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# New Drug Update August 2023

EMPAA

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# Statement of Disclosure

- ✓ I have no relevant conflicts of interest to report
- ✓ This presentation will include a discussion of unlabeled or investigational use of therapies that have not yet been approved by the FDA
- ✓ This presentation will focus on therapies approved by the FDA within the last year; this is not an all-inclusive review of new drugs approved in the past year

# Learning Objectives

**At the conclusion of this educational activity, participants will be able to:**

**List** notable therapies approved by the FDA within the last year and their indications for use.

**Describe** important considerations for therapies approved by the FDA within the last year regarding patient selection, dose, and administration.

**Identify** novel therapies with limited therapeutic alternatives.

# Learning Assessment Questions

## True or False?

Roctavian (valoctocogene roxaparvovec) is indicated for Duchenne muscular dystrophy (DMD).

## Which of the following are important considerations for Leqembi (lecanemab) regarding patient selection?

1. Severity of dementia
2. Presence of amyloid beta
3. Qualified prescriber participating in a registry
4. All of the above

## True or False?

Jesduvroq (daprodustat) has a Boxed Warning for thrombotic vascular events, including major adverse cardiovascular events (MACE).

# Our Discussion Today

1. Overview of FDA New Drug Approval Patterns
2. Gene and Cell Therapy Products
3. Oncology
4. Neurology
5. Diabetes/Cardiology/Nephrology
6. Rare Diseases
7. Other Conditions
8. Immunomodulators
9. Biosimilars

**Bethany Holderread, Pharm.D.**

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Bethany assists Mercer's Medicaid clients with the management of pharmacy benefits.

# 1
# Overview of FDA New Drug Approval Patterns

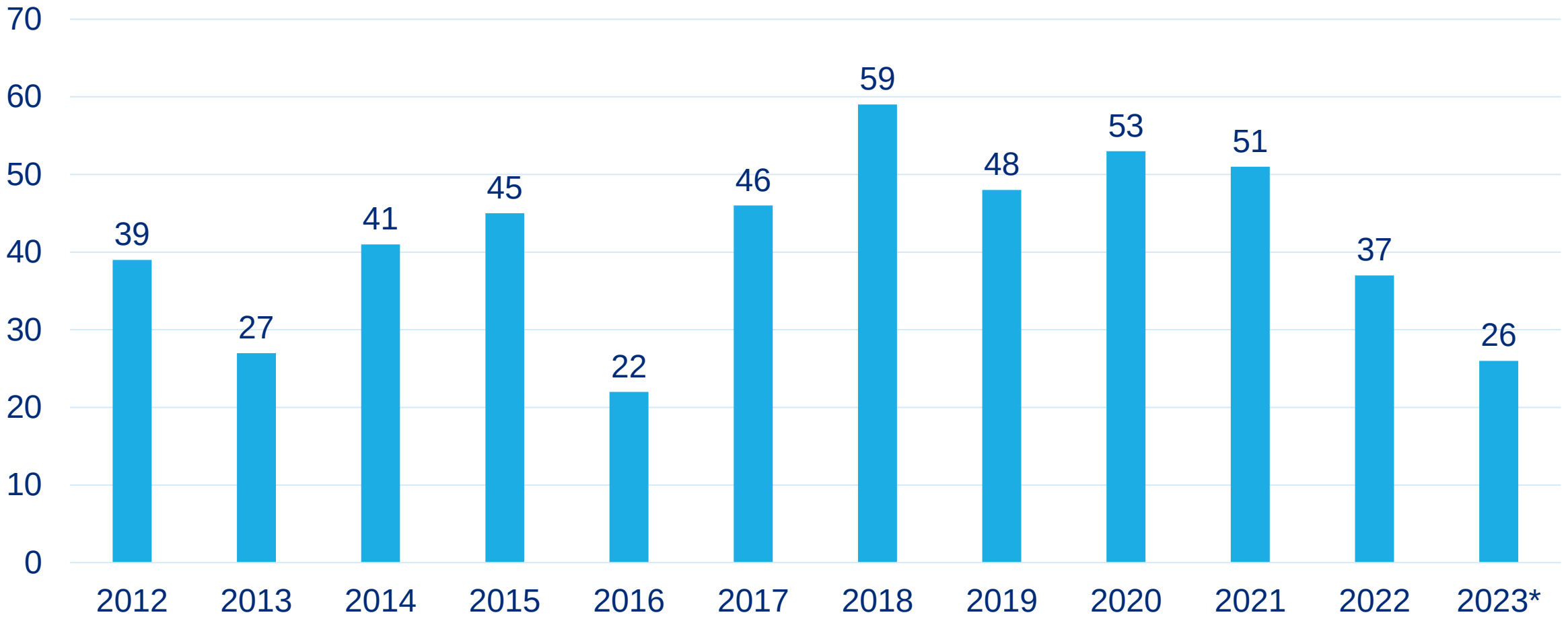
**Random State Fact 1:**

In Alabama, it's illegal to put an ice cream cone in your back pocket.



Livermore. https://www.delish.com/food/g3802/weirdest-food-facts-by-state/.

# FDA Approval Trends: New Molecular Entities




FDA. New Drug Therapy Approvals. https://www.fda.gov/drugs/development-approval-process-drugs/new-drugs-fda-cders-new-molecular-entities-and-new-therapeutic-biological-products. Accessed 07/11/2023.

* As of 07/11/2023

# Summary of 2022 Novel Approvals

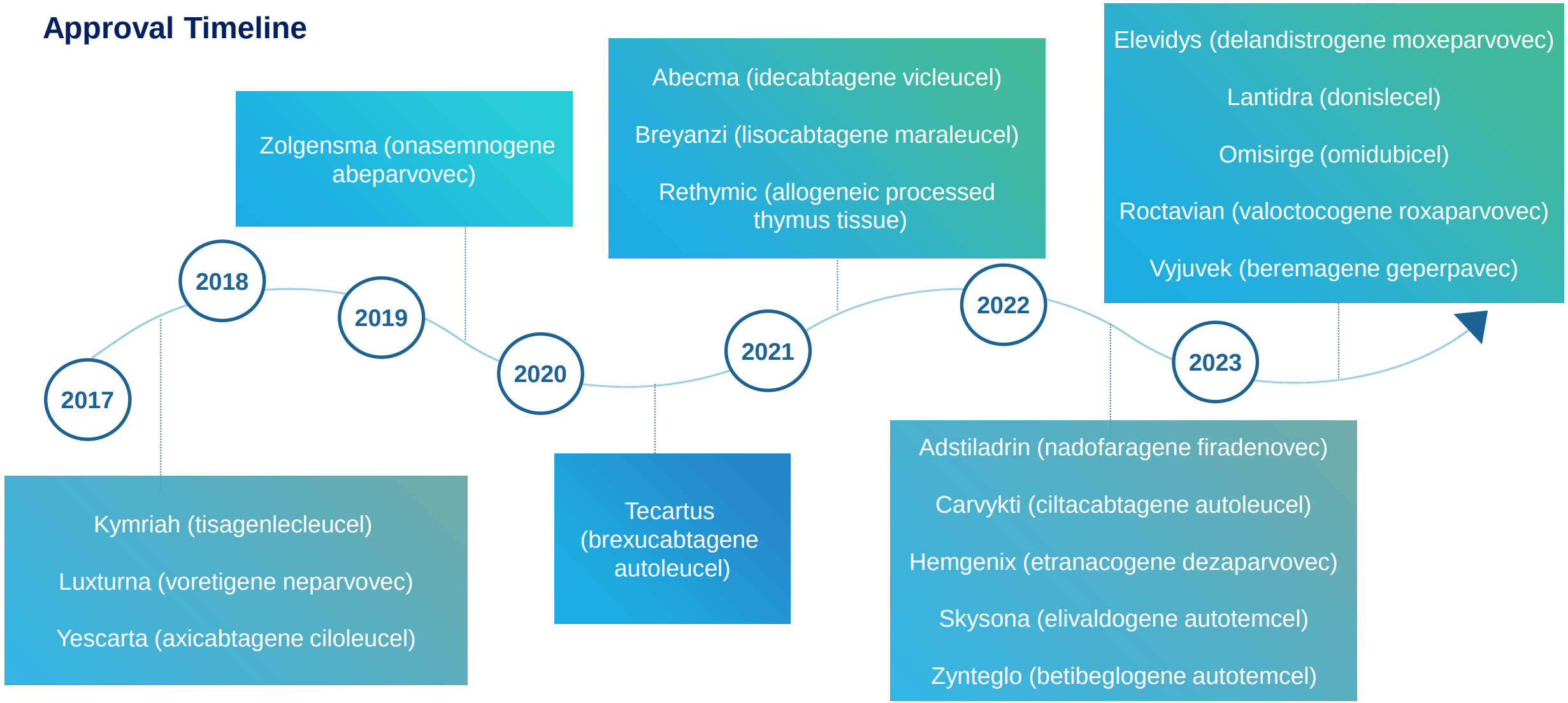
**At Least One Expedited Development or Review Method: 65%**

| | | |
|---|---|---|
| First-in-Class 54% | Orphan 54% | Fast Track 32% |
| Breakthrough Therapy 35% | Priority Review 57% | Accelerated Approval 16% |

FDA. New Drug Therapy Approvals. https://www.fda.gov/drugs/development-approval-process-drugs/new-drugs-fda-cders-new-molecular-entities-and-new-therapeutic-biological-products. Accessed 06/12/2023.

# Approved Gene and Cell Therapy Products

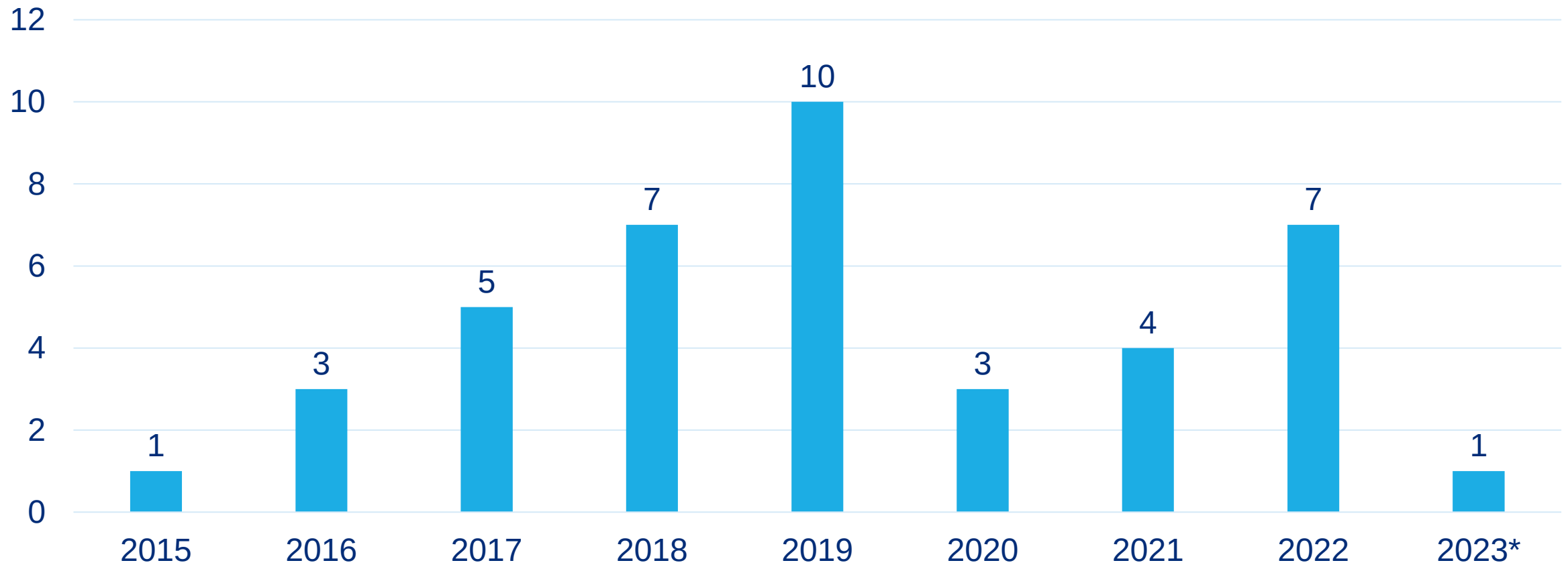
## Approval Timeline




FDA. Approved Cellular and Gene Therapy Products. https://www.fda.gov/vaccines-blood-biologics/cellular-gene-therapy-products/approved-cellular-and-gene-therapy-products Accessed 07/11/2023.
NOTE: Imlygic (talimogene laherparepvec) and Provenge (sipuleucel-T) not included on timeline

# Biosimilar Approvals

## FDA has approved a total of 41 biosimilar products for 11 different reference products since 2015




FDA. FDA-Approved Biosimilar Products. https://www.fda.gov/drugs/biosimilars/biosimilar-product-information. Accessed 07/11/2023.
*As of 07/11/2023.

# 2
# Gene and Cell Therapy Products

**Random State Food Fact 2:**

In Gainesville, Georgia, it's illegal to eat a chicken sandwich with a fork.



Livermore. https://www.delish.com/food/g3802/weirdest-food-facts-by-state/.

# 2nd Half 2022-2023 Gene and Cell Therapy Products

| Drug Name | Approval Date | Indication |
|---|---|---|
| Elevidys (delandistrogene moxeparvovec) | June 2023 | Duchenne muscular dystrophy (DMD) |
| Lantidra (donislecel) | June 2023 | Type 1 diabetes |
| Roctavian (valoctocogene roxaparvovec) | June 2023 | Hemophilia A |
| Vyjuvek (beremagene geperpavec) | May 2023 | Wounds in patients with dystrophic epidermolysis bullosa |
| Omisirge (omidubicel) | April 2023 | Following myeloablative conditioning to reduce the time to neutrophil recovery |
| Adstiladrin (nadofaragene firadenovec) | Dec 2022 | Bacillus Calmette-Guérin unresponsive non-muscle invasive bladder cancer |
| Hemgenix (etranacogene dezaparvovec) | Nov 2022 | Hemophilia B |
| Skysona (elivaldogene autotemcel) | Sept 2022 | Cerebral adrenoleukodystrophy |
| Zynteglo (betibeglogene autotemcel) | Aug 2022 | Transfusion-dependent β-thalassemia |

FDA. Approved Cellular and Gene Therapy Products. https://www.fda.gov/vaccines-blood-biologics/cellular-gene-therapy-products/approved-cellular-and-gene-therapy-products. Accessed 07/11/2023.

# Roctavian (valoctocogene roxaparvovec)

| | |
|---|---|
| **Indication** | Adult males with severe Hemophilia A |
| **Market Landscape** | • Estimated 30,000 to 33,000 people with hemophilia in the U.S. with the majority having hemophilia A<br>• Estimated 2,500 patients eligible to receive Roctavian<br>• Patients currently use high-cost prophylactic factor VIII or Hemlibra (emicizumab) |
| **Clinical Studies** | 112 patients experienced a mean annualized bleed rate (ABR) reduction of 52% through the end of follow-up (median of 3 years) |
| **Dosing** | One-time IV infusion |
| **Important Considerations** | • Baseline antibodies to adeno-associated virus serotype 5 (AAV5) testing<br>• Baseline inhibitor testing<br>• Baseline liver testing and follow-up<br>• Corticosteroids required in majority of patients |
| **Cost** | $2.9 million for the one-time treatment |

FDA. Roctavian. Accessed 07/2023.
IPD Analytics. Roctavian Update.

# Lantidra (donislecel)

| | |
|---|---|
| **Indication** | Type 1 diabetes (T1D) |
| **Market Landscape** | Target population: patients unable to achieve target blood glucose levels because of frequent episodes of severe hypoglycemia despite intervention |
| **Clinical Studies** | • 30 patients with T1D and hypoglycemia unawareness<br>• Patients received 1 to 3 infusions<br>• 4 subjects (13.3%) were insulin independent <1 year, 12 subjects (36.7%) for 1 to 5 years, and 9 subjects (33.3%) for > 5 years |
| **Dosing** | Single infusion into the hepatic portal vein (may require up to 3 infusions) |
| **Important Considerations** | • First allogeneic cellular therapy approved for T1D<br>• Procedure for infusion complications<br>• Immunosuppressives needed to maintain the islet cell viability |
| **Cost** | Not yet available |

FDA. Lantidra. Accessed 07/2023.
IPD Analytics. Lantidra Update.

# Elevidys (delandistrogene moxeparvovec)

| | |
|---|---|
| **Indication** | Duchenne muscular dystrophy (DMD) |
| **Market Landscape** | • Estimated U.S. prevalence ranges between 10,000 and 15,000 males<br>• First gene therapy for ambulatory DMD patients 4-5 years of age<br>• Current treatment consists of exon skipping therapies and glucocorticoids |
| **Clinical Studies** | • 41 patients aged 4 to 7 years with mutation between exons 18 to 58<br>• Results found change in dystrophin levels; ambulatory data conflicting |
| **Dosing** | One-time, single IV dose |
| **Important Considerations** | • Approval in a narrower population, rather than age range of patients studied in clinical trials<br>• Contraindicated in patients with deletion in exon 8 or exon 9 in DMD gene<br>• Baseline testing for the presence of anti-AAVrh74<br>• Liver monitoring, myocarditis, and immune-mediated myositis |
| **Cost** | $3.2 million for one-time treatment |

FDA. Elevidys. Accessed 07/2023.
IPD Analytics. Elevidys Update.

# 3
# Oncology

**Random State Food Fact 3:**

Kansas really is flatter than a pancake: Scientists proved it when they compared the topography of Kansas against that of a pancake from IHOP.



Livermore. https://www.delish.com/food/g3802/weirdest-food-facts-by-state/.

# Novel 2023 Oncology Approvals

| Drug Name | Approval Date | Indication |
|---|---|---|
| Columvi (glofitamab) | June 2023 | Diffuse large B-cell lymphoma (DLBCL) |
| Posluma (flotufolastat F 18) | May 2023 | To use with positron emission tomography imaging in prostate cancer |
| Epkinly (epcoritamab) | May 2023 | Relapsed or refractory DLBCL |
| Zynyz (retifanlimab) | Mar 2023 | Metastatic or recurrent Merkel cell carcinoma |
| Orserdu (elacestrant) | Jan 2023 | Advanced or metastatic breast cancer |
| Jaypirca (pirtobrutinib) | Jan 2023 | Relapsed or refractory mantle cell lymphoma (MCL) |

FDA. Novel Drug Approvals for 2023. https://www.fda.gov/drugs/new-drugs-fda-cders-new-molecular-entities-and-new-therapeutic-biological-products/novel-drug-approvals-2023. Accessed 07/11/2023.

# Novel 2nd Half 2022 Oncology Approvals

| Drug Name | Approval Date | Indication |
|---|---|---|
| Lunsumio (mosunetuzumab) | Dec 2022 | Relapsed or refractory follicular lymphoma |
| Krazati (adagrasib) | Dec 2022 | Non-small cell lung cancer |
| Rezlidhia (olutasidenib) | Dec 2022 | Relapsed or refractory acute myeloid leukemia |
| Elahere (mirvetuximab soravtansine) | Nov 2022 | Recurrent ovarian cancer |
| Tecvayli (teclistamab) | Oct 2022 | Relapsed or refractory multiple myeloma |
| Imjudo (tremelimumab) | Oct 2022 | Unresectable hepatocellular carcinoma |
| Lytgobi (futibatinib) | Sept 2022 | Intrahepatic cholangiocarcinoma |
| Rolvedon (eflapegrastim) | Sept 2022 | Reduce infections associated with febrile neutropenia |

FDA. Novel Drug Approvals for 2022. https://www.fda.gov/drugs/new-drugs-fda-cders-new-molecular-entities-and-new-therapeutic-biological-products/novel-drug-approvals-2022. Accessed 07/11/2023.

# Jaypirca (pirtobrutinib)

| | |
|---|---|
| **Indication** | Relapsed or refractory mantle cell lymphoma (MCL) after at least 2 lines of therapy, including a Bruton's tyrosine kinase inhibitor (BTKi) |
| **Market Landscape** | • MCL is a form of non-Hodgkin lymphoma (NHL), comprising 3 to 10% of NHL cases in the United States (estimated 80,550 new cases of NHL)<br>• Mostly affects males between 60 and 70 years of age<br>• Alternative therapies include Imbruvica (ibrutinib), Calquence (acalabrutinib), Brukinsa (zanubrutinib), and Tecartus (brexucabtagene autoleucel) |
| **Clinical Studies** | • Clinical trial included 120 patients with MCL previously treated with a BTKi<br>• Overall response rate 50%; included a complete response rate of 13%<br>• Median time to response 1.8 months; median duration of response 8.3 months |
| **Dosing** | 200 mg orally once daily |
| **Important Considerations** | • First non-covalent BTKi which could re-establish BTK inhibition in patients previously treated with a covalent BTKi<br>• Taken until disease progression or unacceptable toxicity |
| **Cost** | $255,500 annually |

Jaypirca Prescribing Information
IPD Analytics Jaypirca New Drug Review.

# 4
# Neurology

**Random State Food Fact 4:**

Up until the late 1990s, in Lehigh, Nebraska, it was illegal to sell donut holes.



Livermore. https://www.delish.com/food/g3802/weirdest-food-facts-by-state/.

# Novel 2nd Half 2022-2023 Neurology Approvals

| Drug Name | Approval Date | Indication |
|---|---|---|
| Qalsody (tofersen) | April 2023 | Amyotrophic lateral sclerosis (ALS) with SOD1 gene mutation |
| Daybue (trofinetide) | Mar 2023 | Rett syndrome |
| Zavzpret (zavegepant) | Mar 2023 | Migraine |
| Skyclarys (omaveloxolone) | Feb 2023 | Friedrich's ataxia (FA) |
| Leqembi (lecanemab) | Jan 2023 | Alzheimer's disease (AD) |
| Briumvi (ublituximab) | Dec 2022 | Relapsing forms of multiple sclerosis |
| Relyvrio (sodium phenylbutyrate/taurursodiol) | Sept 2022 | ALS |
| Amvuttra (vutrisiran) | Jun 2022 | Polyneuropathy of hereditary transthyretin-mediated amyloidosis |

FDA. Novel Drug Approvals for 2023. https://www.fda.gov/drugs/new-drugs-fda-cders-new-molecular-entities-and-new-therapeutic-biological-products/novel-drug-approvals-2023. Accessed 07/11/2023.

# Leqembi (lecanemab)

| | |
|---|---|
| **Indication** | Alzheimer's disease (AD) in patients with mild cognitive impairment or mild dementia stage of disease |
| **Market Landscape** | • About 5 million Americans 65 years of age and older have mild cognitive impairment due to AD<br>• Second FDA-approved anti-amyloid monoclonal antibody for AD following Aduhelm (aducanumab) in 2021; initial uptake of therapies limited by CMS national coverage determination (NCD) |
| **Clinical Studies** | Phase 3 CLARITY AD trial: Leqembi slowed the rate of cognitive and functional decline by 27% at 18 months, compared with placebo |
| **Dosing** | 10 mg/kg administered IV every 2 weeks |
| **Important Considerations** | • CMS NCD changed upon traditional approval<br>• Warning regarding amyloid-related imaging abnormalities (ARIA)<br>• Institute for Clinical and Economic Review (ICER)'s cost-effective annual list price range: $8,500 to $20,600 |
| **Cost** | $26,500 per year, based on an average patient weight of 75 kg |

Leqembi Prescribing Information
IPD Analytics Leqembi New Drug Review.

# Skyclarys (omaveloxolone)

| | |
|---|---|
| **Indication** | Friedrich's ataxia (FA) |
| **Market Landscape** | • Estimated 5,000 patients with diagnosed FA in the United States<br>• Only available treatments for FA prior to Skyclarys were supportive therapies that address specific symptoms |
| **Clinical Studies** | • MOXIe Part 2 study: Skyclarys demonstrated –2.41-point difference on modified Friedreich's Ataxia Rating Scale compared to placebo<br>• Results indicate less impairment with Skyclarys treatment potentially resulting in approximately 2 years of disease progression |
| **How Supplied** | 50 mg oral capsules |
| **Dosing** | 150 mg by mouth once daily |
| **Important Considerations** | • First disease-specific therapy for FA<br>• Liver monitoring |
| **Cost** | Wholesale acquisition cost (WAC) of $375,136 annually based on a dose of 150mg daily |

Skyclarys Prescribing Information
IPD Analytics Skyclarys New Drug Review.

# 5
# Diabetes/ Cardiology/ Nephrology

**Random State Food Fact 5:**

There is more shrimp consumed in Las Vegas every day (60,000 lbs!) than the rest of the country combined.



Livermore. https://www.delish.com/food/g3802/weirdest-food-facts-by-state/.

# Novel 2nd Half 2022-2023 Diabetes/ Cardiology/ Nephrology Approvals

| Drug Name | Approval Date | Indication |
|---|---|---|
| Inpefa (sotagliflozin) | May 2023 | Heart failure |
| Filspari (sparsentan) | Feb 2023 | Proteinuria in adults with primary immunoglobulin A nephropathy |
| Jesduvroq (daprodustat) | Feb 2023 | Anemia caused by chronic kidney disease (CKD) in adults on dialysis |
| Brenzavvy (bexagliflozin) | Jan 2023 | Type 2 diabetes |
| Tzield (teplizumab) | Nov 2022 | Delay the onset of stage 3 type 1 diabetes |

FDA. Novel Drug Approvals for 2023. https://www.fda.gov/drugs/new-drugs-fda-cders-new-molecular-entities-and-new-therapeutic-biological-products/novel-drug-approvals-2023. Accessed 07/11/2023.

# Jesduvroq (daprodustat)

| | |
|---|---|
| **Indication** | Anemia caused by chronic kidney disease (CKD) in adults on dialysis |
| **Market Landscape** | • More than 500,000 patients in the United States receiving dialysis<br>• Will likely compete with erythropoiesis-stimulating agents (ESAs)<br>• Two other oral hypoxia-inducible factor prolyl hydroxylase (HIF-PH) inhibitors were expected prior to Jesduvroq but received complete response letters (CRLs) |
| **Clinical Studies** | • Jesduvroq noninferior to ESAs in both dialysis-dependent (DD) and non–dialysis-dependent (NDD) patients<br>• Jesduvroq only approved for the DD population, due to safety concerns |
| **How Supplied** | 1 mg, 2 mg, 4 mg, 6 mg, and 8 mg oral tablets |
| **Dosing** | Orally once daily |
| **Important Considerations** | • Boxed Warning for thrombotic vascular events, including major adverse cardiovascular events (MACE)<br>• First oral treatment for anemia caused by CKD<br>• CMS' Transitional Drug Add-on Payment Adjustment (TDAPA) implications |
| **Cost** | Not yet available |

Jesduvroq Prescribing Information.
IPD Analytics Jesduvroq New Drug Review.

# 6
# Rare Diseases

**Random State Food Fact 6:**

When Teddy Roosevelt visited Tennessee in 1907, he was served Maxwell House coffee and came up with the phrase, "good 'til the last drop."



Livermore. https://www.delish.com/food/g3802/weirdest-food-facts-by-state/.

# Novel 2nd Half 2022-2023 Rare Disease Approvals

| Drug Name | Approval Date | Indication |
|---|---|---|
| Ngenla (somatrogon) | June 2023 | Growth failure due to inadequate secretion of endogenous growth hormone |
| Elfabrio (pegunigalsidase alfa) | May 2023 | Fabry disease (FD) |
| Joenja (leniolisib) | Mar 2023 | Activated phosphoinositide 3-kinase delta syndrome |
| Lamzede (velmanase alfa) | Feb 2023 | Non-central nervous system manifestations of alpha-mannosidosis |
| Xenpozyme (olipudase alfa) | Aug 2022 | Acid Sphingomyelinase Deficiency |

FDA. Novel Drug Approvals for 2023. https://www.fda.gov/drugs/new-drugs-fda-cders-new-molecular-entities-and-new-therapeutic-biological-products/novel-drug-approvals-2023. Accessed 07/11/2023.

# Elfabrio (pegunigalsidase alfa)

| | |
|---|---|
| **Indication** | Adults with confirmed Fabry disease (FD) |
| **Market Landscape** | • Type 1 affects approximately 1 in 40,000 to 60,000 males; Type 2, the most common phenotype by 3- to 10-fold, may affect up to 1 in 1,500 to 4,000 males<br>• Current treatments are IV enzyme replacement therapy (ERT), Fabrazyme (agalsidase beta) and Galafold (migalastat), an oral chemical chaperone |
| **Clinical Studies** | • Met noninferiority margin with agalsidase beta in a head-to-head study in treatment-experienced adults with FD<br>• Median eGFR slope with Elfabrio was -2.514 mL/min/1.73 $m^2$/year vs. -2.155 mL/min/1.73 $m^2$/year with Fabrazyme |
| **How Supplied** | Single-dose vial |
| **Dosing** | 1 mg/kg given via IV infusion every 2 weeks |
| **Important Considerations** | • Elfabrio is a PEGylated ERT and considered a “biobetter” of Fabrazyme<br>• Single-dose gene therapy for FD being studied |
| **Cost** | Approximately $430,000 annually for an adult patient |

Elfabrio Prescribing Information.
IPD Analytics Elfabrio New Drug Review.

# 7
# Other Conditions

**Random State Food Fact 7:**

Vermont loves syrup so much that when McDonald's debuted their Fruit and Maple Oatmeal (not made with real maple syrup), Vermont's Agency of Agriculture took official action to have McDonald's change the item for all 28 Vermont locations to add a tablespoon of pure Vermont maple syrup.



Livermore. https://www.delish.com/food/g3802/weirdest-food-facts-by-state/.

# Novel 2023 Other Approvals

| Drug Name | Approval Date | Indication |
|---|---|---|
| Litfulo (ritlecitinib) | June 2023 | Severely patchy hair loss |
| Paxlovid (nirmatrelvir, ritonavir) | May 2023 | COVID-19 |
| Xacduro (sulbactam, durlobactam) | May 2023 | Hospital-acquired bacterial pneumonia and ventilator-associated bacterial pneumonia caused by Acinetobacter baumannii-calcoaceticus complex |
| Miebo (perfluorhexyloctane) | May 2023 | Signs and symptoms of dry eye disease (DED) |
| Veozah (fezolinetant) | May 2023 | Hot flashes caused by menopause |
| Rezzayo (rezafungin) | Mar 2023 | Candidemia and invasive candidiasis |

FDA. Novel Drug Approvals for 2023. https://www.fda.gov/drugs/new-drugs-fda-cders-new-molecular-entities-and-new-therapeutic-biological-products/novel-drug-approvals-2023. Accessed 07/11/2023.

# Novel 2nd Half 2022 Other Approvals

| Drug Name | Approval Date | Indication |
|---|---|---|
| NexoBrid (anacaulase) | Dec 2022 | Eschar in adults with thermal burns |
| Xenoview (hyperpolarized Xe-129) | Dec 2022 | To evaluate pulmonary function and imaging |
| Sunlenca (lenacapavir) | Dec 2022 | HIV infections that cannot be treated with other available treatments |
| Rebyota (fecal microbiota, live) | Nov 2022 | Recurrent Clostridioides difficile infection (CDI) |
| Omlonti (oomidenepag isopropyl) | Sept 2022 | Open-angle glaucoma or ocular hypertension |
| Terlivaz (terlipressin) | Sept 2022 | Hepatorenal syndrome |

FDA. Novel Drug Approvals for 2022. https://www.fda.gov/drugs/new-drugs-fda-cders-new-molecular-entities-and-new-therapeutic-biological-products/novel-drug-approvals-2022. Accessed 06/19/2023.

# Rebyota (fecal microbiota, live)

| | |
|---|---|
| **Indication** | Recurrent CDI |
| **Market Landscape** | • 500,000 patients with CDI in the U.S. each year; 15% to 30% experience reoccurrence with 40% of those experiencing a 2nd recurrence<br>• Guidelines suggest Zinplava (bezlotoxumab) for 1st recurrence of CDI<br>• Fecal microbiota transplantation (FMT) is recommended in patients experiencing 2 or more recurrences of CDI |
| **Clinical Studies** | • 262 adults with ≥ 1 CDI recurrences<br>• Absence of CDI diarrhea 8 weeks after treatment: 70.6% with Rebyota vs. 57.5% with placebo<br>• >90% remained free of CDI through 6 months; sustained clinical response at 6 months not statistically significant between Rebyota (65.5%) and placebo (56.5%) |
| **How Supplied** | 150 mL single-dose rectal suspension |
| **Dosing** | Single-rectal administration 24 to 72 hours after the last dose of antibiotics |
| **Important Considerations** | • 1st FDA approved microbiome therapy<br>• Manufactured from human fecal matter |
| **Cost** | $9,000 per dose |

Rebyota Prescribing Information.
IPD Analytics. Rebyota Payer Update.

# Miebo (perfluorhexyloctane)

| | |
|---|---|
| **Indication** | Signs and symptoms of dry eye disease (DED) |
| **Market Landscape** | • DED diagnosed in about 16.4 million adults in the United States<br>• Will compete with other topical DED products including Restasis and generics, Xiidra (lifitegrast), and Tyrvaya (varenicline solution) nasal spray |
| **Clinical Studies** | • Two Phase 3 clinical trials (GOBI and MOJAVE), included a total of 1,217 patients with DED: Miebo consistently met primary endpoints of total corneal fluorescein staining and eye dryness score |
| **How Supplied** | Multiple-dose ophthalmic solution |
| **Dosing** | One drop four times daily |
| **Important Considerations** | Unique mechanism of action that addresses tear evaporation |
| **Cost** | $771 per 3 ML bottle |

Miebo Prescribing Information.
IPD Analytics. Miebo New Drug Review.

# Veozah (fezolinetant)

| | |
|---|---|
| **Indication** | Moderate-to-severe vasomotor symptoms (VMS), or hot flashes, caused by menopause |
| **Market Landscape** | • 64% of females 40 to 65 years of age in the United States were menopausal with VMS occurring in approximately 75% to 80% of menopausal women<br>• Menopausal hormone therapy (MHT) is the gold standard for VMS; paroxetine mesylate 7.5 mg is a nonhormonal option |
| **Clinical Studies** | • 12-week SKYLIGHT 1 and 2 trials found a statistically significant reduction from baseline in frequency and severity of VMS at Weeks 4 and 12<br>• Veozah demonstrated long-term endometrial health in a 52-week trial |
| **How Supplied** | 45 mg oral tablet |
| **Dosing** | 45 mg tablet orally once daily |
| **Important Considerations** | • First in class treatment<br>• Liver monitoring<br>• ICER recommended range of $2,000 to $2,500 per year for non-MHTs |
| **Cost** | WAC is $6,692 per year |

Veozah Prescribing Information.
IPD Analytics. Veozah New Drug Review.

# 8 Immunomodulators

**Random State Food Fact 8:**

Wisconsin is the only state that offers a Master Cheesemaker program, which takes three years to complete. Oh, and you need 10 years of cheese making experience before you can even apply.



Livermore. https://www.delish.com/food/g3802/weirdest-food-facts-by-state/.

# Novel 2nd Half 2022 Immunomodulator Approvals

| Drug Name | Approval Date | Indication |
|---|---|---|
| Rystiggo (rozanolixizumab) | June 2023 | Generalized myasthenia gravis |
| Sotyktu (deucravacitinib) | Sept 2022 | Moderate-to-severe plaque psoriasis |
| Spevigo (spesolimab) | Sept 2022 | Generalized pustular psoriasis flares |

FDA. Novel Drug Approvals for 2023. https://www.fda.gov/drugs/new-drugs-fda-cders-new-molecular-entities-and-new-therapeutic-biological-products/novel-drug-approvals-2023. Accessed 07/11/2023.

# Rystiggo (rozanolixizumab)

| | |
|---|---|
| **Indication** | Generalized myasthenia gravis (gMG) in adults who are positive for anti-acetylcholine receptor (AChR) or anti-muscle-specific tyrosine kinase (MuSK) antibodies |
| **Market Landscape** | • Prevalence of gMG is 100–350 patients per 1 million individuals.<br>• AChR-positive ~85% of patients with MG & MuSK-positive ~ approximately 6%<br>• Current treatments include Vyvgart Hytrulo (efgartigimod alfa/hyaluronidase), Soliris (eculizumab), & Ultomiris (ravulizumab) |
| **Clinical Studies** | Rystiggo change of −3.4 points in the primary endpoint (Myasthenia Gravis Activities of Daily Living [MG-ADL] total score) versus −0.78 points in the placebo group over 6-week study |
| **How Supplied** | 280 mg/2 mL (140 mg/mL) in a single-dose vial |
| **Dosing** | Subcutaneous infusion once weekly for 6 weeks |
| **Important Considerations** | • Only approved treatment for both of the two most common subtypes of gMG<br>• Must be administered by an infusion pump at a rate of 20 mL/hr |
| **Cost** | WAC is $290,400 per year |

Rystiggo Prescribing Information.
IPD Analytics. Rystiggo New Drug Review.

# 9
# Biosimilars

**Random State Food Fact 9:**

South Carolina is actually the birthplace of BBQ.



Livermore. https://www.delish.com/food/g3802/weirdest-food-facts-by-state/.

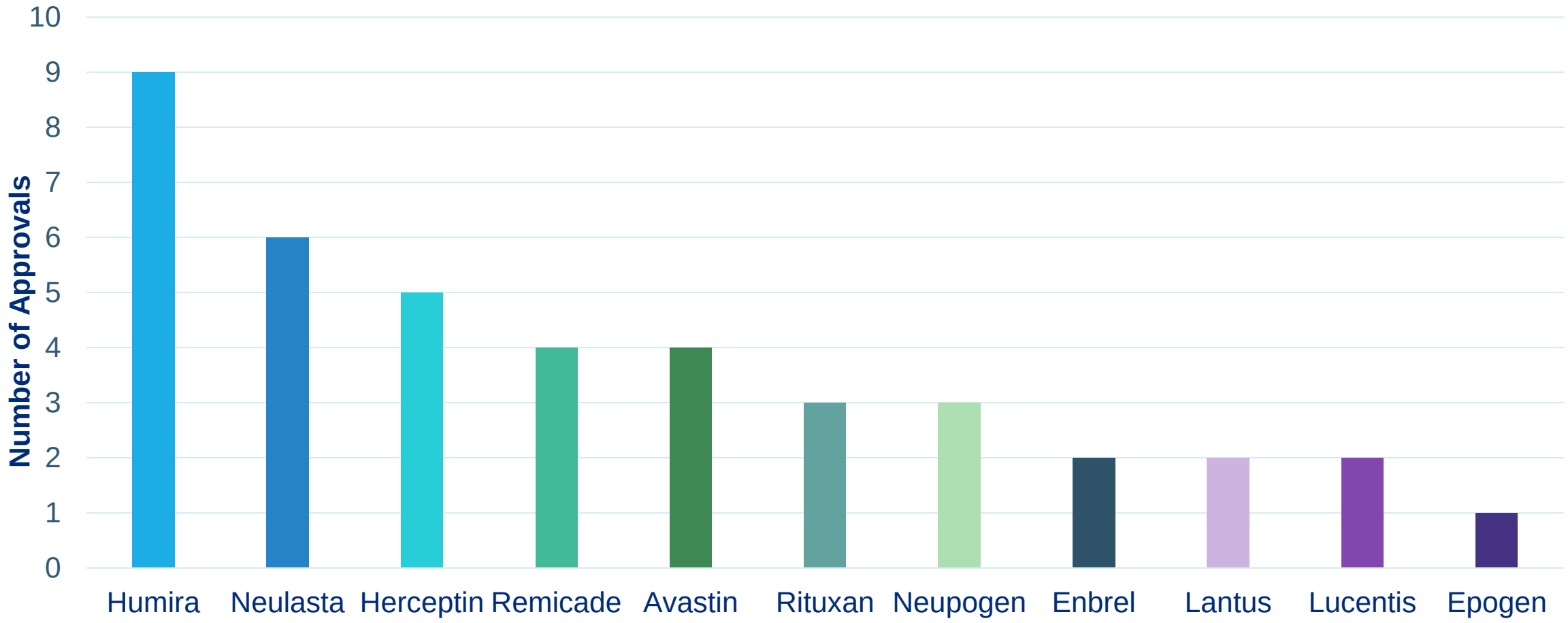
# Biosimilar Approvals in 2nd Half 2022-2023

| Drug Name | Approval Date | Reference Product |
|---|---|---|
| Yuflyma (adalimumab-aaty) | May 2023 | Humira |
| Idacio (adalimumab-aacf) | Dec 2022 | Humira |
| Releuko (filgrastim-ayow) | Feb 2022 | Neupogen |
| Fylnetra (pegfilgrastim-pbbk) | May 2022 | Neulasta |
| Stimufend (pegfilgrastim-fpgk) | Sept 2022 | Neulasta |
| Vegzelma (bevacizumab-adcd) | Sept 2022 | Avastin |
| Alymsys (bevacizumab-maly) | April 2022 | Avastin |
| Cimerli (ranibizumab-eqrn) | Aug 2022 | Lucentis |

FDA. FDA-Approved Biosimilar Products. https://www.fda.gov/drugs/biosimilars/biosimilar-product-information. Accessed 07/11/2023.

# Biosimilar Approvals

## FDA has approved a total of 41 biosimilar products for 11 different reference products since 2015




FDA. New Drug Therapy. https://www.fda.gov/drugs/development-approval-process-drugs/new-drugs-fda-cders-new-molecular-entities-and-new-therapeutic-biological-products. Approvals. Accessed 07/11/2023.
FDA. FDA-Approved Biosimilar Products. https://www.fda.gov/drugs/biosimilars/biosimilar-product-information. Accessed 07/11/2023.

# Conclusions

**26**

**Novel FDA drug approvals in 2023 thus far**

**5**

**Gene and cell therapy approvals in 2023 thus far**

## Oncology

**continues to grow at a rapid pace with the most novel approvals in 2023 thus far**

## Cell and Gene therapy

**approvals are accelerating**

## Specialty therapy

**approvals continue to increase, including those for neurology and rare diseases**

# Learning Assessment Questions

## True or False?

Roctavian (valoctocogene roxaparvovec) is indicated for Duchenne muscular dystrophy (DMD).

## Which of the following are important considerations for Leqembi (lecanemab) regarding patient selection?

1. Severity of dementia
2. Presence of amyloid beta
3. Qualified prescriber participating in a registry
4. All of the above

## True or False?

Jesduvroq (daprodustat) has a Boxed Warning for thrombotic vascular events, including major adverse cardiovascular events (MACE).

# Questions

**Bethany Holderread, Pharm.D.**

**Acknowledgements:**
Payal Kotadiya, April Lindquist

# Abbreviations

| | |
|---|---|
| **AChR** | Anti-acetylcholine receptor |
| **AD** | Alzheimer's disease |
| **ALS** | Amyotrophic lateral sclerosis |
| **ARIA** | Amyloid-related imaging abnormalities |
| **BTKi** | Bruton's tyrosine kinase inhibitor |
| **CAR-T** | Chimeric antigen receptor T-cell |
| **CDI** | Clostridioides difficile infection |
| **CKD** | Chronic kidney disease |
| **CMS** | Centers for Medicare & Medicaid Services |
| **COVD-19** | Coronavirus disease |
| **CRL** | Complete response letter |
| **DD** | Dialysis-dependent |
| **DED** | Dry eye disease |
| **DMD** | Duchenne muscular dystrophy |
| **DLBCL** | Diffuse large B-cell lymphoma |
| **eGFR** | Estimated glomerular filtration rate |
| **ERT** | Enzyme replacement therapy |
| **ESA** | Erythropoiesis-stimulating agents |
| **FA** | Friedrich's ataxia |
| **FD** | Fabry disease |
| **FDA** | United States Food and Drug Administration |
| **FMT** | Fecal microbiota transplantation |
| **gMG** | Generalized myasthenia gravis |
| **HIF-PH** | **H**ypoxia-inducible factor prolyl hydroxylase |
| **ICER** | Institute for Clinical and Economic Review |
| **IV** | Intravenous |
| **M** | Meter |
| **MACE** | Major adverse cardiovascular events |
| **MCL** | Mantle cell lymphoma |
| **MG-ADL** | Myasthenia gravis activities of daily living |
| **MHT** | Menopausal hormone therapy |
| **Min** | Minute |
| **mL** | Milliliter |
| **MuSK** | Muscle-specific tyrosine kinase |
| **NCD** | National coverage decision |
| **NDD** | Non-dialysis dependent |
| **NHL** | non-Hodgkin lymphoma |
| **TDAPA** | Transitional Drug Add-on Payment Adjustment |
| **VMS** | Vasomotor symptoms |
| **WAC** | Wholesale acquisition cost |

Mercer

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