Recommendations for the evaluation of investigational agents in pediatric MS

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1. Exposure of pediatric MS patients to new therapeutic agents should occur in the context of carefully designed clinical trials. Off-label use of emerging therapies is generally discouraged, with the hope that pediatric MS patients may be enrolled in well-controlled, robust clinical trials evaluating appropriate agents.
2. New and emerging therapies of high potency and potentially serious or life-threatening toxicity, should have a reasonable period of post-hoc safety information from adults before consideration for study in the pediatric age group. Re-examination for pediatric indications emphasizing safety and tolerability in this age group is then reasonable.
3. For new and emerging therapies with proven efficacy demonstrated by phase III trials in adults and favorable side effect profiles, appropriate studies in pediatric MS should be conducted to evaluate safety and efficacy.
4. Pediatric MS studies could be developed in tandem with phase III adult RRMS trials only if available safety data demonstrates a favorable risk/benefit ratio, information exists on drugs with the same or similar mechanism of action or if sufficient safety data in children treated with the drug for other disorders already exists.
5. Placebo-controlled trials in pediatric MS should be of brief duration and should have rigorous monitoring to ensure a rescue strategy for children in the placebo arm who experience rapid accrual of physical, cognitive or MRI burden of disease.
6. In situations where use of a specific therapy is restricted to very small patient populations (such as patients with severe or refractory MS), a clinical trial may not be feasible. In this situation, a prospective registry of all treated patients should be employed to capture both short-term and long-term safety and tolerability.
7. All pediatric MS clinical trials should include a long-term prospective registry to capture information about long-term safety and development and fertility parameters.