December 14 Closure of NOPR (NaF PET)

On December 15, 2015, the Centers for Medicare & Medicaid Services (CMS) issued a final decision memorandum (www.cms.gov/medicare-coverage-database/details/nca-decision-memo.aspx?NCAId=279) pertaining to coverage of bone PET with 18F-sodium fluoride (NaF PET) to identify bone metastasis. This National Coverage Decision (NCD) extended for 24 mo the requirement for coverage with evidence development (CED) under §1862(a)(1)(E) of the Social Security Act for NaF PET to identify bone metastasis of cancer as contained in section 220.6.19B of the Medicare National Coverage Determinations Manual. Current NCD and Medicare coverage for NaF PET (under CED) is thus scheduled to expire on December 14.

The National Oncologic PET Registry (NOPR) Working Group has submitted a request to CMS asking that coverage for NaF PET in bone metastasis be reconsidered in light of additional analyses of NOPR data along with Medicare claims data. A coverage decision in response to this reconsideration request is dependent on publication in peer-reviewed journals of these additional analyses. Questions to be addressed with data from these new studies include whether the addition of NaF PET imaging leads to: a change in patient management to more appropriate palliative care; a change in patient management to more appropriate curative care; improved quality of life; or improved survival. The timing of the coverage decision is currently unknown. However, it is unlikely that CMS will issue a coverage decision before the expiration of the current NCD.

At midnight EST on December 14, the NOPR website will no longer allow new case registrations. In addition, all planned NOPR-covered NaF PET studies must have been performed (and the PET Completion Form entered into the database) no later than December 14 at midnight EST. For any study performed on or before that date, entry of the PET Report Submission Form, the PET Scan Assessment Form, and the Post-PET Case Report Form will be allowed (within the time limits specified in the protocol). Participating sites should not schedule NaF PET studies to be performed for Medicare patients after December 14, until a new NCD is issued by CMS.

At the closeout of NOPR (NaF PET), balances in individual PET facility escrow accounts will be refunded. However, to ensure that funds are refunded properly, each PET facility must submit a refund request to: OPTOUT_NOPR@acr.org. The request should include the following information: NOPR facility ID number, NOPR facility name, NOPR facility address, name of person submitting request, phone number of person submitting request, and date of request. NOPR will acknowledge receipt of requests and send a refund check payable to the NOPR facility indicated in the request.

NIH Partners with Industry on Immunotherapy

The National Institutes of Health (NIH) and 11 leading biopharmaceutical companies announced on October 12 the launch of the Partnership for Accelerating Cancer Therapies (PACT), a 5-y public/private research collaboration totaling $215 million as part of the Cancer Moonshot. PACT will initially focus on efforts to identify, develop, and validate robust biomarkers to advance new immunotherapy treatments. The partnership will be managed by the Foundation for the National Institutes of Health (FNIH), with the Food and Drug Administration serving in an advisory role.

"We have seen dramatic responses from immunotherapy, often eradicating cancer completely for some cancer patients," said NIH Director Francis S. Collins, MD, PhD. "We need to bring that kind of success—and hope—for more people and more types of cancers, and we need to do it quickly."

PACT will facilitate systematic and uniform clinical testing of biomarkers to advance understanding of the mechanisms of response and resistance to cancer therapy. The research conducted under the partnership will also integrate immune and other related oncology biomarkers into clinical trials by defining a set of standardized biomarkers to be tested across a variety of studies. This approach will allow for consistent generation of data, uniform and harmonized assays to support data reproducibility, comparability of data across trials, and discovery and validation of new biomarkers for immunotherapy and related combinations. PACT will also facilitate information sharing by all stakeholders to better coordinate clinical efforts, align investigative approaches, reduce duplication, and facilitate more high-quality trials.

PACT partners include AbbVie (North Chicago, IL); Amgen (Thousand Oaks, CA); Boehringer Ingelheim Pharma GmbH & Co. KG (Germany); Bristol-Myers Squibb (New York, NY); Celgene Corporation (Summit, NJ); Genentech, a member of the Roche Group (San Francisco, CA); Gilead Sciences (Foster City, CA); GlaxoSmithKline plc (Brentford, UK); Janssen Pharmaceutical Companies of Johnson & Johnson (Raritan, NJ); Novartis (Basel, Switzerland); and Pfizer, Inc. (New York, NY). Additional support has been provided by the Pharmaceutical Research and Manufacturers Association. The 11 partner organizations will contribute up to $1 million per year for 5 y through the FNIH for a total private sector contribution of $55 million. NIH will contribute $160 million over the 5 y of the partnership, pending availability of funds.

The National Cancer Institute also recently awarded cooperative agreements to support 4 Cancer Immune Monitoring and Analysis Centers (CIMACs) and a Cancer Immunologic Data Commons...
FDA Clears 7T MR Device

On October 12, the U.S. Food and Drug Administration (FDA) cleared the first 7-Tesla (7T) magnetic MR imaging device, more than doubling the static magnetic field strength available for use in the United States. The Magnetom Terra (Siemens Medical Solutions, Inc.; Malvern, PA) is the first 7T MR imaging system cleared for clinical use in the United States. “The overall image quality of MRI improves with higher magnetic field strength,” said Robert Ochs, PhD, director of the Division of Radiological Health in the FDA Center for Devices and Radiological Health. “The added field strength allows for better visualization of smaller structures and subtle pathologies that may improve disease diagnosis.”

The FDA reviewed the Magnetom Terra through the 510(k) premarket clearance pathway and based its clearance on comparison to a predicate device and acquisition of sample clinical images. The agency reviewed the safety of the radiofrequency subsystem through computational modeling, simulations, and rigorous experimental validation. The manufacturer also provided data from a study of 35 healthy patients that compared images using the 7T device and those acquired with a 3T device. Board-certified radiologists reviewed the images and confirmed that those acquired on the 7T device were of diagnostic quality and, in some cases, an improvement over imaging at 3T. The Magnetom Terra is for patients who weigh more than 66 lbs and is limited to examinations of the head, arms, and legs.

U.S. Food and Drug Administration

FDA Adverse Event Search Tool

On September 28, the U.S. Food and Drug Administration (FDA) launched a new user-friendly search tool that improves access to data on adverse events associated with drug and biologic products through the FDA Adverse Event Reporting System (FAERS). The tool is designed to make it easier for consumers, providers, and researchers to access this information. “Tools like the FDA Adverse Event Reporting System are critical to the FDA’s ability to help ensure the greatest level of transparency and help patients and providers make safe use of drug and biologic products after they are approved by the FDA,” said FDA Commissioner Scott Gottlieb, MD. The new dashboard enables users to search for and organize data by criteria such as drug/biologic product, age of patient, type of adverse event, year the adverse event occurred, or within a specific timeframe. The FDA also hopes the increased transparency will spur submission of more detailed and complete reports from consumers, health care professionals, and others by making it easier to see other reports that the FDA receives and search the database for similar observations.

The FDA uses FAERS for surveillance, such as looking for new safety concerns that might be related to a marketed product, evaluating a manufacturer’s compliance with reporting regulations, and responding to outside requests for information. The reports in FAERS are evaluated by clinical reviewers in the FDA Center for Drug Evaluation and Research and Center for Biologics Evaluation and Research to monitor the safety of products after they are marketed. If a potential safety concern is identified in FAERS, additional evaluation is performed.

“Our focus on safety extends beyond approval,” said Janet Woodcock, MD, director of the FDA Center for Drug Evaluation and Research. “In fact, our staff spends a lot of time looking at FAERS reports received regarding approved drug and biologic products, and these reports can be very valuable components of our safety assessments. By giving people a better understanding of these data, and the associated limitations, we hope the new interface will encourage people to submit more complete reports.”

The FDA cautioned that the FAERS data have limitations that should be taken into account. For example, although FAERS contains reports on adverse events associated with a specific drug or biologic, this does not mean that the drug or biologic caused the adverse event. FAERS data by themselves are not an indicator of the safety profile of the drug or biologic. The FDA also maintains adverse event reporting programs and databases for foods, dietary supplements, and cosmetics; medical devices; and vaccines. The agency encourages health care professionals and consumers to report adverse events or quality problems experienced with the use of drug and biologic products to www.fda.gov/medwatch/report.htm.

U.S. Food and Drug Administration

Sharpless Sworn in as New NCI Director

Norman E. “Ned” Sharpless, MD, took the oath of office on October 17 to become the 15th director of the National Cancer Institute (NCI), part of the National Institutes of Health (NIH). He succeeds Harold E. Varmus, MD, who stepped down as director in March 2015. Douglas R. Lowy, MD, served as NCI’s acting director in the interim.

“It is an honor to welcome Dr. Sharpless to the Department of Health and Human Services and the National Institutes of Health,” said Acting Health and Human Services Secretary Eric D.
Hargan, JD. “We are grateful to Dr. Lowy for his service as acting director, and we look forward to Dr. Sharpless playing an integral role in this administration’s aggressive efforts to advance cancer research and cures for cancer patients.”

“Dr. Sharpless is an outstanding scientist, clinician, and administrator, and we are very fortunate to have him join the NIH leadership team,” said NIH Director Francis S. Collins, MD, PhD. “I look forward to his insight, influence, and partnership at NCI, as cancer research is experiencing an unprecedented era of rapid progress.”

Sharpless was most recently director of the NCI-designated Lineberger Comprehensive Cancer Center at the University of North Carolina School of Medicine (Chapel Hill), where he also was the Wellcome Distinguished Professor in Cancer Research. As a practicing oncologist he specialized in the care of patients with hematologic cancers. He is the author of more than 150 original scientific papers, reviews, and book chapters and is an inventor on 10 patents. His research has focused on the molecular biology of cancer and aging.

“I am honored and humbled to assume this role at NCI, the world’s premier cancer research institution,” said Sharpless. “This is an exciting moment for cancer research, as new discoveries and technological improvements are accelerating our progress against cancer, an ancient and unrelenting foe.”

National Institutes of Health

GTEx Atlas of DNA and Gene Expression

Researchers funded by the National Institutes of Health (NIH) announced on October 11 the completion of a detailed atlas documenting the stretches of human DNA that influence gene expression. This atlas was designed to serve as a critical resource for the scientific community interested in the ways in which individual genomic variation leads to biological differences across human tissues and cell types. The atlas is the culmination of work from the Genotype–Tissue Expression (GTEx) Consortium.

“GTEx was unique, because its researchers explored how genomic variation affects the expression of genes in individual tissues, across many individuals, and even within an individual,” said Simona Volpi, PharmD, PhD, program director for GTEx at the National Human Genome Research Institute (NHGRI; Bethesda, MD), who oversaw parts of the project. Researchers involved in the GTEx Consortium collected data from more than 53 different tissue types (including brain, liver, and lung) from autopsy, organ donations, and tissue transplant programs. These tissues came from more than 950 donors.

The project continues to house a biobank of collected tissue samples, as well as extracted DNA and RNA for future studies by independent researchers. Summary-level data are available to the public through the GTEx Portal (www.gtexportal.org/home/), and the most recent release of raw data has been submitted to the Database of Genotypes and Phenotypes (www.ncbi.nlm.nih.gov/gap), an archive of results from studies that investigate genomic contributions to phenotype. GTEx launched in 2010 and concluded in the summer of 2017. It was supported by the NIH Common Fund and administered by NHGRI, the National Institute of Mental Health, and the National Cancer Institute.

GTEx data is already being used to help researchers understand the mechanisms of gene expression in a variety of tissues, which will ultimately better inform knowledge about gene misregulation in the context of disease. GTEx data can also be used to better understand variations in gene expression that underlie differences among healthy individuals. Although the GTEx project has officially ended, plans for future work are already underway. The Enhancing GTEx project, which began in 2013, extends GTEx’s efforts by combining gene expression studies with additional data, such as protein expression. This work is being conducted on the same tissues from the GTEx project.

National Institutes of Health