

Compassionate Allowances Conditions 1-278

Ip36 Deletion Syndrome	Ataxia Telangiectasia Atypical Teratoid/Rhabdoid Tumor	Choroid Plexus Carcinoma	Esthesioneuroblastoma
Acute Leukemia Adrenal Cancer with distant metastases or inoperable, unresectable, or recurrent	Batten Disease	Chronic Idiopathic Intestinal Pseudo Obstruction	Ewing Sarcoma
Adult Non-Hodgkin Lymphoma	Beta Thalassemia Major	Chronic Myelogenous Leukemia (CML) - Blast Phase	Farber Disease (FD) - Infantile
Adult Onset Huntington Disease	Bilateral Optic Atrophy - Infantile	CIC-rearranged Sarcoma	Fatal Familial Insomnia
Aicardi-Goutieres Syndrome	Bilateral Retinoblastoma	Coffin-Lowry Syndrome	Fibrodysplasia Ossificans Progressiva
Alexander Disease (ALX) - Neonatal and Infantile	Bladder Cancer with distant metastases or inoperable or unresectable	Congenital Myotonic Dystrophy	Fibrolamellar Cancer
Allan-Herndon-Dudley Syndrome	Blastic Plasmacytoid Dendritic Cell Neoplasm	Congenital Lymphedema	Follicular Dendritic Cell Sarcoma- metastatic or recurrent
Alobar Holoprosencephaly	Breast Cancer with distant metastases or inoperable or unresectable	Congenital Zika Syndrome	FOXP1 Syndrome
Alpers Disease	CACH – Vanishing White Matter Disease – Infantile and Childhood Onset forms	Cornelia de Lange Syndrome - Classic Form	Friedreichs Ataxia (FRDA)
Alpha Mannosidosis - Types II and III	Calciphylaxis	Corticobasal Degeneration	Frontotemporal Dementia (FTD), Picks Disease -Type A - Adult Fryns Syndrome
ALS/Parkinsonism Dementia Complex	Canavan Disease (CD)	Creutzfeldt-Jakob Disease (CJD) - Adult	Fucosidosis - Type I
Alstrom Syndrome	Carcinoma of Unknown Primary Site	Cri du Chat Syndrome	Fukuyama Congenital Muscular Dystrophy
Alveolar Soft Part Sarcoma	Cardiac Amyloidosis – AL Type	De Sanctis Cacchione Syndrome	Fulminant Giant Cell Myocarditis
Amegakaryocytic Thrombocytopenia	Caudal Regression Syndrome - Types III and IV	Degos Disease – Systematic	Galactosialidosis - Early and Late Infantile Types
Amyotrophic Lateral Sclerosis (ALS) - Adult	CDKL5 Deficiency Disorder	Desmoplastic Mesothelioma	Gallbladder Cancer
Anaplastic Adrenal Cancer – Adult with distant metastases or inoperable, unresectable or recurrent	Cerebro Oculo Facio Skeletal (COFS) Syndrome	Desmoplastic Small Round Cell Tumors	Gaucher Disease (GD) - Type 2
Anaplastic Ependymoma	Cerebrotendinous Xanthomatosis	Dravet Syndrome	Gerstmann-Straussler-Scheinker Disease
Angelman Syndrome	Charlevoix-Saguenay Spastic Ataxia	Duchenne Muscular Dystrophy - Adult	Giant Axonal Neuropathy
Angiosarcoma	Child Lymphoblastic Lymphoma	Early-Onset Alzheimer’s Disease	Glioblastoma Multiforme (Brain Cancer)
Angioimmunoblastic T-cell Lymphoma	Child Lymphoma	Edwards Syndrome	Glioma Grade III and IV
Aortic Atresia	Child Neuroblastoma with distant metastases or recurrent	Eisenmenger Syndrome	Glutaric Acidemia - Type II
Aplastic Anemia	Cholangiocarcinoma	Endometrial Stromal Sarcoma	GM1 Gangliosidosis - Infantile and Juvenile Forms
Astrocytoma - Grade III and IV	Chondrosarcoma with multimodal therapy	Endomyocardial Fibrosis	Head and Neck Cancers
		Ependyoblastoma (Child Brain Cancer)	Heart Transplant Graft Failure
		Erdheim Chester Disease	Heart Transplant Wait List - 1A/1B
		Esophageal Cancer	

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

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Peripheral Nerve Cancer – metastatic or recurrent	Retinopathy of Prematurity - Stage V	Small Intestine Cancer	Ventricular Assist Device Recipient – Left, Right, or Biventricular
Peritoneal Mesothelioma	Rett (RTT) Syndrome	Smith Lemli Opitz Syndrome	Walker Warburg Syndrome
Peritoneal Mucinous Carcinomatosis	Revesez Syndrome	Soft Tissue Sarcoma - with distant metastases or recurrent	Wolf-Hirschhorn Syndrome
Perry Syndrome	Rhabdomyosarcoma	Spinal Muscular Atrophy (SMA) - Types 0 And 1	Wolman Disease
Phelan- McDermid Syndrome	Rhizomelic Chondrodysplasia Punctata	Spinal Nerve Root Cancer - metastatic or recurrent	Xeroderma Pigmentosum
Pineoblastoma – Childhood	Richter Syndrome	Spinocerebellar Ataxia	X-Linked Lymphoproliferative Disease
Pitt Hopkins Syndrome	Roberts Syndrome	Superficial Siderosis of the Central Nervous System	X-Linked Myotubular Myopathy
Pleural Mesothelioma	Rubinstein-Taybi Syndrome	Stiff Person Syndrome	Zellweger Syndrome
Pfeiffer Syndrome - Types 2 and 3	Salivary Cancers	Stomach Cancer - with distant metastases or inoperable, unresectable or recurrent	
Pompe Disease – Infantile	Sandhoff Disease	Subacute Sclerosing Panencephalitis	
Pontocerebellar Hypoplasia	Sarcomatoid Carcinoma of the Lung – Stages II-IV	SYNGAP1-related NSID	
Posterior Cortical Atrophy	Sarcomatoid Mesothelioma	Tabes Dorsalis	
Primary Central Nervous System Lymphoma	Schindler Disease - Type 1	Tay Sachs Disease - Infantile Type	
Primary Effusion Lymphoma	SCN8A-related Epilepsy with Encephalopathy	Taybi-Linder Syndrome	
Primary Omental Cancer	Seckel Syndrome	Tetrasomy 18p	
Primary Peritoneal Cancer	Secondary Adenocarcinoma of the Brain	Thanatophoric Dysplasia - Type 1	
Primary Progressive Aphasia	Severe Combined Immunodeficiency - Childhood	Thyroid Cancer	
Progressive Bulbar Palsy	Single Ventricle	Transplant Coronary Artery Vasculopathy	
Progressive Multifocal Leukoencephalopathy	Sinonasal Cancer	Tricuspid Atresia	
Progressive Supranuclear Palsy	Sjögren-Larsson Syndrome	Trisomy 9	
Prostate Cancer - Hormone	Skin Malignant Melanoma – with Metastases	Ullrich Congenital Muscular Dystrophy	
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		
Refractory Hodgkin Lymphoma			
Renal Amyloidosis – AL Type			
Renpenning Syndrome			