

# THE NEW ROLE OF ACADEMIA IN DRUG DEVELOPMENT

New Thinking, New Competencies, New Results

*Driving New Paradigms in Cancer Research*

December 2010



## **Executive Summary**

A recent town hall meeting offered an opportunity to explore how government, nonprofit organizations, and academic institutions can define new models of working with the private sector to enhance drug development efforts and bring safer, more effective drugs to the market more efficiently. While the challenge to innovative drug development can be great, our investments in biomedical research are providing promising opportunities to capitalize on emerging science. The following recommendations are based on a series of expert panel discussions, and can ensure that the promise of scientific research translates into reality, benefiting the health of the nation.

### **ACADEMIC INSTITUTIONS**

- Universities, in collaboration with industry, should establish models for intellectual property (IP) and technology transfer processes that will become widely adapted and trusted, removing many of today's hurdles to licensing and other opportunities to commercialize early innovation to translational outcomes.
- Universities should develop translational research training programs so current students, who will be future scientists, will be trained in next-generation techniques, applications, project management, collaboration models, and regulatory science.
- University science leaders must identify areas in which external expertise is needed from nonprofits, industry, and government, and establish a focus on entrepreneurship.
- Universities must support new models from the top (Chancellor level) down.
- Research institutions and commercial entities should establish policies to enable pre-competitive and timely sharing of critical data that will aid target development and future drug discoveries.

### **CONGRESS**

- Congress should fully fund the Cures Acceleration Network (CAN).
- The federal government should empower the Food and Drug Administration (FDA) with the scientific capabilities and resources to conduct robust review and approval processes that ensure a thorough evaluation of the risks and benefits of new therapies.

### **FOOD DRUG & ADMINISTRATION (FDA)**

- The FDA should define necessary parameters that take into consideration the differences for development of therapies for rare and neglected diseases.

The FDA should define new regulatory paths that accommodate the shifts in translational science, including emerging ideas associated with the incorporation of biomarkers, nanotechnology, personalized medicine, and informatics.

### **NATIONAL INSTITUTES OF HEALTH (NIH)**

- The National Institutes of Health (NIH) should invest in additional clinical and translational science awards (CTSAs) across the country and fund CTSA centers that bring unique capabilities and translational research and academic commercialization contributions to the consortium.
- Federal agencies that fund translational research programs also should provide or require education about commercialization, including opportunities, challenges, and regulatory mandates.

### **STAKEHOLDERS**

- All stakeholders (academia, industry, government, and disease philanthropy) in collaboration should develop streamlined, standardized clinical trial processes.
- Organizations with a vested interest in commercializing new therapies for patients should promote “team science” incentives for translational research.

# The New Role of Academia in Drug Development

## New Thinking, New Competencies, New Results *Driving New Paradigms in Cancer Research*

### Introduction

For decades, scientific research leading to new drugs and diseases treatments has substantially improved Americans' quality of life and catalyzed economic growth. In the United States, from 1975 to 2002, survival rates for certain cancers rose from 50 percent to 68 percent.<sup>1</sup> Meanwhile, declining mortality from 1970 to 2000 is estimated to have added more than \$3 trillion a year to U.S. economic activity.<sup>2</sup>

However, despite a significant increase over time of public and private investment in biomedical research, the rate at which new treatments are developed appears to be stagnant. Roughly the same number of drugs was approved by the U.S. Food and Drug Administration (FDA) in 2008 as were approved in 1950. At the same time, the cost of funding a breakthrough drug is rising by 13.4 percent annually.<sup>3</sup> As new drugs become more expensive to produce, research dollars have been shifted to research that holds the most potential for blockbuster market returns. Many argue that this has resulted in replicative and risk-averse research, rather than bold innovation.

On July 6, 2010, the Council for American Medical Innovation, Friends of Cancer Research, Kansas Bioscience Authority, the Ewing Marion Kauffman Foundation, and The University of Kansas Cancer Center co-sponsored a town hall meeting in Kansas City, Missouri. Gathered on this day in the heartland of the United States were thought leaders from across the nation—academia, government, industry, and nonprofit patient organizations—all committed to the single shared goal of accelerating the human-health return on our nation's investment in biomedical research. A rare event, this town hall produced a wide range of observations on how the fragile intersection between all of these key contributors to the nation's innovation ecosystem can be bolstered and sustained.

The town hall's theme, *New Thinking, New Competencies, and New Results*, spoke to the need for leaders in government, academia, industry, and venture philanthropy to go beyond traditional collaborations and pathways to drug development and disease treatment. Leaders from each of these fields shared their perspectives. To understand

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<sup>1</sup> The Council for American Medical Innovation (CAMI), *Gone Tomorrow: A Call to Promote Medical Innovation, Create Jobs, and Find Cures in America*, prepared by the Battelle Technology Partnership Practice, June 10, 2010, citing National Cancer Institute, Surveillance Epidemiology and End Results, <http://seer.cancer.gov/faststats/index.php>, accessed 27 May 2010.

<sup>2</sup> CAMI, *Gone Tomorrow*, citing Kevin M. Murphy and Robert H. Topel, "The Value of Health and Longevity," NBER Working Paper No. W11405, June 2005.

<sup>3</sup> Munos, Bernard, "Lessons from 60 years of pharmaceutical innovation," *Nature Reviews Drug Discovery*, December 2009.

how “new thinking, competencies and results” might be applied in the real world, the forum participants considered how the new models could be applied to the search for cancer cures and treatments.

This white paper outlines observations presented during the town hall and captures potential policy directions suggested by the participants for policymakers and national leaders in biomedical research and development. While the white paper captures the comments and opinions of those who presented at the town hall, two limitations apply:

- (i) any comments attributed to an individual are not intended to be direct quotes, but rather a general reflection of what was said; and
- (ii) this white paper is not an official document of any of the sponsoring organizations; rather, the policy directions included below are those presented by participants during the town hall meeting.

With these caveats in mind, the co-sponsors of this event present this white paper with the hope that it will inspire debate, confirm areas of concern, offer new directions, and add measurably to the development of policy directions that will support the new role for academia in drug development.

### **The Current Environment**

The process for successfully taking scientific discoveries from laboratories to routine patient use often involves multiple entities—government, academic research institutions, nonprofits and disease advocacy groups, venture capitalists, and private companies. These contributors don’t always work together as partners, and therefore the development process itself is plagued with gaps and inefficiencies. Among the most commonly cited shortcomings:

- The federal and university research apparatus alone often lacks the competencies and resources to move innovation that is being generated by today’s \$50 billion federal investment in biomedical research beyond early stage development. The traditional academic technology transfer mission, for the most part, has been designed to license early stage research, often funded by government and carried out by academic institutions, to those with expertise in early phase product development. The challenge exists today to develop new paradigms for the commercialization of academic-based innovation so that rich returns spring from the federal investment in basic research and do not languish before being translated into therapies that improve human health.
- Across the spectrum, data are guarded to protect proprietary interests. Universities, because of the inherent culture and the rewards based predominately upon publication, tend to be overly protective of their science. Former NIH director Elias Zerhouni, MD has described this as a “pervasive

proprietary mindset” in the research community, and says changing it is “a moral imperative.”<sup>4</sup>

- The period between early stage research and clinical trials is colloquially referred to as the “Valley of Death,” because it is here that many potential breakthroughs languish. A number of factors converge to contribute to this growing problem: prohibitive costs, unclear market potential, lack of capital, regulatory unpredictability, and lack of training in how to bring a discovery from the lab through the early process of commercialization.
- Venture capital, which has driven success in funding medical innovation, has waned in recent years. In 2009, the amount of venture capital devoted to bioscience was less than in 2005.<sup>5</sup> With the drying up of the capital markets, venture capital has been intolerant of investment risk.
- The FDA’s regulatory science capabilities are outdated, and the approval process for new drugs and treatments can be ad hoc, confusing, and inefficient. This is occurring at a time when investment capital is seeking predictability and, thus, calls for regulatory reform are linked directly with the rebuilding of a robust biomedical innovation ecosystem.
- While all parties have good intentions, patient needs often are subordinated to a focus on the development process.

These obstacles to the efficient flow of ideas “from molecule to the marketplace,” which impede improvements to both human and economic health, call for new models that will pave the way for scientific research to lead to new drugs for patients.

New models should be collaborative, with government, academia, nonprofits, advocacy groups, the venture capital community, and private industry all making commercialization of patient treatments their highest priority. Collaboration will require new platforms for data sharing and an end to the “pervasive proprietary mindset.” The FDA will need an enhanced scientific foundation to ensure predictable, efficient, and appropriate review of new compounds and treatments developed by innovators. Higher education institutions, which host the bulk of early stage science research, will have to become better equipped to move advances toward commercialization.

### **Observations from Leadership**

***“The federal government plays a central role in funding scientific research and development, and laying the groundwork for future discoveries, but collaboration and team science increasingly are***

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<sup>4</sup> Zerhouni, Elias, “Peer-to-Peer Sharing Spurs Scientific Innovation,” *Science Translational Medicine*, December 2009.

<sup>5</sup> CAMI, *Gone Tomorrow*, proprietary calculations based on Thomson Reuters VentureXpert data.

***required. And, collaboration is not a process that can be driven by the government alone."***

The nation's federal health enterprise leaders—Kathleen Sebelius, Secretary of Health and Human Services; Francis Collins, MD, Director of the National Institutes of Health; and Margaret Hamburg, MD, Commissioner of the Food and Drug Administration—offered their perspectives on the future of drug discovery, development, and commercialization.

These leaders were optimistic about academia's role in facilitating the development of new therapies from basic research discoveries, and they believe the federal government can promote a role for academic institutions and nonprofits in advancing new therapies through early phase proof-of-concept. Significantly, all three leaders acknowledged that the NIH system was established as a vehicle for basic research, not as a system to translate research into marketable treatments.

Comments made by Secretary Sebelius, Director Collins, and Commissioner Hamburg suggested that collaboration is crucial to success in future drug discoveries and development. This includes collaboration among federal agencies, and between agencies and external partners, including academic institutions, nonprofits, and industry.

In a conversation moderated by CAMI Chairman and former House Majority Leader Dick Gephardt, Director Collins and Commissioner Hamburg agreed that, given the essential functions NIH and FDA play at the two ends of the drug development and marketing pipeline, improving collaboration between the agencies likely would improve the process of translating research into therapies approved for patients. A new NIH-FDA Joint Leadership Council is a first step toward better alignment and collaboration. Collins and Hamburg see the Joint Leadership Council as a way to help accelerate pathways for cures by ensuring that each agency recognizes the needs of the other—regulatory considerations should be an integral component of biomedical research planning, and the regulatory review process should incorporate the latest science practiced at NIH and other research institutions.

Filling gaps in the drug development process, providing critical, publicly accessible tools, and becoming the facilitator of new linkages between institutions are viewed, increasingly, as key roles for NIH. The NIH is being asked to leverage the return on federally funded infrastructure and emerging technologies through new approaches, such as the molecular libraries and high throughput screening centers. In return, these targeted investments are expected to increase the speed and efficiency with which researchers can identify biochemical interactions and develop new potential therapies.

One example of this is in the field of rare disease research. Today, one in ten Americans is affected by a rare disease. In the United States, sixty of the seventy-one cancer types are considered rare and account for 25 percent of adult tumors. The eleven non-rare cancer types in the United States include prostate, breast,

lung/bronchus, colon, uterus, bladder, melanoma, rectum, ovary, non-Hodgkin lymphoma, and kidney/renal pelvis neoplasms.<sup>6</sup> Research on diseases with small populations does not hold the same potential return on investment that research on diseases affecting larger populations does, yet the risks associated with drug development for these conditions remains high. The NIH's Therapeutics for Rare and Neglected Diseases (TRND) initiative is a seminal program that seeks to de-risk research into treatments for rare and neglected diseases. TRND recognizes that these conditions tend to receive less (if any) private capital for research supporting pre-clinical work, and which require collaborative efforts by academic institutions, foundations, industry, and government researchers and clinicians to be successful.<sup>7</sup>

A key component of collaboration is sharing data. NIH should explore with stakeholders new mechanisms that make it possible to share and access research data, while maintaining appropriate privacy standards. This would facilitate longitudinal research such as comparative effectiveness studies and safety data collection over time to ultimately improve health outcomes. This is especially important for building on the knowledge of current therapies to enhance research in new areas, for example, repurposing known therapies for new uses. TRND seeks to advance this goal by publishing data on both successes and failures to better inform future research.

The government officials and town hall participants agreed that government alone cannot drive the collaboration process. The Obama Administration was credited with conducting outreach to the public, soliciting a range of input, and providing opportunities for participation from external experts.

Facing the reality of tight budgets and constrained resources, building support for a renewed commitment to medical innovation will require a better understanding of the economic impact of the health care system among the public and policymakers. According to the Congressional Budget Office, while health care accounts for some 16 percent of United States gross domestic product (GDP), and employs more workers than any other industry, the benefits of medical innovation to economic growth and productivity are not always clear to people outside the field.

A recent report from the Council for American Medical Innovation concluded that jobs emerging from investment in biomedical industries exceed the national average private-sector wage by more than \$24,000.<sup>8</sup> The report also cited research that estimated the economic cost associated with chronic diseases such as heart disease, diabetes, and cancer at \$1.3 trillion a year.<sup>9</sup> Curing or alleviating those conditions could help to increase the rate of U.S. economic growth.

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<sup>6</sup> Greenlee, Robert T., et al., "The Occurrence of Rare Cancers in U.S. Adults, 1995–2004," *Public Health Reports*, January/February 2010.

<sup>7</sup> NIH release, "NIH Announces New Program to Develop Therapeutics for Rare and Neglected Diseases," May 20, 2009.

<sup>8</sup> CAMI, *Gone Tomorrow*.

<sup>9</sup> CAMI, *Gone Tomorrow*, citing Ross DeVol and Armen Bedroussian, with Anita Charuworn, Anusuya Chatterjee, In Kyu Kim, Soojung Kim, and Kevin Klowden, *An Unhealthy America: The Economic Burden*

In addition to steps the federal government can take to accelerate innovation, state and regional efforts to optimize the conduct of biomedical research are critical for national success. For example, the University of Kansas and its regional partners have collaborated in an effort that shows great promise for spurring economic growth and development within regional economies. By weeding out duplicative research, the collaborative model can trim costs and direct research dollars and venture capital to its most productive uses.

During the town hall meeting, recommendations offered in the sessions with Secretary Sebelius, Dr. Collins, and Dr. Hamburg emphasized both providing adequate funding and promoting commercialization of promising research. Adequate federal funding is crucial for both the FDA and NIH in efforts to fund external research and, for the FDA, to better serve as the designated regulatory authority. Moreover, the FDA cannot function on user fees alone and should instead have access to a stable funding stream.

### **Panel 1: New Thinking**

Topics included consideration of: (a) industry-academia collaborative agreements and how diverse institutions collaborate to close the risk-reward gap; (b) key capabilities, shared resources, and culture transformations that must be in place to allow academia to take on a new role in drug development; (c) new tools and needed systems to promote innovation and early phase commercialization within academic institutions and cancer research centers; and (d) bioscience workforce initiatives to train the next generation of medical innovators.

Panelists: Roy Jensen, MD, The University of Kansas Cancer Center; Garry Neil, MD, Johnson & Johnson; Lesa Mitchell, vice president, Kauffman Foundation; Tony Atala, MD, Wake Forest University School of Medicine; and, Barbara Kunz, Battelle, president, Health and Life Sciences Global Business

***“Improving the rate at which the scientific community successfully develops disease treatments requires innovative thinking and collaboration among academia, industry, and nonprofits.”***

***“The new collaborative model is not as much about who is paying for what, but about what skills a sector brings to the table”***

***“Translation is not an event; it is a matter of will.”***

The panel discussion on *New Thinking* addressed how academia and industry can better collaborate to successfully and efficiently translate scientific discoveries into new therapies.

Moving an idea from basic research into clinical study (through the “Valley of Death,” the stage during which many potential therapies languish for lack of funding) is becoming increasingly difficult and costly. This early “proof-of-concept” phase requires an average of two to four years of work and up to \$50 million—part of an overall discovery process that costs hundreds of millions of dollars over more than a decade.<sup>10</sup>

There is a strong need for new therapies that are safe, effective, and quick to market. Secretary Sebelius noted that the number of drugs approved in 2010 is the same as the number approved in 1950—an indication that, even though science has advanced, the complexities of the development process may be holding back progress in translating science into treatments. Furthermore, while some 7,000 diseases affect the human family, only 600 have treatments. Fewer than 3 percent of rare diseases have an FDA-approved treatment, yet one in ten Americans is affected by a rare disease.

Why do new ideas languish?

Despite attention over the past five years, increased funding is needed for translational and transformative research and projects that intend to bridge the “Valley of Death.” The current system, including NIH’s RO1 grant mechanism, remain heavily focused on basic science and often ignores late-stage, translational bench-to-bedside research.

Another key barrier is the lack of translational expertise among universities, which are rich sources of discovery, as well as lack of incentives for team science and scarce funding for early stage translational activities. Academic scientists often lack a well-developed understanding of how new therapies reach the market. With a broader view of the entire drug-development process, researchers can help to advance it. This kind of training in commercialization should become a key component of the Clinical and Translational Science Award infrastructure.

Yet, progress is occurring in certain areas. Recent multi-sector collaboration models have shown to be effective at addressing the challenges frequently encountered during early stage drug discovery.

Bernard Munos, a strategist at Eli Lilly, noted that, since the early 1980s, large pharmaceutical and biotechnology companies’ share of new FDA-approved drugs has declined from about 75 percent to about 35 percent. At the same time, small companies’

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<sup>10</sup> NIH release, “NIH Announces New Program to Develop Therapeutics for Rare and Neglected Diseases,” May 20, 2009.

share has increased from about 23 percent to nearly 70 percent.<sup>11</sup> Munos noted the growth of venture capital funding as a significant factor. He also suggested that small firms have the ability to fill research needs that large companies—with pressure to find and fund blockbuster drugs—overlook, including rare and neglected diseases.

Munos proposed, “If large companies could organize innovation networks to harness the scientific diversity of biotechnology companies and academic institutions, and combine it with their own development expertise, they might be able to reverse the forces that are undermining the current research model; that is, they might be able to lower their costs and increase their output.” One aspect that could be addressed is the current lack of coordination in the clinical trial system. A recent report from the Institute of Medicine identified several inefficiencies with the NCI’s Cooperative Group trial infrastructure, including the lack of administrative coordination when starting new clinical trials.<sup>12</sup> Currently, the infrastructure is recreated every time a new trial is run—a requirement that adds costs and uncertainty. A template model to address this burden could improve the efficiency of clinical trials well beyond oncology.

Improving the rate at which the scientific community successfully develops disease treatments requires innovative thinking and collaboration among academia, industry, and nonprofits. The new collaborative model is not as much about who is paying for what, but about what skills a sector brings to the table. This new concept of “team science” must move beyond the academic institution to support collaboration with government, nonprofits, and industry.

Chris Austin, MD of NIH noted that translation is not an event; it is a matter of will. From funding to system engineering, it is a dynamic process in which each group plays a unique role. The missing links in this process, regulatory science and data sharing, need to be added to ensure the development process is completed successfully more often.

A new opportunity to enhance collaboration and facilitate translational research is the Cures Acceleration Network (CAN). Established as a part of the Patient Protection and Affordable Care Act of 2010, once funded, CAN will be housed in the NIH, which is directly charged with speeding the process of translating research into treatments for a host of diseases, including cancer, diabetes, Parkinson’s, Alzheimer’s, and autism.

#### Emerging Policy Considerations:

- Research institutions and commercial entities should establish policies to enable timely sharing of pre-competitive data that will aid future drug discoveries.

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<sup>11</sup> Munos, Bernard, “Lessons from 60 years of pharmaceutical innovation,” *Nature Reviews Drug Discovery*, December 2009.

<sup>12</sup> Mendelsohn, J., Moses, H., et al. “A National Cancer Clinical Trials System for the 21st Century: Reinvigorating the NCI Cooperative Group Program,” Institute of Medicine, April 15, 2010.

- Federal agencies that fund translational research programs also should provide or require education about commercialization, including opportunities, challenges, and regulatory mandates.
- Organizations with a vested interest in commercializing new therapies for patients should promote “team science” incentives for translational research.
- Congress should fully fund the Cures Acceleration Network (CAN).

## Panel 2: New Competencies

Topic ideas included consideration of: (a) cross-disciplinary FDA Centers of Excellence in Regulatory Science; engaging schools of science, engineering, business, and other related areas of academic focus with departments of medicine; (b) how industry and academia might begin to meet each other halfway in this new innovation business environment; and (c) high throughput screening in academia as an example of a center of excellence.

Panelists: Gail Cassell, PhD, Eli Lilly & Co; Chris Austin, MD, National Chemical Genomics Center, National Institutes of Health; Jesse Goodman, MD, Federal Drug Administration; Stephen P. Spielberg, MD, PhD, Children’s Mercy Hospital; Jim Baxendale, MS, MBA, University of Kansas

***“Next-generation science is needed to speed much-needed therapies to market. This calls for new models in applied and regulatory science, with new considerations for clinical trials and the incorporation of biomarkers, nanotechnology, personalized medicine, and informatics.”***

***“Universities should develop strategies for managing innovation rather than limiting their focus on technology transfer.”***

A panel discussing *New Competencies* focused on how collaborations can aid the development of new tools to evaluate medical products and establish best practices for commercial agreements.

Panelists and participants discussed the FDA’s reliance on outdated science when determining product safety and efficacy. The approval process also can be erratic, which can leave companies that seek approvals unsure of the proper protocol to follow and frustrated at the lack of communication from the agency.

To improve the FDA's level of scientific expertise, recommendations included increased collaboration among academic centers, industry, and the FDA that could help support regulatory science programs and develop core competencies for product development. This must be done in a way that brings transparency and trust to these multi-party discussions, and does not view the sharing of opinion, experience, and information as a threat to the FDA's regulatory role.

To improve academic institutions' capacity for commercialization, institutions with a specific area of expertise could serve as "centers of excellence" for routine interactions and research. For example, most universities do not have medicinal chemistry, drug delivery, pharmacokinetics/pharmacodynamics (PK/PD), and toxicology expertise that is required for translational work and, therefore, could benefit from access to institutions that specialize in this work.

Appropriate protections of academic-based intellectual property (IP) are essential for medical innovation to thrive. Yet, allowing this system to become complex and inefficient defeats the very purpose that IP procedures are designed to advance. This conundrum is a recurring theme in the discussion of improving commercialization rates of university research.

Academic institutions need a robust infrastructure to deal with regulation of material transfer agreements and IP issues when entering into commercial partnerships. It is essential for universities to find the right balance in protecting the value of their early stage work, while allowing it to move ahead in the development process. Universities should develop strategies for managing innovation rather than limiting their focus on technology transfer. Multi-sector collaboration to develop best practice guidelines could help streamline the establishment of new commercial agreements.

Next-generation science is needed to speed much-needed therapies to market. This calls for new models in applied and regulatory science with new considerations for clinical trials and the incorporation of biomarkers, nanotechnology, personalized medicine, and informatics. To ensure the successful adoption of new technologies for the long term, the future scientific workforce will need to be trained in this new approach to collaborative biomedical research and development.

Academia does not have to become "just like industry," but it should attempt to adopt and adapt industry-like competencies where required for the university to assume its new role in drug development. This includes the ability to bring project management expertise to a drug development plan, the assurance that investigator-sponsors understand their new role and responsibilities as it pertains to the FDA, and the development of early phase clinical trial capabilities that match industry competence.

## Emerging Policy Considerations:

- The federal government should empower the FDA with the scientific capabilities and resources to ensure a thorough evaluation of the risk and benefits of new therapies.
- The FDA should define new regulatory paths that accommodate the shifts in translational science, including emerging ideas associated with the incorporation of biomarkers, nanotechnology, personalized medicine, and informatics.
- Universities, in collaboration with industry, should establish models for intellectual property and technology transfer processes that will become widely adapted and trusted, removing many of today's hurdles to licensing and other opportunities to commercialize early innovation.
- The NIH should invest in additional clinical and translational science awards (CTSAs) across the country and fund CTSA centers that bring unique capabilities, translational research, and academic commercialization contributions to the consortium.
- Universities should develop translational research training programs so current students, who will be future scientists, will be trained in next-generation techniques, applications, project management, collaboration models, and regulatory science.

## Panel 3: New Results

Topic ideas included: (a) public-private partnership models to advance drug discovery, bringing industry, academia, nonprofits, and state and local government together to create the best opportunity for finding new and novel drugs to established, rare, and unproven disease targets; (b) collaboration to facilitate repurposing of potentially beneficial agents; and (c) the role of commercialization in job creation

Panelists: Scott Weir, PhD, The University of Kansas Cancer Center, Institute for Advancing Medical Innovation; Lou DeGennaro, PhD, Leukemia & Lymphoma Society; Tom Thornton, president and chief executive officer, Kansas Bioscience Authority; Michael Weingarten, SBIR Development Center, National Cancer Institute

***“We would get closer to cancer cures if funders required collaboration at the point of sharing data, as well as a holistic and multi-disciplinary***

***approach to development. Patients should be at the center of all thinking and action.”***

***“At the center of this new collaborative model is an understanding that separate interests are working together to change the current culture that has not been delivering needed therapies as quickly as possible. A common shared goal in this new model is the notion that everyone is winning when a new therapy gets to a patient; it is not about single ownership of drug discoveries.”***

As in most endeavors, and in science especially, the proof is in the product. Dr. Ellen Sigal, founder of Friends of Cancer Research, observed that, while there were about 800 groups involved in cancer research ten years ago, today there are thousands. There is a hunger for results and a need to develop platforms that foster collaboration-focused results and tangible metrics.

Funders can play a part in driving collaboration. The Leukemia & Lymphoma Society’s Dr. Lou DeGennaro stated that we would get closer to cancer cures if funders required collaboration at the point of sharing data, as well as a holistic and multi-disciplinary approach to development. Patients should be at the center of all thinking and action. Dr. Frank Douglas, Austen BioInnovation Institute in Akron and Kauffman Foundation, reported that, with a patient-centered focus, the Leukemia & Lymphoma Society and the University of Kansas’ Institute for Advancing Medical Innovation have taken an idea into a Phase I trial in thirteen months—a reflection of the universal goal of reducing cycle time and increasing the number of successful outcomes.

Funders such as nonprofit disease philanthropy groups and federal agencies distributing small business innovation research grants are beginning to bridge the divide between basic research findings and clinically beneficial therapies. New models of providing economic capital also are serving as conduits to finding the right partners to commercialize proof-of-concept findings, and are showing signs of success. Emphasizing early stage collaboration between academic institutions, government, and industry also makes it possible to “de-risk” drug discoveries for handoff to the private sector to complete the commercialization process.

In addition to developing collaboration with development expertise, regulatory expertise must be engaged as well. Since many of the early successes of this new model focus on therapies for rare and neglected diseases, the FDA must be ready to conduct timely and effective reviews of drugs in this classification. There also is a need to address risk/benefit analysis for this specific population, as it often varies from the amount of risk/benefit that other patient populations have come to expect and tolerate.

At the center of this new collaborative model is an understanding that separate interests are working together to change the current culture, which has not been delivering needed therapies as quickly as possible. A common shared goal in this new model is the notion that everyone is winning when a new therapy gets to a patient; it is not about single ownership of drug discoveries.

Emerging Policy Considerations:

- FDA should define necessary parameters that take into consideration the difference for development of therapies for rare and neglected diseases.
- Universities must support new models from the top (Chancellor level) down.
- University science leaders must identify areas in which external expertise is needed from nonprofits, industry, and government, and establish a focus on entrepreneurship.

#### **Panel 4: What This All Means for Cancer**

Panelists: Roy Jensen, MD, The University of Kansas Cancer Center; Ellen Sigal, PhD, founder, Friends of Cancer Research; Steven Averbuch, MD, Bristol-Myers Squibb Company; Frank Douglas, MD, PhD, Austen BioInnovation Institute in Akron and Kauffman Foundation

***“The National Cancer Act of 1971 was designed to establish demonstration projects, but never mentioned curing cancer. To support and highlight translational research and a requirement for cures, there is a need to design a new research paradigm that makes finding cures an explicit goal.”***

The final town hall panel focused on how this new model of collaboratively developing and bringing to market innovative treatments can be applied to the search for cancer therapies and cures.

The National Cancer Act of 1971 was designed to establish demonstration projects, but never mentioned curing cancer. To support and highlight translational research and a requirement for cures, there is a need to design a new research paradigm that makes finding cures an explicit goal.

Panelists discussed the impact of multi-sector collaborations and public-private partnerships on academic research and commercial development, as well as the need

for new policies to facilitate the success of new models that ultimately will create a paradigm shift in the battle against cancer.

In addition to recommendations highlighted throughout the forum, a key recommendation emerging from the final session is that project leads at universities, companies, and nonprofits must remain involved throughout each step of the process. For example, an academic lead cannot simply hand off legal work to tech transfer offices and IP lawyers. When this happens, it is easy to lose sight of end goals, which could jeopardize progress. As Bernard Munos has noted, all the contributors in the research and development process “must be shepherded towards a goal.”<sup>13</sup>

## **Conclusion**

There is strong evidence of a breakdown in the drug development and medical innovation processes. Researchers across sectors—government, academic institutions, and industry—continue to make extraordinary scientific breakthroughs. But, in any given year, only half as many new drugs are approved for patient use as were approved a half-century ago.

*New Thinking, New Competencies, and New Results* offered an opportunity to explore how government, nonprofit organizations, and academic institutions can define new models of working with the private sector to enhance drug development efforts and bring safer, more effective drugs to the market more efficiently. While participants at the town hall focused specifically on cancer treatments, a more efficient and effective drug development process would benefit patients affected by other diseases, as well.

New models of medical innovation must involve a greater degree of collaboration among all participants in the drug development process, with more data sharing, a broader understanding of how early stage work drives commercialization, and a commitment to ensuring that the FDA is equipped with the latest science so that it can expeditiously bring safer scientific advances to patients waiting for cures.

There are no easy solutions to make a highly complex series of processes more efficient, but the ideas and recommendations generated at this town hall will be useful to all stakeholders in designing new models to drive the development of treatments and cures in the twenty-first century.

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<sup>13</sup> Munos, Bernard, “Can open-source R&D reinvigorate drug research?” *Nature Reviews Drug Discovery*, August 18, 2006.