



U.S. FOOD & DRUG  
ADMINISTRATION

CENTER FOR DRUG EVALUATION AND RESEARCH

# Drug Trials Snapshots Summary Report 2022



May 2023

# Introduction

## Welcome to the FDA's Center for Drug Evaluation and Research's (CDER's) Drug Trials Snapshots Summary Report

Since January 2015, CDER has shared information on the diversity of participants in clinical trials through the transparency initiative called Drug Trials Snapshots.

This year's annual report summarizes the 2022 Drug Trials Snapshot program, complementing [CDER's annual report, \*Advancing Health Through Innovation: New Drug Therapy Approvals 2022\*](#), published on January 20, 2023 by providing information on the diversity of participants in the clinical trials relied upon for approval of novel therapies. The approved therapies span a wide range of medical conditions including ones that largely affect pediatric patients, diseases affecting only males or only females, common diseases that affect a large proportion of the population in the United States, and rare (or orphan) diseases with a smaller number of patients in the United States and around the world. Given the varied diseases being targeted, including in men's and women's health issues, looking at patient populations by individual drug or therapeutic area gives the clearest insight into patient diversity in clinical trials specific to a disease, rather than summary statistics of all approved therapies combined.

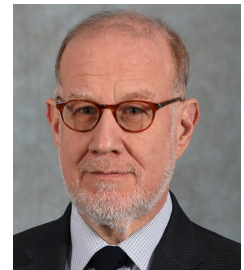
We hope this information is helpful to promote dialogue on the appropriate representation of different subgroups in clinical trials. We welcome your feedback on the Drug Trial Snapshot program and ideas you may have regarding how FDA can enhance the information provided in each Snapshot. You can share your thoughts by sending an email to [Snapshots@fda.hhs.gov](mailto:Snapshots@fda.hhs.gov).



---

**Patrizia Cavazzoni, M.D.**  
Director, CDER

---



---

**Peter Stein, MD**  
Director, OND

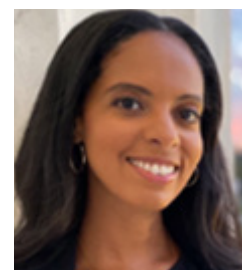
---



---

**Mary Thanh Hai, MD**  
Deputy Director, OND

---



---

**Aden Asefa, MPH**  
DTS Lead, OND

---



## 2022 Summary Statistics

(January 1, 2022 – December 31, 2022)

In 2022, CDER approved 37 novel drugs, either as new molecular entities (NMEs) under new drug applications (NDAs) or as new therapeutic biologics under biologics license applications (BLA). Twenty (54%) of these novel drugs were first-in-class, meaning they have mechanisms of action different from those of existing therapies. In addition, 20 (54%) of these approvals were for the treatment of rare or “orphan” diseases (diseases that affect fewer than 200,000 people in the U.S.).

In addition to summarizing baseline demographic data from pivotal trials supporting approval of each novel drug, the 2022 annual report also highlights several novel therapeutics for highly prevalent conditions based on information from publicly available FDA reviews.

Demographic data for each approved novel drug is organized by the following categories to best reflect the organizational structure of CDER’s Office of New Drugs, which oversees these drug development programs.

- **Heart, Blood, Kidney, and Endocrine Diseases**
- **Autoimmune, Inflammatory, and Lung Diseases**
- **Infectious Diseases**
- **Neurological and Psychiatric Disorders**
- **Cancers**
- **Other Advances in Drug Therapies**

We hope information from this annual report will help promote further dialogue on how drug development can be enhanced to improve knowledge about the safety and effectiveness of therapies across the diverse patient community in the United States.





## HEART, BLOOD, KIDNEY AND ENDOCRINE DISEASES

In 2022, eight novel therapies were approved for medical conditions affecting the heart, blood, kidney, or endocrine system (Table 1). Five of the eight novel therapies were approved to treat a rare disease (Camzyos, Enjaymo, Pyrukynd, Terlivaz and Vonjo). Six of the eight novel approvals were first-in-class (Camzyos, Enjaymo, Mounjaro, Pyrukynd, Terlivaz, and Tzield).

Among the novel therapies approved is Mounjaro, a medication that activates two hormone receptors to improve glucose control in adults with type 2 diabetes (T2D), as an addition to diet and exercise. Approximately 35 million Americans have Type 2 diabetes (T2D), about 10% of the U.S. population.<sup>1</sup> Effectiveness of Mounjaro was established in five global pivotal trials evaluating its use alone (monotherapy) or in combination with other approved diabetes medications, randomizing a total of 6,278 patients, of which 6,263 received study treatment. Of the 6,263 patients, 23.1% were from trial sites in North America, 38.6% in Central/South America and

<sup>1</sup> <https://www.niddk.nih.gov/health-information/health-statistics/diabetes-statistics>



Mexico, 28.5% in the European Union (EU)/United Kingdom/Ukraine, 4.6% in Asia (excluding Japan), 2.7% in Japan, and 2.4 % in the rest of the world. A total of 2,822 (45%) patients were females and 2,082 (33%) were 65 years of age or older. Baseline race categories collected included White (5,037; 80%), Black or African American (224; 4%), Asian (424; 7%), American Indian or Alaska Native (505; 8%), Native Hawaiian or Pacific Islander (10; <1%), and Multiple (59; 1%). Notable in this program was the enrollment of approximately 25% and 11% American Indian or Alaska Natives in two separate trials. Patients in these racial categories are often underrepresented in diabetes drug development programs. Hispanic/Latino ethnicity was reported in 2,917 (47%) patients. In one trial, approximately 70% of patients were identified as Hispanic/Latino ethnicity.



## Table 1. Novel Therapies Approved to Treat Heart, Blood, Kidney, and Endocrine Diseases

Trade Name	Active Ingredient	Indication	Total N	% Female	% White†	% Black†	% Asian†	% Hispanic‡	% ≥ 65 years <sup>€</sup>
<b>Camzyos*</b>	Mavacamten	To treat certain classes of obstructive hypertrophic cardiomyopathy	251	40.6%	91.2%	2.4%	2.4%	4.8%	33.9%
<b>Enjaymo*</b>	Sutimlimab-jome	To decrease the need for red blood cell transfusion due to hemolysis in cold agglutinin disease	24	62.5%	12.5%	0%	12.5%	0%	79.2%
<b>Mounjaro</b>	Tirzepatide	To improve blood glucose control in diabetes, in addition to diet and exercise	6,263	45%	80%	4%	7%	47%	33%
<b>Pyrukynd*</b>	Mitapivat	To treat hemolytic anemia in pyruvate kinase deficiency	107	64%	75%	-	10%	3%	5%
<b>Rolvedon</b>	Eflapegrastim-xnst	To decrease the incidence of infection in patients with non-myeloid malignancies receiving myelosuppressive anti-cancer drugs associated with clinically significant incidence of febrile neutropenia	643	100%	77.1%	11.8%	8.4%	16.6%	37.8%
<b>Terlivaz*</b>	Terlipressin	To improve kidney function in adults with hepatorenal syndrome with rapid reduction in kidney function	300	40%	90%	6%	2%	15%	18%
<b>Tzield</b>	Teplizumab-mzww	To delay the onset of Stage 3 type 1 diabetes	773	36%	72%	1%	26%	5%	0%
<b>Vonjo*</b>	Pacritinib	To treat intermediate or high-risk primary or secondary myelofibrosis in adults with low platelets	63	44%	95%	0	0.02%	NA	69.8%

NA — data not available

\* orphan disease

† The percentages of all other races combined (American Indian, Alaska Native, Native Hawaiian or other Pacific Islander, Other, Unknown/Unreported) adds up to 100% of race category

‡ The percentage of Non-Hispanic and Unknown/Unreported ethnicity adds up to 100% of ethnicity category

€ For programs that analyzed age by a different threshold, the age cutoff is provided. 0% represent conditions that are predominantly in pediatric population.



## AUTOIMMUNE, INFLAMMATORY, AND LUNG DISEASES

In 2022, six novel therapies were approved for autoimmune, inflammatory, or lung diseases (Table 2). Three of the six novel approvals were first-in-class (Sotyktu, Spevigo and Vtama). Two of the six novel therapies were approved to treat a rare disease (Nexobrid and Spevigo).

Cibinqo and Sotyktu were approved for two highly prevalent dermatologic conditions.

Cibinqo is approved for the treatment of moderate-to-severe atopic dermatitis (AD) that is not adequately controlled by other drugs. Approximately 16.5 million U.S. adults have AD, nearly 40% being moderate-to-severe.<sup>2</sup> Effectiveness of Cibinqo was evaluated in three global pivotal trials randomizing a total of 1,615 patients: two trials included adolescents and adults and one enrolled adults only. Of the 1,615 patients randomized in these trials, 334 (21%) were in the United States, 757 (47%) were female, 124 (8%) were between 12 and 18 yrs of age, inclusive, and 89 (6%) were 65 years of age or older. Baseline race categories collected included White (1,117; 69%), Black or African American (88; 5%), Asian (189; 12%), American Indian or Alaska Native (10; <1%), Multiple (190; 12%), and Missing (14; <1% %).

Sotyktu is approved for the treatment of moderate-to-severe plaque psoriasis in patients who are candidates for systemic therapy or phototherapy. Effectiveness of Sotyktu was evaluated in two global pivotal trials randomizing a total of 1,684 patients of which 559 (33%) were female and 169 (10%) were 65 years of age or older. Baseline race categories collected included White (1,467; 87%), Black or African American (32; 2%), Asian (165; 10%), American Indian or Alaska Native (7; <1%), Other (13; <1% %).

<sup>2</sup> [https://nationaleczema.org/research/eczema-facts/#:~:text=Prevalence%20of%20childhood%20%26%20adult%20atopic%20dermatitis%20\(AD\)&text=An%20estimated%2016.5%20million%20U.S.,with%20moderate%20to%20severe%20disease.&text=80%25%20of%20individuals%20affected%20by,to%206%20years%20of%20age.](https://nationaleczema.org/research/eczema-facts/#:~:text=Prevalence%20of%20childhood%20%26%20adult%20atopic%20dermatitis%20(AD)&text=An%20estimated%2016.5%20million%20U.S.,with%20moderate%20to%20severe%20disease.&text=80%25%20of%20individuals%20affected%20by,to%206%20years%20of%20age.)

## Table 2. Novel Therapies Approved to Treat Autoimmune, Inflammatory, and Lung Diseases

Trade Name	Active Ingredient	Indication	Total N	% Female	% White <sup>†</sup>	% Black <sup>†</sup>	% Asian <sup>†</sup>	% Hispanic <sup>‡</sup>	% ≥ 65 years <sup>€</sup>
<b>Cibinqo</b>	Abrocitinib	To treat refractory, moderate-to-severe atopic dermatitis	1,615	47%	69%	5%	12%	NA	5%
<b>Daxxify</b>	daxibotulinumtoxinA-lanm	To treat moderate-to-severe glabellar lines associated with corrugator and/or procerus muscle activity	609	87%	86%	5%	4%	17%	30%
<b>Nexobrid*</b>	Anacaulase-bcdb	To remove eschar in adults with deep partial thickness or full thickness thermal burns	331	28%	81%	10%	3%	9%	5%
<b>Sotyktu</b>	Deucravacitinib	To treat moderate-to-severe plaque psoriasis	1,684	33%	87%	2%	10%	NA	10%
<b>Spevigo*</b>	Spesolimab-sbzo	To treat generalized pustular psoriasis flares	53	68%	45%	NA	55%	0	4%
<b>Vtama</b>	Tapinarof	To treat plaque psoriasis	1,025	42.5%	84.9%	4.6%	6.9%	14.9%	13.6%

NA — data not available

\* orphan disease

† The percentages of all other races combined (American Indian, Alaska Native, Native Hawaiian or other Pacific Islander, Other, Unknown/Unreported) adds up to 100% of race category

‡ The percentage of Non-Hispanic and Unknown/Unreported ethnicity adds up to 100% of ethnicity category

€ For programs that analyzed age by a different threshold, the age cutoff is provided. 0% represent conditions that are predominantly in pediatric population.





## INFECTIOUS DISEASES

In 2022, FDA approved new therapies to treat HIV infection, vulvovaginal yeast infection, and *Helicobacter pylori* infection (See Table 3).<sup>3</sup> Two were first-in-class approvals (Sunlenca and Voquezna).

The U.S. Centers for Disease Control and Prevention estimates that approximately 1.2 million people in the U.S. are living with HIV. There are more than two dozen antiretroviral drugs approved to treat HIV infection that when used in combination have reduced the amount of viral load in the body that impairs the immune system. Despite the availability of many effective treatments, treatment failure can occur as a result of drug resistance or intolerance, and for these patients, there are limited options. Sunlenca is approved for use in combination with other antiretroviral(s) for the treatment of HIV-1 infection in heavily treatment-experienced adults with multidrug resistant infection. Effectiveness of Sunlenca came from one multicenter study that enrolled 36 patients whose HIV infections were resistant to multiple classes of HIV medications. This trial included 18 (25%) females and the mean age was 50.2 yrs, ranging from 23 to 78 yrs. Baseline race categories collected included White (29; 40%), Black (27; 38%), Asian (15; 21%), and Not Reported (1; 1%).

<sup>3</sup> <https://www.cdc.gov/hiv/statistics/overview/index.html>

### Table 3. Novel Therapies Approved to Treat Infectious Disease

Trade Name	Active Ingredient	Indication	Total N	% Female	% White <sup>†</sup>	% Black <sup>†</sup>	% Asian <sup>†</sup>	% Hispanic <sup>‡</sup>	% ≥ 65 years <sup>€</sup>
<b>Sunlenca</b>	Lenacapavir	To treat adults with HIV whose HIV infections cannot be successfully treated with other available treatments due to resistance, intolerance, or safety considerations	36	28%	44%	44%	8%	28%	75% (≥50 yrs)
<b>Vivjoa</b>	Oteseconazole	To reduce the incidence of recurrent vulvovaginal candidiasis (RVVC) in females with a history of RVVC who are not of reproductive potential	871	100%	75%	17%	6%	15%	<1%
<b>Voquezna Triple Pak and Voquezna Dual Pak</b>	Vonoprazan, amoxicillin, clarithromycin	To treat H. pylori infection	992	63%	90%	7%	NA	26%	21%

NA — data not available

\* orphan disease

† The percentages of all other races combined (American Indian, Alaska Native, Native Hawaiian or other Pacific Islander, Other, Unknown/Unreported) adds up to 100% of race category

‡ The percentage of Non-Hispanic and Unknown/Unreported ethnicity adds up to 100% of ethnicity category

€ For programs that analyzed age by a different threshold, the age cutoff is provided. 0% represent conditions that are predominantly in pediatric population.



## NEUROLOGICAL AND PSYCHIATRIC DISORDERS

In 2022, there were five novel therapies approved to treat neurological and psychiatric disorders (See Table 4); one was a first-in-class (Ztalmy) and three were approved to treat rare diseases (Amvuttra, Relyvrio and Ztalmy). Two approvals (Briumvi and Quviviq) were approved for highly prevalent conditions in the U.S.

Briumvi is an antibody administered as an infusion approved for the treatment of relapsing forms of multiple sclerosis (MS). MS is the most common cause of non-traumatic neurologic disability in young adults and is estimated to affect approximately 1 million people in the U.S.<sup>4</sup> Effectiveness of Briumvi was evaluated in two pivotal trials conducted at sites in North America and Europe, randomizing a total of 1,094 patients, of which 1,089 received treatment (mITT). Of the 1,089 patients, 88 (8%) were from the U.S., 699 (64%) were female and 379 (35%) were 40 years of age or older. Baseline race categories collected included White (1,067; 98%), Black or African American (17; 1.6%), Asian (0), American Indian or Alaska Native (1; <1%), and Other (4; <1%). Hispanic/Latino ethnicity was reported in 18 (2%) patients.

Quviviq is approved for the treatment of insomnia in adults based on efficacy results from two global pivotal trials randomizing a total of 1,854 patients, 625 (34%) were from the U.S. A total of 1,262 (68%) of the study population were female and 727 (39%) were 65 years of age or older. Baseline race categories collected included White (1,650; 89%), Black or African American (148; 8%), Asian (44; 2%), American Indian or Alaska Native (3; <1%), Native Hawaiian or Pacific Islander (4; <1%), and Other (3; <1%). Hispanic/Latino ethnicity was reported in 193 (10%) patients.

<sup>4</sup> Wallin MT, Culpepper WJ, Campbell JD, et al. The prevalence of MS in the United States: A population-based estimate using health claims data. *Neurology*. 2019;92(10):e1029-e1040.



## Table 4. Novel Therapies Approved to Treat Neurological and Psychiatric Disorders

Trade Name	Active Ingredient	Indication	Total N	% Female	% White <sup>†</sup>	% Black <sup>†</sup>	% Asian <sup>†</sup>	% Hispanic <sup>†</sup>	% ≥ 65 years <sup>€</sup>
<b>Amyvuttra*</b>	Vutrisiran	To treat polyneuropathy of hereditary transthyretin-mediated amyloidosis	122	35%	71%	3%	17%	9.8%	38%
<b>Briumvi</b>	Ublituximab-xiiy	To treat relapsing forms of multiple sclerosis	1,089	64%	98%	1.6%	0%	2%	35% (≥40)
<b>Quviviq</b>	Daridorexant	To treat insomnia	1,854	68%	89%	8%	2%	10%	39%
<b>Relyvrio*</b>	Sodium phenylbutyrate and taurursodiol	To treat amyotrophic lateral sclerosis (ALS)	137	32%	95%	2%	2%	5%	23%
<b>Ztalmy*</b>	Ganaxolone	To treat seizures in cyclin-dependent kinase-like 5 deficiency disorder	101	79%	92%	NA	5%	NA	0%

NA — data not available

\* orphan disease

† The percentages of all other races combined (American Indian, Alaska Native, Native Hawaiian or other Pacific Islander, Other, Unknown/Unreported) adds up to 100% of race category

‡ The percentage of Non-Hispanic and Unknown/Unreported ethnicity adds up to 100% of ethnicity category

€ For programs that analyzed age by a different threshold, the age cutoff is provided. 0% represent conditions that are predominantly in pediatric population.



## CANCERS

In 2022, CDER approved 10 novel therapies to treat a variety of cancers (See Table 5). Six of the 10 novel approvals were first-in-class (Elahere, Kimmtrak, Lunsumio, Opdualag, Pluvicto, and Tecvayli) and all but one (Pluvicto) were approvals for rare diseases.

Pluvicto is a radioligand therapeutic agent approved for the treatment of adult patients with prostate-specific membrane antigen (PSMA)-positive, metastatic, castration-resistant (cancer that grows despite reduced amounts of testosterone) prostate cancer who have received at least two prior therapies, including an androgen receptor pathway inhibitor and taxane-based chemotherapy. Prostate cancer is the second leading cause of cancer-related deaths among men in the U.S. In 2020, approximately 191,930 new cases of prostate cancer and 33,330 deaths were estimated in the U.S.<sup>5</sup> While most cases of prostate cancers diagnosed at an early stage have an indolent course, local tumor progression and metastatic disease may develop in the long-term. Patients with an initial response to androgen deprivation therapy (ADT) may develop metastatic disease that progresses to a hormone-insensitive stage of disease with 5-year survival rate of approximately 30%. Effectiveness of Pluvicto in this patient population came from one trial that enrolled 831 male patients across 82 study sites, 45 (55%) of which were located in the U.S. A total of 626 (75.3%) patients were 65 years of age or older. Baseline race categories collected included White (721; 87%), Asian (20; 2.4%), Black or African American (55; 7%), Other (2; <1%) or Missing (33; 4%). Hispanic/Latino ethnicity was reported in 14 (1.7%).

<sup>5</sup> Siegel, RL et al. Cancer Statistics, 2020. *CA Cancer J Clin.* 2021 Jan;71(1):7-33.

## Table 5. Novel Therapies Approved to Treat Cancers

Trade Name	Active Ingredient	Indication	Total N	% Female	% White <sup>†</sup>	% Black <sup>†</sup>	% Asian <sup>†</sup>	% Hispanic <sup>‡</sup>	% ≥ 65 years <sup>€</sup>
<b>Elahere*</b>	Mirvetuximab soravtansine-gynx	To treat patients with recurrent ovarian cancer that is resistant to platinum therapy	106	100%	96%	0%	2%	2%	44%
<b>Imjudo*</b>	Tremelimumab-actl	To treat unresectable hepatocellular carcinoma	782	15%	46%	2%	49%	5%	50%
<b>Kimtrak*</b>	Tebentafusp-tebn	To treat unresectable or metastatic uveal melanoma	378	50%	87%	NA	NA	2%	49%
<b>Krazati*</b>	Adagrasib	To treat KRAS G12C-mutated locally advanced or metastatic non-small cell lung cancer in adults who have received at least one prior systemic therapy	112	55%	83%	8%	4.5%	2.7%	51%
<b>Lunsumio*</b>	Mosunetuzumab-axgb	To treat adults with relapsed or refractory follicular lymphoma, a type of non-Hodgkin lymphoma	90	39%	82%	4%	9%	8%	33%
<b>Lytgobi*</b>	Futibatinib	To treat intrahepatic cholangiocarcinoma harboring fibroblast growth factor receptor 2 (FGFR2) gene fusions or other rearrangements	103	56%	50%	8%	29%	2%	22%
<b>Opdualag*</b>	Nivolumab and relatlimab-rmbw	To treat unresectable or metastatic melanoma	714	42%	97%	<1%	NA	6%	46%
<b>Pluvicto</b>	Lutetium Lu 177 vipivotide tetraxetan	To treat prostate-specific membrane antigen-positive metastatic castration-resistant prostate cancer	831	0%	87%	7%	2%	1.7%	75%
<b>Rezlidhia*</b>	Olutasidenib	To treat relapsed or refractory acute myeloid leukemia with a susceptible isocitrate dehydrogenase-1 (IDH1) mutation	153	48%	46%	3%	3%	NA	65%
<b>Tecvayli*</b>	Teclistamab-cqyv	To treat relapsed or refractory multiple myeloma among adults who have received at least four specific lines of therapy	110	44%	91%	5%	3%	12%	53%
<b>Relyvrio*</b>	Sodium phenylbutyrate and taurursodiol	To treat amyotrophic lateral sclerosis (ALS)	137	32%	95%	2%	2%	5%	23%

NA — data not available

\* orphan disease

† The percentages of all other races combined (American Indian, Alaska Native, Native Hawaiian or other Pacific Islander, Other, Unknown/Unreported) adds up to 100% of race category

‡ The percentage of Non-Hispanic and Unknown/Unreported ethnicity adds up to 100% of ethnicity category

€ For programs that analyzed age by a different threshold, the age cutoff is provided. 0% represent conditions that are predominantly in pediatric population.



## OTHER ADVANCES IN DRUG THERAPIES

In other areas (Table 6), CDER approved two novel therapies for medical imaging (Elucirem and Xenoview), two novel therapies to treat eye conditions (Omlonti and Vabysmo), and one novel therapy for a rare enzyme deficiency disorder (Xenpozyme). Xenoview and Xenpozyme are first-in-class approvals.

Omlonti was approved for the reduction of elevated intraocular pressure (IOP) in patients with open-angle glaucoma or ocular hypertension. Glaucoma is a life-long progressive disease that is characterized by irreversible damage to the optic nerve. It is the leading cause of irreversible blindness in the U.S. and approximately 3 million Americans have glaucoma. Effectiveness of Omlonti was evaluated in 1,203 patients enrolled in 3 pivotal trials, one conducted in Asia with data coming from 369 (31%) patients, and two conducted in the U.S. with data coming from 834 (69%) patients. Of the overall trial population, 663 (55%) were female and 560 (47%) were 65 years of age or older. Baseline race categories collected included White (577; 48%), Asian (393; 33%), Black or African American (226; 19%), American Indian or Alaska Native (2; <1%), Other (0) or Multiple (8; <1%).

Vabysmo was approved for two prevalent eye conditions: neovascular (wet) age-related macular degeneration (nAMD) and diabetic macular edema (DME). For nAMD, a leading cause of severe vision loss worldwide, effectiveness of Vabysmo was evaluated in 1,329 patients enrolled in two global pivotal trials. The first trial had 149 study sites in 15 countries with 53 sites (36%) in the U.S. The second trial had 122 study sites in 20 countries with 41 sites (34%) in the U.S. A total of 793 (60%) of study participants were female and 788 (59%) were 75 years of age or older. Baseline race categories collected included White (1,153; 87%), Asian (126; 9%), Black or African American (10; <1%), American Indian or Alaska Native (4; <1%), Unknown (34; 3%) or Multiple (2; <1%).

Diabetic retinopathy and DME are complications of type 1 and 2 diabetes. The most common cause of vision loss from diabetic retinopathy is DME. Effectiveness of Vabysmo for DME was evaluated in 1,891 patients enrolled in two global pivotal trials. The first trial had 179 study sites in 16 countries with 83 (46%) sites in the U.S. The second trial had 174 study sites in 24 countries with 50 (29%) sites in the U.S. A total of 750 (40%) of study participants were female and 812 (43%) were 65 years or older. Baseline race categories collected included White (1,486; 79%), Asian (186; 19%), Black or African American (124; 7%), American Indian or Alaska Native (19; 1%), Unknown (65; 3%) or Multiple (4; <1%).

## Table 6. Novel Therapy Approvals in Other Areas

Trade Name	Active Ingredient	Indication	Total N	% Female	% White <sup>†</sup>	% Black <sup>†</sup>	% Asian <sup>†</sup>	% Hispanic <sup>‡</sup>	% ≥ 65 years <sup>€</sup>
<b>Elucirem</b>	Gadopiclenol	To detect and visualize lesions, together with MRI, with abnormal vascularity in the central nervous system and the body	551	57%	78%	2%	12%	13%	34%
<b>Omlonti</b>	Omidenepag isopropyl	To reduce elevated intraocular pressure in patients with open angle glaucoma or ocular hypertension	1,203	55%	48%	19%	32%	10%	47%
<b>Vabysmo (nAMD)</b>	Faricimab-svoa	To treat neovascular (wet) aged-related macular degeneration and diabetic macular edema (nAMD)	1,329	59%	87%	1%	9%	NA	59% (≥75)
<b>Vabysmo (DME)</b>	Faricimab-svoa	To treat diabetic macular edema (DME)	1,891	40%	79%	7%	19%	NA	43%
<b>Xenoview</b>	Hyperpolarized Xe-129	To evaluate pulmonary function and imaging	83	31%	88%	11%	1%	1%	43%
<b>Xenpozyme*</b>	Olipudase alfa-rpcp	To treat acid sphingomyelinase deficiency	38	58%	90%	N/A	5%	26%	3%

NA — data not available

\* orphan disease

† The percentages of all other races combined (American Indian, Alaska Native, Native Hawaiian or other Pacific Islander, Other, Unknown/Unreported) adds up to 100% of race category

‡ The percentage of Non-Hispanic and Unknown/Unreported ethnicity adds up to 100% of ethnicity category

€ For programs that analyzed age by a different threshold, the age cutoff is provided. 0% represent conditions that are predominantly in pediatric population.



## CONCLUSIONS

CDER approved 37 novel therapies for a broad range of diseases in 2022. Over 27,000 patients contributed to the advancement of science and medicine through their participation in the pivotal studies supporting these approvals. This annual report summarizes the demographic information across a range of populations studied. The report also highlighted nine novel therapies approved for the treatment of 10 highly prevalent conditions in the U.S., providing additional information obtained from publicly available FDA reviews. All of the pivotal trials supporting each of these highlighted approvals were multinational. For programs reporting number of patients by country, the percentage of patients from the U.S. ranged between 8 to 69%. For programs reporting number of study sites by country, the percentage of sites in the U.S. ranged between 29 to 55% of total sites. For diseases affecting both sexes, the percentage of females evaluated ranged between 25 to 68%.

Whites comprised the majority of patients enrolled in most of the pivotal trials supporting approval of all 37 novel therapies, followed by Asians and Blacks. Some programs had notable enrollment of Asians [Lytgobi (29%), Spevigo (55%), Ztalmy (45%), Imjudo (49%), Omlonti (33%)] and Blacks [Omlonti (19%), Vivjoa (17%) and Sunlenca (38%)]. Further assessment of prevalence by racial subgroups for these diseases and factors that may have contributed to higher inclusion of patients



in these two racial categories in these clinical trials may inform approaches in other therapeutic areas where representation by certain relevant demographic characteristics is low. Overall, representation in race categories of Native American or Alaska Native and Native Hawaiian or Pacific Islander remains low. However, two of the five pivotal trials supporting approval of a novel diabetes medicine (Mounjaro) enrolled 25% and 11% patients in the Native American or Alaska Native race category. Historically, patients in this race category contribute less than 1–3% of trial participants in diabetes drug development programs and further investigation into the Mounjaro program may help identify targeted approaches that can improve enrollment of patients in this racial subgroup in future programs.

Presenting demographic data by drug and therapeutic area in the annual summary report will facilitate tracking of trends in diversity in clinical trials by therapeutic area over time and identifying programs that have achieved lower or greater enrollment of under-represented patient populations. FDA is committed to working with sponsors to identify barriers to overcome and to leverage best practices that contribute towards improving patient diversity in clinical trials and hope the information obtained from Drug Trials Snapshots can contribute to this dialogue.

## ACKNOWLEDGEMENTS

We acknowledge and appreciate the diligence of FDA review staff in providing additional analyses to support our transparency goals related to diversity and inclusion in clinical trials to support novel new drugs.

We also acknowledge the following contributors to this report:

Robert Abugov, PhD, Senior Mathematical Statistician, Division of Biometrics II, Office of Biostatistics, Office of Translational Sciences (OTS), CDER

Lawrence Allan, Senior Regulatory Project Manager, Business Process Operations Staff, Office of Program Operations (OPO), Office of New Drugs (OND), CDER

Ariel Armstrong, PhD candidate, Student Trainee, OND, CDER

Kevin Bugin, PhD, Deputy Director for Operations, OND, CDER

Jinzhong Liu, PhD, Acting Director, Clinical Data Science Staff, Immediate Office, OND, CDER

Cathryn Lee, CRNP, MS, Staff Director, Program Development, Implementation and Management Staff, Office of Program Operations, OND, CDER

Mark Rothman, PhD, Director, Division of Biometrics II, Office of Biostatistics, OTS, CDER

Jizu Zhi, PhD, MS, Lead Data Scientist, Clinical Data Science Staff, Immediate office, OND, CDER



**U.S. FOOD & DRUG**  
ADMINISTRATION

U.S. Food and Drug Administration  
[www.fda.gov](http://www.fda.gov)

Center for Drug Evaluation and Research  
U.S. Food and Drug Administration  
10903 New Hampshire Avenue  
Silver Spring, Maryland 20993