# REPRESENTATIVENESS OF RCT PARTICIPANTS

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## Background

Randomized controlled trials (RCTs) are viewed as the 'gold standard' in clinical research. However the external validity of these studies is not guaranteed. Within the HEADS study (Helmet therapy Assessment in Deformed Skulls) it is possible to compare the characteristics of participating infants of an RCT with those of non-participating infants.

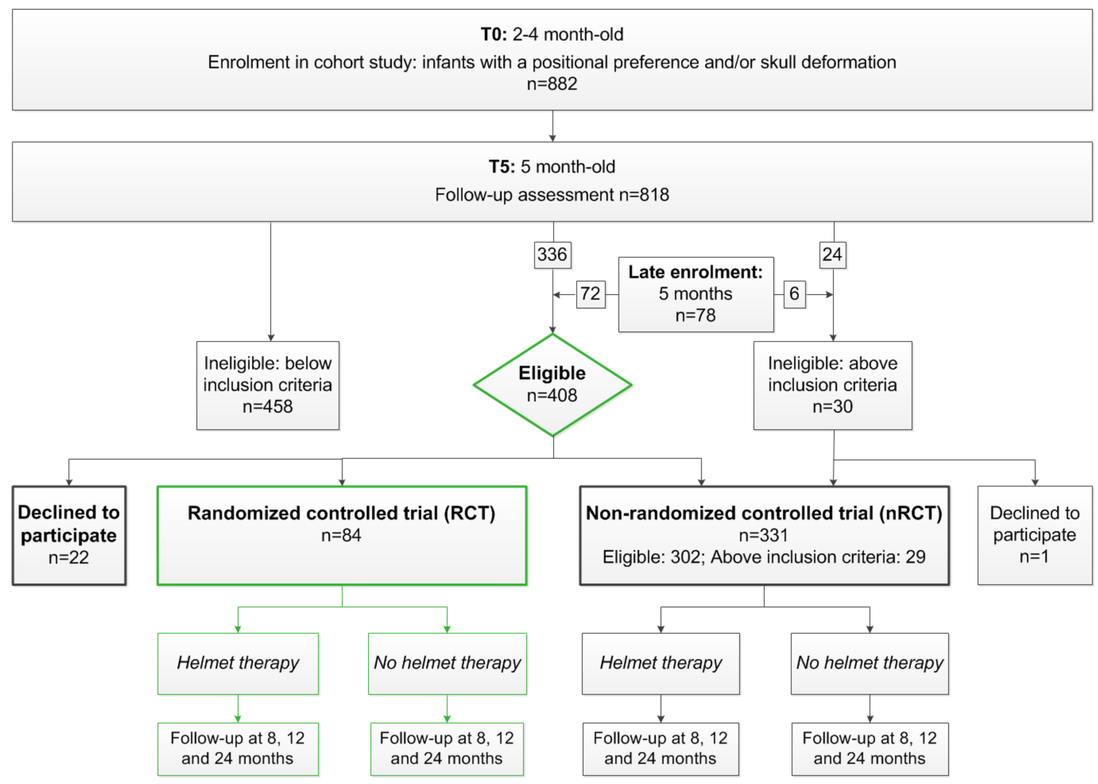
## Methods

Randomized controlled trial (RCT) nested in a cohort study, comparing the effects and costs of helmet therapy and no helmet therapy in infants with skull deformation. Parallel with the RCT, a non-randomized controlled trial (nRCT) is carried out in infants whose parents did not want to participate in the RCT. In the nRCT parents themselves made the decision to start therapy or not.

**T0:** Enrolment in cohort

**T5:** Follow-up cohort & Late-enrolment

**Eligibility criteria:** five month-old, moderate to severe skull deformation, no congenital muscular torticollis or dysmorphisms.




**Representativeness of the RCT population** was determined by comparing background and clinical characteristics of the RCT participants with those of non-participants (nRCT & Declined to participate). After univariate testing, a multivariate regression analysis was performed.

## Results

| Background characteristics (T0) | RCT-participants n=84 | Non-participants n=324 | p |
|---|---|---|---|
| Male gender | 59 (70%) | 205 (63%) | 0.32 |
| Firstborn | 39 (48%) | 161 (52%) | 0.48 |
| Age: months | 5.1 ± 0.3 | 5.2 ± 0.4 | 0.05 |
| Relevant health problems | 7 (9%) | 36 (12%) | 0.45 |
| Educational level parents | | | **0.03** |
| *Low* | 21 (28%) | 52 (17%) | |
| *Medium* | 33 (43%) | 118 (39%) | |
| *High* | 22 (29%) | 131 (44%) | |
| Anxiety level parents: STAI Trait[1] | 30 ± 10.0 | 31 ± 8.3 | 0.51 |
| Dutch ethnicity | 78 (93%) | 288 (89%) | 0.29 |
| Enrolment at T0 | 59 (70%) | 277 (86%) | **0.00** |

| Clinical characteristics (T5) | RCT-participants n=84 | Non-participants n=324 | p |
|---|---|---|---|
| Asymmetric flattening skull: ODDI[2] | 108.0 ±3.7 | 107.8 ± 3.4 | 0.38 |
| Symmetric flattening skull: CPI[3] | 91.6 ± 6.6 | 91.8 ± 6.8 | 0.85 |
| Motor development: low AIMS z-score[4] | 29 (36%) | 140 (46%) | 0.08 |
| Low Satisfaction Outcome Score - assessor | 68 (85%) | 226 (70%) | **0.01** |
| Low Satisfaction Outcome Score - parents | 63 (78%) | 206 (64%) | 0.05 |
| Parents concerned | 35 (43%) | 82 (27%) | **0.00** |

Preliminary data; presented as n (%) or mean ± SD; max 34 missing data per variable.
[1] State Trait Anxiety Inventory, range 20-80
[2] Oblique Diameter Difference Index (ODDI), minimum 100%
[3] Cranio Proportional Index (CPI), average 80%
[4] Alberta Infant Motor Scale (AIMS), low score is <-1SD

A multivariate analysis showed that only the moment of enrolment in the study (enrolment in cohort at T0 versus late-enrolment at T5) was related to participation in the RCT (enrolment at T0: Exp(B)=0.41; 95% CI=0.20-0.85; p=0.02).

## Conclusion

Participants of the RCT within the HEADS study were more likely to enrol via late-enrolment at T5. On average RCT-participants had parents with a lower level of education, received a lower assessor's satisfaction outcome score and had more concerned parents compared to non-participants. Adjusted for these variables, only the moment of enrolment was related to RCT-participation. In the final analysis of the RCT we will test whether variables that were different between RCT-participants and non-participants influence the effectiveness of the intervention.

A comparison of the effects of helmet therapy in the RCT and the nRCT will provide relevant information on the usefulness of data from non-randomized studies compared to randomized studies.

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