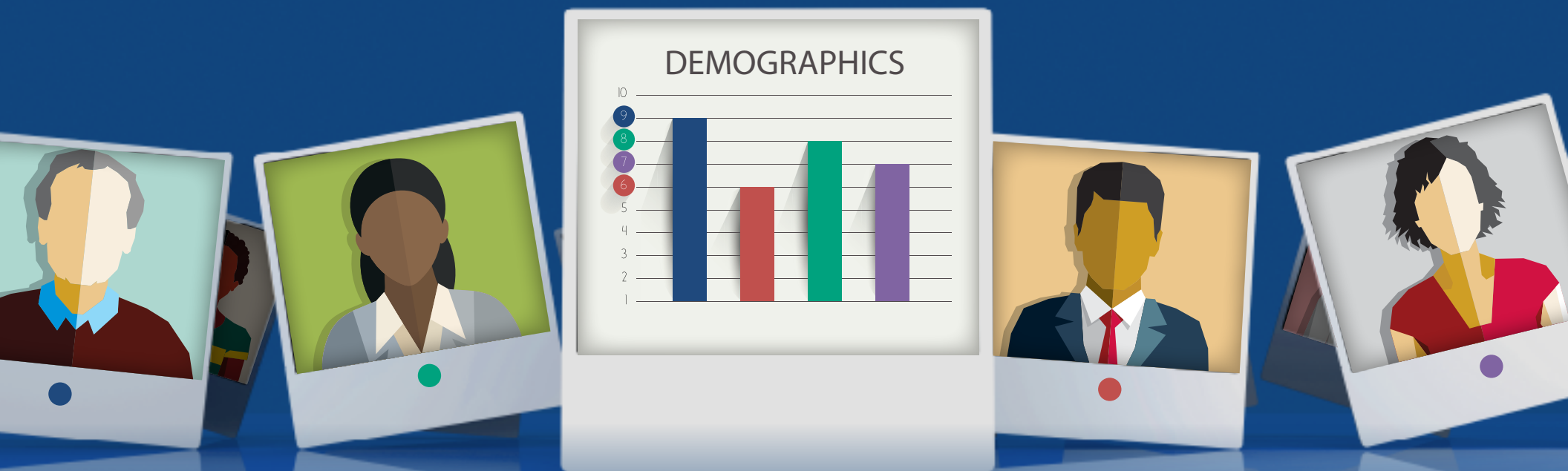


2017 DRUG TRIALS SNAPSHOTS SUMMARY REPORT



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 minority groups, has become of greater interest to the general public. As part of the 2012 Food and Drug Administration Safety and Innovation Act (FDASIA 907), the U.S. Congress required the U.S. Food and Drug Administration (FDA) to report on the diversity of participants in clinical trials and the extent to which safety and efficacy data are based on demographic factors such as sex, age, and race. Recognizing the lack of easily accessible information about participation in drug trials, CDER created the transparency initiative called the Drug Trials Snapshots.

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, and age 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 been publishing 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 and better understanding of the drug development process. This year's report summarizes the 2017 Drug Trials Snapshot program and the extent to which safety and efficacy data are 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, and age participating in the clinical trials used to approve the drug. In addition to demographics, this year's report also details the percentage of clinical trial participants based on ethnicity and geographic location.

We hope this information is helpful to promote dialogue on the appropriate representation of different subgroups in clinical trials and welcome your feedback.



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2017 Summary Statistics

(Jan 1, 2017 - Dec 31, 2017)

In 2017, CDER approved 46 novel drugs*, either as new molecular entities (NMEs) under New Drug Applications (NDAs) or as new therapeutic biologics under Biologics License Applications (BLAs). Overall, 59,030 patients participated in these trials. Subpopulation demographics are presented below.

Table 1. Demographic Subgroups in 2017

DEMOGRAPHIC SUBGROUPS	WOMEN	WHITE	ASIAN	BLACK or AFRICAN AMERICAN	HISPANIC	AGE 65 AND OLDER	UNITED STATES
PARTICIPANT AVERAGE	55%	77%	11%	7%	14%	32 %	34%

*Data presented in this report are from 47 snapshots as one drug was approved for 2 different diseases (populations).

More insight into demographics for all 46 CDER approved NMEs are provided below in Table 2.

Table 2. Percentage of Subpopulations* - All Approvals (2017)

BRAND NAME	INDICATION	WOMEN	WHITE	ASIAN	BLACK or AFRICAN AMERICAN	HISPANIC	AGE 65 and OLDER	UNITED STATES
ALIQOPA	Treatment of relapsed follicular lymphoma	54%	83%	9%	0%	6%	48%	13%
ALUNBRIG	Treatment of metastatic non-small cell lung cancer	57%	67%	31%	1%	6%	23%	NR
AUSTEDO	Treatment of chorea associated with Huntington's disease	44%	92%	0%	6%	0%	19%	93%
BAVENCIO	Treatment of metastatic Merkel cell carcinoma	26%	92%	3%	0%	6%	75%	58%

Table 2. Percentage of Subpopulations* - All Approvals (2017) *continued*

BRAND NAME	INDICATION	WOMEN	WHITE	ASIAN	BLACK or AFRICAN AMERICAN	HISPANIC	AGE 65 and OLDER	UNITED STATES
BAXDELA	Treatment of acute bacterial skin and skin structure infection	37%	86%	0%	5%	28%	14%	52%
BENZNIDAZOLE	Treatment of Chagas disease	46%	NR	NR	NR	NR	0%	0%
BESPONSA	Treatment of B-cell acute lymphocytic leukemia	41%	71%	17%	2%	NR	37%	47%
BEVYXXA	Prevention of venous thromboembolism	54%	93%	<1%	2%	11%	90%	7%
BRINEURA	Treatment of Batten disease	63%	96%	4%	0%	4%	0%	13%
CALQUENCE	Treatment of mantle cell lymphoma	20%	74%	0%	3%	3%	65%	36%
DUPIXENT	Treatment of atopic dermatitis	42%	67%	24%	6%	4%	4%	34%
EMFLAZA	Treatment of Duchenne muscular dystrophy	0%	94%	1%	0%	NR	0%	NR
FASENRA	Treatment of eosinophilic asthma	63%	80%	12%	3%	20%	13%	15%
GIAPREZA	To increase blood pressure in septic or other distributive shock	39%	80%	4%	10%	5%	48%	74%
HEMLIBRA	To prevent or reduce bleeding in patients with hemophilia A	0%	56%	26%	11%	12%	3%	28%
IDHIFA	Treatment of relapsed acute myelogenous leukemia	49%	77%	<1%	6%	9%	60%	83%

Table 2. Percentage of Subpopulations* - All Approvals (2017) *continued*

BRAND NAME	INDICATION	WOMEN	WHITE	ASIAN	BLACK or AFRICAN AMERICAN	HISPANIC	AGE 65 and OLDER	UNITED STATES
IMFINZI	Treatment of urothelial carcinoma	28%	64%	20%	3%	NR	62%	49%
INGREZZA	Treatment of tardive dyskinesia	42%	57%	<1%	39%	28%	18%	100%
KEVZARA	Treatment of rheumatoid arthritis	82%	81%	6%	3%	39%	13%	14%
KISQALI	Treatment of HR-positive, HER2-negative breast cancer	100%	82%	8%	3%	10%	44%	32%
MACRILEN	Diagnosis of adult growth hormone deficiency	42%	86%	3%	2%	NR	2%	24%
MAVYRET	Treatment of chronic hepatitis C genotypes 1, 2, 3, 4, 5 or 6	44%	80%	12%	6%	9%	14%	32%
MEPSEVII	Treatment of mucopolysaccharidosis VII	67%	75%	0%	0%	50%	0%	100%
NERLYNX	Treatment of HER2 positive breast cancer	100%	81%	13%	3%	2%	12%	32%
OCREVUS	Treatment of multiple sclerosis	61%	92%	0%	4%	11%	0%	26%
OZEMPIC	Treatment of type 2 diabetes mellitus	42%	70%	22%	6%	16%	34%	28%
PARSABIV	Treatment of secondary hyperparathyroidism in patients with chronic kidney disease on hemodialysis	40%	66%	3%	28%	13%	35%	52%
PREVYMIS	Prevention of cytomegalovirus infection in allogeneic hematopoietic stem cell transplant	42%	82%	10%	2%	7%	16%	36%

Table 2. Percentage of Subpopulations* - All Approvals (2017) *continued*

BRAND NAME	INDICATION	WOMEN	WHITE	ASIAN	BLACK or AFRICAN AMERICAN	HISPANIC	AGE 65 and OLDER	UNITED STATES
RADICAVA	Treatment of amyotrophic lateral sclerosis	39%	0%	100%	0%	0%	30%	0%
RHOPRESSA	Treatment of open angle glaucoma	62%	73%	2%	25%	19%	54%	100%
RYDAPT	Treatment of acute myelogenous leukemia	56%	38%	2%	2%	2%	0%	33%
RYDAPT	Treatment of systemic mastocytosis	36%	93%	0%	1%	3%	45%	41%
SILIQ	Treatment of plaque psoriasis	31%	91%	3%	3%	NR	6%	22%
SOLOSEC	Treatment of bacterial vaginosis	100%	46%	1%	50%	15%	0%	100%
STEGLATRO	Treatment of type 2 diabetes mellitus	48%	77%	13%	5%	24%	26%	28%
SYMPROIC	Treatment of opioid-induced constipation	62%	80%	1%	18%	8%	15%	85%
TREMFYA	Treatment of plaque psoriasis	29%	82%	14%	2%	6%	5%	20%
TRULANCE	Treatment of chronic idiopathic constipation	79%	72%	3%	23%	41%	11%	98%
TYMLOS	Treatment of postmenopausal osteoporosis	100%	80%	16%	3%	24%	81%	2%
VABOMERE	Treatment of complicated urinary tract infection	66%	93%	2%	1%	8%	35%	3%
VERZENIO	Treatment of HR-positive, HER2-negative breast cancer	100%	61%	27%	2%	12%	36%	19%

Table 2. Percentage of Subpopulations* - All Approvals (2017) *continued*

BRAND NAME	INDICATION	WOMEN	WHITE	ASIAN	BLACK or AFRICAN AMERICAN	HISPANIC	AGE 65 and OLDER	UNITED STATES
VOSEVI	Treatment of hepatitis C genotypes 1, 2, 3, 4, 5 or 6	23%	84%	2%	12%	7%	16%	57%
VYZULTA	Treatment of glaucoma	58%	74%	1%	24%	12%	52%	91%
XADAGO	Treatment of Parkinson's disease	33%	41%	59%	<1%	2%	38%	8%
XEPI	Treatment of impetigo	44%	53%	4%	34%	17%	3%	23%
XERMELO	Treatment of carcinoid syndrome diarrhea	50%	90%	0%	1%	0%	49%	32%
ZEJULA	Treatment of ovarian cancer	100%	87%	3%	1%	3%	35%	70%

*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

Therapeutic Areas in 2017

The four main therapeutic areas for 2017 were: Oncology, Infectious Diseases, Neurology and Endocrinology and Metabolism. Demographics for these approvals are provided in Tables 3, 4, 5 and 6.

Oncology

A total of 7,045 patients participated in the trials that led to the approvals of 12 new drugs. Overall, 83% of all participants were women, 74% were White, 12% were Asian, 2% were Black or African American, 4% were Hispanic, 26% were 65 years and older, and 34% were from the United States.

Demographics for individual approvals is provided in the Table 3.

Table 3. Percentage of Subpopulations-Oncology Approvals 2017

BRAND NAME	INDICATION	WOMEN	WHITE	ASIAN	BLACK or AFRICAN AMERICAN	HISPANIC	AGE 65 and OLDER	UNITED STATES
ALIQOPA	Treatment of relapsed follicular lymphoma	54%	83%	9%	0%	6%	48%	13%
ALUNBRIG	Treatment of metastatic non-small cell lung cancer	57%	67%	31%	1%	6%	23%	NR
BAVENCIO	Treatment of metastatic Merkel cell carcinoma	26%	92%	3%	0%	6%	75%	58%
BESPONSA	Treatment of B-cell acute lymphocytic leukemia	41%	71%	17%	2%	NR	37%	47%
CALQUENCE	Treatment of mantle cell lymphoma	20%	74%	0%	3%	3%	65%	36%
IDHIFA	Treatment of relapsed acute myelogenous leukemia	49%	77%	<1%	6%	9%	60%	83%
IMFINZI	Treatment of urothelial carcinoma	28%	64%	20%	3%	NR	62%	49%
KISQALI	Treatment of HR-positive, HER2-negative breast cancer	100%	82%	8%	3%	10%	44%	32%

Table 3. Percentage of Subpopulations-Oncology Approvals 2017 *continued*

BRAND NAME	INDICATION	WOMEN	WHITE	ASIAN	BLACK or AFRICAN AMERICAN	HISPANIC	AGE 65 and OLDER	UNITED STATES
NERLYNX	Treatment of HER2 positive Breast cancer	100%	81%	13%	3%	2%	12%	32%
RYDAPT	Treatment of acute myelogenous leukemia	56%	38%	2%	2%	2%	0%	33%
RYDAPT	Treatment of systemic mastocytosis	36%	93%	0%	1%	3%	45%	41%
VERZENIO	Treatment of HR-positive, HER2-negative breast cancer	100%	61%	27%	2%	12%	36%	19%
ZEJULA	Treatment of ovarian cancer	100%	87%	3%	1%	3%	35%	70%

NR=not reported

Infectious Diseases

A total of 7,026 patients participated in the trials that led to the approvals of 8 new drugs. Overall, 45% of all participants were women, 76% were White, 6% were Asian, 11% were Black or African American, 13% were Hispanic, 14% were 65 years and older, and 41% were from the United States.

Demographics for individual approvals is provided in the Table 4.

Table 4. Percentage of Subpopulations- Infectious Diseases Approvals 2017

BRAND NAME	INDICATION	WOMEN	WHITE	ASIAN	BLACK or AFRICAN AMERICAN	HISPANIC	AGE 65 and OLDER	UNITED STATES
BAXDELA	Treatment of acute bacterial skin and skin structure infection	37%	86%	0%	5%	28%	14%	52%
BENZNIDAZOLE	Treatment of Chagas disease	46%	NR	NR	NR	NR	0%	0%
MAVYRET	Treatment of chronic hepatitis C genotypes 1, 2, 3, 4, 5 or 6	44%	80%	12%	6%	9%	14%	32%

Table 4. Percentage of Subpopulations- Infectious Diseases Approvals 2017 *continued*

BRAND NAME	INDICATION	WOMEN	WHITE	ASIAN	BLACK or AFRICAN AMERICAN	HISPANIC	AGE 65 and OLDER	UNITED STATES
PREVYMIS	Prevention of cytomegalovirus infection in allogeneic hematopoietic stem cell transplant	42%	82%	10%	2%	7%	16%	36%
SOLOSEC	Treatment of bacterial vaginosis	100%	46%	1%	50%	15%	0%	100%
VABOMERE	Treatment of complicated urinary tract infection	66%	93%	2%	1%	8%	35%	3%
VOSEVI	Treatment of hepatitis C genotypes 1, 2, 3, 4, 5 or 6	23%	84%	2%	12%	7%	16%	57%
XEPI	Treatment of impetigo	44%	53%	4%	34%	17%	3%	23%

NR=not reported

Neurology

A total of 4,610 patients participated in the trials that led to the approvals of 6 new drugs. Overall, 48% of all participants were women, 68% were White, 23% were Asian, 6% were Black or African American, 9% were Hispanic, 14% were 65 years and older, and 23% were from the United States.

Demographics for individual approvals is provided in the Table 5.

Table 5. Percentage of Subpopulations-Neurology Approvals 2017

BRAND NAME	INDICATION	WOMEN	WHITE	ASIAN	BLACK or AFRICAN AMERICAN	HISPANIC	AGE 65 and OLDER	UNITED STATES
AUSTEDO	Treatment of chorea associated with Huntington's disease	44%	92%	0%	6%	0%	19%	93%
EMFLAZA	Treatment of Duchenne muscular dystrophy	0%	94%	1%	0%	NR	0%	NR
INGREZZA	Treatment of tardive dyskinesia	42%	57%	<1%	39%	28%	18%	100%

Table 5. Percentage of Subpopulations-Neurology Approvals 2017 *continued*

BRAND NAME	INDICATION	WOMEN	WHITE	ASIAN	BLACK or AFRICAN AMERICAN	HISPANIC	AGE 65 and OLDER	UNITED STATES
OCREVUS	Treatment of multiple sclerosis	61%	92%	0%	4%	11%	0%	26%
RADICAVA	Treatment of amyotrophic lateral sclerosis	39%	0%	100%	0%	0%	30%	0%
XADAGO	Treatment of Parkinson's disease	33%	41%	59%	<1%	2%	38%	8%

NR=not reported

Endocrinology and Metabolism

A total of 15,777 patients participated in the trials that led to the approvals of 5 new drugs. Overall, 49% of all participants were women, 73% were White, 17% were Asian, 7% were Black or African American, 19% were Hispanic, 36% were 65 years and older, and 27% were from the United States.

Demographics for individual approvals is provided in the Table 6.

Table 6. Percentage of Subpopulations-Endocrinology and Metabolism Approvals 2017

BRAND NAME	INDICATION	WOMEN	WHITE	ASIAN	BLACK or AFRICAN AMERICAN	HISPANIC	AGE 65 and OLDER	UNITED STATES
MACRILEN	Diagnosis of adult growth hormone deficiency	42%	86%	3%	2%	NR	2%	24%
OZEMPIC	Treatment of type 2 diabetes mellitus	42%	70%	31%	6%	16%	34%	28%
PARSABIV	Treatment of secondary hyperparathyroidism in patients with chronic kidney disease on hemodialysis	40%	66%	3%	28%	13%	35%	52%
TYMLOS	Treatment of postmenopausal osteoporosis	100%	80%	16%	3%	24%	81%	2%
STEGLATRO	Treatment of type 2 diabetes mellitus	48%	77%	13%	5%	24%	26%	28%

NR=not reported



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