4. Lessons Learned From the NICHD Pediatric Pharmacology Units

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Approximately 80% of the prescription drugs marketed in the U.S. were approved and marketed for adult use, but have not been adequately studied in infants and children or approved by the FDA for use by children. The PPRU was funded by NICHD in 1994 as a cooperative agreement to bring together pediatricians and pharmacologists at five academic centers with the pharmaceutical industry to conduct pediatric pharmacology research. In the ensuing five years, the Network performed over 100 studies in children.

The FDA Modernization Act of 1997 (FDAMA) encouraged pharmaceutical companies to study drugs in children by providing a six-month extension of exclusivity for marketed drugs. This has been a powerful incentive for new pediatric drug trials. The impact of FDAMA on PPRU-sponsored pediatric drug trials is reflected in the increase of trials since 1998. To date, FDAMA has grant exclusivity to 22 marketed drug products that successfully completed pediatric studies. The PPRU Network performed all or part of the clinical trials to support the exclusivity determination for eight of these 22 drugs. During 1998-1999, pediatric labeling was added to six marketed products based on studies conducted under FDAMA provisions. The PPRU conducted studies to support the labeling changes of four of these six products. In 1999, 54 protocols were active and in the first six months of 2000, 55 protocols were active with a total enrollment exceeding 1,000 patients and an increase in pharmaceutical sponsors. To accommodate the increased demand, the PPRU was expanded in 1999 from 7 to 13 centers and a data center added. The PPRU mission also was expanded to include studies of bioavailability, formulations, metabolism, and efficacy of current and new drugs; age-specific labeling; new classes of drugs; pharmacogenetic and modeling studies; and education. This talk will discuss the history, organization, and accomplishments of the PPRU and the Neonatal Research Networks in conducting drug trials, as well as challenges for the future, including the current U.S. regulatory environment.

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