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FDA fosters development of therapies for children with rare diseases

by from the Food and Drug Administration Office of Pediatric Therapeutics, Division of Pediatric and Maternal Health, and Office of Orphan Products

The Food and Drug Administration's (FDA's) Orphan Drug Designation program provides financial incentives to sponsors that develop drugs and biologics intended for the treatment, diagnosis or prevention of rare diseases/disorders that affect fewer than 200,000 people or when they are not expected to recover the costs of research and development of the treatment for a specific indication.

Sponsor participation in the orphan drug designation program continues to grow. Since the program began in 1983, the FDA has received more than 5,700 requests for orphan drug designations; more than 1,000 requests were received in 2016.

The program has enabled the development and marketing of almost 600 drugs and biologic products for rare diseases. A significant proportion of orphan drug designations are for products that receive marketing approval for use in children. In 2016, the FDA approved products for the diagnosis or treatment of 39 orphan diseases or conditions of which 14 were indicated for use in the pediatric population (see table). Two products with orphan drug designations (eteplirsen for treatment of Duchenne muscular dystrophy and nusinersen for treatment of spinal muscular atrophy) also were awarded vouchers that provided expedited review of the drugs by the FDA.

Orphan drug products with pediatric indications approved in 2016

Orphan drug product	Rare disease/condition
Dysport (botulinum toxin type A)	muscle contractures in pediatric cerebral palsy patients
Ilaris (canakinumab)	• hyperimmunoglobulinemia D syndrome/mevalonate kinase deficiency • familial Mediterranean fever • tumor necrosis factor receptor associated periodic syndrome
Defitelio (defibrotide)	hepatic veno-occlusive disease
Exondys 51 (eteplirsen)	Duchenne muscular dystrophy
Netspot (Gallium [Ga-68]-N-[(4,7,10-Tricarboxymethyl-1,4,7,10-tetraazacyclododec-1-yl) acetyl]-D-phenylalanyl-L-cysteinyl-L-tyrosyl-D-tryptophanyl-L-lysyl-L-threoninyl-L-cysteinyl-L-threonine-cyclic (2-7) disulfide)	localization of neuroendocrine tumors
Orkambi (lumacaftor/ivacaftor)	cystic fibrosis
Vermox (mebendazole)	gastrointestinal infections caused

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	by *Ascaris lumbricoides* (roundworm) and *Trichuris trichiura* (whipworm)
Provayblue (methylene blue)	methemoglobinemia
Orfadin (nitisinone)	tyrosinemia type 1
Spinraza (nusinersen)	spinal muscular atrophy
Anthim (obiltoxaximab)	inhalation anthrax
Zemplar (paricalcitol)	pediatric hyperparathyroidism
Idelvion (recombinant fusion protein linking coagulation factor IX with albumin)	factor IX deficiency (hemophilia B)
Crestor (rosuvastatin)	pediatric homozygous familial hypercholesterolemia

Resource

- FDA orphan drug product designation database,