Children's Hospital Association Statement for the Record

Energy and Commerce Committee Hearing, "Evaluating Approaches to Diagnostic Test Regulation and the Impact of the FDA's Proposed Rule"

March 21, 2024

On behalf of the nation's 200+ children's hospitals and the children and families we serve, thank you for holding this hearing, "Evaluating Approaches to Diagnostic Test Regulation and the Impact of the FDA's Proposed Rule." We share the goals of this committee and the FDA of protecting public health by assuring the safety and effectiveness of diagnostic tests, particularly for children with serious or complex medical needs. Therefore, we believe it is essential to understand the unique pediatric considerations of the FDA's proposed rule or any other approaches to regulating laboratory-developed tests (LDT). It is imperative that diagnostic test regulation protect children's access to life-saving tests and timely care.

Role of LDTs in Pediatric Health Care

Children are not just little adults. They are constantly growing and developing, and their health care needs and the delivery system to meet those needs are different from those of adults. Pediatric health care requires specialized medications, diagnostics, tests, therapeutics, and equipment that the nation's children's hospitals provide. LDTs fill a critical gap in the practice of pediatric medicine as they allow for accurate, timely, accessible, and high-quality testing for many pediatric conditions for which no commercial test exists or where an existing FDA-approved test does not meet current pediatric clinical needs. They are critical to children's hospitals' ability to provide timely, cost-effective, and high-quality diagnostics and care for all children, and particularly for children in need of treatment for rare and difficult-to-diagnose pediatric disorders.

LDTs developed and used in pediatric health care settings account for all stages of childhood development, from newborn through adolescence and young adulthood, and address numerous genetic and heritable diseases, pediatric cancers, and acquired conditions that are not well-represented in adult health care practice. FDA itself has recognized four subpopulations within the pediatric population – neonates, infants, children, and adolescents[1]—and the importance and challenges of developing age-appropriate treatments and diagnostics for the pediatric population. For example, tests that are effective in adolescents cannot necessarily be used on neonates without modification. Pediatric-related LDTs provide those age-appropriate diagnostic tools.

Children's Hospitals' use of LDTs

There are numerous FDA-approved tests that could potentially be used for children but are not validated for such use. Children's hospitals' clinical laboratories either develop tests from scratch that are needed by their patients or perform the extensive validation work needed to demonstrate that an FDA-approved test for adults can safely and reliably be used for children. Those in-house tests (LDTs) offer precise and accurate results and they are a critical component of lifesaving treatment plans designed for children.

[1] See, e.g., Pediatric Medical Devices, FDA.

Children's hospital laboratories offer several hundred in-house LDTs or modified FDA tests. These tests are all developed and validated following requirements specified by the Clinical Laboratory Improvement Amendments of 1988 (CLIA) in laboratories tightly regulated and further accredited under CLIA and by their states, the College of American Pathologists, or the Joint Commission—in accordance with the CLIA regulation. These existing regulatory measures ensure the quality of this testing, which is usually developed in partnership with pediatric clinical providers to meet well-defined clinical needs.

These pediatric-related LDTs include tests for diseases/diagnoses that are related to infancy or childhood; tests that are approved for adults but must be altered or modified for pediatric use; tests for pediatric rare and orphan diseases; tests that cannot be done—or are not done—by adult-focused laboratories; and tests that are run in hospitals for immediate pediatric patient care.

For example, pediatric LDTs are used when there are no FDA-approved alternatives available for time-sensitive tests to enable pediatric specialists to make immediate clinical decisions for children. These include the test used to diagnose childhood leukemia, which may be individualized within different children's hospital laboratories for the specific child. The curative treatment for children with leukemia is bone marrow or stem cell transplantation, and the genetic test used to monitor the health of the bone marrow transplant after it happens is also an LDT.

There are also numerous situations in which the instructions for use for an FDA-approved test do not include the parameters needed to use the test in the pediatric population. These include tests with instructions for use that exclude pediatric age ranges. Therefore, children's hospitals routinely develop different reference ranges to inform age-appropriate clinical decision-making. For example, the test used to determine bleeding risk in pediatric surgical patients is an LDT because there is no FDA-approved test available for use in patients under the age of 18 years.[2]

Furthermore, adult-focused laboratories often do not have the pediatric-specific instrumentation to care for children of varying ages. LDTs allow children's hospital laboratories to serve pediatric patients of all ages (newborns, infants, small children, and even older children) through the use of age-appropriate equipment—such as extremely small sample sizes and equipment such as microtainer tubes for testing low birthweight preterm newborns.

Impact of Proposed FDA Rule

We believe the proposed FDA rule on LDTs will have serious implications for children's hospitals' ability to provide timely diagnostics for the nation's children. We are particularly concerned with the disproportionate impact that the proposed rule will have on children – especially those enrolled in Medicaid—and their access to the vital testing, screening, diagnostics, and care that they need. Medicaid is the largest insurer of children in the U.S. and, on average, covers one-half of children's hospitals' patients. For some children's hospitals, closer to three-quarters or more of its patients are enrolled in Medicaid. Between one-third and one-half of Medicaid-covered children have

[2] For additional examples of pediatric-related LDTs, see CHA Comments on FDA Laboratory Developed Tests Proposed Rule.

special health care needs.[3] These children frequently face higher disease exposure risks and are more likely to depend on LDTs for their specialized pediatric health care.

It is important to note that, though children's hospitals account for only 2% of hospitals in the U.S., they account for about 45% of all hospital days for children on Medicaid. As a result of the heavy reliance on Medicaid, which often under-reimburses for costs of care, the budgets of children's hospital laboratories are tight and the financial resources and staff needed to pursue the large number of complex reviews under the proposed rule will be in addition to resources already used to meet the stringent regulatory and accreditation requirements under CLIA, the College of American Pathologists, the Joint Commission, state standards, etc.

Furthermore, as we note above, we know that the for-profit sector has not—and likely will not—step in to make tests for pediatric and orphan diseases as the market is too small to be profitable. Similar to the development of new pharmaceuticals, which are usually developed for adults, children are often left behind in the development of commercial testing, given the small market and highly specialized nature of pediatric diseases. As a result, many needed tests for children, including those with rare, uncommon and often life-threatening, diseases will no longer be available with significant negative implications for their overall health and wellbeing.

Given the critical and unique role that LDTs play in pediatric health care, we believe that any regulatory or congressional action affecting pediatric-related LDTs must continue to protect children in need of these life-saving diagnostics. Therefore, we have recommended to the FDA that its final rule continue general enforcement discretion approach for all hospital and health system LDTs, and at a minimum, enforcement discretion for pediatric-related LDTs.

Additional Considerations

While we are not opposed to congressional or regulatory action to give special consideration to LDTs developed by academic medical centers (AMCs), we remind the committee that there are pediatric-specific LDTs that are developed or modified by children's hospitals that are not affiliated with an AMC. Therefore, we believe that consideration must specifically be given to those tests that are developed to meet the specific needs of infants, children, and all those impacted by pediatric diseases—regardless of where the tests are developed.

We also believe that, at a minimum, currently marketed pediatric-related LDTs must be grandfathered in any regulatory structure to ensure that children continue to have access to the specialized clinical diagnostics and care that children's hospitals provide. In the absence of a grandfathering provision, it is likely that some, if not many, children's hospital laboratories will be unable to make the substantial administrative and financial investments that would be required to prepare submissions for FDA review for the range of LDTs currently in use. As a result, they

[3] Medicaid Access in Brief-Children and Youth with Special Health Care Needs (macpac.gov); Children with Special Health Care Needs: Coverage, Affordability, and HCBS Access | KFF.

would have to make extremely difficult decision to consider abandoning existing effective pediatric-related tests putting their child patients at risk.

However, a grandfathering provision will **not** fully ensure that children have access to the tests and health care they need. In addition to a measure to protect tests currently in use, measures must be put into place to support the ability of children's hospitals to continue to develop—and innovate with—new diagnostics that are safe and clinically effective and drive pediatric cures and treatment.

Thank you for the opportunity to highlight the unique implications of the regulation of LDTs for pediatric health care. We know this committee shares our commitment to ensuring that children continue to have access to life-saving diagnostics and timely care. We also join with the committee and the FDA in supporting appropriate regulatory oversight of LDTs in the commercial marketplace and in ensuring that all diagnostics and safe and effective. We look forward to continuing to work with you to meet the health care needs of the nation's children.