U.S. House Committee on Energy and Commerce
Subcommittee on Health

Hearing on Programs Affecting Safety and Innovation in Pediatric Therapies

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Submitted for the Record

By

Donald P. Lombardi
President and CEO
Institute for Pediatric Innovation
One Broadway - 14th Floor
Cambridge, MA 02142
Office: 1.617.401.2376
Mobile: 1.617.309.7912
Fax: 1.617.758.4101
Email: donald.lombardi@pediatricinnovation.org

Key Points

1. Standard approaches for commercializing innovations don't work for pediatrics, including the system for transferring Federally funded research results from academic and medical research institutions to the for profit sector as contemplated under the Bayh Dole Act of 1980.

2. A solution requires novel forms of public-private, nonprofit-for profit collaboration to carefully assess what products are needed and to drive innovation toward these needs.

3. Legislation must be designed to provide incentives and support for such collaborations, while avoiding unwanted consequences.

Introduction

Good Morning. My name is Donald Lombardi and I am President and CEO of the Institute for Pediatric Innovation. We are a Cambridge, Massachusetts based nonprofit organization dedicated to increasing the quality of care of children by bringing more appropriate medical devices and drugs to the clinical treatment of pediatric patients.

Shortcomings of the technology transfer system

As others have testified, the medical device industry and the pharmaceutical industry have not invested effectively in commercializing devices and drugs that have been specifically designed for care of pediatric patients. I will address how the academic technology transfer system also fails to deliver the products needed for pediatric care.

I was the Chief Intellectual Property Officer of Children's Hospital Boston, responsible for managing that organization's compliance with the Bayh Dole Act of 1980. This law gives Federal grant recipients the right and mandate to transfer intellectual property arising from Federally sponsored research to for-profit companies. The intent is that companies will develop the products and make them commercially available, thereby benefiting the public. Companies licensed by Children's Hospital brought numerous products into clinical development and onto the market, resulting in tens of millions of dollars of revenue. However, very little of this commercial success resulted in new technology for the care to pediatric patients. My experience at Children's Hospital Boston is mirrored in the experience of technology transfer officers at all major pediatric institutions. Nearly all of the successful products arising from these institutions were focused on the clinical care of adults, because companies and investors who licensed the technologies were motivated to apply them for the largest and easiest-to-access adult markets.

Need for novel collaborations between public, private, nonprofit and for-profit organizations

We carried out an assessment of why even premiere pediatric institutions proved largely unable to cause products to be developed for treating children. We found that the traditional technology transfer

process focuses primarily on new basic biology discoveries made by research scientists. Most of this research lacks commercial proof-of-principle and requires significant investment even before testing in humans. Further, the program did not engage clinicians – i.e., doctors, nurses, and other patient care professionals – who know the most about the products needed for day-to-day care of patients. The scope of the problem demanded that it be approached on a national, multi-institutional basis. To address these issues, we created the Institute for Pediatric Innovation (IPI) as a nonprofit 501(c)3 organization. The IPI plan includes the following components:

1. IPI pediatric hospital consortium and need analysis. Before selecting products or technologies for development, we must understand the needs and priorities for pediatric and neonatal care. IPI has assembled a small consortium of leading pediatric care and research hospitals and is convening a board of pediatric experts to help assess the needs of the patients. The Hospitals are providing access to their clinicians and clinical operations to help identify those needs that can most impact quality of care, patient safety, and patient, parent and care-giver satisfaction. IPI will compare the information obtained from the clinicians with published market studies to fully understand the needs of the pediatric market.

2. Product selection criteria. IPI is comparing the information provided by the clinical thought leaders with needs and requirements for commercial feasibility in order to set priorities on areas for initial focus. Of the products with the greatest potential to save and improve lives, we will focus on those to which the members of the consortium can add most value in product design and clinical testing.

3. Product opportunity analyses. In its first year, IPI will select 5-7 products representing the greatest clinical need and greatest opportunity for near-term product realization:

- *Reformulated pharmaceuticals*. Existing pharmaceutical products that require different dosage or delivery forms for pediatric patient populations will be one focus. Today, physicians and nurses are forced to guess on dosage and use medication that has not been studied in the neonate or pediatric population. This opens the door for severe allergic reactions, overdosing or under dosing to treat severe infections, gastroenterology infections or urinary infections. In fact, new drug formulations often wait 2-3 years after the approval of a drug to even begin the dosing studies for pediatric and neonatal patients.

- *Re-engineered devices*. Today's physicians and nursing staff are forced to "jerry rig" products to save the lives of their small patients. Many medical products used in the adult population today, need be made not only smaller but also more flexible to avoid injury to a small body. Use of different materials, different anatomical curves and entry points and other factors need to be optimized for the pediatric patient. Alternative (FDA-approved) materials may provide better engineered products for neonate or pediatric care, optimizing the outcomes, and reducing hospital stays, potentially saving billions of dollars for the healthcare system. One day in the NICU for example, costs slightly over $40,000.
- *"Product imagination" brainstorming.* With market research and clinician input in hand, and with the support of a corporate partner interested in new products for neonatal care, IPI will conduct workshops with clinicians, design engineers, and market specialists to develop innovative product concepts for use in Neonatal Intensive Care Units. This initial project will serve as a basis for developing design practices that IPI will apply with corporate partners in other clinical areas such as pediatric orthopedics, cardiology, gastro-intestinal, neurosurgery and surgery.

4. Corporate collaboration. As a non-profit organization, IPI is only a part of the solution. In order to effect a major change in the current focus of device and pharmaceutical companies, IPI needs to collaborate with large corporations that will market the re-engineered or novel pediatric products to the hospital communities. Industry needs incentives to participate in improving pediatric care – such as grants, or additional federal tax exemptions – to offset the change their risk/reward calculus.

5. Access to government, foundation, philanthropic, investor, and corporate resources to finance product development. The consortium approach can reduce risk by identifying products that the pediatric market most needs. The IPI opportunity analyses will better qualify product opportunities. However, while many corporations are willing to contribute resources to the cause, they have not yet developed pediatric specific initiatives within their corporation, a critical goal of the success for IPI. As a nonprofit organization, IPI expects to be able to access philanthropic and government funds that do not require a return on investment to help finance the development phase of certain products.

Recommended guidelines for legislation

In seeking to help remedy the gap in availability of products optimized for pediatric care, Congress should:

- Create a reliable data source of clinical needs for which there is a demonstrable demand
- Provide the funding required to bring together clinicians, medical scientists, technologists, and businesses to direct innovation toward those needs
- Create incentives for industry and other stakeholders to collaborate in developing needed products
- Look for ways to expedite clinical testing and approval without risk to patient safety
- Avoid creating processes that add no value, create additional burdens or disincentives, or reduce the stakeholders' ability to meet clinical and scientific needs in pediatrics
- Assure that legislation meets the needs of all stakeholders including industry, FDA, and care-givers
- Remember that investments in improving children's health have the highest social return in quality adjusted life years