

dostarlimab, and antibodies directed at TIM3 and LAG3. —*Elie Dolgin* ■

## New Endpoints, Programs Used for Drug Approvals

The FDA approved 59 new drugs in 2018—a record number. Of that total, 17 are novel cancer agents, two of which were greenlighted with nontraditional data, according to a report released by the agency's Center for Drug Evaluation and Research (CDER; available at [www.fda.gov](http://www.fda.gov)).

The document “emphasizes some of the many innovative ways we were able to evaluate safety and efficacy for these new therapies,” CDER Director Janet Woodcock, MD, writes in the introduction.

Take apalutamide (Erleada; Janssen), for example. This antiandrogen, used to treat nonmetastatic castration-resistant prostate cancer, is the first drug approved based on metastasis-free survival instead of a traditional

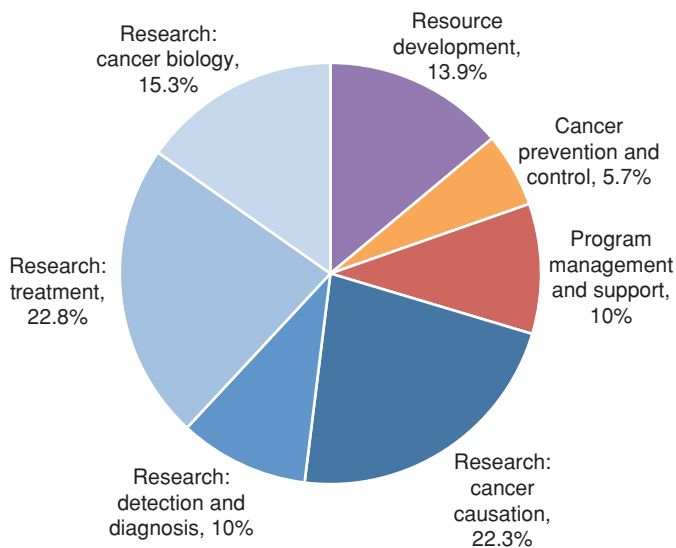
clinical trial endpoint, such as overall survival or progression-free survival.

In addition, the FDA approved lutetium Lu 177 dotatate (Lutathera; Advanced Accelerator Applications), a radioactive drug for gastroenteropancreatic neuroendocrine tumors, a group of rare cancers with few treatments. The approval was based on safety and efficacy data from an expanded access program through which patients can receive experimental drugs when no approved therapy is available. “This use of data represents an innovative way to... approve a needed therapy,” the report explains.

Five biosimilars related to cancer treatment hit the market in 2018 as well, the highest annual tally to date. “As patents and exclusivity protections for biologics expire in the United States, we can expect many more biosimilars to be submitted for approval,” Woodcock writes. “More products on the market means greater competition that can lead to increased access to therapies and lower costs to patients.” —*Suzanne Rose* ■

## BY THE NUMBERS

NCI Budget Breakdown, FY 2018



For fiscal year (FY) 2018, which ended on September 30, the total NCI budget was approximately \$5.928 billion, including \$496 million for the 21st Century Cures Act, \$300 million of which was earmarked for the Beau Biden Cancer Moonshot (<https://www.cancer.gov/about-nci/budget/fact-book>). The agency allocated 70.4% of its budget to research. Funds for resource development were mostly devoted to cancer centers.

For more news on cancer research, visit *Cancer Discovery* online at <http://cancerdiscovery.aacrjournals.org/> CDNews.

## NOTED

The chimeric antigen receptor (CAR) T-cell therapy **tisagenlecleucel (Kymriah; Novartis)** elicits lasting responses in certain relapsed/refractory leukemias and lymphomas, according to updated results of phase II trials presented at the 2018 American Society of Hematology Annual Meeting in San Diego, CA, in December. In the ELIANA trial, 79 children and young adults with acute lymphoblastic leukemia had an overall response rate (ORR) of 82% and an 18-month overall survival rate (OS) of 70%. In the JULIET trial, 115 adult patients with diffuse large B-cell lymphoma had an ORR of 54% and a median OS of 11.1 months (*N Engl J Med* 2019;380:45–56).

The FDA approved **tagraxofusp-erzs** (Elzonris; Stemline Therapeutics) for blastic plasmacytoid dendritic cell neoplasm (BPDCN) in patients age 2 and older. In a phase I trial, the drug elicited responses in seven of 13 patients. Tagraxofusp-erzs is the first drug approved for BPDCN.

Researchers identified molecular features of neuroblastomas associated with prognosis (*Science* 2018;362:1165–70). They found that patients whose tumors lacked telomere maintenance mechanisms had the highest survival rates, whereas those whose tumors had the maintenance mechanisms plus RAS and/or p53 pathway mutations had the worst outcomes.

U.S. Surgeon General Jerome Adams, MD, released an advisory about electronic cigarette (e-cigarette) use among youths (available at: <https://e-cigarettes.surgeon-general.gov/>). He declared the uptake of e-cigarettes among children “an epidemic,” and described the potential harms of using them. He also called for more aggressive steps to prevent youth access, outlining actions that can be taken by parents, teachers, health professionals, and state and local governments.

The U.S. cancer death rate has decreased continuously for the past 25 years, according to a report published by the American Cancer Society (*CA Cancer J Clin* 2019;69:7–34). Overall, the cancer mortality rate dropped 27% between 1991 and 2016, although cancer remains the second leading cause of death after heart disease.