Liver transplantation in severe methylmalonic acidemia: the sooner, the better

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We read with interest the article by Niemi et al. and the editorial by Sloan et al. on liver or combined liver-kidney transplantation in methylmalonic acidemia (MMA). The implications of the encouraging experience by these transplant approaches reported in the former piece are deeply analyzed and correctly commented in the latter. Main Authors' statements are that liver or liver-kidney transplantation in MMA, although not curative, results in excellent long-term survival and stabilization of neurocognitive picture. Open questions concern three issues: 1) who should be transplanted; 2) when transplantation should be performed; 3) which kind of approach (isolated liver or combined liver-kidney trasnsplantation) should be proposed.

Concomitantly with the publication of these interesting papers, we independently published our experience in liver transplantation for neonatal-onset, cobalamin-unresponsive MMA, providing arguments for answering the abovementioned questions. First, given the awful natural course of the disease in spite of scrupulous medical management, we are persuaded that the organ transplant approach should be considered in all patients with neonatal-onset, cobalamin-unresponsive MMA. In our series liver transplantation, indeed, allowed an excellent clinical course both in the perioperative and in the long-term follow-up, consistent with Niemi and Colleagues' results. Second, we are convinced that transplantation should be performed early, ideally within the first year of life, so as to avoid repeated metabolic decompensation and consequent neurological deterioration. Third, we believe that isolated liver transplantation should be the therapeutic option in MMA patients, providing the greatest mutase enzyme activity (5-fold greater than kidney transplantation), sufficient for preserving renal function and avoiding or (at least) delaying kidney transplantation.

On the light of these independent reports, early liver transplantation currently appears to be the best therapeutic option in patients with severe MMA. Although not fully curative, this approach will certainly ensure healthier clinical outcome with respect to traditional medical management.