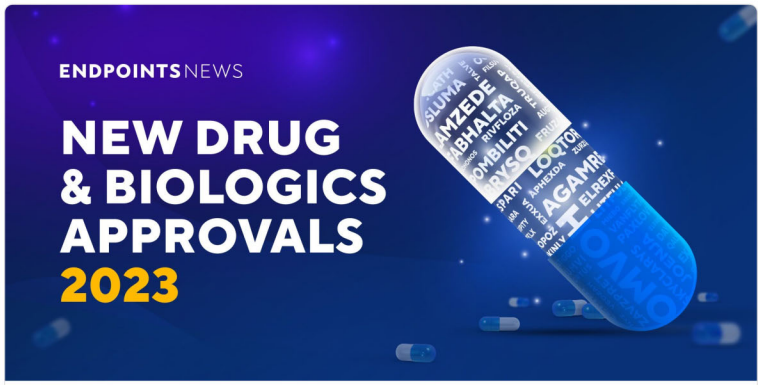
ENDPOINTS NEWS



January 3, 2024 03:30 PM EST
FDA+

All the new drugs approved in 2023: Alzheimer's and gene therapy cap a five-year high

ENDPOINTS

After kicking off 2023 with a potential megablockbuster in Eisai and Biogen's new Alzheimer's drug Leqembi, in totality the FDA signed off on 10 more novel drug approvals in 2023 when compared to last year.

The 57 novel drugs approved in 2023 (not counting gene therapies or vaccines) are the most since 2018 — the year of Gilead's blockbuster HIV drug Biktarvy, which generated $10.4 billion in 2022 sales.

But peak 2023, as in 2022, came with approvals of new cell and gene therapies. In the case of 2023, the two approvals on Dec. 8 were for new sickle cell gene therapies, including Vertex and CRISPR Therapeutics' Casgevy, the first CRISPR-based treatment in the US. Worth watching in 2024: how bluebird's decision to price Lyfgenia nearly $1 million more than Casgevy will play out.

test the bounds of the commercial markets in the US and Europe, where these potential cures have so far yet to muster wider coverage or access. With the backing of CBER's chief Peter Marks, however, be prepared to see more of the same with respect to cell and gene therapy approvals in the coming years.

What to watch for in 2024: CMS will disclose its negotiated prices on Sept. 1 for the first 10 Part D drugs (not kicking in until 2026), and all eyes will be on CMS' disclosure of net prices, with the investment bank Leerink Partners noting that biopharma companies may push back as net prices are not commonly disclosed publicly. — *Zachary Brennan*

Leqembi ↓	Elfabrio ↓
Brenzavvy ↓	Veozah ↓
Orserdu ↓	Miebo ↓
Jaypirca ↓	Epkinly ↓
Jesduvroq ↓	Vyjuvek ↓
Lamzede ↓	Xacduro ↓
Filspari ↓	Posluma ↓
Altuviiio ↓	Paxlovid ↓
Skyclarys ↓	Inpefa ↓
Zavzpret ↓	Abrysvo ↓
Daybue ↓	Columvi ↓
Rezzayo ↓	Elevidys ↓
Zynyz ↓	Litfulo ↓
Joenja ↓	Rystiggo ↓
Qalsody ↓	Ngenla ↓

Beyfortus ↓	Bimzelx ↓
Cyfendus ↓	Zilbrysq ↓
Vanflyta ↓	Omvoh ↓
Balfaxar ↓	Agamree ↓
Xdemvy ↓	Loqtorzi ↓
Izervay ↓	Fruzaqla ↓
Zurzuvae ↓	Defencath ↓
Talvey ↓	Augtyro ↓
Elrexfio ↓	Truqap ↓
Sohonos ↓	Ryzneuta ↓
Veopoz ↓	Ogsiveo ↓
Aphexda ↓	Fabhalta ↓
Ojjaara ↓	Casgevy ↓
Exxua ↓	Lyfgenia ↓
Pombiliti ↓	Filsuvez ↓
Rivfloza ↓	Wainua ↓
Velsipity ↓	

$7 billion annually, according to Eisai

APPROVAL DATE:
01/06/2023

1. Leqembi

Active ingredient: lecanemab-irmb

Indication: To treat Alzheimer's disease

Snapshot: Adherents of the amyloid hypothesis were vindicated in July when Leqembi became the first drug in 20 years to win a full-throated FDA approval for Alzheimer's disease. By targeting amyloid proteins in the brains of people in the early stages of the disease, an 18-month course of the antibody infusion, given every two weeks, slowed cognitive decline by 27%. A weekly injectable form could come in 2024, along with a competing antibody from Eli Lilly. With their modest effects and the possible risks of brain bleeding and swelling, it's still unclear how many patients will get the drugs, but Eisai hopes global sales of Leqembi could reach $7 billion by 2030. — *Ryan Cross*

COMPANY:
TheracosBio

PRICE:
$47.85 plus shipping for a 30-day supply through Mark Cuban's Cost Plus Drugs

PEAK SALES ESTIMATE:
N/A

APPROVAL DATE:
01/23/2023

Snapshot: SGLT2 inhibitor Brenzavvy was approved in January, but by July, it had already joined Mark Cuban's Cost Plus Drugs plan for exclusive distribution. TheracosBio, founded in 2000 with a mission to bring low-cost drugs to market, reached out to Cuban's company shortly after it formed in 2022 with the idea to potentially partner and compete against drugs like Invokana, Farxiga and Jardiance. Its approval was based on Phase III studies that showed reduced A1C and fasting blood sugar after 24 weeks of use. Additional benefits shown in the trials, although not approved indications, included cardiovascular risk reductions, weight loss and blood pressure reduction. — *Beth Snyder Bulik*

COMPANY:
Menarini Group

PRICE:
$21,000 per month

PEAK SALES ESTIMATE:
N/A

APPROVAL DATE:
01/27/2023

3. Orserdu

Active ingredient: elacestrant

Indication: To treat estrogen receptor-positive, human epidermal growth factor receptor 2-negative, ESR1-mutated, advanced or metastatic breast cancer with disease progression following at least one line of endocrine therapy

Snapshot: The Menarini Group and Radius Health got their oral SERD drug Orserdu, or elacestrant, approved in January. It's a drug class that has flummoxed some of the biggest

COMPANY:
Eli Lilly

PRICE:
$21,000 per 30 days of therapy at the 200 mg dose

PEAK SALES ESTIMATE:
$1.59 billion – Bloomberg analyst consensus

APPROVAL DATE:
01/27/2023

4. Jaypirca

Active ingredient: pirtobrutinib

Indication: To treat relapsed or refractory mantle cell lymphoma in adults who have had at least two lines of systemic therapy, including a BTK inhibitor + chronic lymphocytic leukemia or small lymphocytic lymphoma (CLL/SLL) who have received at least two prior lines of therapy, including a Bruton's tyrosine kinase (BTK) inhibitor and a BCL-2 inhibitor

Snapshot: Eli Lilly received two accelerated approvals for its BTK inhibitor Jaypirca to bookend the year. In January, the FDA gave the green light to Jaypirca for mantle cell lymphoma, marking Lilly's entrance into the BTK field. The approval was for patients whose cancers had returned or stopped responding to treatment, and had at least two prior treatment regimens, including a previous BTK inhibitor. In December, the pill was approved with similar terms for chronic lymphocytic leukemia and small lymphocytic lymphoma. Unlike other approved BTK inhibitors — such as Imbruvica, Calquence and Brukinsa — Lilly's drug is the first on

COMPANY:
GSK

PRICE:
Approximately $700 per 30 days of treatment with 6mg tablets for the median patient cohort

PEAK SALES ESTIMATE:
$269.4 million – Bloomberg analyst consensus

APPROVAL DATE:
02/01/2023

5. Jesduvroq

Active ingredient: daprodustat

Indication: To treat anemia caused by chronic kidney disease for adults on dialysis for at least four months

Snapshot: GSK managed to get Jesduvroq across the finish line after a bumpy development process, but not without the FDA slapping on a safety warning for an increased risk of death, heart attack, stroke and blood clots. Use of the drug was also limited to patients who have been on dialysis for at least four months. Jesduvroq, also known as daprodustat, won approval after the FDA rejected a similar drug from AstraZeneca and FibroGen called roxadustat, citing the need to run another pivotal trial. Getting the drug to patients elsewhere has been a challenge, with GSK announcing plans in July to pull its applications from European and other markets. — *Max Gelman*

COMPANY:
Chiesi

PRICE:
$4,000 per vial

PEAK SALES ESTIMATE:
N/A

APPROVAL DATE:
02/16/2023

6. Lamzede

Active ingredient: velmanase alfa-tycv

Indication: To treat non-central nervous system manifestations of alpha-mannosidosis

Snapshot: Chiesi's velmanase alfa-tycv is an enzyme supplement designed to treat non-central nervous system manifestations of an ultra-rare progressive disease known as alpha-mannosidosis. It occurs when the enzyme α-mannosidase is deficient, resulting in recurrent chest and ear infections, hearing loss, distinctive facial features, muscle weakness and abnormalities in cognition, vision, the skeleton and joints. — *Jared Whitlock*

COMPANY:
Travere Therapeutics

PRICE:
$9,900 for a 30-day supply; $120,000 per year

PEAK SALES ESTIMATE:
N/A

Indication: To reduce proteinuria in adults with primary immunoglobulin A nephropathy at risk of rapid disease progression

Snapshot: The FDA's accelerated approval of Travere Therapeutics' treatment for a rare kidney disease called IgAN was a win for the company in what's becoming an increasingly crowded field. Travere, which has its roots as one of Martin Shkreli's old companies, suffered a blow later in the year when a confirmatory trial failed, leading to layoffs and casting doubts on the drug's future. The company says it will press ahead with a try for full approval, but between the lackluster data and the REMS requirement for the drug, it may be a challenge. — *Jared Whitlock*

COMPANY:
Sanofi

PRICE:
N/A

PEAK SALES ESTIMATE:
€2.3 billion – Barclays

APPROVAL DATE:
02/23/2023

8. Altuviiio

Active ingredient: Recombinant Fc-VWF-XTEN fusion protein

Indication: To treat adults and children with hemophilia A (congenital factor VIII deficiency)

Snapshot: Altuviiio added to Sanofi's portfolio of approved

ing disorder this year as the company plans to request approval for the Alnylam-allied fitusiran. — *Kyle LaHucik*

COMPANY:
Reata Pharmaceuticals

PRICE:
$370,000 per year

PEAK SALES ESTIMATE:
N/A

APPROVAL DATE:
02/28/2023

9. Skyclarys

Active ingredient: omaveloxolone

Indication: To treat Friedreich's ataxia

Snapshot: After raising serious efficacy questions that led to a three-month extension of the FDA's review, the agency in late February ultimately approved Reata Pharmaceuticals' Skyclarys (omaveloxolone) for a rare genetic disease known as Friedreich's ataxia, which affects the body's nerves, for those 16 years and older. The rare disease affects about 6,000 Americans and 22,000 individuals globally. Reata didn't stay solo for long: Biogen snapped up the biotech in July for $7.3 billion, following a bidding war with another buyer. — *Zachary Brennan*

COMPANY:
Pfizer

PRICE:
$1,100 per 6 single-dose nasal sprays

PEAK SALES ESTIMATE:
$1.13 billion – Bloomberg analyst consensus

APPROVAL DATE:
03/09/2023

10. Zavzpret

Active ingredient: zavegepant

Indication: To treat acute migraine with or without aura in adults

Snapshot: In March, Zavzpret became the third drug in Pfizer's migraine portfolio with an acute indication and the first-and-only CGRP intranasal spray delivery method approved. Zavzpret came to Pfizer in 2022 through the $11.6 billion purchase of Biohaven. The drug's approval, based on two studies, met co-primary endpoints of pain freedom and freedom from the most bothersome symptoms of migraine two hours after dosage. The intranasal drug adds an alternative for patients with migraine who can't take pills due to nausea or vomiting. — *Beth Bulik*

COMPANY:
Acadia Pharmaceuticals

PRICE:
$585,000 on average annually

Active ingredient: trofinetide

Indication: To treat Rett syndrome

Snapshot: Daybue is the first drug for a rare neurodevelopmental disorder called Rett syndrome which is often mistaken for autism or cerebral palsy and primarily affects girls and women. Acadia Pharmaceuticals licensed the synthetic peptide from Neuren Pharmaceuticals in 2018. The drug's mechanism is unclear, but in 2021, a clinical trial showed that it significantly improved caregiver and physician assessments of a patient's symptoms. Acadia believes Rett syndrome affects up to 9,000 patients in the US, with half that number currently diagnosed. — *Ryan Cross*

COMPANY:
Cidara Therapeutics

PRICE:
$1,950 per vial (200mg)

PEAK SALES ESTIMATE:
N/A

APPROVAL DATE:
03/22/2023

12. Rezzayo

Active ingredient: rezafungin

Indication: To treat candidemia and invasive candidiasis

Snapshot: It's been more than a decade since there was a new treatment for the fungal infections candidemia and inva-

ung

COMPANY:
Incyte

PRICE:
$14,240 per vial

PEAK SALES ESTIMATE:
$361.6 million – Bloomberg analyst consensus

APPROVAL DATE:
03/22/2023

13. Zynyz

Active ingredient: retifanlimab-dlwr

Indication: To treat metastatic or recurrent locally advanced Merkel cell carcinoma

Snapshot: After the FDA rejected it for a type of anal cancer, Incyte's PD-1 inhibitor Zynyz won an accelerated approval from the agency in March to treat metastatic or recurrent locally advanced Merkel cell carcinoma, a rare and fast-growing skin cancer. The approval was based on positive results from the company's single-arm POD1UM-201 study, which is ongoing and will serve as the confirmatory trial. Zynyz is the eighth PD-1/PD-L1 inhibitor to win FDA approval. — *Lia DeGroot*

COMPANY:
Pharming Group

PRICE:
$45,000 for a 30-day supply

PEAK SALES ESTIMATE:
N/A

APPROVAL DATE:
03/24/2023

14. Joenja

Active ingredient: leniolisib

Indication: To treat activated phosphoinositide 3-kinase delta syndrome

Snapshot: Joenja scored approval from the FDA in March as the first and only approved treatment for activated phosphoinositide 3-kinase delta (PI3Kδ) syndrome, which triggers immunodeficiency in patients. The drug belongs to Pharming Group and marks the company's second commercial product. Joenja is still under consideration from the EMA, which is predicted to make a decision in the first quarter of 2024. — *Anna Brown*

COMPANY:
Biogen

PRICE:
$14,230 per dose

Active ingredient: tofersen

Indication: To treat amyotrophic lateral sclerosis in adults who have a SOD1 gene mutation

Snapshot: The FDA's approval of Qalsody was only for a tiny fraction of amyotrophic lateral sclerosis (ALS) patients with a SOD1 mutation — about 300 people in the US — but the decision may influence future treatment options for many more people with ALS and other neurodegenerative diseases. After the drug failed to hit its primary endpoint in a Phase III study, the FDA granted accelerated approval to Qalsody based on longer-term follow-up results, as well as the drug's ability to reduce neurofilament, a protein shed by damaged neurons that is tied to many neurodegenerative conditions. In March, an FDA adcomm voted unanimously that Qalsody's impact on neurofilament could predict clinical benefit, and the FDA approval reiterated that the protein could be a surrogate biomarker. — *Lei Lei Wu*

COMPANY:
Seres Therapeutics

PRICE:
$17,500 per course (three days)

PEAK SALES ESTIMATE:
$850 million – TD Cowen

APPROVAL DATE:
04/26/2023

16. Vowst

Active ingredient: fecal microbiota spores, live-brpk

that is purified from human stool. The move came a few months after Ferring Pharmaceuticals also gained a green light to market a *C. difficile* medicine, known as Rebyota. — *Kyle LaHucik*

COMPANY:
GSK

PRICE:
$280 per dose

PEAK SALES ESTIMATE:
GSK expects peak sales greater than £3 billion

APPROVAL DATE:
05/03/2023

17. Arexvy

Active ingredient: RSVPreF3

Indication: To prevent lower respiratory tract disease caused by RSV in adults 60 years and older

Snapshot: Arexvy was the first approved RSV vaccine in the US. GSK's chief commercial officer Luke Miels said at a November investor conference that Arexvy has taken over 71% of what's expected to be a multibillion-dollar market for RSV preventatives. It's in direct competition with Pfizer's Abrysvo, which was approved in May. — *Nicole DeFeudis*

COMPANY:
Chiesi Global Rare Diseases and Protalix BioTherapeutics

PRICE:
$4,135.11 for a 20-milligram vial; yearly price of about $350,000 to $400,000

PEAK SALES ESTIMATE:
N/A

APPROVAL DATE:
05/09/2023

18. Elfabrio

Active ingredient: pegunigalsidase alfa-iwxj

Indication: To treat confirmed Fabry disease

Snapshot: Enzyme replacement therapy Elfabrio is the second approved drug using Protalix's platform, which produces recombinant proteins with plant cells. The Israel-based biotech partnered with Chiesi to sell Elfabrio. The two companies originally applied for an accelerated approval in 2021, which the agency rejected. The rare kidney disease now has a handful of treatment options, including Elfabrio, Amicus Therapeutics' Galafold, and Sanofi's Fabrazyme. — *Andy Dunn*

COMPANY:

19. Veozah

Active ingredient: fezolinetant

Indication: To treat moderate to severe hot flashes caused by menopause

Snapshot: Astellas originally expected a decision on its non-hormonal hot flash treatment Veozah in February, having spent a priority review voucher to speed up the process by four months. But the FDA took extra time to review the package and didn't approve it until May, based on three Phase III trials. The company priced the drug considerably higher than ICER's previously recommended range of $2,000 to $2,500 a year. — *Ngai Yeung*

COMPANY:
Bausch + Lomb and Novaliq

PRICE:
$771 for a one-month supply (3 mL of Miebo in a 5mL bottle, or approximately 270 drops)

PEAK SALES ESTIMATE:
N/A

APPROVAL DATE:
05/18/2023

20. Miebo

Active ingredient: perfluorohexyloctane

lions of Americans and is one of the most common eye surface disorders, the company said. — *Ngai Yeung*

COMPANY:
AbbVie and Genmab

PRICE:
$37,500 per month

PEAK SALES ESTIMATE:
N/A

APPROVAL DATE:
05/19/2023

21. Epkinly

Active ingredient: epcoritamab-bysp

Indication: To treat relapsed or refractory diffuse large B-cell lymphoma (not otherwise specified) and high-grade B-cell lymphoma after two or more lines of systemic therapy

Snapshot: AbbVie and Genmab came in second to Roche and Genentech's Lunsumio when they got an Epkinly approval in May, but the CD3xCD20 drug is the first subcutaneous bispecific antibody approved as a third-line treatment for relapsed/refractory large B cell lymphoma. The approval put a bow on a 2020 alliance between the companies when AbbVie paid $750 million upfront to partner with Genmab and promised as much as $3.15 billion in milestones. The pair plans to keep testing the drug for other blood cancers. Epkinly was one of four bispecific T cell engagers approved by the FDA in 2023 in large B cell lymphoma or multiple

COMPANY:
Krystal Biotech

PRICE:
$24,250 per vial, with the average patient using 26 vials per year; Annual cost of about $631,000 per patient

PEAK SALES ESTIMATE:
N/A

APPROVAL DATE:
05/19/2023

22. Vyjuvek

Active ingredient: beremagene geperpavec-svdt

Indication: To treat wounds in patients 6 months of age and older with dystrophic epidermolysis bullosa with mutation(s) in the collagen type VII alpha 1 chain (COL7A1) gene

Snapshot: Vyjuvek in May became the first approved treatment in the US for "butterfly children" — kids born with a rare genetic condition called dystrophic epidermolysis bullosa that makes their skin incredibly fragile. The gel treatment, marketed by Krystal Biotech, is applied to wounded skin, and unlike other gene therapies which can only be dosed once, the herpes virus-based gene therapy gel can be re-applied. Krystal Biotech is now investigating an eye drop version of the treatment that could potentially help patients with vision complications or blindness from the genetic condition. — *Lei Lei Wu*

Phase 3 was 7-10 days

PEAK SALES ESTIMATE:
~$110 million – Goldman Sachs

APPROVAL DATE:
05/23/2023

23. Xacduro

Active ingredient: sulbactam, durlobactam

Indication: To treat hospital-acquired bacterial pneumonia and ventilator-associated bacterial pneumonia caused by susceptible isolates of *Acinetobacter baumannii-calcoaceticus* complex

Snapshot: In May, the FDA approved Xacduro for certain pneumonia infections that are most frequently seen in hospitals. The dual antibiotic targets a type of bacteria called *Acinetobacter baumannii-calcoaceticus* complex, which predominantly causes pneumonia and can be highly resistant to current drugs. Innoviva subsidiary Entasis Therapeutics pushed the antibiotic forward after AstraZeneca cut the program as part of a larger retreat from antibiotics research. The FDA highlighted the importance of the approval for these difficult-to-treat infections — but whether Xacduro can succeed commercially in a market that makes it difficult for new antibiotics to turn a profit remains to be seen. — *Lei Lei Wu*

COMPANY:
Blue Earth Diagnostics

PRICE:
$4,775

PEAK SALES ESTIMATE:

Indication: To use with positron emission tomography (PET) imaging in certain patients with prostate cancer

Snapshot: The FDA approved Posluma, a positron emission tomography imaging agent, for individuals with metastasized prostate cancer who are candidates for therapy or with suspected recurrence based on elevated PSA levels. — *Jared Whitlock*

COMPANY:
Pfizer

PRICE:
$1,390 for a five-day course

PEAK SALES ESTIMATE:
$18.9 billion in 2022

APPROVAL DATE:
05/25/2023

25. Paxlovid

Active ingredient: nirmatrelvir, ritonavir

Indication: To treat mild-to-moderate Covid-19 in adults at high risk for progression to severe Covid-19

Snapshot: Covid-19 pandemic stalwart Paxlovid needs little introduction. The protease inhibitor won a full FDA approval in May to treat mild-to-moderate Covid-19 in high-risk adults after being on the market since December 2021 under an emergency use authorization. It raked in $19 billion in

COMPANY:
Lexicon Pharmaceuticals

PRICE:
$598 per month

PEAK SALES ESTIMATE:
Consensus sees peak sales of $697 million in 2031; Jefferies sees peak sales reaching $332 million

APPROVAL DATE:
05/26/2023

26. Inpefa

Active ingredient: sotagliflozin

Indication: To treat heart failure and related hospitalizations, including type 2 diabetes and chronic kidney disease

Snapshot: The FDA approved Lexicon's heart failure drug Inpefa in May. The sodium-glucose cotransporter 2 (SGLT2) inhibitor is used to reduce the risk of cardiovascular death, as well as heart failure-related hospitalization in adults with cardiovascular-related diseases, such as type 2 diabetes. Lexicon only won approval after a series of hiccups, including a 2019 FDA rejection of Inpefa as a treatment for diabetes, which led partner Sanofi to cancel its $1.7 billion deal with the company a few months later. — *Anna Brown*

COMPANY:
Pfizer

PRICE:
$295 per dose

PEAK SALES ESTIMATE:
N/A

APPROVAL DATE:
05/31/2023

27. Abrysvo

Active ingredient: RSVpreF

Indication: To prevent lower respiratory tract disease caused by RSV in adults 60 years and older

Snapshot: Abrysvo was approved in May for the prevention of RSV in older adults, then again in August to help protect newborns when administered to women between 32 and 36 weeks of pregnancy. It's the first maternal vaccine to help protect newborns against lower respiratory tract disease from RSV through 6 months of age. —*Nicole DeFeudis*

COMPANY:
Roche

PRICE:
$350,000 for an 8.5 month course

PEAK SALES ESTIMATE:
$1.23 billion – Bloomberg analyst consensus

Indication: To treat diffuse large B-cell lymphoma, not otherwise specified, or large B-cell lymphoma arising from follicular lymphoma after two or more lines of systemic therapy

Snapshot: Roche expanded its bispecific antibody franchise in June with an accelerated approval of Columvi as a third-line treatment for an aggressive form of non-Hodgkin's lymphoma. It's the company's second CD20xCD3 therapy designed to connect T cells to blood cancers, equipped with two antibody arms targeting the blood cell marker CD20 to make it more potent than its predecessor, Lunsumio. In a Phase I/II trial, 56% of patients responded to an 8.5-month course of Columvi, and 43% had a complete response. — *Ryan Cross*

COMPANY:
Sarepta Therapeutics

PRICE:
$3.2 million

PEAK SALES ESTIMATE:
N/A

APPROVAL DATE:
06/22/2023

29. Elevidys

Active ingredient: delandistrogene moxeparvovec-rokl

Indication: To treat ambulatory pediatric patients aged 4 through 5 years with Duchenne muscular dystrophy (DMD) with a confirmed mutation in the DMD gene

Snapshot: The FDA's accelerated approval of Sarepta's

on the data. — *Lei Lei Wu*

COMPANY:
Pfizer

PRICE:
$49,000 per year

PEAK SALES ESTIMATE:
$914.6 million – Bloomberg analyst consensus

APPROVAL DATE:
06/23/2023

30. Litfulo

Active ingredient: ritlecitinib

Indication: To treat severely patchy hair loss

Snapshot: Pfizer may have followed behind Eli Lilly's Olumiant in getting its drug Litfulo approved for alopecia, but the New York drug giant won FDA clearance for both adults and teens, giving it access to a broader patient population. Litfulo comes with a boxed safety warning, like other JAK inhibitors. In Litfulo's pivotal trial, 23% of patients on the 50 mg dose had 80% or more scalp hair coverage after six months, compared to just 2% in the placebo group. Litfulo is also being studied to treat vitiligo, ulcerative colitis and Crohn's disease. — *Max Gelman*

COMPANY:
UCB

PRICE:
$6,050 per vial

PEAK SALES ESTIMATE:
$800 million – Bloomberg analyst consensus

APPROVAL DATE:
06/26/2023

31. Rystiggo

Active ingredient: rozanolixizumab-noli

Indication: To treat generalized myasthenia gravis in adults who are anti-acetylcholine receptor- or anti-muscle-specific tyrosine kinase antibody-positive

Snapshot: The FDA in June approved UCB's Rystiggo to treat generalized myasthenia gravis, a rare autoimmune disease. The Belgian company said it's the first option to treat the two main subtypes of the disease. The approval, which was granted priority review, came on the heels of argenx's myasthenia gravis drug Vyvgart Hytrulo. Rystiggo works by targeting the neonatal Fc receptor in hopes of reducing levels of immunoglobulin G antibodies, which impede the messaging between nerves and muscles. — *Lia DeGroot*

COMPANY:
Pfizer and OPKO Health

PRICE:
$8,300 monthly list price, assuming a 35 kg child

Active ingredient: somatrogon-ghla

Indication: To treat growth failure due to inadequate secretion of endogenous growth hormone

Snapshot: Pfizer and the Miami-based healthcare company OPKO Health started working together in 2014 on a longer-acting treatment for growth hormone deficiency. Nearly a decade later, and after an initial rejection in 2022, the FDA approved Ngenla in June for children as young as three years old. Pfizer is in charge of selling the drug, which will compete against the Danish drugmaker Ascendis Pharma's Skytrofa, a weekly injection like Pfizer's drug. — *Andy Dunn*

COMPANY:
CellTrans

PRICE:
N/A

PEAK SALES ESTIMATE:
N/A

APPROVAL DATE:
06/28/2023

33. Lantidra

Active ingredient: donislecel

Indication: To treat type 1 diabetes

Snapshot: Lantidra is the first cell therapy approved to treat type 1 diabetes. The treatment, which involves taking pancre-

COMPANY:
BioMarin

PRICE:
$2.9 million

PEAK SALES ESTIMATE:
$2.2 billion, per Biopharma Dive

APPROVAL DATE:
06/29/2023

34. Roctavian

Active ingredient: valoctocogene roxaparvovec-rvox

Indication: To treat adults with severe hemophilia A without antibodies to adeno-associated virus serotype 5 detected by an FDA-approved test

Snapshot: Roctavian is the first gene therapy approved to treat hemophilia A, a genetic bleeding disorder. It was approved in June following a 2020 rejection and a review extension in 2023, and BioMarin hopes to turn it into a key blockbuster drug. — *Nicole DeFeudis*

Commercial: $495; Vaccines for children program: $395

PEAK SALES ESTIMATE:
$3 billion – Jefferies

APPROVAL DATE:
07/17/2023

35. Beyfortus

Active ingredient: nirsevimab-alip

Indication: To prevent respiratory syncytial virus (RSV)

Snapshot: In July, the FDA approved Beyfortus, an antibody drug co-developed by Sanofi and AstraZeneca. Beyfortus, also known as nirsevimab, is given as a one-time intramuscular injection to infants, protecting them from respiratory syncytial virus, or RSV. Beyfortus is the latest in a suite of new medicines against RSV, a virus that typically puts 58,000 to 80,000 young children in the US in the hospital every year, according to the CDC. — *Jared Whitlock*

COMPANY:
Emergent BioSolutions

PRICE:
N/A

PEAK SALES ESTIMATE:
N/A

APPROVAL DATE:
07/20/2023

36. Cyfendus

causes anthrax, *Bacillus anthracis*. While the two-dose vaccine received full approval in 2023, it has been provided to the US government for years under pre-emergency use authorization. — *Nicole DeFeudis*

COMPANY:
Daiichi Sankyo

PRICE:
$546 per tablet for 7.7 mg and 26.5 mg doses

PEAK SALES ESTIMATE:
N/A

APPROVAL DATE:
07/20/2023

37. Vanflyta

Active ingredient: quizartinib

Indication: To use as part of a treatment regimen for newly diagnosed acute myeloid leukemia that meets certain criteria

Snapshot: Daiichi Sankyo won FDA approval for Vanflyta in July as a frontline treatment for acute myeloid leukemia. This came after the agency delayed a decision on the approval in April — extending the PDUFA date by three months to review more data from the company. As well as being used as a first-line treatment in combination with chemo, Vanflyta was also approved as a maintenance monotherapy. — *Anna Brown*

COMPANY:
Octapharma

PRICE:
N/A

PEAK SALES ESTIMATE:
N/A

APPROVAL DATE:
07/21/2023

38. Balfaxar

Active ingredient: prothrombin complex concentrate, human-lans

Indication: To help restore blood coagulation in patients on blood thinners before urgent surgeries or invasive procedures

Snapshot: Balfaxar is approved for adults who need an urgent reversal of blood thinners such as warfarin ahead of surgeries or other invasive procedures. While blood thinners are prescribed to more than 2.4 million Americans to reduce the risk of blood clots, they also put those patients at a higher risk of heavy bleeding in surgery. — *Nicole DeFeudis*

COMPANY:
Tarsus Pharmaceuticals

PRICE:
$1,850 for six-week treatment course

39. Xdemvy

Active ingredient: lotilaner

Indication: To treat Demodex blepharitis

Snapshot: Tarsus' first-ever drug approval, Xdemvy, came earlier than expected in July, a month before its FDA deadline. It's the first-ever drug approved to treat Demodex blepharitis, eye irritation and inflammation caused by tiny mites that are often mistaken for allergy symptoms. The eye drops are a reformulation of a veterinary drug used to prevent fleas and ticks, and Tarsus' studies showed significant improvement in both mite eradication (52% of treated patients) and reduction of yellowish eyelid collarettes (89% of treated patients) at six weeks. — *Beth Bulik*

COMPANY:
Iveric Bio

PRICE:
$2,100 per single-dose vial

PEAK SALES ESTIMATE:
FY2025 – peak sales of ¥200 billion to ¥400 billion (~1.4 billion – 2.8 billion USD) -Astellas; Global peak sales ¥500 billion – Jefferies

APPROVAL DATE:
08/04/2023

40. Izervay

Active ingredient: avacincaptad pegol

first ever approval for the condition, which is a leading cause of blindness, earlier in the year. The back-to-back nods set up a showdown over safety and efficacy. Izervay's use was capped at 12 months, whereas Apellis' Syfovre has no limitations, but the biotech presented more data in September hoping to expand its label. — *Kyle LaHucik*

COMPANY:
Biogen and Sage Therapeutics

PRICE:
$15,900 for a one-time two-week course

PEAK SALES ESTIMATE:
$655.7 million – Bloomberg analyst consensus

APPROVAL DATE:
08/04/2023

41. Zurzuvae

Active ingredient: zuranolone

Indication: To treat postpartum depression

Snapshot: After decades without any pharmaceutical progress, mothers suffering from postpartum depression finally have a powerful option. The fast-acting drug Zurzuvae can dramatically lift moods in a matter of days. It's a highly awaited sequel to Sage's onerous 60-hour infusion for the condition that was a commercial failure. Zurzuvae was inspired by a natural hormone whose falling levels may trigger postpartum depression and target GABA receptors in the brain. But Zurzuvae's effectiveness was murky in major depressive disorder, a far larger market, leading the FDA to re-

COMPANY:
Johnson & Johnson

PRICE:
$45,000 per month

PEAK SALES ESTIMATE:
$2.38 billion – Bloomberg analyst consensus

APPROVAL DATE:
08/09/2023

42. Talvey

Active ingredient: talquetamab-tgvs

Indication: To treat adults with relapsed or refractory multiple myeloma who have received at least four prior therapies

Snapshot: Johnson & Johnson is on a roll in blood cancer. Talvey's accelerated approval gives the company five marketed multiple myeloma drugs, and it joins a growing arsenal of medicines for multiple myeloma, including J&J and Legend Biotech's CAR-T Carvykti, Bristol Myers Squibb's Abecma, Pfizer's newly cleared Elrexfio and others. The bispecific antibody, injected under the skin weekly or biweekly, goes after the CD3 receptor on the T cell's surface and GPRC5D on multiple myeloma cells, among other cells. It comes with a boxed warning for cytokine release syndrome. — *Kyle LaHucik*

PEAK SALES ESTIMATE:
$733.2 million – Bloomberg analyst consensus

APPROVAL DATE:
08/14/2023

43. Elrexfio

Active ingredient: elranatamab-bcmm

Indication: To treat adults with relapsed or refractory multiple myeloma who have received at least four prior lines of therapy

Snapshot: Pfizer expects that Elrexfio could become a "multibillion-dollar franchise" following its accelerated approval in August for adults with multiple myeloma. The subcutaneous injection was cleared for relapsed or refractory multiple myeloma patients who have previously received at least four prior therapies, including a proteasome inhibitor, an immunomodulatory agent and an anti-CD38 monoclonal antibody. It works by targeting BCMA on myeloma cells and CD3 on T cells, which prompts the T cells to attack the myeloma cells. — *Lia DeGroot*

COMPANY:
Ipsen

PRICE:
Estimated annual list price of $624,000

PEAK SALES ESTIMATE:
Over €100 million – Ipsen

APPROVAL DATE:
08/16/2023

fibrodysplasia ossificans progressiva

Snapshot: Ipsen's Sohonos was approved for the treatment of the ultra-rare disease fibrodysplasia ossificans progressiva despite the FDA's questions over its efficacy and safety. The agency's OK came after a 2022 rejection, in which regulators asked for more data from existing studies. Ipsen resubmitted its application and said Sohonos should come with a boxed warning about premature growth plate closure. The drug also eventually got a 10-4 vote in favor of its benefit-risk profile from the FDA's Endocrinologic and Metabolic Drugs Advisory Committee, and approval followed. — *Katherine Lewin*

REGENERON

COMPANY:
Regeneron

PRICE:
$34,600 per single-use vial

PEAK SALES ESTIMATE:
N/A

APPROVAL DATE:
08/18/2023

45. Veopoz

Active ingredient: pozelimab-bbfg

Indication: To treat patients 1 year and older with CD55-deficient protein-losing enteropathy (PLE), also known as CHAPLE disease

Snapshot: Veopoz is approved to treat CHAPLE disease, a rare but potentially life-threatening immune disease that affects fewer than 100 people worldwide. It targets a protein called complement factor C5, which is involved in the active

COMPANY:
BioLineRx

PRICE:
$5,900 per vial

PEAK SALES ESTIMATE:
N/A

APPROVAL DATE:
09/08/2023

46. Aphexda

Active ingredient: motixafortide

Indication: To use with filgrastim (G-CSF) to mobilize hematopoietic stem cells to the peripheral blood for collection and subsequent autologous transplantation in patients with multiple myeloma

Snapshot: Autologous stem cell transplantation is part of the standard of care for multiple myeloma patients, a process in which a patient's stem cells are removed and reintroduced after treatment with chemotherapy. But with some patients, there's trouble collecting enough hematopoietic stem cells in one round, leading to sessions that can last hours a day for up to five days. BioLineRx's drug, Aphexda, is used in combination with filgrastim to make the cells easier to collect. — *Nicole DeFeudis*

COMPANY:
GSK

PRICE:
$26,900
for a 30-tablet bottle

PEAK SALES ESTIMATE:
$861 million – Bloomberg analyst consensus

APPROVAL DATE:
09/15/2023

47. Ojjaara

Active ingredient: momelotinib

Indication: To treat intermediate or high-risk myelofibrosis in adults with anemia

Snapshot: Ojjaara's approval in September for myelofibrosis patients with anemia capped off a long turnaround story for the JAK inhibitor. The drug was shelved by Gilead after a failed study and then licensed by Sierra Oncology for only $3 million upfront in 2018. After Sierra successfully steered the candidate through a new late-stage study, GSK swooped in with $1.9 billion to buy the company. Ojjaara was the fourth JAK inhibitor approved for the rare bone marrow cancer, but clinical studies suggest that, unlike earlier JAK inhibitors, it can help control anemia instead of causing or worsening it — a boon for a patient population in which many develop anemia. — *Lei Lei Wu*

COMPANY:
Fabre-Kramer Pharmaceuticals

PRICE:
N/A

Active ingredient: gepirone

Indication: To treat major depressive disorder

Snapshot: The biotech industry got a surprise in late September when a largely forgotten company said it had received FDA approval for its major depressive disorder tablet Exxua. The move came about three months later than expected because of "major amendments" to the filing that needed to be addressed, but the approval was actually two decades later than the drug's developers had initially liked. Houston-based Fabre-Kramer Pharmaceuticals and its partners received multiple no-gos from the FDA and its outside advisors dating all the way back to 2002. The drug shuffled through the hands of Organon, GSK and back to Fabre-Kramer during its multi-decade journey. — *Kyle LaHucik*

COMPANY:
Amicus Therapeutics

PRICE:
$650,000 for Pombiliti and Opfolda for a patient weighing approximately 70 kg

PEAK SALES ESTIMATE:
$706.8 million – Bloomberg analyst consensus

APPROVAL DATE:
09/28/2023

49. Pombiliti

Active ingredient: cipaglucosidase alfa-atga

Indication: To treat late-onset Pompe disease

COMPANY:
Novo Nordisk

PRICE:
Rivfloza will be priced "to reflect its demonstrated clinical value and benefit to those living with PH1," a spokesperson said.

PEAK SALES ESTIMATE:
N/A

APPROVAL DATE:
09/29/2023

50. Rivfloza

Active ingredient: nedosiran

Indication: To lower urinary oxalate levels in patients 9 years and older with primary hyperoxaluria type 1 and relatively preserved kidney function

Snapshot: Rivfloza is an RNAi therapy developed by Dicerna to treat a rare kidney disease that's marked by an excessive production of oxalate, a substance that can combine with calcium to cause kidney stones. The drug lowers urinary oxalate levels, and will be available in early 2024, according to Novo Nordisk. It will compete with Alnylam's rival Oxlumo, which was approved in 2020. — *Nicole DeFeudis*

COMPANY:
Pfizer

PRICE:
$6,164 for a 30-day bottle

PEAK SALES ESTIMATE:
$2.02 billion – Bloomberg analyst consensus

APPROVAL DATE:
10/12/2023

51. Velsipity

Active ingredient: etrasimod

Indication: To treat moderate to severe plaque psoriasis in adults who are candidates for systemic therapy or phototherapy

Snapshot: Velsipity was at the center of Pfizer's $6.7 billion acquisition of Arena Pharmaceuticals. It's the second selective sphingosine-1-phosphate (S1P) receptor modulator approved to treat ulcerative colitis behind Bristol Myers Squibb's Zeposia, though it will also contend with a variety of other treatments from steroids to biologics. Velsipity and Zeposia are contraindicated in patients with certain heart issues and carry warnings for bradyarrhythmia, where the heart rate temporarily slows down. — *Nicole DeFeudis*

10/17/2023

52. Bimzelx

Active ingredient: bimekizumab

Indication: To treat moderate to severe plaque psoriasis in adults who are candidates for systemic therapy or phototherapy

Snapshot: UCB's Bimzelx overcame headwinds at the company's Belgian manufacturing plant to win US approval in plaque psoriasis in October, two years after a thumbs-up in Europe. The IL-17A/IL-17F inhibitor was rejected by the FDA in early 2022 and faced further delays in its review process later that year. After satisfying the FDA that the manufacturing issues were resolved, Bimzelx was launched in the US in November. The Belgian drugmaker believes the product can reach sales of at least €4 billion ($4.23 billion), despite warnings on its label for liver monitoring and suicidal ideation. — *Ayisha Sharma*

COMPANY:
UCB

PRICE:

PEAK SALES ESTIMATE:
$528 million – TD Cowen

APPROVAL DATE:
10/17/2023

53. Zilbrysq

Active ingredient: zilucoplan

receptor (AChR) antibody-positive. Unlike traditional C5-targeting monoclonal antibodies, Zilbrysq can be used alongside intravenous immunoglobulin and plasma exchange without supplemental dosing. According to TD Cowen analysts, Zilbrysq's once-daily subcutaneous dosing regimen lines up with patient wants, who projected at least €500 million ($528 million) in sales. — *Ayisha Sharma*

COMPANY:
Eli Lilly

PRICE:
$9,593 per month for initial IV infusion then $10,360 per under-the-skin injection thereafter

PEAK SALES ESTIMATE:
$1.51 billion – Bloomberg analyst consensus

APPROVAL DATE:
10/26/2023

54. Omvoh

Active ingredient: mirikizumab-mrkz

Indication: To treat ulcerative colitis

Snapshot: Lilly entered the big inflammatory bowel disease market thanks to a second chance with Omvoh. The pharma giant had ditched plans to request approval in psoriasis in 2021, then moved forward in ulcerative colitis but received an FDA rejection in April because of manufacturing issues. Those issues were resolved and the FDA gave it a nod in October. The Indianapolis drugmaker had recently beefed up its autoimmune pipeline via the $2.4 billion purchase of DICE Therapeutics. In a similar area, Lilly seeks to mark a turn-

COMPANY:
Santhera

PRICE:
N/A

PEAK SALES ESTIMATE:
$500 million – 2021 estimate from Santhera

APPROVAL DATE:
10/26/2023

55. Agamree

Active ingredient: vamorolone

Indication: To treat Duchenne muscular dystrophy

Snapshot: After multiple setbacks, Santhera's commitment to Duchenne muscular dystrophy finally paid off when its steroid-based drug Agamree won FDA approval in October. Agamree was meant to be filed in 2022, but third-party manufacturing issues delayed the process by several months. The approval gave a much-needed win, after Santhera's previous Duchenne candidate, idebenone, failed a Phase III trial in 2020 and was discontinued. Agamree is designed to address the problem of dose-limiting toxicities linked with more traditional steroids. — *Ayisha Sharma*

PEAK SALES ESTIMATE:
$200 million peak in US – Coherus; 2030 sales of $160 million – TD Cowen

APPROVAL DATE:
10/27/2023

56. Loqtorzi

Active ingredient: toripalimab-tpzi

Indication: To treat recurrent or metastatic nasopharyngeal carcinoma when used together with or following other therapies

Snapshot: Junshi Biosciences made history in China by bringing the first homegrown PD-1 to the market five years ago. Together with partner Coherus, it also beat much bigger players in ushering the first Chinese checkpoint inhibitor into the US, winning approval for Loqtorzi in patients with recurrent or metastatic nasopharyngeal carcinoma across all lines of treatment. The approval followed an initial rejection and subsequent review delay. Going after a cancer type that's prevalent in southeast Asia but relatively rare in the US, Junshi and Coherus won over the FDA with clinical data from just China and several other Asian countries — something that the agency deemed insufficient in bigger indications. — *Amber Tong*

COMPANY:
Takeda

PRICE:
N/A

PEAK SALES ESTIMATE:
$144.9 million – Bloomberg analyst consensus

Indication: To treat refractory, metastatic colorectal cancer

Snapshot: The FDA's approval for Fruzaqla may have gone to Takeda, but it's also a momentous decision for Hutchmed, which first won approval for the VEGF receptor kinase inhibitor in China in 2018 under a different name. Takeda bought the ex-China global rights for $400 million upfront at the beginning of the year. In clinical trials, the oral drug was shown to improve both overall survival and progression-free survival in patients with previously treated metastatic colorectal cancer, a space that Takeda says has seen few new treatments over the years. — *Amber Tong*

COMPANY:
CorMedix

PRICE:
N/A

PEAK SALES ESTIMATE:
N/A

APPROVAL DATE:
11/15/2023

58. Defencath

Active ingredient: taurolidine, heparin

Indication: To reduce the incidence of catheter-related bloodstream infections in adults with kidney failure receiving chronic hemodialysis through a central venous catheter

Snapshot: Defencath is approved to reduce catheter-related bloodstream infections in adults with kidney failure. Its use is limited to patients who are receiving chronic hemodialysis

COMPANY:
Bristol Myers Squibb

PRICE:
$29,000 per month for adult patients

PEAK SALES ESTIMATE:
$997.4 million – Bloomberg analyst consensus

APPROVAL DATE:
11/15/2023

59. Augtyro

Active ingredient: repotrectinib

Indication: To treat ROS1-positive non-small cell lung cancer

Snapshot: Augtyro scored approval in November, adding another treatment option for adult patients with locally advanced or metastatic ROS1-positive non-small cell lung cancer. It's a rival to Pfizer's Xalkori and Roche's Rozlytrek in ROS1-positive NSCLC, and marked a payoff for Bristol Myers Squibb after it acquired the drug when it bought Turning Point Therapeutics for $4.1 billion in June 2022. — *Katherine Lewin*

PRICE:$22,922 FOR A 28-DAY SUPPLY OF THE MEDICINE

PEAK SALES ESTIMATE:
$1.44 billion – Bloomberg analyst consensus

APPROVAL DATE:
11/16/2023

60. Truqap

Active ingredient: capivasertib

Indication: To treat breast cancer that meets certain disease criteria

Snapshot: With the approval of Truqap, AstraZeneca expanded its breast cancer portfolio to offer patients who have hormone receptor-positive, HER2-negative tumors another targeted therapy option. However, the label comes with a restriction: The AKT inhibitor is approved in combination with AstraZeneca's own Faslodex to treat a subset of patients whose tumors harbor certain genetic alterations, a narrower population than some analysts expected based on late-stage data showing progression-free survival improvement for all-comers. Testing is required to prescribe the drug. AstraZeneca first discovered Truqap via a longtime alliance with Otsuka subsidiary Astex. — *Amber Tong*

COMPANY:
Evive Biotech and Acrotech Biopharma

PRICE:
N/A

PEAK SALES ESTIMATE:
N/A

Indication: To treat neutropenia

Snapshot: Evive and Acrotech secured approval in November for Ryzneuta to decrease the incidence of febrile neutropenia in adults with non-myeloid malignancies who are receiving myelosuppressive cancer drugs. It's the first innovative biologic independently developed by Evive Biotech, a subsidiary of China-based Yifan Pharmaceutical. — *Zachary Brennan*

COMPANY:
SpringWorks Therapeutics

PRICE:
$29,000 per 30-day supply

PEAK SALES ESTIMATE:
$1.66 billion – Bloomberg analyst consensus

APPROVAL DATE:
11/27/2023

62. Ogsiveo

Active ingredient: nirogacestat

Indication: To treat adults with progressing desmoid tumors who require systemic treatment

Snapshot: Ogsiveo was approved in November following a three-month delay after regulators said they needed more time to review responses they requested from the company. The drug was licensed from Pfizer, which had tested it in a Phase I trial across solid tumor types. It was later discovered

COMPANY:
Novartis

PRICE:
$550,000 per year

PEAK SALES ESTIMATE:
More than $3B for all indications in late-stage development – Novartis

APPROVAL DATE:
12/05/2023

63. Fabhalta

Active ingredient: iptacopan

Indication: To treat paroxysmal nocturnal hemoglobinuria

Snapshot: Fabhalta was approved to treat paroxysmal nocturnal hemoglobinuria, a rare disorder where red blood cells break up early. The twice-daily oral pill rivals AstraZeneca's Soliris and Ultomiris infusions, which have been widely used for years and generated more than $5.7 billion in 2022 sales. However, Fabhalta carries a boxed warning for serious infections caused by encapsulated bacteria, and its use will be limited under a Risk Evaluation and Mitigation Strategy (REMS) program. — *Nicole DeFeudis*

PRICE:
$2.2 million

PEAK SALES ESTIMATE:
$2.35 billion – Bloomberg analyst consensus

APPROVAL DATE:
12/08/2023

64. Casgevy

Active ingredient: exagamglogene autotemcel

Indication: To treat sickle cell disease (SCD) in patients 12 years and older

Snapshot: Dec. 8 was an important day for sickle cell patients as the FDA approved two new gene therapies to treat the disease, including Casgevy, the first CRISPR treatment in the US. The decision capped decades of research and follows approvals in the UK in mid-November for sickle cell disease and transfusion-dependent beta thalassemia. Vertex will take the lead on manufacturing and commercialization and plans to open around 50 authorized treatment centers across the US, chief operating officer Stuart Arbuckle told *Endpoints News*. — *Nicole DeFeudis*

COMPANY:
bluebird bio

PRICE:
$3.1 million

PEAK SALES ESTIMATE:

Indication: To treat sickle cell disease (SCD) in patients 12 years and older

Snapshot: Lyfgenia was one of two gene therapies for sickle cell approved on Dec. 8, in addition to Vertex and CRISPR's Casgevy. However, bluebird said it would price Lyfgenia nearly $1 million more than Casgevy, raising questions about access. The company also failed to secure an anticipated priority review voucher that it was hoping would bolster its cash reserves and instead turned to a combination of public and private financing. — *Nicole DeFeudis*

COMPANY:
Chiesi

PRICE:
Chiesi spokesperson said the list price will be "competitively priced"

PEAK SALES ESTIMATE:
N/A

APPROVAL DATE:
12/18/2023

66. Filsuvez

Active ingredient: birch triterpenes

Indication: To treat wounds associated with dystrophic and junctional epidermolysis bullosa

Snapshot: Filsuvez is approved to treat wounds associated with junctional and dystrophic forms of epidermolysis bul-

COMPANY:
Ionis/AstraZeneca

PRICE:
"Consistent" with Onpattro and Amvuttra (around $450,000 per year)

PEAK SALES ESTIMATE:
$1.57 billion – Bloomberg analyst consensus

APPROVAL DATE:
12/21/2023

67. Wainua

Active ingredient: eplontersen

Indication: To treat hereditary transthyretin-mediated amyloid polyneuropathy (ATTRv-PN)

Snapshot: Wainua picked up approval in December as AstraZeneca and Ionis position it as an alternative to two of Alnylam's drugs for hereditary transthyretin-mediated amyloid polyneuropathy, or ATTRv-PN. It will likely be compared most to Amvuttra, which, like Wainua, is an injectable drug. AstraZeneca and Ionis are also shooting for the larger ATTR-CM population, finishing enrollment in July. — *Max Gelman*

AUTHOR

ENDPOINTS
editor@endpointsnews.com

Life science leaders, innovators and the investment community will convene in
Francisco this January for what is arguably the biggest week of the healthcare
nering calendar. Ahead of this, Astellas' Chief Scientific Officer, Yoshitsugu Shi
Head of Business Development, Issei Tsukamoto, and Primary Focus Lead for

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