New Drug Update 2024: CNS, Behavioral Health, Musculoskeletal, Immunology, Oncology, Ocular

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April 5-7, 2024

OHIO PHARMACISTS ASSOCIATION

1 VOICE VISION TEAM

Disclosure Statement

- Karen L. Kier has no relevant financial relationship(s) with ineligible companies to disclose.

  and

- None of the planners for this activity have relevant financial relationships with ineligible companies to disclose.

Learning Objectives

At the completion of this activity, the participant will be able to:

1. review the pharmacology and therapeutics of selected prescription medications released to the market within the past year;

2. state the indications and clinical applications of the medications presented, and how they compare to current therapies;

3. list the most common adverse effects, toxicities, and significant drug-drug and drug-food interactions reported; and

4. explain important patient/caregiver counseling information for these medications.

MUSCULOSKELETAL

denosumab-bbdz (Jubbonti) Injection

March 5, 2024 Sandoz
Osteoporosis

- RANK ligand (RANKL) inhibitor interchangeable biosimilar to Prolia (denosumab) used in osteoporosis
- primary and secondary bone loss
- 60mg/mL (1mL prefilled syringe or vial)
- 60 mg SC q 6 months
- Upper arm, upper thigh, or abdomen
- 1000mg calcium and 400IU vitamin D per day

palovarotene (Sohonos) Capsules

August 16, 2023 Ipsen
Fibrodysplasia Ossificans Progressiva

- retinoid for the reduction in volume of new heterotopic ossification in adults and pediatrics aged 8 and older for females and 10 years and older for males with fibrodysplasia ossificans progressiva (FOP)
- very rare genetic connective tissue disorder characterized by the abnormal development of bone in areas of the body where bone is not normally present
- impacts 400 people in the U.S. and 900 people globally
- 5 mg once daily
- flares: 20 mg once daily for 4 weeks followed by 10 mg once daily for 8 weeks for a total of 12 weeks
- only dosage form is a 1 mg capsule
- BBW: contraindicated in pregnancy and may cause fetal harm
- significant drug-drug interactions: CYP 3A4 substrate

tocilizumab-bavi (Tofidence) Injection

September 29, 2023 Biogen
Rheumatoid Arthritis, Polyarticular Juvenile Idiopathic Arthritis, Juvenile Idiopathic Arthritis

- interleukin-6 (IL-6) receptor antagonist biosimilar to Actemra for treatment of moderately to severely active rheumatoid arthritis, polyarticular juvenile idiopathic arthritis and systemic juvenile idiopathic arthritis
- first tocilizumab biosimilar approved in the United States
- IV formulation only
- ? availability

tocilizumab-aazg (Tyenne) Injection

March 7, 2024 Fresenius Kabi USA
Rheumatoid Arthritis, Giant Cell Arteritis, Polyarticular Juvenile Idiopathic Arthritis, Juvenile Idiopathic Arthritis

- interleukin-6 (IL-6) receptor antagonist biosimilar to Actemra
- RA, giant cell arteritis, polyarticular juvenile idiopathic arthritis, and systemic juvenile idiopathic arthritis
- first tocilizumab biosimilar with both IV and subcutaneous formulations approved by the FDA
- globally in more than 10 countries
- ? availability

adalimumab-aaty (Yuflyma) Injection

May 23, 2023 Celltrion USA

Rheumatoid arthritis, juvenile idiopathic arthritis, psoriatic arthritis, ankylosing spondylitis, Crohn's disease, ulcerative colitis, plaque psoriasis, and hidradenitis suppurativa

- tumor necrosis factor (TNF) blocker biosimilar to Humira
- treatment of RA, juvenile idiopathic arthritis, psoriatic arthritis, ankylosing spondylitis, Crohn’s disease, ulcerative colitis, plaque psoriasis, hidradenitis suppurativa, and uveitis
- high-concentration (100mg/mL) and citrate-free formulation
- pre-filled syringe and autoinjector
- company is also seeking an interchangeability designation

adalimumab-ryvk (Simlandi) Injection

February 23, 2024 Alvotech and Teva Pharmaceutical Industries
Rheumatoid Arthritis, Juvenile Idiopathic Arthritis, Psoriatic Arthritis, Ankylosing Spondylitis, Crohn's Disease, Ulcerative Colitis, Plaque Psoriasis, Hidradenitis Suppurativa, Uveitis

- tumor necrosis factor (TNF) blocker interchangeable biosimilar to Humira
- first high-concentration, citrate-free biosimilar interchangeable biosimilar
- 40mg/0.4ml dose
- 88% of Humira scripts in US are for the high dose
- approved for RA, juvenile idiopathic arthritis, psoriatic arthritis, ankylosing spondylitis, Crohn’s disease, ulcerative colitis, plaque psoriasis, hidradenitis suppurativa, and uveitis
- Phase 3 trials of Simlandi were in chronic plaque psoriasis

tofersen (Qalsody) Injection

April 25, 2023 Biogen

Amyotrophic Lateral Sclerosis

- antisense oligonucleotide for amyotrophic lateral sclerosis (ALS) in adults with a superoxide dismutase 1 (SOD1) gene mutation
- first treatment to target a genetic cause of ALS
- accelerated approval based on reduction in plasma neurofilament light chain (surrogate marker)
- ongoing Phase 3 ATLAS study for confirmation, this study is of those with mutation but no symptoms of ALS
- 28-week randomized, double-blind, placebo-controlled clinical study VALOR in those with ALS symptoms (tofersen could be added to existing therapies), slower decline than placebo group
- loading dose: Intrathecal: 100 mg every 14 days for 3 doses
- maintenance dose: Intrathecal: 100 mg every 28 days, starting 28 days following 3rd loading dose
- 100 mg/15mL injection, refrigerated, protect from light
- ADRs: serious neurologic events including myelitis, papilledema and elevated intracranial pressure, and aseptic meningitis

delandistrogene moxeparvovec-rokl (Elevidys) Suspension for Intravenous Infusion

June 22, 2023 Sarepta Therapeutics

Duchenne Muscular Dystrophy

- adeno-associated virus vector-based gene therapy indicated for ambulatory pediatric patients aged 4 through 5 years with Duchenne muscular dystrophy (DMD) with a confirmed mutation in the DMD gene
- accelerated approval
- contraindicated in patients with any deletion in exon 8 and/or exon 9 in the *DMD* gene
- single-dose gene transfer therapy

vamorolone (Agamree) Oral Suspension

October 26, 2023
Duchenne Muscular Dystrophy

Catalyst Pharmaceuticals

- corticosteroid for Duchenne muscular dystrophy in patients 2 years of age and older
- novel corticosteroid, reduction in adverse events, related to bone health, growth trajectory, and behavior
- 2 to 6 mg/kg/day, extending for a period of up to 48 months in clinical trials
- FDA approved dose: 6 mg/kg once daily with a meal; may decrease to 2 mg/kg once daily based on tolerability
- maximum of 300 mg/day
- oral suspension 40 mg/mL, 100 mL bottle
- orange flavored

givinostat (Duvyzat) oral suspension

March 21, 2024
Duchenne Muscular Dystrophy

Italfarmaco Group

- histone deacetylase inhibitor for Duchenne muscular dystrophy in 6 years and older
- priority review, orphan drug and rare pediatric disease designations
- novel mechanism of action
- multicentre, randomised, double-blind, placebo-controlled phase 3 EPIDYS trial
- total of 179 ambulant boys six years of age or older received either Duvyzat twice daily or placebo
- statistically significant and clinically meaningful difference in time to complete the four-stair climb assessment (surrogate endpoint)
- 10mg/mL suspension administered orally as 2 oral doses daily on a full stomach
- ADRs: Diarrhea, increase lipid levels, lowers platelet count

CNS

eplontersen (Wainua) Injection

December 21, 2023 Ionis Pharmaceuticals and AstraZeneca
Hereditary Transthyretin-Mediated Amyloid Polyneuropathy (ATTRv-PN)

- transthyretin-directed antisense oligonucleotide for treatment of the polyneuropathy of hereditary transthyretin-mediated amyloidosis in adults
- rare disease due to mutations in the gene encoding transthyretin (TTR) and characterized by multisystem extracellular deposition of amyloid, leading to dysfunction of different organs and tissues
- specifically renal, cardiac, and ocular
- Carpal tunnel syndrome and neuropathies
- hypertrophic cardiomyopathy
- SC: 45 mg once monthly, autoinjector, 45mg per 0.8ml (one syringe)
- stored in refrigerator, remove 30 minutes before injection
- decrease vitamin A, vitamin A supplementation is recommended

atidarsagene autotemcel (Lenmeldy) infusion

March 18, 2024
Metachromatic Leukodystrophy

Orchard Therapeutics

- autologous hematopoietic stem cell gene therapy for children with metachromatic leukodystrophy
- first therapy approved for this rare, fatal genetic disorder
- caused by a mutation in the gene responsible for encoding the enzyme arylsulfatase A leading to neurological damage and developmental regression due to the accumulation sulfatides (fats) in the brain
- inserting one or more functional copies of the human *ARSA* gene *ex vivo* into the genome of a patient's own hematopoietic stem cells using a lentiviral vector
- 37 pediatric patients, 12 years of follow-up
- extended overall survival and resulted in the preservation of motor function and cognitive skills
- significant side effect profile including thrombosis and infections
- weight-based infusion

natalizumab-sztn (Tyruko) Injection

August 24, 2023 Sandoz

Multiple Sclerosis, Crohn's Disease

- integrin receptor antagonist biosimilar to Tysabri, approved for multiple sclerosis and Crohn’s disease
- first and only FDA-approved biosimilar for relapsing forms of MS
- monotherapy for the treatment of relapsing forms of multiple sclerosis, to include clinically isolated syndrome, relapsing-remitting disease, and active secondary progressive disease
- BBW: progressive multifocal leukoencephalopathy
- REMS program
- Jan 2024 was available in Germany, First quarter 2024 for the US

efgartigimod alfa and hyaluronidase-qvfc (Vyvgart Hytrulo) Injection

June 20, 2023
Myasthenia Gravis

Halozyme Therapeutics

- neonatal Fc receptor blocker and endoglycosidase combination for generalized myasthenia gravis (gMG) in adult patients who are anti-acetylcholine receptor (AChR) antibody positive
- Vyvgart was approved in Dec 2021 (IV once weekly for 4 weeks)
- Not interchangeable
- Vyvgart Hytrulo is a SC 1,008 mg fixed dose over 30-90 seconds
- once weekly injections for four weeks
- administered by a healthcare professional
- missed dose can be given if within 3 days, otherwise return to weekly schedule for total of 4 doses
- efgartigimod alfa 1,008 mg/hyaluronidase 11,200 units volume is 5.6 mL

efgartigimod alfa and hyaluronidase-qvfc (Vyvgart Hytrulo) Injection

- administer using a 25G, 12-inch tubing, PVC winged infusion set with a maximum priming volume of 0.4 mL
- allow refrigerated product to reach room temperature (15 minutes)
- yellowish, clear to opalescent solution
- significant CYP3A4 drug-drug interactions
- Phase 3 ADAPT-SC study—bridged individuals from the ADAPT trial for the IV therapy to the SC therapy
- no live vaccines during active treatment

rozanolixizumab-noli (Rystiggo) Injection

June 26, 2023 UCB
Myasthenia Gravis

- neonatal Fc receptor blocker for generalized myasthenia gravis in adults who are anti-acetylcholine receptor (AChR) or antimuscle-specific tyrosine kinase (MuSK) antibody positive
- two most common subtypes of gMG
- subcutaneous infusion
- Phase 3 MycarinG study
- significant difference favoring rozanolixizumab-noli was observed in the MG-ADL total score change from baseline [-3.4 points vs -0.8 points in the placebo group ($p<0.001$)]
- dose according to body weight once weekly for 6 weeks
- subsequent cycles may be administered based on clinical evaluation and no sooner than 63 days from the start of the previous cycle\
- 280 mg/2 mL
- lower right or left part of abdomen below the navel

zilucoplan (Zilbrysq) Injection

October 17, 2023 UCB, Inc.

Myasthenia gravis

- peptide inhibitor of complement component 5 (C5 inhibitor) for generalized myasthenia gravis in adults who are anti-acetylcholine receptor (AChR) antibody positive
- SC daily dose based on *actual* body weight, <56 kg 16.6mg, ≥56 to <77 kg 23mg, ≥77 kg 32.4mg
- prefilled syringes of 16.6 mg/0.416 mL, 23 mg/0.574 mL, 32.4 mg/0.81 mL
- allow prefilled syringe to reach room temperature prior to administration
- avoid skin that is scarred, tender, red, bruised, or hard
- rotate injection sites.
- do not attempt to remove air bubbles from prefilled syringe
- can be injected by patient with training or HC professional

zilucoplan (Zilbrysq) Injection

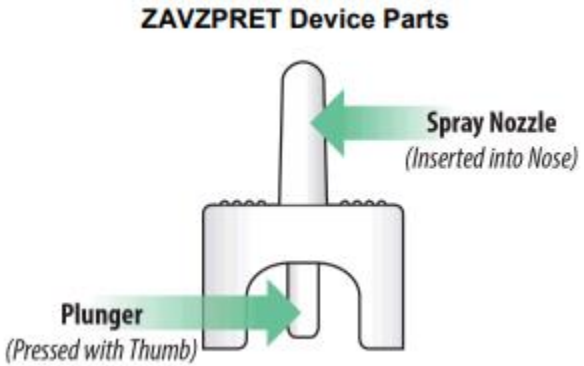
- vaccinate with meningococcal vaccine (serogroups A, C, W, and Y and serogroup B) at least 2 weeks prior to initiation (ZILBRYSQ REMS program due to risk of meningococcal disease)
- RAISE study was a multi-center, Phase 3, randomized, double-blind, placebo-controlled study to assess the efficacy, safety profile, and tolerability in adults with AChR antibody-positive gMG
- pts were randomized in a 1:1 ratio to SC daily injections of 0.3 mg/kg or placebo for 12 weeks
- rapid, consistent, and statistically significant benefits in different patient-and-clinician reported outcomes at Week 12 in mild-to-severe
- ADRs: injection site reactions, upper respiratory tract infection, diarrhea
- Avoid in pregnancy, crosses placenta and may cause fetal harm

zavegepant (Zavzpret) Nasal Spray

March 9, 2023 Pfizer

Migraine

- calcitonin gene-related peptide (CGRP) receptor antagonist for *acute treatment*
- migraines with and without aura
- placebo controlled trials
- nasal spray 10mg (device looks like Narcan)
- 10mg once in 24 hours, do not repeat in that time frame
- single spray in one nostril
- no more than 8 uses in 30 day period



zavegepant (Zavzpret) Nasal Spray

- 15 minute for early pain relief
- about 24% were pain free at 2 hours
- about 40% reported response to most bothersome symptoms at 2 hours
- ADRs: dysgeusia and taste disorders (less than 18%), nose irritation
- avoid in patients with severe liver or renal dysfunction
- avoid with intranasal decongestants (if use then at least 1 hour after zavegepant)

rizatriptan (RizaFilm) Oral Film

April 14, 2023 IntelGenx
Migraine

- serotonin (5-HT) 1B/1D receptor agonist (triptan) oral film for the acute migraine
- Maxalt® is oral tablets and ODT (generics available)
- formulation of oral thin film is to launch 1st quarter 2024
- 10mg film considered equivalent to oral and ODT rizatriptan
- do not remove from pouch until ready for use
- place oral film on the tongue
- film disintegrates within 2 minutes
- No water needed and can be swallowed with saliva

gepirone (Exxua) Extended-Release Tablets

September 22, 2023 Fabre-Kramer Pharmaceuticals
Major Depressive Disorder

- azapirone antidepressant for adults with major depressive disorder
- novel mechanism of action selectively targets the serotonin 1A receptor
- 18.2 mg once daily, may increase to 36.3 mg once daily on day 4 based on response and tolerability
- further increase to 54.5 mg once daily on day 7 and 72.6 mg once daily on day 14 based on response and tolerability
- maximum dosage is 72.6 mg once daily
- taper needed if discontinuation of drug if pt has been taking for more than 4 weeks
- limited data on switching between antidepressants (cross-titration)
- allow 14 days to elapse between discontinuing an MAOI and initiation of gepirone

gepirone (Exxua) Extended-Release Tablets

- Dosing for older adults or those with CrCl less than 50 mL/min or liver disease CP 2: 18.2 mg once daily, may increase to 36.3 mg once daily on day 7 based on response and tolerability, maximum dosage 36.3 mg once daily
- administer with food at approximately the same time every day
- swallow tablets whole, do not split, crush, or chew
- baseline ECG maybe recommended due to QTc prolongation
- Potential fetal harm, if pregnant women is exposed then report to National Pregnancy Registry for Antidepressants
- ADRs: nausea, dizziness, headache, fatigue, insomnia, suicidal ideations, serotonin syndrome
- Significant drug-drug interactions: major CYP 3A4 substrate

zuranolone (Zurzuvae) Capsules

August 4, 2023
Postpartum Depression

Sage Therapeutics
CIV

- neuroactive steroid gamma-aminobutyric acid (GABA) A receptor positive modulator for postpartum depression in adults
- First oral therapy, brexanolone (Zulresso () IV infusion was approved in 2019
- 50 mg once daily in the evening for 14 days
- may reduce to 40 mg once daily in the evening based on tolerability
- 20 mg, 25 mg, 30 mg capsules available
- administer with a fat-containing food (400 to 1,000 calories, 25% to 50% fat) for adequate absorption
- driving impairment due to CNS depressant effects
- advise patients not to drive or engage in other potentially hazardous activities until at least 12 hours after administration for the 14-day treatment
- missed dose: administer the next dose at the regularly scheduled time the next evening
- significant drug-drug interactions; CYP3A4 substrate
- abuse potential (C-IV)
- SKYLARK Study treatment rapidly improved symptoms of PPD at Day 15 and as early as Day 3 with sustained effect to Day 45

acetaminophen and ibuprofen (Combogesic IV) Injection

October 17, 2023
Postoperative Pain

AFT Pharmaceuticals

- analgesic and nonsteroidal anti-inflammatory drug (NSAID) combination for postoperative pain
- warning for dosage errors
- 100 mL vial contains 1 g acetaminophen and 300 mg ibuprofen
- <50 kg: Acetaminophen 15 mg/kg (in combination ibuprofen 4.5 mg/kg) every 6 hours as needed (max: APAP 750 mg/ibuprofen 225 mg as single dose or APAP 3 g/ibuprofen 900 mg per 24 hours)
- ≥50 kg: Acetaminophen 1 g (in combination ibuprofen 300 mg) every 6 hours as needed (max: APAP 4 g/ibuprofen 1.2 g per 24 hours)
- Combogesic tablets approved in US March 2023 as acetaminophen 325 mg and ibuprofen 97.5 mg (availability?)

trofinetide (Daybue) Oral Solution

March 10, 2023
Rett Syndrome

Acadia Pharmaceuticals

- synthetic analog of the amino-terminal tripeptide of IGF-1 for Rett syndrome
- glycine-proline-glutamate analog
- Rare, genetic mutation predominately in girls (extremely rare in boys)
- rarely inherited
- infants seem healthy during their first 6 months, but over time, rapidly lose coordination, speech, and use of the hands
- symptoms may then stabilize for years
- lifespan is shortened, average 40-50
- adult and pediatric patients two years of age and older
- first and only drug approved for Rett syndrome

trofinetide (Daybue) Oral Solution

- Phase 3 LAVENDER study evaluating the efficacy and safety of trofinetide versus placebo in 187 female patients with Rett syndrome 5 to 20 years of age
- statistical improvement in both caregiver and healthcare professional scores
- ADRs: diarrhea (82%), vomiting (29%)
- stop all laxatives prior to initiation of therapy
- diarrhea may result in dehydration
  - Mild to moderate: antidiarrheals and hydration
  - Severe: may require dose adjustment of discontinuation
  - Monitor weight

trofinetide (Daybue) Oral Solution

Adults and Adolescents

- Dosing: weight 35 to <50 kg: 10 g twice daily or ≥50 kg: 12 g twice daily

Children ≥2 years

- 9 to <12 kg: Oral: 5 g twice daily
- 12 to <20 kg: Oral: 6 g twice daily
- 20 to <35 kg: Oral: 8 g twice daily

- supplied as 200 mg/mL (450 mL)
- contains fd&c red #40, methylparaben sodium, propylparaben sodium
- strawberry flavor
- do not administered a missed dose or a vomited dose, wait until next dose is scheduled

sodium oxybate (Lumryz) Granules for Extended-Release Oral Suspension

May 1, 2023
Narcolepsy

Avadel Pharmaceuticals
C-III

- once-nightly for excessive daytime sleepiness and cataplexy in adults with narcolepsy
- extended-release, once nightly formulation versus twice nightly already FDA approved (Xyrem)
- Controlled substance
- REMS program
- Orphan Drug Exclusivity
- Phase 3 REST-ON clinical study compared to placebo
- Not compared to Xyrem in clinical trials or Xywav (oxybate salts)

sodium oxybate (Lumryz) Granules for Extended-Release Oral Suspension

- 4.5 g at bedtime, may increase by 1.5 g at weekly intervals
- recommended dosage range: 6 to 9 g once nightly
- maximum dose: 9 g
- Dosage forms available 4.5 g , 6 g, 7.5 g, 9 g
- High sodium content 9 g = 1,640 mg of sodium
- Abuse potential
- ADRs: CNS and respiratory depression, abnormal dreams, night terrors, suicidal ideation
- Significant drug-drug interactions with other CNS depressants

IMMUNOLOGY

immune globulin intravenous, human-stwk (Alyglo) Liquid for Intravenous Injection

December 15, 2023 GC Biopharma USA
Primary Immunodeficiency Syndrome

- 10% immune globulin liquid for IV injection for primary humoral immunodeficiency in adults
- phase 3-- prospective, open-label, single-arm, historically controlled, multicenter phase 3 study to assess the efficacy and safety
- primary outcome variable was acute serious bacterial infections

pozelimab-bbfg (Veopoz) Injection

August 18, 2023
CHAPLE Disease

Regeneron Pharmaceuticals

- complement C5 inhibitor for adults and pediatrics 1 year of age and older with CHAPLE disease
- very rare inherited immune disease causing the complement part of the immune system to become overactive
- complement system attacking the body’s own cells
- 100 patients worldwide
- single loading dose of pozelimab 30 mg/kg IV on day 1, followed by subcutaneous weekly weight-based doses

iptacopan (Fabhalta) Capsules

December 5, 2023
Paroxysmal Nocturnal Hemoglobinuria

Novartis Pharmaceuticals

- first-in-class, complement factor B inhibitor for the treatment of adults with paroxysmal nocturnal hemoglobinuria (PNH)
- PNH is an acquired mutation making red blood cells susceptible to premature destruction by the complement system
- characterized by hemolysis, bone marrow failure, and thrombosis in varying combinations and levels of severity
- Phase III APPLY-PNH trial in patients with residual anemia (hemoglobin < 10 g/dL) despite prior anti-C5 treatment (standard of care)
- specific conversions are recommended from anti-C5 to iptacopan
- 200 mg twice daily
- ADRs: headache, nasopharyngitis, bacterial and viral infections
- REMS: must receive vaccinations for encapsulated bacteria

leniolisib (Joenja) Tablets

March 24, 2023 Pharming Group N.V.
Activated PI3K-Delta Syndrome

- kinase inhibitor activated phosphoinositide 3-kinase delta (PI3Kδ) syndrome (APDS)
- rare and progressive primary immunodeficiency
- first identified in 2013, infections start in infancy with most common are frequent and severe infections of the ears, sinuses, and upper and lower respiratory tracts
- enlarged lymph nodes and spleen
- higher risk of developing lymphoma
- first treatment approved in the US
- adult and pediatric patients 12 years of age and older

leniolisib (Joenja) Tablets

- weight ≥45 kg: 70 mg twice daily, approximately every 12 hours
- administer with or without food
- if patient vomits ≤1 hour after dose, readminister dose as soon as possible; if patient vomits >1 hour after dose, do not readminister dose
- Available as 70mg tablet
- ADRs: neutropenia (monitor WBC)
- 12-week randomized, placebo-controlled study in 31 patients aged 12 years and older demonstrated clinical efficacy of 70mg twice daily over placebo
- reduction in lymph node size and increase in B cells

bimekizumab-bkzx (Bimzelx) Injection

October 17, 2023 UCB
Plaque Psoriasis

- humanized interleukin-17A and interleukin-17F antagonist for moderate to severe plaque psoriasis in adults
- first IL-17A and IL-17F inhibitor
- 3 Phase 3, multicenter, randomized, placebo and/or active comparator-controlled trials (BE READY, BE VIVID and BE SURE)
- 320 mg (given as two SC injections of 160 mg each) at Weeks 0, 4, 8, 12 and 16, then every 8 weeks
- ≥120 kg, a dose of 320 mg every 4 weeks after week 16 may be considered
- available as an auto-injector or a prefilled syringe 160 mg/mL
- patient may self inject if trained or HC professional can administer
- infection and malignancy risk, monitor
- vaccinate prior to starting therapy and no live vaccines during therapy

ustekinumab-auub (Wezlana) Injection

October 31, 2023 Amgen
Plaque Psoriasis, Psoriatic Arthritis, Crohn's Disease, Ulcerative Colitis

- human interleukin-12 and -23 antagonist interchangeable biosimilar to Stelara for plaque psoriasis, psoriatic arthritis, Crohn’s disease, and ulcerative colitis
- launch expected in early 2025 with patent expiration of Stelara

BEHAVIORAL HEALTH

risperidone (Uzedy) Extended-Release Injectable Suspension

April 28, 2023
Schizophrenia

Teva Pharmaceuticals and MedinCell

- long-acting injectable atypical antipsychotic for schizophrenia
- first SC, long-acting formulation utilizing SteadyTeq™, a copolymer technology that controls the steady release of risperidone
- blood concentrations are reached within 6-24 hours of a single dose
- initiation of treatment requires no loading dose or oral supplementation
- Approved in adults, study involved ages 13 and up
- two Phase 3 trials: RISE Study and SHINE Study
- Prefilled SC syringes: 50 mg/0.14 mL; 75 mg/0.21 mL; 100 mg/0.28 mL; 125 mg/0.35 mL; 150 mg/0.42 mL; 200 mg/0.56 mL; 250 mg/0.7 mL
- Perseris™ is given once a month SC, Risperdal Consta® is usually given every 2 weeks (IM), Uzedy™ is given once a month or once every 2 months SC, Rykindo® is usually given every 2 weeks (IM)
- Conversions recommended between products

aripiprazole (Abilify Asimtufii) Extended-Release Injectable Suspension

April 27, 2023
Schizophrenia, Bipolar Disorder

Otsuka Pharmaceutical

- long-acting injectable atypical antipsychotic for schizophrenia and bipolar I disorder
- IM: every 2 months, single-chamber, prefilled syringe
- must be administered by healthcare professional, gluteal injection
- pharmacokinetic bridging study which was a 32-week, open-label, multiple-dose, randomized, parallel-arm, multicenter study, compared to Abilify Maintena
- conversion charts available, missed doses, adherence
- 960 mg once every 2 months (56 days)
- may be given up to 2 weeks before or 2 weeks after the 2-month schedule
- overlap *oral* aripiprazole or other *oral* antipsychotic for 14 days during initiation
- 720 mg/2.4 mL and 960 mg/3.2 mL (volume of fluid requires gluteal injection)

nalmefene hydrochloride (Opvee) Nasal Spray

May 22, 2023
Opioid Overdose

Opiant Pharmaceuticals

- opioid antagonist for treatment of opioid overdose
- IV formulation already available, now as a nasal spray
- 12 years and older
- AHA recommends naloxone first with repeated doses before use of nalmefene
- 2.7 mg (contents of 1 nasal spray) as a single dose in 1 nostril
- may repeat every 2 to 5 minutes as needed in alternating nostrils
- new nasal spray is required for each dose
- packaging with either 1 spray or 2
- nalmefene has a longer duration of action and a higher affinity for opioid receptors compared to naloxone

naloxone hydrochloride (RiVive) Nasal Spray

July 28, 2023
Opioid Overdose

Harm Reduction Therapeutics

- OTC opioid antagonist nasal spray for emergency treatment of opioid overdose
- 3mg strength
- no company, entity, or individual will profit from sales

buprenorphine (Brixadi) Extended-Release Injection

May 23, 2023 Braeburn
Opioid Use Disorder C-III

- partial opioid agonist for opioid use disorder
- extended-release subcutaneous injection, injected and prepared by HC professional
- both weekly and monthly injections available—not interchangeable
- Brixadi: 64 mg/0.18 mL; 96 mg/0.27 mL; 128 mg/0.36 mL
- Brixadi (Weekly): 8 mg/0.16 mL; 16 mg/0.32 mL; 24 mg/0.48 mL; 32 mg/0.64 mL [contains alcohol]
- need to have started on other buprenorphine therapy or had a single dose of transmucosal
- conversion charts available
- restricted distribution: Brixadi Risk Evaluation and Mitigation Strategy
- clinical trials compared to SL formulation and found to be noninferior

ONCOLOGY

denosumab-bbdz Injection (Wyost)

March 5, 2024 Sandoz

Osteolytic Bone Lesions of Multiple Myeloma, Osteolytic Bone Metastases of Solid Tumors, Giant Cell Tumor of Bone, Hypercalcemia of Malignancy

- RANK ligand (RANKL) inhibitor
- First interchangeable biosimilar to Xgeva (denosumab)
- Approved for same indications as Xgeva
- 120 mg/1.7 mL (70 mg/mL) injection
- Supplement with calcium and vitamin D

melphalan (Hepzato) Lyophilized Powder for Injection

August 14, 2023
Uveal Melanoma

Delcath Systems

- alkylating drug used as a liver-directed treatment for adults with uveal melanoma with unresectable hepatic metastases
- melphalan for *intra-arterial* infusion via percutaneous hepatic perfusion (PHP) is available only through a restricted program under a REMS program called the Hepzato Kit REMS
- FOCUS Study median duration of response 14 months

lifileucel (Amtagvi) Suspension for Intravenous Infusion

February 16, 2024 Iovance Biotherapeutics
Melanoma

- tumor-derived autologous T cell immunotherapy used for adult patients with unresectable or metastatic melanoma
- FDA granted accelerated approval
- Had to have previous treatment with a PD-1 blocking antibody, and if BRAF V600 mutation positive, a BRAF inhibitor with or without a MEK inhibitor
- a Phase 3 trial, TILVANCE-301
- collect and expand a patient's unique T cells from a portion of their tumor
- returns billions of the patient's T cells back to the body
- One-time treatment

eflornithine (Iwilfin) Tablets

December 13, 2023 US WorldMeds
Neuroblastoma

- ornithine decarboxylase inhibitor used to reduce the risk of relapse in adult and pediatric patients with high-risk neuroblastoma
- maintenance therapy
- multi-site, single-arm, externally controlled study of children with high-risk neuroblastoma who received eflornithine as maintenance therapy following standard of care treatment, including immunotherapy
- 52% reduction in the risk of relapse and a 68% reduction in the risk of death
- 192 mg tablets, orally, with or without food, twice daily for two years
- tablet contains corn starch
- ADRs: hearing loss, otitis media, pneumonia, and diarrhea
- monitor: CBC, liver function, hearing
- Significant drug-drug interactions

bevacizumab-tnjn (Avzivi) Injection

December 6, 2023 Bio-Thera Solutions
Colorectal Cancer, Non-Small Cell Lung Cancer, Glioblastoma Multiforme, Renal Cell Carcinoma, Cervical Cancer, Ovarian Cancer, Fallopian Tube Cancer, Peritoneal Cancer

- vascular endothelial growth factor (VEGF) inhibitor biosimilar to Avastin
- treatment of colorectal cancer, non-small cell lung cancer, glioblastoma, renal cell carcinoma, cervical cancer, and epithelial ovarian, fallopian tube, or primary peritoneal cancer
- second approved biosimilar for Avastin

fruquintinib (Fruzaqla) Capsules

November 8, 2023 Takeda
Colorectal Cancer

- highly selective and potent oral inhibitor of VEGFR-1, -2 and -3 for previously treated metastatic colorectal cancer
- first novel chemotherapy-free treatment option approved in the U.S. regardless of biomarker status
- 5 mg once daily on days 1 to 21 of each 28-day cycle; continue until disease progression or unacceptable toxicity
- 1 mg and 5 mg capsules, 1 mg contains tartrazine

capivasertib (Truqap) Tablets

November 16, 2023 AstraZeneca
Breast Cancer

- AKT inhibitor in combination with fulvestrant for advanced hormone receptor-positive, HER2-negative breast cancer or metastatic breast cancer with one or more biomarker alterations (PIK3CA, AKT1 or PTEN)
- first-in-class, potent, adenosine triphosphate (ATP)-competitive inhibitor of all three AKT isoforms (AKT1/2/3)
- concurrently, the FDA approved a companion diagnostic test to detect PIK3CA, AKT1 and PTEN
- CAPItello-291 Phase III trial (NEJM), reduced risk of disease progression or death by 50% versus fulvestrant alone
- 400mg is administered twice daily according to an intermittent dosing schedule of 4 days on and 3 days off, dose reductions can occur with side effects or toxicity
- avoid grapefruit
- significant drug-drug interactions
- evaluate fasting blood glucose and HbA_{1c} and optimize blood glucose prior to capivasertib initiation
- monitor for derm and GI toxicities especially in older adults
- Dosage forms include 160 mg and 200 mg tablets

repotrectinib (Augtyro) Capsules

November 15, 2023 Bristol Myers Squibb
Non-Small Cell Lung Cancer

- tyrosine kinase inhibitor (TKI) for patients with ROS1-positive locally advanced or metastatic non-small cell lung cancer (NSCLC)
- TRIDENT-1 study, an open-label, single-arm, Phase 1/2 trial evaluating in TKI-naïve and TKI-pretreated patients
- median duration of response (mDOR) was 34.1 months
- 40 mg capsules
- 160 mg once daily for 14 days, then increase to 160 mg twice daily; continue until disease progression or unacceptable toxicity
- Same time each day, swallow capsules whole--do not open, chew, crush, or dissolve capsules
- Dosing reductions based on ADRs or toxicity
- Significant drug-drug interactions

toripalimab-tpzi (Loqtorzi) Injection

October 27, 2023
Nasopharyngeal Carcinoma

Coherus Biosciences

- programmed death receptor-1 (PD-1)- blocking antibody for nasopharyngeal carcinoma (NPC)
- combined with cisplatin and gemcitabine for first-line treatment of adults with metastatic or recurrent locally advanced NPC
- monotherapy for adults with recurrent, unresectable, or metastatic NPC with disease progression on or after platinum-containing chemotherapy
- JUPITER-02 Phase 3 study, 37% risk reduction in death compared to chemotherapy alone

efbemalenograstim alfa-vuxw (Ryzneuta) Injection

November 16, 2023 Evive Biotech
Neutropenia Associated with Chemotherapy

- leukocyte growth factor to reduce duration of febrile neutropenia in patients treated with chemotherapy
- adult patients with non-myeloid malignancies receiving myelosuppressive anti-cancer
- multi-center, randomized, multi-dose, active-controlled study comparing the efficacy and safety of efbemalenograstim to pegfilgrastim (Neulasta®)
- novel dimeric G-CSF long-acting fusion protein without PEGylation or Tween-80
- stronger G-CSF receptor activation properties and avoid the potential problem with allergic reactions
- SC: 20 mg once per chemotherapy cycle
- administer ≥24 hours after cytotoxic chemotherapy

retifanlimab-dlwr (Zynyz) Injection

March 22, 2023 Incyte
Merkel Cell Carcinoma

- programmed death receptor-1 (PD-1)-blocking antibody for Merkel cell carcinoma
- metastatic or recurrent locally advanced Merkel cell carcinoma
- rare type of skin cancer that usually appears as a flesh-colored or bluish-red nodule, often on the face, head or neck
- tends to grow fast and to spread quickly
- POD1UM-201 trial, an open-label, multiregional, single-arm study
- objective response rate (ORR) of 52%
- duration of response (DOR) ranged from 1.1 to 24.9+ months
- ADRs: fatigue, musculoskeletal pain
- 500 mg intravenously every four weeks until disease progression, unacceptable toxicity, for up to 24 months

tislelizumab-jsgr (Tevimbra) Injection

March 13, 2024 BeiGene
Esophageal Carcinoma

- programmed death receptor-1 (PD-1) blocking antibody for unresectable or metastatic esophageal squamous cell carcinoma (ESCC) after prior chemotherapy did not include a PD-(L)1 inhibitor
- available 2nd half of 2024
- RATIONALE 302 trial, primary endpoint of a statistically significant and clinically meaningful survival benefit
- 8.6 months compared to 6.3 months with standard chemotherapy

abiraterone acetate and niraparib (Akeega) Tablets

August 11, 2023
Prostate Cancer

Janssen Pharmaceuticals

- CYP17 inhibitor and a poly (ADP-ribose) polymerase (PARP) inhibitor combination indicated with prednisone for adults with deleterious or suspected deleterious BRCA-mutated metastatic castration-resistant prostate cancer
- first-and-only dual action tablet
- niraparib 200 mg/abiraterone acetate 1,000 mg once daily (with prednisone)
- continue until disease progression or unacceptable toxicity
- should also receive a gonadotropin-releasing hormone (GnRH) analog concurrently or had a bilateral orchiectomy
- prednisone is for managing adrenocortical insufficiency
- Phase 3 MAGNITUDE, median radiographic PFS of 19.5 months

epcoritamab-bysp (Epkinly) Injection

May 19, 2023 AbbVie
Diffuse Large B-Cell Lymphoma

- bispecific CD20-directed CD3 T-cell engager for treatment of adult patients with relapsed or refractory diffuse large B-cell lymphoma (DLBCL)
- Phase 1/2 EPCORE NHL-1 clinical trial
- overall response rate of 61%
- complete response rate of 38%
- median duration of response of 15.6 months
- Black box warning for cytokine release syndrome

glofitamab-gxbm (Columvi) Injection

June 15, 2023

Genentech

Diffuse Large B-Cell Lymphoma

- bispecific CD20-directed CD3 T-cell engager for treatment of relapsed or refractory diffuse large B-cell lymphoma, not otherwise specified (DLBCL, NOS) or large B-cell lymphoma (LBCL) arising from follicular lymphoma, after two or more lines of systemic therapy
- accelerated approval based on response rate and durability of response in the Phase I/II NP30179
- median duration of response was 1.5 years

talquetamab-tgvs (Talvey) Injection

August 9, 2023
Multiple Myeloma

Janssen Pharmaceutical

- first-in-class, bispecific GPRC5D-directed CD3 T-cell engager for patients with heavily pretreated multiple myeloma
- relapsed or refractory multiple myeloma who have received at least 4 prior lines of therapy, including a proteasome inhibitor, an immunomodulatory agent, and an anti-CD38 antibody
- 33% of pts had a complete response to SC injection
- median duration was 9 to 9.5 months
- Boxed Warning for cytokine release syndrome and neurologic toxicity including immune effector cell-associated neurotoxicity syndrome

motixafortide (Aphexda) Lyophilized Powder for Injection

September 8, 2023 BioLineRx
Hematopoietic Stem Cell Mobilization

- hematopoietic stem cell mobilizer indicated in combination with filgrastim (G-CSF) to mobilize hematopoietic stem cells to the peripheral blood for collection and subsequent autologous transplantation in patients with multiple myeloma
- one dosage of motizafortide plus filgrastim enabled a majority of patients to achieve the collection goal of ≥ 6 million hematopoietic stem cells

momelotinib (Ojjaara) Tablets

September 15, 2023 GSK
Myelofibrosis

- JAK1/JAK2 and activin A receptor type 1 (ACVR1) inhibitor for myelofibrosis patients with anemia
- blood cancer affecting approximately 25,000 pts in the US
- myelofibrosis pts are estimated to develop anemia over the course of the disease, and over 30% will discontinue treatment due to anemia
- 200 mg once daily until disease progression or unacceptable toxicity
- MOMENTUM phase 3 trial evaluated transfusion requirements and enlargement of the spleen

quizartinib (Vanflyta) Tablets

July 20, 2023 Daiichi Sankyo
Acute Myeloid Leukemia

- oral FLT3-ITD (FMS-like tyrosine kinase-3-internal tandem duplication) inhibitor for the treatment of FLT3-ITD positive acute myeloid leukemia
- newly diagnosed AML
- 37% of newly diagnosed AML are a *FLT3* gene mutation
- first, novel MOA
- combined with standard cytarabine and anthracycline induction and cytarabine consolidation
- 22% reduction in the risk of death compared to standard chemotherapy alone
- 17.7 mg, 26.5 mg tablets available
- REMS program due to QTc prolongation

OTHER

letibotulinumtoxinA-wlbg (Letybo) Powder for Injection

February 29, 2024 Hugel, Inc.
Glabellar Lines

- acetylcholine release inhibitor and a neuromuscular blocking agent
- temporary improvement in moderate to severe glabellar lines associated with corrugator and/or procerus muscle activity
- frown lines between the eyebrows
- positive results from 3 phase III studies
- Different dosing from other botulinumtoxins
- Inject 4 units into each of the 5 sites (2 injections each corrugator muscle and 1 in procerus muscle) for a total dose of 20 units per treatment session
- Do not administer more frequently than every 3 months
- Available late 2024

hyaluronic acid and lidocaine (Skinvive by Juvederm) Injectable Gel

May 15, 2023 Abbvie
Facial Wrinkles

- intradermal microdroplet injection containing a dermal filler and a local anesthetic to improve skin smoothness of the cheeks
- adults over the age of 21
- results lasting through six months
- unmet need in the skin quality category
- “improve smoothness of the cheeks leading to a lasting glow”

iloprost (Aurlumyn) Injection

February 13, 2024 Eicos Sciences
Frostbite
prostacyclin mimetic for severe frostbite in adults to reduce the risk of finger and toe amputations

- Better known as treatment for pulmonary arterial hypertension (PAH)—approved in 2004
- open-label, controlled trial that randomized 47 adults with severe frostbite
- 0% amputations in 16 patients in the ilopost group
- Priority Review and Orphan Drug designations
- 6 hours daily of infusion for up to 8 days
- ADRs: headache, flushing, heart palpitations, fast heart rate, nausea, vomiting, dizziness, and hypotension

beremagene geperpavec-svdt (Vyjuvek) Topical Gel

May 19, 2023
Epidermolysis Bullosa

Krystal Biotech

- herpes-simplex virus type 1 vector-based gene therapy for the wounds in patients dystrophic epidermolysis bullosa (DEB)
- dystrophic epidermolysis bullosa is a rare genetic disorder usually identified at birth
- blisters form in the dermis (middle of skin), lack the glue to keep together
- blisters can form on lining of organs
- 6 months and older
- gene therapy designed to address the genetic root cause of DEB by delivering functional copies of the human *COL7A1* gene to provide wound healing and sustained functional COL7 protein expression
- First FDA approved gene therapy with redosing
- Dose is based on wound size –dosing charts available, applied by HC professional
- Not to touch or scratch application area
- Dosed in plaque-forming units, 5 billion PFU/2.5 mL

birch triterpenes (Filsuvez) Topical Gel

December 19, 2023
Epidermolysis Bullosa

Chiesi Global Rare Diseases

- topical birch bark extract for treatment of wounds associated with dystrophic epidermolysis bullosa and junctional epidermolysis bullosa
- adult and pediatric patients 6 months of age and older
- apply a 1-mm layer to the affected wound surface with dressing changes until wound is healed
- do not apply to mucus membranes
- tube is for single use only and immediate use upon opening
- discard any unused portion
- Availability unknown

adapalene, benzoyl peroxide and clindamycin phosphate (Cabtreo) Topical Gel

October 20, 2023 Bausch Health Companies
Acne

- triple-combination for the topical treatment of acne vulgaris in adults and pediatrics 12 years of age and older
- clindamycin 1.2%/adapalene 0.15%/benzyl peroxide 3.1%
- 20 g and 50 g pumps, white to off-white, opaque gel
- contains propylene glycol
- Phase 3 clinical trials reported a 50% treatment success and approximately 75% reduction in both inflammatory and noninflammatory lesions at week 12
- ADRs: site reactions

avacincaptad pegol (Izervay) Intravitreal Solution

August 4, 2023 Iveric Bio

Geographic Atrophy

- complement C5 protein inhibitor for geographic atrophy (GA) secondary to age-related macular degeneration (AMD)
- intravitreal: 2 mg (0.1 mL) into affected eye(s) once monthly (about every 21 to 35 days) for up to 12 months
- GATHER1 and GATHER2 Phase 3 clinical trials evaluated the safety and efficacy of monthly 2 mg intravitreal administration
- lowing of disease progression was observed as early as 6 months with up to a 35% reduction in the first year
- ADRs: Conjunctival hemorrhage, visual changes
- monitor IOP and optic nerve

aflibercept (Eylea HD) Injection

August 18, 2023 Regeneron Pharmaceuticals
Macular Degeneration, Diabetic Macular Edema, Diabetic Retinopathy

- higher dose formulation of vascular endothelial growth factor (VEGF) inhibitor aflibercept for neovascular (wet) age-related macular degeneration (nAMD), diabetic macular edema (DME), and diabetic retinopathy (DR)
- intravitreal 8 mg (0.07 mL) every 4 weeks for the first 3 months
- then 8 mg every 8 to 16 weeks in wAMD and DME
- then every 8 to 12 weeks for DR
- 48-week results of PULSAR and PHOTON compared 8mg to 2mg afilibercept
- demonstrating non-inferior and clinically equivalent vision gains at 48 weeks with both 12- and 16-week dosing regimens after only 3 initial monthly doses
- advantage is the extended dosing regimen of eye injections with HD

travoprost (iDose TR) Intracameral Implant

December 13, 2023 Glaukos Corporation
Glaucoma, Open Angle, Glaucoma/Intraocular Hypertension

- long-duration prostaglandin analog indicated for the reduction of intraocular pressure (IOP) in patients with open-angle glaucoma (OAG) or ocular hypertension (OHT)
- one time single implant of 75 mcg, medical-grade titanium
- two prospective, randomized, multicenter, double-masked, Phase 3 pivotal trials
- compared to topical timolol ophthalmic solution, 0.5% BID
- ADRs: red eyes, bright light bothers eyes

phenylephrine hydrochloride and tropicamide (Mydcombi) Ophthalmic Spray

May 5, 2023 Eyenovia
Pupillary Dilation

- alpha-1 adrenergic receptor agonist and anticholinergic fixed-combination ophthalmic spray to induce mydriasis for diagnostic procedures and in conditions where short-term pupil dilation is desired
- 1% phenylephrine/2.5% tropicamide
- uses a unique Optejet device (not an injection—spray)
- can be used for in office pupil dilation or cataract surgery

phentolamine mesylate (Ryzumvi) Ophthalmic Solution

September 25, 2023 Ocuphire Pharma
Reversal of Pharmacologically Induced Mydriasis

- alpha adrenergic blocker for the treatment of pharmacologically-induced mydriasis (pupil dilation)
- reverse mydriasis produced by adrenergic agonists (e.g., phenylephrine) or parasympatholytic (e.g., tropicamide) agents
- 1 or 2 drops into each dilated eye following eye exam or procedure
- when using 2 drops, instill 2nd drop 5 minutes after the first
- effect within 30 minutes
- maximum effect: 60 to 90 minutes
- Duration: 24 hours
- 2024 release—first half

pilocarpine hydrochloride (Qlosi) Ophthalmic Solution

October 17, 2023 Orasis Pharmaceuticals
Presbyopia

- Low-dose formulation of the approved cholinergic agonist pilocarpine indicated presbyopia in adults
- 0.4% strength compared to Vuity 1.25%
- used daily, or as needed, up to twice per day
- effective 20 minutes after administration
- can last up to 8 hours
- improve near vision without impacting distance or night vision
- preservative-free formulation
- Availability 1st half of 2024

lotilaner (Xdemvy) Ophthalmic Solution

July 25, 2023 Tarsus Pharmaceuticals
Demodex Blepharitis

- isoxazoline ectoparasiticide for demodex blepharitis (eyelid disease caused by mites)
- first and only FDA approved for demodex blepharitis, collarettes are telltale sign
- 0.25%
- 1 drop into affected eye(s) twice daily for 6 weeks
- wait at least 5 minutes in between application of other eye drops
- remove contact lenses prior to administration and wait 15 minutes before reinserting
- do not touch tip of container to any surface, the eyelids or fingers, or surrounding areas
- 1.5 mL, 10 mL sizes [contains edetate (edta) disodium, polyoxyl/peg-35 castor oil(cremophor el)]
- ADRs: burning, stinging, chalazion (small bump in the eyelid caused by a blockage of a tiny oil gland)

perfluorohexyloctane (Miebo) Ophthalmic Solution

May 18, 2023
Dry Eye Disease

Bausch + Lomb Corporation and Novaliq

- semifluorinated alkane for signs and symptoms of dry eye disease
- first treatment for DED directly targeting tear evaporation
- GOBI and MOJAVE, two phase 3 pivotal clinical trials with 1217 patients (randomized 1:1 to perfluorohexyloctane or hypotonic saline), 57 day trials
- 1 drop into affected eye(s) 4 times daily
- 1.338 g/mL total 3 mL
- preservative free, do not touch eye with device
- Remove contact lenses prior to administration and wait at least 30 minutes before reinserting
- ADRs: red eyes, blurred vision

cyclosporine (Vevye) Ophthalmic Solution

May 30, 2023 Novaliq
Dry Eye Disease

- calcineurin inhibitor immunosuppressant for signs and symptoms of dry eye disease
- 0.1% (2ml bottle), solution
- novel vehicle, water-free excipient, fast acting, no anti-microbial preservatives, oils or surfactants
- other products such as Restatis, Verkazia, Cequa
- contact lenses may be reinserted 15 minutes following administration
- store at 59°F to 77°F, do not freeze or refrigerate

exagamglogene autotemcel (Casgevy) Suspension for Intravenous Infusion

December 8, 2023 Vertex Pharmaceuticals and CRISPR Therapeutics
Sickle Cell Disease, Beta Thalassemia

- CRISPR/Cas9 genome-edited cell therapy for sickle cell disease and transfusion-dependent beta-thalassemia (approved Jan 2024)
- 12 years and older
- Patient assistance program
- one-time treatment for sickle cell disease with frequent vaso occlusive crises
- made specifically for each patient, using the patient's own edited blood stem cells, and increases the production of a special type of hemoglobin called hemoglobin F

lovotibeglogene autotemcel (Lyfgenia) Suspension for Intravenous Infusion

December 8, 2023
Sickle Cell Disease

Bluebird Bio

- autologous hematopoietic stem cell-based gene therapy for the treatment of patients 12 years of age or older with sickle cell disease and a history of vaso-occlusive events
- one-time gene therapy
- adding a functional β-globin gene to patients' own hematopoietic stem cells
- Small clinical trials-- Severe vaso-occlusive events were resolved in 30/32 patients, 88.2% experienced no vaso-occlusive events at all
- ADRs: stomatitis, thrombocytopenia, neutropenia, febrile neutropenia, anemia, and leukopenia
- Some concern with trials with a different version– hematologic malignancy
- Only at qualified treatment centers-- single dose for infusion containing a suspension of CD34+ cells provided in 1 to 4 infusion bags

ritlecitinib (Litfulo) Capsules

June 23, 2023 Pfizer
Alopecia

- covalent kinase inhibitor for severe alopecia areata in adults and adolescents 12 years and older
- 50mg once daily
- Significant warnings including CV mortality, serious infections, and cancers
- monitor WBC and platelets
- administer all age-appropriate vaccines prior to starting therapy
- no live vaccines during treatment
- do not combine with JAK inhibitors or other immunosuppresives
- tablet contains blue dye

nirogacestat (Ogsiveo) Tablets

November 27, 2023
Desmoid Tumors

SpringWorks Therapeutics

- gamma secretase inhibitor indicated for progressing desmoid tumors
- desmoid tumors are noncancerous growths, fibroblasts from connective tissue
- adults
- 150 mg twice daily; continue until disease progression or unacceptable toxicity, dosage adjustments in package labeling based on side effects or toxicity
- administer with or without food, avoid grapefruit, Seville oranges, and starfruit
- swallow tablets whole; do not break, crush, or chew
- monitor for increases in liver enzymes
- Significant drug-drug interactions

References

Notable drug approvals, MPR website, https://www.empr.com/?s=new+drugs&published=12months

FDA access database
FDA safety alerts
Drugs.com
Lexicomp database
Manufacturer websites
PubMed

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