CHILD NEUROLOGY SOCIETY From the President



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Clinical Trials for Neurological Diseases – The NEXT Generation

Three decades ago, most of the children with acute lymphoblastic leukemia achieved initial disease remission after chemotherapy, only to later relapse and die. In a stunning reversal, about 95% of the children with this form of leukemia now survive. During this time we have also made major progress in the treatment of many other forms of childhood cancer, including tumors affecting the brain. How did this transformation come about, and are there lessons that might be applied to other serious diseases of children?

The development of new chemotherapeutic agents and more effective drug combinations and dosing undoubtedly improved cancer treatment results in children. But it was the advent of collaborative clinical trial groups for childhood cancer that allowed new drugs and new drug combinations to be efficiently evaluated. The National Cancer Institutesupported Children's Oncology Group now includes some 8,000 childhood cancer experts at over 200 children's hospitals. Over 90% of the children and adolescents with cancer in the United States are treated at one of these facilities, and more than 60% of children with cancer are enrolled in one or more clinical trials. In contrast, only a small fraction of adults with cancer enter clinical trials, and progress has been less remarkable for the tumors that commonly occur in adults.

Collaboration on this scale allows even uncommon disorders to be studied more rapidly and more meaningfully. Embedding the study plan into a treatment roadmap facilitates participation and ensures a uniform treatment approach aside from the study variables. Although the accumulation of data is slower for uncommon conditions, we still learn from every enrolled patient. A similar network would also facilitate treatment trials for neurological diseases.

Several disease-specific research collaborative groups have formed in the last several years, but developing and maintaining a clinical trials network, even on a smaller scale, necessitates planning, effective infrastructure, and adequate funding. The recently created Network for Excellence in Neuroscience Clinical Trials (NeuroNEXT) program provides a mechanism to rapidly evaluate new selected techniques or treatments for neurological disorders. The program promotes interaction between multiple academic centers, foundations, and industry, and it is specifically designed to include both children and adults. NeuroNEXT has twentyfive approved centers, but investigators from other centers can be included for specific projects.

The NeuroNEXT program makes it feasible to rapidly apply new scientific discoveries and to evaluate promising therapies for neurological disorders. This is an excellent opportunity to spur the development of novel treatments, particularly those that would have languished without the benefit of an established trials network and the infrastructure to maintain it. Perhaps it will also enable us to learn more of the lessons that every patient has to teach us.