2 Design tables, worksheets and checklists

Table 2.1 Protocol content and suggested features (ProtDoc.Tab)

The study protocol is the foundation of the trial. It is the first document produced and is likely revised several times over the course of the trial. It drives all other documents and publications in the trial. It serves as the basis for IRB submissions and as the road map for the trial.

Outside cover
Title (e.g., Protocol for the XYZ Trial)
Version number
Version date

Inside cover
Title
Version number
Version date
Print date
Document custodian

Table of contents (with page nos.)

History page (cumulative summary of changes from all previous versions)

Body of document
Print font: 11 or 12 point
Page orientation: Portrait
Margins (1" left, right, top, and bottom)
Page numbering: Continuous; upper right hand corner

References

Appendices
Glossary
Consent form
Design summary
etc.

Sample table of contents for ADAPT[1] (version 1.4; 19 Nov 2002; http://jhuccs1.us/adapt/documents.htm)

Table 2.1. Protocol content and suggested features

Table 2.2 Suggestions for development of study handbooks and manuals of operations (HandBk.Tab)

A. General

- Identify major topics or functions for which handbooks/manuals are required (e.g., clinic operations, data intake and processing, laboratory procedures, etc.)
- Develop draft table of contents for each required handbook/manual and submit for review and comment by the leadership group of the trial before development
- Develop methods and procedures for data collection with input from key study personnel, including clinicians, statisticians, clinic coordinators, laboratory technicians, and the like
- Strive to ensure that written material contained in handbooks/manuals is concise and devoid of complex sentences and esoteric language
- Test the adequacy of each handbook/manual by having it reviewed by individuals who will use it
- Release for use only after it has been reviewed and approved by study leadership

B. Organization

- Each handbook/manual should have a unique official name
- The name of the handbook/manual, date of release, version or edition number, and name of the individual or group responsible for its distribution should be indicated on the title page of the document
- Include a detailed table of contents, along with a listing of tables and figures in the document
- Include a subject index and glossary
- Chapters in manuals should be divided into numbered subsections; the accompanying numbers and titles should appear in the table of contents of the document
- Left-hand page margins should be wide enough to keep text from being obscured or lost when pages are photocopied or bound (e.g., at least 1" for standard 8½" x 11" pages assembled in loose-leaf notebooks or pressure binders)
- Right-hand page margins should be wide enough to allow room for user notes (e.g., at least 0.75" for standard 8½ x 11" pages). The same is true of top and bottom margins
- Boldface or other fonts should be used to identify key phrases, definitions, and important procedural statements
- Pages should be numbered sequentially from beginning to the end of a document; avoid numbering by chapter or section
- Page numbers should appear in the same location throughout the document regardless of page orientation (preferably upper right-hand corner)

C. Suggested maintenance aids

- Responsibility for review and revision of handbook/manual should be assigned to a specific individual or group
- A specific individual should be given responsibility for keeping track of revisions made to a handbook/manual and for making certain users of the handbook/manual are supplied with updates as they are produced
- Each new version of a handbook/manual should be identified with a version date and should indicate the date and version number of the document it replaces
- Large documents that are subject to frequent updates should be kept in loose-leaf binders to facilitate page replacements and to simplify photo-reproduction of pages in the document

Table 2.2 Study handbooks and manuals of operations

- Individual pages that are updated and inserted in an existing version of a document as replacements should include the revision date in the top or bottom right-hand corner of the pages

Table 2.3 Sample size specification table (SampSize.Tab)

When: After the design is set

Who: Study statistician

Purpose: To set down details of the sample size design

Definitions

class of trial - Class in regard to the nature of treatment effect to be detected as specified in sample size or power calculations for the trial: superiority trial, equivalence trial, noninferiority trial, and inferiority trial.

primary treatment group comparison - 1. The treatment group comparison of primary importance to the trial as specified in the study protocol and as used in sample size calculations. 2. One of several such comparisons in a trial involving multiple treatment groups, e.g., r-1 comparisons in a trial involving t treatment groups (t-1 test treatment groups and one control treatment) in which each of the t-1 treatments are compared against the control treatment.

treatment group - The group of persons assigned to receive a specified treatment in a trial

A. Identifying information

1. Study name: _____

2. Form completed by: _____

3. Date completed (day-month-year) . . . . . __—___—_____

B. Basic design features

4. Sample size design
   ( ) Fixed
   ( ) Sequential
   ( ) Open
   ( ) Closed

5. Treatment structure
   ( ) Parallel
   ( ) Crossover

Table 2.3 Sample size specification table

6. Number of treatment groups (including the control or comparison treatment group)
   ( ) Two
   ( ) ≥ three

   No. of primary treatment group comparisons . . . . . No. ___

   Specify primary treatment group comparisons

   _____

7. Primary outcome measure
   ( ) Change
   ( ) Event
      ( ) Death
      ( ) Cause specific death
      ( ) Disease
      ( ) Re-occurrence of disease
      ( ) Worsening of disease
      ( ) Other (specify)

      _____

   ( ) Other (specify)

   _____

8. Planned length of treatment and followup

   Anniversary closeout . . . . . Length _____ mos

   Common closing date . . . . . Length: Min _____ mos Max _____ mos

9. Planned length of followup same as planned length of treatment?
   ( ) Yes
   ( ) No

   Length of treatment . . . . . _____ mos

   Length of followup after treatment . . . . . Min _____ mos Max _____ mos

C. Sample size

10. Class of trial
   ( ) Superiority trial
   ( ) Equivalence trial
   ( ) Noninferiority trial
   ( ) Inferiority trial

Table 2.3 Sample size specification table

11. Sample size design
    ( ) Open sequential sample size design

    ( ) Closed sequential sample size design
    Minimum sample size . . . . . _____

    Maximum sample size . . . . . _____

    ( ) Fixed sample size design
    Total . . . . . _____

    Sample size per treatment group . . . . . _____

12. Sample size cited in item 11 is the result of (check one):
    ( ) Pragmatics (answer items 13 and 14)
    ( ) Sample size calculation

13. What is the driving pragmatic constraint:
    ( ) Money
    ( ) Availability of suitable people for study
    ( ) Other (specify)

    _____

14. Power with proposed sample size (provide a table of power values for different treatment differences)

    Power table here

15. Basis for sample size calculation; list details below, including the type I and II levels of error protection, the size of the difference to be detected, and the formula used for the calculation

    Specifications here

Table 2.4 Outcome specification table (Outcome.Tab)

When: During the design phase of the trial

Who: Study leaders

Purpose: To designate the primary outcome measure for use in the trial

Definitions

design variable - The variable used for determining sample size in planning a trial. Usually synonymous with primary outcome but need not be.

designed subgroup comparison - A subgroup comparison specified in the study protocol, especially one based on a sample size calculation when the trial was designed.

outcome measure - [trials] An observation variable recorded for a treatment unit at one or more time points after enrollment for the purpose of assessing the effect of a study treatment. 2. A measurement or observation used to measure the effect of an experimental variable. syn: **outcome variable**

primary outcome measure - That measure, among two or more in a trial, considered to be of primary importance in its design (e.g., the one used for the sample size calculation) or analysis; may be a continuous measure or an event depending on the trial.

secondary outcome measure - 1. A measure of relevance to a secondary objective of a trial. 2. A measure specified in the study protocol as secondary. 3. A measure specified as secondary in a study publication.

subgroup comparison - A comparison of treatment groups within a specified subgroup of people to assess treatment effect; subgroup typically defined by disease state or history on entry or by entry baseline or demographic characteristics.

surrogate outcome measure - An outcome measure used as a substitute for some other outcome. In trials, usually one that is known to be or presumed to be predictive of a clinical event and that, when used as a basis for designing a trial, leads to an estimated sample size or duration of followup that is less than that required for detecting a meaningful difference using the clinical event as an outcome measure.

A. Identifying information

1. Study name: _____

2. Form completed by: _____

3. Date completed (day-month-year) . . . . . __ __—__ __ __—__ __ __ __

Table 2.4 Outcome specification table

B. Primary outcome measure

4. Design variable
   ( ) Identical to primary outcome measure
   ( ) Different then the primary outcome measure (specify design variable)

   _____

5. Primary outcome measure (check one)
   ( ) All cause mortality
   ( ) Death due to a specific cause
   ( ) Clinical event
   ( ) Cause specific death or related nonfatal event
   ( ) Change measure
   ( ) Composite measure (specify)

   _____

   ( ) Surrogate measure (specify)

   _____

6. Clinical relevance of the outcome measure
   ( ) High
   ( ) Intermediate
   ( ) Marginal

7. Scientific rationale for choice of primary outcome measure (check one)
   ( ) Previous trials suggesting effect
   ( ) Evidence of effect from observational studies
   ( ) Deductive

8. Is the measure a surrogate measure?
   ( ) No
   ( ) Yes, indicate what the measure is a surrogate for and the scientific basis to supporting its use as a surrogate

   _____

   _____

   _____

Table 2.4 Outcome specification table

9. Is the outcome measure specified as primary in the study protocol?
   ( ) No (explain)

   _____

   ( ) Yes

C. Other outcome measures

10. Secondary outcome measures mentioned in the study protocol?
    ( ) No
    ( ) Yes; list

    _____

    _____

11. Safety outcome measures? (list)

    _____

    _____

D. Subgroup comparisons

12. Treatment comparisons by demographic or baseline entry characteristics mentioned in the study protocol?
    ( ) No
    ( ) Yes, answer items 13 and 14

13. Comparisons mentioned (check all that apply)
    ( ) Treatment group by gender
    ( ) Treatment group by age at entry
    ( ) Treatment group by ethnic origin
    ( ) Treatment group by disease state
    ( ) Other (specify)

    _____

14. Are any of the comparisons indicated in item 13 designed?
    ( ) No
    ( ) Yes (specify)

    _____

Table 2.5 Treatment specification table (Trt.Tab)

When: In the design phase of a trial

Who: Study leaders

Purpose: To specify the treatment design for the trial

Definitions

factorial treatment design - A type of treatment design in which one treatment (factor) is crossed (full or partial) with another treatment (factor). For example, a 2 x 2 factorial design in a trial with a parallel treatment design to evaluate the usefulness of counseling to reduce sodium intake and caloric intake in relation to blood pressure control; in its simplest form with just two levels for each factor (counseling or no counseling) yields four counseling regimens: AB, **A**B, A**B**, and **AB**, where **A** represents counseling for sodium reduction and A denotes absence of such counseling, and where **B** represents counseling for caloric restriction and B denotes absence of such counseling.

complete factorial - 1. A factorial design in which each treatment appears in combination with every other treatment; e.g., the treatment combinations AB, **A**B, A**B**, and **AB** in a parallel treatment trial involving test treatments **A** and **B** and matching placebos A and B. 2. A design in which a specified subset of treatments appear in combination with every other treatment in that subset; e.g., the treatment combinations represented above as part of a larger set involving treatments **C** and **D** not appearing in combination with **A** or **B** or their matching placebos.

incomplete factorial - 1. A factorial design in which only some of the combinations of factors, conditions, or treatments possible in a complete factorial appear, are used, or are administered; e.g., the combinations **A**B, **AB**, and AB in a trial involving the treatments **A** and **B** and matching placebos, A and B, respectively. 2. Any factorial arrangement that is incomplete, even if complete for a defined subset of the factors, conditions, or treatments represented, e.g., an arrangement in which the combinations **A**B, **AB**, A**B**, and AB are represented but in a trial involving a third treatment, **C**, not used in combination with **A**, A, **B**, or B.

treatment design - The portion of the study design that specifies the treatments to be evaluated, the nature of the treatment structure, the treatment assignment design, and the way in which the treatments are to be administered.

treatment modality, **treatment mode** - The method or agent used to treat, ameliorate, or prevent disease or to improve health; in regard to a test treatment in a trial, the general class of method or agent by which effect is to be achieved, e.g., via surgery, medical treatment, radiation, electrotherapy, drug, biologic, device, diet modification, dietary supplement, counseling, or etc.

treatment structure - The interrelation of treatment groups represented in a trial; parallel treatment structure, crossover treatment structure, factorial treatment structure.

Table 2.5 Treatment specification table

A. Identifying information

1. Study name: _____

2. Form completed by: _____

3. Date completed (day-month-year) . . . . . __-___-____

B. Treatment design

4. Test treatment groups
   Number . . . . . ____

   Test trt group 1 _____

   Test trt group 2 _____

   Test trt group 3 _____

   Test trt group 4 _____

5. Control/comparison treatment groups
   Number . . . . . ____

   Ctrl/comparison group 1 _____

   Ctrl/comparison group 2 _____

6. Treatment structure
   ( ) Crossover
      ( ) Complete
      ( ) Incomplete
   ( ) Parallel
      ( ) Uncrossed
      ( ) Crossed
         ( ) Complete factorial structure
         ( ) Incomplete factorial structure

7. Method of treatment assignment (check one)
   ( ) Randomization
   ( ) Other
      ( ) Systematic; order seen, day of the week, morning-afternoon
      ( ) Other (specify)

      _____

Table 2.5 Treatment specification table

8. Unit of assignment
   - ( ) Person
   - ( ) Part of person (e.g., eyes in an eye trial; specify)

     _____
   - ( ) Aggregate of persons
     - ( ) Household
     - ( ) Hospital ward
     - ( ) Census tract
     - ( ) Other (specify) _____

9. System of assignment?
   - ( ) Random
     - ( ) Simple
     - ( ) Restricted
   - ( ) Haphazard
   - ( ) Systematic/alternation
   - ( ) Deterministic
   - ( ) Deterministic with random component
   - ( ) Other (specify)

     _____

10. Assignment ratio?
    - ( ) Fixed
      - ( ) Uniform
      - ( ) Nonuniform (specify) . . . . . _____
    - ( ) Adaptive
      - ( ) Baseline (specify variable(s) used for adaptation)

        _____
      - ( ) Outcome (specify outcome used for adaptation)

        _____
      - ( ) Play-the-winner (specify outcome)

        _____
      - ( ) Other (specify)

        _____

Table 2.5 Treatment specification table

11. Treatment modalities represented in items 4 and 5 (check all that apply)
    ( ) Drug
    ( ) Vaccine
    ( ) Biologic
    ( ) Device
    ( ) Surgery
    ( ) Radiation
    ( ) Dietary regimen/diet supplement
    ( ) Exercise
    ( ) Educational/training regimen
    ( ) Placebo/sham
    ( ) Other (specify)

    _____

C. Treatment administration

12. Treatment masking
    ( ) None
    ( ) Single masked
        ( ) Patient masked
        ( ) Physician masked

    ( ) Double masked

13. Treatment application schedule
    ( ) Single application (specify point of application relative to point of treatment assignment and dosage)

    _____

    ( ) Multiple applications
        ( ) Daily to end of followup (specify dosage schedule; if double-masked drug trial specify pills per day)

        _____

        ( ) Other (specify schedule and dosage)

        _____

D. Other administrative and operational issues

14. Do any of the test treatments represented in item 4 require an IND or IDE?
    ( ) No
    ( ) Yes (specify holder of the IND or IDE)

    _____

Table 2.5 Treatment specification table

15. Are any of the test treatments represented in item 4 drugs, biologics, or devices?
    ( ) No
    ( ) Yes (specify suppliers)

    _____

    _____

16. Is the trial a double-masked drug trial?
    ( ) No
    ( ) Yes
    Who is the supplier of matching placebos
    ( ) The same as the supplier of the test treatments
    ( ) Other (specify)

    _____

WS 2.1 Terminology worksheet (Defns.WS)

When: Early in the course of design, prior to the development of key study documents

Who: Key leadership personnel; typically, in the case of a multicenter trial, personnel in the coordinating center

Purpose: To establish terminology conventions for use in development of key study documents

A. Identifying information

1. Study name: _____

2. Form completed by: _____

3. Date completed (day-month-year) . . . . . __-___-____

B. Generic terms (language conventions to be used in study documents and publications)

4. Person screened for enrollment into the trial
   ( ) Screenee
   ( ) Study candidate
   ( ) Other specify _____

5. Person enrolled in the trial
   ( ) Study patient (typical in treatment trials)
   ( ) Study participant (typical in trials involving study of healthy people or in settings where there is a desire to avoid medical connotations)
   ( ) Study subject
   ( ) Other (specify)

   _____

6. Collective name for body of persons enrolled in trial
   ( ) Study population
   ( ) Other (specify)

   _____

7. Experimental variable (i.e., the variable represented in the assignment process for the trial)
   ( ) Treatment/study treatment
   ( ) Intervention/study intervention
   ( ) Regimen/study regimen
   ( ) Arm/study arm
   ( ) Other (specify)

8. Name used for designating any one of study groups created by the assignment process regardless of whether test or control treatment
   ( ) Treatment group (recommended)
   ( ) Study group
   ( ) Other (specify)

   _____

9. Name used when referring to any of the study groups except the group receiving the control or comparison treatment
   ( ) Test-assigned group
   ( ) Test treated-assigned group
   ( ) Test treatment group
   ( ) Study group (not recommended)
   ( ) Other (specify)

   _____

10. Name used when referring to the control or comparison treatment in the trial
    ( ) Control-assigned group
    ( ) Control group
    ( ) Comparison-assigned group
    ( ) Comparison group
    ( ) Other (specify)

    _____

11. If control treatment is a placebo, name of person assigned to that treatment
    ( ) Placebo-assigned patient/participant
    ( ) Placebo patient/participant (avoid)
    ( ) Other (specify)

    _____

12. If the control treatment is a placebo, name of group assigned to that treatment
    ( ) Placebo-assigned group
    ( ) Placebo control-assigned group
    ( ) Placebo control group
    ( ) Placebo group (avoid)
    ( ) Other (specify)

    _____

13. Generic name for any outcome measure
    ( ) Outcome measure
    ( ) Event (avoid)
    ( ) Endpoint (avoid)
    ( ) Other (specify)

    _____

14. Generic name for observation variable
    ( ) Variable
    ( ) Parameter (avoid)
    ( ) Other (specify)

    _____

15. Name of study head?
    ( ) Principal investigator
    ( ) Study chair
    ( ) Other (specify)

    _____

16. Generic name of place where study persons are enrolled and followed?
    ( ) Study clinic
    ( ) Field site
    ( ) Other (specify)

    _____

17. Generic name of entire research team
    ( ) Research group
    ( ) Study group (not recommended)
    ( ) Other (specify)

    _____

18. For multicenter trial define:
    Center/study center (Recommended: An operational unit in the structure of a trial responsible for performing specified functions in one or more stages of the trial; e.g., a clinical center or resource center.)

    _____

    _____

Resource center (Recommended: Any center providing expertise and support in a differentiated study structure; in multicenter trials, usually any of the following: data coordinating center, treatment coordinating center, coordinating center, and project office; may also include data center, central laboratory, reading center, and quality control center if heads represented in the leadership structure of the trial are located in that center.)

_____

_____

19. Generic name for heads of centers:
    ( ) Center director
    ( ) Other (specify)

    _____

20. Name of the key leadership body (typically steering committee)

    _____

21. Give the name of the body responsible for treatment effects monitoring
    ( ) Data and safety monitoring committee (DSMC)
    ( ) Data and safety monitoring board (DSMB)
    ( ) Data monitoring committee (DMC)
    ( ) Policy data monitoring board (PDMB)
    ( ) Treatment effects monitoring committee (TEMC)
    ( ) Other (specify)

    _____

22. Name of site/center responsible for data processing and analysis
    ( ) Data center
    ( ) Biostatistics center
    ( ) Data coordinating center
    ( ) Coordinating center
    ( ) Other (specify)

    _____

C. Definitions

23. The point defining the end of the baseline period of observation and the start of the treatment and followup period of observation (Recommended: The point at which a person is assigned to treatment)

    _____

24. The point at which a person is counted as enrolled in the trial (Recommended: The point at which assignment is revealed to clinic personnel)

    _____

25. The point at which a person is counted as a dropout (Recommended: When the person misses a specified number of followup visits or when a person actively withdraws from followup)

    _____

26. Scheduled followup visit
    ( ) A followup visit specified in the study protocol to be done within a specified time window
    ( ) Other (specify)

    _____

27. Unscheduled followup visit (aka interim visit)
    ( ) A followup visit that is not part of the followup data collection protocol
    ( ) Other (specify)

    _____

28. Missed clinic visit (Recommended: A clinic visit, as per the schedule specified in the study protocol, that was not made within the permissible time window for the visit)

    _____

29. Lost to followup (Recommended: Typically a dropout for observations that require clinic visits)

    _____

30. Consent (Recommended: Signed dated consent on IRB-approved consent form)

    _____

31. Baseline data (Recommended: Data collected after consent for screening through to the point of treatment assignment as marked by the point at which treatment assignment is revealed to clinic personnel)

    _____

32. Baseline period (Recommended: From first screening visit up to the point of treatment assignment)

    _____

33. Followup (Recommended: A process involving periodic contact with persons enrolled in a trial for the purpose of administering the assigned treatment(s), observing the effects of treatment(s), modifying the course of treatment(s), or for collecting required followup data.)

---

34. Randomized (Recommended: The condition of having been assigned to a treatment via a random process; normally considered to have occurred when the treatment assignment is revealed to any member of the clinic staff, e.g., when an envelope containing the treatment assignment is opened at the clinic.)

---

35. Time window (Recommended: The time interval for performing a specified activity or procedure. In trials and other followup studies, usually the window for performing a specified examination or type of data collection, such as for a baseline or followup visit.)

---

36. Intention to treat (Recommended: A philosophy in which there is an intent to account for all persons enrolled in a trial and to perform analyses by assigned treatment, regardless of observed course of treatment.)

---

37. Subgroup (A subpart or subset of a study population distinguished by a characteristic or set of characteristics, especially, in the case of trials, such a subpart or subset as distinguished by one or more baseline characteristics.)

---

38. Stopping rule (A rule for determining when to terminate or alter the treatment protocol of a trial based on the observed treatment difference for an outcome of interest; usually some function of a p-value produced by a designated test statistic evaluated at specified points in the course of the trial.)

---

39. Stopping guidelines (A guide as to size or type of treatment differences that may cause treatment effects monitors to recommend stopping or altering a trial. Not to be used interchangeably with stopping rule. Use stopping guideline instead of stopping rule if the rule is used simply as a guide as to when a stop or alteration may be indicated.)

---

40. Multiple look (Treatment comparisons made at two or more time points over the course of a trial; especially when done in relation to treatment effects monitoring and where they may lead to alteration of the treatment protocol.)

    _____

41. Protocol violation (A protocol departure considered to be serious, e.g., administration of the wrong treatment or enrollment of an ineligible person.)

    _____

42. Terms avoided (check all that apply)
    ( ) Treatment failure (presumptive)
    ( ) Informed consent (wishful thinking in the absence of information to indicate consent is truly informed)
    ( ) Endpoint (often interpreted by clinic staff to be synonymous with cessation of followup; not the case except where the "endpoint" is death)
    ( ) Placebo patient (no such person; use placebo-assigned)
    ( ) Drop-in (a person who receives a study treatment different than the one assigned in a trial; use inconsistent with analysis by original treatment assignment)
    ( ) Other (specify)

    _____

    _____

    _____

D. Abbreviations and labels

43. Abbreviations to be used in study documents and publications

Clinic (Recommended: Cl) . . . . . _____

Screening (Recommended: Scr) . . . . . _____

Screening visit (Recommended: ScrV) . . . . . _____

Baseline (Recommended: Bl) . . . . . _____

Baseline visit (Recommended: BlV) . . . . . _____

Followup (Recommended: Fu) . . . . . _____

Followup visit (Recommended: FuV) . . . . . _____

Treatment (Recommended: Trt) . . . . . _____

Treatment visit (Recommended: TrtV) . . . . . _____

44. Labels for treatment groups in treatment effects monitoring reports and publications

Test trt 1 . . . . . _____

Test trt 2 . . . . . _____

Test trt 3 . . . . . _____

Test trt 4 . . . . . _____

Test trt 5 . . . . . _____

Test trt 6 . . . . . _____

Control trt 1 . . . . . _____

Control trt 2 . . . . . _____

45. In multicenter trials, the letter codes for clinics to be displayed in performance monitoring reports

Clinic #	Location	Code
Cl 1		
Cl 2		
Cl 3		
Cl 4		
Cl 5		
Cl 6		
Cl 7		
Cl 8		
Cl 9		
Cl 10		

46. Other labels

Prime leadership committee . . . . . _____

Data center . . . . . _____

Monitoring committee . . . . . _____

WS 2.2 Name and acronym worksheet (BigName.WS)

When: On initiation of funding for a study or on creation of a multistudy structure

Who: Study chair or director of coordinating center with sign-off by study officers and steering committee

Purpose: To choose a study name or an umbrella name and a related acronym or acrostic

Related forms: WS 17

Definitions

umbrella name - A name chosen to characterize a collection of studies as preformed under a common corporate structure, e.g., as represented in a study network

study name - A name chosen to characterize a particular study

Reminders and recommendations

Name

- Choose in favor of brevity, crispness, and succinctness
- Avoid redundant terms like "controlled" in "randomized controlled trial" and unnecessary descriptors like "clinical" in "clinical trial"
- Avoid catchy or "cute" names
- Avoid restrictive terms likely to render a name obsolete by subsequent expansion to activities not covered by the name
- Choose in favor of neutral, nonpromotional, names
- For umbrella names consider in conjunction with likely names of particular studies; avoid likely redundancies or contradictory terms when used in conjunction with the name of a specific study
- Keep other likely uses in mind, as in funding applications, publications, presentations, and other written references
- Avoid use of contrived, unprintable, characters
- Avoid choosing to create a desired or "cute" acronym
- Avoid names producing undesirable or vulgar acronyms (Reminder: A meaningless sequence of letters in one language can have meaning in another)
- Be cautious of regional geographical descriptors such as U.S. or Europe that require change if the activity eventually expands beyond the limit represented by the descriptor
- Avoid ill-defined regional terms such as "National" and "International"
- Once chosen, avoid variations of name

Acronym

- Useful for shorthand nomenclature in study documents and publications
- Ideally, choose to be of six or fewer characters
- Include base term in study name, e.g., "trial" as in NETT – National Emphysema Treatment Trial
- Include "network" or other like terms in umbrella name
- Avoid choosing name simply to produce pronounceable acronym

- Be wary of pronounceable acronym, e.g., MRFIT (Multiple Risk Factor Intervention Trial); converted by critics to Ms Fit because trial included only males
- Avoid variations
- Keep likely contractions of name and acronym in mind when choosing

A. Identifying information

1. Form completed by: _____

2. Date completed (day-month-year) . . . . . __—___—____

3. Form completed for (check one):
   ( ) Study name
   ( ) Umbrella name

B. Specifications

4. Maximum number of words, exclusive of articles and connectors . . . . . _____

5. Maximum number of characters, including spaces and punctuation . . . . . _____

6. Base descriptor for activities represented under the name
   ( ) Study(ies)
   ( ) Project(s)
   ( ) Program(s)
   ( ) Other (specify)

   _____

C. Descriptors and modifiers

7. Generic descriptors (check all that apply)
   ( ) Clinical
   ( ) Cooperative
   ( ) Multicenter
   ( ) National
   ( ) International
   ( ) Randomized
   ( ) Controlled
   ( ) Mask; blind
   ( ) Prospective
   ( ) Retrospective
   ( ) Surveillance
   ( ) Followup
   ( ) Other (specify)

   _____

8. Disease descriptor in name?
   ( ) No
   ( ) Yes (specify below)

   _____

9. Population descriptors in name?
   ( ) No
   ( ) Yes
      ( ) Age (e.g., infants, children, adults, elderly) _____
      ( ) Gender (male, men, boys; female, women, girls) _____
      ( ) Race/ethnic origin _____
      ( ) Other _____

10. State indicators in name (e.g., pregnant, healthy, normal, diseased, abnormal, etc.)?
    ( ) No
    ( ) Yes (specify)

    _____

11. Other descriptor terms in name?
    ( ) No
    ( ) Yes (specify)

    _____

D. Candidate names and acronyms

12. Candidate names

    a. _____

    b. _____

    c. _____

    d. _____

13. Candidate acronyms

    a. . . . . _____

    b. . . . . _____

    c. . . . . _____

    d. . . . . _____

E. Official name and acronym

14. Proposed official name

    _____

    No. of characters . . . . . ____

    No. of words, excluding articles and connectors . . . . . ____

15. Proposed official acronym . . . . . _____

16. Name of approving study body: _____

17. Date of approval: . . . . . __ __—__ __ __—__ __ __ __

WS 2.3 Study logo worksheet (Logo.WS)

When: Early in the stage of the study

Who: Study chair or director of coordinating center with sign-off by study officers and steering committee

Purpose: To provide a printable brand for identifying documents from the study

Related form: WS 2.2

Reminders and recommendations

- Useful for branding study documents such as handbooks, manuals, data reports, study forms and letterheads
- If in colors, choose colors easily seen and distinguishable under adverse light conditions; choose and arrange colors so that content remains intelligible when reproduced in black and white
- Reject logos that are easily degraded when copied or that become unreadable when copied several times on different machines and under different intensity settings

A. Identifying information

1. Study name: _____

2. Completed by: _____

3. Date completed (day-month-year) . . . . . __—___—____

B. Specifications and desired characteristics

4. Content:
   ( ) Study acronym or acrostic included?
   ( ) Study name?
   ( ) Slogan or motto?

   _____

   ( ) Figures, objects, special characters, or symbols?

   _____

   ( ) Other

   _____

5. Characteristics:
   ( ) Black and white?
   ( ) Colored? (list colors)

   _____

   ( ) Still readable when photocopied?
   ( ) Word processor importable?
   ( ) Other

   _____

6. Uses
   ( ) Letterhead
   ( ) Headers or footers of forms
   ( ) Covers of manuals, handbooks, and study reports
   ( ) Consent documents
   ( ) Materials given to study participants during the study
   ( ) Other

   _____

C. Candidate logos

Sample A

Sample B

Sample C

D. Official logo

E. Sign-off

7. Name of accepting body: _____

8. Date of acceptance: . . . . . __ __—__ __ __—__ __ __ __

WS 2.4 Data sharing worksheet (DataGive.WS)

When: Complete in design phase of trial; review and update over the course of the trial

Who: Director of data center

Purpose: To enumerate data sharing expectations and responsibilities

Definitions

de-identified data - Data stripped of personal identifiers; data contained in a limited dataset.[6]

De-identification, as spelled out in HIPAA,[5] involves deleting for persons studied, their relatives, household members, and employers:

- Names
- Any geocodes that identify an individual household such as street address or Post Office Box Number
- Telephone numbers
- Fax numbers
- Electronic mail addresses
- Social Security Numbers
- Medical record numbers
- Health plan beneficiary identifiers
- Account numbers
- Certificate/license numbers
- Vehicle identifiers and serial numbers, including license plate numbers
- Medical device identifiers and serial numbers
- Web universal resource locators (URL)
- Internet Protocol (IP) address numbers
- Biometric identifiers, including finger and voice prints
- Full face photographic images

Datasets must also be devoid of:

- Geographic subdivisions designations smaller than a state (i.e., county, city, town, precinct)
- 5 or 9 digit ZIP codes (1st three digits allowable in most cases)
- All elements of dates (except year) directly related to an individual, including dates of birth or death, dates of health care services or health care claims (de-identified datasets cannot contain birth dates; file may contain the individual's age expressed in years, months, days, or hours, as appropriate, except for individuals aged 90 or above; such persons to be identified simply as being 90 or above)
- Any other unique identifying number, characteristic, or code that could be used to identify the individual (supplier of data may affix codes to allow user to associate data with persons, provided codes cannot be used to re-identify persons)

identified data - Data identifiable to a person by Id number or other personal identifiers.

WS 2.4 Data sharing worksheet

A. Identifying information

1. Form completed by: _____

2. Date completed (day-month-year) . . . . . __—___—_____

3. Types of data sharing planned (check all that apply)
   ( ) Data sharing within the investigator group (**Section B**)
   ( ) Data sharing external to the investigator group
   ( ) Voluntary external data sharing (**Section C**)
   ( ) Mandated external data sharing (**Section D**)

B. Internal data sharing (Data sharing within the investigator group)

4. Internal data sharing planned?
   ( ) Yes
   ( ) No (explain; internal sharing is the norm)

   _____

   _____

5. Mode of sharing during study (check all that apply)?
   ( ) None planned (explain)

   _____

   ( ) On demand via access to study database
   ( ) Via request to data center

   ( ) Other (specify) _____

6. Mode of sharing on completion of study (check all that apply)?

   ( ) None planned (explain)

   _____

   ( ) Finished dataset supplied to investigators via data center
      ( ) Supplied identified
      ( ) Supplied de-identified

   ( ) Other (specify)

   _____

7. Signed agreement by investigators receiving data to not identify persons studied and to not copy or provide data to persons outside the investigator group?
   ( ) Yes
   ( ) No (explain)

_____

C. Voluntary external data sharing (Data sharing with persons external to the investigator group and in which study investigators determine whether to share; typically external voluntary sharing carries conditions including the right of investigators to review uses prior to presentation or publication; data may be provided identified or de-identified depending on agreement.)

8. Voluntary data sharing planned?
   ( ) Yes
   Mode
   ( ) Passive (by request; no announcement of willingness to entertain requests for sharing)
   ( ) Active (by announcement of willingness to share on study website or in study publications)
   ( ) No

9. Procedure for reviewing requests (check all that apply)?
   ( ) Study chair/PI
   ( ) Study officers
   ( ) Steering committee
   ( ) Study officers & steering committee
   ( ) Other (specify)

_____

10. Sharing of interim treatment results?
    ( ) No (the norm)
    ( ) Yes (explain)

_____

11. Entertainment of external requests for analyses?
    ( ) No
    ( ) Yes
    ( ) Via analyses done by study data center without cost to requestor
    ( ) Via analyses by study data center with costs covered by requestor
    ( ) By requestor via data provided by the study data center
    ( ) Other (specify)

_____

12. Entertainment of external requests for study datasets?
    ( ) No
    ( ) Yes
        ( ) Supplied de-identified without use restriction
        ( ) Supplied de-identified with use restriction
        ( ) Supplied identified with use restriction
        ( ) Other (specify)

        _____

    Cost of preparation
    ( ) Supplied without cost to requestor
    ( ) Cost of preparation covered by requestor
    ( ) Other (specify)

        _____

D. Mandated external data sharing (Data sharing external to the investigator group mandated by the sponsoring agency and in which requests for data are answered by providing requestors de-identified data typically without investigator approval or constraints on use.)

13. Work scope of funding award includes provisions for mandated external data sharing?
    ( ) Yes
    ( ) No

14. Consent form includes mention of intent to provide access to de-identified data by people outside the investigator group?
    ( ) Yes
    ( ) No

15. Is the planned sample size per treatment group sufficiently large to make de-identification possible with minimal risk of probabilistic identification?
    ( ) Yes
    ( ) No

16. Reconciliations
    If Item 13 is answered Yes and Item 14 is answered No, explain the inconsistency

    _____

WS 2.4 Data sharing worksheet

Note

If Item 15 is answered No and Item 13 is answered Yes, revise work scope to exclude mandatory external data sharing or inform funding agency of unwillingness to engage in unrestricted release of study datasets

17. Mode of mandatory external data sharing
    ( ) Submit datasets to sponsor to deal with requests for datasets
    ( ) Submit datasets to 3rd party custodian to deal with requests for datasets
    ( ) Study data center to serve as custodian
    ( ) Other (specify)

    _____

18. Mode of announcing availability of datasets for external sharing (check all that apply)
    ( ) Study website
    ( ) Study publications
    ( ) Other (specify)

    _____

19. Types of datasets to be made available to persons external to the investigator group (check all that apply)
    ( ) Datasets corresponding to datasets used in publications
    ( ) Finished dataset containing all study data, whether or not used in previous publications
    ( ) Other (specify)

    _____

20. Time when de-identified data to be made (check all that apply)
    ( ) In relation to individual study publications (e.g., by announcing availability of dataset supporting a publication in the publication)
    ( ) Finished dataset compiled after the end of data collection and after editing and "cleanup" even if the investigator group is still working on publications
    ( ) Finished dataset after cessation of paper writing activities by the investigator group
    ( ) Finished dataset as supplied by data center in final year of support even if investigator group still working on publications
    ( ) Other (specify)

    _____

CL 2.1 Treatment design synopsis checklist (TrtDesig.CL) (per Coronary Drug Project)[4]

When: After the trial is designed

Who: Senior people in the coordinating center

Purpose: To provide a synopsis of study design for use in study documents and in producing study publications

1. Trial type
- (x) Superiority
- ( ) Equivalence
- ( ) Inferiority
- ( ) Demonstration
- ( ) Other

2. Trial phase
- ( ) 1
- ( ) 2
- ( ) 3
- (x) 4
- ( ) Other

3. Purpose
- (x) Prevention
  - ( ) Primary
  - (x) Secondary
- ( ) Treatment
- ( ) Other

4. Test treatment(s)

Estrogen (ESG) mixed conjugated equine estrogen; two estrogen treatment groups: one receiving a dosage of 2.5mg/day and another receiving 5.0mg/day; Premarin®

Colfibrate (CPIB) ethyl alpha parachlorophenoxy-isobutyrate; Atromid-S®

Dextrothyroxine (DT4); Choloxin®

Nicotinic acid (NICA)

5. Primary outcome measure
- ( ) Event
  - (x) Death, any cause; 5 yr mortality
  - ( ) Death, specific cause
  - ( ) Morbid clinical event
  - ( ) Other
- ( ) Change measure
- ( ) Other

6. Treatment groups

Test treatment groups
- ( ) 1
- ( ) 2
- ( ) 3
- (x) 4 or more

Control treatment groups
- (x) 1
- ( ) 2
- ( ) 3 or more

Total no. of treatment groups
- ( ) 2
- ( ) 3
- ( ) 4
- (x) 5 or more

7. Control treatment(s)
- ( ) Observation only
- (x) Matching placebo
- ( ) Sham procedure
- ( ) Standard medical care
- ( ) Other

8. Treatment structure
- (x) Parallel
  - ( ) Full factorial structure
  - ( ) Partial factorial structure
  - (x) Independent (uncrossed)
- ( ) Crossover
- ( ) Other

9. Treatment assignment design
- (x) Randomized
- ( ) Systematic
- ( ) Physician judgment
- ( ) Patient choice
- ( ) Other

10. Assignment unit
- ( ) Geographical area
- ( ) Household
- (x) Person
- ( ) Person part
- ( ) Other

11. Assignment ratio
- ( ) Uniform (same across treatment groups)
- (x) Non-uniform (specify); 1 to 2.5, per treatment group relative to control treatment, i.e., 1:1:1:1:1:2.5

12. Treatment administration
- ( ) Unmasked
  - ( ) Fully unmasked
  - ( ) Partially unmasked
- (x) Masked
  - ( ) Single-masked
  - (x) Fully double-masked
  - ( ) Partial double-masked

13. Treatment modalities
- (x) Drugs
  - ( ) Pills
  - ( ) Injections
  - ( ) Implants
  - ( ) Other
- ( ) Medical device
- ( ) Surgery
- ( ) Radiation
- ( ) Dietary
- ( ) Behavior modification
- ( ) Other

14. Bias control procedures
- (x) Concealment of assignments until issue
- (x) Masked treatment administration
- (x) Shielding of investigators from interim results
- (x) Independent treatment effects monitoring
- ( ) Other

15. Sample size requirement
- ( ) Not stated
- (x) Specified

16. Sample size rationale
- (x) Calculated
- ( ) Pragmatic
- ( ) Unspecified

17. Variance control procedures
- (x) Randomization
- (x) Stratification; clinic and two risk groups
- (x) Blocking of assignments
- ( ) Other

18. Primary treatment comparison in trials with more than one test-treated group

(x) Simple, i.e., comparison of individual test-treated groups vs control-treated group

( ) Complex: Simple comparison plus:

- ( ) Test-treated group compared with other test-treated groups
- ( ) Combinations of test-treated groups compared with control-treated group
- ( ) Other