# First data of the RIKADA study: risk stratification of children and adolescents with primary cardiomyopathies

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## Introduction

Primary cardiomyopathies (CMP) and their outcome in children and adolescents are heterogeneous. Some are diagnosed prenatally; others are first diagnosed with sudden cardiac death (SCD) or acute heart failure. The aim of the RIKADA study is to give a systematic characterization of these patients and first degree family members, and develop a risk stratification which allows detecting patients with high risk for heart failure or SCD.

## Methods

We performed preliminary analyses of the first index patients enrolled into the RIKADA study between February 2014 and October 2016. The study design consists of physical examination, ECG, echocardiography, if applicable cardiopulmonary exercise testing, cardiovascular magnetic resonance (CMR) imaging, and blood analyses including molecular genetic testing.

Follow-up examinations are performed every three years in index patients, every six years in siblings (until the age of 21 years).

## Results

By October 2016, 48 patients with CMP (35% dilated CMP, DCM; 29% hypertrophic CMP, HCM; 19% left ventricular noncompaction CMP, LVNC; 10% restrictive CMP, RCM; 4% arrhythmogenic right ventricular CMP, ARVC; 2% combined HCM/LVNC) were included (Figure 1).

Median age (range) of index patients was 8.5 (0.1-18) years, 60% male, BSA 1.07 (0.2-2.2) m2. 25% showed heart failure signs. Pro brain natriuretic peptide (proBNP) was 1496.0 (6.9-78247.0) pg/ml (39/48); maximum oxygen consumption 33.5 (12.9-48.2) ml/kg/min (21/48). CMR: LV-EF 57.5 (21-88) % (22/48), LV-EDV 95.35 (50.6-227.1) ml/m² (22/48). 4 underwent heart transplantation, 2 died. Between CMP groups there were significant differences in LV-EF (p=0.0065) and LV-EDV (p=0.0201). DCM patients had significantly higher proBNP levels (p<0.001) and LV volumes (p<0.001), and lower LV-EF (p<0.001) compared to the LVNC group.

Frequencies of NYHA groups III (5/7) and IV (4/4) were higher in DCM and RCM patients. Furthermore, proBNP levels were higher in these groups (DCM: 3655 (6.9-78247.0) pg/ml; RCM: 2384 (951.6-13059.0) pg/ml; Figure 2).

## Conclusions

Morphologic and functional data significantly varied between the CMP groups, especially between DCM and LVNC. Patients with DCM and RCM patients seem to be at higher risk for severe disease courses.

Further follow-ups will enable a detailed risk stratification and may detect groups which benefit from an earlier intensive pharmacological heart failure treatment.

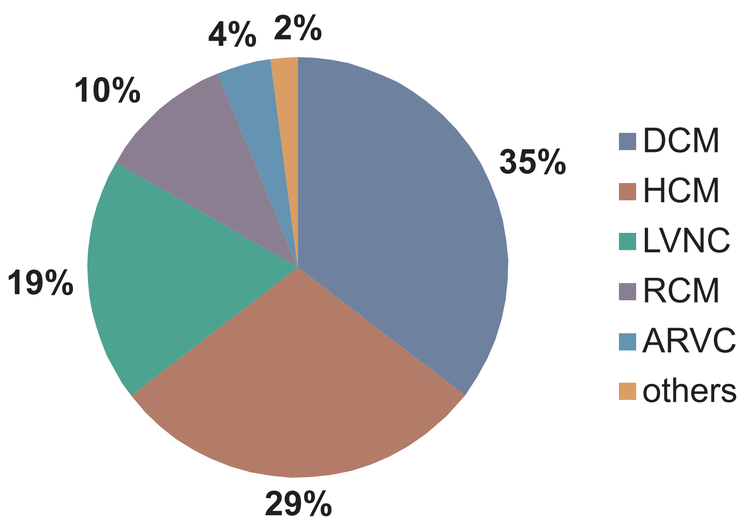


**Fig. 1:** Distribution of diagnoses in the CMP cohort

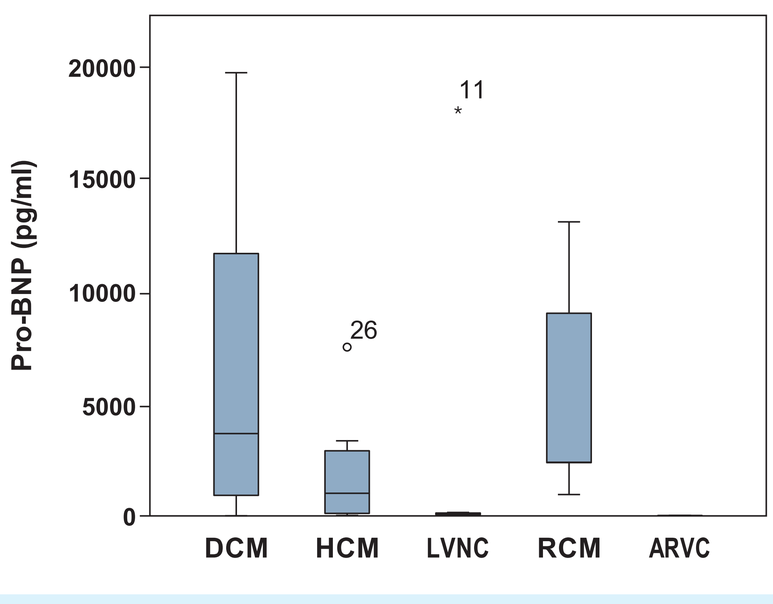


**Fig. 2:** Distribution of pro-BNP levels (pg/ml) according CMP group. DCM vs. LVNC p<0.001.

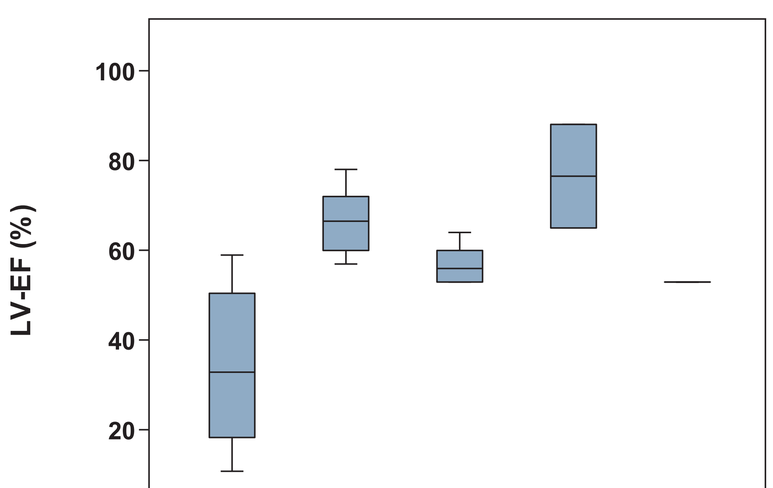


**Fig. 3:** LV-EF (%) in CMR according to CMP group (p=0.0065). DCM vs. LVNC p<0.001.

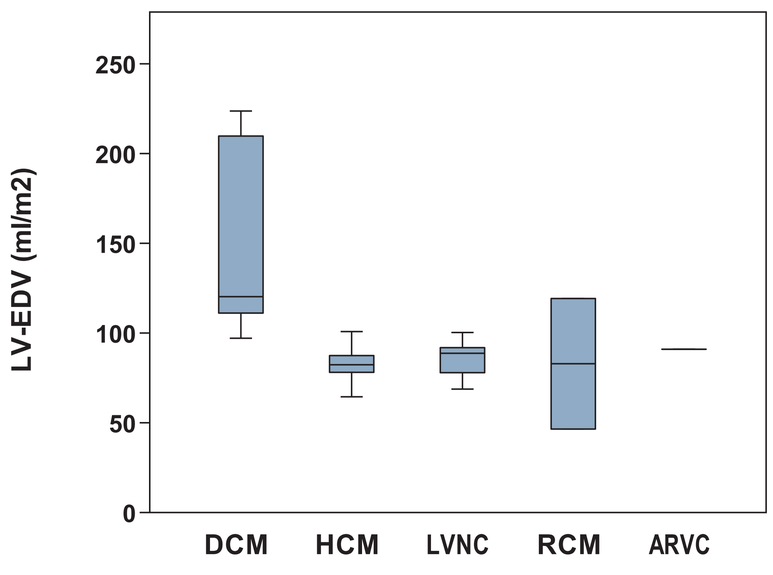


**Fig. 4:** LV-EDV (ml/m²) in CMR according to CMP group (p=0.0201). DCM vs. LVNC p<0.001.