Pediatric regulatory science, clinical trial networks hold promise for studies

CSDD benchmarks industry experience with pediatric studies initiative

- Pediatric regulatory science and pediatric clinical trial networks are believed to hold the greatest promise for improving pediatric drug development.

- Progress is being made on BPCA and PREA goals.

- Most respondents to a Tufts CSDD survey said pediatric study complexity has increased more than 50% since 2008.

- Patient recruitment/retention and study modifications were identified as the factors most responsible for driving up pediatric study costs.

- Resources dedicated to pediatric studies have increased across most R&D functions.

Nearly 20 years ago, the United States Food and Drug Administration (FDA), R&D-based biopharmaceutical companies, pediatric health care practitioners, and child health advocates embarked on an ambitious initiative to improve the development of medicines designed to meet the unique needs of children. That effort led to new policies and a regulatory framework to foster pediatric research: the Best Pharmaceuticals for Children Act of 2002 (BPCA) and the Pediatric Research Equity Act of 2003 (PREA). Since then, hundreds of medicines originally developed for adults have been assessed for their potential utility in children. After being tested for safety and efficacy in pediatric studies, more than 600 drugs and biologicals have been labeled with specific information to inform safer administration and dosing in children.

In 2007, Tufts CSDD assessed drug developers’ experience following the first decade of these pediatric studies initiatives. Much has changed since then, including the need to conduct pediatric studies earlier in development across all age groups, with appropriate formulations, in the context of a changing and increasingly complex research environment. This report updates a previous CSDD study (see Tufts CSDD Impact Report 2007 March/April,9[2]) and assesses changes in pediatric drug development since 2008 resulting from the FDA Amendments Act of 2007 (FDAAA) and the FDA Safety and Innovation Act of 2012 (FDASIA).