Maryland
DEPARTMENT OF HEALTH

The Most Expensive Drugs Approved in 2023: How did we get here and what to expect next?

Saturday,
October 21, 2023

A continuing education seminar
jointly sponsored by the
MDH Office of Pharmacy Services, MedChi, APA,
and Kepro, an Acentra Health company.

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DEPARTMENT OF HEALTH

Continuing Medical Education (CME) &
Pharmacy Continuing Education (ACPE) Seminar

THE MOST EXPENSIVE DRUGS APPROVED IN 2023: HOW DID WE GET HERE AND WHAT TO EXPECT NEXT?

Virtual Live Program
on
Saturday, October 21, 2023

8:50 am – Introductions	Maryland Department of Health Office of Pharmacy Services
9:00 am – Presentation	Maria Lowe, PharmD, BCPS Director of Pharmaceutical Intelligence Institute for Clinical and Economic Review
11:00 am – Closing Remarks	Maryland Department of Health Office of Pharmacy Services
11:15 am - Adjourn	

The views and opinions expressed by the speakers are not necessarily the views and opinions of the State of Maryland Department of Health.

****This event will be recorded for future use.***
By attending, you agree to participate in audio and/or visual recording*

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MedChi designates this live virtual activity for a maximum of (2) *AMA PRA Category 1 Credit(s)*^{TM}. Evaluation form is to be completed for the presentation attended. Physicians should claim only the credit commensurate with the extent of their participation in the activity.

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Dr. Lowe states that she does not have relevant financial relationship with commercial interests and will be discussing “Off-Label” uses of products or devices. This information is on file with Kepro, an Acentra Health company.

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Activity Type: Knowledge-Based

The Most Expensive Drugs Approved in 2023

How did we get here and what to expect next.

Maria M. Lowe, PharmD, BCPS
mlowe@icer.org | Director of Pharmaceutical Intelligence

ICER
INSTITUTE FOR CLINICAL AND ECONOMIC REVIEW

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Introduction
Disclaimers and Disclosures

- Non-biased **overview** of the pipeline: **not all-inclusive**
- This CPE program **will** include discussion of **non-FDA approved (off-label)** medication use
- Maria Lowe, PharmD, BCPS declares that she has **no actual or potential conflicts of interest** to disclose in relation to this presentation
  - **None of her statements** about drugs in the pipeline should be construed as indicating which, if any, of these drugs may be reviewed by ICER in the future

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Introduction
Objectives

By the end of this presentation
You should be able to…

- Describe **recent trends** in the FDA approval process
- Summarize **select high-cost products** approved by the FDA in 2023
- Identify **agents in late stages of development** with high-cost potential

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Where Are We?

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The Most Expensive Drugs in the US
2023

Hemgenix® (etranacogene dezaparvovec-drlb) Gene therapy indicated for adults with **hemophilia B** **FDA approved:** 11/22/2022		**$3.5 million** per dose
Skysona® (elivaldogene autotemcel) Gene therapy indicated to slow the progression of neurologic dysfunction in boys 4-17 years of age with early, active **CALD** **FDA approved:** 9/16/2022 *(accelerated approval)*		**$3 million** per dose
Zynteglo® (betibeglogene autotemcel) Gene therapy Indicated for the treatment of adult and pediatric patients with **ß-thalassemia** who require regular RBC transfusions **FDA approved:** 8/17/2022		**$2.8 million** per dose
Zolgensma® (onasemnogene abeparvovec-xioi) Gene therapy indicated to treat children less than two years of age with spinal muscular atrophy **FDA approved:** 5/24/2019		**$2.25 million** per dose

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CALD: cerebral adrenoleukodystrophy, RBC: red blood cell

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How Did We Get Here?

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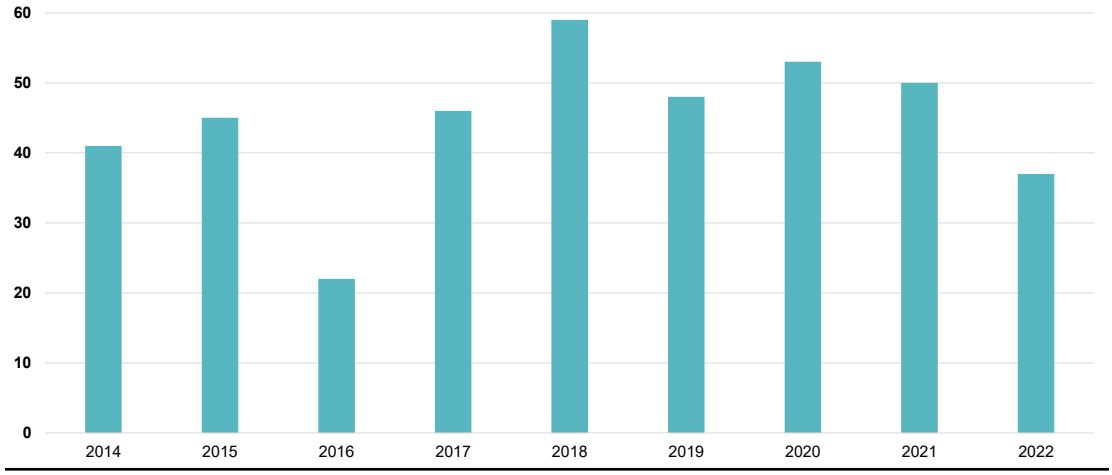
FDA Approvals
Novel Products Approved by CDER



CDER: Center for Drug Evaluation and Research

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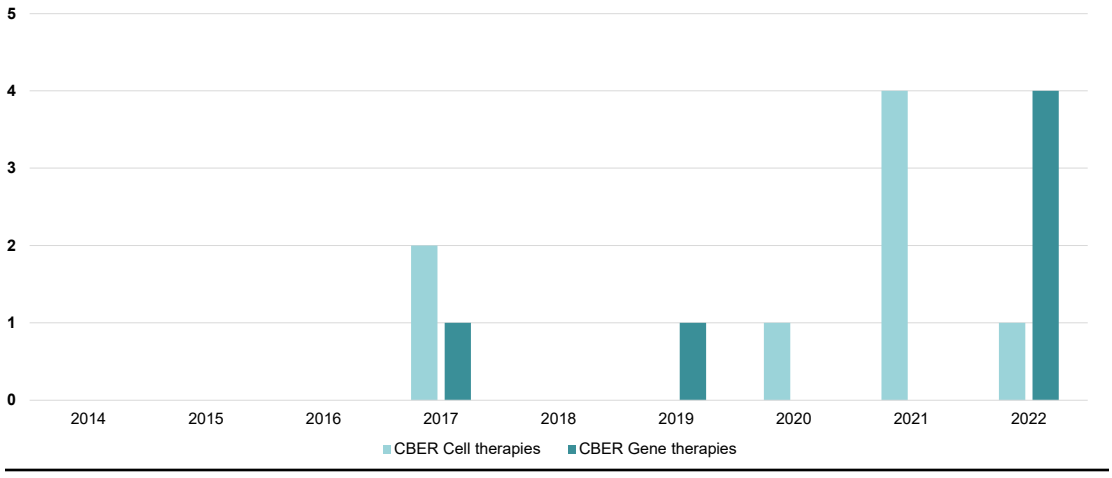
FDA Approvals
Cell and Gene Therapies Approved by CBER



CBER: Center for Biologic Evaluation and Research

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FDA Approvals
2023 So Far…

What's happening with the FDA so far this year?

Approvals appear to be up…
- **26 novel products** approved in **1H2023** (vs. 16 in 1H2022)

Ad. Comms appear to be up…
- **17 ad comm.** meetings in **1H2023** (vs. 14 in 2022)

Stories that stand out…
- **IRA** and its impact on drug development
- **Obesity management** market has taken off (bolstered by CV outcomes data)
- **Adalimumab biosimilars** are finally here
- **Psychedelics** are nearing FDA approval

Ad. Comm.: advisory committee, CV: cardiovascular, IRA: Inflation Reduction Act

FDA Approvals
2023 So Far…

And how about with gene and cell therapies?

Approvals may trend higher than last year…
- **3 gene therapies** and **2 cell therapies** in **1H2023**

Expect continued growth and evolution in this space…
- **CBER** and **OTAT**
- FDA appears to have an appetite for **regulatory flexibility**
- Role of accelerated approval pathway
- First **CRISPR-based** gene therapy undergoing FDA review

CBER: Center of Biologics Research and Evaluation, CRISPR: Clustered Regularly Interspaced Short Palindromic Repeats, OTAT: Office of Tissues and Advanced Therapies

What Happens Next?

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Cardiovascular

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Cardiovascular
2023 Approvals*

Inpefa®
(Sotagliflozin)
Developer(s): Lexicon Pharmaceuticals, Inc.
FDA approved: 5/26/2023
Price: $

- Oral, **small molecule**
- Sodium-glucose cotransporter 2 indicated to **reduce the risk of cardiovascular death, hospitalization for heart failure, and urgent heart failure visits** in adults with: heart failure or type 2 diabetes mellitus, chronic kidney disease, and other cardiovascular risk factors

*current as of 9/27/2023

Cardiovascular
Agents in Development: Heart Failure with Preserved Ejection Fraction

Mitiperstat
[AZD-4831]
Developer: AstraZeneca PLC (lead)
Price (estimate): $

- Oral, **myeloperoxidase inhibitor**
- **Phase II/III**

Finerenone
(Kerendia®)
Developer(s): Bayer AG (lead)
Price (estimate): $

- Oral, **MRA**
- **Phase III** *(for expanded approval)*

Semaglutide
(Ozempic®,Wegovy®)
Developer(s): Novo Nordisk A/S (lead)
Price (estimate): $

- SC, **GLP-1 agonist**
- **Phase III** *(for expanded approval)*

Tirzepatide
(Mounjaro®)
Developer(s): Eli Lilly and Company (lead), Mitsubishi Tanabe Pharma Corporation (partner)
Price (estimate): $

- SC, **GLP-1/GIP agonist**
- **Phase III** *(for expanded approval)*

GIP: glucose-dependent insulinotropic polypeptide receptor, GLP-1: glucagon-like peptide-1 receptor, MRA: mineralocorticoid receptor antagonist, SC: subcutaneous

Cardiovascular

Agents in Development: Pulmonary Arterial Hypertension (PAH)

Sotatercept [MK-7962]

Developer(s): Merck & Co., Inc. (lead), Bristol Myers Squibb Company (partner), Acceleron Pharma, Inc. (former), Celgene Corporation (former)
Price (estimate): $-$$

Description/Details

- SC, every 21 days (add-on therapy)
- **Activin receptor type IIA-Fc (ActRIIA-Fc) fusion protein**

Regulatory Status

- Seeking approval for the treatment of **PAH**
- Regulatory review; FDA Decision expected **3/26/2024**

Trial Results

- Phase III **STELLAR** trial[1]
  - ↑ **exercise capacity** over baseline vs placebo
  - ↑ **6MWD by 40.8 meters** (95% CI, 27.5-54.1; p<0.001) at week 24

[1]Hoeper MM et al. NEJM. 2023;388:1478-1490
6MWD: 6-minute walk distance, BLA: biologic license application, CI: confidence interval, SC: subcutaneous, PAH: pulmonary arterial hypertension

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Central Nervous System

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Central Nervous System
2023 Approvals*

Daybue® (Trofinetide)
Developer(s): ACADIA Pharmaceuticals Inc. (lead), Neuren Pharmaceuticals Limited (partner)
FDA approved: 3/10/2023
Price: $

- Oral solution, **small molecule**
- Indicated for the treatment of **Rett syndrome** in adults and pediatric patients ≥ 2 years

Exxua® (Gepirone)
Developer(s): ACADIA Pharmaceuticals Inc. (lead), Neuren Pharmaceuticals Limited (partner)
FDA approved: 9/22/2023
Price: $

- Oral ER tablets, **small molecule**
- Indicated for the treatment of **major depressive disorder** in adults

Zavzpret® (Zavegepant)
Developer(s): Pfizer Inc. (lead), Bristol Myers Squibb Company (partner), Royalty Pharma plc (partner)
FDA approved: 3/9/2023
Price: $

- Nasal spray, **small molecule**
- Calcitonin gene-related peptide receptor antagonist indicated for the **acute treatment of migraine** with or without aura

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*current as of 9/27/2023
ER: extended-release

Central Nervous System
2023 Approval: Amyotrophic Lateral Sclerosis (ALS)

Qalsody® (Tofersen)

Developer(s): Biogen, Inc. (lead), Ionis Pharmaceuticals, Inc. (partner)
FDA approved: 4/25/2023 *(accelerated approval)*
Price: $$

Description/Details

- Intrathecal, **antisense oligonucleotide** designed to inhibit production of SOD1
- Indicated for the treatment of **ALS** in adults who have a mutation in the **SOD1 gene**
- **Dosing:**
  - Loading: 3 doses every 14-days
  - Maintenance: 1 dose every 28 days

Trial Results

- Phase III **VALOR** trial[1]
  - 28-week, RCT, DB, PC (N=108)
  - **Less decline** over baseline in **ALSFRS-R** (difference vs. placebo not statistically significant)
  - **Lower levels of NfL** at week 28 over baseline (difference vs. placebo 0.40, 95% CI: 0.33, 0.49; P<0.0001)

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[1]Qalsody package insert, 4/25/2023
ALS: amyotrophic lateral sclerosis, ALSFRS-R: ALS functional rating scale revised, DB: double-blind, CI: confidence interval, NfL: neurofilament light, PC: placebo-controlled, RCT: randomized controlled trial, SOD: superoxide dismutase 1

Central Nervous System
Agents in Development: Amyotrophic Lateral Sclerosis (ALS)

NurOwn® (autologous MSC-NTF cells)

Developer(s): BrainStorm Cell Therapeutics Inc. (lead)
Price (estimate): $$-$$$

Description/Details
- IV/intrathecal autologous mesenchymal **stem cells** secreting neurotrophic factors

Regulatory Status
- Seeking approval for the treatment of **ALS**
- FDA decision expected **12/8/2023**

Trial Results
- Phase III **BCT-002-US** trial[1]
  - **Reduced NfL** at week 20 vs. placebo (p<0.05)
  - Participants with **greater ALSFRS-R decline** from baseline at week 28 had **higher baseline NfL values**, r=-0.33, p=0.0064

[1]BrainStorm press release, 7/7/2023
ALS: amyotrophic lateral sclerosis, ALSFRS-R: ALS functional rating scale revised, BLA: biologic license application, IV: intravenous, MSC: mesenchymal stromal cells, NfL: neurofilament light, NTF: neutrophilic factors

Central Nervous System
2023 Approval: Alzheimer's Disease (AD)

Leqembi® (Lecanemab)

Developer(s): Eisai Co., Ltd. (lead), BioArctic AB (partner), Biogen, Inc. (partner)
FDA approved: 1/6/2023
Price: $$

Description/Details
- IV, **anti-beta amyloid monoclonal antibody**
- Indicated for the treatment of **Alzheimer's disease** (treatment should be initiated in patients with MCI or mild dementia stage of disease)
- **Dosing:**
  - IV infusion every 2 weeks

Trial Results
- Phase III **CLARITY AD**[1] trial
  - MC, DB (N=1795)
  - **CDR-SB**: adjusted LSM change from baseline at 18 months **1.21** with lecanemab vs **1.66** with placebo (difference, -0.45; 95% CI: -0.67, -55.6; P<0.001)

[1]van Dyck CH et al.NEJM.2023;388:9-21
AD: Alzheimer's disease, CDR-SB: Clinical Dementia Rating-Sum of Boxes, CI: confidence interval, DB: double-blind, IV: intravenous, LSM: least-squares mean, MC: multicenter, MCI: mild cognitive impairment

Central Nervous System
Agents in Development: Alzheimer's Disease (AD)

Donanemab [LY3002813]

Developer(s): Eli Lilly and Company (lead)
Price (estimate): $$

Description/Details
- IV, once-monthly
- **Anti-beta amyloid antibody**

Regulatory Status
- Seeking approval for the treatment of **amyloid-positive early symptomatic AD**
- FDA decision expected **2023** (full approval)

Trial Results
- Phase III **TRAILBLAZER-ALZ 2**[1] trial
  - **Primary outcome**: **35% slowing of decline on iADRS** (p<0.0001)
  - **Secondary outcome**: **35% slowing of decline on CDR-SB** (p<0.0001)

[1]Lilly press release, 5/3/2023
AD: Alzheimer's disease, CDR-SB: Clinical Dementia Rating-Sum of Boxes, iADRS: integrated Alzheimer's Disease Rating Scale, IV: intravenous

Central Nervous System
2023 Approvals: Duchenne Muscular Dystrophy (DMD)

Elevidys® (delandistrogene moxeparvovec-rokl)

Developer(s): Sarepta Therapeutics, Inc. (lead), Roche Holding AG (partner)
FDA approved: 6/22/2023 *(accelerated approval)*
Price: $$$$

Description/Details
- IV, **viral gene therapy (AAV vector)** designed to deliver a micro-dystrophin-encoding gene
- Indicated for the treatment of ambulatory pediatric patients aged 4 through 5 years with **DMD** (with confirmed mutation in DMD gene)
- **Dosing**:
  - Single-dose infusion

Trial Results
- Phase II **Study 102**[1]
  - **Part 1**: randomized, DB, PC (N=41)
  - Mean NSAA total score: **19.8** (vs. 22.6 with placebo)
- Phase Ib **ENDEAVOR trial**[1]
  - OL, MC (N=20)
  - Micro-dystrophin mean change from baseline: **54.2**

[1]Elevidys package insert, 6/2023
AAV: adeno-associated virus, DB: double-blind, DMD: Duchenne muscular dystrophy, IV: intravenous, MC: multi-center, NSAA: North Star Ambulatory Assessment, OL: open-label, PC: placebo-controlled

Central Nervous System
Agents in Development: Duchenne Muscular Dystrophy (DMD)

Fordadistrogene movaparvovec [PF-06939926]

Developer(s): Pfizer, Inc. (lead), Bamboo Therapeutics, Inc. (former)
Price (estimate): $$$$

Description/Details
- IV, **viral (AAV) gene therapy** designed to deliver functional copy of mini-dystrophin gene

Regulatory Status
- In development for the treatment of **DMD**
- Phase III

Trial Results
- Phase III **CIFFREO** - ongoing
- Phase Ib **Single Ascending Dose** trial[1]
  - Treatment group - **N=19**; external control - **N=60**
  - **5.6-point** improvement in **NSAA** at 1 year

[1]Pfizer Fourth Quarter 2021 Earnings Teleconference Presentation, 2/8/2022
AAV: adeno-associated virus, DMD: Duchenne muscular dystrophy, IV: intravenous, NSAA: North Star Ambulatory Assessment

Central Nervous System
Agents in Development: Duchenne Muscular Dystrophy (DMD)

Givinostat [ITF-2357]

Developer(s): Italfarmaco S.p.A. (lead)
Price (estimate): $-$$

Description/Details
- Oral suspension, twice-daily
- **Class I and II histone deacetylase inhibitor**

Regulatory Status
- Seeking approval for the treatment of **DMD**
- Regulatory review; PDUFA = **12/21/2023**

Trial Results
- Phase III **Ambulant Patients (EPIDYS)**[1] trial
  - Treatment with givinostat resulted in a **slower decline** in mean change in time to climb 4 stairs over baseline (difference vs placebo 1.78 seconds, p=0.0345)

[1]Italfarmaco Group press release, 6/25/2022
DMD: Duchenne muscular dystrophy, PDUFA: Prescription Drug User Fee Act

Central Nervous System
Agents in Development: Duchenne Muscular Dystrophy (DMD)

Ataluren (Translarna)
Developer(s): PTC Therapeutics, Inc. (lead), University of Pennsylvania (partner)
Price (estimate): $-$$

- Oral, **protein restoration therapy**
- **Phase III**

Pamrevlumab [FG3019]
Developer(s): FibroGen, Inc. (lead), Bristol Myers Squibb Company (partner)
Price (estimate): $$

- IV, **CTGF inhibitor**
- **Phase III**

Vamorolone [VBP-15]
Developer(s): Santhera Pharmaceuticals (lead), Catalyst Pharmaceuticals, Inc. (partner), Idorsia Pharmaceuticals (partner), Johnson & Johnson (partner), Reveragen BioPharma, Inc. (partner), Sperogenix Therapeutics Limited (partner), Actelion Pharmaceuticals Ltd. (former)
Price (estimate): $-$$

- Oral, **glucocorticoid analogue**
- **Regulatory review**; PDUFA = **10/26/2023**

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CTGF: connective tissue growth factor, IV: intravenous, PDUFA: Prescription Drug User Fee Act

Central Nervous System
2023 Approvals: Friedrich's Ataxia

Skyclarys® (Omaveloxolone)

Developer(s): Biogen, Inc. (lead), AbbVie Inc. (partner), Reata Pharmaceuticals, Inc. (partner)
FDA approved: 2/28/2023
Price: $$-$$$

Description/Details

- Oral, **small molecule** Nrf2 pathway activator
- Indicated for the treatment of **Friedreich's ataxia** in adults and adolescents 16 years and older
- **Dosing:**
  - 3 capsules once daily

Trial Results

- Phase II **MOXIe** trial[1]
  - Randomized, DB, PC (N=103)
  - **Lower mFARS scores** relative to placebo at week 48 (-1.56 vs 0.85 LSM change from baseline; -2.41 treatment difference, 95% CI: -4.32, -0.51, P=0.0138)

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[1]Skyclarys package insert, 2/28/2023
CI: confidence interval, DB: double-blind, LSM: least squares mean, mFARS: modified Friedreich's Ataxia Rating Scale, Nrf2: nuclear erythroid 2-related factor 2, PC: placebo-controlled

Central Nervous System

2023 Approvals: ~~Major Depressive Disorder (MDD)~~/Postpartum Depression

Zurzuvae® (Zuranolone)

Developer(s): Sage Therapeutics, Inc. (lead), Biogen, Inc. (partner), Shionogi & Co. Ltd. (partner)
FDA approved: 8/4/2023
Price: $

Description/Details

- Oral, **small molecule**, neuroactive steroid GABA- A receptor positive modulator
- Indicated for the treatment of **postpartum depression** in adults
- **Dosing:**
  - Oral once daily (in evening) for 14 days

Trial Results

	LSM Change from Baseline	Placebo	Placebo-subtracted difference (95%CI)
SKYLARK	-15.6	-11.6	-4.0 (-6.3, -1.7)
ROBIN	-17.8	-13.6	-4.2 (-6.9, -1.5)

[1]Zurzuvae package insert, 8/4/2023
CI: confidence interval, GABA: gamma-aminobutyric acid, HAMD-1t: 17-item Hamilton depression rating scale, LSM: least squares mean

Central Nervous System

Agents in Development: Major Depressive Disorder (MDD)

Zuranolone [SAGE-217]

Developer(s): Sage Therapeutics, Inc. (lead), Biogen, Inc. (partner), Shionogi & Co. Ltd. (partner)
Price (estimate): $

Description/Details

- Oral, once-daily, short-course
- **Neuroactive steroid**

Regulatory Status

- Seeking approval for the treatment of **MDD**
- FDA declined approval **8/4/2023**

Trial Results

- Conflicting trial results:
  - Phase III **CORAL** trial
  - Phase III **MOUNTAIN** trial
  - Phase III **RAINFOREST** trial
  - Phase III **SHORELINE** trial
  - Phase III **WATERFALL** trial

MDD: major depressive disorder

Central Nervous System
Agents in Development: Major Depressive Disorder (MDD)

Psilocybin [COMP360]

Developer(s): COMPASS Pathways (lead)
Price (estimate): $-$$

Description/Details
- Oral, **synthetic psychedelic (psilocybin)** administered **with psychological support**

Regulatory Status
- In development for the treatment of **MDD**
- Phase III trials

Trial Results
- Phase II **Dose-ranging** trial[1] (N=79)
  - LSM change in **MADRS** from baseline to week 3
    - **−12.0** for 25 mg
    - **−7.9** for 10 mg
    - **−5.4** for 1 mg

[1]Goodwin GM et al, N Engl J Med 2022; 387:1637-1648
LSM: least squares mean, MADRS: Montgomery-Asberg Depression Rating Scale, MDD: major depressive disorder

Central Nervous System
Agents in Development: Major Depressive Disorder (MDD)

Ansofaxine hydrochloride [LY03005]
Developer: Luye Pharma Group, Ltd. (lead)
Price (estimate): $
- Oral, **triple reuptake inhibitor**
- **Regulatory review**; NDA submitted **12/2019**

Aticaprant [CERC-501, JNJ-67953964]
Developer: Johnson & Johnson (lead), Avalo Therapeutics, Inc. (partner), Eli Lilly and Company (partner)
Price (estimate): $
- Oral, **selective KOR antagonist**
- **Phase III**

Dextromethadone [REL-1017]
Developer(s): Relmada Therapeutics, Ind. (lead)
Price (estimate): $
- Oral, **NMDA receptor antagonist**
- **Phase III**

KOR: kappa opioid receptor antagonist, MDD: major depressive disorder, NDA: new drug application, NMDA: N-methyl-D-asparate, PDUFA: Prescription Drug User Fee Act, sNDA: supplemental new drug application

Central Nervous System
Agents in Development: Major Depressive Disorder (MDD)

Lumateperone (Caplyta®)
Developer(s): Intra-Cellular Therapies, Inc. (lead), Bristol Myers Squibb Company (partner)
Price (estimate): $

- Oral, atypical antipsychotic
- **Phase III,** sNDA for adjunctive treatment of MDD expected 2H204

Seltorexant [JNJ-42847922]
Developer: Johnson & Johnson (lead), Minerva Neurosciences, Inc. (partner), Royalty Pharma plc (partner)
Price (estimate): $

- Oral, orexin 2 receptor antagonist
- **Phase III**; NDA possible 2020-2023

Ulotaront [SEP-363856]
Developer(s): Sumitomo Pharma Co., Ltd. (lead), Otsuka Pharmaceutical Co. Ltd. (partner), PsychoGenics Inc. (partner), Sunovion Pharmaceuticals Inc. (Former)
Price (estimate): $

- Oral, TAAR1 agonist
- **Phase III**

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MDD: major depressive disorder, NDA: new drug application, sNDA: supplemental new drug application, TAAR1: trace amine-associated receptor 1

Central Nervous System
Agents in Development: Post-Traumatic Stress Disorder (PTSD)

Midomafetamine (MDMA)-Assisted Therapy

Developer(s): Multidisciplinary Association for Psychedelic Studies (lead)
Price (estimate): $-$$

Description/Details
- Oral, MDMA combined with guided psychotherapy

Regulatory Status
- In development for the treatment of PTSD
- Phase III trials; NDA expected 2H2023

Trial Results
- Phase III MAPP1 and MAPP2[1] trials
  - MDMA-assisted therapy improved PTSD as measured by CAPS-5 Total Severity scores (≥ 6 months after last dose)
  - Effects maintained at 1 year; low incidence of relapse

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[1]MAPS Public Benefit Corporation press release, 4/5/2023
CAPS-5: Clinician-Administered PTSD scale for DSM-5, MDMA: methylenedioxymethamphetamine, NDA: new drug application, PTSD: post-traumatic stress disorder

Central Nervous System
Agents in Development: Schizophrenia

Xanomeline/ Trospium [KarXT]

Developer(s): Karuna Therapeutics, Inc. (lead), PureTech Health plc (partner), Zai Lab Ltd. (partner)
Price (estimate): $

Description/Details
- Oral, twice-daily
- **Muscarinic acetylcholine receptor agonist** and a **muscarinic antagonist**

Regulatory Status
- In development for the treatment of **schizophrenia**
- Phase III trials; NDA expected **3Q2023**

Trial Results
- Phase III **EMERGENT**[1,2] trials (inpatient)
  - **9.6-point ↓ PANSS** vs placebo (-21.2 vs. -11.6, p<0.0001) at Week 5
  - **8.4-point ↓ PANSS** vs placebo (-20.6 vs. -12.2, p<0.0001) at Week 5

[1]Karuna press release, 3/20/2023
[2]Presentation to American Society of Clinical Psychopharmacology, 5/30/2023
NDA: new drug application, PANSS: Positive and Negative Syndrome Scale

Central Nervous System
Agents in Development: Schizophrenia

Brilaroxazine [RP5063]
Developer: Reviva Pharmaceuticals Holdings, Inc. (lead)
Price (estimate): $

- Oral, **atypical antipsychotic**
- **Phase III**

Emraclidine [CVL-231]
Developer: Cerevel Therapeutics Holdings, Inc. (lead)
Price (estimate): $

- Oral, **PAM**
- **Phase III**

Evenamide [NW3509]
Developer(s): Newron Pharmaceuticals S.p.A. (lead)
Price (estimate): $

- Oral, **VGSC modulator**
- **Phase III**

Iclepertin [BI-425809]
Developer(s): Boehringer Ingelheim GmbH (lead)
Price (estimate): $

- Oral, **GlyT1 inhibitor**
- **Phase III**

GlyT1: glycine neurotransmitter transporter, PAM: positive allosteric modulator, VGSC: voltage gated sodium channel

Dermatology

Dermatology
2023 Approvals: Epidermolysis Bullosa

Vyjuvek® (beremagene geperpavec-svdt)

Developer(s): Krystal Biotech, Inc. (lead)
FDA approved: 5/19/2023
Price: $$$

Description/Details

- Topical, **gene therapy (viral)**
- Indicated for treatment of wounds in patients 6 months of age and older with **DEB** with mutation(s) in the COL7A1 gene
- **Dosing:**
  - Apply once weekly until wounds close

Trial Results

- Phase III **GEM-3** trial[1]
  - RCT, DB, intra-subject PC (N=31)

	Wk. 22/24 or 24/26	Wk. 8/10 or 10/12
Complete wound closure (b-vec)	20 (65%)	21 (68%)
Complete wound closure (placebo)	8 (26%)	7 (23%)
Treatment difference	39% (p=0.012)	45% (p=0.003)

ICER

[1]Vyjuvek package insert, 5/19/2023

COL7A1: collagen type VII alpha 1 chain, DEB: dystrophic epidermolysis bullosa, Wk: week

Dermatology
Agents in Development: Epidermolysis Bullosa

Dabocemagene autoficel ("D-Fi") [FCX007]

Developer(s): Castle Creek Biosciences, Inc. (lead), Fibrocell Science, Inc. (partner), Precigen, Inc. (partner)
Price (estimate): $$$

Description/Details
- Topical/intradermal, ex-vivo, autologous, cell-based **viral (lentivirus vector) gene therapy**

Regulatory Status
- In development for the treatment of **recessive dystrophic epidermolysis bullosa**
- Phase III

Trial Results
- Phase I/II **RDEB** trial[1] (N=6)
  - **80% (8/10)** of treated chronic wounds achieved **complete wound healing** at 12 weeks

[1]Castle Creek Biosciences press release 2019 (accessed via Citeline's Biomedtracker)
RDEB: recessive dystrophic epidermolysis bullosa, RMAT: regenerative medicine advanced therapy

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Dermatology
Agents in Development: Epidermolysis Bullosa

EB101 gene-corrected cell therapy

Developer(s): Abeona Therapeutics Inc. (lead)
Price (estimate): $$$

Description/Details
- Topical/intradermal, ex-vivo, autologous, **viral (retrovirus vector) gene-corrected skin grafts**

Regulatory Status
- In development for the treatment of **recessive dystrophic epidermolysis bullosa**
- Phase III; BLA submitted **9/26/2023**

Trial Results
- Phase III **VIITAL** trial[1]
  - More wounds with **≥50% healing at 6 months** (81.4% vs 16.3% with placebo)

[1]Abeona Therapeutics Press Release, 5/11/2023
BLA: biologic license application

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Endocrine/Metabolic

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Endocrine/Metabolic
2023 Approvals*

Brenzavvy® (Bexagliflozin)
Developer(s): TheracosBio,LLC (lead)
FDA approved: 1/20/2023
Price: $

- Oral, **small molecule**
- Indicated to improve glycemic control in adults with **type 2 diabetes mellitus** as an adjunct to diet and exercise

Elfabrio® (Pegunigalsidase alfa)
Developer(s): Chiesi Farmaceutici S.p.A. (lead), Protalix BioTherapeutics, Inc. (partner)
FDA approved: 5/9/2023
Price: $$

- IV, **biologic**
- Indicated for the treatment of **Fabry Disease**

Lamzede® (Velmanse alfa-tycv)
Developer(s): Chiesi Farmaceutici S.p.A. (lead), Zymenex A/S (partner)
FDA approved: 2/16/2023
Price: $$$

- IV, **biologic**
- Indicated for the treatment of non-central nervous system manifestations of **alpha-mannosidosis**

Ngenla® (Somatrogon-ghla)
Developer(s): Opko Health (lead), Pfizer Inc. (partner), Prolor Biotech Ltd. (former)
FDA approved: 6/27/2023
Price: $

- SC, **biologic**
- Indicated to treat **growth failure** due to inadequate secretion of endogenous growth hormone

ICER

*current as of 9/27/2023
IV: intravenous, SC: subcutaneous

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Endocrine/Metabolic

Agents in Development: Metachromatic Leukodystrophy (MLD)

Atidarsagene autotemcel ("arsa-cel"), [OTL-200]

Developer(s): Orchard Therapeutics Limited (lead), AGC Therapeutics Limited (partner), GSK plc (partner), AGC Biologics S.p.A. (former)
Price (estimate): $$$$

Description/Details

- IV, ex-vivo, **viral (lentivirus vector) gene therapy** designed to deliver ARSA gene

Regulatory Status

- Seeking approval for **early-onset MLD**
- Regulatory review; FDA decision expected by **3/18/2024**

Trial Results

- Phase II **Italy** trial[1]
  - Significant and clinically meaningful improvement in sMFS vs. disease natural history pre-symptomatic late infantile (p<0.001), pre-symptomatic early juvenile (p=0.042), early-symptomatic early juvenile (p<0.001), MLD subgroups compared to disease natural history)

[1]Orchard Therapeutics Press Release, 8/31/2023
ARSA: arylsulfatase-A, BLA: biologic license application, IV: intravenous, MLD: metachromatic leukodystrophy

Endocrine/Metabolic

Agents in Development: Non-alcoholic Steatohepatitis (NASH)*

Resmetirom [MGL-3196]

Developer(s): Madrigal Pharmaceuticals, Inc. (lead), Roche Holding AG (partner), Synta Pharmaceuticals Corp. (former)
Price (estimate): $

Description/Details

- Oral, once-daily
- **β-selective thyroid hormone agonist**

Regulatory Status

- Seeking approval for **NASH with liver fibrosis**
- Regulatory review; PDUFA = **3/14/2024**

Trial Results

- Phase III **MAESTRO-NASH** trial[1]

Primary Endpoint	Res. 80 mg	Res. 100 mg	Pbo. (n=318)
NASH Resolution with ≥2-point reduction in NAS and no worsening of Fibrosis	**26%** (p<0.0001)	**30%** (p<0.0001)	10%
≥1-stage improvement in fibrosis with no worsening NAS	**24%** (p=0.0002)	**26%** (p<0.0001)	14%

[1]Madrigal Press release, 6/22/2023
*also known as MASH: Metabolic Dysfunction-Associated Steatohepatitis
NAS: NAFLD Activity Score, NASH: non-alcoholic steatohepatitis, NDA: new drug application, PDUFA: Prescription Drug User Fee Act

Endocrine/Metabolic

Agents in Development: Transthyretin Amyloid Cardiomyopathy

Acoramidis
Developer(s): BridgeBio Pharma, Inc. (lead), AstraZeneca PLC (partner), Alexion Pharmaceuticals Inc. (former), Eidos Therapeutics, Inc. (former)
Price (estimate): $$

- Oral, **tetrameric TTR stabilizer**
- **Phase III;** NDA expected **2023**

Eplontersen [IONIS-TTR-LRx]
Developer: Ionis Pharmaceuticals, Inc. (lead), Akcea Therapeutics, Inc. (partner), AstraZeneca PLC (partner)
Price (estimate): $$

- SC, **LICA targeting TTR**
- **Phase III;** sNDA for **ATTR-CM** expected **2024**

Patisiran (Onpattro®)
Developer(s): Alnylam Pharmaceuticals Inc. (lead), Arbutus Biopharma Corporation (partner), GENESIS Pharma S.A. (partner), Ionis Pharmaceuticals Inc. (partner), Medison Pharma Ltd. (partner), Sanofi (partner), taiba-ME (partner)
Price (estimate): $$

- IV, **anti-TTR siRNA**
- **Regulatory review;** PDUFA for sNDA **10/8/2023**

Vutrisiran (Amvuttra®)
Developer: Alnylam Pharmaceuticals Inc. (lead), Sanofi (partner)
Price (estimate): $$

- SC, **anti-TTR siRNA**
- **Phase III; (**for expanded approval)

ATTR-CM: transthyretin amyloid cardiomyopathy, IV: intravenous, LICA ligand-conjugated antisense drug, NDA: new drug application, PDUFA: Prescription Drug User Fee Act, SC: subcutaneous, siRNA: small interfering ribonucleic, sNDA: supplemental new drug application, TTR: transthyretin

Endocrine/Metabolic

2023 Approvals: Type 1 Diabetes

Lantidra® (donislecel-jujn)

Developer(s): CellTrans, Inc. (lead)
FDA approved: 6/28/2023
Price: $$

Description/Details

- IV, **allogeneic pancreatic islet cell therapy**
- Indicated to treat adults with **T1DM unable to approach target HbA1c** due to repeated episodes of severe hypoglycemia despite intensive diabetes management and education*
- **Dosing:**
  - Initial infusion with option for second infusion 1 year after 1st, and option for 3rd infusion 1 year after 2nd

Trial Results

	Mean (max, min)
Study 1 (OL, SA, N=10)	5.1 (0.2, 12.8)
Study 2 (OL, SA, N=20)	3.2 (0, 9.9)

*to be used in conjunction with concomitant immunosuppression
[1]**Lantidra package insert, 6/28/2023**
HbA1c: hemoglobin A1c, IV: intravenous, OL: open label, SA: single-arm, T1DM: type 1 diabetes

Endocrine/Metabolic
Agents in Development: Weight Management

Tirzepatide

Developer(s): Eli Lilly and Company (lead), Mitsubishi Tanabe Pharma Corporation (partner)
Price (estimate): $

Description/Details
- SC, once-weekly
- **GLP-1/GIP agonist**

Regulatory Status
- Seeking approval* for the treatment of **adults with obesity/overweight**
- Regulatory review; FDA decision expected **2023**

Trial Results
- Phase III **SURMOUNT-1** trial[1]
  - Weight ↓ **15.0%** (5 mg), **19.5%** (10 mg) and **20.9%** (15 mg), vs **3.1%** with placebo

[1]**Jastreboff AM et al.NEJM.2022;387:205-216**
*Unclear if will be expanded approval or separate branded product
GIP: glucose-dependent insulinotropic polypeptide, GLP-1: glucagon-like peptide-1, SC: subcutaneous

Endocrine/Metabolic
Agents in Development: Weight Management

Cagrilintide + Semaglutide (CagriSema) **Developer(s):** Novo Nordisk A/S (lead) **Price (estimate):** $	• SC, **Amylin and GLP-1 analogue** • **Phase III**
Orforglipron [LY3502970] **Developer:** Eli Lilly and Company (lead), Chugai Pharmaceutical Co., Ltd. (partner) **Price (estimate):** $	• Oral, **GLP-1 agonist** • **Phase III**
Retatutide [LY3437943] **Developer:** Eli Lilly and Company (lead) **Price (estimate):** $	• SC, **GLP-1/GIP/glucagon agonist** • **Phase III**
Semaglutide (Rybelsus®) **Developer(s):** Novo Nordisk A/S (lead), Emisphere Technologies, Inc. (partner), Merck & Co. (partner) **Price (estimate):** $	• Oral, **GLP-1 agonist** • **Phase III;** sNDA expected **2023**

GIP: glucose-dependent insulinotropic polypeptide receptor, GLP-1: glucagon-like peptide-1 receptor, sNDA: supplemental new drug application,

Hematology/~~Oncology~~

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Hematology
2023 Approvals: Hemophilia A

Roctavian®
(valoctocogene roxaparvovec-rvox)

Developer(s): BioMarin Pharmaceutical Inc. (lead), St. Jude Children's Research Hospital (partner), University College London (partner)
FDA approved: 6/20/2023
Price: $$$$

Description/Details

- IV, **gene therapy (viral)**
- Indicated for the treatment of adults with **severe hemophilia A** (congenital factor VIII deficiency) without pre-existing antibodies to AAV serotype 5
- **Dosing:**
  - Single-dose infusion

Trial Results

- Phase III **GENEr8-1** trial[1]
  - Reduced **Mean ABR** (bleeds/year): **2.6** (vs. 5.4 at baseline)
  - Reduced **Median ABR** (bleeds/year): **0.3** (vs. 3.3 at baseline)

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[1]Roctavian package insert, 6/20/2023
AAV: adeno-associated virus, ABR: annualized bleeding rate, IV: intravenous

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Hematology
Agents in Development: Beta Thalassemia

Exagamglogene Autotemcel ("exa-cel") [CTX001]

Developer(s): Vertex Pharmaceuticals Incorporated (lead), Anagenesis Biotechnologies (partner), Bayer AG (partner), CRISPR Therapeutics AG (partner)
Price (estimate): $$$$

Description/Details
- IV, ex-vivo, CRISPR-Cas9 **gene therapy** designed to reduce levels of BCL11A

Regulatory Status
- Seeking approval for the treatment of **transfusion-dependent beta thalassemia**
- FDA decision expected by **3/30/2024**

Trial Results
- Phase I/II/II **CLIMB THAL-111** trial[1]
  - **88.9%** of patients achieved transfusion-independence for at least 12 consecutive months
  - Mean duration of transfusion-independence = **20.5 months**, max. duration = **40.7 months**

[1]CRISPR Press Release, 6/9/2023
Cas9: CRISPR-associated protein, CRISPR: Clustered Regularly Interspaced Short Palindromic Repeats, IV: intravenous

Hematology
Agents in Development: Hemophilia A

Giroctocogene fitelparvovec [PF-07055480]

Developer(s): Pfizer Inc. (lead), Sangamo Therapeutics, Inc.
Price (estimate): $$$$

Description/Details
- IV, ex-vivo, **viral (rAAV) gene therapy** designed to deliver a copy of factor VIII gene

Regulatory Status
- In development for the treatment of **moderate to severe hemophilia A**
- Phase III; BLA filing planned **2H2024**

Trial Results
- Phase III **AFFINE** trial - ongoing
- Phase I/II **Alta (study 1603)** trial[1]
  - Mean **ABR = 0** for 1st year post-infusion
  - Mean **ABR = 1.4** (n=5 patients with ≥2 years follow-up)

[1]Presentation to Annual Meeting and Exposition of the American Society of Hematology, 12/2021
ABR: annualized bleeding rate, BLA: biologic license application, IV: intravenous, rAAV: recombinant adeno-associated virus

Hematology
Agents in Development: Hemophilia B

Fidanacogene elaparvovec [SPK-9001]

Developer(s): Pfizer Inc. (lead), Roche Holding AG (partner), Spark Therapeutics, Inc. (former)
Price (estimate): $$$$

Description/Details
- IV, ex-vivo, **viral (AAV) gene therapy** designed to deliver a copy of the factor IX gene

Regulatory Status
- Seeking approval for the treatment of **hemophilia B**
- FDA decision expected **2Q2024**

Trial Results
- Phase III **BENEGENE-2** trial[1]
  - **71%** reduction in mean ABR vs FIX prophylaxis (1.3 vs. 4.43, p<0.0001)

[1]Pfizer Press Release, 12/29/2022
ABR: annualized bleed rate, FIX: factor IX, IV: intravenous

Hematology
Agents in Development: Sickle Cell Disease (SCD)

Exagamglogene Autotemcel ("exa-cel") [CTX001]

Developer(s): Vertex Pharmaceuticals Incorporated (lead), Anagenesis Biotechnologies (partner), Bayer AG (partner), CRISPR Therapeutics AG (partner)
Price (estimate): $$$$

Description/Details
- IV, ex-vivo, CRISPR-Cas9 **gene therapy** designed to reduce levels of BCL11A

Regulatory Status
- Seeking approval for treatment of **severe SCD**
- FDA decision expected by **12/8/2023**

Trial Results
- Phase I/II/III **CLIMB-SCD-121** trial[1]
  - **94.1%** were VOC free for ≥ 12 consecutive months (95% CI: 71.3%, 99.9%; p=0.0001)
  - **100%** free from VOC-related hospitalizations for ≥12 consecutive months (95% CI: 80.5%, 100.0%; p<0.0001)

[1]CRISPR Press Release, 6/9/2023
Cas9: CRISPR-associated protein, CI: confidence interval, CRISPR: Clustered Regularly Interspaced Short Palindromic Repeats, IV: intravenous, SCD: sickle cell disease, VOC: vaso-occlusive crisis

Hematology
Agents in Development: Sickle Cell Disease (SCD)

Lovotibeglogene autotemcel ("Lovo cel") (LentiGlobin)

Developer(s): bluebird bio (lead)
Price (estimate): $$$$

Description/Details
- IV, ex-vivo, **viral (lentiviral vector) gene therapy** designed to deliver modified β-globin gene

Regulatory Status
- Seeking approval for the treatment of **SCD**
- FDA decision expected **12/20/2023**

Trial Results
- Phase I/II **HGB-206** trial[1]
  - **96%** were sVOE free through 24 months of follow-up
  - A single sVOE occurred in one patient experiencing persistent anemia

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[1]**bluebird bio press release, 12/10/2022**
IV: intravenous, SCD: sickle cell disease, sVOE: severe vaso-occlusive events

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Immunology

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Immunology
2023 Approvals*

Filspari® (Sparsentan)
Developer(s): Travere Therapeutics, Inc. (Lead), Bristol Myers Squibb Company (Partner), CSL Vifor (Partner), Ligand Pharmaceuticals, Inc. (Partner)
FDA approved: 2/17/2023
Price: $$

- Oral, **small molecule**
- Indicated to reduce proteinuria in adults with **primary immunoglobulin A nephropathy** at risk of rapid disease progression

Litfulo® (Ritlecitinib)
Developer(s): Pfizer Inc. (Lead)
FDA approved: 6/23/2023
Price: $

- Oral, **small molecule**
- Indicated for the treatment of **severe alopecia areata** in adults and adolescents 12 years and older

Veopoz® (Pozelimab-bbfg)
Developer(s): Regeneron Pharmaceuticals, Inc. (lead), Alnylam Pharmaceuticals Inc. (partner)
FDA approved: 8/18/2023
Price: $$?

- IV/SC, **biologic**
- Indicated to treat patients 1 year old and older with **CD55-deficient protein-losing enteropathy (PLE)**, also known as **CHAPLE disease**

*current as of 9/27/2023
IV: intravenous, SC: subcutaneous

Immunology
Agents in Development: Leukocyte Adhesion Deficiency-I (LAD-1)

RP-L201

Developer(s): Rocket Pharmaceuticals Inc. (lead)
Price (estimate): $$$$

Description/Details
- IV, ex-vivo, **viral (lentivirus) gene therapy** designed to deliver ITGB2 gene

Regulatory Status
- Seeking approval for the treatment of **LAD-1**
- BLA submitted **8/10/2023**

Trial Results
- Phase I/II **LAD-1** trial[1]
  - **100% OS** at 12 months post-infusion (9 patients with 12-24 months of available follow-up)
  - Evidence of resolution of LAD-I-related skin rash and restoration of wound repair capabilities

[1]Rocket Pharmaceuticals Press Release, 5/19/2023
BLA: biologic license application, IV: intravenous

Immunology
2023 Approvals: Myasthenia Gravis

Rystiggo® (Rozanolixizumab-noli)

Developer(s): UCB S.A. (lead)
FDA approved: 6/26/2023
Price: $$

Description/Details
- SC infusion, **anti-FcRn antibody**
- Indicated for the treatment of **generalized myasthenia gravis** in adults who are anti-AChR or anti-MuSK antibody positive
- **Dosing**:
  - Treatment cycle: weekly SC infusion for 6 weeks

Trial Results
- Phase III **MG0003** trial[1]
  - RCT, MC, DB (N=200)

	Roz. 7mg/kg	Roz. 10mg/kg
MG-ADL total score (LSM)	-3.4	-3.4
Difference vs. placebo (95% CI)	-2.6 (-4.1, -1.2)	-2.6 (-4.1, -1.2)
QMG total score (LSM)	-5.4	-6.7
Difference vs. placebo (95% CI)	-3.5 (-5.6, -1.6)	-4.8 (-6.8, -2.9)

[1]Rystiggo package insert, 6/26/2023
AChR: anti-acetylcholine receptor, DB: double-blind, FcRn: neonatal Fc receptor, MuSK: muscle-specific tyrosine kinase, PC: placebo-controlled, RCT: randomized controlled trial, Roz. Rozanolixizumab

Immunology
Agents in Development: Myasthenia Gravis

Zilucoplan [RA101495SC]

Developer(s): UCB S.A (lead), Ra Pharmaceuticals, Inc. (former)
Price (estimate): $$

Description/Details
- SC, once-daily
- **Complement (C5) inhibitor**

Regulatory Status
- Seeking approval for the treatment of **myasthenia gravis**
- Regulatory review; FDA decision expected **2H2023**

Trial Results
- Phase III **RAISE** trial[1]
  - Treatment with zilucoplan resulted in **higher responder rates**…
    - MG-ADL: 73% vs. 46%, p<0.001
    - QMG: 58% vs 33%, p=0.0012

[1]UCB press release, 6/30/2023
MG-ADL: myasthenia gravis activities of daily living, QMG: quantitative myasthenia gravis, SC: subcutaneous

Immunology
Agents in Development: Paroxysmal Nocturnal Hemoglobinuria (PNH)

Crovalimab [RG-6107, CH-7092230]
Developer(s): Roche Holding AG (lead), Chugai Pharmaceutical Co., Ltd. (partner)
Price (estimate): $$

- IV/SC, **complement (C5) inhibitor**
- **Regulatory review;** BLA submitted **7/2023**

Danicopan [ALXN2040]
Developer(s): AstraZeneca PLC (lead), Achillion Pharmaceuticals, Inc. (former), Alexion Pharmaceuticals Inc. (former)
Price (estimate): $$

- Oral, **factor D inhibitor**
- **Regulatory review;** NDA submitted for PNH with EVH **2Q2023**

Iptacopan [LNP023]
Developer(s): Novartis AG (lead)
Price (estimate): $$

- Oral, **factor B inhibitor**
- **Regulatory review;** NDA submitted **2Q2023**

BLA: biologic license application, EVH: extravascular hemolysis, NDA: new drug application, PNH: paroxysmal nocturnal hemoglobinuria

Immunology
Agents in Development: Psoriasis

Bimekizumab (Bimzelx)

Developer(s): UCB S.A. (lead)
Price (estimate): $

Description/Details
- SC, every 8 weeks
- **Anti-IL17a and IL-17F**

Regulatory Status
- Seeking approval for the treatment of **moderate to severe plaque psoriasis**
- Regulatory review; FDA decision expected **3Q2023**

Trial Results
- Phase III BE trials
  - **BE SURE**[1]: 86.2% PASI 90 at week 16 vs 47.2% with adalimumab Humira® (p<0.001)
  - **BE VIVID**[2]: 85% PASI 90 at week 16 vs 50% ustekinumab (Stelara®)

[1]Warren et al. *NEJM*.2021;385:130-141., [2]Reich et al. *Lancet*.2021;397(10273):487-498.
IL: interleukin, PASI 90/100: 90/100% improvement in Psoriasis Area and Severity index, SC: subcutaneous

Infectious Diseases

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Infectious Diseases
2023 Approvals*

Abrysvo® (RSV vaccine)
Developer(s): Pfizer In. (lead)
FDA approved: 5/31/2023
Price: $

- IM, **vaccine**
- Indicated for the **prevention of LRTD caused by RSV** in adults ≥60 years and infants from birth through 6 months when administered during pregnancy

Arexvy® (RSV vaccine, adjuvanted)
Developer(s): GSK plc (lead), Agenus Inc. (partner)
FDA approved: 5/3/2023
Price: $

- IM, **vaccine**
- Indicated for the **prevention of LRTD caused by RSV** in adults ≥60 years

Cyfendus® (Anthrax vaccine adsorbed, adjuvanted)
Developer(s): Emergent BioSolutions (lead)
FDA approved: 7/20/2023
Price: ?

- IM, **vaccine**
- Indicated for **post-exposure prophylaxis** of disease following exposure to ***Bacillus anthracis*** in persons 18-65 years in conjunction with recommended antibacterial drugs

Paxlovid® (Nirmatrelvir, ritonavir)
Developer(s): Pfizer Inc. (lead)
FDA approved: 5/25/2023
Price: ?

- Oral, **small molecule**
- Indicated for the **treatment of mild-to-moderate COVID-19** in adults at high risk for progression to severe COVID-19, including hospitalization and death

ICER

*current as of 9/27/2023
COVID-19: coronavirus disease 2019, IM: intramuscular, LRTD: lower respiratory tract disease, RSV: respiratory syncytial virus

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Infectious Diseases
2023 Approvals*

Rezzayo® (Rezafungin)
Developer(s): Melinta Therapeutics, Inc. (lead), Cidara Therapeutics, Inc. (partner), Mundipharma International Limited (partner)
FDA approved: 3/22/2023
Price: $

- IV, **small molecule**
- Indicated for the treatment of patients ≥ 18 years of age who have limited or no alternative options for the treatment of **candidemia and invasive candidiasis**

Vowst® (fecal microbiota spores)
Developer(s): Seres Therapeutics, Inc. (lead), Aimmune Therapeutics, Inc. (partner), Nestle Health Science (partner)
FDA approved: 4/26/2023
Price: $

- Oral, **microbiome product**
- Indicated to **prevent the recurrence of *Clostridioides difficile* infection (CDI)** in individuals ≥ 18 years of age following antibacterial treatment for recurrent CDI

Xacduro® (sulbactam, durlobactam)
Developer(s): Innoviva, Inc. (lead), Zai Lab Ltd. (partner), Entasis Therapeutics Holdings (former)
FDA approved: 5/23/2023
Price: $

- IV, **small molecule**
- Indicated for patients ≥ 18 years of age for the treatment of **HABP and VABP** caused by susceptible isolates of *Acinetobacter baumannii-calcoaceticus* complex

*current as of 9/27/2023
HABP: hospital-acquired bacterial pneumonia, IV: intravenous, VABP: ventilator-associated bacterial pneumonia

Infectious Diseases
2023 Approvals: Respiratory Syncytial Virus (RSV)

Beyfortus® (Nirsevimab-alip)

Developer(s): AstraZeneca PLC (lead), AIMM Therapeutics B.V. (partner), Sanofi (partner)
FDA approved: 7/17/2023
Price: $

Description/Details

- IM, **IgG1 RSV-preF antibody**
- Indicated for prevention of **RSV LRTD** in neonates and infants born during 1st RSV season and children ≤ 24 months vulnerable to severe disease through 2nd season
- **Dosing**:
  - Single IM dose per RSV season

Trial Results

	Incidence (nirsevimab)	Incidence (placebo)	Efficacy*
Healthy Preterm Infants	2.6%	9.5%	70.1% (95% CI: 52.3, 81.2)
MELODY	1.2%	5.0%	74.9% (95% CI: 50.6,87.3)

[1]Beyfortus package insert, 7/17/2023
CI: confidence interval, IgG: immunoglobulin, IM: intramuscular, LRTD: lower respiratory tract disease, RSV: respiratory syncytial virus, RSV-preF: respiratory syncytial virus protein-directed fusion

Other

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Other
2023 Approvals*

Izervay® (avacincaptad pegol)
Developer(s): Astellas Pharma, Inc. (lead), Archemix Corp. (partner), DelSiTech Ltd. (partner), Iveric Bio, Inc. (former)
FDA approved: 8/4/2023
Price: $

- Intravitreal, **oligonucleotide**
- Complement inhibitor indicated for the treatment of **geographic atrophy** secondary to **age-related macular degeneration**

Jesduvroq® (daprodustat)
Developer(s): GSK plc (lead), Kyowa Kirin Co., Ltd (partner)
FDA approved: 2/1/2023
Price: $

- Oral, **small molecule**
- Hypoxia-inducible factor prolyl hydroxylase inhibitor indicated for the treatment of **anemia due to CKD** in adults receiving dialysis for at least 4 months

Miebo® (perfluorhexyloctane)
Developer(s): Bausch Health Companies Inc. (lead), Novaliq GmbH (partner), Senju Pharmaceutical Co., Ltd (partner)
FDA approved: 5/18/2023
Price: $

- Topical (ocular), **small molecule**
- Indicated for the treatment of signs and symptoms of **dry eye disease**

ICER

*current as of 9/27/2023
CKD: chronic kidney disease

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Other
2023 Approvals*

Sohonos® (palovarotene)
Developer(s): Ipsen SA (lead), Roche Holding AG (partner), Clementia Pharmaceuticals, Inc. (former)
FDA approved: 8/16/2023
Price: ?

- Oral, **small molecule**
- Retinoid indicated for reduction in the volume of new heterotopic ossification in adults and children ≥ 8 years for females and ≥ 10 years for males with **fibrodysplasia ossificans progressive**

Veozah® (fezolinetant)
Developer(s): Astellas Pharma, Inc. (lead), Ogeda S.A. (former)
FDA approved: 5/12/2023
Price: $

- Oral, **small molecule**
- Neurokinin 3 receptor antagonist indicated for the treatment of moderate to severe **vasomotor symptoms due to menopause**

Xdemvy® (lotilaner)
Developer(s): Tarsus Pharmaceuticals Inc. (lead), LianBio (partner)
FDA approved: 7/25/2023
Price: $

- Topical (ocular), **small molecule**
- Anti-parasitic indicated for the treatment of **Demodex blepharitis**

*current as of 9/27/2023

Bringing It All Together

Pipeline Preview 2023
Bringing it all together

Where do we go from here?

Total Approvals likely to surpass 2022…
- 2023 will likely see more novel approvals vs. last year
- Launch prices will (likely) continue to increase

Keep your eyes on…
- **Cell and gene therapies:** space is growing rapidly – unanswered questions remain
- **Obesity management:** expect growth in this space for some time
- **Psychedelics:** with first product in this space nearing FDA approval, expect this to be a hot topic for the year to come

Questions?

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Notes

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