Introduction: Dilated cardiomyopathy (DCM) is a heterogeneous group of diseases linked by a common phenotype of cardiac chamber dilation. The prognosis is varying according to the underlying aetiology; however DCM is a serious disorder and according to literature up to 50% of children die or receive a heart transplant in the first five years after diagnosis.

Design and setting: Our institutional database was retrospectively screened for patients below the age of ≤3 years diagnosed between January 01, 2006 and December 31, 2011 with DCM (n=50). An extensive initial diagnoses program identified a cause for DCM in 72% of the patients (28% idiopathic). The patients were treated based on the actual guidelines. However, keeping in mind the poor outcome of these patients, we also used individual drug therapy and compassionate therapy approaches like bone marrow derived progenitor cell therapy or pulmonary artery banding in selected cases.

Results: The median age at presentation was 4.9 months. The median follow up was 19.7 months. Kaplan-Meier analysis of survival after DCM diagnosis revealed a 1-year survival of 97% and a 5-year survival of 86%. The rate of freedom from death or transplantation was 77% at 1 year and 67% at 5 years. The patients that survived and were not heart transplanted (median follow up 34.3 months) showed a significant increase in clinical condition (NYHA classification class 3.25 +/-0.85 to 1.42 +/- 0.72), BNP (3436 ±4132pg/ml to 453+/-±965), FS (14.18 +/-6.25 to 24.61 +/-10.64) and LVEDD (3.49 +/-3.32 to 0.55 +/-2.68).

Conclusion: With the present data we can show that the rate of death or transplantation can be reduced distinctly in very young children with dilated cardiomyopathy. Our therapy was based on the actual guidelines combined with personal compassionate therapeutical approaches.