21st CENTURY CURES 2.0

TITLE I: PUBLIC HEALTH

Section___: National Testing and Response Strategy for Current and Future Pandemics

Challenge

We must improve our nation's surveillance and testing capabilities to support the U.S. response to this and future pandemics. Capabilities and strategies for testing, data sharing infrastructure, vaccine, therapeutic and other medical supply readiness can help win the battle against COVID-19 and future pandemics. While we applaud the testing provisions in recently-passed legislation, we need a more comprehensive set of actions now, and once the COVID-19 battle is won, we must ensure that efforts taken today can serve as a foundation for unique strategies and efforts to address future pandemics.

Policy: The President, acting through the Secretary of Health and Human Services, shall be required to implement a national testing, data sharing infrastructure, vaccine, therapeutic, and medical supply readiness strategy to mitigate current and future pandemics. The purpose of the strategy is to identify and implement the actions necessary to ensure the nation can win the battle against COVID-19 and future pandemics. Actions could include such things as:

- Testing strategies to help foster expedient results and personalized medical responses for patients and communities, including medically underserved populations and those in medically underserved areas. These strategies could include deploying point-of-care testing as well as testing at non-medical sites;
- Establishing and implementing methods for data sharing from testing to be used to inform surveillance and other pandemic monitoring and response efforts;
- Developing a plan to help get Americans back to work safely;
- Modernizing and expanding domestic drug manufacturing, including through the use of continuous manufacturing; and
- Establishing a plan to develop and administer vaccines and therapeutics.

This strategy will include specific actions that need to be taken, and deadlines for implementing such actions, to improve our nation's capabilities immediately.

Section ___: COVID-19 Rare Disease Support Program

Challenge

As our country battles the COVID-19 pandemic, one of the groups facing immense difficulties are financially vulnerable individuals with rare diseases. Given the unique nature of their diseases, they are the most vulnerable to provider and resource disruption and shortages, especially in rural areas. During this COVID-19 pandemic, these individuals and their families are facing unforeseen expenses related to disruptions to where they traditionally receive their treatments and disruptions of their critical support services, among others. Financially vulnerable rare disease patients do not have the ability to bear these additional costs.

Policy: This policy would help financially vulnerable individuals with rare diseases and their families by providing financial assistance for COVID-19-related expenses. By providing funds to a "COVID-19 Rare Disease Support Program" to help them with these unforeseen expenses, Congress could help ensure these individuals receive the care and support they need.

Section ___: Pandemic Preparedness Program for Patients

Challenge

During our battle with COVID-19, patients have faced enormous challenges because of provider and resource shortages. These shortages have caused numerous health challenges for patients and their families. Unfortunately, we know that once we win our battle against COVID-19, there will be future pandemics that could cause additional challenges for patients. We need to help patients be prepared when a pandemic or other public health emergencies happen so that we as a nation are best able to respond.

Policy: This policy would establish a federal grant program for organizations that work to provide support and other resources to help support access and the cost of medical care for patients and their families for this or a future pandemic. The grants would require eligible organizations to prepare a plan that identifies:

- the challenges faced during the COVID-19 pandemic;
- potential challenges during future pandemics;
- how they will overcome these challenges during the next pandemic;
- how their efforts can connect to local, state, and federal efforts in ways that promote cohesion; and
- what patients, their families, and their providers can do to ensure they can overcome those challenges.

Section ___: Improving U.S. Pandemic Preparedness and Response through Support of Antimicrobial Resistance Product Commercialization

Challenge

As current and past pandemics have shown, secondary infections that result from viral or other infectious diseases are one of the leading causes of death. The antibiotics that will be needed to treat patients suffering from bacterial and fungal infections are also largely responsible for the quality of care U.S. patients receive when not under pandemic conditions. The problem, quite simply, is that our ability to rely on the availability of these important medical products is lower today than it was even two years ago. Several medical product manufacturer bankruptcies over the last twelve months means that if nothing is done to reverse this trend, more company failures will follow as soon as this year.

The Administration, through agencies such as the ASPR and BARDA, have been working with industry to support new product development. However, these efforts have been constrained in the past by resources and a regulatory inability to collaborate with manufacturers in ways that promote the shared interests of all.

The original 21st Century Cures Act took steps to increase regulatory flexibility as it relates to the review and market approval of medical products. Now we need an initiative to address the commercial market failures of these FDA-approved products.

Policy: This provision would provide the Secretary of Health and Human Services, acting through the ASPR, the resources and regulatory authorities necessary to fix the commercial market for new antibiotics. In doing so, the Secretary shall ensure:

- that federal support can be provided for both pre-market and post market costs that can lead to company bankruptcy;
- that consideration is given to funding amount differentials that encourage both novel antibiotics and non-novel antibiotics that address a critical need;
- that a national strategy for how to get ahead of drug resistance in the future, including steps that can be taken to secure funding support from other countries and international bodies.

Section ___: Vaccine and Immunization Programs

Challenge

Vaccination is a proven public health success and a cost-effective strategy to help the immune system develop antibodies to protect individuals and communities from disease. Infectious diseases, despite some being vaccine-preventable, can travel quickly through a community and infect large numbers of people. As can be seen in the coronavirus

pandemic, it is imperative for communities to maintain high vaccination rates to foster "herd immunity" to protect against spreadable diseases.

Policy (Vaccine education): This policy would improve education of all Americans on the importance of vaccines. Controversies have raised concern about the safety of vaccines, from autism to polio vaccine contamination. Hysteria surrounding vaccine-associated risks has resulted in a declining number of vaccinations in developed countries.

Policy (Vaccine surveillance): This policy would strengthen and support the capacity of the Immunization Information System (IIS) within the Centers for Disease Control and Prevention. CDC's IIS is a confidential, population-based, computerized database that:

- Creates or supports effective interventions such as client and provider reminder systems;
- Determines client vaccination status for decisions made by health care providers, local health departments, and schools.

TITLE II: CAREGIVER INTEGRATION

Section ___: Educational programs and training for caregivers

Challenge

The most important site for receiving care for many Americans is not an operating room or clinical trial site but actually at home. Millions of patients require care from an informal caregiver, often a close family member or friend, who provides essential care, often with no formal training or appropriate compensation. In talking with caregivers from our districts, they often discuss their need for additional education on how to best to provide care services for their loved one or friend. They also share their concern regarding where to find high quality educational resources and information they can trust. A place where they could find reliable educational resources would provide them with piece of mind and help them provide better care to the family member or friend for whom they are providing care.

Clinical visits and interventions can also be extended with proper training for caregivers on how to deliver the interventions at home, maximizing health outcomes and keeping costs low. However, coverage of caregiver skills training does not exist and educational resources for caregivers are limited and not uniformly available. Lack of proper at home interventions can result in patient injury and overutilization of hospital stays. Further, hospitals are often not the appropriate setting of care for the needs of patients. Research and evaluation into caregiver skills training as an effective health intervention is also lacking in the U.S.

Finally, our current battle against COVID-19 has shown just how critical these caregivers are and made the need for educational programs and training even more immediate.

Policy: This provision would authorize grants for educational programs and training for caregivers to learn skills which would allow them to augment a care team and complement, not compete with, a clinical visit. These educational programs would include specialized training in medication adherence and injections, complementary strategies to ensure adherence to physical and occupational therapy regimens, nutritional compliance, and other services provided in the home.

TITLE III: PATIENT ENGAGEMENT IN HEALTH CARE DECISION-MAKING

Section ___: Increasing Health Literacy to Promote Better Outcomes for Patients

Challenge

Improving health literacy and access to health information will improve outcomes. Why? Patients will better understand their diseases or conditions, and thus they will be empowered to become part of the decision-making process and take steps to get well. The COVID-19 crisis has shown us how critical improving health literacy and access to health information is.

Policy: This provision would require CMS to issue an RFI regarding ways the agency can work with federally subsidized health care program stakeholders to encourage and promote greater health literacy of individuals. This RFI will solicit feedback for:

- Identifying culturally competent, evidence-based interventions that have been proven to improve health literacy in populations served by these programs
- Health literacy approaches that can be used by Medicare or Medicaid plans or providers that has been proven or shows promise to reduce cost
- Health literacy approaches that can be used by Medicare or Medicaid plans or providers that have been proven to increase patient satisfaction or improve the quality of care for at-risk populations, including holistic and non-medication-based forms of care
- Suggestions for how CMS can encourage the use of proven health literacy interventions through its payment policies under Medicare or Medicaid
- Health Insurance Literacy (In network, deductive, co-insurance, co-pay, difference between payors, etc.
- Improved health literacy contributes to shared decision-making/alignment with patient values.

TITLE IV: CLINICAL TRIALS

Section ___: Diversity in Clinical Trials

Challenge

Diversity in clinical trials is essential to ensuring medical products are safer and more effective for patients. While some progress has been made, it is clear we have a long way to go. It remains essential that regulators, industry, patients and researchers look for ways to continue to improve diversity. To that end, we put forward the following policies.

Policy (Medicaid): One issue patients face is the cost associated with clinical trials, especially those who are the most vulnerable. This policy would ensure that Medicaid covers the routine care costs of clinical trial participation for enrollees with life-threatening conditions. The financial pain caused by the COVID-19 pandemic has made enacting this policy even more urgent.

Policy (Update FDASIA Action Plan): In Section 907 of the Food and Drug Administration Safety and Innovation Act (FDASIA), Congress required FDA to publish a report "addressing the extent to which clinical trial participation and the inclusion of safety and effectiveness data by demographic subgroups including sex, age, race, and ethnicity, is included in applications submitted to the Food and Drug Administration." As part of the report, Congress required FDA to conduct an analysis on demographic subgroups representation in clinical trials. Congress also required FDA to provide an action plan with recommendations on improving issues identified in the report. FDA published this report in 2013 and the action plan in 2014. The plan included 27 action items.

This provision would require FDA to provide an update on implementation of the items included in the action plan.

Policy (Awareness): Increasing awareness and understanding of clinical trials is another important component to address this challenge. Under this policy, Congress would require HHS to utilize its multiple programs and communication channels to increase awareness and understanding. As part of this requirement, HHS would be required to publicize clinicaltrials.gov and other clinical trial resources through communication channels such as the Medicare Explanation of Benefits.

Policy (Make Clinicaltrials.gov More User Friendly): Under this policy, HHS would be required to convene a task force on making clinicaltrials.gov more user- and patient-friendly. This task force must include NIH, FDA, academic researchers, and patient organizations to focus on the administrative burden and utility of this database for all stakeholders.

Section ___: Trial Sites at Care Sites

Challenge

Today, Medicare patients and others are not able to access some covered medical services at the trial site if they are involved in a clinical trial. The impact of these barriers to care at a trial site places a great strain on those patients who are using clinical trials for their own care. The current pandemic and shortages of providers and supplies only exacerbates these problems especially in rural areas of the country with fewer care options. More should be done to overcome these barriers and improve the care of patients currently in clinical trials.

Policy: CMS, in collaboration with the FDA and NIH, shall take steps to improve access to already covered medical services at clinical trial sites. In doing so, CMS shall seek the input of those who operate clinical trials in order to identify current barriers and ways to overcome them.

TITLE V: FDA

Section ___: Coordinated FDA Approach on Digital

Challenge: Digital health technologies, including software, data, wearable devices and mobile applications, provide extraordinary opportunities to improve the lives of patients, whether it is how they receive treatments, access information, participate in clinical trials or monitor their health. We took steps in the first Cures to foster the development of these technologies, including at FDA, through inclusion of the Software Act. Currently, there are multiple efforts at FDA to advance the vision of Cures on digital technologies. As policymakers, one thing we must do is ensure that the regulatory framework that the FDA is currently developing is collaborative and inclusive of the various centers within the agency. The goal is not to require one regulatory standard, but to seek areas where common regulatory considerations can be carried through all.

Policy: To address these issues and continue to move forward on digital technologies, this provision would require the FDA Commissioner to work with CDER, CBER and CDRH to establish collaborative regulatory alignment where advisable. In doing so, the FDA must include the following:

- How the Commissioner will coordinate the work of CDER, CBER and CDRH on digital technology issues;
- Guidance on:
    - The use of digital endpoints for regulatory review, including the validation and qualification of digital endpoints/biomarkers;
    - The acceptance of decentralized trials, including the use of digital health technologies;
    - The use of digital health technologies in patient-focused development of products; and
- Describe how FDA will coordinate with foreign regulators to ensure harmonization on the regulation and use of digital health technologies.

Section ___: FDA Grant-making Authority and Funding

Challenge

The original 21st Century Cures Act helped encourage the use of innovative clinical trial design and continue the utilization of patient-focused drug development. Through the efforts of the FDA, patients as well as stakeholders, advancements have been made but the science in these areas still needs to be further developed. Further developing the science would enable the drug development process to be more efficient and further centered on the "real world" needs of patients. As we have seen in the response to the COVID-19 pandemic, the utilization of innovative trial designs is critical as safe and effective treatments and vaccines are developed.

Policy: This provision would authorize funds to enable the Food and Drug Administration to provide grants in the area of innovative clinical trial design and patient-focused drug development to further build the science in these areas. Included in the area of innovative clinical trial design is complex adaptive, Bayesian, and other novel clinical trial designs. In the area of patient-focused drug development, further study on clinical outcome assessment and patient experience data and incorporation of digital health tools and real-world data/evidence should be prioritized.

Section ___: Increasing Use of Real-World Data/Evidence

Challenge

Data and optimal utilization and analysis of that data holds the promise of greater insights that can fuel the development of new cures. Fostering the development and utilization of real-world data (RWD) and real-world evidence (RWE) was a major priority of 21st Century Cures. To date, RWE has mainly been utilized within FDA to improve pharmacovigilance of approved drugs and to support label extensions of approved drugs. We are encouraged by FDA's efforts, but we believe RWE holds additional promise across other federal agencies. To speed up the promise of RWE use, we propose the following policies.

Policy (RWE in Breakthrough and Accelerated Approval): Congress established the Breakthrough Therapy Designation (BTD) in 2012 to expedite the development and review of drugs with substantial preliminary clinical activity that treat serious conditions with a large unmet medical need. At the same time, Congress expanded the Accelerated Approval pathway to allow drugs for serious conditions that filled an unmet medical need be approved based on an effect on a surrogate endpoint or an intermediate clinical endpoint that is reasonably likely to predict drug's clinical benefit. These policies have been a tremendous success, and we believe that further incorporating RWE could help build on that success.

Under this provision, FDA would be required to issue a guidance on utilizing RWE in BTD and Accelerated Approval products as part of post-approval commitments (e.g., additional clinical trials for special and underrepresented populations). Such topics may include:

- Acceptable endpoints and outcomes measures
- Data quality standards
- Data transparency requirements
- Study design considerations

Policy (Develop federal government approach): This policy would require the Secretary of Health and Human Services to establish a consistent and clear regulatory framework for the recognition and utilization of real-world evidence. The goals of the initiative shall be to:

- Outline a framework for the collection of consistent and usable RWD and
- Identify ways other federal agencies can use RWE.

Policy (Patients): Another essential policy is to ensure that patients are the center of RWE. To do this, the provision would require the establishment of a task force, comprised of patient groups, CMS, FDA and private sector representatives, to develop a list of recommendations on ways to encourage patients to engage in RWD generation.

Section ___: Improve FDA-CMS communication regarding transformative new therapies

Challenge

To ensure timely access to innovative products, we need to foster better communication and collaboration between the agency in charge of reviewing new product applications (FDA) and the agency in charge of making coverage determinations (CMS). Improving the communication and collaboration between these two agencies would afford CMS the opportunity to access additional expertise and insight when considering the functional aspects of a new product, including for purposes of innovative coverage design intended to best take advantage of a products ability to increase care quality and reduce costs.

One area where this communication and collaboration would be particularly valuable is the Breakthrough Therapy Designation, which applies to new products that treat a serious or life-threatening illness for which preliminary clinical evidence indicates that the drug may demonstrate a "substantial improvement over existing therapies." A vast majority of products receiving the Breakthrough Therapy Designation from FDA achieve successful approval; thus the period between when a designation is granted and the product is approved by FDA could provide an important opportunity to initiate the payment process.

Policy (FDA-CMS Coordination): This provision would establish an automatic communication requirement between FDA and CMS for products granted Breakthrough Therapy designations. The communication requirement would commence upon the grant of the designation and would continue through the collection of any RWE post-FDA-approval.

TITLE VI: CMS Modernization

Section ___: TBD

Challenge

The U.S. health care system today can be described as a system in transition. While alternatives to individual coverage and reimbursement rules are being tested within demonstration projects through such bodies as CMMI, the overall pace of modernization must be improved to keep pace with technological and scientific advances.

We are interested in efforts to modernize the entire coverage and care delivery system in the United States. For patients suffering from chronic or severe illnesses and conditions, technology advancements may hold the key to increasing the quality of their care and lowering costs. Advancements in the use of cell and gene therapies holds great promise for the future of patient care. Digital therapeutics hold hope that medical services can increase medical service access for patients wherever they may preside. Our increasing understanding and use of information hold the promise of creating personalized coverage and reimbursement approaches for various patient populations.

To best allow for new technologies and treatments to benefit patients, modern and systemic approaches to coverage and reimbursement needed. We understand that modernization efforts like the one we are contemplating need be carefully done so as to ensure that the considerations of all parties are balanced in ways that ultimately benefit patients.

We therefore would like additional feedback and ideas on the questions below and will consider all responses as we move forward in the coming months.

Questions to Support Possible Policy Development

1. General Coverage Modernization

    - Are current coverage and reimbursement rules for new medical products under federally-financed health programs that are outdated or in need of reform? If so, what are they?
    - Are the current coverage and reimbursement approaches to new medical products or other modern technologies adequate to keep up with the pace of innovation? If not, why?
    - What barriers and issues exist for patients who transition from private insurance to Medicare?

2. Cell and Gene Therapies

    - Are there barriers that impede or otherwise slow coverage for new cell and gene therapy products? If so, what are they?
    - Are there improvements that can be made under federally-financed health programs to improve coverage and patient access of these therapies?

3. Medical Products for Small Patient Populations

    - What are the biggest impediments to new cures development for these important populations?
    - What steps can policymakers take to address these impediments, if any?

4. Genomic Sequencing

    - Are there barriers that impede or otherwise slow coverage of genome sequencing?
    - Are there improvements that can be made under federally-financed health programs to improve coverage and patient access of genomic sequencing?

5. Breakthrough Coverage

- Are there barriers that impede coverage of technologies and therapies approved through FDA's breakthrough technologies and therapies pathways?
- How do we expedite coverage while at the same time ensuring that additional evidence can be collected?

TITLE VII: Technical Provisions

Section ___: Technical Provisions

Policy (Breakthrough and RMAT Designations): Congress authorized both the Breakthrough Therapy (BT) and the Regenerative Medicine Advanced Therapy (RMAT) designation programs at FDA with the goal of expediting the development and review of drugs that preliminary clinical evidence indicates could benefit patients living with serious or life-threatening diseases or conditions. However, FDA has taken the position that it will only review designation requests if there is an active investigational new drug (IND) application in place, despite the fact that a sponsor may choose not to submit an NDA for legitimate reasons (e.g. clinical studies occur in foreign countries due to small patient populations for rare disease). This policy would remedy this unforeseen impediment to sponsors which have collected scientifically valid preliminary clinical evidence outside of the U.S. to receive the benefits of these designations for investigational drugs that otherwise meet the BT and RMAT criteria.

Policy (Electronic Visit Verification): Section 12006 of 21st Century Cures (21CC) requires that all states mandate the use of electronic visit verification (EVV) systems for Medicaid-funded personal care services (PCS) and home health care services (HHCS). This policy would remove geo-tracking and biometrics within EVV systems to account for changes in service location and address privacy concerns.