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| **artbin: evaluation of sample size and power in randomized controlled trials with a binary outcome** |

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| --- | --- | --- | --- | --- |
| Developer .AB/PR |  |  | | |
| Date of prog creation……23/03/2004 | Date of last change (doc)...09/06/2022 |  | | |
| Reviewer .........................EMZ | Artbin version…………..….2.0.1 |  | | |
| Review date 13/11/2018 | Initials (reviewer).. ..EMZ |  |  |  |
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Work overview

A Stata Program was created to calculate sample size and power in trials with a binary outcome, named artbin.

artbin calls the sub-program:

* art2bin.ado

Action 1: Creating the program

Original work

The program was originally created by A. Babiker and P. Royston on 23/03/2004, and subsequently expanded in collaboration with F. Barthel and M. Parmar.

Original testing

Performed at the time by the program authors.

File changes

Updates to the program functionality were implemented up to 09/06/2022.

Action 2: Further testing the program artbin

Original work

The program artbin.ado underwent verification using published paper lists of sample size and power calculations by EMZ, for further details please see below. Please note that artbin.ado provides the *total* sample size (2n), whereas the papers provide the sample size per treatment arm (n). The verification testing files are called artbin\_testing\_*[x]*.do where *x = 1,2,3,4.*

Error messages and boundary limits were also examined using the testing file artbin\_errortest\_8.do.

*Original testing 1: Verification (sample size)*

| **Trial** | **Outcome** | **Reference** | **Parameters (α one-sided unless otherwise stated)** | | **Reference n** | | **artbin code** | **artbin result /2**  **2n/2** |
| --- | --- | --- | --- | --- | --- | --- | --- | --- |
| Non-inferiority | Binary | Blackwelder 1982, Table 3 | | p = 90%, d = 20%, α = 5%, β = 10% | 39 | artbin, pr(0.1 0.1) margin(0.2) alpha(0.1) power(0.9) wald | | 39 |
| Non-inferiority | Binary | Julious 2011, Table 4 | | p = 70%, d = 5%, α = 2.5%, β = 10% | 1766 | artbin, pr(0.3 0.3) margin(0.05) alpha(0.05) power(0.9) wald | | 1766 |
| Non-inferiority | Binary | Pocock 2003 | | p = 85%, d = 15%, α\* = 5%, β = 10% | 120 | artbin, pr(0.15 0.15) margin(0.15) alpha(0.05) power(0.9) wald | | 120 |
| Non-inferiority | Binary | Sealed envelope calculator | | p = 80%, d = 10%, α = 10%, β = 20% | 145 | artbin, pr(0.2 0.2) margin(0.1) alpha(0.2) power(0.8) wald | | 145 |
| Non-inferiority | Binary | Julious 2011, Table 4 | | p = 90%, d = 5%, α = 2.5%, β = 10% | 757 | artbin, pr(0.1 0.1) margin(0.05) alpha(0.05) power(0.9) wald | | 757 |
| Non-inferiority | Binary | Julious 2011, Table 4 | | p = 75%, d = 20%, α = 2.5%, β = 10% | 99 | artbin, pr(0.25 0.25) margin(0.2) alpha(0.05) power(0.9) wald | | 99 |
| Non-inferiority | Binary | Julious 2011, Table 4 | | p = 80%, d = 15%, α = 2.5%, β = 10% | 150 | artbin, pr(0.2 0.2) margin(0.15) alpha(0.05) power(0.9) wald | | 150 |
| Non-inferiority | Binary | Julious 2011, Table 4 | | p = 85%, d = 5%, α = 2.5%, β = 10% | 1072 | artbin, pr(0.15 0.15) margin(0.05) alpha(0.05) power(0.9) wald | | 1072 |
| Superiority | Binary | Pocock 1983 | | p 1 = 90%, p 2 = 95%, α\* = 5%, β = 10% | 578 | artbin, pr(0.05 0.1) alpha(0.05) power(0.9) wald | | 578 |
| Superiority | Binary | Sealed envelope calculator | | p 1 = 90%, p 2 = 80%, α = 10%, β = 20% | 155 | artbin, pr(0.1 0.2) alpha(0.1) power(0.8) wald | | 155 |

*\* Note: Pocock assumes a 2-sided confidence interval will be produced for non-inferiority trials.*

*Original testing 2: Comparing sample size from artbin to that of ssi*

| **Trial** | **Outcome** | **Parameters (α one-sided unless otherwise stated)** | | **Ssi code** | | **Ssi**  **2n** | | **artbin code** | | **artbin result**  **2n** | |
| --- | --- | --- | --- | --- | --- | --- | --- | --- | --- | --- | --- |
| Non-inferiority | Binary | p0 = 0.95, p1 = 0.9, α = 5%, β = 10% | ssi .05 .05, alpha(.05) power(.9) non | | 652 | | artbin, pr(.05 .05) margin(0.05) alpha(0.1) power(0.9) wald | | 652 | |
| Non-inferiority | Binary | p0 = 0.8, p1 = 0.7, α = 5%, β = 20% | ssi 0.2 0.1, alpha(.05) power(.8) non | | 396 | | artbin, pr(0.2 0.2) margin(0.1) alpha(0.1) power(0.8) wald | | 396 | |
| Non-inferiority | Binary | p0 = 0.7, p1 = 0.6, α = 2.5%, β = 30% | ssi 0.3 0.1, alpha(.025) power(.7) non | | 520 | | artbin, pr(0.3 0.3) margin(0.1) alpha(0.05) power(0.7) wald | | 520 | |
| Non-inferiority | Binary | p0 = 0.5, p1 = 0.2, α = 2.5%, β = 10% | ssi 0.5 0.3, alpha(.025) power(.9) non | | 118 | | artbin, pr(0.5 0.5) margin(0.3) alpha(0.05) power(0.9) wald | | 118 | |
| Non-inferiority | Binary | p0 = 0.4, p1 = 0.35, α = 5%, β = 20% | ssi 0.6 0.05, alpha(.05) power(.8) non | | 2376 | | artbin, pr(0.6 0.6) margin(0.05) alpha(0.1) power(0.8) wald | | 2376 | |
| Non-inferiority | Binary | p0 = 0.2, p1 = 0.1, α = 2.5%, β = 30% | ssi 0.8 0.1, alpha(.025) power(.7) non | | 396 | | artbin, pr(0.8 0.8) margin(0.1) alpha(0.05) power(0.7) wald | | 396 | |

*Original testing 3: Verification (power)*

| **Trial** | **Outcome** | **Reference** | **Parameters (α one-sided unless otherwise stated)** | | **Reference power** | | **artbin code** | **artbin result**  **power** |
| --- | --- | --- | --- | --- | --- | --- | --- | --- |
| Non-inferiority | Binary | Blackwelder 1982, Table 3 | | p = 90%, d = 20%, α = 5%, 2n = 78 | 0.9 | artbin, pr(0.1 0.1) margin(0.2) alpha(0.1) n(78) wald | | 0.903 |
| Non-inferiority | Binary | Julious 2011, Table 4 | | p = 70%, d = 5%, α = 2.5%, 2n = 3532 | 0.9 | artbin, pr(0.3 0.3) margin(0.05) alpha(0.05) n(3532) wald | | 0.9 |
| Non-inferiority | Binary | Pocock 2003 | | p = 85%, d = 15%, α\* = 5%, 2n = 240 | 0.9 | artbin, pr(0.15 0.15) margin(0.15) alpha(0.05) n(240) wald | | 0.902 |
| Non-inferiority | Binary | Sealed envelope calculator | | p = 80%, d = 10%, α = 10%, 2n = 290 | 0.8 | artbin, pr(0.2 0.2) margin(0.1) alpha(0.2) n(290) wald | | 0.802 |
| Non-inferiority | Binary | Julious 2011, Table 4 | | p = 90%, d = 5%, α = 2.5%, 2n = 1514 | 0.9 | artbin, pr(0.1 0.1) margin(0.05) alpha(0.05) n(1514) wald | | 0.9 |
| Non-inferiority | Binary | Julious 2011, Table 4 | | p = 75%, d = 20%, α = 2.5%, 2n = 198 | 0.9 | artbin, pr(0.25 0.25) margin(0.2) alpha(0.05) n(198) wald | | 0.901 |
| Non-inferiority | Binary | Julious 2011, Table 4 | | p = 80%, d = 15%, α = 2.5%, 2n = 300 | 0.9 | artbin, pr(0.2 0.2) margin(0.15) alpha(0.05) n(300) wald | | 0.901 |
| Non-inferiority | Binary | Julious 2011, Table 4 | | p = 85%, d = 5%, α = 2.5%, 2n = 2144 | 0.9 | artbin, pr(0.15 0.15) margin(0.05) alpha(0.05) n(2144) wald | | 0.9 |
| Superiority | Binary | Pocock 1983 | | p 1 = 90%, p 2 = 95%, α\* = 5%, 2n = 1156 | 0.9 | artbin, pr(0.05 0.1) alpha(0.05) n(1156) wald | | 0.897 |
| Superiority | Binary | Sealed envelope calculator | | p 1 = 90%, p 2 = 80%, α = 10%, 2n = 310 | 0.8 | artbin, pr(0.1 0.2) alpha(0.1) n(310) wald | | 0.801 |

Action 3: Validating the program art2bin

Original work

The program art2bin.ado underwent verification using published paper lists of sample size calculations by EMZ, for further details please see below. The paper used was Julious 2011 (Table 4) which lists sample sizes for various p0, p1 and deltas. Please note that art2bin.ado provides the *total* sample size (2n), whereas the paper provides the sample size per treatment arm (n). The verification testing file is called artbin\_testing\_*[x]*.do where *x = 1,2,3,4.*

In addition, the sample size result from art2bin.ado was compared to that given by niss (written by Patrick Phillips).

*Original testing 1: Verification (sample size)*

| **Trial** | **Outcome** | **Reference** | **Parameters (α one-sided unless otherwise stated)** | | **Reference n** | | **art2bin code** | **art2bin result /2**  **2n/2** |
| --- | --- | --- | --- | --- | --- | --- | --- | --- |
| Non-inferiority | Binary | Julious 2011,  Table 4 | | p0 = 0.7, p1 = 0.9, d=0.2, α = 2.5%, β = 10% | 20 | artbin, pr(0.3 0.1) margin(0.2) alpha(0.025) onesided(1) power(0.9) wald alg | | 20 |
| Non-inferiority | Binary | Julious 2011,  Table 4 | | p0 = 0.75, p1 = 0.85, d=0.1, α = 2.5%, β = 10% | 83 | artbin, pr(0.25 0.15) margin(0.1) alpha(0.025) onesided(1) power(0.9) wald alg | | 83 |
| Non-inferiority | Binary | Julious 2011,  Table 4 | | p0 = 0.8, p1 = 0.7, d=0.15, α = 2.5%, β = 10% | 1556 | artbin, pr(0.2 0.3) margin(0.15) alpha(0.025) onesided(1) power(0.9) wald alg | | 1556 |
| Non-inferiority | Binary | Julious 2011,  Table 4 | | p0 = 0.85, p1 = 0.8, d=0.1, α = 2.5%, β = 10% | 1209 | artbin, pr(0.15 0.2) margin(0.1) alpha(0.025) onesided(1) power(0.9) wald alg | | 1209 |
| Non-inferiority | Binary | Julious 2011,  Table 4 | | p0 = 0.9, p1 = 0.9, d=0.05, α = 2.5%, β = 10% | 757 | artbin, pr(0.1 0.1) margin(0.05) alpha(0.025) onesided(1) power(0.9) wald alg | | 757 |
| Non-inferiority | Binary | Julious 2011,  Table 4 | | p0 = 0.7, p1 = 0.75, d=0.15, α = 2.5%, β = 10% | 105 | artbin, pr(0.3 0.25) margin(0.15) alpha(0.025) onesided(1) power(0.9) wald alg | | 105 |
| Non-inferiority | Binary | Julious 2011,  Table 4 | | p0 = 0.75, p1 = 0.75, d=0.2, α = 2.5%, β = 10% | 99 | artbin, pr(0.25 0.25) margin(0.2) alpha(0.025) onesided(1) power(0.9) wald alg | | 99 |
| Non-inferiority | Binary | Julious 2011,  Table 4 | | p0 = 0.8, p1 = 0.9, d=0.05, α = 2.5%, β = 10% | 117 | artbin, pr(0.2 0.1) margin(0.05) alpha(0.025) onesided(1) power(0.9) wald alg | | 117 |
| Non-inferiority | Binary | Julious 2011,  Table 4 | | p0 = 0.85, p1 = 0.85, d=0.1, α = 2.5%, β = 10% | 268 | artbin, pr(0.15 0.15) margin(0.1) alpha(0.025) onesided(1) power(0.9) wald alg | | 268 |
| Non-inferiority | Binary | Julious 2011,  Table 4 | | p0 = 0.9, p1 = 0.85, d=0.1, α = 2.5%, β = 10% | 915 | artbin, pr(0.1 0.15) margin(0.1) alpha(0.025) onesided(1) power(0.9) wald alg | | 915 |

*Original testing 2: Comparing sample size from art2bin to that of niss*

| **Trial** | **Outcome** | **Parameters (α one-sided unless otherwise stated)** | | **niss code** | | **niss**  **2n** | | **art2bin code** | | **art2bin result**  **2n** | |
| --- | --- | --- | --- | --- | --- | --- | --- | --- | --- | --- | --- |
| Non-inferiority | Binary | p0 = 0.7, p1 = 0.9, d=0.2, α = 2.5%, β = 10% | niss 0.7 0.9 0.2, alpha(0.025) power(0.9) aratio(1) | | 40 | | artbin, pr(0.3 0.1) margin(0.2) alpha(0.025) onesided power(0.9) wald alg | | 40 | |
| Non-inferiority | Binary | p0 = 0.75, p1 = 0.85, d=0.1, α = 2.5%, β = 10% | niss 0.75 0.85 0.1, alpha(0.025) power(0.9) aratio(1) | | 166 | | artbin, pr(0.25 0.15) margin(0.1) alpha(0.025) onesided power(0.9) wald alg | | 166 | |
| Non-inferiority | Binary | p0 = 0.8, p1 = 0.7, d=0.15, α = 5%, β = 10% | niss 0.8 0.7 0.15, alpha(0.05) power(0.9) aratio(1) | | 2536 | | artbin, pr(0.2 0.3) margin(0.15) alpha(0.05) onesided power(0.9) wald alg | | 2536 | |
| Non-inferiority | Binary | p0 = 0.85, p1 = 0.8, d=0.1, α = 2.5%, β = 10% | niss 0.85 0.8 0.1, alpha(0.025) power(0.9) aratio(1) | | 2418 | | artbin, pr(0.15 0.2) margin(0.1) alpha(0.025) onesided power(0.9) wald alg | | 2418 | |
| Non-inferiority | Binary | p0 = 0.9, p1 = 0.9, d=0.05, α = 5%, β = 10% | niss 0.9 0.9 0.05, alpha(0.05) power(0.9) aratio(1) | | 1234 | | artbin, pr(0.1 0.1) margin(0.05) alpha(0.05) onesided power(0.9) wald alg | | 1234 | |
| Non-inferiority | Binary | p0 = 0.7, p1 = 0.75, d=0.15, α = 2.5%, β = 10% | niss 0.7 0.75 0.15, alpha(0.025) power(0.9) aratio(1) | | 210 | | artbin, pr(0.3 0.25) margin(0.15) alpha(0.025) onesided power(0.9) wald alg | | 210 | |
| Non-inferiority, unequal allocation ratios | Binary | p0 = 0.7, p1 = 0.9, d=0.2, α = 2.5%, β = 10%, allocation ratio 1:2 | niss 0.7 0.9 0.2, alpha(0.025) power(0.9) aratio(2) | | 51 | | artbin, pr(0.3 0.1) margin(0.2) alpha(0.025) onesided power(0.9) aratio(2) wald alg | | 51 | |
| Non-inferiority, unequal allocation ratios | Binary | p0 = 0.75, p1 = 0.85, d=0.1, α = 2.5%, β = 10%, allocation ratio 1:3 | niss 0.75 0.85 0.1, alpha(0.025) power(0.9) aratio(3) | | 243 | | artbin, pr(0.25 0.15) margin(0.1) alpha(0.025) onesided power(0.9) aratio(3) wald alg | | 243 | |
| Non-inferiority, unequal allocation ratios | Binary | p0 = 0.8, p1 = 0.7, d=0.15, α = 5%, β = 10%, allocation ratio 1:4 | niss 0.8 0.7 0.15, alpha(0.05) power(0.9) aratio(4) | | 3640 | | artbin, pr(0.2 0.3) margin(0.15) alpha(0.05) onesided power(0.9) aratio(4) wald alg | | 3640 | |
| Non-inferiority, unequal allocation ratios | Binary | p0 = 0.85, p1 = 0.8, d=0.1, α = 2.5%, β = 10%, allocation ratio 1:2 | niss 0.85 0.8 0.1, alpha(0.025) power(0.9) aratio(2) | | 2618 | | artbin, pr(0.15 0.2) margin(0.1) alpha(0.025) onesided power(0.9) aratio(2) wald alg | | 2618 | |
| Non-inferiority, unequal allocation ratios | Binary | p0 = 0.9, p1 = 0.9, d=0.05, α = 5%, β = 10%, allocation ratio 1:4 | niss 0.9 0.9 0.05, alpha(0.05) power(0.9) aratio(4) | | 1928 | | artbin, pr(0.1 0.1) margin(0.05) alpha(0.05) onesided power(0.9) aratio(4) wald alg | | 1928 | |
| Non-inferiority, unequal allocation ratios | Binary | p0 = 0.7, p1 = 0.75, d=0.15, α = 2.5%, β = 10%, allocation ratio 1:3 | niss 0.7 0.75 0.15, alpha(0.025) power(0.9) aratio(3) | | 287 | | artbin, pr(0.3 0.25) margin(0.15) alpha(0.025) onesided power(0.9) aratio(3) wald alg | | 287 | |

**Or*iginal testing 3: Comparing sample size from art2bin continuity corrected superiority trials to that of power***

| **Trial** | **Outcome** | **Parameters (α two-sided unless otherwise stated)** | | **power code** | | **power**  **n** | | **art2bin code** | | **art2bin result**  **2n/2** | |
| --- | --- | --- | --- | --- | --- | --- | --- | --- | --- | --- | --- |
| Superiority, continuity corrected | Binary | p0 = 0.95, p1 = 0.9, α = 5%, β = 10% | power twoproportions 0.05 0.1, alpha(0.05) power(0.9) continuity | | 621 | | artbin, pr(0.05 0.1) margin(0) alpha(0.05) power(0.9) ccorrect alg | | 621 | |
| Superiority, continuity corrected | Binary | p0 = 0.97, p1 = 0.93, α = 5%, β = 5% | power twoproportions 0.03 0.07, alpha(0.05) power(0.95) continuity | | 818 | | artbin, pr(0.03 0.07) margin(0) alpha(0.05) power(0.95) ccorrect alg | | 818 | |
| Superiority, continuity corrected | Binary | p0 = 0.9, p1 = 0.8, α = 5%, β = 15% | power twoproportions 0.1 0.2, alpha(0.05) power(0.85) continuity | | 247 | | artbin, pr(0.1 0.2) margin(0) alpha(0.05) power(0.85) ccorrect alg | | 247 | |
| Superiority, continuity corrected | Binary | p0 = 0.9, p1 = 0.99, α = 2.5%, β = 20% | power twoproportions 0.1 0.01, alpha(0.025) power(0.80) continuity | | 143 | | artbin, pr(0.1 0.01) margin(0) alpha(0.025) power(0.8) ccorrect alg | | 143 | |
| Superiority, continuity corrected | Binary | p0 = 0.85, p1 = 0.8, α = 10%, β = 10% | power twoproportions 0.15 0.2, alpha(0.1) power(0.90) continuity | | 1027 | | artbin, pr(0.15 0.2) margin(0) alpha(0.1) power(0.90) ccorrect alg | | 1027 | |
| Superiority, continuity corrected | Binary | p0 = 0.7, p1 = 0.9, α = 5%, β = 10% | power twoproportions 0.3 0.1, alpha(0.05) power(0.90) continuity | | 92 | | artbin, pr(0.3 0.1) margin(0) alpha(0.05) power(0.90) ccorrect alg | | 92 | |

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