



Comments of Small Biotechnology Business Coalition¹

For the September 9, 2014

House Energy & Commerce Committee Hearing:

21st Century Cures: Examining the Regulation of Laboratory Developed Tests

Small biotechnology companies are the primary developers of innovative diagnostic tests that address unmet medical needs. We estimate that there are over 800 small companies in the U.S. developing and validating tests to aid in earlier and more definitive diagnosis and in therapeutic decision making.

On July 31, 2014, the Food and Drug Administration (FDA) notified Congress that it intends to issue Draft Guidance to regulate Laboratory Developed Tests (LDTs). A widespread concern among small diagnostics companies is that the high costs of compliance with these anticipated regulations in the face of unpredictable commercial returns would prevent them from attracting the capital needed to bring their products to market. Our companies already face general disinterest from the venture capital community as a result of very low reimbursement rates for diagnostic test coupled with the difficulty of obtaining patent protection for novel biomarkers or test as a result of two recent U.S. Supreme Decisions that considerably narrowed patent protection for this subject matter.²

FDA regulation of LDTs should not be permitted to progress unless exemptions are provided for small companies developing innovative tests that address unmet medical needs. Our organization is proposing a new regulatory mechanism for simultaneously encouraging innovation while at the same time protecting patients and delivering tests that can substantially improve disease outcomes. This concept is modeled after the FDA's Small Business Nutrition Labelling Exemption and the Humanitarian Device Exemption

¹ www.SmallBiotechCoalition.org The SBBC was founded in February 2010 to promote government policies that aid the estimated 2000+ independently owned, privately held small biotech companies. The vast majority of these companies are financed through the SBIR program and individual ("Angel") investors rather than VCs.

² [Myriad v. AMP](#) and [Mayo v. Prometheus](#)

The proposed exemption would apply to innovative tests developed by small businesses that would otherwise be deemed to be Class III diagnostics (most complex, high risk and novel intended uses) requiring premarket approval (PMA). These diagnostics—to be known as —“Small Business Developed Innovative Tests” or — “SBDIT”s—could instead be subject to a new — “provisional PMA” that would permit marketing and administration of the test to up to 8,000 U.S. patients per year or \$8 million in annual revenue.

An application for a small business provisional PMA would be similar in both form and content to a regular PMA application, but without the same amount of clinical trial data typically required for PMAs. FDA could reject only those applications that pose an unreasonable or significant risk to patients, and where the likely benefit to health is clearly outweighed by the risks, taking into account the probable risks and benefits of currently available devices or alternative testing paradigms. Additionally, the applicant must demonstrate that no comparable tests are on the market, and that they could not otherwise bear the cost of a traditional PMA. In this regard the standard would be very similar to that of a Humanitarian Device Exemption (HDE).

This exemption would help break the “Catch-22” facing most innovative small diagnostics companies who face the challenge of accessing capital to fund expensive clinical studies without conclusive evidence that their test will gain regulatory approval and marketplace acceptance.

Data derived from the first few years of marketing could be collected and analyzed becoming the equivalent of a large scale, prospective clinical study that would otherwise be prohibitively expensive for small companies. During this provisional period companies could ascertain market demand for their test and in some cases obtain reimbursement from CMS and/or private payers. This would significantly decrease uncertainty for investors permitting the company to more readily obtain funding for more research and development and product improvements.

The Humanitarian Device Exemption (HDE) was established based on the recognition that companies` research and development costs typically exceed their market returns for devices and diagnostics addressing small patient populations (under 4,000 U.S. patients per year). HDEs provide an incentive for the development of products for these diseases by eliminating the requirement of proving efficacy. The economic rationale for HDEs also supports similar incentives for small business notwithstanding the fact that the later ultimately may address substantially larger markets. Investigational Device Exemptions (IDEs) are designed for devices and diagnostics for which effectiveness data is being gathered prior to marketing. The proposed small business exception also permits the gathering of this data but in the context of a limited initial marketing campaign to early adopters.

For further information please contact:

Small Biotechnology Business Coalition
9710 Traville Gateway Drive, #325
Rockville, MD 20850
301-917-6538
policy@SmallBiotechCoalition.org