Unique study approval ID (Sweden): 2019-05490

Official title: Improving care for children with congenital heart disease by cardiovascular biomarker profiling and advanced non-invasive cardiac imaging techniques.

Statistical analysis plan (date): 25th of Oct. 2020

Aims:

Diagnostic test evaluations of the cardiovascular system in paediatric controls and cases of defined congenital heart disease lesions, in line with e.g. STARD guidelines, to improve the early prediction of the studied outcome (need for open heart surgery or cardiac catheter intervention in cases).

Diagnostic methods:

1. Echocardiography evaluation:

- selected echocardiographic measure (test individual measures) will be selected to predict outcome (need for open heart surgery or cardiac catheter intervention)

- selected echocardiographic measures (test group of measures) will be selected to predict outcome (as above)

2. Cardiac magnetic resonance (CMR) evaluation:

- selected CMR measure (test individual measures) will be selected to predict outcome (as above)

- selected CMR measures (test group of measures) will be selected to predict outcome (as above)

3. Cardiovascular biomarkers:

- selected cardiovascular biomarker (test individual markers) will be selected to predict outcome (as above)

- selected cardiovascular biomarkers (test groups of markers) will be selected to predict outcome (as above)

Data collection:

- estimation of sample size based on previous studies (power 0.8, alpha 0.05)

- clinical data collection according to predefined criteria (anonymised electronic database)

- electronic storage of echocardiography / cardiac magnetic resonance imaging for analysis

- storage of cardiovascular biomarker samples under standardised conditions until batch analysis

- anonymised data analysis using established statistical methods

Data analysis:

- descriptive statistics / frequency tables of all enrolled subjects (n, %, mean/SD, median/IQR).

- parametric / non-parametric tests to compare controls and cases and changes in cases over follow-up period of study

- logistic regression modelling to identify risk factors / create models that are suitable to predict outcome in cases (need for open heart surgery or cardiac catheter intervention)

- receiver operating characteristics curves (ROC) / area under the curve (AUC) analysis for proposed new diagnostic tests that may help to predict outcome in cases (see above)

- propose risk factor scoring system to predict outcome in cases (see above)