International Conference on

Pediatric Pharmacology and Therapeutics

12th International Conference on

Pediatric, Perinatal and Diagnostic Pathology

July 13-14, 2018 | Toronto, Canada

Regression of neonatal Cardiac rhabdomyoma in two months through low-dose Everolimus therapy

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Cardiac Rhabdomyoma (CR) is the most common cardiac tumor in newborns. Approximately 75% of cases are associated with tuberous sclerosis complex. Although these tumors usually spontaneously regress after 2 years of age, they can be life-threatening when they obstruct major cardiac inflow or outflow pathways. Everolimus is an inhibitor of mammalian target of rapamycin, reducing its production of the proteins harmartin and tuberin. Everolimus has demonstrated a remarkable suppression effect in children with tuberous sclerosis complex at doses of 4.7–5.6 mg/M2/day and serum trough levels of 5–15 ng/mL. Since 2012, five case reports of neonates with CR have also reported the tumor-regressing effect of everolimus. However, the optimal dosage for neonates is still unknown. Over the past 2 years, we have deliberately used a low dose everolimus regimen (0.3–0.67 mg/ M2/ day) in three neonates with large CRs, in an effort to maintain serum trough levels at 3–7 ng/mL. In all three cases, the tumors regressed smoothly within 2 months. Regarding the drug's side effect of predisposing patients to infection, we observed that adenovirus pneumonia occurred in one case at 3 months of age, and chicken pox occurred in another case at 9 months of age; both recovered smoothly. Our three cases of neonatal CR demonstrate that a low-dose everolimus regimen is an effective treatment for tumor regression.

Biography

As a director of pediatric cardiology and pediatric intensive care unit, Jeng-Sheng Chang is specialized in diagnosis, management and follow-up of the congenital and acquired heart diseases in children. His team is team also integrated other subspeciality teams, including cardiovascular surgeon, anesthesiology, Pharmacists, respiration therapist, nurse practitioners and nutrionist, etc., for combining care of patients with critical disease his patient-centered projects for the critical heart disease children have won the grands for quality improvement, Taiwan Institute of Health and Welfare, 2015-2016 two successive years. The open heart surgery for congenital heart disease, interventional cardiac catheterization, Extracorporeal Membranous oxygenator(ECMO) therapy, and Kawasaki disease, both in clinical management and researches, are among our best works. His researches in Kawasaki disease, collaborative with the bench team in Central Research Institute, have published important papers in the Journals of Nature Genetics and Circulation Research. A biomarker for early detection of Kawasaki disease has also been put on the markets of Asian countries.

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