109	clinical trials
for two proportions, 125	adaptive, 248
	Bayesian methods, 255
Pharmacodynamic experiments, 253	cluster randomized trials, 247
Pharmacokinetic experiments, 229, 253	combination treatments, 251
Pharmacogenetic experiments, 253	ethical considerations, 252
Pilot studies, 26	general considerations, 243-246
Power	multiple tests, 250
assumed versus actual power, 59-60, 196	noninferiority, 249
computed using noncentrality parameter,	Phase I, 246–247, 260
188, 199–200, 221, 347	Phase II, 246–248, 260, 262
definition of, 188	cancer trials, 247
conditional, 10	Phase III, 246–248
difficulty assessing power with	Phase IV, 248
nonparametric methods,	repeated measurements, 249–250
323–324, 326	software output, 261–263
in clinical studies, 252–253	using conditional power and predictive
observed, 9	power, 257
obtained using Lynch's tables for	using costs, 256
experimental designs,	using historical controls, 250
207–208, 210	using internal pilot studies, 250
of control charts, 280	using simulation, 246–247
predictive, 10	vaccine efficacy studies, 251
realistic values of power used in sample	cluster sampling, 345
size determination, 39	confidence intervals, 33–34

control charts	power, 185–186
CUSUM and EWMA charts	unequal sample sizes, 186-187
subgroup size considerations, 290–292	one factor, more than two fixed levels 187–192
determining subgroup size	hand calculation formulas, 28–29
using software simulations, 281–283	unequal sample sizes, 195–196
for Stage 1, 279–280, 302	Plackett–Burman designs, 220–222
for Stage 2, 280, 302	ray designs, 225
variable subgroup size in Stage 2, 279	repeated measures designs, 212–214
correlation, 172–176	split-plot designs, 222–224
confidence intervals, 174–175	strip-plot designs, 222–224
intraclass correlation, 175	two factors, 203–205
two correlations, 175–176	use of Excel spreadsheet, 226
epidemiology, 342	useful tables, 233
estimating population totals, 38	with random factors, 227–228
estimating sigma without data, 27	zero patient designs, 228
estimating the number of unseen species,	factor analysis, 346
351	for miscellaneous types of studies,
experimental designs	352–353
2^k designs, 205–209	genome-wide association studies, 351
2 ² design, 206	in auditing, 37
unreplicated, 206–207	inaccuracy of calculations, 17
2^{k-p} designs, 209–210	longitudinal studies, 342
Analysis of Covariance (ANCOVA),	maximum error of estimation, 34-37,
196–197	86–91, 111–112
Bayesian, 229	relative precision, 93–94
block designs	using prediction bound for sigma, 86
incomplete block designs	microarray studies, 343
balanced, 198	means
partially balanced, 199	Bayesian methods, 81
randomized complete block designs,	one mean, 58–63
197–198	confidence interval, 63–65, 84–88
completely randomized designs, 197	adaptive, 85
crossover designs, 202, 215-218, 230	exponential distribution, 71
detecting conditional effects, 210–211	unequal sample sizes, 74
for computer experiments, 228–229	using upper confidence bound on
for nonnormal responses, 225–226	sigma, 64, 67–68
Poisson distribution, 226	approach investigated, 68
Latin square designs, 199–203	two means, 71–81
Graeco-Latin square designs,	dependent samples, 78–81, 90–91
202–203	confidence interval on difference
using multiple Latin squares,	between means, 90–91
200–201	using coefficient of variation, 65–66
multiple comparisons, 192–193	using the t distribution, 70
nested designs, 224–225	robustness, 70
nested factor, 224	measures of agreement, 350
nested factorial, 224	meta-analyses, 343 multilevel modeling, 349
One factor, two fixed levels, 154-157	minuever modeling 349

Sample size determination (<i>Continued</i>)	rates
multinomial probabilities, 129–130	assuming Poisson distribution, 130,
multivariate methods, 346–348	133
Hotelling's T ² procedure, 346–348	one rate, 130–132
MANOVA (multivariate analysis of	pilot study, 132
variance), 346	two rates, 132–135
national security, 352	using variance stabilizing
nonparametric tests	transformation, 134
contingency tables, 334	receiver operating characteristic ROC
Kruskal-Wallis one-way ANOVA, 331	curves, 343
obstacles in sample size determination,	regression models
329	Cox regression, 167–169, 171
Mann-Whitney test, 327-330	possible problems using maximum
McNemar's test, 334	likelihood, 168
quasi-likelihood, 334-335	Draper-Smith rule of thumb, 148
rank correlation coefficients, 335	effect sizes, 150
sample size determined using	logistic regression, 155
asymptotic relative	assuming use of Wald test, 158,
efficiency, 325, 329	162
sign test, 331–334	complexities, 156-157
van Elteren test, 331	disagreements among software
Wilcoxon one-sample test, 324–325	solutions, 160
for paired data, 327	exact logistic regression, 167
not provided in most studies with results	software, 167
not having statistical	multiple logistic regression,
significance, 57	163–165
poor ad hoc approaches, 19	measurement error, 165
prediction intervals, 349–350	applet, 165
process capability indices, 297–298	possible problems interpreting
proportions	coefficients, 163
multiple proportions, 126–127	simple rule of thumb for
chi-square test, 127	determining sample size, 164
one proportion, 103	using variance inflation factor,
Bayesian approaches, 115	163
differing software solutions,	concern over such use,
108–109	163–164
exact approach, 113-114	ordinal logistic regression, 166–167
with arcsine transformation, 104	polytomous logistic regression, 165
with continuity correction, 107–108	simple logistic regression, 156–162
two proportions, 115–126	binary covariate, 162
approximation, 118	using Demidenko's applet, 158
arcsine transformation, 116	using two-sample t-test approach,
Bayesian method, 126	157
correlated proportions (McNemar's	new approach using Wetz criterion
test), 123–124	in simple linear regression, 147–148
equivalence tests, 124–125	in multiple linear regression,
linear trend (Cochran–Armitage	151–154
test), 125–126	nonlinear regression, 172
unequal sample sizes, 120	obstacles in simple linear regression,
with continuity correction, 119–120	146

problems interpreting coefficients of	Software
random regressors, 150	East, 260
obstacles in multiple linear regression,	ExpDesign Studio, 260
150	G*Power, 47, 84, 117, 131, 136, 170,
Poisson regression, 169–172	174–175, 230–231,
exact Poisson regression, 169-170	326–327, 329, 336, 346
using Soper's applet, 148-149, 152	Java applets, 45–47, 135, 158, 174, 186,
reliability and life testing, 301, 317–318	260, 317
sample surveys, 344	Lenth's applet, 22–23, 46, 58, 65, 74, 77,
vegetation surveys, 344	80, 109, 117, 121, 131–132,
sequential sample sizes, 343–344	135, 138, 147, 149, 174,
Slovin's formula, 20	186–187, 190, 195,
spatial statistics, 350	197–198, 200–202, 212,
standard deviation, 81	220, 222–224, 227–228, 230
structural equation modeling, 348–349	Mathematica, 189, 336
survey results, 259	MATLAB, 189
survival studies, 307	MINITAB, 7, 46, 60, 62, 65, 76, 79–80,
Cox proportional hazards model,	84–85, 89, 93–94, 105–106,
314–315	109, 113, 117, 121–122,
adjusting for covariates, 315	128, 131–133, 135, 175,
effect on sample size, 315	185–186, 188, 195, 198,
Freedman method, 311–312	204–205, 208–209, 212,
joint modeling of longitudinal and	217, 220–221, 228,
survival data, 315–316	230–231, 288, 301, 327
logrank test, 308–311	macro for computing power for fixed
multistage designs, 316	effects balanced designs, 230
number of deaths needed, 310	MLPowSim, 349
sample size challenges, 308	N Solution, 260
suggested formulas, 310-311	nQuery Advisor, 7, 27, 46, 65, 74-77,
Tarone–Ware test, 313	84–85, 87–90, 93–94, 106,
Wilcoxon-Breslow-Gehan test	109, 117, 121, 125–127,
lack of sample size software, 313	131, 136, 138, 154, 160, 162,
test reliability, 351	164, 174, 187, 195, 205, 217,
tolerance intervals, 298–300	231–232, 312, 316, 329,
using coefficient of variation, 113	331, 336, 350
using finite population correction factor	PASS, 7, 27, 29–30, 33, 35, 46–47, 60,
(fpc), 36–39, 195	62, 65, 72, 74–77, 79, 82–85,
using simplified, approximate formulas,	87–94, 103, 106, 109–110,
72–74	113, 117, 121–127, 130–131,
using standard deviation units, 26	133–135, 147–149,
variances, 81–84	154–155, 158–162, 164,
more than two, 84	168–171, 174–176, 187,
one variance, 81–83	194–198, 202–204,
approximation, 82-83	213–214, 216–218, 220,
confidence interval, 91–92	231–232, 260, 262,
one-sided confidence bound,	282–285, 287–288,
92–93	290–293, 301, 311–312,
two variances, 83	316, 318, 324, 326–329,
dependent samples, 84	331–334, 336, 346–347,
using Levene's test, 84	350

Software (Continued) SiZ, 46, 109, 121, 131, 158, 160-162, Power and Precision, 7, 46, 60, 62, 71, 164, 217, 350 75-76, 80, 84, 93-94, 104, Soper's applets, 148-149, 152-154, 173, 106-107, 109, 116-117, 348 121-122, 125, 131, 135-136, S-Plus, 24 Stata, 7, 46, 94-95, 121, 131, 135-136, 153–154, 158, 161, 162, 164, 174–176, 187, 189–190, 158, 168, 170, 173, 262, 195, 197, 201-203, 230, 316-317, 342 316 STATGRAPHICS, 301 R code/packages, 24, 69, 135, 189, 220, Standardized effect sizes, 42–43 328, 342–343 criticism, 42–43 SAS Software/programs, 45, 75, 81, 94, Superiority testing, 11 161, 198, 230, 232, 263, 331, 346 Value of information methods, 17, 256