DETAILED LISTING OF COMPASSIONATE ALLOWANCE CASES

ACUTE LEUKEMIA	Acute Lymphoblastic Leukemia, Acute Myeloid Leukemia
ADRENAL CANCER	Adrenal Carcinoma, Adrenocortical Carcinoma, Adrenocortical Cancer, Cancer of the Adrenal Cortex, Carcinoma of the Adrenal Cortex
ADULT NON-HODGKIN LYMPHOMA	Lymphoma - non-Hodgkin's; Lymphocytic lymphoma; Histiocytic lymphoma; Lymphoblastic lymphoma; Cancer - non-Hodgkin's lymphoma
ADULT ONSET HUNTINGTON DISEASE	Huntington's chorea; Huntington's Disease
AICARDI - GOUTIERES SYNDROME	AGS; Cree Encephalitis; Encephalopathy with Basal Ganglia Calcification; Pseudo-TORCH Syndrome; Pseudotoxoplasmosis Syndrome; Familial Infantile Encephalopathy with Intracranial Calcification and Chronic Cerebrospinal Fluid Lymphocytosis
ALEXANDER DISEASE (ALX) - NEONATAL AND INFANTILE	Alexander Syndrome, Dysmyelogenic Leukodystrophy, Dysmyelogenic Leukodystrophy-Megalobare, Fribrinoid Degeneration of Astrocytes-Infantile type, Fibrinoid Leukodystrophy-Infantile type, Hyaline Panneuropathy, Leukodystrophy with Rosenthal Fibers, Megalencephaly with Hyaline Inclusion, Megalencephaly with Hyaline Panneuropathy
ALLAN-HERNDON-DUDLEY SYNDROME	Allan-Herndon Syndrome; X-linked Intellectual Deficit with Hypotonia; Monocarboxylate Transporter 8 Deficiency; MCT8 Deficiency; MCT8 Specific Thyroid Hormone Cell Transporter Deficiency; MCT8 SLC16A2
ALOBAR HOLOPROSENCEPHALY	Holoprosencephaly; HPE; Holoprosencephaly 1 Alobar; Familial Alobar Holoprosencephaly; Holoprosencephaly Sequence
ALPERS DISEASE	Alpers Syndrome; Alpers Progressive Infantile Poliodystrophy; Alpers Huttenlocher Syndrome; AHS; Progressive Neuronal Degeneration of Childhood; PNDC; Progressive Sclerosing Poliodystrophy; Progressive Infantile Poliodystrophy; Progressive Cerebral Poliodystrophy; Poliodystrophia Cerebri
ALPHA MANNOSIDOSIS-TYPES II AND III	Alpha Mannosidosis Types II/III Early Onset Forms; Alpha-D-mannosidosis; Alpha-mannosidase B deficiency; Alpha-mannosidase deficiency; Lysosomal alpha B mannosidosis; Lysosomal alpha-D-mannosidase deficiency; Mannosidosis lipid storage disease; Inborn error of metabolism; MAN2B1

	gene abnormality; Mannosidosis
ALSTROM SYNDROME	ALMS; ALMS1; Alstrom-Halgren Syndrome; Alstrom syndrome 1
ALVEOLAR SOFT PART SARCOMA	ASP Sarcoma; ASPS
AMEGAKARYOCYTIC THROMBOCYTOPENIA	Inherited Bone Marrow Failure Syndrome (IBMFS); Congenital Amegakaryocytic Thrombocytopenia (CAMT)
AMYOTROPHIC LATERAL SCLEROSIS (ALS) - ADULT	Aran-Duchenne, Gehrig's Disease, Lou Gehrig's Disease, Motor Neuron Disease
ANAPLASTIC ADRENAL CANCER - ADULT	Adrenal Carcinoma, Anaplastic Adrenal Cancer
ANGELMAN SYNDROME	AS
ANGIOSARCOMA	Primary Angiosarcoma; Secondary Angiosarcoma
AORTIC ATRESIA	Aortic Valve Atresia; Aortic Valve Stenosis
APLASTIC ANEMIA	Acquired Aplastic Anemia; Anemia Aplastic; Bone Marrow Failure; Idiopathic Aplastic Anemia; Secondary Aplastic Anemia; Severe Aplastic Anemia
ASTROCYTOMA - GRADE III AND IV	Astrocytoma Grade III: anaplastic astrocytoma, anaplastic malignant astrocytoma, Astrocytoma Grade IV: glioblastoma multiforme(GBM), glioblastoma, mixed glioblastoma sarcoma, gliosarcoma astrocytoma grade IV, giant cell glioblastoma astrocytoma, spongioblastoma multiforme
ATAXIA TELANGIECTASIA	Boder-Sedgwick Syndrome; Louis-Bar Syndrome; Ataxia Telangiectasia syndrome; A-T; Cerebello-oculocutaneous Telangiectasia
ATYPICAL TERATOID/RHABDOID TUMOR	AT/RT; Central Nervous System AT/RT; CNS AT/RT; Malignant AT/RT; Childhood Atypical Teratoid / Rhabdoid Tumor; Childhood AT/RT
BATTEN DISEASE	Neuronal Ceroid Lipofuscinoses (NCL); Ceroid Neuronal Lipofuscinosis (CNL); Spielmeyer-Vogt-Sjogren-Batten disease; Haltia-Santavuori; Jansky-Beilschowsky
BETA THALASSEMIA MAJOR	Beta Thalassemia Major Syndrome; Beta Thalassemia Major Disease; Thalassemia Major; Cooley Anemia; Cooley Anemia

DETAILED LISTING OF COMPASSIONATE ALLOWANCE CASES

	Disease; Cooley Anemia Syndrome; Erythroblastic Anemia of Childhood; Microcythemia Major; Mediterranean Anemia Major; Beta Zero Thalassemia
BILATERAL OPTIC ATROPHY - INFANTILE	Infantile Bilateral Optic Atrophy; Bilateral Optic Neuropathy; Idiopathic Bilateral Optic Atrophy; Congenital Optic Atrophy; Pediatric Bilateral Optic Atrophy
BILATERAL RETINOBLASTOMA	Malignant Neoplasm, Retina
BLADDER CANCER	Invasive Bladder Cancer, Bladder Carcinoma, Invasive Bladder Carcinoma, Transitional Cell Carcinoma of the Bladder, Transitional Cell Cancer of the Bladder, Squamous Cell Carcinoma of the Bladder, Squamous Cell Cancer of the Bladder, Adenocarcinoma of the Bladder, Urinary Cancer, Urinary Carcinoma
BREAST CANCER	Breast Carcinoma (Stage IV), Metastatic Breast Carcinoma, Metastatic Breast Cancer, Ductal Carcinoma of the Breast (Stage IV), Metastatic Ductal Carcinoma, Metastatic Ductal Cancer, Lobular Carcinoma of the Breast Stage (IV), Metastatic Lobular Cancer, Metastatic Lobular Carcinoma, Recurrent Breast Cancer
CANAVAN DISEASE (CD)	Aminoacylase-2 (ACY2) Deficiency, Aspartoacylase (ASPA) Deficiency, Canavan's Leukodystrophy, Spongy Degeneration of the Central Nervous System or Neuroaxis, Van Bogaert-Bertrand Syndrome
CARCINOMA OF UNKNOWN PRIMARY SITE	Cancer of Unknown Primary Origin; CUP; Origin of Unknown Primary Site; Occult Primary Malignancy; Occult Primary Cancer; Malignant Neoplasms of Unknown site; Malignant Neoplasms of Unknown Origin
CARDIAC AMYLOIDOSIS - AL TYPE	Light Chain Cardiac Amyloidosis; Cardiac Amyloidosis Light Chain Disease; Primary Cardiac Amyloidosis
CAUDAL REGRESSION SYNDROME - TYPES III AND IV	Caudal Dysplasia Sequence; Caudal Regression Sequence; Caudal Dysgenesis Syndrome; Sacral Regression; Sacral Agenesis; Lumbo Sacral Agenesis; Sacral Defect with Anterior Meningocele
CEREBRO OCULO FACIO SKELETAL (COFS) SYNDROME	Cockayne Syndrome-Classical Type I, Cockayne Syndrome-Congenital Type II, Pena Shokeir Syndrome Type II

DETAILED LISTING OF COMPASSIONATE ALLOWANCE CASES

CEREBROTENDINOUS XANTHOMATOSIS	CTX; Van Bogaert-Scherer-Epstein Disease; Xanthomatosis Cerebrotendinous; Cerebral Cholesterosis
CHILD LYMPHOBLASTIC LYMPHOMA	
CHILD LYMPHOMA	Non-Hodgkin Lymphoma; Diffuse Large B-cell Lymphoma;B-cell Lymphoma;T-cell Lymphoma; Peripheral T-cell Lymphoma; Follicular Lymphoma; Burkitt Lymphoma; Anaplastic Large Cell Lymphoma
CHILD NEUROBLASTOMA	Congenital Neuroblastoma; Sympathicoblastoma; Stage IVS Neuroblastoma; Pepper's syndrome; Schwannian Stroma-Poor Neuroblastoma
CHONDROSARCOMA	Chondrosarcoma Grade III; Dedifferentiated Chondrosarcoma; Mesenchymal Chondrosarcoma
CHRONIC IDIOPATHIC INTESTINAL PSEUDO OBSTRUCTION	Chronic Intestinal Pseudo Obstruction; Intestinal Pseudo Obstruction; Congenital Idiopathic Intestinal Pseudo Obstruction; Primary Intestinal Pseudo Obstruction
CHRONIC MYELOGENOUS LEUKEMIA (CML) - BLAST PHASE	Chronic Myeloid Leukemia (Blast phase), CML (Blast phase), Chronic Granulocytic Leukemia (Blast phase)
COFFIN-LOWRY SYNDROME	Coffin Syndrome; Coffin Lowry Disease
CONGENITAL LYMPHEDEMA	Congenital Hereditary Lymphedema; Primary Lymphedema; Congenital Primary Lymphedema; Milroy disease
CORNELIA DE LANGE SYNDROME - CLASSIC FORM	Classic Form Cornelia de Lange Syndrome; CDLS1, Brachmann-De Lange Syndrome; BDLS; CdLS; de Lange Syndrome; Amsterdam Dwarfism; Bushy Syndrome
CORTICOBASAL DEGENERATION	CBD; Cortical-Basal Ganglionic Degeneration; Cortico-Basal Ganglionic Degeneration; CBGD
CREUTZFELDT-JAKOB DISEASE (CJD) - ADULT	Jakob-Creutzfeldt Disease, Jakobs Disease, Subacute Spongiform Encephalopathy, Variant (V-CJD) Bovine Spongiform Encephalopathy (BSE), Fatal Familial Insomnia (FFI), Gerstmann-Straussler-Scheinker (GSS) Disease, Prion disease
CRI DU CHAT SYNDROME	5p-Syndrome; Cat's Cry Syndrome; Le Jeune Syndrome; Chromosome 5p-Syndrome; 5p deletion syndrome

DETAILED LISTING OF COMPASSIONATE ALLOWANCE CASES

DE SANCTIS CACCHIONE SYNDROME	De Sanctis-Cacchione Syndrome; Xerodermic Idiocy; Xeroderma Pigmentosum with Neurological Manifestation
DEGOS DISEASE	Degos-Kohlmeier Disease; Degos Syndrome; Kohlmeier-Degos Disease; Malignant Atrophic Papulosis; Kohlmeier disease
DRAVET SYNDROME	Severe Myoclonic Epilepsy Of/In Infancy; SMEI Syndrome; Epilepsy with Polymorphic Seizures; Polymorphic Epilepsy in Infancy; PMEI
EARLY-ONSET ALZHEIMER'S DISEASE	Presenile dementia; Presenile Alzheimer's disease; Young-onset Alzheimer's disease; Familial AD; FAD; AD; EOAD
EDWARDS SYNDROME	Trisomy 18; Chromosome 18; Trisomy 18 Complete; Complete Trisomy 18 Syndrome; Trisomy E Syndrome
EISENMENGER SYNDROME	Eisenmenger Complex; Eisenmenger Disease; Eisenmenger Reaction; Eisenmenger Physiology
ENDOMETRIAL STROMAL SARCOMA	Endometrial Stromal Sarcoma Grade III/IV; High Grade Endometrial Stromal Sarcoma; ESS; Undifferentiated Uterine Sarcoma; UUS
ENDOMYOCARDIAL FIBROSIS	EMF; Davies Disease; Fibroelastic Endocarditis, Loeffler Endomyocardial Fibrosis with Eosinophilia; Loeffler Fibroplastic Parietal Endocarditis
EPENDYMOBLASTOMA (CHILD BRAIN CANCER)	Childhood Ependymoma; Ependymal Tumors; Neuroectodermal Tumors, Primitive.
ERDHEIM CHESTER DISEASE	Erdheim Chester Syndrome; Lipoid Granulomatosis; Non-Langerhans Cell Histiocytosis; Polyostotic Sclerosing Histiocytosis
ESOPHAGEAL CANCER	Adenocarcinoma of the Esophagus, Squamous cell carcinoma of the Esophagus
ESTHESIONEUROBLASTOMA	Olfactory Neuroblastoma; Skull Based Olfactory Neuroblastoma; Intracranial Olfactory Neuroblastoma; Recurrent Esthesioneuroblastoma
EWING SARCOMA	Ewing Tumor; Ewing Sarcoma of Bone, Ewing Sarcoma of Soft Tissue; Primitive Neuroectodermal Tumor; PNET; Askin Tumor; Diffuse Bone Endothelioma; Endothelial Myeloma; Bone Endothelioma; Endothelial Sarcoma of Bone; Extraosseous

	Ewing Sarcoma
FARBER'S DISEASE (FD) - INFANTILE	Acid Ceramidase Deficiency, Disseminated Lipogranulomatosis, Farbers Syndrome
FATAL FAMILIAL INSOMNIA	Insomnia Fatal Familial
FIBRODYSPLASIA OSSIFICANS PROGRESSIVA	FOP; Myositis Ossificans Progressiva; Progressive Myositis Ossificans; Progressive Ossifying Myositis; Munchmeyer Disease
FOLLICULAR DENDRITIC CELL SARCOMA	Follicular Dendritic Cell Tumor/Sarcoma; (FDCT/S); Dendritic Cell Sarcoma
FRIEDREICH'S ATAXIA (FRDA)	Friedreich's Disease, Friedreich's Tabes, Hereditary Ataxia-Friedreich's type, Hereditofamilial Spinal Ataxia
FRONTOTEMPORAL DEMENTIA (FTD), PICK'S DISEASE -TYPE A - ADULT	Frontotemporal Lobar Degeneration, Dementia with Lobar Atrophy and Neuronal Cytoplasmic Inclusions, Diffuse Degenerative Cerebral Disease, Lobar Atrophy of the brain, Pick Disease of the brain-Type 1, Wilhelmsen-Lynch Disease
FRYNS SYNDROME	FRNS; Diaphragmatic Hernia, Abnormal Face, and Distal Limb Anomalies; Congenital Diaphragmatic Hernia; CDH
FUCOSIDOSIS- TYPE I	Alpha-L-Fucosidase Deficiency; ALF; Fucosidosis Infantile Type; Fucosidosis Severe
FUKUYAMA CONGENITAL MUSCULAR DYSTROPHY	Cerebromuscular dystrophy, Fukuyama type; FCMD; Fukuyama CMD; Fukuyama muscular dystrophy; Fukuyama Syndrome; Fukuyama type congenital muscular dystrophy; Muscular dystrophy congenital, Fukuyama type; Muscular dystrophy congenital progressive, with intellectual disability; Muscular dystrophy congenital, with central nervous system involvement; Polymicrogyria with muscular dystrophy
FULMINANT GIANT CELL MYOCARDITIS	Fulminant GCM; Fulminant Myocarditis; Fulminant Non-ischemic Dilated Cardiomyopathy
GALACTOSIALIDOSIS - EARLY AND LATE INFANTILE TYPES	Protective Protein/Cathepsin A Deficiency ; PPCA deficiency; Cathepsin A Deficiency of GSL; Deficiency of Cathepsin A; Lysosomal Protein Deficiency; Neuraminidase with Beta galactosidase deficiency; Goldberg Syndrome
GALLBLADDER CANCER	Cholangiocarcinoma, Klatskin tumor, Biliary Duct cancer

DETAILED LISTING OF COMPASSIONATE ALLOWANCE CASES

GAUCHER DISEASE (GD) - TYPE 2	Gaucher disease-Type 2; GD2; Gaucher disease, infantile cerebral; Gaucher disease, acute neuronopathic type; Gaucher's disease type 2; Gaucher syndrome type 2
GIANT AXONAL NEUROPATHY	
GLIOBLASTOMA MULTIFORME (BRAIN CANCER)	Grades III and IV Astrocytoma; Malignant Glioma; Anaplastic Glioma; Brain Cancer; Adult Brain Tumor; GBM
GLIOMA - GRADE III AND IV	High Grade Malignant Glioma; Malignant Glioma Grade III; Malignant Glioma Grade IV; Glioblastoma; Anaplastic Astrocytoma; Undifferentiated Glioma; Anaplastic Glioma
GLUTARIC ACIDEMIA - TYPE II	Electron transfer flavoprotein deficiency; EMA; ETFA deficiency; ETFB deficiency; ETFDH deficiency; Ethylmalonic-adipicaciduria; GA II; Glutaric acidemia, type 2; MAD; MADD; Multiple acyl-CoA dehydrogenase deficiency; Multiple FAD dehydrogenase deficiency
HEAD AND NECK CANCERS	Squamous Cell Carcinoma of the nasal cavity, sinuses, lips, mouth, nose, tonsils, tongue, throat, or larynx (voice box); Adenocarcinoma of the nasal cavity, sinuses, lips, mouth, nose, tonsils, tongue, throat, or larynx (voice box); Squamous Cell Cancer of the nasal cavity, sinuses, lips, mouth, nose, tonsils, tongue, throat, or larynx (voice box); Metastatic Squamous Cell Neck Cancer; Metastatic Squamous Cell Neck Carcinoma; Head and Neck Carcinomas. Small Cell or Oat Cell Carcinoma.
HEART TRANSPLANT GRAFT FAILURE	Graft Rejection; Tissue Rejection; Organ Rejection; Primary Graft Dysfunction; Cardiac Allograft Vasculopathy
HEART TRANSPLANT WAIT LIST - 1A/1B	Cardiac Transplant; Transplant-Heart
HEMOPHAGOCYTIC LYMPHOHISTIOCYTOSIS, FAMILIAL TYPE	FHLH types 1, 2, 3, 4 and 5; FHL; HLH; HPLH; Familial Erythrophagocytic Lymphohistiocytosis; Erythrophagocytic Lymphohistiocytosis; Familial Histiocytic Retulosis
HEPATOBLASTOMA	Pediatric Embryonal Hepatoma; Pediatric Hepatoblastoma; Liver Cancer - children; HB; HBL
HEPATOPULMONARY SYNDROME	Hepatopulmonary Syndrome Type I/II; Hepato Pulmonary Syndrome

DETAILED LISTING OF COMPASSIONATE ALLOWANCE CASES

HEPATORENAL SYNDROME	Hepatorenal Syndrome Type I/II; Hepato Renal Syndrome
HISTIOCYTOSIS SYNDROMES	Langerhans Cell Histiocytosis; LCH; Histiocytosis X; Malignant Histiocytosis syndrome T-cell lymphoma; Non-Langerhans Cell Histiocytosis; Hemophagocytic Syndrome; Pulmonary Histiocytosis X; Nonlipid Reticuloendotheliosis; Pulmonary Langerhans Cell Granulomatosis; Hand-Schuller-Christian disease; Letterer-Siwe disease; Rosai-Dorfman disease; Sinus Histiocytosis with Massive Lymphadenopathy.
HOYERAAL-HREIDARSSON SYNDROME	Hoyeraal-Hreidarsson Disease; Cerebellar Hypoplasia with Pancytopenia; Progressive Pancytopenia Immunodeficiency Cerebellar Hypoplasia
HUTCHINSON-GILFORD PROGERIA SYNDROME	HGPS; Hutchinson-Gilford Syndrome; Progeria Syndrome; Progeria of Childhood
HYDRANENCEPHALY	Hydroanencephaly
HYPOCOMPLEMENTEMIC URTICARIAL VASCULITIS SYNDROME	Hypocomplementemic Vasculitis; Urticarial Vasculitis; McDuffie Syndrome
HYPOPHOSPHATASIA - PERINATAL (LETHAL) AND INFANTILE ONSET TYPES	Alkaline Phosphotase Deficiency; Perinatal Lethal Hypophosphatasia; Hypophosphatasia Perinatal Lethal Form; Perinatal Rathburn Disease; Phosphoethanolaminuria
HYPOPLASTIC LEFT HEART SYNDROME	HLHS; Aortic and Mitral Atresia with Hypoplastic Left Heart Syndrome
I CELL DISEASE	Mucolipidosis Type II; MLType II; Inclusion Cell disease; Mucolipidosis II Alpha/Beta; ML II; Mucolipidosis 2; ML 2; GNPTA; Leroy Disease; N-acetylglucosamine 1 phosphotransferase deficiency; ML disorder type 2
IDIOPATHIC PULMONARY FIBROSIS	Idiopathic Diffuse Interstitial Pulmonary Fibrosis; IPF; Pulmonary Fibrosis; Cryptogenic Fibrosing Alveolitis; CFA; Fibrosing Alveolitis; Usual Interstitial Pneumonitis; UIP; Diffuse Fibrosing Alveolitis; Familial Idiopathic Pulmonary Fibrosis (FIPF).
INFANTILE FREE SIALIC ACID STORAGE DISEASE	ISSD; Sialuria Infantile Form; Infantile Sialic Acid Storage Disorder; Free Sialic Acid Storage Disease; N-acetylneuraminic acid storage disease; NANA Storage Disease; Sialuria Finnish Type

DETAILED LISTING OF COMPASSIONATE ALLOWANCE CASES

INFANTILE NEUROAXONAL DYSTROPHY (INAD)	Prenatal or Connatal Neuroaxonal Dystrophy, Seitelberger Disease
INFANTILE NEURONAL CEROID LIPOFUSCINOSES	Haltia-Santavuori disease; CNL1; INCL; NCL; Finnish form of NCL; Santavuori-Haltia disease
INFLAMMATORY BREAST CANCER	Inflammatory Breast Carcinoma, IBC, Locally Advanced Breast Cancer
INTRACRANIAL HEMANGIOPERICYTOMA	Primary Intracranial Hemangiopericytoma; Infantile Intracranial Hemangiopericytoma; Multifocal Intracranial Hemangiopericytoma
JERVELL AND LANGE-NIELSEN SYNDROME	Jervell Lange Syndrome; Jervell Nielsen Disease; Nielsen Syndrome; Cardio Auditory Syncope Syndrome; Cardioauditory Syndrome of Jervell and Lange-Nielsen; Surdo Cardiac Syndrome; Long QT Syndrome
JOUBERT SYNDROME	Agenesis of Cerebellar Vermis; Cerebello-Oculo-Renal Syndrome; Cerebellooculorenal Syndrome 1; Cerebelloparenchymal Disorder 4; Joubert-Bolthauser Syndrome; Joubert Syndrome and Related Disorders; Joubert Syndrome Type A; Pure Joubert Syndrome; Classic Joubert Syndrome
JUNCTIONAL EPIDERMOLYSIS BULLOSA LETHAL TYPE	JEB; JEB-Herlitz; Lethal Junctional Epidermolysis Bullosa; Junctional Epidermolysis Bullosa Gravis; Dystrophic Epidermolysis Bullosa; Hemidesmosomal Epidermolysis Bullosa; Herlitz disease
JUVENILE ONSET HUNTINGTON DISEASE	Juvenile Huntington Disease; Juvenile HD; JHD; Early-Onset HD
KIDNEY CANCER	Kidney Carcinoma, Renal Cell Cancer (RCC), Renal Cell Carcinoma, Wilms Tumor, Renal Pelvis Carcinoma, Renal Adenocarcinoma, Clear Cell Sarcoma of the Kidney, Rhabdoid Tumor of the Kidney, Neuroepithelial Tumor of the Kidney, Diffuse Hyperplastic Perilobar Nephroblastomatosis
KRABBE DISEASE (KD) - INFANTILE	Beta Galactocerebrosidase (GALC) Deficiency, Galactosylceramide Deficiency, Galactosylceramide Lipidosis, Globoid Cell Leukodystrophy (GLD), Krabbe Leukodystrophy, Sphingolipidoses, Krabbe type
KUFS DISEASE TYPE A AND TYPE B	Adult Neuronal Ceroid Lipofuscinosis; Adult NCL; NCL Type 4; CLN4A; Kufs Disease; Kufs Type Neuronal Ceroid

	Lipofuscinosis
LARGE INTESTINE CANCER	Colon Cancer, Colon Carcinoma, Colorectal Cancer, Colorectal Carcinoma, Rectal Cancer, Rectal Carcinoma, Large Bowel Cancer, Large Bowel Carcinoma, Large Intestine Adenocarinoma
LATE INFANTILE NEURONAL CEROID LIPOFUSCINOSES	Jansky-Bielchowsky disease; CNL2; LINCL
LEIGH'S DISEASE	Leigh Necrotizing Encephalopathy; Leigh's Syndrome; Necrotizing Encephalomyelopathy of Leigh's; SNE; Subacute Necrotizing Encephalopathy
LEIOMYOSARCOMA	Leiomyosarcoma of the Uterus; Leiomyosarcoma of Vascular Origin; Leiomyosarcoma of the Bone; Leiomyosarcoma of the Retroperitoneum; Leiomyosarcoma Stage IV; Leiomyosarcoma Stage III; High Grade Leiomyosarcoma
LEPTOMENINGEAL CARCINOMATOSIS	Leptomeningeal Cancer; Neoplastic Meningitis; Carcinomatous Meningitis; Leptomeningeal Metastasis; Leptomeningeal Carcinoma; Meningeal Metastasis
LESCH-NYHAN SYNDROME (LNS)	Hereditary Hyperuricemia and Choreoathetosis Syndrome, Hyperuricemia Choreoathetosis-Self mutilation Syndrome, Hyperuricemia-Oligophrenia, Hypoxanthine-Guanine Phosphoribosyltranferase Deficiency (HGPRT), Hypoxanthine phosphoribosyltransferase Deficiency (HPRT), Juvenile Gout-Choreoathetosis and Intellectual Disability Syndrome, Lesch Nyhan Disease, Nylan Syndrome
LEWY BODY DEMENTIA	Lewy Body Disease; Diffuse Lewy Body Disease; Dementia with Lewy Bodies; Autosomal Dominant Diffuse Lewy Body Disease; Cortical Lewy Body Dementia; Lewy Body Variant of Alzheimer Disease; Parkinson Disease with Dementia; Senile Dementia of Lewy Type
LIPOSARCOMA	Dedifferentiated Liposarcoma; Pleomorphic Liposarcoma; Inflammatory Liposarcoma; Spindle Cell Liposarcoma; Myxoid Liposarcoma
LISSENCEPHALY	Lissencephaly Type I; LIS1; Classical Lissencephaly; X-Linked Lissencephaly; XLIS; Lissencephaly with Agenesis of the Corpus Callosum; Lissencephaly with Cerebellar Hypoplasia; Microlissencephaly; Miller-Dieker Syndrome

DETAILED LISTING OF COMPASSIONATE ALLOWANCE CASES

LIVER CANCER	Hepatocellular (Liver) cancer, Intrahepatic Bile Duct Cancer, Liver Cancer, Hepatocellular Carcinoma
LOWE SYNDROME	LS; Cerebro-oculo-renal Syndrome; Cerebro-oculo-renal Dystrophy; Lowe Oculocerbrorenal Syndrome; Lowe's Disease; Oculocerebrorenal Dystrophy; OCRL; Oculocerebrorenal Syndrome; OCR; Oculo-cerebrorenal Syndrome of Lowe; Phosphatidylinositol-4,5 Bisphospate-5-Phosphatase Deficiency
LYMPHOMATOID GRANULOMATOSIS - GRADE III	Lymphomatoid Granulomatosis High Grade; Polymorphic Reticulosis; Lymph Angiitis and Granulomatosis; Malignant Lymph Angiitis and Granulomatosis; Pulmonary Angiitis; Lymphoproliferative Disease; LG High Grade; LG
MALIGNANT BRAINSTEM GLIOMAS - CHILDHOOD	Childhood Malignant Brainstem Glioma; Malignant Brainstem Glioma- Childhood Diffuse Intrinsic; Malignant Brainstem Glioma; Diffuse Intrinsic Pontine Gliomas; DIPG; Malignant Brain Tumor; Pediatric Malignant Brain Tumor; Malignant Brain Tumor - Children
MALIGNANT ECTOMESENCHYMOMA	Mature Ectomesenchymoma; Gangliorhabdomyosarcoma; Rhabdomyosarcoma with Ganglionic Differentiation
MALIGNANT GASTROINTESTINAL STROMAL TUMOR	Gastrointestinal Stromal Neoplasm; Gastrointestinal Stromal Sarcoma
MALIGNANT GERM CELL TUMOR	Pediatric Malignant Germ Cell Tumor; Adult Malignant Germ Cell Tumor
MALIGNANT MULTIPLE SCLEROSIS	Malignant MS; Marburg Variant Multiple Sclerosis, Marburg Variant MS; Fulminant Multiple Sclerosis; Aggressive MS; Advanced MS
MALIGNANT RENAL RHABDOID TUMOR	Malignant Rhabdoid Tumor of the Kidney; Malignant RT
MANTLE CELL LYMPHOMA (MCL)	B-cell lymphoma, Non-Hodgkin's Lymphoma
MAPLE SYRUP URINE DISEASE	MSUD; Branched-Chain Ketoaciduria; Branched-Chain Alpha-Ketoacid Dehydrogenase Deficiency; BCKD Deficiency
MARSHALL-SMITH SYNDROME	

DETAILED LISTING OF COMPASSIONATE ALLOWANCE CASES

MASTOCYTOSIS - TYPE IV	Leukemic Mastocytosis; Mast Cell Leukemia; MCL; Lymphadenopathic Mastocytosis; Type IV Leukemia
MECP2 DUPLICATION SYNDROME	Trisomy Xq28; Distal Duplication Xq; Telomeric Duplication Xq; Lubs-Arena Syndrome; Lubs X-linked Intellectual Deficit Syndrome; Intellectual Deficit X-linked Lubs Type
MEDULLOBLASTOMA	
MENKES DISEASE - CLASSIC OR INFANTILE ONSET FORM	Classical Menkes Disease; Menkes Syndrome; X-linked Copper Deficiency; Congenital Hypocupremia
MERKEL CELL CARCINOMA	Merkel Cell Cancer; Trabecular Cancer; Apudoma of Skin; Small Cell Neuroepithelial Tumor of Skin; Primary Small Cell Carcinoma of Skin; Toker Tumor; Primary Cutaneous Neuroendocrine Tumor; Malignant Trichodiscoma; Neuroendocrine Carcinoma of the Skin
MEROSIN DEFICIENT CONGENITAL MUSCULAR DYSTROPHY	Merosin Deficient CMD; Laminin alpha-2; LAMA2; Muscular Dystrophy Congenital; Merosin Positive CMD; LAMM; MDCMD; Congenital Muscular Dystrophy with laminin-alpha-2 deficiency; MDC1A; Classic CMD LAMA2
METACHROMATIC LEUKODYSTROPHY (MLD) - LATE INFANTILE	Arylsulfatase A Deficiency (ARSA), Cerebroside Sulfatase Deficiency, Metachromatic Form of Diffuse Cerebral Sclerosis, Metachromatic Leukoencephalopathy, Scholz-Bielchowsky-Henneberg Diffuse Cerebral Sclerosis, Scholz-Greenfield Disease, Sulphatide Lipidosis, Sulphatidosis, Van Bogaert-Nijssen Disease
MITRAL VALVE ATRESIA	Mitral Atresia; MA
MIXED DEMENTIAS	Dementia due to multiple etiologies; Vascular dementia Alzheimer's disease (VaD); Parkinson's dementia; Diffuse Lewy-Body dementia; Frontotemporal dementia (Pick's disease); Huntington's dementia; Prion dementia; Progressive Supranuclear Palsy (PSP)
MPS I HURLER SYNDROME	Hurler Syndrome; Hurler Syndrome type IH; Alpha-L-iduronate deficiency; Mucopolysaccharidosis type I; MPS I; MPS I H; Lipochondrodystrophy; Pfaundler-Hurler syndrome; Hurler-Pfaundler syndrome
MPS II HUNTER SYNDROME	Hunter Syndrome; Mucopolysaccharidosis type II; Iduronate sulfatase deficiency; MPS Disorder; MPS II

DETAILED LISTING OF COMPASSIONATE ALLOWANCE CASES

MPS III SANFILIPPO SYNDROME	Sanfilippo Syndrome; Mucopolysaccaridoses III; MPS III ; Sanfilippo Type III; Oligophrenic polydystrophy; Polydystrophic oligophrenia
MUCOSAL MALIGNANT MELANOMA	Primary Mucosal Melanomas; Extracutaneous Malignant Melanomas (EMM); Primary Sinonasal Mucosal Melanoma (SNMM); Anorectal Melanoma (ARM); Mucosal Melanoma of the Head and Neck; Melanoma of the Esophagus; Melanoma of the Male Genito-Urinary Tract; Vulval Melanoma; Vaginal Melanoma; Mucosal Melanoma; Buccal Melanoma; Oral Melanoma
MULTICENTRIC CASTLEMAN DISEASE	MCD; Multicentric Plasma Cell Variant of Castleman's Disease; Idiopathic Multicentric Castleman's Disease; HIV-Associated Multicentric Castleman Disease
MULTIPLE SYSTEM ATROPHY	MSA; Shy-Drager Syndrome; Neurologic Orthostatic Hypotension; Shy-McGee-Drager Syndrome; Parkinson's Plus Syndrome; Striatonigral Degeneration; Sporadic Olivopontocerebellar Atrophy; MSA-P; MSA-C
MYOCLONIC EPILEPSY WITH RAGGED RED FIBERS	MERRF; Myoclonus with Epilepsy with Ragged Red Fibers; MERRF Syndrome; Myoencephalopathy Ragged Red Fiber Disease; Fukuhara syndrome
NEONATAL ADRENOLEUKODYSTROPHY	NALD; Neonatal ALD; Peroxisomal Biogenesis Disorder (PBD); Zellweger Syndrome Spectrum (ZSS)
NEPHROGENIC SYSTEMIC FIBROSIS	Nephrogenic Fibrosing Dermopathy; NSD; NSF
NEURODEGENERATION WITH BRAIN IRON ACCUMULATION - TYPE 1 AND 2	NBIA-1; NBIA-2; Hallervorden-Spatz Syndrome; (HSS); Pantothenate Kinase Associated Neurodegeneration; PKAN; Pigmentary Degeneration of Globus Pallidus and Substantia Nigra Red Nucleus, Neuroferritinopathy, Infantile; Neuroaxonal Dystrophy; INAD
NFU-1 MITOCHONDRIAL DISEASE	Multiple Mitochondrial Dysfunction Syndrome 1; Multiple Mitochondrial Dysfunction Syndrome Type 1; MMDS 1; NFU1 iron-sulfur cluster scaffold homolog (S. cerevisiae)
NIEMANN-PICK DISEASE (NPD) - TYPE A	Acute Neuronopathic form-type A-classic infantile form, Niemann Disease, Sphingomyelin Lipidosis, Sphingomyelinase Deficiency
NIEMANN-PICK DISEASE-	NPD; NPC; NPD type C; Niemann-Pick type II;

DETAILED LISTING OF COMPASSIONATE ALLOWANCE CASES

TYPE C	Sphingomyelinase deficiency; Neuronal Cholesterol Lipidosis
NON-SMALL CELL LUNG CANCER	Squamous Cell Lung Carcinoma, Squamous Cell Lung Cancer, Large Cell Lung Carcinoma, Large Cell Lung Cancer, Adenocarcinoma of the Lung, Non-Small Cell Lung Cancer, Non-Small Cell Lung Carcinoma, Lung Carcinoma
NONKETOTIC HYPERGLYCINEMIA	Glycine Encephalopathy; Hyperglycinemia Nonketotic; Isolated Nonketotic Hyperglycinemia
OBLITERATIVE BRONCHIOLITIS	Bronchiolitis Obliterans; Constrictive Bronchiolitis
OHTAHARA SYNDROME	Early Infantile Epileptic Encephalopathy with Burst Suppression; EIEE
OLIGODENDROGLIOMA BRAIN CANCER- GRADE III	Anaplastic Oligodendroglioma
ORNITHINE TRANSCARBAMYLASE (OTC) DEFICIENCY	Hyperammonemia Type II, Hyperammonemia due to Ornithine Transcarbamylase Deficiency, Ornithine Carbamyltransferase Deficiency
ORTHOCHROMATIC LEUKODYSTROPHY WITH PIGMENTED GLIA	Pigmented Type of Orthochromatic Leukodystrophy; Pigmentary Orthochromatic Leukodystrophy; POLD; Orthochromatic Leukodystrophy with Pigmented Glia Cells; Adult Onset Leukodystrophy; Adult Onset Leukoencephalopathy with Axonal Spheroids and Pigmented Glia; ALSP
OSTEOGENESIS IMPERFECTA (OI) - TYPE II	Osteogenesis Imperfecta Congenita (OIC), Vrolik Disease (OI Type 2A)
OSTEOSARCOMA	Osteogenic Sarcoma; Chondroblastic Osteocarcoma; Multifocal Osteosarcoma; Metastatic Osteosarcoma; Fibroblastic Osteosarcoma; Central Osteosarcoma; Conventional Central Osteosarcoma; Classical Osteosarcoma; Osteoblastic Sarcoma; Central Osteogenic Sarcoma; Sclerosing Osteosarcoma
OVARIAN CANCER	Ovarian Epithelial Carcinoma, Ovarian Epithelial Cancer, Ovarian Carcinoma
PALLISTER-KILLIAN SYNDROME	Pallister Mosaic Syndrome; Pallister-Killian Mosaic Syndrome; Killian Syndrome; Teschler-Nicola/Killian Syndrome; Tetrasomy 12p Syndrome; Tetrasomy 12p Mosaic Syndrome;

DETAILED LISTING OF COMPASSIONATE ALLOWANCE CASES

	Isochromosome 12p Syndrome
PANCREATIC CANCER	Exocrine cancer, Pancreatic Adenocarcinoma
PARANEOPLASTIC PEMPHIGUS	PNP; Paraneoplastic Autoimmune Multi-organ Syndrome
PATAU SYNDROME	Trisomy 13; Trisomy 13 Syndrome; Complete Trisomy 13 Syndrome; D Trisomy 13 Syndrome
PEARSON SYNDROME	Pearson Marrow Pancreas Syndrome; Sideroblastic Anemia with Marrow Cell Vacuolization and Exocrine Pancreatic Dysfunction; Pearson Anemia
PELIZAEUS-MERZBACHER DISEASE - CLASSIC FORM	Classic PMD; Pelizaeus-Merzbacher Brain Sclerosis; Adult Pelizaeus-Merzbacher Disease; Classic Pelizaeus-Merzbacher Disease
PELIZAEUS-MERZBACHER DISEASE - CONNATAL FORM	Connatal Pelizaeus-Merzbacher Disesase; Connatal PMD; Cockayane-Pelizaeus-Merzbacher Disease; Type II Connatal Pelizaeus-Merzbacher Disease; Severe PMD
PERIPHERAL NERVE CANCER	Malignant Peripheral Nerve Sheath Tumor; Malignant Neurilemmoma; MPNST; Sporadic Neurofibromatosis Type I; Neurofibromatosis Type I; Malignant Neurofibrosarcoma; Malignant Schwannoma; Malignant Tumor of the PNS; Malignant Neoplasm of the PNS; Malignant PNS Tumor; Malignant PNS Neoplasm; Neurosarcoma
PERITONEAL MESOTHELIOMA	Malignant Mesothelioma of the Peritoneum
PERITONEAL MUCINOUS CARCINOMATOSIS	Primary Peritoneal Surface Malignancy; Invasive Peritoneal Mucinous Carcinomatosis
PERRY SYNDROME	Parkinsonism with alveolar hypoventilation and mental depression
PHELAN-MCDERMID SYNDROME	Phelan-McDermid Disease; Deletion 22q13 Syndrome; 22q13 Deletion Syndrome; Chromosome 22q13.3 Syndrome; Monosomy 22q13
PLEURAL MESOTHELIOMA	Malignant Mesothelioma of the Pleura
POMPE DISEASE - INFANTILE	Acid Maltase Deficiency (AMD), Alpha-1,4 Glucosidase Deficiency, Cardiomegalia Glycogenica Diffusa, Generalized

	Glycogenosis (Cardiac), Glycogen Storage Disease type II , Glycogenosis type II, Lysosomal Glucosidase Deficiency
PRIMARY CENTRAL NERVOUS SYSTEM LYMPHOMA	PCNSL; Primary CNS Lymphoma; Reticulum Cell Sarcoma; Diffuse Histiocytic Lymphoma; Brain Lymphoma; Cerebral Lymphoma; Primary Lymphoma of the Central Nervous System; Lymphoma-Brain
PRIMARY EFFUSION LYMPHOMA	PEL; Body Cavity Lymphoma; Body Cavity-Based Lymphoma; AIDS - Related Lymphoma
PRIMARY PROGRESSIVE APHASIA	PPA; Semantic Dementia
PROGRESSIVE BULBAR PALSY	Progressive Bulbar Atrophy; Bulbar Paralysis; Bulbar Palsy; Fazio-Londe Syndrome ; Fazio-Londe Disease
PROGRESSIVE MULTIFOCAL LEUKOENCEPHALOPATHY	PML
PROGRESSIVE SUPRANUCLEAR PALSY	Richardson-Steele-Olszewski syndrome; Steele-Richardson-Olszewski syndrome; Richardson Syndrome; Nuchal Dystonia Dementia Syndrome; Progressive Supranuclear Ophthalmoplegia; Supranuclear Palsy-Progressive; PSP
PROSTATE CANCER-HORMONE REFRACTORY DISEASE	Jewett Stage D2 Prostate Cancer; Stage D2 Metastatic Prostate Cancer; Hormone Refractory Metastatic Prostate Cancer; Metastatic Castration-Resistant Prostate Cancer
PULMONARY ATRESIA	Pulmonary valve atresia with intact ventricular septum; PA-IVS; Pulmonary valve atresia with ventricular septal defect; PA-VSD
PULMONARY KAPOSI SARCOMA	Pulmonary KS; PKS
RETINOPATHY OF PREMATURITY - STAGE V	ROP Stage V; Retinopathy of Prematurity Type V; Retrolental Fibroplasia
RETT (RTT) SYNDROME	MECP2 Related Disorder, RTT, RTS
REVESZ SYNDROME	Revesz-DeBuse Syndrome; Revesz-Debuse Disease; Revesz Disorder; Exudative Retinopathy with Bone Marrow Failure; Exudative Retinopathy with Bone Marrow Failure and Cerebellar Hypoplasia
RHABDOMYOSARCOMA	Alveolar Rhabdomyosarcoma; Embryonal

DETAILED LISTING OF COMPASSIONATE ALLOWANCE CASES

	Rhabdomyosarcoma; Sarcoma Botryoides; Soft Tissue Sarcoma
RHIZOMELIC CHONDRODYSPLASIA PUNCTATA	RCDP; RCP; Chondrodysplasia Punctata Rhizomelic; Rhizomelic Chondrodysplasia Punctata Classic Type; Rhizomelic Chondrodysplasia Punctata Type 1; RCDP1; Rhizomelic Chondrodysplasia Punctata Type 2; RCDP2; Rhizomelic Chondrodysplasia Punctata Type 3; RCDP3
ROBERTS SYNDROME	Roberts Disease; Appelt-Gerken-Lenz Syndrome; Appelt-Gerken-Lenz Disease; Hypomelia Hypotrichosis Facial Hemangioma Syndrome; Hypomelia Hypotrichosis Facial Hemangioma Disease; Pseudothaliodomide Syndrome; Roberts SC Phocomelia Disease; Tetraphocomelia-Cleft-Palate Syndrome
SALIVARY CANCERS	Salivary Glands Cancer, Anaplastic Small Cell Carcinoma of the Salivary Glands, Adenosquamous Carcinoma of the Salivary Glands, Anaplastic Small Cell Carcinoma, Adenosquamous Carcinoma
SANDHOFF DISEASE	Gangliosidosis GM2 type II, Gangliosidosis Beta Hexosaminidase B Deficiency, Hexosaminidases A and B Deficiency
SCHINDLER DISEASE - TYPE I	Neuroaxonal Dystrophy Schindler type; Alpha-N-Acetylgalactosaminidase Deficiency Type 1; NAGA Deficiency Type 1; Alpha NAGA Deficiency Schindler Type; Schindler Disease Type 1 Infantile Onset; Schindler Disease Infantile Type; Schindler Disease Classic Form
SECKEL SYNDROME	Seckel Type Dwarfism; Seckel Type Premordial Dwarfism; Microcephalic Primordial Dwarfism Seckel Type; Seckel Syndrome Types 1-4; Nanocephalic Dwarfism
SEVERE COMBINED IMMUNODEFICIENCY-CHILDHOOD	Pediatric Severe Combined Immunodeficiency; X-Linked SCID; Adenosine Deaminase Deficiency; ADA-SCID; Classical X-linked SCID; Bubble Boy Disease
SINGLE VENTRICLE	Common Ventricle; Common-Inlet Left Ventricle; Double-Inlet Left Ventricle; Double-Inlet Ventricle
SINONASAL CANCER	Sinonasal Malignancy; Sinonasal Undifferentiated Carcinoma; SNUC; Highly Aggressive Undifferentiated Carcinoma of the Nasal Cavity and Paranasal Sinuses

DETAILED LISTING OF COMPASSIONATE ALLOWANCE CASES

SJÖGREN-LARSSON SYNDROME	Fatty Acid Alcohol Oxidoreductase Deficiency; FALDH Deficiency; Fatty Aldehyde Dehydrogenase Deficiency; Congenital Icthyosis Mental Retardation Spasticity Syndrome; Ichthyosis Spastic Neurologic Mental Retardation Disorder; Congenital Ichthyosis Oligophrenia and Spastic Paresis Syndrome; Ichthyosis Oligophrenia and Spastic Tetraplegia Syndrome; Ichthyosis Oligophrenia Syndrome
SKIN MALIGNANT MELANOMA	Cutaneous Melanoma; Metastatic Melanoma of the Skin; Skin Malignant Melanoma; Malignant Melanoma
SMALL CELL CANCER OF THE LARGE INTESTINE	Cancer of the Colon, Colorectal Small Cell Cancer, Small Cell Carcinoma of the Large Intestine
SMALL CELL CANCER OF THE PROSTATE	Cancer of the Prostate Gland, Prostatic Cancer, Prostatic Carcinoma, Small Cell Carcinoma of the Prostate
SMALL CELL CANCER OF THE THYMUS	Thymic Small Cell Carcinoma; Small Cell Carcinoma of the Thymus
SMALL CELL CANCER OF THE FEMALE GENITAL TRACT	Small-Cell Carcinoma of the Uterus; Small-Cell Carcinoma of the Vagina; Small-Cell Carcinoma of the Vulva; Small-Cell Carcinoma of the Endometrium; Small-Cell Carcinoma of the Fallopian Tube; Small-Cell Cancer of the Ovary; Small-Cell Cancer of the Cervix;Vaginal Small-Cell Carcinoma; Vaginal Small-Cell Cancer; Primary Vaginal Small-Cell Carcinoma; Poorly Differentiated Neuroendocrine Cancer of the Vaginal Tract
SMALL CELL LUNG CANCER	Small Cell Lung Carcinoma, Oat cell Lung cancer, Mixed small cell/large cell Lung carcinoma, Combined small cell Lung carcinoma
SMALL INTESTINE CANCER	Small Intestine Adenocarcinoma, Small Intestine Sarcoma, Small Intestine Gastrointestinal Stromal Tumor, Small Intestine Carcinoid, Small Intestine Carcinoma
SMITH LEMLI OPITZ SYNDROME	SLO Syndrome; SLOS; RSH Syndrome; DHCR7 Deficiency; Smith-Lemli-Optiz Syndrome Type II
SOFT TISSUE SARCOMA	Malignant Soft Tissue Sarcoma; Malignant Soft Tissue Tumor; Malignant Mesenchymal Tumor; Mesenchymal Sarcoma
SPINAL MUSCULAR ATROPHY (SMA) - TYPES 0 AND 1	Prenatal onset arthrogryposis multiplex congenital (SMA0), Werdnig-Hoffman disease-Infantile Muscular Atrophy (SMA1)

DETAILED LISTING OF COMPASSIONATE ALLOWANCE CASES

SPINAL NERVE ROOT CANCER	Tumor-Spinal cord; Spinal Root Neoplasm; Spinal Cord Tumor
SPINOCEREBELLAR ATAXIA	SCA; Infantile-onset Spinocerebellar Ataxia; Autosomal Dominant Spinocerebellar Ataxia (ADSCA)
STIFF PERSON SYNDROME	Stiff Man Syndrome; Stiffperson's Syndrome; Moersch-Woltmann Syndrome; Moersch-Woltman Condition; SPS; SMS; Stiff Baby Syndrome; Focal Stiff Person Syndrome; Stiff Limb Syndrome; Jerking Stiff Person Syndrome; Progressive Encephalomyelitis with Rigidity and Myoclonus; PERM
STOMACH CANCER	Gastric Cancer, Gastric Carcinoma, Stomach Carcinoma
SUBACUTE SCLEROSING PANENCEPHALITIS	SSPE; Dawson Disease; Dawson’s Encephalitis; Panencephalitis Subacute Sclerosing; Subacute Inclusion Body Encephalitis
TABES DORSALIS	Progressive Locomotor Ataxia; Locomotor Ataxia; Syphilitic Spinal Sclerosis; Syphilitic Myelopathy
TAY SACHS DISEASE, INFANTILE TYPE	Infantile-onset or infantile form TSD; Amaurotic Familial Idiocy; Amaurotic Familial Infantile Idiocy; Cerebromacular Degeneration; GM2 Gangliosidosis Type 1; GM2 Gangliosidosis (B variant); HexA deficiency; Hexosaminidase A deficiency; Hexosaminidase b-subunit deficiency (variant B); Infantile Cerebral Ganglioside; Infantile Cerebral Ganglioside Lipidosis; Tay-Sachs Sphingolipidosis
THANATOPHORIC DYSPLASIA, TYPE 1	TD1; TDI; Dwarf, Thanatophoric; Thanatophoric Dwarfism; Thanatophoric Short Stature
THE ALS/PARKINSONISM DEMENTIA COMPLEX	The Amyotrophic Lateral Sclerosis/Parkinsonism Dementia Complex; ALS/PDC; ALS/PDC syndrome of Guam; Kii ALS-PDC; Lytico-Bodig Motor Neuron Disease; Guam Disease; PDALS; Parkinsonism Dementia-ALS complex
THYROID CANCER	Anaplastic Thyroid Carcinoma, Anaplastic Thyroid Cancer
TRANSPLANT CORONARY ARTERY VASCULOPATHY	Transplant Cardiac Allograft Vasculopathy; Cardiac Transplant Vasculopathy
TRICUSPID ATRESIA	Tricuspid Valve Atresia
ULLRICH CONGENITAL MUSCULAR DYSTROPHY	UCMD; Ullrich Disease; Ullrich Scleroatonic Muscular Dystrophy; Muscular Dystrophy Scleroatonic

DETAILED LISTING OF COMPASSIONATE ALLOWANCE CASES

URETER CANCER	Metastatic Transitional Cell Carcinoma of the Ureter (Stage IV), Squamous Cell Cancer of the Ureter, Squamous Cell Carcinoma of the Ureter, Adenocarcinoma of the Ureter, Ureter Carcinoma
USHER SYNDROME TYPE I	Usher Syndrome I; Usher Disease; Usher-Hallgren Syndrome; Halgren Disease; RP-Dysacusis Syndrome; Dystropia Retinae Dysacusis Disease; Graefe-Usher Syndrome; Retinitis Pigmentosa Deafness Syndrome
VENTRICULAR ASSIST DEVICE RECIPIENT- LEFT, RIGHT, OR BIVENTRICULAR	VAD; Left Ventricular Assist Device Recipient; LVAD Recipient; Right Ventricular Assist Device Recipient; RVAD Recipient; Biventricular Assist Device Recipient; BiVAD Recipient; Heart Pump Recipient; Implantable Ventricular Assist Device Recipient; VAD Recipient; Long Term Ventricular Assist Device Recipient; VAD Recipient; Left Ventricular Assist System Recipient; LVAS Recipient; Heart Assist System Implantation Recipient
WALKER WARBURG SYNDROME	WWS; WWS Muscular Dystrophy; Warburg syndrome; Pagon Syndrome; Chemke Syndrome; Hydrocephalus, Agyria and Retinal Dysplasia; HARD +/- Syndrome; HARDE syndrome; Muscle-Eye-Brain disease; Cerebro-oculomuscular syndrome; Lissencephaly type II; Oculocerebral malformation
WOLF-HIRSCHHORN SYNDROME	WHS; Chromosome 4p Deletion Syndrome; Chromosome 4p Monosomy; Del(4p) Syndrome; Monosomy 4p; Partial Monosomy 4p; Pitt-Rogers-Danks Syndrome; PRDS
WOLMAN DISEASE	Acid Lipase disease; Cholesterol Ester Storage disease; Acid Cholesterol Ester Hydrolase deficiency, Wolman Type; Lysosomal Acid Lipase deficiency, Wolman Type
X-LINKED LYMPHOPROLIFERATIVE DISEASE	Duncan Syndrome; Duncan Disease; Epstein-Barr Virus-Induced Lymphoproliferative Disease in Males; Immunodeficiency-5 (IMD5); EBV Susceptibility (EBVS); Purtilo syndrome
X-LINKED MYOTUBULAR MYOPATHY	Myotubular Myopathy; X-Linked Centronuclear Myopathy; Centronuclear Myopathy; Classic X-Linked Myotubular Myopathy
XERODERMA PIGMENTOSUM	XP; DeSanctis-Cacchione Syndrome; Xeroderma Pigmentosum Variant Type; XP-V
ZELLWEGER SYNDROME	ZS; Peroxisomal Biogenesis Disorder (PBD); Zellweger Spectrum

	Syndrome (ZSS); Cerebro-hepato-renal Syndrome