

Rare Disease	Drugs Being Repurposed
Neurodegeneration with brain iron accumulation disorders	Deferiprone
Superficial siderosis	Deferiprone
Generalized lymphatic anomaly, Gorham-Stout disease, Kaposiform lymphangiomatosis, Central conducting lymphatic anomaly	Sirolimus, Everolimus, Alpelisib, Trametinib, Bisphosphonates NOS
CLOVES syndrome	Alpelisib
SLC6A1 epileptic encephalopathy	Glycerol Phenylbutyrate
Congenital disorders of glycosylation	Acetazolamide
GM1 gangliosidosis	Acetylleucine, Miglustat
Mucopolidosis type IV	Cyclodextrin NOS, Fingolimod
Limb-girdle muscular dystrophy	Domagrozumab
Pemphigus, Pemphigoid	Dupilumab, Rituximab
Pachyonychia congenita	Sirolimus, Erlotinib, Chemotherapy NOS
Aicardi-Goutieres syndrome	Baricitinib
STXBP1-related disorders	Glycerol Phenylbutyrate
Jansen's metaphyseal chondrodysplasia	Inverse Agonist NOS
Neurofibromatosis	Brigatinib, Bevacizumab, Everolimus, Trametinib, Cabozantinib, Abemaciclib, Selumetinib
Hypothalamic-pituitary brain tumors, Hypothalamic obesity	Oxytocin
Castleman disease	Sirolimus, Ruxolitinib
SYNGAP1-related syndrome	Clonidine, Lovastatin
Acromegaly	Semaglutide
Hereditary hemorrhagic telangiectasia	Bevacizumab, Pazopanib, Sirolimus, Pomalidomide
CAMK2 mutation disorders	Verapamil, Lithium, Divalproex, Lamotrigine, Sodium Oxybate, Baclofen
PTEN hamartoma tumor syndrome	Everolimus
Neutropenia	Mavorixafor
Smith-Kingsmore syndrome	Sirolimus
Pulmonary alveolar proteinosis	Sargramostim
Infantile neuroaxonal dystrophy	Desipramine
Generalized arterial calcification of infancy, Autosomal recessive hypophosphatemic rickets type 2	Bisphosphonates NOS
Batten disease	Miglustat
RUNX1 mutations	Imatinib, Etanercept, Sirolimus, Dasatinib, Taselisib, Idelalisib
Fanconi anemia	Afatinib, Gefitinib
Eosinophilic diseases	Benralizumab, Dupilumab, Lirentelimab, Tezepelumab, Budesonide
Primary sclerosing cholangitis	Ursodeoxycholic Acid, Norursodeoxycholic Acid, Vancomycin, Fenofibrate, Statins NOS, Sulfasalazine, Cilofexor, Volixibat
Snyder-Robinson syndrome	Sodium Phenylbutyrate, Eflornithine
Giant axonal neuropathy	Sirolimus, Tacrolimus, Prednisone, Metformin, Estradiol Valerate, Tazarotene
Congenital muscular dystrophy	Omigapil
Alkaptonuria	Nitisinone
Chordoma	Afatinib, Cetuximab, Pemetrexed, Palbociclib, Nivolumab
Lymphangiomyomatosis	Sirolimus
ALS	Sunitinib, Bosutinib, Tauroursodeoxycholic Acid