Angiotensin II type 1-receptor blocker therapy in pediatric patients with Marfan syndrome - Update after six years of experience with particular regard to tolerability.

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Introduction: Marfan syndrome MFS is an inherited connective tissue disorder in which aortic root dilatation remains the significant indicator for morbidity and mortality. After recent publication of the pediatric multicenter study and publication of our monocentric data in 2013 concerning the effectiveness and tolerability of therapy with angiotensin II type 1-receptor blocker therapy (ARB) vs beta blocker (BB) we would like to present an actual update after six years of experience.

Methods: We identified 114 pediatric patients with confirmed MFS. Indication for medical prophylaxis was found in 54 patients. All patients were subjected to standardized diagnostic program including echocardiography, MRI and clinical examination according to revised Ghent Criteria. We examined the effectiveness of medical therapy with ARB (n=34) on the growth of the sinus valsalvae (SV) with comparison of z-scores of SV before treatment and during follow up and compared these data to patients treated with BB (n=20) only.

Results: Treatment by ARB and BB leads to significant reduction of SV dilatation (p<0.05). The deviation of SV enlargement from normal as expressed by the rate of change in z-scores was significantly reduced by a mean difference of −0.57±0.65 z-scores (p<0.05) under ARB therapy and by a mean difference of −0.42±0.55 z-scores (p<0.05) under BB therapy. The prophylactic effect of ARB and BB on aortic root dilatation is similar in both groups (p>0.05). Therapy with BB was discontinued in 20% (4/20) due to intolerance. Therapy was not discontinued in the ARB group (0/34).

Conclusions: The prophylactic effect of ARB and BB in pediatric patients with MFS is similar but tolerability of ARB is clearly superior. Furthermore the inhibition of TGF-ß signaling by ARB which is supposed to contribute to the pathogenesis of MFS has to be considered. Clinical study results to confirm this hypothesis are still missing.