Growth hormone therapy in children with Prader-Willi syndrome

To the Editor:

Recently, Carrel et al[1] reported that growth hormone (GH) treatment for 12 months improved mobility skill acquisition, especially motor abilities and speech, in infants and toddlers affected by Prader-Willi syndrome (PWS) only when treatment started before 18 months of age. We report on the results obtained in 4 boys with PWS, 4.5 to 9.0 years of age, after GH therapy for 24 months (1 mg/m^2 per day, all days) on the intelligence quotient, examined by the WPPSI or Wechsler Intelligence Scale for Children-R tests,[2] and the neurological performances, examined by the Touwen test.[3]

Intelligence quotient did not show any improvement: At baseline, the scores ranged from 0.47 to 0.70 (moderate impairment); after 24 months, they ranged from 0.40 to 0.81.

At baseline, posture, dyskinesia, and visual system explored by the Touwen test were normal in all children, whereas the remaining 7 skills were variably impaired. The balance of trunk, coordination of extremities, fine manipulative ability, gross motor functions, and quality of motility were impaired in all children; sensorimotor apparatus was impaired in 2; and associated movements were impaired in 1. After treatment, a significant improvement occurred in 6 of the 7 skills; for balance of trunk, 2 of 4 had normalization; coordination of extremities, 4 of 4 (normalization in 3); fine manipulative ability, 1 of 4; gross motor function, 4 of 4 (normalization in 2); quality of motility, 3 of 4; sensorimotor apparatus, 2 of 2 (normalization). No improvement was observed in associated movements.

It is possible that a more prolonged therapy than that used by Carrell et al might explain the beneficial effects of GH on the neurological motor dysfunction in these young patients with PWS.

Patients with PWS after GH therapy for 24 months can show improvement in several motor functions, even if treatment started at school age.

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Reply

To the Editor:

The thoughtful response of Franzese et al prompts a clarification that is critical to interpreting the results of our 2004 article in the context of the findings of our earlier work and that of others. Although the 12-month data from our ongoing longitudinal study revealed that GH treatment before age 18 months improved mobility skill acquisition, this conclusion was not exclusive of or in contradiction to our previous findings that GH therapy improved the strength and agility skills in older children with PWS.[1]

We applaud the pilot work of Franzese et al for their interest in examining the efficacy of a 24-month protocol for GH treatment. They used Touwen's Test for Children with Minor Neurological Dysfunction to evaluate the neurologic outcome of the 4 boys that they followed during GH treatment. Of concern, however, are studies critiquing the lack of sufficient construct, content, and criterion validity for Touwen's test.[2,3] The problem that challenges us and unites us with Franzese et al is to identify a mechanism of action for GH therapy as it relates to motor outcome. We hold that this will be best facilitated by randomized, controlled studies with sufficient numbers of subjects and psychometrically sound instruments to address the complexity of the motor issues in this population.

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