

Compassionate Allowances (CAL) Conditions (300)

1p36 Deletion Syndrome	Atypical Teratoid/Rhabdoid Tumor	Child Neuroblastoma – with distant metastases or recurrent	Erdheim Chester Disease
Acute Leukemia	Au-Kline Syndrome	Child T-Cell Lymphoblastic Lymphoma	Esophageal Cancer
Adrenal Cancer with distant metastases or inoperable, unresectable or recurrent	Bainbridge-Ropers Syndrome	Cholangiocarcinoma	Esthesioneuroblastoma
Adult Heart Transplant Wait List – Status Levels 14	Batten Disease	Chondrosarcoma – with multimodal therapy	Ewing Sarcoma
Adult Non-Hodgkin Lymphoma	Beta Thalassemia Major	Choroid Plexus Carcinoma	Farber Disease (FD) – Infantile
Adult Onset Huntington Disease	Bilateral Anophthalmia	Chronic Idiopathic Intestinal Pseudo Obstruction	Fatal Familial Insomnia
Aicardi-Goutieres Syndrome	Bilateral Optic Atrophy – Infantile	Chronic Myelogenous Leukemia (CML) – Blast Phase	Fibrodysplasia Ossificans Progressiva
Alexander Disease (ALX) – Neonatal and Infantile	Bilateral Retinoblastoma	CIC-rearranged Sarcoma	Fibrolamellar Cancer
Allan-Herndon-Dudley Syndrome	Bladder Cancer with distant metastases or inoperable or unresectable	Coffin-Lowry Syndrome	Follicular Dendritic Cell Sarcoma – metastatic or recurrent
Alobar Holoprosencephaly	Blastic Plasmacytoid Dendritic Cell Neoplasm	Congenital Lymphedema	FOXP1 Syndrome
Alpers Disease	Breast Cancer – with distant metastases or inoperable or recurrent	Congenital Myotonic Dystrophy	Friedreich’s Ataxia (FRDA)
Alpha Mannosidosis – Types II and III	CACH – Vanishing White Matter Disease – Congenital, Infantile, and Early Childhood Onset Forms	Congenital Zika Syndrome	Frontotemporal Dementia (FTD), Pick’s Disease, Type A – Adult
ALS/Parkinsonism Dementia Complex	Calciophylaxis	Cornelia de Lange Syndrome –Classic Form	Fryns Syndrome
Alstrom Syndrome	Canavan Disease (CD)	Corticobasal Degeneration	Fucosidosis – Type I
Alveolar Soft Part Sarcoma	Carcinoma of Unknown Primary Site	Costello Syndrome	Fukuyama Congenital Muscular Dystrophy
Amegakaryocytic Thrombocytopenia	Cardiac Amyloidosis – AL Type	Creutzfeldt-Jakob Disease (CJD) – Adult	Fulminant Giant Cell Myocarditis
Amyotrophic Lateral Sclerosis (ALS) – Adult	Carey-Fineman-Ziter Syndrome	Cri du Chat Syndrome	Galactosialidosis – Early and Late Infantile Types
Anaplastic Adrenal Cancer – Adult with distant metastases or inoperable, unresectable or recurrent	Caudal Regression Syndrome – Types III and IV	Degos Disease – Systematic	Gallbladder Cancer
Anaplastic Ependymoma	CDKL5 Deficiency Disorder	DeSanctis Cacchione Syndrome	Gaucher Disease (GD) – Type 2
Anaplastic Thyroid Cancer	Cerebro Oculo Facio Skeletal (COFS) Syndrome	Desmoplastic Mesothelioma	Gerstmann-Straussler-Scheinker Disease
Angelman Syndrome	Cerebrotendinous Xanthomatosis	Desmoplastic Small Round Cell Tumors	Giant Axonal Neuropathy
Angioimmunoblastic T-Cell Lymphoma	Charlevoix-Saguenay Spastic Ataxia	Dravet Syndrome	Glioblastoma Multiforme (Adult Brain Cancer)
Angiosarcoma	Child Heart Transplant Wait List – Status Levels 1A/1B	Duchenne Muscular Dystrophy — Adult	Glioma – Grade III and IV
Aortic Atresia	Child Lymphoblastic Lymphoma	Early-Onset Alzheimer’s Disease	Glutaric Acidemia – Type II
Aplastic Anemia	Child Lymphoma	Edwards Syndrome (Trisomy 18)	GM1 – Gangliosidosis – Infantile & Juvenile Forms
Astrocytoma – Grade III and IV	Child Medulloblastoma	Eisenmenger Syndrome	Harlequin Ichthyosis – Child
Ataxia Telangiectasia		Endometrial Stromal Sarcoma	Head and Neck Cancers – with distant metastasis or inoperable or unresectable
		Endomyocardial Fibrosis	
		Ependyoblastoma (Child Brain Cancer)	

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Heart Transplant Graft Failure	Krabbe Disease (KD) – Infantile	Megalencephaly-Capillary Malformation Syndrome	Nonketotic Hyperglycinemia
Hematopoietic Stem Cell Transplantation	Kufs Disease – Type A and B	Menkes Disease – Classic or Infantile Onset Form	Non-Small Cell Lung Cancer
Hemophagocytic Lymphohistiocytosis – Familial Type (FHLH)	Large Intestine Cancer – with distant metastasis or inoperable, unresectable or recurrent	Merkel Cell Carcinoma – with metastases	NUT Carcinoma
Hepatoblastoma	Late Infantile Neuronal Ceroid	Merosin Deficient Congenital Muscular Dystrophy	Obliterative Bronchiolitis
Hepatocellular Carcinoma	Lipofuscinoses	Metachromatic Leukodystrophy (MLD) – Late Infantile	Ohtahara Syndrome
Hepatopulmonary Syndrome	Leber Congenital Amaurosis	Metastatic Endometrial Adenocarcinoma	Oligodendroglioma Brain Cancer – Grade III
Hepatorenal Syndrome	Leigh’s Disease	Microvillus Inclusion Disease –Child	Ornithine Transcarbamylase (OTC) Deficiency
Histiocytic Malignancies	Leiomyosarcoma	Mitral Valve Atresia	Orthochromatic Leukodystrophy with Pigmented Glia
Histiocytosis Syndromes	Leptomeningeal Carcinomatosis	Mixed Dementias	Osteogenesis Imperfecta (OI) – Type II
Hoyeraal-Hreidarsson Syndrome	Lesch-Nyhan Syndrome (LNS)	Mowat-Wilson Syndrome	Osteosarcoma, formerly known as Bone Cancer
Hutchinson-Gilford Progeria Syndrome	Lewy Body Dementia	MPS I	Ovarian Cancer – with distant metastases or inoperable or unresectable
Hydranencephaly	Liposarcoma – metastatic or recurrent	MPS II	PACS1 Syndrome
Hypocomplementemic Urticarial Vasculitis Syndrome	Lissencephaly	MPS III	Pallister-Killian Syndrome
Hypophosphatasia – Perinatal (Lethal) and Infantile Onset Types	LMNA-related Congenital Muscular Dystrophy	Mucosal Malignant Melanoma	Pancreatic Cancer
Hypoplastic Left Heart Syndrome	Lowe Syndrome	Multicentric Castleman Disease	Paraneoplastic Cerebellar Degeneration
I Cell Disease	Lymphomatoid Granulomatosis – Grade III	Multiple System Atrophy	Paraneoplastic Pemphigus
Idiopathic Pulmonary Fibrosis	Malignant Brainstem Gliomas – Childhood	Myelodysplastic Syndrome with Excess Blasts	Patau Syndrome (Trisomy 13)
Infantile Free Sialic Acid Storage Disease	Malignant Ectomesenchymoma	Myoclonic Epilepsy with Ragged Red Fibers Syndrome	Pearson Syndrome
Infantile Neuroaxonal Dystrophy (INAD)	Malignant Gastrointestinal Stromal Tumor	Neonatal Adrenoleukodystrophy	Pelizaeus-Merzbacher Disease – Classic Form
Infantile Neuronal Ceroid Lipofuscinoses	Malignant Germ Cell Tumor	Neonatal Marfan Syndrome	Pelizaeus-Merzbacher Disease – Connatal Form
Inflammatory Breast Cancer (IBC)	Malignant Multiple Sclerosis	Nephrogenic Systemic Fibrosis	Pericardial Mesothelioma
Intracranial Hemangiopericytoma	Malignant Renal Rhabdoid Tumor	Neurodegeneration with Brain Iron Accumulation – Types 1 and 2	Peripheral Nerve Cancer – metastatic or recurrent
Jervell and Lange-Nielsen Syndrome	Mantle Cell Lymphoma (MCL)	NFU-1 Mitochondrial Disease	Peritoneal Mesothelioma
Joubert Syndrome	Maple Syrup Urine Disease	Nicolaides-Baraitser Syndrome	Peritoneal Mucinous Carcinomatosis
Junctional Epidermolysis Bullosa – Lethal Type	Marshall-Smith Syndrome	Niemann-Pick Disease (NPD) – Type A	Perry Syndrome
Juvenile Onset Huntington Disease	Mastocytosis – Type IV	Niemann-Pick Disease – Type C	Pfeiffer Syndrome – Types II and III
Kidney Cancer – inoperable or unresectable	MECP2 Duplication Syndrome		
Kleefstra Syndrome	Megacystis Microcolon Intestinal Hypoperistalsis Syndrome		

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