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Pediatric information added to labeling of 76 drugs, biologics in 2024

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The Food and Drug Administration (FDA) continues to increase the availability of pediatric use information in the labeling for drugs and biologics.

Between January and December 2024, pediatric use information was added to the labeling of 51 drugs and 25 biologics pursuant to laws that encourage or require pediatric studies. In addition, dozens of orphan drugs were approved for pediatric patients with rare diseases, including the first approved treatments for the neurological manifestations of Niemann-Pick disease type C (see table).

A pediatric labeling change refers to any update to a product's labeling to add information about safety, effectiveness or dosing in children. Many, but not all, labeling changes represent a new FDA approval for an indication in children. Several in 2024 represented approvals for novel drugs with active ingredient(s) never before approved or marketed in the U.S., such as the following:

- **Ebglyss** (lebrikizumab-lbkz) subcutaneous injection, an interleukin-13 antagonist to treat moderate-to-severe atopic dermatitis in patients 12 years and older who weigh at least 40 kilograms (kg) and whose disease is not adequately controlled with topical prescription therapies or when those therapies are not advisable;
- **Sofdra** (sofpironium) topical gel, an anticholinergic to treat primary axillary hyperhidrosis in patients 9 years and older;
- **Zelsuvmi** (berdazimer) topical gel, a nitric oxide-releasing agent to treat molluscum contagiosum in patients 1 year and older; and
- **Zevtera** (ceftobiprole medocaril sodium) intravenous injection, a cephalosporin antibacterial to treat community-acquired bacterial pneumonia in patients 3 months and older.

Other approvals in 2024 represented important strides in pediatric drug development, including the following:

- **Femlyv** (norethindrone acetate and ethinyl estradiol) is the first orally disintegrating tablet approved for the prevention of pregnancy. The formulation expands access to contraception for individuals who have trouble swallowing pills. The active ingredients in Femlyv have been approved in the U.S. as an oral tablet since 1968.
- **Onyda XR** (clonidine hydrochloride) oral suspension is the first liquid nonstimulant medication indicated for the treatment of attention-deficit/hyperactivity disorder. It is indicated in patients 6 years and older as a monotherapy or adjunctive to stimulant medications. Administration is once daily at bedtime.
- **Farxiga** (dapagliflozin) oral tablets, **Invokana** (canagliflozin) oral tablets and **Xigduo XR** (dapagliflozin and metformin hydrochloride extended-release) oral tablets received FDA approval as an adjunct to diet and exercise to improve glycemic control in patients 10 years and older with type 2 diabetes mellitus.
- Several **oxycodone** products were labeled with new pharmacokinetic and safety information for breastfeeding infants whose mothers receive oxycodone treatment during lactation. Data from lactation studies indicated that oxycodone is present in breastmilk and that maternal doses of less than 60 milligrams/day of an immediate-release formulation are unlikely to result in clinically relevant exposures in breastfed infants. However, based on known adverse effects in adults, infants exposed to oxycodone through breastmilk should be monitored for excess sedation and respiratory depression. Withdrawal symptoms can occur in breastfed infants when maternal administration of an opioid analgesic is stopped or when breastfeeding is stopped.

In 2024, labeling was updated for several products to reflect that safety and effectiveness have been evaluated but *not* established in pediatric patients, including Onglyza (saxagliptin) oral tablets and Kombiglyze XR (saxagliptin/metformin hydrochloride) extended-release oral tablets.

Effectiveness of saxagliptin was not demonstrated in a 26-week, placebo-controlled, double-blind randomized clinical trial with a 26-week safety extension in 164 pediatric patients ages 10-17 years with inadequately controlled type 2 diabetes mellitus.

Similarly, Ofev (nintedanib) oral capsules were evaluated, but effectiveness was not demonstrated in pediatric patients ages 6-17 years with fibrosing interstitial lung diseases.

The safety and effectiveness of several oncology drugs, including Imjudo (tremelimumab-actl), Inlyta (axitinib) and Lenvima (lenvatinib), were evaluated but not established for treatment of solid tumors in pediatric patients.

Additional examples of pediatric product approvals in 2024

Trade name (generic name) and dosage form	Pediatric indication approved in 2024 (see prescribing information for all indications)	Pediatric labeling change date
*Aqneursa (levacetylleucine) oral suspension	Treatment of neurological manifestations of Nieman-Pick disease type C in patients weighing at least 15 kg	Sept. 24, 2024
Avycaz (ceftazidime/avibactam) intravenous injection	Treatment of certain infections caused by susceptible gram-negative microorganisms: complicated intraabdominal infections, used in combination with metronidazole; complicated urinary tract infections, including pyelonephritis; and hospital-acquired bacterial pneumonia and ventilator-associated bacterial pneumonia in neonates (at least 31 weeks' gestational age) and infants younger than 3 months (previously approved in patients 3 months and older)	Jan. 26, 2024
Benlysta (belimumab) subcutaneous injection	Treatment of active systemic lupus erythematosus and active lupus nephritis in patients 5 years and older who are receiving standard therapy (previously approved as an intravenous injection; in 2024, the subcutaneous autoinjector formulation was approved for pediatric patients 5 years and older, allowing children to receive treatment at home)	May 16, 2024
Bkemv (eculizumab-aeeb) intravenous injection	Treatment of atypical hemolytic uremic syndrome to inhibit complement-mediated thrombotic microangiopathy (the first interchangeable biosimilar to Soliris [eculizumab])	May 28, 2024
*Crenessity (crinecerfont) oral capsules and oral solution	Adjunctive treatment to glucocorticoid replacement to control androgens in patients 4 years and older with classic congenital adrenal hyperplasia	Dec. 13, 2024
Fibryga (fibrinogen [human]) intravenous injection	Fibrinogen supplementation in bleeding patients with acquired fibrinogen deficiency	July 31, 2024
Imkeldi (imatinib) oral solution	Treatment of newly diagnosed Philadelphia chromosome positive chronic myeloid leukemia (Ph+ CML) in chronic phase and acute lymphoblastic leukemia (Ph+ ALL) in combination with chemotherapy	Nov. 22, 2024
Legubeti (acetylcysteine) powder packets for oral solution	Antidote to prevent or lessen hepatic injury, which may occur following the ingestion of a potentially hepatotoxic quantity of acetaminophen	Feb. 13, 2024

*Miplyffa (arimoclomol) oral capsules	Treatment of neurological manifestations of Niemann-Pick disease type C (NPC), in combination with miglustat, in patients 2 years and older (first treatment for the neurological manifestations of NPC)	Sept. 20, 2024
Neffy (epinephrine) nasal spray	Emergency treatment of type I allergic reactions, including anaphylaxis, in patients who weigh at least 30 kg (first nasal spray for treatment of anaphylaxis)	Aug. 9, 2024
*Ojemda (tovorafenib) oral suspension	Treatment of patients 6 months and older with relapsed or refractory pediatric low-grade glioma harboring a BRAF fusion or rearrangement, or BRAF V600 mutation (first FDA approval for this indication)	April 23, 2024
Orkambi (lumacaftor/ivacaftor) oral tablets	Treatment of cystic fibrosis in patients 1 year and older who are homozygous for the F508del mutation in the CFTR gene	Dec. 13, 2024
Palforzia (peanut [*Arachis hypogaea*] allergen powder-dnfp) oral powder	Mitigation of allergic reactions, including anaphylaxis, that may occur with accidental exposure to peanut in patients 1 through 3 years with a confirmed diagnosis of peanut allergy (previously approved for patients 4 years and older)	July 26, 2024
Veklury (remdesivir) intravenous injection	Treatment of COVID-19 in patients from birth to less than 28 days of age weighing at least 1.5 kg who are hospitalized or have mild-to-moderate COVID-19 and are at high risk for progression to severe COVID-19, including hospitalization or death (previously approved for patients 28 days and older)	Feb. 28, 2024
Xolair (omalizumab) subcutaneous injection	Reduction of allergic reactions (type I), including anaphylaxis, that may occur with accidental exposure to one or more foods in patients 1 year and older with IgE-mediated food allergy (first FDA approval for this indication)	Feb. 16, 2024

*Designated as an orphan drug

The FDA's Office of Pediatric Therapeutics and Office of New Drug's Division of Pediatrics and Maternal Health contributed to this article.

Resources

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- Orphan drug designations and approvals
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