

**University of Massachusetts Medical School**  
**Testimony for U.S. Senate Committee on Appropriations**

It is an honor to submit this testimony to the United States Senate Committee on Appropriations for its full committee hearing on *Driving Innovation through Federal Investments*. Thank you to Chairwoman Mikulski, Ranking Member Shelby and all of the committee members for this opportunity.

As you know, investment in medical research by the National Institutes of Health (NIH) has yielded tremendous improvements in human health. Today, individuals impacted by serious conditions such as cancer, stroke, heart disease, diabetes and HIV have longer life expectancies than ever before due to advancements from NIH-funded research. NIH research has a long and proven track record of yielding treatments and therapies that dramatically improve health, quality of life, and life expectancy across the entire spectrum of disease, from these relatively common ailments to rare conditions that impact fewer people but in an equally devastating manner.

This progress is reflected in breakthroughs from NIH-sponsored research at the University of Massachusetts Medical School. Since its founding over four decades ago, UMMS has had a significant impact in biomedical research, over 80% of which has historically been from NIH. The impact of this research has been felt locally in Central Massachusetts and around the world. UMMS researchers have been recognized as world leaders, for example, in RNA interference (“RNAi”), immunology, diabetes, gene therapy, neurodegenerative disease, and anti-HIV therapy.

From Dr. John Sullivan’s participation in the 1980’s in discovery and development of nevirapine for prevention of maternal-to-infant transmission of HIV, to major medical breakthroughs in 2013 by Dr. Katherine Luzuriaga and Dr. Jeanne Lawrence in HIV and Down Syndrome, respectively, UMMS researchers have been at the forefront of remarkable NIH-supported breakthroughs for decades. Early last year, Dr. Luzuriaga, who began her career in Dr. Sullivan’s lab, reported the first case of a “functional cure” in an HIV-positive infant along with colleagues from Johns Hopkins and the University of Mississippi, a discovery that earned the three women recognition on *Time Magazine*’s 2013 list of the 100 Most Influential People in the World. Just three months later, Dr. Lawrence reported a laboratory breakthrough that offers hope for silencing the extra chromosome responsible for Down Syndrome after she discovered a naturally occurring chromosome “off switch” that can be rerouted to neutralize the extra chromosome responsible for this genetic disorder.

Between these notable outcomes in the 1980’s and 2013, UMMS researchers have contributed to countless advances in medical knowledge and therapies with funding support from the NIH. This likewise is the case at institutions across the country, and the results are truly astounding. The average American lives six years longer today than in the 1970s, largely because of pioneering NIH investments that generated historic medical advances such as those by Drs. Sullivan, Luzuriaga and Lawrence. Childhood leukemia, heart disease, cervical cancer, HIV, rotavirus – the course of history of each of these diseases and many others have been transformed due treatments or preventive measures that resulted from NIH-supported research.

Just last month, Dr. Luzuriaga and her colleagues announced that a second baby born with HIV appears to have been cleared of infection after early and aggressive treatment, providing further evidence that early therapy may be the key to achieving remission. At a recent symposium, she stated: “Twenty years ago, I don’t think we would have thought we’d get to the point where we’d talk about a functional cure.” Yet today, as the result of years of research supported by the NIH, she and her colleagues understand that viral reservoirs, which serve as the barrier to cure, “are laid down very early in infection, leading to the concept that initiating treatment within hours of birth will be necessary to eradicate infection.” Consider for a moment the immense potential this new knowledge holds for patients impacted by HIV and their families. What seemed unimaginable two decades ago has become reality today. And just imagine what can become reality over the next two decades *if* we continue to invest in research.

In addition to advances resulting from clinical research, NIH-sponsored basic research at UMMS is transforming our understanding of biology and opening exciting new pathways of research into a variety of diseases. In 2006, Dr. Craig Mello of UMMS was the co-recipient of the Nobel Prize in Medicine along with his research colleague, Dr. Andrew Fire of Stanford University, as a result of their discovery that short strands of RNA can silence the expression of targeted genes – so-called “RNA interference” or “RNAi.” Dr. Mello’s research on a worm opened up an entirely new and promising field of medical research focused upon utilizing RNA interference to silence the production of proteins associated with a wide variety of human diseases (as well as opening up avenues in other fields of biological sciences, including agriculture). Today, academic and commercial medical researchers alike are investigating multiple delivery methods to transport strands of interfering RNA to block production of specific proteins and thereby develop therapies for a variety of diseases, such as cancer, HIV, Huntington’s disease, skin diseases and hemophilia, just to name a few. The possibilities are truly astounding. Again, what seemed unimaginable a short time ago has now become reality. And again, imagine what may become reality in the near future *if* we continue to invest in research.

Mid-career researchers at UMMS likewise are involved in projects that reflect the promise of investment in science. For example, Dr. Oliver Rando received worldwide media coverage in 2010 for a research discovery regarding the impact of paternal diet on metabolism in offspring and Dr. Brian Lewis studies the molecular mechanisms underlying pancreatic cancer. The work product of these two mid-career investigators and their peers at UMMS and elsewhere holds amazing potential for human health. Their work buttresses the hope that what may seem unimaginable today will become reality *if* we continue to invest in research.

Unfortunately though, the work of Drs. Rando and Lewis are being negatively impacted by decisions to reduce federal research investment. Last summer, the grant that funded Dr. Rando’s research came to an end and he did not receive funding for multiple meritorious applications he submitted to continue the work. In Dr. Lewis’s case, he and his team work most effectively by simultaneously investigating numerous potential avenues against pancreatic cancer, which requires securing multiple grants. But under current funding circumstances they have not been able to do so. Meritorious and highly ranked applications by both researchers, which would have been funded in previous years, have not been funded. As a result, talented young trainees and employees in their labs have been released and promising research directions have been abandoned or not embarked upon. Sadly, among the young researchers impacted by reductions in force are diverse and talented investigators from groups underrepresented in medical research,

the very people who are instrumental to increasing diversity in the field. A leader in recruiting individuals from underrepresented backgrounds into science, Dr. Lewis now has to watch as mentees abandon their dreams of being research scientists because they have difficulty envisioning productive careers in the current fiscal climate.

Established investigators likewise are being hampered now by reductions in federal funding. For example, Nobel Prize recipient Dr. Mello withstood cuts of 3.5 percent on an NIH grant and Dr. Luzuriaga withstood reductions of 6 to 8 percent on two research grants in the same year her research breakthrough inspired *Time Magazine* to name her to its 100 Most Influential People in the World list. The impact of cuts upon UMMS research over two years has been substantial: in 2011, our faculty had 380 NIH awards amounting to \$180.5 million, yet by the end of 2013 this was reduced to 302 awards amounting to \$130.7 million. We were fortunate to secure increases in non-federal funding sources during that period, but our entire research portfolio still decreased from over \$277 million to \$233.5 million. We and other institutions now face the difficult challenge of watching as many researchers are forced to reduce the scope of their projects, thereby slowing the pace of advancement against disease and impacting the careers of talented junior researchers and lab employees.

Yet, in spite of these setbacks, we at UMMS are optimistic that federal policymakers will see the wisdom in reinvesting, rather than disinvesting, in medical research. We fully support the Ad Hoc Group for Medical Research's recommendation that Congress recognize the NIH as a critical national priority by providing at least \$32 billion in funding in FY 2015. The medical and economic case is simply too compelling not to reinvest. If you reinvest, new therapies resulting from research at UMMS and elsewhere will improve patients' lives and ensure that NIH's track record as the world's foremost medical research agency continues. Just as Dr. Sullivan's research in the 1980's yielded therapies that have saved literally thousands of lives across the globe, so too will more recent UMMS breakthroughs produce major advances in human health.

Our work will enhance America's global economic competitiveness as well. As you know, NIH funding is vital to the health of the national economy, as commercialization of new findings creates skilled jobs, new products and long-term economic growth. Here again, UMMS is a case in point. In an era in which a declining industrial base severely impacted most old manufacturing centers, the City of Worcester bucked the trend by gaining population and gaining jobs in medical research-related fields. From 2005 to 2009, employment in the "education and health services" sector (as defined by the U.S. Bureau of Labor Statistics) grew by 10.6 percent in the City of Worcester and 11.6 percent in Worcester County.<sup>1</sup> Though this sector does not consist entirely of research jobs, here in Central Massachusetts it includes a substantial number of positions attributable to NIH-funded research. This sector now is the largest in this former industrial hub, employing some 45 percent of workers in the City of Worcester and almost one-third in the county.<sup>2</sup> Moreover, thousands of additional jobs that do not fall within "education and health services" are attributable to the presence of an NIH-supported research center – most notably at private life sciences companies that cite UMMS's presence as a factor in their decision to locate in Worcester. These companies range in size from large, multinational employers such

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<sup>1</sup> *Benchmarking Economic Development in Worcester: 2011*, Worcester Regional Research Bureau, March 2011.

<sup>2</sup> *Benchmarking Economic Development in Worcester: 2012*, Worcester Regional Research Bureau, March 2012.

as AbbVie (formerly Abbott Bioresearch Center) to small startups. The commercial impact of research is reflected as well by our licensing data: UMMS has 182 active licenses with 105 companies.

The full health and economic impact of today's medical research may be difficult to predict precisely, but telltale indicators are evident. For example, patent applications resulting from NIH-funded research demonstrate clinical and economic potential, even though a product may not yet be on the market. UMMS data provides a compelling example: from 2001 to 2012, UMMS filed 817 patent applications, virtually all of which were attributable to NIH-funded research.

The NIH can continue to be the world's leader in biomedical research, but only if it is provided with sufficient funding to invest in promising research by the nation's leading scientists. Across the country, institutions like UMMS possess the physical and intellectual infrastructure and have long track records of success in producing medical breakthroughs and catalyzing economic development. America's global competitors are increasing spending on medical research and undertaking aggressive efforts to attract young researchers to relocate (including their native-born scientists who have trained and worked in the U.S.), but they cannot yet match our infrastructure and track record. *However, this could change.* Reinvestment now is absolutely critical to secure our standing, retain our talent and ensure our future is as impressive as our track record. Reinvestment will enable us to continue to blaze a path toward cures and therapies to debilitating conditions; continue to expand our local, state and national economies; continue as the world leader in innovation; and continue to be a nation that attracts and retains talent rather than exports talent to other countries.

We at UMMS are optimistic that you will reinvest in research because we as a nation have come too far to turn back now. We have devoted substantial national treasure to education, training and physical infrastructure to lay the foundation for research success. Institutions such as UMMS have followed suit with our own investments to advance this national vision. Together, building upon this foundation, we jointly invested in research to position the United States as the world's preeminent medical research leader. That research investment, built upon the solid intellectual and physical infrastructure foundation we jointly established, has yielded an absolutely enormous return. Whether measured by advances in human health or by national economic output, it has been an unparalleled success and is the envy of nations across the world.

This is the tremendous legacy of federal policymakers' wise and insightful decisions. The case is simply too compelling to consider turning back now.