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Pediatric information added to labeling of 75 drugs, biologics in 2022

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The Food and Drug Administration (FDA) continues efforts to increase the availability of pediatric information in the labeling for drugs and biologics. Between January and December 2022, pediatric information was added to the labeling of more than 75 drugs and biologics, covering a broad range of therapeutic areas.

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 represent important strides in pediatric product development. For example:

- Boostrix (tetanus toxoid, reduced diphtheria toxoid and acellular pertussis vaccine, adsorbed [Tdap]) intramuscular injection approval is expanded to include immunization during the third trimester of pregnancy to prevent pertussis in infants younger than 2 months. The FDA's approval for Boostrix

previously included use during pregnancy to protect the vaccinated individual. This is the first vaccine approved for use during pregnancy specifically to prevent a disease in young infants.

- Cabenuva (cabotegravir extended-release; rilpivirine extended-release) intramuscular injection approval as a complete regimen for treatment of HIV-1 infection is expanded to include adolescents 12 years and older and weighing at least 35 kilograms (kg). This is the first intramuscular injectable HIV drug approved for adolescents. Cabenuva can be administered monthly or every two months, providing a potential alternative to traditional daily oral HIV treatment regimens for some patients.
- Pedmark* (sodium thiosulfate) intravenous injection is approved to reduce the risk of hearing loss (ototoxicity) associated with cisplatin, a platinum-based chemotherapeutic, in pediatric patients ages 1 month and older with localized, nonmetastatic solid tumors. This is the first product approved for this indication. Cisplatin is used to treat a variety of cancers and can cause severe ototoxicity, particularly in pediatric patients in whom the prevalence of hearing loss is estimated to be 40% to 60%.
- Tzield (teplizumab-mzwv) subcutaneous injection is approved to delay the onset of stage 3 type 1 diabetes in patients 8 years and older who have stage 2 type 1 diabetes. This is the first disease-modifying therapy approved for type 1 diabetes. Although Tzield does not prevent or cure type 1 diabetes, treatment can delay the need for exogenous insulin therapy and its associated risks and intensive regimen. This delay of approximately two years is considered clinically meaningful, particularly because type 1 diabetes often presents in patients younger than 10 years who may face challenges with complex disease management.
- Zynteglo (betibeglogene autotemcel) intravenous injection is an autologous hematopoietic stem cell-based gene therapy that was approved for the treatment of patients with beta-thalassemia who require regular red blood cell transfusions. This is the first cell-based gene therapy approved for this indication. Effectiveness was established based on achievement of transfusion independence; 89% of 41 patients receiving Zynteglo achieved transfusion independence.

Some pediatric labeling changes may reflect new safety information relevant to children or may add information about clinical studies that failed to demonstrate efficacy in children.

In 2022, labeling was updated for several products to reflect that safety and effectiveness have been evaluated but *not* established in pediatric patients, including Cimzia (certolizumab pegol) subcutaneous injection for treatment of moderately to severely active Crohn’s disease, Halaven (eribulin mesylate) intravenous injection for treatment of relapsed or refractory solid tumors and lymphomas, Cotellic (cobimetinib) tablets for treatment of solid tumors, Gilotrif (afatinib) tablets for treatment of solid tumors with known ErbB pathway deregulation and Geodon (ziprasidone hydrochloride) capsules for treatment of bipolar I disorder.

Additional examples of pediatric product approvals in 2022

Trade name (generic name)	Indication and labeling change	Pediatric labeling change date
Benlysta (belimumab) intravenous injection	Treatment of patients 5 years and older with active lupus nephritis	July 26, 2022
CellCept (mycophenolate mofetil) tablet, capsule, oral suspension, intravenous injection	Prophylaxis of organ rejection of allogenic heart and liver transplants in pediatric recipients 3 months and older	June 6, 2022
Comirnaty (COVID-19 vaccine, mRNA) intramuscular injection	Active immunization to prevent coronavirus disease 2019 (COVID-19) caused by severe acute respiratory syndrome coronavirus 2 (SARS-CoV-2) in individuals 12 years and older	July 8, 2022
Dupixent* (dupilumab) subcutaneous injection	Treatment of eosinophilic esophagitis in patients 12 years and older weighing at least 40 kg	May 20, 2022
Imbruvica* (ibrutinib) capsules	Treatment of chronic graft vs. host disease after failure of one or more lines of systemic therapy in patients 1 year and older	Aug. 24, 2022
Priorix (Measles, mumps and rubella vaccine, live) subcutaneous injection	Active immunization for the prevention of measles, mumps and rubella in individuals 12 months and older	June 3, 2022
Qsymia (phentermine and topiramate) extended-release capsules	Adjunct to a reduced-calorie diet and increased physical activity for chronic weight management in pediatric patients 12 years and older with body mass index (BMI) in the 95th percentile or greater standardized for age and sex (obesity)	June 24, 2022
Sezaby* (phenobarbital sodium) intravenous injection	Treatment of neonatal seizures in term and preterm infants	Nov. 17, 2022
SMOFlipid (lipid injectable emulsion) intravenous injection	Source of calories and essential fatty acids for parenteral nutrition in pediatric patients, including term and preterm neonates, when oral or enteral nutrition is not possible, insufficient or contraindicated	March 22, 2022
Stelara (ustekinumab) subcutaneous injection	Treatment of active psoriatic arthritis in patients 6 years and older	July 29, 2022
Tpoxx* (tecovirimat) intravenous injection	Treatment of human smallpox disease in adults and pediatric patients weighing at least 3 kg	May 18, 2022
Triumeq PD (abacavir, dolutegravir and lamivudine) tablets for oral suspension	Treatment of HIV-1 infection in pediatric patients weighing 10 to less than 25 kg	March 30, 2022
Vaxneuvance (pneumococcal 15-valent conjugate vaccine) intramuscular injection	Active immunization for the prevention of invasive disease caused by *Streptococcus pneumoniae* in individuals 6 weeks and older	June 17, 2022
Veklury (remdesivir) intravenous injection	Treatment of COVID-19 in patients 28 days and older weighing at least 3 kg	April 25, 2022
Wegovy (semaglutide) subcutaneous injection	Adjunct to a reduced-calorie diet and increased physical activity for chronic weight management in patients 12 years and older with an initial BMI at the 95th percentile or greater standardized for age and sex (obesity)	Dec. 23, 2022
Xofluza (baloxavir marboxil) tablets and granules for oral suspension	Treatment and post-exposure prophylaxis of influenza in patients 5 years and older	Aug. 11, 2022

* Designated as an orphan product

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

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