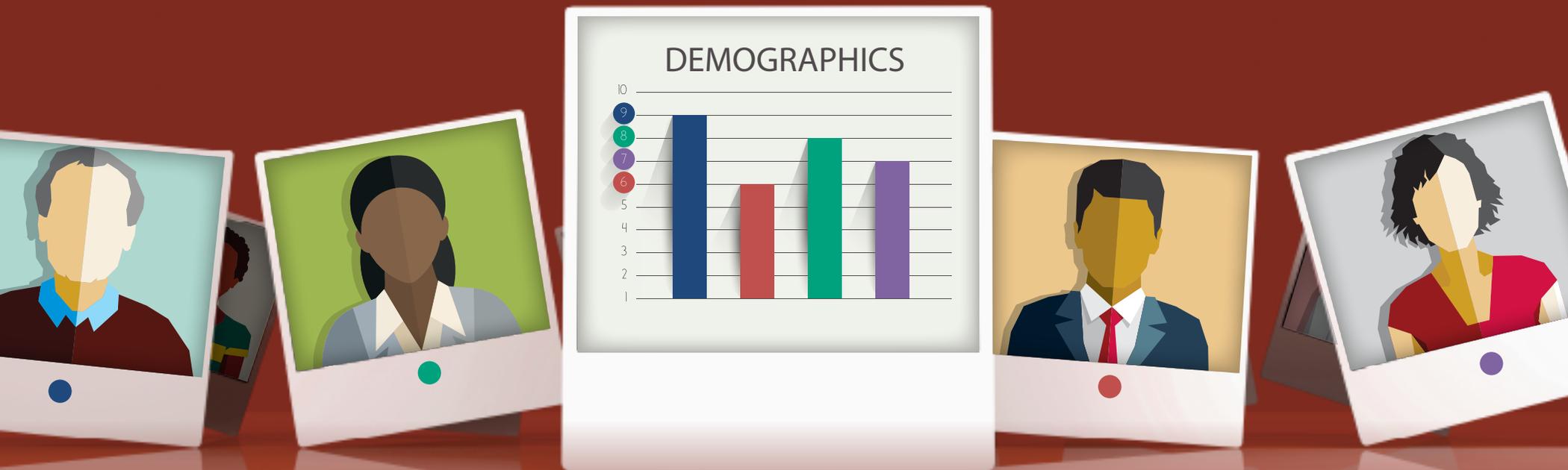


2018 DRUG TRIALS SNAPSHOTS SUMMARY REPORT



Introduction

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

Every year, CDER approves a number of novel drugs based upon review of safety and efficacy measures from sponsor submitted clinical trial data. Participation in these clinical trials has varied greatly, from tens to thousands of patients. In recent years, the representation of certain subgroups, such as women and people of racial and ethnic minority groups, has become of greater interest to the general public. CDER created the transparency initiative, called the Drug Trials Snapshots, to share information on the diversity of participants in clinical trials and the extent to which safety and efficacy data is based on demographic factors such as sex, age, race and ethnicity.

Snapshots are data posted online in a standardized format after approval of a novel drug that is either a New Molecular Entity (NME) or original biologic (BLA) product. They show who participated in the pivotal clinical trials used to approve the drug and stratify the data by sex, race, age and ethnicity subgroups. Further, the Snapshots provide statements on whether there were any observed differences in safety and efficacy by demographic subgroups at the time of approval. Since January 2015, CDER has published a Drug Trial Snapshot for each novel drug approved within a month of the official approval date.

Our Summary Reports are another commitment to enhancing transparency. This year's report summarizes the 2018 Drug Trials Snapshot program, highlighting 59 novel drugs, and the extent to which safety and efficacy data is based on demographic factors. We provide an overall average of each demographic group followed by a more detailed table of the percent representation of sex, race, age and ethnicity participating in the clinical trials used to approve the drug.

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.



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2018 Summary Statistics

(Jan 1, 2018 - Dec 31, 2018)

In 2018, CDER approved **59** novel drugs*, either as new molecular entities (NMEs) under New Drug Applications (NDAs) or as new therapeutic biologics under Biologics License Applications (BLAs). Overall, **43,966** patients participated in these trials. Subpopulation demographics from these trials are presented below.

Table 1. Demographic Subgroups in 2018

DEMOGRAPHIC SUBGROUPS	WOMEN	WHITE	BLACK or AFRICAN AMERICAN	ASIAN	HISPANIC	AGE 65 AND OLDER	UNITED STATES
AVERAGE	56%	69%	11%	10%	14%	15%	47%

*Data presented in this report are from 60 snapshots as one drug was approved for two indications.

More insight into demographics for all 59 CDER approved NMEs are provided below in Table 2.

Table 2. Percentage of Subpopulations* - All Approvals (2018)

BRAND NAME	INDICATION	WOMEN	WHITE	BLACK or AFRICAN AMERICAN	ASIAN	HISPANIC	AGE 65 and OLDER	UNITED STATES
AEMCOLO	Treatment of traveler’s diarrhea in adults	50%	83%	1%	14%	3%	9%	0%
AIMOVIG	Preventive treatment of migraine in adults	85%	15%	7%	1%	7%	0%	49%
AJOVY	Preventive treatment of migraine in adults	87%	80%	9%	10%	10%	2%	73%

Table 2. Percentage of Subpopulations* - All Approvals (2018) *continued*

BRAND NAME	INDICATION	WOMEN	WHITE	BLACK or AFRICAN AMERICAN	ASIAN	HISPANIC	AGE 65 and OLDER	UNITED STATES
AKYNZEO	Prevention of the nausea and vomiting in adults receiving chemotherapy	40%	100%	<1	<1	NR	32%	1%
ANNOVERA	Pregnancy prevention in women	100%	70%	14%	4%	30%	0%	67%
ASPARLAS	Treatment of acute lymphoblastic leukemia (ALL) in children and young adults	38%	70%	5%	4%	17%	0%	62%
BRAFTOVI**	Treatment of melanoma (a type of skin cancer) when used with another drug binimetinib	41%	91%	0%	3%	9%	29%	9%
BIKTARVY	Treatment of human immunodeficiency virus 1 (HIV-1) infection in adults	13%	63%	29%	3%	21%	2%	65%
COPIKTRA	Treatment of chronic lymphocytic leukemia (CLL), small lymphocytic lymphoma (SLL), and follicular lymphoma (FL)	35%	92%	3%	< 1	11%	61%	30%
CRYSVITA	Treatment of X-linked hypophosphatemia	60%	83%	3%	11%	8%	< 1	59%
DAURISMO	Treatment of newly diagnosed acute myeloid leukemia (ALL) in those who cannot tolerate induction chemotherapy.	28%	98%	<1	2%	NR	98%	23%
DIACOMIT	Treatment of seizures associated with Dravet syndrome in patients 2 years and older taking clobazam	53%	NR	NR	NR	NR	0%	0%
DOPTELET	Treatment of adults with low platelet count	35%	60%	3%	33%	18%	25%	19%
ELZONRIS	Treatment of blastic plasmacytoid dendritic cell neoplasm (BPDCN) in adults and children	23%	90%	4%	3%	10%	56%	100%

Table 2. Percentage of Subpopulations* - All Approvals (2018) *continued*

BRAND NAME	INDICATION	WOMEN	WHITE	BLACK or AFRICAN AMERICAN	ASIAN	HISPANIC	AGE 65 and OLDER	UNITED STATES
EMGALITY	Preventive treatment of migraine in adults	85%	77%	8%	6%	21%	< 1	62%
EPIDIOLEX	Treatment of seizures in two rare and severe forms of epilepsy.	46%	86%	4%	2%	2%	0%	75%
ERLEADA	Treatment of prostate cancer that has not spread to other parts of the body (non-metastatic)	0%	66%	6%	12%	1%	88%	28%
FIRDAPSE	Treatment of Lambert-Eaton myasthenic syndrome (LEMS) in adults	61%	89%	1%	1%	13%	20%	61%
GALAFOLD	Treatment of Fabry disease in adults	64%	97%	0%	0%	6%	1%	30%
GAMIFANT	Treatment of patients with primary hemophagocytic lymphohistiocytosis	53%	65%	9%	15%	NR	0%	24%
ILUMYA	Treatment of moderate to severe plaque psoriasis	29%	80%	3%	14%	9%	9%	33%
KRINTAFEL	Prevention of malaria relapse caused by the parasite <i>Plasmodium vivax</i>	25%	2%	11%	19%	70%	2%	0%
LIBTAYO	Treatment of advanced skin cancer (squamous cell carcinoma)	15%	96%	1%	1%	3%	72%	62%
LOKELMA	Treatment of hyperkalemia in adults	41%	85%	12%	2%	33%	57%	90%
LORBRENA	Treatment of specific type of lung cancer	58%	49%	1%	37%	NR	18%	34%

Table 2. Percentage of Subpopulations* - All Approvals (2018) *continued*

BRAND NAME	INDICATION	WOMEN	WHITE	BLACK or AFRICAN AMERICAN	ASIAN	HISPANIC	AGE 65 and OLDER	UNITED STATES
LUCEMYRA	Treatment of symptoms associated with opioid withdrawal during abrupt opioid discontinuation.	28%	67%	22%	<1	17%	<1	100%
LUMOXITI	Treatment of hairy cell leukemia in adults	21%	90%	1%	1%	6%	39%	44%
LUTATHERA	Treatment of somatostatin receptor-positive gastro-entero-pancreatic neuroendocrine tumors (GEP-NETs)	49%	82%	4%	< 1	3%	49%	58%
MEKTOVI**	Treatment of melanoma (a type of skin cancer) when used with another drug encorafenib							
MOTEGRITY	Treatment of chronic idiopathic constipation (CIC) in adults	76%	76%	3%	19%	NR	15%	3%
MOXIDECTIN	Treatment of onchocerciasis in patients 12 years of age and older	36%	0%	100%	0%	0%	9%	0%
MULPLETA	Treatment of adults with low platelet count	41%	50%	<1	47%	8%	37%	9%
NUZYRA	Treatment of bacterial skin infections known as acute bacterial skin and skin structure infections (ABSSSI) caused by certain bacteria	42%	92%	4%	< 1	35%	7%	83%
NUZYRA	Treatment of community-acquired bacterial pneumonia (CABP)	45%	92%	2%	5%	3%	42%	< 1
OLUMIANT	Treatment of adult patients with rheumatoid arthritis (RA) whose disease was not well controlled using RA medications called Tumor Necrosis Factor (TNF) antagonists	80%	67%	3%	25%	25%	18%	22%

Table 2. Percentage of Subpopulations* - All Approvals (2018) *continued*

BRAND NAME	INDICATION	WOMEN	WHITE	BLACK or AFRICAN AMERICAN	ASIAN	HISPANIC	AGE 65 and OLDER	UNITED STATES
OMEGA VEN	Treatment of parenteral nutrition-associated cholestasis (PNAC)	42%	63%	12%	4%	20%	0%	100%
ONPATTRO	Treatment of nerve damage in adult patients with hereditary transthyretin-mediated amyloidosis	26%	73%	2%	23%	12%	42%	19%
ORILISSA	Treatment of moderate to severe pain associated with endometriosis	100%	88%	9%	1%	15%	0%	75%
OXERVATE	Treatment of neurotrophic keratitis	62%	90%	3%	NR	10%	47%	31%
PALYNZIQ	Lowering the blood levels of phenylalanine in adults with phenylketonuria (PKU)	50%	98%	1%	0%	3%	0%	100%
PIFELTRO	Treatment of HIV-1 infection in adults	16%	61%	21%	9%	28%	1%	30%
POTELIGEO	Treatment of adults with mycosis fungoides or Sézary syndrome	42%	70%	10%	5%	4%	50%	54%
REVCOVI	Treatment of adenosine deaminase severe combined immune deficiency (ADA-SCID).	40%	30%	10%	40%	30%	0%	60%
SEYSARA	Treatment of certain types of acne vulgaris in patients 9 years and older	58%	77%	16%	3%	27%	0%	100%
SYMDEKO	Treatment of cystic fibrosis	50%	97%	1%	<1	3%	0%	0%
TAKHZYRO	Prevention of attacks of hereditary angioedema in people 12 years and older	70%	90%	8%	2%	4%	4%	7%

Table 2. Percentage of Subpopulations* - All Approvals (2018) *continued*

BRAND NAME	INDICATION	WOMEN	WHITE	BLACK or AFRICAN AMERICAN	ASIAN	HISPANIC	AGE 65 and OLDER	UNITED STATES
TALZENNA	Treatment of adults with a specific form of breast cancer	98%	70%	3%	11%	11%	9%	36%
TAVALISSE	Treatment of adults with low platelet count due to chronic immune thrombocytopenia (ITP)	61%	93%	3%	3%	3%	26%	13%
TEGSEDI	Treatment of nerve damage in adult patients with hereditary transthyretin-mediated amyloidosis	31%	93%	2%	2%	14%	43%	48%
TIBSOVO	Treatment of adults with acute myeloid leukemia (AML) that have a mutation in a gene called IDH1 and whose disease has come back or has not improved after previous treatment(s)	50%	63%	6%	3%	5%	37%	83%
TPOXX	Treatment of smallpox disease	59%	69%	28%	1%	11%	10%	100%
TROGARZO	Treatment of human immunodeficiency virus 1 (HIV-1) infection in adults.	15%	55%	33%	10%	25%	5%	90%
ULTOMIRIS	Treatment of adults with paroxysmal nocturnal hemoglobinuria (PNH)	47%	47%	3%	39%	6%	14%	4%
VITRAKVI	Treatment of solid tumors which are metastatic and beyond surgical resection or do not have any other therapeutic options	48%	72%	8%	3%	11%	21%	88%
VIZIMPRO	Treatment of advanced non-small cell lung cancer	60%	23%	<1	77%	0%	40%	0%
XERAVA	Treatment of complicated intra-abdominal infections caused by bacteria	43%	98%	< 1	< 1	2%	30%	5%

Table 2. Percentage of Subpopulations* - All Approvals (2018) continued

BRAND NAME	INDICATION	WOMEN	WHITE	BLACK or AFRICAN AMERICAN	ASIAN	HISPANIC	AGE 65 and OLDER	UNITED STATES
XOFLUZA	Treatment of the flu (influenza) in people 12 years of age and older	50%	26%	6%	67%	15%	0%	34%
XOSPATA	Treatment of relapsed or refractory acute myeloid leukemia with one specific gene mutation	53%	62%	5%	24%	5%	41%	57%
YUPELRI	Maintenance treatment of adults with a lung disease called COPD	51%	90%	9%	1%	4%	48%	100%
ZEMDRI	Treatment of adults who have a complicated urinary tract infection	53%	99%	0%	0%	1%	46%	1%

*The percentage of all other races combined (American Indian or Alaska Native, Native Hawaiian or other Pacific islander, Other, Unknown/Unreported) makes up to 100% of race category.

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

The percentage of patients from anywhere else in the world makes up to 100% of geographic category.

NR=not reported

**BRAFTOVI and MEKTOVI were approved using the same clinical trial population.

Therapeutic Areas in 2018

The three main therapeutic areas for 2018 were: Oncology, Infectious Diseases, and Neurology. Demographics for these approvals are provided in Tables 3, 4, and 5.

Oncology

A total of 5,157 patients participated in the trials that led to the approvals of 17 new drugs. Overall, 38% of all participants were women, 68% were White, 15% were Asian, 4% were Black or African American, 4% were Hispanic, 50% were 65 years and older, and 36% were from the sites in the United States.

Demographics for individual approvals is provided in Table 3.

Table 3. Percentage of Subpopulations - Oncology Approvals 2018

BRAND NAME	INDICATION	WOMEN	WHITE	BLACK or AFRICAN AMERICAN	ASIAN	HISPANIC	AGE 65 and OLDER	UNITED STATES
ASPARLAS	Treatment of acute lymphoblastic leukemia (ALL) in children and young adults	38%	70%	5%	4%	17%	0%	62%
BRAFTOVI*	Treatment of melanoma (a type of skin cancer) when used with another drug binimetinib	41%	91%	0%	3%	9%	29%	9%
COPIKTRA	Treatment of chronic lymphocytic leukemia (CLL), small lymphocytic lymphoma (SLL), and follicular lymphoma (FL)	35%	92%	3%	< 1	11%	61%	30%
DAURISMO	Treatment of newly diagnosed acute myeloid leukemia (ALL) in those who cannot tolerate induction chemotherapy.	28%	98%	<1	2%	NR	98%	23%
ELZONRIS	Treatment of blastic plasmacytoid dendritic cell neoplasm (BPDCN) in adults and children	23%	90%	4%	3%	10%	56%	100%
ERLEADA	Treatment of prostate cancer that has not spread to other parts of the body (non-metastatic)	0%	66%	6%	12%	1%	88%	28%

Table 3. Percentage of Subpopulations - Oncology Approvals 2018 *continued*

BRAND NAME	INDICATION	WOMEN	WHITE	BLACK or AFRICAN AMERICAN	ASIAN	HISPANIC	AGE 65 and OLDER	UNITED STATES
LIBTAYO	Treatment of advanced skin cancer (squamous cell carcinoma)	15%	96%	1%	1%	3%	72%	62%
LORBRENA	Treatment of specific type of lung cancer	58%	49%	1%	37%	NR	18%	34%
LUMOXITI	Treatment of hairy cell leukemia in adults	21%	90%	1%	1%	6%	39%	44%
LUTATHERA	For the treatment of somatostatin receptor-positive gastro-entero-pancreatic neuroendocrine tumors (GEP-NETs)	49%	82%	4%	< 1	3%	49%	58%
MEKTOVI*	Treatment of melanoma (a type of skin cancer) when used with another drug encorafenib							
POTELIGEO	Treatment of adults with mycosis fungoides or Sézary syndrome	42%	70%	10%	5%	4%	50%	54%
TALZENNA	Treatment of adults with a specific form of breast cancer	98%	70%	3%	11%	11%	9%	36%
TIBSOVO	Treatment of adults with acute myeloid leukemia (AML) that have a mutation in a gene called IDH1 and whose disease has come back or has not improved after previous treatment(s)	50%	63%	6%	3%	5%	37%	83%
VITRAKVI	Treatment of solid tumors which are metastatic and beyond surgical resection or do not have any other therapeutic options	48%	72%	8%	3%	11%	21%	88%
VIZIMPRO	Treatment of advanced non-small cell lung cancer	60%	23%	<1	77%	0%	40%	0%
XOSPATA	Treatment of relapsed or refractory acute myeloid leukemia with one specific gene mutation	53%	62%	5%	24%	5%	41%	57%

NR=not reported

* BRAFTOVI and MEKTOVI were approved using the same clinical trial population

Infectious Diseases

A total of 12,404 patients participated in the trials that led to the approvals of 11 new drugs. Overall, 33% of all participants were women, 61% were White, 10% were Asian, 23% were Black or African American, 17% were Hispanic, 11% were 65 years and older, and 33% were from the sites in the United States.

Demographics for individual approvals is provided in Table 4.

Table 4. Percentage of Subpopulations - Infectious Diseases Approvals 2018

BRAND NAME	INDICATION	WOMEN	WHITE	BLACK or AFRICAN AMERICAN	ASIAN	HISPANIC	AGE 65 and OLDER	UNITED STATES
AEMCOLO	Treatment of traveler's diarrhea in adults	50%	83%	1%	14%	3%	9%	0%
BIKTARVY	Treatment of human immunodeficiency virus 1 (HIV-1) infection in adults	13%	63%	29%	3%	21%	2%	65%
KRINTAFEL	Prevention of malaria relapse caused by the parasite, Plasmodium vivax	25%	2%	11%	19%	70%	2%	0%
MOXIDECTIN	Treatment of onchocerciasis in patients 12 years of age and older	36%	0%	100%	0%	0%	9%	0%
NUZYRA	Treatment of bacterial skin infections known as acute bacterial skin and skin structure infections (ABSSSI) caused by certain bacteria	42%	92%	4%	< 1	35%	7%	83%
NUZYRA	Treatment of community-acquired bacterial pneumonia (CABP)	45%	92%	2%	5%	3%	42%	< 1
PIFELTRO	Treatment of HIV-1 infection in adults	16%	61%	21%	9%	28%	1%	30%

Table 4. Percentage of Subpopulations - Infectious Diseases Approvals 2018 *continued*

BRAND NAME	INDICATION	WOMEN	WHITE	BLACK or AFRICAN AMERICAN	ASIAN	HISPANIC	AGE 65 and OLDER	UNITED STATES
TPOXX	Treatment of smallpox disease	59%	69%	28%	1%	11%	10%	100%
TROGARZO	Treatment of human immunodeficiency virus 1 (HIV-1) infection in adults.	15%	55%	33%	10%	25%	5%	90%
XERAVA	Treatment of complicated intra-abdominal infections caused by bacteria	43%	98%	< 1	< 1	2%	30%	5%
XOFLUZA	Treatment of the flu (influenza) in people 12 years of age and older	50%	26%	6%	67%	15%	0%	34%
ZEMDRI	Treatment of adults who have a complicated urinary tract infection	53%	99%	0%	0%	1%	46%	0.5%

NR=not reported

Neurology

A total of 7,401 patients participated in the trials that led to the approvals of 8 new drugs. Overall, 79% of all participants were women, 60% were White, 6% were Asian, 7% were Black or African American, 12% were Hispanic, 3% were 65 years and older, and 60% were from the sites in the United States.

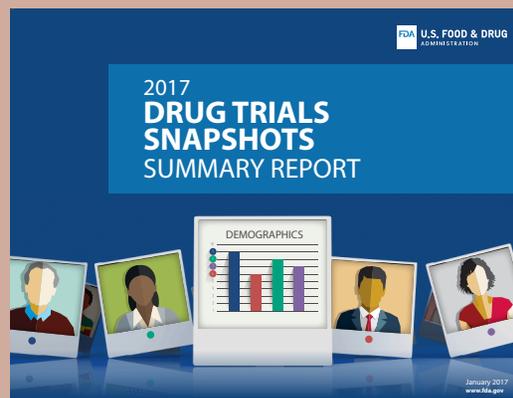
Demographics for individual approvals is provided in Table 5.

Table 5. Percentage of Subpopulations - Neurology Approvals 2018

BRAND NAME	INDICATION	WOMEN	WHITE	BLACK or AFRICAN AMERICAN	ASIAN	HISPANIC	AGE 65 and OLDER	UNITED STATES
AIMOVIG	Preventive treatment of migraine in adults	85%	15%	7%	1%	7%	0%	49%
AJOVY	Preventive treatment of migraine in adults	87%	80%	9%	10%	10%	2%	73%
DIACOMIT	Treatment of seizures associated with Dravet syndrome in patients 2 years of age and older taking clobazam	53%	NR	NR	NR	NR	0%	0%
EMGALITY	Preventive treatment of migraine in adults	85%	77%	8%	6%	21%	< 1	62%
EPIDIOLEX	Treatment of seizures in two rare and severe forms of epilepsy.	46%	86%	4%	2%	2%	0%	75%
FIRDAPSE	Treatment of Lambert-Eaton myasthenic syndrome (LEMS) in adults	61%	89%	1%	1%	13%	20%	61%
ONPATTRO	Treatment of nerve damage in adult patients with hereditary transthyretin-mediated amyloidosis	26%	73%	2%	23%	12%	42%	19%
TEGSEDI	Treatment of nerve damage in adult patients with hereditary transthyretin-mediated amyloidosis	31%	93%	2%	2%	14%	43%	48%

NR=not reported

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