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Tacrolimus significantly improves symptoms in children with myasthenia gravis refractory to prednisone

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Myasthenia gravis is a common autoimmune neurologic disease in China. More than half of myasthenia gravis cases are children (or juvenile) who predominantly manifested isolated ocular weakness. However, pyridostigmine and oral prednisone could improve the symptoms to some degree; the children will display multiple side effects such as Cushing reaction and retarded stature. In attempt to overcome the flaws of prednisone therapy and to obtain more stabilized improvement of myasthenia gravis symptoms, we aimed to test the efficacy and safety of tacrolimus, a novel immunosuppressant, in a cohort of myasthenia gravis children. Fourteen children with myasthenia gravis (aged more than 5 and 14 years) who had not achieved satisfactory improvement or/and suffered from severe side effects of oral prednisone were recruited between January 2014 and May 2015 at Tongji Hospital. Only one child withdrew from the study because he complained of slight dizziness and nausea, and the remaining 13 children completed the therapy for 1 year. After the treatment with tacrolimus (1-2 mg per day), doses of prednisone were significantly decreased, the myasthenia gravis symptoms, evaluated by quantitative myasthenia gravis score, myasthenia gravis-specific manual muscle testing and myasthenia gravis-activities of daily living score in the children were significantly improved at weeks 12 and thereafter. In addition, 10 (76.9%) patients had completely stopped using prednisone, accordingly, the major side effects were reversed. The titer of acetylcholine autoantibodies significantly dropped from 1.96 ± 2.62 nmol/L to 0.70 ± 1.04 nmol/L ($P < 0.05$). During the follow-up, no severe adverse events were reported. Our result provided a novel therapy for children with myasthenia gravis who did not respond well to oral prednisone or/and had major side effects of oral prednisone. Tacrolimus is safe in children and is able to stabilizing improve the isolated ocular weakness of children with myasthenia gravis.

Biography

Mengcui Gui has her expertise in Neuroimmunology. She along with her team has already established clinical data and biological information collecting system on myasthenia gravis, multiple sclerosis, myolitis, etc. Also, she has built the animal model after years of experience in research, evaluation and administration both in hospital and education institutions.

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