

Compassionate Allowances (CAL) Conditions (314)

1p36 Deletion Syndrome	Ataxia Telangiectasia	Child Heart Transplant Wait List – Status Levels 1A/1B	Eisenmenger Syndrome
Acute Leukemia	Atypical Teratoid/Rhabdoid Tumor	Child Lymphoblastic Lymphoma	Endometrial Stromal Sarcoma
Adenylosuccinate Lyase Deficiency – Neonatal Form and Type 1	Au-Kline Syndrome	Child Lymphoma	Endomyocardial Fibrosis
Adrenal Cancer with Distant Metastases or Inoperable, Unresectable or Recurrent	Bainbridge-Ropers Syndrome	Child Medulloblastoma	Ependymoblastoma (Child Brain Cancer)
Adult Heart Transplant Wait List – Status Levels 14	Baraitser-Winter Syndrome	Child Neuroblastoma – with Distant Metastases or Recurrent	Erdheim Chester Disease
Adult Non-Hodgkin Lymphoma	Batten Disease	Child T-Cell Lymphoblastic Lymphoma	Esophageal Cancer
Adult-Onset Huntington Disease	Beare-Stevenson Cutis Gyrata Syndrome	Cholangiocarcinoma	Esthesioneuroblastoma
Aicardi-Goutieres Syndrome	Beta Thalassemia Major	Chondrosarcoma – with Multimodal Therapy	Ewing Sarcoma
Aicardi Syndrome	Bilateral Anophthalmia	Choroid Plexus Carcinoma	Farber Disease (FD) – Infantile
Alexander Disease (ALX) – Neonatal and Infantile	Bilateral Optic Atrophy – Infantile	Chronic Idiopathic Intestinal Pseudo Obstruction	Fatal Familial Insomnia
Allan-Herndon-Dudley Syndrome	Bilateral Retinoblastoma	Chronic Myelogenous Leukemia (CML) – Blast Phase	Fibrodysplasia Ossificans Progressiva
Alobar Holoprosencephaly	Bladder Cancer – with Distant Metastases or Inoperable or Unresectable	CIC-Rearranged Sarcoma	Fibrolamellar Cancer
Alpers Disease	Blastic Plasmacytoid Dendritic Cell Neoplasm	Coffin-Lowry Syndrome	Follicular Dendritic Cell Sarcoma – Metastatic or Recurrent
Alpha Mannosidosis – Types II and III	Bohring-Opitz Syndrome	Congenital Lymphedema	FOXG1 Syndrome
ALS/Parkinsonism Dementia Complex	Breast Cancer – with Distant Metastases or Inoperable or Recurrent	Congenital Myotonic Dystrophy	Friedreich’s Ataxia (FRDA)
Alstrom Syndrome	CACH – Vanishing White Matter Disease – Congenital, Infantile, and Early Childhood Onset Forms	Congenital Zika Syndrome	Frontotemporal Dementia (FTD), Pick’s Disease, Type A – Adult
Alveolar Soft Part Sarcoma	Calciophylaxis	Cornelia de Lange Syndrome – Classic Form	Fryns Syndrome
Amegakaryocytic Thrombocytopenia	Canavan Disease (CD)	Corticobasal Degeneration	Fucosidosis – Type I
Amyotrophic Lateral Sclerosis (ALS) – Adult	Carcinoma of Unknown Primary Site	Costello Syndrome	Fukuyama Congenital Muscular Dystrophy
Anaplastic Adrenal Cancer – Adult with Distant Metastases or Inoperable, Unresectable or Recurrent	Cardiac Amyloidosis – AL Type	Creutzfeldt-Jakob Disease (CJD) – Adult	Fulminant Giant Cell Myocarditis
Anaplastic Ependymoma	Carey-Fineman-Ziter Syndrome	Cri du Chat Syndrome	Galactosialidosis – Early and Late Infantile Types
Anaplastic Thyroid Cancer	CASK-Related Gene Disorders	Degos Disease – Systematic	Gallbladder Cancer
Angelman Syndrome	Caudal Regression Syndrome – Types III and IV	DeSanctis Cacchione Syndrome	Gaucher Disease (GD) – Type 2
Angioimmunoblastic T-Cell Lymphoma	CDKL5 Deficiency Disorder	Desmoplastic Mesothelioma	Gerstmann-Straussler-Scheinker Disease
Angiosarcoma	Cerebro Oculo Facio Skeletal (COFS) Syndrome	Desmoplastic Small Round Cell Tumors	Giant Axonal Neuropathy
Aortic Atresia	Cerebrotendinous Xanthomatosis	Dravet Syndrome	Glioblastoma Multiforme (Adult Brain Cancer)
Aplastic Anemia	Charlevoix-Saguenay Spastic Ataxia	Duchenne Muscular Dystrophy – Adult	Glioma – Grade III and IV
Astrocytoma – Grade III and IV		Edwards Syndrome (Trisomy 18)	Glutaric Acidemia – Type II

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GM1 – Gangliosidosis – Infantile and Juvenile Forms	Junctional Epidermolysis Bullosa – Lethal Type	Maple Syrup Urine Disease	Neurodegeneration with Brain Iron Accumulation – Types 1 and 2
Harlequin Ichthyosis – Child	Juvenile Onset Huntington Disease	Marshall-Smith Syndrome	NFU-1 Mitochondrial Disease
Head and Neck Cancers – with Distant Metastasis or Inoperable or Unresectable	Kidney Cancer – Inoperable or Unresectable	Mastocytosis – Type IV	Nicolaides-Baraitser Syndrome
Heart Transplant Graft Failure	Kleefstra Syndrome	MECP2 Duplication Syndrome	Niemann-Pick Disease (NPD) – Type A
Hematopoietic Stem Cell Transplantation	Krabbe Disease (KD) – Infantile	Megacystis Microcolon Intestinal Hypoperistalsis Syndrome	Niemann-Pick Disease – Type C
Hemophagocytic Lymphohistiocytosis – Familial Type (FHLH)	Kufs Disease – Type A and B	Megalencephaly-Capillary Malformation Syndrome	Nonketotic Hyperglycinemia
Hepatoblastoma	Lafora Disease	Menkes Disease – Classic or Infantile Onset Form	Non-Small Cell Lung Cancer
Hepatocellular Carcinoma	Large Intestine Cancer – with Distant Metastasis or Inoperable, Unresectable or Recurrent	Merkel Cell Carcinoma – with Metastases	NUT Carcinoma
Hepatopulmonary Syndrome	Late Infantile Neuronal Ceroid Lipofuscinoses	Merosin Deficient Congenital Muscular Dystrophy	Obliterative Bronchiolitis
Hepatorenal Syndrome	Leber Congenital Amaurosis	Metachromatic Leukodystrophy (MLD) – Late Infantile	Ohtahara Syndrome
Hepatosplenic T-Cell Lymphoma	Leigh’s Disease	Metastatic Endometrial Adenocarcinoma	Oligodendroglioma Brain Cancer – Grade III
Histiocytic Malignancies	Leiomyosarcoma	Microvillus Inclusion Disease – Child	OPHN1 Syndrome
Histiocytosis Syndromes	Leptomeningeal Carcinomatosis	Mitral Valve Atresia	Ornithine Transcarbamylase (OTC) Deficiency
Hoyeraal-Hreidarsson Syndrome	Lesch-Nyhan Syndrome (LNS)	Mixed Dementias	Orthochromatic Leukodystrophy with Pigmented Glia
Hutchinson-Gilford Progeria Syndrome	Lewy Body Dementia	Mowat-Wilson Syndrome	Osteogenesis Imperfecta (OI) – Type II
Hydranencephaly	Liposarcoma – Metastatic or Recurrent	MPS I	Osteosarcoma, formerly known as Bone Cancer
Hypocomplementemic Urticarial Vasculitis Syndrome	Lissencephaly	MPS II	Ovarian Cancer – with Distant Metastases or Inoperable or Unresectable
Hypophosphatasia – Perinatal (Lethal) and Infantile Onset Types	LMNA-Related Congenital Muscular Dystrophy	MPS III	PACS1 Syndrome
Hypoplastic Left Heart Syndrome	Lowe Syndrome	Mucosal Malignant Melanoma	Pallister-Killian Syndrome
I Cell Disease	Lymphomatoid Granulomatosis – Grade III	Multicentric Castleman Disease	Pancreatic Cancer
Idiopathic Pulmonary Fibrosis	Malignant Brainstem Gliomas – Childhood	Multiple System Atrophy	Paraneoplastic Cerebellar Degeneration
Infantile Free Sialic Acid Storage Disease	Malignant Ectomesenchymoma	Myelodysplastic Syndrome with Excess Blasts	Paraneoplastic Pemphigus
Infantile Neuroaxonal Dystrophy (INAD)	Malignant Gastrointestinal Stromal Tumor	Myoclonic Epilepsy with Ragged Red Fibers Syndrome	Patau Syndrome (Trisomy 13)
Infantile Neuronal Ceroid Lipofuscinoses	Malignant Germ Cell Tumor	Neonatal Adrenoleukodystrophy	Pearson Syndrome
Inflammatory Breast Cancer (IBC)	Malignant Migrating Partial Seizures of Infancy (MMPST)	Neonatal Marfan Syndrome	Pelizaeus-Merzbacher Disease – Classic Form
Intracranial Hemangiopericytoma	Malignant Multiple Sclerosis	Nephrogenic Systemic Fibrosis	Pelizaeus-Merzbacher Disease – Connatal Form
Jervell and Lange-Nielsen Syndrome	Malignant Renal Rhabdoid Tumor		
Joubert Syndrome	Mantle Cell Lymphoma (MCL)		

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