

Compassionate Allowances Conditions 1-266

Acute Leukemia	Atypical Teratoid/Rhabdoid Tumor	Chronic Myelogenous Leukemia (CML) - Blast Phase	Fatal Familial Insomnia
Adrenal Cancer with distant metastases or inoperable, unresectable, or recurrent	Batten Disease	CIC-rearranged Sarcoma	Fibrodysplasia Ossificans Progressiva
Adult Non-Hodgkin Lymphoma	Beta Thalassemia Major	Coffin-Lowry Syndrome	Fibrolamellar Cancer
Adult Onset Huntington Disease	Bilateral Optic Atrophy - Infantile	Congenital Myotonic Dystrophy	Follicular Dendritic Cell Sarcoma – metastatic or recurrent
Aicardi-Goutieres Syndrome	Bilateral Retinoblastoma	Congenital Lymphedema	Friedreichs Ataxia (FRDA)
Alexander Disease (ALX) - Neonatal and Infantile	Bladder Cancer with distant metastases or inoperable or unresectable	Congenital Zika Syndrome	Frontotemporal Dementia (FTD), Picks Disease -Type A - Adult
Allan-Herndon-Dudley Syndrome	Blastic Plasmacytoid Dendritic Cell Neoplasm	Cornelia de Lange Syndrome - Classic Form	Fryns Syndrome
Alobar Holoprosencephaly	Breast Cancer with distant metastases or inoperable or unresectable	Corticobasal Degeneration	Fucosidosis - Type I
Alpers Disease	CACH – Vanishing White Matter Disease – Infantile and Childhood Onset forms	Creutzfeldt-Jakob Disease (CJD) - Adult	Fukuyama Congenital Muscular Dystrophy
Alpha Mannosidosis - Types II and III	Canavan Disease (CD)	Cri du Chat Syndrome	Fulminant Giant Cell Myocarditis
ALS/Parkinsonism Dementia Complex	Carcinoma of Unknown Primary Site	De Sanctis Cacchione Syndrome	Galactosialidosis - Early and Late Infantile Types
Alstrom Syndrome	Cardiac Amyloidosis – AL Type	Degos Disease – Systematic	Gallbladder Cancer
Alveolar Soft Part Sarcoma	Caudal Regression Syndrome - Types III and IV	Desmoplastic Mesothelioma	Gaucher Disease (GD) - Type 2
Amegakaryocytic Thrombocytopenia	CDKL5 Deficiency Disorder	Desmoplastic Small Round Cell Tumors	Gerstmann-Straussler-Scheinker Disease
Amyotrophic Lateral Sclerosis (ALS) - Adult	Cerebro Oculo Facio Skeletal (COFS) Syndrome	Dravet Syndrome	Giant Axonal Neuropathy
Anaplastic Adrenal Cancer – Adult with distant metastases or inoperable, unresectable or recurrent	Cerebrotendinous Xanthomatosis	Duchenne Muscular Dystrophy - Adult	Glioblastoma Multiforme (Brain Cancer)
Angelman Syndrome	Charlevoix-Saguenay Spastic Ataxia	Early-Onset Alzheimer’s Disease	Glioma Grade III and IV
Angiosarcoma	Child Lymphoblastic Lymphoma	Edwards Syndrome	Glutaric Acidemia - Type II
Angioimmunoblastic T-cell Lymphoma	Child Lymphoma	Eisenmenger Syndrome	GM1 Gangliosidosis - Infantile and Juvenile Forms
Aortic Atresia	Child Neuroblastoma with distant metastases or recurrent	Endometrial Stromal Sarcoma	Head and Neck Cancers
Aplastic Anemia	Chondrosarcoma with multimodal therapy	Endomyocardial Fibrosis	Heart Transplant Graft Failure
Astrocytoma - Grade III and IV	Choroid Plexus Carcinoma	Ependymoblastoma (Child Brain Cancer)	Heart Transplant Wait List - 1A/1B
Ataxia Telangiectasia	Chronic Idiopathic Intestinal Pseudo Obstruction	Erdheim Chester Disease	Hemophagocytic Lymphohistiocytosis - Familial Type
		Esophageal Cancer	
		Esthesioneuroblastoma	
		Ewing Sarcoma	
		Farber Disease (FD) - Infantile	

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Hepatoblastoma	Krabbe Disease (KD) - Infantile	Menkes Disease - Classic or Infantile Onset Form	Non-Small Cell Lung Cancer
Hepatopulmonary Syndrome	Kufs Disease Type A and B	Merkel Cell Carcinoma - with metastases	Obliterative Bronchiolitis
Hepatorenal Syndrome	Large Intestine Cancer	Merosin Deficient Congenital Muscular Dystrophy	Ohtahara Syndrome
Histiocytosis Syndromes	Late Infantile Neuronal Ceroid Lipofuscinoses	Metachromatic Leukodystrophy (MLD) - Late Infantile	Oligodendroglioma Brain Cancer - Grade III
Hoyeaaal-Hreidarsson Syndrome	Leigh's Disease	Microvillus Inclusion Disease -- Child	Ornithine Transcarbamylase (OTC) Deficiency
Hutchinson-Gilford Progeria Syndrome	Leiomyosarcoma	Mitral Valve Atresia	Orthochromatic Leukodystrophy with Pigmented Glia
Hydranencephaly	Leptomeningeal Carcinomatosis	Mixed Dementias	Osteogenesis Imperfecta (OI) - Type II
Hypocomplementemic Urticarial Vasculitis Syndrome	Lesch-Nyhan Syndrome (LNS)	Mowat-Wilson Syndrome	Osteosarcoma, formerly known as Bone Cancer - with distant metastases, or inoperable or unresectable
Hypophosphatasia - Perinatal (Lethal) and Infantile Onset Types	Lewy Body Dementia	MPS I Hurler Syndrome	Ovarian Cancer - with distant metastases or inoperable or unresectable
Hypoplastic Left Heart Syndrome	Liposarcoma - metastatic or recurrent	MPS II Hunter Syndrome	Pallister-Killian Syndrome
I Cell disease	Lissencephaly	MPS III Sanfilippo Syndrome	Pancreatic Cancer
Idiopathic Pulmonary Fibrosis	Liver Cancer	Mucosal Malignant Melanoma	Paraneoplastic Pemphigus
Infantile Free Sialic Acid Storage Disease	Lowe Syndrome	Multicentric Castleman Disease	Patau Syndrome
Infantile Neuroaxonal Dystrophy (INAD)	Lymphomatoid Granulomatosis - Grade III	Multiple System Atrophy	Pearson Syndrome
Infantile Neuronal Ceroid Lipofuscinoses	Malignant Brain Stem Gliomas - Childhood	Myelodysplastic Syndrome with Excess Blasts	Pelizaeus-Merzbacher Disease - Classic Form
Inflammatory Breast Cancer	Malignant Ectomesenchymoma	Myoclonic Epilepsy with Ragged Red Fibers Syndrome	Pelizaeus-Merzbacher Disease - Connatal Form
Intracranial Hemangiopericytoma	Malignant Gastrointestinal Stromal Tumor	Neonatal Adrenoleukodystrophy	Pericardial Mesothelioma
Jervell and Lange-Nielsen Syndrome	Malignant Germ Cell Tumor	Nephrogenic Systemic Fibrosis	Peripheral Nerve Cancer – metastatic or recurrent
Joubert Syndrome	Malignant Multiple Sclerosis	Neurodegeneration with Brain Iron Accumulation - Type 1 and Type 2	Peritoneal Mesothelioma
Junctional Epidermolysis Bullosa - Lethal Type	Malignant Renal Rhabdoid Tumor	NFU-1 Mitochondrial Disease	Peritoneal Mucinous Carcinomatosis
Juvenile Onset Huntington Disease	Mantle Cell Lymphoma (MCL)	Nicolaides-Baraister Syndrome	Perry Syndrome
Kidney Cancer	Maple Syrup Urine Disease	Niemann-Pick Disease (NPD) - Type A	Phelan- McDermid Syndrome
Kleefstra Syndrome	Marshall-Smith Syndrome	Niemann-Pick Disease-Type C	Pitt Hopkins Syndrome
	Mastocytosis - Type IV	Nonketotic Hyperglycinemia	Pleural Mesothelioma
	MECP2 Duplication Syndrome		
	Medulloblastoma - with metastases		
	Megacystis Microcolon Intestinal Hypoperistalsis Syndrome		

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NUT Carcinoma	Salivary Cancers	Stomach Cancer - with distant metastases or inoperable, unresectable or recurrent
Pfeiffer Syndrome - Types 2 and 3	Sandhoff Disease	Subacute Sclerosing Panencephalitis
Pompe Disease – Infantile	Sarcomatoid Mesothelioma	SYNGAP1-related NSID
Pontocerebellar Hypoplasia	Schindler Disease - Type 1	Tabes Dorsalis
Posterior Cortical Atrophy	SCN8A-related Epilepsy with Encephalopathy	Tay Sachs Disease - Infantile Type
Primary Central Nervous System Lymphoma	Seckel Syndrome	Taybi-Linder Syndrome
Primary Effusion Lymphoma	Secondary Adenocarcinoma of the Brain	Tetrasomy 18p
Primary Peritoneal Cancer	Severe Combined Immunodeficiency - Childhood	Thanatophoric Dysplasia - Type 1
Primary Progressive Aphasia	Single Ventricle	Thyroid Cancer
Progressive Bulbar Palsy	Sinonasal Cancer	Transplant Coronary Artery Vasculopathy
Progressive Multifocal Leukoencephalopathy	Sjögren-Larsson Syndrome	Tricuspid Atresia
Progressive Supranuclear Palsy	Skin Malignant Melanoma – with metastases	Ullrich Congenital Muscular Dystrophy
Prostate Cancer - Hormone Refractory Disease - or with visceral metastases	Small Cell Cancer (Large Intestine, Ovary, Prostate, or Thymus)	Ureter Cancer – with distant metastases or inoperable or unresectable or recurrent
Pulmonary Atresia	Small Cell Cancer of the Female Genital Tract	Usher Syndrome - Type I
Pulmonary Kaposi Sarcoma	Small Cell Lung Cancer	Ventricular Assist Device Recipient – Left, Right, or Biventricular
Refractory Hodgkin Lymphoma	Small Intestine Cancer	Walker Warburg Syndrome
Renal Amyloidosis – AL Type	Smith Lemli Opitz Syndrome	Wolf-Hirschhorn Syndrome
Renpenning Syndrome	Soft Tissue Sarcoma - with distant metastases or recurrent	Wolman Disease
Retinopathy of Prematurity - Stage V	Spinal Muscular Atrophy (SMA) - Types 0 And 1	Xeroderma Pigmentosum
Rett (RTT) Syndrome	Spinal Nerve Root Cancer - metastatic or recurrent	X-Linked Lymphoproliferative Disease
Revesez Syndrome	Spinocerebellar Ataxia	X-Linked Myotubular Myopathy
Rhabdomyosarcoma	Superficial Siderosis of the Central Nervous System	Zellweger Syndrome
Rhizomelic Chondrodysplasia Punctata	Stiff Person Syndrome	
Richter Syndrome		
Roberts Syndrome		
Rubinstein-Taybi Syndrome		