

Compassionate Allowances Conditions 1-225

Acute Leukemia	Batten Disease	Degos Disease	Glioblastoma Multiforme (Brain Cancer)
Adrenal Cancer with distant metastases or inoperable, unresectable, or recurrent	Beta Thalassemia Major	Dravet Syndrome	Glioma Grade III and IV
Adult Non-Hodgkin Lymphoma	Bilateral Optic Atrophy - Infantile	Early-Onset Alzheimer's Disease	Glutaric Acidemia - Type II
Adult Onset Huntington Disease	Bilateral Retinoblastoma	Edwards Syndrome	Head and Neck Cancers
Aicardi-Goutieres Syndrome	Bladder Cancer with distant metastases or inoperable or unresectable	Eisenmenger Syndrome	Heart Transplant Graft Failure
Alexander Disease (ALX) - Neonatal and Infantile	Breast Cancer with distant metastases or inoperable or unresectable	Endometrial Stromal Sarcoma	Heart Transplant Wait List - 1A/1B
Allan-Herndon-Dudley Syndrome	Canavan Disease (CD)	Endomyocardial Fibrosis	Hemophagocytic Lymphohistiocytosis - Familial Type
Alobar Holoprosencephaly	Carcinoma of Unknown Primary Site	Ependymblastoma (Child Brain Cancer)	Hepatoblastoma
Alpers Disease	Cardiac Amyloidosis – AL Type	Erdheim Chester Disease	Hepatopulmonary Syndrome
Alpha Mannosidosis - Types II and III	Caudal Regression Syndrome - Types III and IV	Esophageal Cancer	Hepatorenal Syndrome
ALS/Parkinsonism Dementia Complex	Cerebro Oculo Facio Skeletal (COFS) Syndrome	Esthesioneuroblastoma	Histiocytosis Syndromes
Alstrom Syndrome	Cerebrotendinous Xanthomatosis	Ewing Sarcoma	Hoyeaal-Hreidarsson Syndrome
Alveolar Soft Part Sarcoma	Child Lymphoblastic Lymphoma	Farber Disease (FD) - Infantile	Hutchinson-Gilford Progeria Syndrome
Amegakaryocytic Thrombocytopenia	Child Lymphoma	Fatal Familial Insomnia	Hydranencephaly
Amyotrophic Lateral Sclerosis (ALS) - Adult	Child Neuroblastoma with distant metastases or recurrent	Fibrodysplasia Ossificans Progressiva	Hypocomplementemic Urticarial Vasculitis Syndrome
Anaplastic Adrenal Cancer – Adult with distant metastases or inoperable, unresectable or recurrent	Chondrosarcoma	Follicular Dendritic Cell Sarcoma	Hypophosphatasia - Perinatal (Lethal) and Infantile Onset Types
Angelman Syndrome	Chronic Idiopathic Intestinal Pseudo Obstruction	Friedreichs Ataxia (FRDA)	Hypoplastic Left Heart Syndrome
Angiosarcoma	Chronic Myelogenous Leukemia (CML) - Blast Phase	Frontotemporal Dementia (FTD), Picks Disease -Type A - Adult	I Cell disease
Aortic Atresia	Coffin-Lowry Syndrome	Fryns Syndrome	Idiopathic Pulmonary Fibrosis
Aplastic Anemia	Congenital Lymphedema	Fucosidosis - Type I	Infantile Free Sialic Acid Storage Disease
Astrocytoma - Grade III and IV	Cornelia de Lange Syndrome - Classic Form	Fukuyama Congenital Muscular Dystrophy	Infantile Neuroaxonal Dystrophy (INAD)
Ataxia Telangiectasia	Corticobasal Degeneration	Fulminant Giant Cell Myocarditis	Infantile Neuronal Ceroid Lipofuscinoses
Atypical Teratoid/Rhabdoid Tumor	Creutzfeldt-Jakob Disease (CJD) - Adult	Galactosialidosis - Early and Late Infantile Types	Inflammatory Breast Cancer
	Cri du Chat Syndrome	Gallbladder Cancer	
	De Sanctis Cacchione Syndrome	Gaucher Disease (GD) - Type 2	
		Giant Axonal Neuropathy	

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Intracranial Hemangiopericytoma	Malignant Renal Rhabdoid Tumor	Niemann-Pick Disease-Type C	Pompe Disease - Infantile
Jervell and Lange-Nielsen Syndrome	Mantle Cell Lymphoma (MCL)	Nonketotic Hyperglycinemia	Primary Central Nervous System Lymphoma
Joubert Syndrome	Maple Syrup Urine Disease	Non-Small Cell Lung Cancer	Primary Effusion Lymphoma
Junctional Epidermolysis Bullosa - Lethal Type	Marshall-Smith Syndrome	Obliterative Bronchiolitis	Primary Progressive Aphasia
Juvenile Onset Huntington Disease	Mastocytosis - Type IV	Ohtahara Syndrome	Progressive Bulbar Palsy
Kidney Cancer	MECP2 Duplication Syndrome	Oligodendroglioma Brain Cancer - Grade III	Progressive Multifocal Leukoencephalopathy
Krabbe Disease (KD) - Infantile	Medulloblastoma - with metastases	Ornithine Transcarbamylase (OTC) Deficiency	Progressive Supranuclear Palsy
Kufs Disease Type A and B	Menkes Disease - Classic or Infantile Onset Form	Orthochromatic Leukodystrophy with Pigmented Glia	Prostate Cancer - Hormone Refractory Disease - or with visceral metastases
Large Intestine Cancer	Merkel Cell Carcinoma - with metastases	Osteogenesis Imperfecta (OI) - Type II	Pulmonary Atresia
Late Infantile Neuronal Ceroid Lipofuscinoses	Merosin Deficient Congenital Muscular Dystrophy	Osteosarcoma, formerly known as Bone Cancer - with distant metastases, or inoperable or unresectable	Pulmonary Kaposi Sarcoma
Leigh's Disease	Metachromatic Leukodystrophy (MLD) - Late Infantile	Ovarian Cancer - with distant metastases or inoperable or unresectable	Retinopathy of Prematurity - Stage V
Leiomyosarcoma	Mitral Valve Atresia	Pallister-Killian Syndrome	Rett (RTT) Syndrome
Leptomeningeal Carcinomatosis	Mixed Dementia	Pancreatic Cancer	Revesez Syndrome
Lesch-Nyhan Syndrome (LNS)	MPS I Hurler Syndrome	Paraneoplastic Pemphigus	Rhabdomyosarcoma
Lewy Body Dementia	MPS II Hunter Syndrome	Patau Syndrome	Rhizomelic Chondrodysplasia Punctata
Liposarcoma - metastatic or recurrent	MPS III Sanfilippo Syndrome	Pearson Syndrome	Roberts Syndrome
Lissencephaly Liver Cancer	Mucosal Malignant Melanoma	Pelizaeus-Merzbacher Disease - Classic Form	Salivary Cancers
Liver Cancer	Multicentric Castleman Disease	Pelizaeus-Merzbacher Disease - Connatal Form	Sandhoff Disease
Lowe Syndrome	Multiple System Atrophy	Peripheral Nerve Cancer – metastatic or recurrent	Schindler Disease - Type 1
Lymphomatoid Granulomatosis - Grade III	Myoclonic Epilepsy with Ragged Red Fibers Syndrome	Peritoneal Mesothelioma	Seckel Syndrome
Malignant Brain Stem Gliomas - Childhood	Neonatal Adrenoleukodystrophy	Peritoneal Mucinous Carcinomatosis	Severe Combined Immunodeficiency - Childhood
Malignant Ectomesenchymoma	Nephrogenic Systemic Fibrosis	Perry Syndrome	Single Ventricle
Malignant Gastrointestinal Stromal Tumor	Neurodegeneration with Brain Iron Accumulation - Type 1 and Type 2	Phelan- McDermid Syndrome	Sinonasal Cancer
Malignant Germ Cell Tumor	NFU-1 Mitochondrial Disease	Pleural Mesothelioma	Sjögren-Larsson Syndrome
Malignant Multiple Sclerosis	Niemann-Pick Disease (NPD) - Type A		Skin Malignant Melanoma
			Small Cell Cancer (Large Intestine, Prostate, or Thymus)

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Small Cell Cancer of the Female
Genital Tract

Small Cell Lung Cancer

Small Intestine Cancer

Smith Lemli Opitz Syndrome

Soft Tissue Sarcoma - with distant
metastases or recurrent

Spinal Muscular Atrophy (SMA)
- Types 0 And 1

Spinal Nerve Root Cancer -
metastatic or recurrent

Spinocerebellar Ataxia

Stiff Person Syndrome

Stomach Cancer - with distant
metastases or inoperable, unresectable or
recurrent

Subacute Sclerosing
Panencephalitis

Tabes Dorsalis

Tay Sachs Disease - Infantile
Type

Thanatophoric Dysplasia - Type
1

Thyroid Cancer

Transplant Coronary Artery
Vasculopathy

Tricuspid Atresia

Ullrich Congenital Muscular
Dystrophy

Ureter Cancer – with distant
metastases or inoperable or unresectable
or recurrent

Usher Syndrome - Type I

Ventricular Assist Device
Recipient – Left, Right, or
Biventricular

Walker Warburg Syndrome

Wolf-Hirschhorn Syndrome

Wolman Disease

X-Linked Lymphoproliferative
Disease

X-Linked Myotubular Myopathy

Xeroderma Pigmentosum

Zellweger Syndrome