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ASSOCIATION FOR CLINICAL ONCOLOGY
KNOWLEDGE CONQUERS CANCER

May 29, 2025

The Honorable Diana DeGette
2111 Rayburn House Office Building
Washington, DC 20515

Dear Representative DeGette,

The Association for Clinical Oncology (ASCO), representing more than 50,000 oncology professionals, appreciates your ongoing commitment to the Cures initiative including the release of your white paper, "A Roadmap for 21st Century Cures" in December 2024. Our members - clinical oncologists, researchers, and other oncology professionals - are dedicated to research that improves patient outcomes and ensuring access to evidence-based care. We are pleased to submit the following comments in response to the Cures 2.1 white paper.

Executive Summary

With over a decade of progress and measurable success, the Cures initiative has driven transformative policy changes. Its previous legislative efforts, the 21st Century Cures Act, significantly accelerated research toward new treatments. A notable outcome of previous initiative is the establishment of the Advanced Research Projects Agency for Health (ARPA-H), which ASCO strongly supports.

ASCO was pleased to respond to the stakeholder request in August 2024 that provided the foundation for the Cures 2.1 white paper.

ASCO's comments on the Cures 2.1 white paper are organized into three key areas: analysis of its focus areas, recommendations for inclusion, and discussion of previously unaddressed issues. We want to reiterate our strong support for policies directly aligned with ASCO's mission: conquering cancer through research, education, and the promotion of the highest quality patient care. Specifically, safeguarding ARPA-H's role as a research innovator, ensuring

accessible and representative clinical trials, and leveraging real-world data are critical to delivering innovative treatments to patients. While the Cures 2.1 white paper aims to significantly advance treatment and research, we encourage you to include two other issues of importance to the oncology landscape: drug shortages and advanced diagnostic testing. Finally, ASCO is contributing new insights regarding Medicare benefit categories for emerging therapies, including local and national coverage determinations and FDA approval processes.

Analysis of Cures 2.1 Focus Areas

ARPA-H

Since its inception, ASCO has supported the goals of the Advanced Research Projects Agency for Health (ARPA-H). We have closely followed the agency's progress over its initial years and anticipate the transformative impact of the high-risk, high-reward projects that will benefit cancer patients across the country. Specifically, funding high-risk, high-reward research fills a gap in our current biomedical research enterprise. The NIH has historically been able to fund research at specific sites, traditionally favoring incremental, hypothesis-driven research, and often basic research. Industry then frequently tackles more translational research including projects that yield lucrative returns on investment. A nimble federal agency, such as ARPA-H, can harness collaboration across multiple sectors, can pursue bold ideas without concern for maintaining a bottom line, and can complement efforts currently underway in our biomedical research enterprise. ARPA-H's unique approach to research is already proving its value in the cancer care space. Some current initiatives include the Advanced Analysis for Precision Cancer Therapy (ADAPT) program to improve treatment responses in tumor biology, the Sonodynamic Therapy for Hematologic Malignancies project to revolutionize cancer therapy with a minimal invasive ultrasound-based approach, and the Precision Surgical Interventions (PSI) for more precise tumor removal.

Congress established ARPA-H to complement NIH's pivotal role with a focus on end product-driven research and development, collaboration across scientific disciplines, and a public-private approach. As ARPA-H matures, we urge Congress to commit to consistent and appropriate funding levels. Sustained support is vital to fostering public-private partnerships, essential for expediting the delivery of groundbreaking innovations, as originally envisioned in Cures 2.0.

Telehealth

Telehealth has proven beneficial to patients and providers by increasing access to care for patients with cancer while reducing treatment burden and disruption to patients' lives. ASCO strongly supports lifting the geographic location and site origination restrictions and making these flexibilities the foundation of telehealth care for years to come. Telemedicine in oncology has been associated with cost savings for patients. An analysis of over 25,000 telemedicine visits by more than 11,600 adults with cancer found that the use of the technology saved patients between $147-$186 dollars per visit by limiting lost productivity and driving-related costs.

The benefits of telehealth include decreasing travel time for patients, potentially eliminating the need to take time off from work or find childcare, providing immediate access to care, which can lead to early detection of health issues and complications, supporting continuity of care, increasing patient autonomy, reducing caregiver burden, and increasing patient satisfaction.[1,2] Additionally, telehealth interventions in chronic disease management demonstrate better patient outcomes and can lead to a decline in hospital admissions/re-admissions, length of hospital stays, emergency department visits, and a reduction in mortality.[3,4] Virtual visits can also provide effective follow-up and enhanced convenience and communication compared with traditional office visits.

Access to Clinical Trials

ASCO has a long-standing dedication to improving access to and enrollment in clinical trials and will continue to advocate for legislation, such as the *Clinical Trails Modernization Act* (H.R.3521), that reduces barriers and makes clinical trials more representative of the population of cancer patients. Our dedication to broadening eligibility criteria for cancer clinical trials will continue as we work with the FDA and other stakeholders to implement

[1] Cox A, Lucas G, Marcu A, et al. Cancer survivors' experience with telehealth: a systematic review and thematic synthesis. J Med Internet Res. 2017;19:e11. https://pubmed.ncbi.nlm.nih.gov/28069561/
[2] Pennell NA, Dicker AP, Tran C, et al. mHealth:Mobile Technologies to Virtually Bring the Patient Into an Oncology Practice. 2017 ASCO Educational Book. https://ascopubs.org/doi/full/10.1200/EDBK_176093
[3]Gros DF, Lancaster CL, López CM, et al. Treatment satisfaction of home-based telehealth versus in person delivery of prolonged exposure for combat-related PTSD in veterans. J Telemed Telecare. 2016;1357633X16671096. Epub 2016 Sep 26 https://pubmed.ncbi.nlm.nih.gov/27672059/
[4] Bashshur RL, Shannon GW, Smith BR, et al. The empirical foundations of telemedicine interventions for chronic disease management. Telemed J E Health. 2014;20(9):769-800. doi:10.1089/tmj.2014.9981 https://pubmed.ncbi.nlm.nih.gov/24968105/

these recommendations, as outlined by the Society in two published manuscripts [5,6]. In the *Road to Recovery* report[7], ASCO recommended that researchers design more pragmatic and efficient clinical trials in order to "maximize learning from trial participants, increase generalizability of research results, integrate trial procedures more easily into standard clinical workflows, and reduce trial costs." Trial designs that closely follow routine care may make it easier for more oncology practices to participate in clinical research and expand patient access to clinical trials. Innovative trial designs, such as master protocols and adaptive designs, could be leveraged to improve trial efficiencies and ensure patients are enrolled in trials most likely to lead to clinically meaningful results. We believe achieving these goals will benefit the entire cancer care continuum.

Utilizing Real World Data in Clinical Setting

Continued efforts to explore and expand the use of Real-World Data (RWD) are important to ensuring data for all patient populations are included in the development of treatments. Sources of RWD include electronic health records, insurance claims, patient registries, and digital health solutions outside of conventional clinical trials. Additionally, RWD can supplement clinical trial data to establish real-world effectiveness and toxicity, especially in oncology.

Clinician-led clinical data registries are major sources of real-world data and are uniquely positioned to drive quality and effective research. When registry data is combined with Medicare, Medicaid, and State Children's Health Insurance Program (CHIP) claims data, registries can facilitate both quality improvement efforts and longitudinal studies. ASCO understands the potential for RWD to provide global benefits and is also aware of the need for thoughtful deployment and monitoring.

One area to consider exploring is federal legislation facilitating communication across the health information technology (HIT) continuum to improve the quality of patient care while protecting the security of data and patient privacy. Legislation should focus on addressing the current barriers to maximize the value of digital health care.

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[5]Kim, E. S., Bruinooge, S. S., Roberts, S., Ison, G., Lin, N. U., Gore, L., Uldrick, T. S., Lichtman, S. M., Roach, N., Beaver, J. A., Sridhara, R., Hesketh, P. J., Denicoff, A. M., Garrett-Mayer, E., Rubin, E., Multani, P., Prowell, T. M., Schenkel, C., Kozak, M., . . . Schilsky, R. L. (2017). Broadening eligibility criteria to make clinical trials more representative: American Society of Clinical Oncology and Friends of Cancer Research Joint Research Statement. *Journal of Clinical Oncology*, *35*(33), 3737–3744. https://doi.org/10.1200/jco.2017.73.7916

[6] Kim, E. S., Uldrick, T. S., Schenkel, C., Bruinooge, S. S., Harvey, R. D., Magnuson, A., Spira, A., Wade, J. L., Stewart, M. D., Vega, D. M., Beaver, J. A., Denicoff, A. M., Ison, G., Ivy, S. P., George, S., Perez, R. P., Spears, P. A., Tap, W. D., & Schilsky, R. L. (2021). Continuing to broaden eligibility criteria to make clinical trials more representative and inclusive: ASCO–Friends of Cancer Research Joint Research Statement. *Clinical Cancer Research*, *27*(9), 2394–2399. https://doi.org/10.1158/1078-0432.ccr-20-3852

[7] Nathan A. Pennell et al. American Society of Clinical Oncology Road to Recovery Report: Learning From the COVID-19 Experience to Improve Clinical Research and Cancer Care. JCO 39, 155-169(2021).
DOI:10.1200/JCO.20.02953

Interoperability must allow the sharing of data across the care continuum- among providers at small and large institutions, community practices, academic settings, imaging centers, laboratories, pharmacies, payers, researchers, and patients. Although adoption of electronic health records has improved the collection of clinical information, gaps in interoperability and structured health data continue to challenge the use of HIT.

Recommendations for Inclusion in Cures 2.1

ASCO strongly recommends consideration of the following critical issues during the development of the Cures 2.1 legislation.

Cancer Drug Shortages are Hindering Innovation

For more than a decade, the oncology community has experienced cancer drug shortages caused by a multitude of factors, including quality issues, manufacturer business decisions, disruptions to raw ingredients and excipient supplies, and natural disasters. Most oncology drugs in shortage are legacy, generic sterile injectables, many of which do not have alternatives, and impact both the pediatric and adult populations. The lack of predictability in the generic cancer drug supply chain can have negative impacts on patients with cancer, including disruptions or delays in treatment, potentially leading to irreversible disease progression.

Shortages are systemic and have long-lasting impacts beyond the immediate availability to patients. They can stifle innovation of future therapies that may be better than the traditional standard of care. On average, review and approval of new cancer therapies takes six months before the treatment becomes readily available to patients.[8] However, drug shortages regularly slow clinical trials of new treatments when those trials rely on the drugs in short supply as the standard of care, sometimes leading to institutions shutting down the trials altogether. According to a National Comprehensive Cancer Network survey conducted in 2024, 43% of cancer centers reported drug shortages negatively impacting clinical trials. The impacts included budgetary changes as the study teams attempt to order/secure product to reserve for trial enrollment, hesitation to open trials with drugs that have shortages, and delays opening some trials due to availability of standard of care drugs.[9]

[8] Lythgoe, M. P., Desai, A., Gyawali, B., et al. (2022). Cancer Therapy Approval Timings, Review Speed, and Publication of Pivotal Registration Trials in the US and Europe, 2010-2019. JAMA Network Open, 5(6), e2216183.
[9] National Comprehensive Cancer Network, Inc. & NCCN Best Practices Committee. (2024). New Information and Survey Results from June 2024. https://www.nccn.org/docs/default-source/oncology-policy-program/nccnbestpracticesdrugshortagesurvey.pdf?sfvrsn=b081351e_6

ASCO members have shared examples of sending patients to an academic center for a clinical trial because the trial was the only guaranteed way patients could get the drugs they needed. Normally patients enroll in trials to further research or gain access to new therapies. In this case, patients are feeling the need to participate in a trial just to receive basic treatment that might not otherwise be available.

In testimony from ASCO member Jason Westin, MD, MS, FACP, during the December 2023 Senate Finance Committee hearing titled Drug Shortages: Examining Supply Challenges, Impacts, and Policy Solutions from a Federal Health Program Perspective, Dr. Westin highlighted the impact a potential shortage of a drug called fludarabine, a crucial component of CAR-T cell therapy. Fludarabine is a cheap and generic drug, initially approved over 30 years ago, and it is an essential component for CAR-T cell therapy. CAR-T is a lifesaving, cutting-edge, "almost science fiction-like technology" that weaponizes the patient's own immune cells to fight their cancer by seeing the cancer cells, the wolf in sheep's clothing hiding in plain sight, but its efficacy is dependent upon being given with fludarabine. Unfortunately, fludarabine has no known effective substitutes.[10]

Recognizing the urgency of this issue in the context of cancer care and clinical trials, ASCO recommends a comprehensive approach to mitigating drug shortages that must encompass the following key recommendations:

- Address economic factors that drive generic manufacturers out of the market and consider stabilizing the market with long-term contracts and guaranteed prices.
- Reward reliable U.S. manufacturing of critical and supportive medications and raw materials through price stabilization and investment in continuous manufacturing or other advanced manufacturing for critical drugs and active pharmaceutical ingredients (APIs).
- Recognize potential shortages earlier by increasing the FDA's visibility into the supply chain so the agency can predict and respond to potential shortages earlier.
- Relay information about potential shortages to health systems and providers to help them prepare for and mitigate possible supply challenges.

[10] Westin, J. R., MD, MS, FACP & Association for Clinical Oncology. (2023). Drug Shortages: Examining Supply Challenges, Impacts, and Policy Solutions from a Federal Health Program Perspective. https://society.asco.org/sites/new-www.asco.org/files/JasonWestinMD-ASCO-WrittenStatement-12.05.2023-Hearing.pdf

Laboratory Developed Test Innovation

ASCO advocates for the inclusion of the *Verifying Accurate Leading-edge IVCT Development (VALID) Act*, which you introduced with Rep. Larry Bucshon in the 118th Congress, in future *Cures* legislation to meet the challenge of ensuring patients' access to advanced diagnostics. Reflecting years of engagement and collaboration between Congress and the stakeholder community, you reintroduced the bill in March 2023, and it had bipartisan support in Congress and broad support from the stakeholder community. It aimed to modernize the regulation of all Laboratory Developed Tests (LDTs) and in vitro diagnostic tests (IVDs) under a single framework under the FDA. ASCO believes there is a need for a flexible, risk-based regulatory system that would incentivize and improve the development of advanced, reliable tests. The *VALID Act* would be an important step forward for patients and their clinicians who expect the highest quality, innovative tests available to diagnose and treat illnesses. Absent these reforms, the current, outdated oversight of the development and quality of these tests will lead to inaccurate and unreliable tests and undermine clinical decision making and patient health outcomes.

Fostering Safe and Effective Artificial Intelligence (AI)

Driving innovation and adoption of AI will depend on researchers and clinicians having access to data that can be easily understood and explained. The limited ability to curate large, complex data sets could lead to poor performance of AI models. Information sharing, balanced appropriately with patient safety and data protection, is the key to truly utilizing AI's potential to derive new clinical insights and improve clinical outcomes, care coordination, and efficiency of care delivery. Federal legislation should facilitate the adoption of standards to promote and drive electronic data exchange while earning public trust. More incentives are needed to create the environment for this to occur.

In the absence of established government or regulatory oversight, ASCO has developed principles for the responsible use of AI in oncology.[11] Our principles highlight concerns with AI systems potentially being trained with limited, and/or poor-quality data sets, leading to inappropriate interpretations. Data reflecting inherent limitations or skewed representations can result in research conclusions that produce negative health [12] Additional guidance will be needed on developing the most appropriate methods, tools,

[11] American Society for Clinical Oncology. American Society for Clinical Oncology (ASCO) Principles for the Responsible Use of Artificial Intelligence in Oncology. https://society.asco.org/sites/new-www.asco.org/files/ASCO-AI-Principles-2024.pdf.
[12] Assistant Secretary of Planning and Evaluation. Office of Health Policy. Report: Trustworthy Artificial Intelligence (TAI) for Patient-Centered Outcomes Research (PCOR)
https://aspe.hhs.gov/sites/default/files/documents/1348a9a067fd4d225981a822dfe25ea5/trustworthy-ai.pdf

and training to provide clinicians with the opportunity to identify, assess, and mitigate inappropriate interpretations of the data.

Efforts are underway to develop AI model evaluation and validation through quality assurance lab testing to ensure the safety and effectiveness of AI models.[13] The need to preserve patient privacy will be very important with the use of AI. There is a greater, emerging need to support research on privacy enhancing technologies such as federated learning and differential privacy.[14,,,17]More incentives are needed for providers and researchers to promote interoperability of data sets across the HIT continuum, to actively explore techniques to help understand AI algorithms (to its extent possible), and to promote research on privacy enhancing technologies.

Additional Considerations

Benefit Categories for Emerging Therapies

ASCO supports the establishment of a pathway for immediate national Medicare coverage of FDA-market authorized breakthrough devices meeting the specified criteria and supports the voluntary, opt-in nature of these programs. In recent years, CMS proposed two possible pathways for authorization of these emerging therapies, the Medicare Coverage of Innovative Technology (MCIT) and Transitional Coverage for Emerging Technologies (TCET). ASCO supported the implementation of MCIT; however, ASCO did not support universal expansion of the newly proposed TCET pathway beyond breakthrough devices.

The MCIT coverage pathway was specifically for Medicare coverage of devices that are designated as part of the Food and Drug Administration's (FDA) Breakthrough Devices Program ("breakthrough devices") and are FDA market authorized. The MCIT pathway would have been voluntary and device manufacturers would notify CMS of their intent to utilize this coverage option. In contrast to the varied local coverage policies with between-MAC and within-MAC differences, the MCIT would have created a pathway for immediate

[13] Shah NH, Halamka JD, Saria S, et al. A Nationwide Network of Health AI Assurance Laboratories. JAMA. 2024;331(3):245–249. doi:10.1001/jama.2023.26930.
[14] Islam MM, Mohammed N, Wang Y, Hu P. Differential Private Deep Learning Models for Analyzing Breast Cancer. Omics Data. Front Oncol. 2022;12:879607. Published 2022 Jun 23. doi:10.3389/fonc.2022.879607.
[15] Karargyris, A., Umeton, R., Sheller, M.J. et al. Federated benchmarking of medical artificial intelligence with MedPerf. Nat Mach Intell 5, 799–810 (2023). https://doi.org/10.1038/s42256-023-00652-2.
[16] The White House. Advancing a Vision for Privacy-Enhancing Technologies. https://www.whitehouse.gov/ostp/news-updates/2022/06/28/advancing-a-vision-for-privacy-enhancingtechnologies.
[17] National Institute of Standards and Technology. U.S. Department of Commerce. NIST Special Publication. Guidelines for Evaluating Differential Privacy Guarantees. https://nvlpubs.nist.gov/nistpubs/SpecialPublications/NIST.SP.800-226.ipd.pdf.

national Medicare coverage of any FDA-market authorized breakthrough device if the device meets criteria outlined in the proposal.

While ASCO supports the establishment of a pathway for breakthrough devices (including diagnostics that are cleared/authorized/approved by FDA as medical devices), we believe that a pathway should address the current patchwork of local coverage and could enhance access nationally for Medicare beneficiaries, thereby increasing clarity and consistency of coverage.

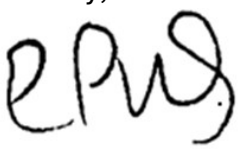
Local Coverage Determination Reform

Reforms to the Medicare local coverage determinations (LCDs) process are necessary to ensure that LCDs do not impede a physician's medical judgment. To fulfill the letter and spirit of the LCD program, the coverage development process must be transparent with qualified health experts and based on sound medical evidence. Congress should take steps to improve transparency and boost accountability in the LCD process. Reform of the LCD process will ensure that the most credible and compelling evidence available is used consistently. By changing the LCD process, Congress can ensure that the latest medical and scientific evidence is considered and that the evidence is not used selectively to deny appropriate coverage to seniors.

Conclusion

ASCO welcomes the opportunity to provide input and collaborate with you on the Cures initiative to accelerate the discovery, development, and delivery of promising new treatments to patients. If you have questions regarding cancer care issues, please contact Jeremy Haines at Jeremy.Haines@asco.org.

Sincerely,



Eric P. Winer, MD, FASCO
Chair of the Board
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