Texas Compassionate Use Program
Qualifying Conditions

- 3-hydroxy-3-methylglutaryl-CoA lyase deficiency
- 5-aminoimidazole-4-carboxamide ribonucleotide transformylase deficiency

A

- Adenylosuccinate synthase Deficiency
- Alexander disease
- Alpers-Huttenlocher syndrome
- ALS (Amyotrophic Lateral Sclerosis)
- Alzheimer's Disease and other dementias
- Amyloidoses
- Argyrophilic Grain Disease
- Aromatic L-amino acid decarboxylase deficiency
- Asparylglucosaminuria
- Ataxia neuropathy spectrum
- Autism and other spectrum disorders

B

- Bidirectional enzyme deficiency
- Biopterin Defects

C

- Canavan disease
- Cancer *
- Central Core
- Cerebral Autosomal Dominant Arteriopathy with Sub-cortical Infarcts and Leukoencephalopathy
- Cerebral Palsy
- Charcot Marie Tooth and related hereditary neuropathies
- Childhood Myocerebrohepatopathy spectrum
- Congenital Disorders of Glycosylation
- Corticobasal Degeneration
- Creatine Disorders
- Creatine Transporter Defect, also known as SLC 6A8
- Creutzfeldt-Jakob Disease
- CTE (Chronic Traumatic Encephalopathy)

D

- Dementia with Lewy Bodies
- Deoxyguanisine kinase deficiency
- Dihydropirimidinase Deficiency
- Dihydropteridine reductase
- Dihydropyrimidine dehydrogenase Deficiency
- Duchenne Muscular Dystrophy

E

- Epilepsy

F

- Facioscapulohumeral Muscular Dystrophy

- Familial or Sporadic Fatal Insomnia
- Familial Spastic Paraplegia
- Farber Disease
- Fatty Acid Oxidation
- Freidreich's Ataxia
- Frontotemporal dementia and parkinsonism linked to chromosome 17 caused by mutations in MAPT gene
- Frontotemporal Lobar Degeneration

G

- Galactosemia
- Galactosialidosis
- Gaucher Type 2 and Type 3
- Gerstmann-Straussler-Scheinker Disease
- Globular Glial Tauopathy
- Glutaric acidemia type 1
- Glycine encephalopathy, also known as non-ketotic hyperglycinemia
- Glycogen Storage-Lysosomal: Pompe Disease
- GM1 gangliosidosis
- GM2 gangliosidosis also known as Tay-sachs and Sandhoff Disease
- Guanidinoacetate methytransferase deficiency
- Guanosine triphosphate cyclohydrolase deficiency

H

- Homocysteine re-methylation defects
- Huntington's Disease
- Hypoxanthine-guanine phosophoribosyltransferase Deficiency also known as Lesch-Nyhan disease

K

- Kearn Sayers Syndrome
- Krabbe
- Kuru

L

- L-2-hydroxyglutaric aciduria
- L-Arginine/glycine amidinotransferase deficiency
- Leukodystrophy
- Lewy Body Disorders
- Long-chain L-3 hydroxyacyl-CoA dehydrogenase deficiency
- Lysosomal Storage Diseases

M

- Mannosidosis
- Manosidosis alpha and beta
- Maple Syrup Urine Disease
- Metachromatic leukodystrophy
- Metal Metabolism
- Methylenetetrahydrofolate reductase deficiency severe variant
- Mitochondrial Conditions
- Mitochondrial Depletion syndromes types 1 through 14
- Mitochondrial Encephalopathy Lactic Acidosis Stroke
- Mitochondrial Encephalopathy Ragged Red Fiber

- Mitochondrial neurogastrointestinal encephalopathy
- Monoamine oxidase deficiency
- Motor Neuron Disease
- MS (Multiple Sclerosis)
- Mucolipidoses
- Mucolipidoses Type II, also known as Inclusion Cell disease
- Mucolipidoses Type III, also known as pseudo-Hurler polydystrophy
- Mucopolysaccaridosis
- Mucopolysaccharidosis Type I, also known as Hurler Syndrome or Scheie Syndrome
- Mucopolysaccharidosis Type II, also known as Hunter Syndrome
- Mucopolysaccharidosis Type III, also known as Sanfilippo A and B
- Mucopolysaccharidosis Type IV, also known as Maroteaux-Lamy
- Mucopolysaccharidosis Type VII, also known as Sly
- Multiple Sulfatase deficiency
- Multiple System Atrophy
- Muscular Dystrophies
- Myoclonic epilepsy myopathy sensory ataxia

N

- Neimann Pick Type A and B
- Neimann Pick Type C
- Neonatal Adrenoleukodystrophy
- Neurodegeneration with brain iron accumulation
- Neurofibrillary Tangle dementia, also known as Primary Age-related Tauopathy
- Neuronal ceroid lipofuscinosis types 1-10 including Batten Disease
- Neuropathy, Ataxia, and Retinitis Pigmentosa
- Neurotransmitter defects

O

- Oligosaccharidoses

P

- Pantothenate Kinase Associated Neurodegeneration
- Parkinson's Disease
- Pelizaeus-Merzbacher disease
- Peripheral Neuropathies
- Peripheral neuropathy types 1 through 4
- Peroxisomal biosynthesis defects
- Peroxisomal Disorders
- Pick Disease
- Polymerase G Related Disorders
- Polyol disorders
- Primary Lateral Sclerosis
- Prion Diseases
- Progressive Choreas
- Progressive dystonias DYT genes 1 through 20
- Progressive Muscular Atrophy
- Progressive Supranuclear Palsy
- Pterin-4-carbinolamine dehydratase
- PTSD *
- Purine and Pyrimidine Defects
- Pyruvate Carboxylase Deficiency
- Pyruvate Dehydrogenase Deficiency

- Pyruvoyl-tetahydropterin synthase

R

- Refsum Disease
- Respiratory chain disorders complex 1 through 4 defects: Co Q biosynthesis defects
- RRM2B-related mitochondrial disease

S

- Salidosis
- Schindler
- Segawa Diease, also known as Dopamine Responsive Dystonia
- Sepiapterin reductase
- Sialidosis
- Spasticity
- Sphingolipidoses
- Spinal Muscular Atrophy
- Spinal-bulbar muscular atrophy
- Spinocerebellar ataxia
- Subacute necrotizing encephalopathy, also known as Leigh syndrome
- SUCLG1-related mitochondrial DNA depletion syndrome, encephalomyopathic form with methylmalonic aciduria
- Synucleinopathies

T

- Tauopathies
- Thymidine Kinase
- Transactive response DNA-binding protein-43 (TDP-43) Proteinopathies
- Trifunctional protein deficiency

V

- Vascular dementia

W

- Wilson Disease

X

- X-linked adrenoleukodystrophy

Z

- Zellweger syndrome

* Effective September 1, 2021