

FDA Approved Drugs with Orphan Designations for one or more indications.

- **302** Original orphan drugs for single rare disease
- **72** Orphans that served the mass market first
- **84** Orphans approved for multiple orphan diseases

302 Original orphan drugs for single rare disease

Acetadote	Acthrel	Adagen	Adcirca
Adreview	Agrylin	Aldurazyme	Alecensa
Alimta	Alinia	Alkeran For Injection	Alomide Ophthalmic Solution
Aloprim	Alphanine	Alprolix	Ammonul
Ampyra	Anascorp	Anavip	Anthim
Anthrasil	Apokyn	Arcalyst	Aromasin
Arranon	Atnativ	Atryn	Avonex
Babybig	Banzel	Beleodaq	Bendeka
Benefix	Berinert	Betapace	Betaseron
Bexxar	Blinicyto	Bosulif	Botulism antitoxin heptavalent (A, B, C, D, E, F, G) (Equine)
Buphenyl	Busulfex	Cafcit	Campath
Caprelsa	Carbaglu	Carnexiv	Cayston
Ceprotrin	Cerdelga	Cerebyx	Ceredase
Cerezyme	Cetylev	Chemet	Chenix
Chirhostim	Cholbam	Cibacalcin	Cinryze
Clolar	Cnj-016	Coagadex	Coartem
Colcrys	Cometriq	Copaxone	Cotellic
Crofab	Curosurf	Cuvposa	Cyanokit
Cystadane	Cystagon	Cystaran	Dacogen
Daunoxome	Defitelio	Depocyt	Diastat Acudial
Digibind	Duopa	Duraclon	Elaprase
Eldepryl	Elelyso	Elitek	Ellence
Elliotts B Solution	Elmiron	Eloctate	Empliciti

Envarsus Xr	Erwinaze	Esbriet	Ethamolin
Evomela	Exondys 51	Exosurf Neonatal	Extraneal
Fabrazyme	Fareston	Farydak	Felbatol
Ferriprox	Firazyr	Fludara	Folotyn
Galzin	Gamimune N	Gastrocrom	Gattex
Gralise	Halfan	Hemangeol	Hetlioz
Hexalen	Hivid	Humate-P	Idamycin
Idelvion	Ifex	Imlygic	Impavido
Increlex	Infasurf	Inomax	Iplex
Juxtapid	Kalydeco	Kanuma	Kogenate
Korlym	Krystexxa	Kuvan	Kynamro
Kyprolis	Lamprene	Lenvima	Letairis
Leustatin	Lidoderm	Lioresal	Lutrepulse
Luveris	Lynparza	Makena	Marqibo
Membraneblue	Mesnex	Metrodin	Metrogel
Mitosol	Moctanin	Mononine	Mozobil
Myalept	Mycobutin	Mylotarg	Myobloc
Naglazyme	Natpara	Nebupent	Neoprofen
Netspot	Neumega	Neutrexin	Ninlaro
Nipent	Nithiodote	Normocarb Hf	Northera
Nplate	Nulojix	Nutrestore	Nutropin Depot
Nymalize	Obizur	Ocaliva	Ofev
Oforta	Oncaspar	Oncoscint Cr/Ov	Onfi
Onivyde	Ontak	Opsumit	Opticrom
Orlaam	Ornidyl	Panhematin	Panretin
Parathar	Paser	Pentam	Pentaspan
Pentetate Calcium Trisodium	Phoslo	Phoxillum	Pomalyst
Portrazza	Praxbind	Priftin	Proamatine/Amatine
Prolastin-C	Protropin	Provayblue	Provigil

Pulmozyme	Purixan	Qualaquin	Qutenza
Radiogardase	Ravicti	Raxibacumab/Abthraxtm	Refacto, Xyntha
Refludan	Remodulin	Renacidin	Repatha
Respigam	Retisert	Retrovir	Riastap
Rifadin	Rifater	Rilutek	Rixubis
Ruconest	Rubraca	Ryanodex	Sabril
Saizen	Sclerosol	Serostim	Simulect
Sirturo	So-Aqueous	Somavert	Spherusol
Spinraza	Stimate	Stivarga	Strensiq
Suboxone	Subutex	Sucraid	Sulfamylon
Supprelin	Supprelin La	Sylvant	Synribo
Syprine	Tagrisso	Targretin	Tasigna
Thiola	Thrombate III	Thyroshield	Tobi
Torisel	Totect	Tracleer	Trasylol
Tretten	Triostat	Trisenox	Tyvaso
Ucephan	Unituxin	Uptravi	Urso 250
Vaccinia Immune Globulin (Human) Intravenous	Valchlor	Valstar	Varizig
Venclexta	Ventavis	Vermox	Vesanoid
Vidaza	Vimizim	Vitrasert	Vonvendi
Voraxaze	Vpriv	Vumon	Wilate
Winrho Sd	Xalkori	Xenazine	Yondelis
Zavesca	Zelboraf	Zenapax	Zevalin
Zinecard	Zirgan	Zolinza	Zometa, Zabel
Zorbtive	Zykadia		

72 Orphans that served the mass market first

Abelcet	Abilify	Abraxane	Actemra
Afinitor	Alphanate	Avastin	Biothrax

Blenoxane	Colazal	Cordarone	Crestor
Cytogam	Didronel	Doxil	Droxia
Durezol	Dysport	Enbrel	Erbix
Evista	Feiba	Gammagard Liquid	Gammaplex
Gamunex(R)-C	Genotropin	Geref	Gonal-F
H.P. Acthar Gel	Halaven	HepaGamB	Herceptin
Horizant	Humira	Infumorph	Intron A
Iressa	Ixiaro	Keveyis	Kineret
Lamictal	Lartruvo	Leucovorin Calcium	Lipiodol
Lupron	Lymphoseek	M.V.I.-12	Marinol
Megace	Methotrexate	Mobic	Neulasta
Neupogen	Norditropin	Novoseven Rt	Ocuflox Ophthalmic Solution
Ozurdex	Prograf	Rapamune	Rebetol
ReVia/Trexan	SulfADIAZine	Sylatron	Synarel
Taxol	Technetium Tc99m Sulfur Colloid	Topamax	Viread
Votrient	Xgeva	Xifaxan	Zemplar

84 Orphans approved for multiple orphan diseases

Actimmune	Adcetris	Adempas	Afinitor
Albenza	Ambisome	Antizol	Arzerra
Avastin	Botox	Carnitor	Corifact
Cresemba	Cyramza	Darzalex	Doxil
Ethyol	Exjade	Flolan	Fusilev
Gazyva	Genotropin	Gilotrif	Gleevec
Gliadel	Humatrope	Humira	Iclusig
Ilaris	Imbruvica	Istodax	Jakafi
Kalbitor	Kcentra	Keytruda	Lariam
Leucovorin Calcium	Leukine	Mekinist	Mepron
Myozyme, Lumizyme	Neupogen	Nexavar	Norditropin

Novantrone	Novoseven	Nutropin	Opdivo
Opdivo and Yervoy	Orfadin	Orkambi	Photofrin
Photrexa	Procrit/Epogen	Procysbi	Proleukin
Promacta	Remicade	Revlimid	Rituxan
Roferon A	Salagen	Sandostatin Lar	Secreflo
Sensipar	Signifor	Soliris	Somatuline Depot
Sprycel	Survanta	Tafinlar	Temodar
Thalomid	Thyrogen	Tindamax	Treanda
Urocit-K	Velcade	Xgeva	Xiaflex
Xuriden	Xyrem	Yervoy	Zydelig

KHN used multiple datasets from the FDA, relying primarily on the orphan drug designations and approvals database. If a drug had an approval date before the first marketing approval date in the orphan database, we put it in the mass market first category.