21st Century Cures Act

What is it and How to Operationalize it

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Disclosures

I have no conflicts of interest to disclose.

The contents of this presentation are my own and do not necessarily reflect the views and/or policies of Matrix Clinical Trials or Matrix Medical Network.

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Objectives

- Describe what is the 21st Century Cures Act
- Describe the 2020 amendment Information Blocking
- Describe how it affects Clinical Research Operations

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Introduction

- The 21st Century Cures Act was signed into law December 13, 2016
- To accelerate the discovery, development, and delivery of 21st century cures
- In 2020, an amendment was approved to address exemptions in data sharing
- V2.0 currently in draft

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Accelerating Research

- Originally the 21st Century Cures Act provided NIH with the flexibility and resources needed to accomplish its mission to improve the health of Americans.
- The Cures Act implemented measures to:
  - alleviate administrative burdens that can prolong the start of clinical trials
  - allow researchers to more easily attend scientific conferences where in-person collaboration can often lead to scientific breakthroughs
  - enhance data sharing among NIH-supported researchers
  - improve privacy protections for research volunteers
  - encourage inclusion of diverse populations represented in clinical research
  - open up new NIH funding opportunities for young investigators

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Innovative Funding

Allocates funding to NIH over each of the next 10 years (starting 2017), for a total of **$4.8 billion.**

All of Us Research Program
https://allofus.nih.gov/

Brain Research through Advancing Innovative Neurotechnologies (BRAIN) Initiative
https://braininitiative.nih.gov/

The Cancer Moonshot
https://www.cancer.gov/research/key-initiatives/moonshot-cancer-initiative

Regenerative Medicine Innovation Project
https://www.nih.gov/rmi

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Advancing Precision Medicine

DHHS is encouraged to carry out a Precision Medicine Initiative to address disease prevention, diagnosis, and treatment.

DHHS must implement secure data sharing and ensure inclusion of a broad range of participants.

The bill revises provisions regarding disclosure by researchers of the identifiable, sensitive information of research subjects.

DHHS must prohibit researchers from disclosing such information from federally funded research to persons not connected to the research

DHHS may exempt identifiable information collected for biomedical research from disclosure under the Freedom of Information Act.

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Advancing Precision Medicine

Researchers may remotely access protected health information if security and privacy safeguards are maintained and the information is not retained.

DHHS must:
Issue guidance regarding an individual's authorization to use protected health information for future research, and

Convene a working group to study the use of protected health information for research.

The NIH may require recipients of grants or cooperative agreements to share scientific data.

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Provisions Directly Related to Clinical Research

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Patient-Focused Drug Development

Amends the Federal Food, Drug, and Cosmetic Act to require the FDA, after approving an application for a new medication, to publish a brief statement on any patient experience data or related information that was part of the application.

Patient experience data is information about the impact of a medical condition or a related therapy on a patient's life and the patient's preferences for treatment.

The FDA must report on its use of patient experience data in regulatory decision-making.

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Patient-Focused Drug Development 2.0 - Draft

Shall require drug manufacturers/ sponsors to collect and report on patient experience data as part of the clinical trial

Shall require FDA to fully consider all patient experience data collected during the clinical trial; and

Shall require reporting of patient experience data in a transparent manner that is uniform, meaningful and informative to patients and providers

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Diversity in Clinical Trials 2.0 - Draft

- Requires an update from FDA on efforts to improve diversity in clinical trials
- Requires a GAO study on barriers to clinical research participation
- Requires DHHS to conduct a public awareness campaign to increase awareness and understanding, particularly in minority communities, of clinical trials
- Establishes a task force on making clinicaltrials.gov more user- and patient-friendly
- Enhancing the Diversity of Clinical Trial Populations - Eligibility Criteria, Enrollment Practices, and Trial Designs Guidance for Industry

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Trial Design and Regulations 2.0 – Draft

- DHHS shall provide grants in area of innovative clinical trial design and patient experience data
- Shall require DHHS to submit report to Congress re: current state of cell and gene therapy regulation and challenges for FDA in the future

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Centers for Medicare and Medicaid Services 2.0 – Draft

- Shall allow Medicare to cover the costs of their beneficiaries in PCORI-funded studies
- Shall establish an automatic communication requirement between FDA and CMS for breakthrough therapy drugs
- Shall require a GAO report on recommendations to enhance Medicare coverage and reimbursement for innovative health technologies
- Shall provide guidance to states on effectively integrating telehealth services (TIKES and CHIP)
- Codify the current Medicare Coverage of Innovative Technology pathway at CMS
- Shall require DHHS to submit report on current capabilities and deficiencies of CMS's computer systems
- Shall increase access to genetic diagnostics for pediatric patients with rare diseases
- Shall require DHHS to provide pilot grant within CMS to test approaches to delivering personalized-medicine consults

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21st Century Cures Act 2.0 – Draft

- Shall authorize the creation of Advanced Research Projects Agency for Health (ARPA-H)
  - To speed transformational innovation in health research
  - To speed application and implementation of health breakthroughs
- Shall provide **$25 Billion** to independent research institutions, public laboratories and universities throughout the country to continue work on thousands of federally-backed projects

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Advancing New Therapies

Food and Drug Administration

The FDA must establish a process to qualify drug development tools (methods, materials, or measures that aid drug development and regulatory review) as reliable for use in supporting approval or investigational use of a drug.
Drug Development Tool Guidance
Medical Device Development Tool Guidance

The FDA may permit the sponsors of new medications that target genes or variant proteins to treat rare, serious conditions to rely upon information submitted for an approved medication that uses the same technology.

This bill amends the Orphan Drug Act to authorize DHHS to defray all the costs of development of orphan drugs (drugs for rare conditions), instead of only certain testing expenses.

DHHS may award grants to institutions of higher education and nonprofits to study and recommend improvements to the process of continuous manufacturing of medications. (Currently, most medications are manufactured in batches.)

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Modern Trial Design and Evidence Development

Food and Drug Administration

The FDA must issue guidance addressing the use of novel clinical trial design in the development and review of drugs.
Oncology
Innovative Designs for Drugs and Biologicals
Adaptive Designs - Drugs and Biologicals
Adaptive Designs - Medical Devices

DHHS must revise the Human Subject Regulations, the FDA Human Subject Regulations, and the vulnerable populations rules to:

- Reduce regulatory duplication and unnecessary delays;
- Modernize the provisions; and
- Protect vulnerable populations, incorporate local considerations, and support community engagement.

The FDA must evaluate and issue guidance on the use of evidence from sources other than clinical trials to support approval of a drug for a new indication.
FDA Guidances

Clinical testing of investigational medical devices and drugs no longer requires the informed consent of the subjects if the testing poses no more than minimal risk to the subjects and includes safeguards.

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Patient Access to Therapies and Information

Food and Drug Administration

Upon request, the FDA must facilitate development and expedite review of regenerative advanced therapies, including cell therapies, therapeutic tissue engineering products, and human cell and tissue products. For a therapy to be eligible, there must be preliminary clinical evidence that the therapy has the potential to address an unmet medical need for a serious condition.

Health care economic information provided to an entity selecting medications for coverage or reimbursement, (e.g., formulary committee of a health insurer), must describe any differences between the information provided regarding a medication and the FDA-approved labeling for that medication.

For certain indications, the FDA may rely upon a summary of clinical data to approve a supplemental application for a medication.

The manufacturer or distributor of an investigational drug for a serious condition must publish a policy for compassionate use of the drug.
FDA Guidance - Individual Patient Expanded Access

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21st Century Cures Act 2.0 – Draft

Food and Drug Administration

- Shall direct DHHS to establish two (2) additional FDA Centers of Excellence
- Shall not require IND application to initiate accelerated approval
- Shall allow use of other evidence to fulfill post-approval study requirements to confirm predicted clinical benefit
- Shall require FDA to publish guidance on the standards and factors for CMC data development and review

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Antimicrobial Innovation and Stewardship

Food and Drug Administration

- DHHS must:
  - Annually publish information on antimicrobial resistance and antimicrobial stewardship;
  - Disseminate guidance and materials regarding antimicrobial stewardship;
  - Continue working with state and local public health departments on antimicrobial resistance programs; and
  - Collect, evaluate, and publish data from the antimicrobial stewardship activities of health care facilities.
- The FDA may, at the request of the drug's sponsor, approve an antibiotic or antifungal drug for use in a limited population if the drug is intended to treat a serious infection in a limited population of patients with unmet medical needs. The FDA's determination of the safety and effectiveness of such a drug must reflect the drug's use in the intended limited population.
- The FDA must identify and publish susceptibility test interpretive criteria for antimicrobial drugs.

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21st Century Cures Act 2.0 – Draft

Developing Antimicrobial Innovations

Include Pioneering Antimicrobial Subscriptions to End Up surging Resistance (PASTEUR) Act

Establish a subscription model to pay for critically-needed novel antimicrobial drugs

Invest in programs to address antimicrobial resistance including Federal payment to companies

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Medical Device Innovations

- Upon a sponsor's request, the FDA must determine whether a device meets the criteria for designation as a breakthrough device.
- The humanitarian device exemption is expanded to allow the FDA to exempt from effectiveness requirements certain medical devices intended to benefit up to 8,000 individuals (previously up to 4,000 individuals).
- Certain software is exempted from requirements for medical devices, including software that provides medical recommendations and the basis for those recommendations to health care professionals.
- Software remains subject to regulation as a medical device if:
  - The software acquires, processes, analyzes, or interprets medical information; or
  - FDA identifies use of the software as reasonably likely to have serious adverse health consequences.
- When assessing a medical device that includes a software function exempted from medical device requirements, the FDA may assess the impact of the software on the functioning of the device.

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Improving Scientific Expertise and Outreach

Food and Drug Administration

- Provides new authority to help FDA improve the ability to recruit and retain scientific, technical, and professional experts and it establishes new expedited product development programs, including:
  - –The Regenerative Medicine Advanced Therapy that offers a new expedited option for certain eligible biologics products.
  - –The Breakthrough Devices program, designed to speed the review of certain innovative medical devices.

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Medical Countermeasures Innovation

DHHS must ensure the issuance of timely and accurate guidelines regarding the use of medical products for countering public health emergencies or material threats.

The Biomedical Advanced Research and Development Authority's (BARDA's) procurement of medical countermeasures no longer requires Presidential approval or an agreement between DHHS and the Department of Homeland Security.

The Paperwork Reduction Act does not apply to voluntary collection of information during a public health emergency or while determining whether there is a public health emergency.

The bill revises provisions regarding FDA authorization of emergency use of unapproved products to include animal drugs and veterinary feed directive drugs.

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21st Century Cures Act 2.0 – Draft

COVID-19

DHHS to survey COVID-19 patients for sources of health coverage, long-term care coverage and disability coverage.

DHHS to convene national virtual meetings with key sector representatives to serve an ongoing long-COVID learning collaborative.

National strategy to address testing, data sharing, administration of vaccines and therapeutics, and medical supply readiness to mitigate future pandemic and public health emergencies.

DHHS to develop plan to help rare disease patients overcome challenges in public health emergencies.

Establishes a federal grant program for organizations to implement the above plan.

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Vaccine Access, Certainty and Innovation

- The Advisory Committee on Immunization Practices must:
  - Consider the use of newly licensed vaccines at each regularly scheduled meeting, and
  - Make timely recommendations for vaccines designated as breakthrough therapies and vaccines that could be used in a public health emergency.
- The CDC must review the processes of the Advisory Committee on Immunization Practices.
- DHHS must:
  - Report on ways to promote innovation in the development of vaccines, and
  - Revise the Vaccine Injury Table to include information on vaccines recommended by the CDC for pregnant women. A mother and child are individually considered for compensation for a vaccine injury from a vaccine administered during pregnancy.

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21st Century Cures Act 2.0 – Draft

- Improve the education of all Americans on the importance of vaccines

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Information Delivery

- Amends the Health Information Technology for Economic and Clinical Health Act (HITECH) to require DHHS to establish a goal, develop a strategy, and make recommendations to reduce regulatory or administrative burdens relating to the use of electronic health records (EHR).
- Requires developers of health IT, for their health IT to be certified, to meet certain requirements, including that the developer not engage in **information blocking**, which is preventing, discouraging, or interfering with the access, exchange, or use of information.

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21st Century Cures Act 2.0 – Draft

- DHHS to submit report to Congress on the efforts to ensure collaboration and alignment across the centers and offices of the FDA with respect of regulation of digital health technologies

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Information Blocking

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Definitions

- **Access** – is the ability or means necessary to make electronic health information (EHI) available for exchange, use, or both.
- **Actors** – defined as:
  - Healthcare providers (Hospital, Long-term care facility, Medical practice, Laboratory, Pharmacy, Healthcare worker/provider)
  - Health Information Network or Health Information Exchange
  - Health IT Developer of Certified IT
- **Exchange** – is the ability for EHI to be transmitted between and among different technologies, systems, platforms, or networks; and it is inclusive of all forms of **transmission such as bi-directional and network-based transmission**
- **Information Blocking** – Business, technical and organizational practices that prevent or materially discourage the **access, exchange or use** of electronic health information when an Actor knows, or (for some Actors like EHR vendors), should know, that these practices are likely to interfere with access, exchange or use of EHI.
- **Use** – is the ability for EHI to be understood and acted upon once accessed or exchanged. "Acted upon" includes the ability to read and write and it is also bi-directional

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Exceptions

1. Preventing Harm
2. Privacy
3. Security
4. Infeasibility
5. Health Information Technology
6. Content and Manner
7. Fees
8. Licensing

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Preventing Harm Exception

- It will not be information blocking for an actor to engage in practices that are reasonable and necessary to prevent harm to a patient or another person, provided certain conditions are met
- Key Conditions of the Exception
  - The actor must hold a reasonable belief that the practice will substantially reduce a risk of harm;
  - The actor's practice must be no broader than necessary;
  - The actor's practice must satisfy at least one condition from each of the following categories:
    - type of risk
    - type of harm
    - implementation basis
  - The practice must satisfy the condition concerning a patient right to request review of an individualized determination of risk of harm.

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Privacy Exception

- It will not be information blocking if an actor does not fulfill a request to access, exchange, or use EHI in order to protect an individual's privacy, provided certain conditions are met.
- To satisfy this exception, an actor's privacy-protective practice must meet at least one of the four sub-exceptions:
  - Precondition not satisfied: If an actor is required by a state or federal law to satisfy a precondition (such as a patient consent or authorization) prior to providing access, exchange, or use of EHI
  - Health IT developer of certified health IT not covered by HIPAA
  - Denial of an individual's request for their EHI consistent with **45 CFR 164.524(a) of the HIPAA Privacy Regulation**
    - These include psychotherapy notes and information compiled for use in a civil, criminal or administrative legal proceedings, information obtained by a non-healthcare provider.
  - Respecting an individual's request not to share information: An actor may choose not to provide access, exchange, or use of an individual's EHI if doing so fulfills the wishes of the individual, provided certain conditions are met.

45 CFR Part 164.524(a) states:

(a) Standard: Access to protected health information

(2) Unreviewable grounds for denial – A covered entity may deny an individual access without providing the individual an opportunity for review

(iii) An individual's access to protected health information created or obtained by a covered health care provider in the course of research that includes treatment may be **temporarily** suspended for as long as the research is in progress, provided that the individual has agreed to the denial of access when consenting to participate in the research that includes treatment, and the covered health care provider has informed the individual that the right of access will be reinstated upon completion of the research.

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Security Exception

- It will not be information blocking for an actor to interfere with the access, exchange, or use of EHI in order to protect the security of EHI, provided certain conditions are met.
- The practice must be:
  - Directly related to safeguarding the confidentiality, integrity, and availability of EHI;
  - Tailored to specific security risks; and
  - Implemented in a consistent and non-discriminatory manner.
- The practice must either implement a qualifying organizational security policy or implement a qualifying security determination.

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Infeasibility Exception

- It will not be information blocking if an actor does not fulfill a request to access, exchange, or use EHI due to the infeasibility of the request, provided certain conditions are met.
- The practice must meet one of the following conditions:
  - Uncontrollable events: The actor cannot fulfill the request for access, exchange, or use of electronic health information due to a natural or human-made disaster, public health emergency, public safety incident, war, terrorist attack, civil insurrection, strike or other labor unrest, telecommunication or internet service interruption, or act of military, civil or regulatory authority.
  - Segmentation: The actor cannot fulfill the request for access, exchange, or use of EHI because the actor cannot unambiguously segment the requested EHI.
  - Infeasibility under the circumstances: The actor demonstrates through a contemporaneous written record or other documentation its consistent and non-discriminatory consideration of certain factors that led to its determination that complying with the request would be infeasible under the circumstances.
- The actor must provide a written response to the requestor within 10 business days of receipt of the request with the reason(s) why the request is infeasible.

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Health IT Performance

- It will not be information blocking for an actor to take reasonable and necessary measures to make health IT **temporarily** unavailable or to degrade the health IT's performance for the benefit of the overall performance of the health IT, provided certain conditions are met.
- The practice must:
  - Be implemented for a period of time no longer than necessary to achieve the maintenance or improvements for which the health IT was made unavailable or the health IT's performance degraded;
  - Be implemented in a consistent and non-discriminatory manner; and
  - Meet certain requirements if the unavailability or degradation is initiated by a health IT developer of certified health IT, HIE, or HIN.

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Content and Manner Exception

- It will not be information blocking for an actor to limit the **content** of its response to a request to access, exchange, or use EHI or the **manner** in which it fulfills a request to access, exchange, or use EHI, provided certain conditions are met.
- On and after 24 months after the publication date of the Cures Act final rule, an actor must respond to a request to access, exchange, or use EHI with EHI as defined in § 171.102 (definitions).
- An actor may need to fulfill a request in an alternative manner when the actor is:
  - Technically unable to fulfill the request in any manner requested; or
  - Cannot reach agreeable terms with the requestor to fulfill the request.

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Fees Exception

- It will not be information blocking for an actor to charge fees, including fees that result in a reasonable profit margin, for accessing, exchanging, or using EHI, provided certain conditions are met.
  - Be based on objective and verifiable criteria that are uniformly applied for all similarly situated classes of persons or entities and requests.
  - Be reasonably related to the actor's costs of providing the type of access, exchange, or use of EHI.
  - Not be based on whether the requestor or other person is a competitor, potential competitor, or will be using the EHI in a way that facilitates competition with the actor.

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Licensing Exception

- It will not be information blocking for an actor to license interoperability elements for EHI to be accessed, exchanged, or used, provided certain conditions are met
- The practice must meet:
  - The negotiating a license conditions: An actor must begin license negotiations with the requestor within 10 business days from receipt of the request and negotiate a license within 30 business days from receipt of the request.
  - The licensing conditions:
    - Scope of rights
    - Reasonable royalty
    - Non-discriminatory terms
    - Collateral terms
    - Non-disclosure agreement
  - Additional conditions relating to the provision of interoperability elements.

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Thank you

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