Reader Response: Clinical trials of disease-modifying agents in pediatric MS: Opportunities, challenges, and recommendations from the IPMSSG

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Waubant et al. criticized placebo-controlled trials in pediatric multiple sclerosis (MS),[1] but failed to address a core challenge. The terms "children" and "pediatrics" have 2 connotative considerations: legal/administrative and physiological. The American Academy of Pediatrics (AAP) defines pediatrics as patients <21-years-old; older with special needs.[2] This is adequate to define administratively which patients pediatricians can/should treat, but inadequate as inclusion criterion for efficacy trials.[3] MS and depression can emerge when the CNS reaches some maturity; epilepsy occurs at any age. There is no "pediatric" MS or depression. Both diseases begin sometimes in minors, but do not change fundamentally at Food and Drug Administration (FDA)/European Medicines Agency (EMA)-defined birthdays (17 years and 18 years, respectively).[4] Separate pediatric drug development in non-neonatals emerged with the acceptance of the children-are-therapeutic-orphans concept by the AAP and FDA.[5] The United States' rewards for separate "pediatric" studies created conflicts of interest in regulatory authorities, academia, and pharmaceutical industry.[3] Today, the FDA accepts adolescents in adult cancer studies and has partially relented to demands for separate "pediatric" studies in dermatology and epilepsy, while the EMA remains unfortunately adamant.[4] Separate "pediatric" efficacy studies in minors with mature bodies are unethical. This challenge goes beyond individual clinical disciplines; but, without clinicians critically addressing it, it will never be resolved.

Disclosure

The authors report no relevant disclosures. Contact journal@neurology.org for full disclosures.

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