Section by Section

Background

When it comes to investments and advancements in biomedical research, the United States has no equal. Its National Institutes of Health (NIH) is the world's largest public source of biomedical research funding with an annual budget of over $28 billion. The NIH is comprised of 27 major institutes and centers, leading the way in cancer, cardiovascular, infectious disease and allergy advancements for health promotion and relief from the burdens of disease.

The private sector is also investing substantial resources in increasing both longevity and quality of life. These companies now invest more than the federal government in biomedical research and development (R&D). Potent pharmaceuticals and cutting edge medical devices provide health care professionals with a therapeutic arsenal that has increased lifespan seven years since 1960 and dropped neonatal mortality four fold. Partnerships between NIH and private industry are not often recognized for their key roles in bringing new treatments to the public, but are of great importance as they have led to life-changing therapies from to Taxol to Claritin to HIV anti-retrovirals.

But how can biomedical R&D proceed even faster? How can partnerships between NIH's Institutes and Centers, disease-based NGO's, biotech companies and small and large pharmaceuticals occur even more frequently? Towards which diseases should our resources be prioritized in the first place? How can NIH and the private sector be more responsive to emerging public health threats such as bioterroism, an avian flu pandemic, antibiotic resistance, and a waning vaccine supply?

Center for Cures

In response to these pressing questions and the capacity of the NIH to address our health needs, Senator Lieberman and Senator Cochran are proposing a $5 billion dollar annual investment to create the American Center for Cures (ACC). The mission of this new NIH Center will be to promote more rapid translation of public and private research into therapies, diagnostics and tools, which can effectively treat and possibly cure diseases of critical importance to domestic and global health. The ACC will enhance NIH's ability to not only pursue fundamental knowledge about the nature and behavior of living systems, *but to apply that knowledge to extend healthy life and reduce the burdens of illness and disability*. This is NIH's mission.

Specifically, the American Center for Cures will:

1) Direct new resources towards the world's most burdensome diseases and towards biomedical, bioengineering, and biotechnological research with the greatest therapeutic impact and promise.

2) Create an ACC national advisory board consisting of key health experts and stakeholders, who will help identify the critical diseases and health threats requiring greater public and private investment.
3) Create a special Health Advanced Research Projects Agency (HARPA) to support innovative multidisciplinary collaborate research between NIH Institutes, between NIH and other federal agencies and between NIH grantees and business partners, for projects with the potential for significant health impact.
4) Create health-centered Federally Funded Research and Development Centers (FFRDC) which will bring together interdisciplinary teams of experts including scientists, clinicians, epidemiologists, and pharmacists for a time limited period to focus on developing therapeutic breakthroughs for important disease entities.
5) Invest further in the development of an expert workforce which will augment the nation's translational research capacity. Such an effort will include training new clinical researchers and bioinformatics professionals.
6) Promote risk-taking and collaboration between NIH Institutes and Centers.
7) Streamline the clinical research process essential to determining if new treatments are effective and safe.
8) Promote the innovative efforts of small to medium sized biotechnology and bioengineering firms who require additional support in key traditionally under-funded stages of product development -- the so called R&D "Valley of Death".
9) Facilitate NIH partnerships with private industry in the preclinical stage of the R&D process so as to formulate a plan for health research translation and commercialization from the outset.
10) Standardize NIH information management systems and reporting requirements of publicly funded research to improve information sharing between the applied science, translational research and business communities.

A section by section summary of the legislation is included below. If you have any questions about this legislation or would like to be an original cosponsor, please contact Wilson Wang (202-224-4042) in the Office of Senator Lieberman or David McClendon in the Office of Senator Cochran (224-5054).

Section 1: Short title
Section 2: Table of contents
Section 3: Findings
Section 4.: Amends Title IV of the Public Health Services Act to establish a new Center at the National Institutes of Health (NIH) called the American Center for Cures (ACC)

Part J- American Center for Cures

Section 499A: Definitions
Section 499B (a): States the mission of the proposed American Center for Cures (ACC), which is to increase the capacity of the NIH to promote translational research between its Institutes and Centers, between the NIH and other Federal agencies and between NIH grantees and business partners so as to speed the development of effective therapies, diagnostics and cures essential to human health and well being.

The ACC shall formulate and implement a strategy for the nation's translational research investment based on (1) a prioritization of biomedical research based on disease burden and research promise, and (2) funding for innovative, multi-disciplinary, and collaborative research

The ACC will be guided in part by a series of "Grand Challenges" or strategic challenges that direct the health research community towards multi-staged projects with the potential to transform the healthcare landscape. Examples include: the creation of a research tool arsenal that enables the country to detect quickly and accurately to acute health threats, such as an avian flu pandemic or a bioterrorism attack; a commitment by researchers and manufacturers from public and private sectors to develop vaccines for the world's most deadly infectious diseases including HIV, tuberculosis, and malaria. Other examples are provided in this section.

Section 499B(b): Establishes a Director of Cures (to be called in this document the "Director") who will administer the ACC. The President of the United States will appoint the Director. The NIH Director will recommend candidates for the Director to the President. The NIH Director will work with the Director to promote the nation's translational research efforts.

The Director will have at his disposal an annual acceleration fund of $5,000,000,000 dollars to provide support for research and development of breakthrough biomedical discoveries and to carry out the purposes of the ACC. No less than one half of the acceleration fund will be allocated to a Health Advanced Research Projects Agency described in Subpart II.

Section 499B(c): Establishes a Cures Council to advise and direct the translational research efforts of the ACC. The Council will be co-chaired by the Director of Cures and the Director of NIH. Membership will include NIH Institute and Center Directors; leaders from at least 9 federal agencies including the Director of the Agency for Healthcare Research and Quality (AHRQ), the Director of the Defense Advanced Research Projects Agency (DARPA), and the President of the Institute of Medicine (IOM); no fewer than three leaders from the small business community; three leaders from large pharmaceutical or biotechnology companies; and three leaders from academia. All Council members will be appointed by the President.

The Council shall establish subcommittees including one of NIH Institute and Center Directors to coordinate research priorities in, and ensure sharing of research agendas among, the Institutes and Centers. The subcommittee shall also coordinate the ACC research agenda with that of the NIH's Institutes and Centers.

The Council shall be aided by the Office of Intramural Risk Opportunity and Mapping of the Office of Technology Transfer established in subpart V.

The Council shall conduct an annual assessment of ACC priorities and progress and make this available to the public in written and electronic forms.

Section 499B(d): The Director of Cures shall prepare and submit, directly to the President for review and transmittal to Congress, an annual budget estimate for the Center.

Subpart 1 - Federally Funded Research and Development Centers

Section 499C: Federally Funded Research and Development Centers (FFRDC's) will serve as sites for multidisciplinary and cross-scientific research within particular areas of health. The Director may establish one or more FFRDC's to carry out activities related to the mission of the ACC. These Centers will establish, as appropriate, technology test beds and incubators, utilize cooperative agreements with the private sector, and conduct large-scale multi-disciplinary translational research projects in health or disease areas which are essential to medical advancement, but lack adequate private sector funding.

The FFRDC's shall consult widely with representatives from private industry, institutions of higher education, nonprofit institutions, other federal governmental agencies, and other federally funded research and development centers.

The Director shall ensure that competitive mechanisms are used to select and to promote the ongoing quality and performance of the FFRDC's.

Contracts between the ACC and FFRDC's shall be for no longer than 7 years, after which time refunding shall be contingent upon approval by the Director and the Cures Council.

Each FFRDC shall biannually submit a report on the activities carried out by the Centers under this section to the Director and the appropriate committees of Congress

For any fiscal year, the Director may use not more than 25 percent of the funds available in the Director's Acceleration Fund for FFRDC's.

Subpart 2 - Health Advanced Research Projects Agency

Section 499d. Technological and scientific innovation often require innovative thinking and significant funding streams that are rapid and are outcomes based. Funds must also encourage expert multidisciplinary collaboration. This section establishes at the ACC a Health Advanced Research Projects Agency (HARPA) for these purposes.

HARPA will be headed by a Director of the Research Projects Agency who will be appointed by the Director of Cures

HARPA shall be composed of not more than 100 expert portfolio managers in key health areas, as determined by the Director of HARPA in conjunction with the Director and Cures Council.

HARPA shall undertake the grand challenges formulated by the Center and encourage innovative, multidisciplinary, and collaborative research between NIH Institutes and Centers, between the NIH and other Federal agencies, and between NIH grantees and business partners

Management and organizing principles include an agency which is small, flexible, entrepreneurial, and non-hierarchical; which empowers portfolio managers to foster research opportunities free from bureaucratic impediments; which seeks to employ the strongest scientific and technical talent in the Nation; which rotates a significant portion of the staff every 3-5 years, which leverages comparable matching investment from other NIH institutes and centers, federal agencies, and from the private and non profit sectors; which creates a translational research model that supports fundamental research breakthroughs, early and late stage applied development, prototyping, knowledge diffusion, and technology deployment; which establishes metrics to evaluate research success; which ensures that

revolutionary research dominates HARPA's agenda and portfolio. Other management and organizing principles are provided.

HARPA activities will include supporting basic and applied research to promote revolutionary technology changes which address health needs. It will advance the development, testing, evaluation, prototyping and deployment of critical health products. Multiple other activities are provided

HARPA will have flexible hiring practices as described in the Strom Thurmond National Defense Authorization Act, 1999.

HARPA will have the authority to flexibly fund projects, including the prompt awarding, releasing, enhancing and withdrawal of monies

HARPA will be funded through the Director's acceleration fund.

Subpart 3 - Clinical Trials

Clinical trials are an essential part of the research and development process. This is where the effectiveness and safety of products are scientifically and systematically investigated. However, clinical trials are complex, expensive, and time-consuming, making it difficult for individuals to perform all the functions necessary to successfully organize and implement clinical trials. This subpart improves how clinical trials are conducted and how their results are disseminated. It also promotes the development of a future clinical research workforce.

Section 499E. Increasing Research Study Participation: The ACC shall create a national electronic clinical trial registry with the National Library of Medicine (NLM) that will announce NIH-funded clinical trials operating throughout the country and will encourage private sector participation in this registry. Special attention will be given to minority groups, who are frequently underrepresented in clinical trials.

Section 499E-1. Grants for Quality Clinical Trial and Execution: The ACC shall provide grants for clinical trial design and execution to academic centers or to small private firms to fund multidisciplinary clinical research teams, whose members may include project managers, clinicians, epidemiologists, and nursing staff.

Section 499E-2. Streamlining the Regulatory Process Governing Clinical Research: This section streamlines the regulatory process governing clinical research, which has become increasingly unwieldy due to necessary but complex patient privacy and safety rules. The ACC shall establish a series of Centralized Institutional Review Boards (CIRB) to ensure human subject safety and well-being for multi-institutional clinical trials. CIRB's shall be established in accordance with professional best practices and Good Clinical Practice (GCP) guidelines.

A CIRB shall be housed at the Institute or Center with expertise on the subject of the clinical trial or outside of the NIH in a public or private institution with comparable expertise and organizational capacity

CIRB's will be funded through user fees or Center funds

The CIRB shall act on behalf, in whole or in part, of the bodies ordinarily responsible for the safety of research subjects in a locality, on a contractual basis.

The CIRB will review and package research applications for facilitated electronic review by local IRB's participating in multi-center clinical trials. Local IRB review can be performed by a subcommittee that is empowered to make decisions in a timely manner. Local IRB's can either accept or reject the CIRB review.

Local IRB's which are part of the CIRB network shall be responsible for taking into consideration local characteristics such as educational level of research subjects to assure sound selection of research subjects and to minimize risks to vulnerable populations.

Each CIRB shall regularly communicate important information electronically to the local institutional review boards.

Section 499E-3. Training Clinical Researchers of the Future: The ACC will augment NIH's investment into programs developing the nation's clinical research workforce. These programs include: the NIH's Mentored Patient-oriented Research Career Development Award, NIH grants to help institutions develop curricula for clinical researchers, and NIH grants to fund participants in clinical science programs, which shall include but not be limited to clinical science certificates or clinical science Masters' Degrees.

Section 499E-4. Clinical Research Study and Clinical Trial: The Director shall commission the Institute of Medicine (IOM) to study the regulations protecting patient safety and anonymity so that in a contemporary clinical research context, a more realistic balance can be achieved between clinical research promotion and regulatory requirements governing research subject safety and privacy. The IOM will issue a written report within eighteen months of the passage of the CURES act which shall consider changes to the current Health Insurance Portability and Accountability Act (HIPAA) to further promote the clinical research endeavor.

Section 499E-5. Authorization of Appropriations for Sections 499E-1-4

Subpart 4 – Valley of Death

Small businesses are major drivers of innovation. Facile, motivated, numerous, and creative, these small businesses can extend the limits of R&D in way large companies with secure product lines are unable to do. However, small businesses often encounter difficulty securing capital in the so called, "Valley of Death" – the period between a research idea with possible application to the time the safety and efficacy of a product is demonstrated in human clinical trials. Common end-pathways within the Valley of Death include development of pharmacological assays, scale-up of production from lab-scale to clinical-trials scale, development of suitable formulations, evaluation of chemical stability, evaluation of materials testing for durability or reactivity, undertaking initial toxicology studies, and planning and implementation of clinical trials.

Section 499F. Small Business Partnerships: The Small Business Innovation Research (SBIR) and Small Business Technology Transfer (STTR) programs are effective major investments in promoting the R&D portfolios of small businesses. SBIR and STTR receive 2.5% and 0.3% of the budgets, respectively, of federal agencies with R&D budgets greater than $100 million dollars. SBIR/STTR grants

and contracts consist of three phases. Phase I plans for product development and procurement. Phase II addresses implementation of the plan. Phase III involves commercialization yet by law is ineligible for SBIR/STTR funding. Management and orientation of SBIR/STTR programs at the NIH can be improved.

This section moves the NIH's SBIR and STTR programs from the Extramural Research Office to the new Office of Bioscientific Enterprise Development (OBED) in the ACC Office of Technology Transfer (OTT).

The NIH currently awards its SBIR and STTR grants and contracts through a peer review process. Now, not less than 35% of SBIR and STTR grants and contracts shall be rewarded on a competitive basis by an OBED program manager with significant managerial, technical, and translational research experience to expertly assess the quality of a SBIR or STTR proposal.

Program managers will place special emphasis on partnering grantees with potential purchasers or investors of technology from the start of the research and development process with potential purchasers or investors including federal agencies such as the NIH.

ACC shall reduce the time between Phase I and Phase II funding to 6 months or less. Currently, grantees can wait up to 5 years to learn whether or not they are a recipient of a phase II grant.

An SBIR/STTR project manager may petition the OTT for Phase III funding from the Director's acceleration fund for projects requiring a supplementary funds to finalize product commercialization. The maximum funding for Phase III funding of a project shall be $2,000,000 for a maximum of 2 years.

All recipients of SBIR/STTR funding are required to report to the OTT whether there was eventual commercial success of the product. OTT shall keep a publicly accessible electronic record of all SBIR/STTR investments in research and development. The record shall include at minimum the following information: the grantee, a description of the funded research, the amount of money awarded in each phase of SBIR/STTR research, and if applicable, the nature of the products developed.

For each fiscal year, the two grants program managers who have had the greatest success in helping to commercialize products may be awarded a bonus up to $10,000.

Section 499F-1. Rapid Access to Intervention Development: The National Cancer Institute of the NIH has a successful translational research program called RAID (Rapid Access to Interventional Development). RAID lends essential expertise and resources to researchers outside of the NIH. OTT shall expand upon this program and establish other RAID programs, designed to accelerate the process of bringing promising and novel discoveries from the laboratory to the clinical trial stage.

RAID awardees have traditionally been selected to receive access to laboratories, facilities and other NIH supports for the pre-clinical development of drugs, biologics, and devices, using the peer review process. Now, not less than 35% of RAID awards shall be awarded on a competitive basis by a program manager with significant managerial, technical, and translational research experience to adequately assess the quality of a project proposal.

Eligible awardees include university researchers, non-profit research organizations, and firms of less than 100 employees in collaboration with one or more university or non-profit organizations.

The Office may discontinue support at any point when the entity fails to meet commercialization success criteria established by the Office.

Examples of RAID support are given. These include advice regarding the investigational new drug or investigational new device filing with the Food and Drug Administration

The Office shall not support products past proof-of-principle clinical trials.

Section 499F-2. Toxicity Studies: Toxicity studies are essential to the development of any drug therapy, but are difficult to stage. The Center for Cures shall support ongoing research into the most efficient methods of screening for human toxicity, including using cell-based and animal model technologies.

OTT may offer support for toxicity studies to private companies licensing NIH intellectual property.

Section 499F-3. Additional funding sources and models: The Director of the Center for Cures may provide acceleration funds for flexible contracts for translational research development to entities that license intellectual property from NIH where such contracts support innovation and commercialization.

Section 499F-4. Authorization of Appropriations for Sections 499F and 499F-1

Subpart 5 - Office of Technology Transfer

The Office of Technology Transfer (OTT) should be one of the NIH's most active entities. It is within the process of technology transfer where basic science research informs applications to health and where ideas are brought from bench to bedside and back to the bench. The OTT should be a library of innovation administered by experts who have experience in linking the translational research community with industry. This subpart improves upon the current research translation authorities of NIH's OTT.

Section 499G. Restructuring: The NIH Office of Technology Transfer in the NIH Director's Office shall be transferred to a new OTT Office in the American Center for Cures.

Section 499G-1. Marketing Function: The OTT office shall create a program for transfer management & support that cultivates industry interest in NIH funded research, reaches out to potential industry partners, coordinates patents from different NIH Institutes and Centers, and manages Cooperative Research and Development Agreements (CRADA's), biological licensing agreements, material transfer agreements, and intellectual property licensing.

To promote government-industry partnerships, the OTT shall create an electronic database within the National Library of Medicine that tabulates translational research efforts occurring at the NIH. The OTT shall hold an annual translational research conference the bring together public and private stakeholders

The OTT shall develop a program for transfer management & support which will be familiar with the NIH's intramural and extramural research portfolio as well as with the interests of small and large biotech and pharmaceutical industries. For those Institutes or Centers with their own OTT offices, the new OTT program for transfer management & support will work closely with those offices to coordinate industry outreach efforts.

As appropriate, OTT shall register CRADA's within a publicly accessible electronic database maintained by NLM

Section 499G-2. Office of Intramural Risk Opportunity and Mapping: An Office of Intramural Risk Mapping within OTT shall oversee the intramural research programs of the NIH to be certain they are complementary, non-duplicative, and distinct from extramural and private programs.

The Office shall identify and map health risks and scientific opportunities and update the data on these topics as necessary to ensure they are current. This information is to be provided to the Cures Council on a biannual basis to help them prioritize the nation's translational research investment

The Office shall make funds available to groups of NIH Institutes and Centers to promote multidisciplinary projects that focus on health risk analysis and corresponding scientific risk opportunity. Preference will go to projects that demonstrate a high degree of collaboration and which address diseases with the great burden or research promise, and that are most likely to result in the development of a therapeutic prototype.

Funding for the Office will come from the Director's Acceleration Fund.

Section 499G-3. Patenting and Licensing Incentives: The OTT shall make every effort to increase licensing to stimulate the availability of products for clinical use. The OTT shall recommend to the Director incentives that create private sector, financial, commercial, and academic interest in the NIH's IP portfolio. These incentives may include extensions of NIH health patents, restoration of NIH health patents, and partnering options to pursue exclusive and nonexclusive licensing to one or multiple partners in the government, industrial, and/or academic sectors.

The Director shall encourage OTT to develop flexible models for contracts that fulfill the needs of industry and the public.

Section 499G-4. Translational Researcher Development: The Director shall oversee development of a curriculum for internships in translational research encompassing rotations through multiple NIH Institutes and Centers, the clinical trial design process, the NLM, and other related disciplines with an emphasis on practical experience.

Tuition grants for extramural translational research programs shall be administered under the supervision of the Director.

The ACC shall train interdisciplinary scientists in the science of risk analysis & mapping through a program of internships and fellowships.

Section 499G-6. Translational Research Training Program: The NIH Director shall ensure that each NIH Institute or Center establishes a translational research training program.

Subpart 6 - Developing Information Systems

The NIH's National Center for Biotechnology Information (NCBI) at the NLM provides essential information resources to scientists worldwide and is the underpinning of much of NIH conducted biomedical research. The NCBI's databases and computational and linkage tools nurture information

sharing and are critical to identifying interconnections, developing insights, and accelerating biomedical breakthroughs.

Section 499H. Advancing National Health Information Infrastructure: The ACC and the NLM shall construct a clinical study registry and clinical results database tracking all NIH-funded clinical trials taking places in the United States and will encourage private sector participation in the registry. This registry and database will expand upon the NLM's current information system and database.

The registry of clinical trials shall include at least the following: clinical trial title, description of the product under study, the hypothesis to be tested, phase of the trial, brief description of the intervention, the study design, duration and location, participation criteria, contact information for the principal investigator, and sponsoring organization.

The databank of clinical trial results shall consist of at least the following: a summary of the results of the trial in a standard, non-promotional summary format, summary data tables with respect to the primary and secondary outcome measures, information on the statistical significance of the results, publications in peer reviewed journals relating to the trial, and a description of the process used to review the results of the trial, including a statement about whether the results were peer reviewed scientists independent of the trial sponsor.

An entity shall register a clinical trial not later than 3 months after submitting the clinical trial protocol to the Food and Drug Administration (FDA) and report clinical trial results not later than 3 months after completing the trial.

Penalties for not registering clinical trials can be loss of federal funding or in cases where a private entity does not receive federal funding, a fine of up to $1,000,000 dollars.

Section 499H-1. Publication Requirement for Research: The Director of the NIH shall require, pursuant to the government purpose license provision 45 CFR 74.36 that for any research funded by the NIH, there will be a standardized report of this research for public viewing. Department of Health and Human Services (DHHS) grantees shall provide the NLM an electronic copy of the final version of all peer-reviewed manuscripts accepted for publication for display on their digital library archive, PubMed Central, within 6 months from the date of its publication.

Failure to submit required information to the NLM within 6 months from the date of publication may result in loss of public funding for federal employees or grantees.

Section 499H-2. Informatics Training and Workforce Development. 21st Century technologies for analyzing DNA, RNA, proteins, and other biologically important molecules are generating a "tsunami of data" which are far beyond the understanding of unaided human cognition, but hold the key to improved understanding of human health and disease. Training of individuals in "clinical bioinformatics" -- translational research that applies computerized analytic methods of molecules, cells, tissues, and body systems to the prevention, diagnosis and treatment of human disease -- will be pivotal to fostering this emerging and important data-intensive field.

The NIH shall develop a multi-faceted approach to increasing the number of persons trained in clinical bioinformatics. This shall include but not be limited to augmenting secondary school science programs, undergraduate degree programs in Bioinformatics, NIH bioinformatics graduate training programs, and Centers of Excellence in Clinical Bioinformatics.

Authorization of Appropriations

Section 499H-3. NLM Expansion of Facilities. In 2002, Congress authorized an expansion of the NLM. These facilities may be essential to the NLM's capacity to fill its numerous informatics functions. The Director will commission the IOM to report to Congress on the impact of not funding the expansion of facilities.

Subpart 7 - Research Tools

Innovation requires proper tools for discovery. These include animal models that can be surrogates for human systems and markers that illuminate otherwise invisible cells, DNA, proteins and viruses. Arguably, the development of research tools is subject to the same market forces as more common end products -- drugs, medical devices, and vaccines.

Section 499I. NIH Research Tool Inventory: The Director of NIH shall direct the head of each NIH Institute and Center to perform an annual review of its research tool inventory for the specific purpose of enabling each Institute and Center to understand processes for research tool distribution, frequency of use, IP status, and utility. Each NIH Institute and Center shall also describe in its review the type and quantity of research tools it desires to obtain in order to better fulfill its R&D goals.

The ACC shall enter this inventory into an electronic research tool database and use this database to oversee the prioritization and funding of new projects to fulfill pressing needs and to encourage promising technologies.

Section 499I-1. Exceptions to Tool Guidelines: The Director of NIH may advise the OTT to provide exceptions to prohibition against patenting and licensing research tools under some appropriate circumstances when exclusive or non-exclusive licensing provides the swiftest, and most efficacious final development of an important health care technology.