

## Compassionate Allowances Conditions 1-287

1p36 Deletion Syndrome	Aplastic Anemia	Child Lymphoma	Edwards Syndrome (Trisomy 18)
Acute Leukemia	Astrocytoma - Grade III and IV	Child Neuroblastoma - with distant metastases or recurrent	Eisenmenger Syndrome
Adrenal Cancer with distant metastases or inoperable, unresectable or recurrent	Ataxia Telangiectasia	Child T-Cell Lymphoblastic Lymphoma	Endometrial Stromal Sarcoma
Adult Heart Transplant Wait List – Status Levels 1-4	Atypical Teratoid/Rhabdoid Tumor	Cholangiocarcinoma	Endomyocardial Fibrosis
Adult Non-Hodgkin Lymphoma	Bainbridge-Ropers Syndrome	Chondrosarcoma - with multimodal therapy	Ependyoblastoma (Child Brain Cancer)
Adult Onset Huntington Disease	Batten Disease	Choroid Plexus Carcinoma	Erdheim Chester Disease
Aicardi-Goutieres Syndrome	Beta Thalassemia Major	Chronic Idiopathic Intestinal Pseudo Obstruction	Esophageal Cancer
Alexander Disease (ALX) - Child	Bilateral Optic Atrophy - Infantile	Chronic Myelogenous Leukemia (CML) - Blast Phase	Esthesioneuroblastoma
Allan-Herndon-Dudley Syndrome	Bilateral Retinoblastoma	CIC-rearranged Sarcoma	Ewing Sarcoma
Alobar Holoprosencephaly	Bladder Cancer with distant metastases or inoperable or unresectable	Coffin-Lowry Syndrome	Farber’s Disease (FD) - Infantile
Alpers Disease	Blastic Plasmacytoid Dendritic Cell Neoplasm	Congenital Lymphedema	Fatal Familial Insomnia
Alpha Mannosidosis - Types II and III	Breast Cancer with distant metastases or recurrent	Congenital Myotonic Dystrophy	Fibrodysplasia Ossificans Progressiva
ALS/Parkinsonism Dementia Complex	CACH – Vanishing White Matter Disease – Infantile and Childhood Onset Forms	Congenital Zika Syndrome	Fibrolamellar Cancer
Alstrom Syndrome	Calciphylaxis	Cornelia de Lange Syndrome - Classic Form	Follicular Dendritic Cell Sarcoma - metastatic or recurrent
Alveolar Soft Part Sarcoma	Canavan Disease (CD)	Corticobasal Degeneration	FOXC1 Syndrome
Amegakaryocytic Thrombocytopenia	Carcinoma of Unknown Primary Site	Costello Syndrome	Friedreich’s Ataxia (FRDA)
Amyotrophic Lateral Sclerosis (ALS) - Adult	Cardiac Amyloidosis – AL Type	Creutzfeldt-Jakob Disease (CJD) - Adult	Frontotemporal Dementia (FTD), Pick’s Disease, Type A - Adult
Anaplastic Adrenal Cancer – Adult with distant metastases or inoperable, unresectable or recurrent	Caudal Regression Syndrome - Types III and IV	Cri du Chat Syndrome	Fryns Syndrome
Anaplastic Ependymoma	CDKL5 Deficiency Disorder	Degos Disease – Systematic	Fucosidosis - Type I
Anaplastic Thyroid Cancer	Cerebro Oculo Facio Skeletal (COFS) Syndrome	DeSanctis Cacchione Syndrome	Fukuyama Congenital Muscular Dystrophy
Angelman Syndrome	Cerebrotendinous Xanthomatosis	Desmoplastic Mesothelioma	Fulminant Giant Cell Myocarditis
Angioimmunoblastic T-Cell Lymphoma	Charlevoix-Saguenay Spastic Ataxia	Desmoplastic Small Round Cell Tumors	Galactosialidosis - Early and Late Infantile Types
Angiosarcoma	Child Heart Transplant Wait List – Status Levels 1A/1B	Dravet Syndrome	Gallbladder Cancer
Aortic Atresia		Duchenne Muscular Dystrophy - Adult	Gaucher Disease (GD) - Type 2
		Early-Onset Alzheimer’s Disease	Gerstmann-Straussler-Scheinker Disease
			Giant Axonal Neuropathy

## Compassionate Allowances Conditions 1-287

Glioblastoma Multiforme (Adult Brain Cancer)	Infantile Neuronal Ceroid Lipofuscinoses	Malignant Ectomesenchymoma	MPS III
Glioma - Grade III and IV	Inflammatory Breast Cancer (IBC)	Malignant Gastrointestinal Stromal Tumor	Mucosal Malignant Melanoma
Glutaric Acidemia - Type II	Intracranial Hemangiopericytoma	Malignant Germ Cell Tumor	Multicentric Castleman Disease
GM1 - Gangliosidosis - Infantile & Juvenile Forms	Jervell and Lange-Nielsen Syndrome	Malignant Multiple Sclerosis	Multiple System Atrophy
Head and Neck Cancers – with distant metastasis or inoperable or unresectable	Joubert Syndrome	Malignant Renal Rhabdoid Tumor	Myelodysplastic Syndrome with Excess Blasts
Heart Transplant Graft Failure	Junctional Epidermolysis Bullosa - Lethal Type	Mantle Cell Lymphoma (MCL)	Myoclonic Epilepsy with Ragged Red Fibers Syndrome
Hemophagocytic Lymphohistiocytosis – Familial Type (FHLH)	Juvenile Onset Huntington Disease	Maple Syrup Urine Disease	Neonatal Adrenoleukodystrophy
Hepatoblastoma	Kidney Cancer – inoperable or unresectable	Marshall-Smith Syndrome	Neonatal Marfan Syndrome
Hepatocellular Carcinoma	Kleefstra Syndrome	Mastocytosis - Type IV	Nephrogenic Systemic Fibrosis
Hepatopulmonary Syndrome	Krabbe Disease (KD) - Infantile	MECP2 Duplication Syndrome	Neurodegeneration with Brain Iron Accumulation - Types 1 and 2
Hepatorenal Syndrome	Kufs Disease - Type A and B	Medulloblastoma	NFU-1 Mitochondrial Disease
Histiocytic Malignancies	Large Intestine Cancer - with distant metastasis or inoperable, unresectable or recurrent	Megacystis Microcolon Intestinal Hypoperistalsis Syndrome	Nicolaides-Baraitser Syndrome
Histiocytosis Syndromes	Late Infantile Neuronal Ceroid Lipofuscinoses	Megalencephaly-Capillary Malformation Syndrome	Niemann-Pick Disease (NPD) - Type A
Hoyeraal-Hreidarsson Syndrome	Leber Congenital Amaurosis	Menkes Disease - Classic or Infantile Onset Form	Niemann-Pick Disease -Type C
Hutchinson-Gilford Progeria Syndrome	Leigh’s Disease	Merkel Cell Carcinoma - with metastases	Nonketotic Hyperglycinemia
Hydranencephaly	Leiomyosarcoma	Merosin Deficient Congenital Muscular Dystrophy	Non-Small Cell Lung Cancer
Hypocomplementemic Urticarial Vasculitis Syndrome	Leptomeningeal Carcinomatosis	Metachromatic Leukodystrophy (MLD) - Late Infantile	NUT Carcinoma
Hypophosphatasia – Perinatal (Lethal) and Infantile Onset Types	Lesch-Nyhan Syndrome (LNS)	Metastatic Endometrial Adenocarcinoma	Obliterative Bronchiolitis
Hypoplastic Left Heart Syndrome	Lewy Body Dementia	Microvillus Inclusion Disease – Child	Ohtahara Syndrome
I Cell Disease	Liposarcoma - metastatic or recurrent	Mixed Dementias	Oligodendroglioma Brain Cancer - Grade III
Idiopathic Pulmonary Fibrosis	Lissencephaly	Mowat-Wilson Syndrome	Ornithine Transcarbamylase (OTC) Deficiency
Infantile Free Sialic Acid Storage Disease	Lowe Syndrome	MPS I	Orthochromatic Leukodystrophy with Pigmented Glia
Infantile Neuroaxonal Dystrophy (INAD)	Lymphomatoid Granulomatosis - Grade III	MPS II	Osteogenesis Imperfecta (OI) - Type II
	Malignant Brain Stem Gliomas - Childhood		Osteosarcoma, formerly known as Bone Cancer
			Ovarian Cancer - with distant metastases or inoperable or unresectable

## Compassionate Allowances Conditions 1-287

PACSI Syndrome	Primary Progressive Aphasia	Secondary Adenocarcinoma of the Brain	Superficial Siderosis of the Central Nervous System
Pallister-Killian Syndrome	Progressive Bulbar Palsy	Severe Combined Immunodeficiency - Childhood	SYNGAP1-related NSID
Pancreatic Cancer	Progressive Multifocal Leukoencephalopathy	Single Ventricle	Tabes Dorsalis
Paraneoplastic Cerebellar Degeneration	Progressive Supranuclear Palsy	Sinonasal Cancer	Tay Sachs Disease - Infantile Type
Paraneoplastic Pemphigus	Prostate Cancer – Hormone Refractory Disease - or with visceral metastases	Sjögren-Larsson Syndrome	Taybi-Linder Syndrome
Patau Syndrome (Trisomy 13)	Pulmonary Atresia	Skin Malignant Melanoma – with Metastases	Tetrasomy 18p
Pearson Syndrome	Pulmonary Kaposi Sarcoma	Small Cell Cancer of the Female Genital Tract	Thanatophoric Dysplasia, Type 1
Pelizaeus-Merzbacher Disease - Classic Form	Refractory Hodgkin Lymphoma	Small Cell Cancer of the Large Intestine	Transplant Coronary Artery Vasculopathy
Pelizaeus-Merzbacher Disease - Connatal Form	Renal Amyloidosis – AL Type	Small Cell Cancer of the Ovary	Tricuspid Atresia
Pericardial Mesothelioma	Renal Medullary Carcinoma	Small Cell Cancer of the Prostate	Trisomy 9
Peripheral Nerve Cancer – metastatic or recurrent	Renpenning Syndrome	Small Cell Cancer of the Thymus	Ullrich Congenital Muscular Dystrophy
Peritoneal Mesothelioma	Retinopathy of Prematurity – Stage V, Bilateral	Small Cell Lung Cancer	Ureter Cancer – with distant metastases or inoperable, or unresectable or recurrent
Peritoneal Mucinous Carcinomatosis	Rett (RTT) Syndrome	Small Intestine Cancer – with distant metastases or inoperable or recurrent	Usher Syndrome - Type I
Perry Syndrome	Revesz Syndrome	Smith Lemli Opitz Syndrome	Ventricular Assist Device Recipient – Left, Right, or Biventricular
Pfeiffer Syndrome - Types II and III	Rhabdomyosarcoma	Snijders Blok-Campeau Syndrome	Walker Warburg Syndrome
Phelan- McDermid Syndrome	Rhizomelic Chondrodysplasia Punctata	Soft Tissue Sarcoma - with distant metastases or recurrent	Wolf-Hirschhorn Syndrome
Pineoblastoma – Childhood	Richter Syndrome	Spinal Muscular Atrophy (SMA) - Types 0 And 1	Wolman Disease
Pitt Hopkins Syndrome	Roberts Syndrome	Spinal Nerve Root Cancer - metastatic or recurrent	Xeroderma Pigmentosum
Plasmablastic Lymphoma	Rubinstein-Taybi Syndrome	Spinocerebellar Ataxia	X-Linked Lymphoproliferative Disease
Pleural Mesothelioma	Salivary Cancers	Stiff Person Syndrome	X-Linked Myotubular Myopathy
Pompe Disease – Infantile	Sandhoff Disease	Stomach Cancer - with distant metastases or inoperable, unresectable or recurrent	Zellweger Syndrome
Pontocerebellar Hypoplasia	Sarcomatoid Carcinoma of the Lung – Stages II-IV	Subacute Sclerosing Panencephalitis	
Posterior Cortical Atrophy	Sarcomatoid Mesothelioma		
Primary Central Nervous System Lymphoma	Schindler Disease - Type 1		
Primary Effusion Lymphoma	SCN8A-related Epilepsy with Encephalopathy		
Primary Omental Cancer	Seckel Syndrome		
Primary Peritoneal Cancer			