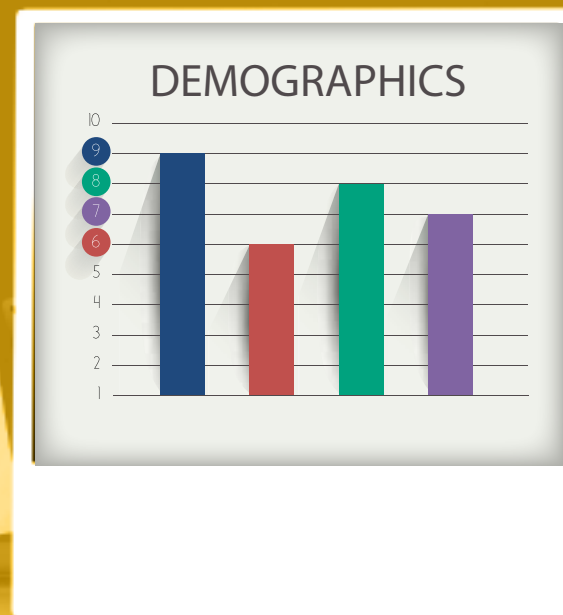


2019 DRUG TRIALS SNAPSHOTS SUMMARY REPORT



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 2019 Drug Trials Snapshot program, highlighting 48 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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2019 Summary Statistics

(Jan 1, 2019 - Dec 31, 2019)

In 2019, CDER approved 48 novel drugs*, either as New Molecular Entities (NMEs) under New Drug Applications (NDAs) or as new therapeutic biologics under Biologics License Applications (BLAs). Overall, 46,391 patients participated in these trials. Subpopulation demographics from these trials are presented below.

Table 1. Demographic Subgroups in 2019

| DEMOGRAPHIC SUBGROUPS | WOMEN | WHITE | BLACK or AFRICAN AMERICAN | ASIAN | HISPANIC | AGE 65 AND OLDER | UNITED STATES |
|-----------------------|-------|-------|---------------------------|-------|----------|------------------|---------------|
| AVERAGE | 72% | 72% | 9% | 9% | 18% | 36% | 40% |

*Data presented in this report are from 49 snapshots as one drug was approved for two indications.

More insight into demographics for all 49 CDER approved NMEs are provided below in Table 2.

Table 2. Percentage of Subpopulations* - All Approvals

| BRAND NAME | INDICATION | WOMEN | WHITE | BLACK or AFRICAN AMERICAN | ASIAN | HISPANIC | AGE 65 and OLDER | UNITED STATES |
|------------|--|-------|-------|---------------------------|-------|----------|------------------|---------------|
| ACCRUFER | Treatment of iron deficiency | 68 | 83 | 12 | 2 | 14 | 41 | 57 |
| ADAKVEO | Treatment of vasoocclusive crisis in sickle cell disease | 55 | 5 | 91 | NR | 24 | 0 | 75 |
| AKLIEF | Treatment of acne vulgaris | 55 | 87 | 7 | 3 | 17 | 0 | 45 |

Table 2. Percentage of Subpopulations* - All Approvals *continued*

| BRAND NAME | INDICATION | WOMEN | WHITE | BLACK or AFRICAN AMERICAN | ASIAN | HISPANIC | AGE 65 and OLDER | UNITED STATES |
|------------------|---|-------|-------|---------------------------------|-------|----------|---------------------|------------------|
| BALVERSA | Treatment of urothelial carcinoma | 21 | 74 | 2 | 5 | 2 | 61 | 22 |
| BEOVU | Treatment of wet age-related macular degeneration | 56 | 86 | 1 | 11 | 8 | 90 | 33 |
| BRUKINSA | Treatment of mantle cell lymphoma | 25 | 21 | 1 | 76 | NR | 39 | 8 |
| CABLIVI | Treatment of an episode of acquired thrombotic thrombocytopenic purpura | 67 | 74 | 17 | 2 | 3 | 6 | 22 |
| CAPLYTA | Treatment of schizophrenia | 24 | 21 | 75 | 1 | 9 | 0 | 100 |
| DAYVIGO | Treatment of insomnia | 76 | 73 | 15 | 11 | 12 | 35 | 54 |
| EGATEN | Treatment of fascioliasis | 58 | NR | NR | NR | NR | 4 | 0 |
| ENHERTU | Metastatic HER2-positive breast cancer | 100 | 51 | 3 | 42 | 6 | 26 | 35 |
| EVENITY | Treatment of osteoporosis in postmenopausal women | 100 | 62 | 2 | 10 | 37 | 82 | 2 |
| EXEM FOAM | Detection of fallopian tubes patency in women with infertility | 100 | NR | NR | NR | NR | NR | 0 |
| FETROJA | Treatment of complicated urinary tract infection | 55 | 96 | NR | 3 | 2 | 53 | 2 |

Table 2. Percentage of Subpopulations* - All Approvals *continued*

| BRAND NAME | INDICATION | WOMEN | WHITE | BLACK or AFRICAN AMERICAN | ASIAN | HISPANIC | AGE 65 and OLDER | UNITED STATES |
|----------------------------|--|-----------|-----------|---------------------------------|-----------|-----------|---------------------|------------------|
| FLUORODOPA F 18 | Detection of certain nerve cells in patients with Parkinsonian syndromes | 32 | NR | NR | NR | NR | 63 | 100 |
| GIVLAARI | Treatment of acute hepatic porphyria | 89 | 78 | 1 | 16 | 9 | 1 | 34 |
| Ga 68 DOTATOC | Detection of somatostatin receptor positive neuroendocrine tumors | 57 | 94 | 2 | 1 | 2 | 18 | 100 |
| IBSRELA | Treatment of irritable bowel syndrome with constipation | 82 | 64 | 31 | 3 | 28 | 8 | 100 |
| INREBIC | Treatment of myelofibrosis | 43 | 92 | 1 | 7 | NR | 45 | 8 |
| JEUVEAU | Temporary improvement of glabellar lines | 91 | 84 | 8 | 2 | 0 | 10 | 100 |
| MAYZENT | Treatment of relapsing forms of multiple sclerosis | 60 | 95 | 1 | 3 | 6 | 0 | 9 |
| NOURIANZ | Treatment of "off episodes" in patients with Parkinson's disease | 49 | 34 | 1 | 64 | NR | 52 | 32 |
| NUBEQA | Treatment of prostate cancer | 0 | 79 | 3 | 13 | 3 | 87 | 9 |
| OXBRYTA | Treatment of sickle cell disease | 59 | 24 | 67 | 1 | 3 | 0 | 36 |
| PADCEV | Treatment of urothelial carcinoma | 30 | 85 | 2 | 9 | 4 | 64 | 94 |

Table 2. Percentage of Subpopulations* - All Approvals *continued*

| BRAND NAME | INDICATION | WOMEN | WHITE | BLACK or AFRICAN AMERICAN | ASIAN | HISPANIC | AGE 65 and OLDER | UNITED STATES |
|--------------------|--|------------|-----------|---------------------------------|-----------|-----------|---------------------|------------------|
| PIQRAY | Treatment of advanced breast cancer | 100 | 66 | 1 | 22 | 14 | 44 | 9 |
| POLIVY | Treatment of diffuse large B- cell lymphoma | 34 | 71 | 4 | 12 | 3 | 61 | 30 |
| PRETOMANID | Treatment of lung tuberculosis | 48 | 1 | 76 | NR | NR | 0 | 0 |
| REBLOZYL | Treatment of anemia in patients with beta thalassemia | 58 | 54 | 1 | 35 | 2 | 0 | 8 |
| RECARBRIO | Treatment of complicated urinary infection | 43 | 92 | 1 | 3 | 6 | 30 | 4 |
| REYVOW | Treatment of acute migraine | 84 | 78 | 18 | 1 | 18 | 4 | 90 |
| RINVOQ | Treatment of rheumatoid arthritis | 79 | 85 | 6 | 7 | 27 | 20 | 26 |
| ROZLYTREK | Treatment of non-small cell lung cancer | 55 | 66 | 5 | 23 | 3 | 25 | 51 |
| ROZLYTREK** | Treatment of certain solid tumors | | | | | | | |
| SCENESSE | Increasing pain-free light exposure in patients with EPP | 47 | 98 | 0 | 1 | NR | 4 | 70 |

Table 2. Percentage of Subpopulations* - All Approvals *continued*

| BRAND NAME | INDICATION | WOMEN | WHITE | BLACK or AFRICAN AMERICAN | ASIAN | HISPANIC | AGE 65 and OLDER | UNITED STATES |
|-----------------------|---|-------|-------|---------------------------------|-------|----------|---------------------|------------------|
| SKYRIZI | Treatment of plaque psoriasis | 30 | 78 | 3 | 17 | 11 | 11 | 40 |
| SUNOSI | Improvement of wakefulness in patients with narcolepsy or obstructive sleep apnea | 49 | 77 | 18 | 2 | 8 | 12 | 90 |
| TISSUEBLUE | Staining the internal limiting membrane | NR | NR | NR | NR | NR | NR | NR |
| TRIKAFTA | Treatment of cystic fibrosis | 50 | 93 | 1 | NR | 4 | 0 | 60 |
| TURALIO | Treatment of tenosynovial giant cell tumor | 59 | 88 | 3 | 3 | 14 | 6 | 38 |
| UBRELVY | Treatment of acute migraine | 89 | 82 | 15 | 1 | 15 | 3 | 100 |
| VYLEESI | Treatment of women with hypoactive sexual desire disorder | 100 | 86 | 12 | 1 | 9 | 0 | 97 |
| VYNDAQEL/ VYNDAMAX | Treatment of certain type of cardiomyopathy | 10 | 81 | 14 | NR | 3 | 91 | 63 |
| VYONDYS 53 | Treatment of Duchenne muscular dystrophy | 0 | 86 | 2 | 6 | 16 | 0 | 81 |
| WAKIX | Treatment of excessive daytime sleepiness in patients with narcolepsy | 50 | 55 | 2 | NR | NR | 6 | 0 |
| XCOPRI | Treatment of partial-onset seizures | 49 | 74 | 3 | 21 | 7 | 2 | 29 |

Table 2. Percentage of Subpopulations* - All Approvals *continued*

| BRAND NAME | INDICATION | WOMEN | WHITE | BLACK or AFRICAN AMERICAN | ASIAN | HISPANIC | AGE 65 and OLDER | UNITED STATES |
|-----------------|---|------------|-----------|---------------------------------|-----------|-----------|---------------------|------------------|
| XENLETA | Treatment of community-acquired bacterial pneumonia | 44 | 79 | 5 | 11 | 8 | 40 | 2 |
| XPOVIO | Treatment of multiple myeloma | 47 | 73 | 17 | 1 | 5 | 48 | 81 |
| ZULRESSO | Treatment of postpartum depression | 100 | 61 | 36 | 1 | 17 | 0 | 100 |

*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

**ROZLYTREK indications were approved using the same clinical trial population

Therapeutic Areas in 2019

The four main therapeutic areas for 2019 were: Hematology, Oncology, Neurology and Psychiatry and Sleep Disorders. Demographics for these approvals are provided in Tables 3, 4, and 5.

Hematology

A total of 1,255 patients participated in the trials that led to the approvals of 6 new drugs. Overall, 64% of all participants were women, 57% were White, 11% were Asian, 25% were Black or African American, 8% were Hispanic, 11% were 65 years and older, and 35% were from sites in the United States.

Demographics for individual approvals is provided in the Table 3.

Table 3. Percentage of Subpopulations - Hematology Approvals

| BRAND NAME | INDICATION | WOMEN | WHITE | BLACK or AFRICAN AMERICAN | ASIAN | HISPANIC | AGE 65 and OLDER | UNITED STATES |
|-----------------|---|-----------|-----------|---------------------------------|-----------|-----------|---------------------|------------------|
| ACCRUFER | Treatment of iron deficiency | 68 | 83 | 12 | 2 | 14 | 41 | 57 |
| ADAKVEO | Treatment of vasoocclusive crisis in sickle cell disease | 55 | 5 | 91 | NR | 24 | 0 | 75 |
| CABLIVI | Treatment of an episode of acquired thrombotic thrombocytopenic purpura | 67 | 74 | 17 | 2 | 3 | 6 | 22 |
| GIVLAARI | Treatment of acute hepatic porphyria | 89 | 78 | 1 | 16 | 9 | 1 | 34 |
| OXBRYTA | Treatment of sickle cell disease | 59 | 24 | 67 | 1 | 3 | 0 | 36 |
| REBLOZYL | Treatment of anemia in patients with beta thalassemia | 58 | 54 | 1 | 35 | 2 | 0 | 8 |

NR=not reported

Oncology

A total of 3,593 patients participated in the trials that led to the approvals of 11 new drugs. Overall, 38% of all participants were women, 73% were White, 18% were Asian, 4% were Black or African American, 5% were Hispanic, 59% were 65 years and older, and 24% were from sites in the United States.

Demographics for individual approvals is provided in the Table 4.

Table 4. Percentage of Subpopulations - Oncology Approvals

| BRAND NAME | INDICATION | WOMEN | WHITE | BLACK or AFRICAN AMERICAN | ASIAN | HISPANIC | AGE 65 and OLDER | UNITED STATES |
|-----------------|---|-------|-------|---------------------------------|-------|----------|---------------------|------------------|
| BALVERSA | Treatment of urothelial carcinoma | 21 | 74 | 2 | 5 | 2 | 61 | 22 |
| BRUKINSA | Treatment of mantle cell lymphoma | 25 | 21 | 1 | 76 | NR | 39 | 8 |
| ENHERTU | Treatment of metastatic HER2-positive breast cancer | 100 | 51 | 3 | 42 | 6 | 26 | 35 |
| INREBIC | Treatment of myelofibrosis | 43 | 92 | 1 | 7 | NR | 45 | 8 |
| NUBEQA | Treatment of prostate cancer | 0 | 79 | 3 | 13 | 3 | 87 | 9 |
| PADCEV | Treatment of urothelial carcinoma | 30 | 85 | 2 | 9 | 4 | 64 | 94 |
| PIQRAY | Treatment of advanced breast cancer | 100 | 66 | 1 | 22 | 14 | 44 | 9 |

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

| BRAND NAME | INDICATION | WOMEN | WHITE | BLACK or AFRICAN AMERICAN | ASIAN | HISPANIC | AGE 65 and OLDER | UNITED STATES |
|------------------|--|-----------|-----------|---------------------------------|-----------|-----------|---------------------|------------------|
| POLIVY | Treatment of diffuse large B-cell lymphoma | 34 | 71 | 4 | 12 | 3 | 61 | 30 |
| ROZLYTREK | Treatment of non-small cell lung cancer | 55 | 66 | 5 | 23 | 3 | 25 | 51 |
| ROZLYTREK | Treatment of certain solid tumors | | | | | | | |
| TURALIO | Treatment of tenosynovial giant cell tumor | 59 | 88 | 3 | 3 | 14 | 6 | 38 |
| XPOVIO | Treatment of multiple myeloma | 47 | 73 | 17 | 1 | 5 | 48 | 81 |

NR=not reported

Neurology

A total of 10,390 patients participated in the trials that led to the approvals of 6 new drugs. Overall, 75% of all participants were women, 77% were White, 10% were Asian, 11% were Black or African American, 13% were Hispanic, 8% were 65 years and older, and 70% were from sites in the United States.

Demographics for individual approvals is provided in the Table 5.

Table 5. Percentage of Subpopulations - Neurology Approvals

| BRAND NAME | INDICATION | WOMEN | WHITE | BLACK or AFRICAN AMERICAN | ASIAN | HISPANIC | AGE 65 and OLDER | UNITED STATES |
|-------------------|--|-----------|-----------|---------------------------------|-----------|-----------|------------------------|------------------|
| MAYZENT | Treatment of relapsing forms of multiple sclerosis | 60 | 95 | 1 | 3 | 6 | 0 | 9 |
| NOURIANZ | Treatment of "off episodes" in patients with Parkinson's disease | 49 | 34 | 1 | 64 | NR | 52 | 32 |
| REYVOW | Treatment of acute migraine | 84 | 78 | 18 | 1 | 18 | 4 | 90 |
| UBRELVY | Treatment of acute migraine | 89 | 82 | 15 | 1 | 15 | 3 | 100 |
| VYONDYS 53 | Treatment of Duchenne muscular dystrophy | 0 | 86 | 2 | 6 | 16 | 0 | 81 |
| XCOPRI | Treatment of partial-onset seizures | 49 | 74 | 3 | 21 | 7 | 2 | 29 |

NR=not reported

Psychiatry and Sleep Disorders

A total of 3,645 patients participated in the trials that led to the approvals of 5 new drugs. Overall, 60% of all participants were women, 60% were White, 6% were Asian, 29% were Black or African American, 10% were Hispanic, 19% were 65 years and older, and 70% were from sites in the United States.

Demographics for individual approvals is provided in the Table 5.

Table 5. Percentage of Subpopulations - Psychiatry and Sleep Disorders Approvals

| BRAND NAME | INDICATION | WOMEN | WHITE | BLACK or AFRICAN AMERICAN | ASIAN | HISPANIC | AGE 65 and OLDER | UNITED STATES |
|-----------------|---|------------|-----------|---------------------------------|-----------|-----------|------------------------|------------------|
| CAPLYTA | Treatment of schizophrenia | 24 | 21 | 75 | 1 | 9 | 0 | 100 |
| DAYVIGO | Treatment of insomnia | 76 | 73 | 15 | 11 | 12 | 35 | 54 |
| SUNOSI | Improvement of wakefulness in patients with narcolepsy or obstructive sleep apnea | 49 | 77 | 18 | 2 | 8 | 12 | 90 |
| WAKIX | Treatment of excessive daytime sleepiness in patients with narcolepsy | 50 | 55 | 2 | NR | NR | 6 | 0 |
| ZULRESSO | Treatment of postpartum depression | 100 | 61 | 36 | 1 | 17 | 0 | 100 |

NR=not reported

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