NORD'S Rare Disease Database

11q Syndrome
13q Syndrome
18p- Syndrome
4 Q Syndrome
5-Oxoprolinuria
ACTH Deficiency
AIDS (Acquired Immune Deficiency Syndrome)
AIDS Dysmorphic Syndrome
APECED Syndrome
Aarskog Syndrome
Aase-Smith Syndrome
Acanthocheilonemiasis
Acanthocytosis
Acanthosis Nigricans
Achalasia
Achard-Thiers Syndrome
Achondroplasia
Acidemia, Isovaleric
Acidemia, Methylmalonic
Acidemia, Propionic
Acne
Acne Rosacea
Acoustic Neuroma
Acrodermatitis Enteropathica
Acrodysostosis
Acromegaly
Adams-Oliver Syndrome
Addison's Disease
Adie Syndrome
Adrenal Hyperplasia, Congenital
Adrenoleukodystrophy
Agammaglobulinemias, Primary
Agenesis of Corpus Callosum
Agranulocytosis, Acquired
Ahumada-del Castillo Syndrome
Aicardi Syndrome
Alagille Syndrome
Albinism
Alexander's Disease
Alkaptonuria
Alopecia Areata
Alpers Disease
Alpha-1-Antitrypsin Deficiency
Alport Syndrome
Alveolitis, Extrinsic Allergic
Alveolitis, Fibrosing
Alzheimer's Disease
Amelogenesis Imperfecta
Amenorrhea, Primary
Amniotic Bands
Amyloidosis
Amyotrophic Lateral Sclerosis (Lou Gehrig's Disease)
Anaphylaxis
Andersen Disease (GSD IV)
Anemia, Aplastic
Anemia, Blackfan-Diamond
Anemia, Fanconi's
Anemia, Hemolytic, Warm Antibody
Anemia, Hemolytic, Acquired Autoimmune
Anemia, Hemolytic, Cold Antibody
Anemia, Hereditary Non-Spherocytic Hemolytic
Anemia, Hereditary Spherocytic Hemolytic
Anemia, Megaloblastic
Anemia, Pernicious
Anemia, Sideroblastic
Anencephaly
Angelman Syndrome
Angioedema, Hereditary
Anindia
Ankylosing Spondylitis
Anocontia
Anorexia Nervosa
Antisocial Personality Disorder
Antithrombin III Deficiency, Congenital
Antley-Bixler Syndrome
Apert Syndrome
Apnea, Infantile
Apnea, Sleep
Apraxia
Arachnoiditis
Arginase Deficiency
Arginino Succinic Aciduria
Arnold-Chiari Syndrome
Arteriovenous Malformation
Arteritis, Giant Cell
Arteritis, Takayasu
Arthritis, Infectious
Arthritis, Psoriatic
Arthrogryposis Multiplex Congenita
Asherman's Syndrome
Asperger's Syndrome
Aspergillosis
Astrocytoma, Malignant
Astrocytoma, Benign
Ataxia Telangiectasia
Ataxia, Friedreich's
Ataxia, Hereditary
Ataxia, Marie's
Atrial Septal Defects
Attention Deficit Hyperactivity Disorder
Autism
Babesiosis
Balantidiasis
Balo Disease
Banti's Syndrome
Barrett Syndrome
Bartonellosis
Bartter's Syndrome
Batten Disease
Beckwith-Wiedemann Syndrome
Behcet's Syndrome
Bejel
Bell's Palsy
Benign Essential Tremor Syndrome
Bernard-Soulier Syndrome
Beryllosis
Biliary Atresia
Binswanger's Disease
Blastomycosis
Blepharospasm, Benign Essential
Bloom Syndrome
Blue Diaper Syndrome
Blue Rubber Bleb Nevus
Botulism
Bowen's Disease
Brain Tumors, General
Branchio-Oculo-Facial Syndrome
Broad Beta Disease
Bronchopulmonary Dysplasia
Brown Syndrome
Brucellosis
Bubonic Plague
Budd-Chiari Syndrome
Buerger's Disease
Bullimia
Bullous Pemphigoid
Burning Mouth Syndrome
CHARGE Association
Cancer, Breast
Cancer, Colon
Cancer, Lung
Cancers, Skin, General
Candidiasis
Carbamyl Phosphate Synthetase Deficiency
Carboxylase Deficiency, Multiple
Carcinoid Syndrome
Carcinoma, Renal Cell
Carcinoma, Squamous Cell
Cardio-auditory Syndrome
Carnitine Deficiency Syndromes, Hereditary
Carnosinemia
Caroli Syndrome
Carpal Tunnel Syndrome
Carpenter Syndrome
Castleman's Disease
Cataracts
Cavernous Hemangioma
Celiac Sprue
Central Core Disease
Central Hypoventilation Syndrome, Congenital
Cerebellar Degeneration, Subacute
Cerebral Palsy
Cerebro-Costo-Mandibular Syndrome
Cerebro-Oculo-Facio-Skeletal Syndrome
Chagas' Disease
Chalazion
Charcot-Marie-Tooth Disease
Chediak-Higashi Syndrome
Chiari-Frommel Syndrome
Chikungunya
Chlamydia
Cholangitis, Primary Sclerosing
Cholecystitis
Cholera
Cholestasis
Chorea, Syndenham's
Choroideremia
Chromosome 11-14 Translocation
Chronic Fatigue Syndrome
Churg-Strauss Syndrome
Ciguatera Fish Poisoning
Cirrhosis, Primary Biliary
Citrullinemia
Cleft Lip and Cleft Palate (Hare lip)
Clubfoot
Coats Disease
Cockayne Syndrome
Coffin-Lowry Syndrome
Coffin-Siris Syndrome
Cohen Syndrome
Colitis, Collagenous
Colitis, Ulcerative
Colorado Tick Fever
Condyloma
Cone Dystrophy
Conn Syndrome
Conradi-Hunermann Syndrome
Conversion Disorder
Cor Biloculare
Cor Triatriatum
Corneal Dystrophy
Cornelia de Lange Syndrome
Cowpox
Craniometaphyseal Dysplasia
Creutzfeldt-Jakob Disease
Cri du Chat Syndrome
Crohn's Disease
Cronkhite-Canada Disease
Crouzon Disease
Cryptococcosis
Cushing Syndrome
Cutis Laxa
Cystic Fibrosis
Cystic Hygroma
Cysticercosis
Cystinosis
Cystinuria
Cytomegalovirus Infection
Dandy-Walker Syndrome
Darier Disease
Dejerine-Sottas Disease
Dengue Fever
Dentin Dysplasia, Radicular
Dentin Dysplasia, Coronal
Dentinogenesis Imperfecta, Type III
Depersonalization Disorder
Dercum Disease
Dermatitis, Atopic
Dermatitis, Contact
Dermatomyositis
Devic Disease
Dextrocardia with Situs Inversus
DiGeorge Syndrome
Diabetes, Insipidus
Diabetes, Insulin-Dependent
Diastrophic Dysplasia
Dilatation of the Pulmonary Artery, Idiopathic
Diverticulitis
Diverticulosis
Down Syndrome
Dracunculosis
Duane Syndrome
Dubin Johnson Syndrome
Dubowitz Syndrome
Duhring Disease
Dupuytren's Contracture
Dysautonomia, Familial
Dyslexia
Dysphonia, Chronic Spasmodic
Dysplasia Polyostotic Fibrous
Dysplasia, Epiphysealis Hemimelica
Dysplasia, Fibrous
Dysplastic Nevus Syndrome
Dysthymia
Dystonia, Torsion
Dystrophy, Asphyxiating Thoracic
Dystrophy, Myotonic
Eales Disease
Eaton-Lambert Syndrome
Ectodermal Dysplasias
Edema, Idiopathic
Ehlers-Danlos Syndrome
Eisenmenger Syndrome
Elephantiasis
Empty Sella Syndrome
Encephalitis, Herpetic
Encephalitis, Rasmussen's
Encephalitis, Japanese
Encephalomyelitis, Myalgic
Endocarditis, Infective
Endometriosis
Endomyocardial Fibrosis
Engelmann Disease
Enterobiasis
Eosinophilia Myalgia
Eosinophilic Fasciitis - NA (JT)
Epidermal Nevus Syndrome
Epidermolysis Bullosa
Epidermolytic Hyperkeratosis
Epididymitis
Epilepsy
Epilepsy, Myoclonic Progressive Familial
Epitheliopathy, Acute Posterior Multifocal Placoid Pigment
Erb's Palsy
Erysipelas
Erythema Multiforme
Erythromelalgia
Exstrophy of the Bladder
FG Syndrome
Fabry Disease
Factor IX Deficiency
Factor XIII Deficiency
Fahr's Disease
Fairbank Disease
Farber's Disease
Fascioliasis
Felty Syndrome
Fetal Alcohol Syndrome
Fiber Type Disproportion, Congenital
Fibroelastolosis, Endocardial
Fibromatosis, Congenital Generalized
Fibrositis
Filariasis
Forbes Disease (GSD III)
Forbes-Albright Syndrome
Forestier's Disease
Formaldehyde Poisoning
Fragile X Syndrome
Fraser Syndrome
Freeman-Sheldon Syndrome
Frey's Syndrome
Froelich's Syndrome
Fructose Intolerance, Hereditary
Fructosuria
Galactosemia
Ganglioside Sialidase Deficiency
Gardner Syndrome
Gastritis, Chronic Erosive
Gastritis, Eosinophilic
Gastritis, Giant Hypertrophic
Gastroesophageal Reflux
Gaucher Disease
Gianotti-Crosti Syndrome
Giardiasis
Gilbert Syndrome
Glioblastoma Multiforme
Glucose-6-Dehydrogenase Deficiency
Glucose-Galactose Malabsorption
Glutaricaciduria I
Glutaricaciduria II
Glycogen Storage Disease VIII
Goldenhar Syndrome
Goodpasture Syndrome
Gordon Syndrome
Gorham's Disease
Gottron's Syndrome
Graft vs Host Disease
Granuloma Annulare
Granulomatosis, Lymphatoid
Granulomatosis, Wegener's
Granulomatous Disease, Chronic
Graves' Disease
Greig Cephalopolysyndactyly Syndrome
Guillain-Barre Syndrome
Hageman Factor Deficiency
Hairy Tongue
Hallermann-Streiff Syndrome
Hallervorden-Spatz Disease
Hand-Foot-Mouth Disease
Hartnup Disease
Hashimoto's Syndrome
Headache, Cluster
Heart Block, Congenital
Heavy Metal Poisoning
Hematuria, Benign Familial
Hemochromatosis, Hereditary
Hemoglobinuria, Paroxysmal Cold
Hemoglobinuria, Paroxysmal Nocturnal
Hemolytic Uremic Syndrome
Hemophilia
Hemorrhagic Telangiectasia, Hereditary
Hepatic Fibrosis, Chronic - NA (VW)
Hepatitis B
Hepatitis, Non-A, Non-B (Hepatitis C)
Hepatitis, Neonatal
Hepatorenal Syndrome
Hermaphroditism, True
Herpes Zoster
Herpes, Neonatal
Hers Disease (GSD VI)
Hiccups, Chronic
Hidradenitis Suppurativa
Hirsprung's Disease
Histidinemia
Hsiicytosis-X
Hodgkin's Disease
Holoprosencephaly
Holt-Oram Syndrome
Homocystinuria
Hunter Syndrome
Huntington's Disease
Hurler Syndrome
Hutchinson-Gilford Syndrome
Hydranencephaly
Hydrocephalus
Hypercholesterolemia
Hyperchylomicronemia
Hyperekplexia
Hyperhidrosis
Hyperkalemia
Hyperlipoproteinemia, Type IV
Hyperoxaluria Type I, Primary
Hyperprolinemia Type II
Hyperprolinemia Type I
Hypertension
Hyperthermia
Hypochondroplasia
Hypoglycemia
Hypokalemia
Hypoparathyroidism
Hypophosphatasia
Hypoplastic Left Heart Syndrome
Hypotension, Orthostatic
Hypothyroidism
Hypotonia, Benign Congenital
I-Cell disease
IGA Nephropathy
Ichthyosis
Ichthyosis Congenita
Ichthyosis Hystrix, Curth-Macklin Type
Ichthyosis Vulgaris
Ichthyosis, Erythrokeratolysis Hiemalis
Ichthyosis, Lamellar Recessive
Ichthyosis, Harlequin Type
Ichthyosis, Erythrokeratodermia Progressiva Symmetrica
Ichthyosis, Peeling Skin Syndrome
Ichthyosis, Tay Syndrome
Ichthyosis, CHILD Syndrome
Ichthyosis, Giroux-Barbeau Syndrome
Ichthyosis, Chanarin Dorfman Syndrome
Ichthyosis, Erythrokeratodermia Variabilis
Ichthyosis, Netherton Syndrome
Ichthyosis, Keratosis Follicularis Spinulosa Decalvans
Ichthyosis, X-Linked
Ichthyosis, Sjogren Larsson Syndrome
Imperforate Anus
Incontinentia Pigmenti
Interstitial Cystitis
Intestinal Pseudoobstruction
Irritable Bowel Syndrome
Isaacs' Syndrome
Ivemark Syndrome
Jarcho-Levin Syndrome
Job Syndrome
Joseph's Disease
Joubert Syndrome
Jumping Frenchmen of Maine
Kallman Syndrome
Kartagener Syndrome
Kawasaki Disease
Kearns-Sayre Syndrome
Keratoconjunctivitis, Vernal
Keratoconus
Keratomalacia
Keratosis, Seborrheic
Kernicterus
Kienboeck Disease
Kikuchi's Disease - NA (VW)
Kinsborne Syndrome
Klinefelter Syndrome
Klippel-Feil Syndrome
Klippel-Trenaunay Syndrome
Kniest Syndrome
Kohler Disease
Korsakoff's Syndrome
Kufs Disease
Kugelberg-Welander Syndrome
Lactose Intolerance
Landau-Kleffner Syndrome
Laron Dwarfism
Larsen Syndrome
Laurence-Moon-Biedl Syndrome
Leber's Congenital Amaurosis
Leber's Optic Atrophy
Legg-Calve-Perthes Syndrome
Legionnaire's Disease
Leigh's Disease
Leiner Disease
Leopard Syndrome
Leprechaunism
Leprosy
Leptospirosis
Lesch-Nyhan Syndrome
Leukemia, Chronic Lymphocytic
Leukemia, Chronic Myelogenus
Leukemia, Hairy Cell
Leukodystrophy, Canavan's
Leukodystrophy, Metachromatic
Leukodystrophy, Krabbe's
Leukodystrophy
Lichen Planus
Lichen Sclerosus
Lipodystrophy
Lissencephaly
Listeriosis
Locked-In Syndrome
Lowe's Syndrome
Lupus
Lyelles Syndrome
Lyme Disease
Lymphadenopathy, Angioimmunoblastic with Dysproteinemia
Lymphangioma, Cavernous
Lymphangiomyomatosis
Lymphedema, Hereditary
Lymphocytic Infiltrate of Jessner
Lymphoma, Gastric, Non-Hodgkin's Type
Macroglossia
Macular Degeneration, Polymorphic
Macular Degeneration
Madelung's Disease
Maffucci Syndrome
Malaria
Malignant Hyperthermia
Mallory-Weiss Syndrome
Manic Depression, Bipolar
Mannosidosis
Maple Syrup Urine Disease
Marcus Gunn Phenomenon
Marfan Syndrome
Marhall-Smith Syndrome
Maroteaux-Lamy Syndrome
Mastocytosis
May-Hegglin Anomaly
McArdle Disease (GSD V)
McCune-Albright Syndrome
Measles
Meckel Syndrome
Mediterranean Fever, Familial
Medium-Chain Acyl CoA Dehydrogenase Deficiency
Medullary Cystic Disease
Medullary Sponge Kidney
Medulloblastoma
Meige Syndrome
Melanoma, Malignant
Melkersson-Rosenthal Syndrome
Melnick-Needles Syndrome
Meniere Disease
Meningioma
Meningitis
Meningitis, Bacterial
Meningitis, Tuberculous
Meningitis, Meningincoccal
Menkе's Disease
Mesenteritis, Retractile
Metaphyseal Chondrodysplasia, McKusick Type
Microvillus Inclusion Disease
Mikulicz Syndrome
Mitral Valve Prolapse Syndrome
Mixed Connective Tissue Disease (MCTD)
Moebius Syndrome
Morquio Syndrome
Motor Neuron Disease
Mountain Sickness, Acute
Moyamoya Disease
Mucha-Haberman Disease
Mucopolysaccharidosis
Multiple Sclerosis
Multiple Sulfatase Deficiency
Mumps
Muscular Dystrophy, Duchenne
Muscular Dystrophy, Batten Turner
Muscular Dystrophy, Becker
Muscular Dystrophy, Emery-Dreifuss
Mutism, Elective
Myasthenia Gravis
Mycosis Fungoides
Myelitis
Myelofibrosis-Osteosclerosis
Myeloma, Multiple
Myoclonus
Myopathy, Scapuloperoneal
Myositis Ossificans
Myositis, Inclusion Body
N-Acetyl Glutamate Synthetase Deficiency
Nail-Patella Syndrome
Narcolepsy
Nelson Syndrome
Nemaline Myopathy
Neurasthenia
Neurofibromatosis
Neuroleptic Malignant Syndrome
Neuropathy, Peripheral
Neuropathy, Hereditary Sensory, Type II
Neuropathy, Congenital Hypomyelination
Neuropathy, Hereditary Sensory, Type
Neuropathy, Giant Axonal
Neutropenia, Cyclic
Neutropenia, Chronic
Nevoid Basal Carcinoma Syndrome
Nezelof's Syndrome
Niemann-Pick Disease
Nocardiosis
Non-Ketotic Hyperglycinemia
Noonan Syndrome
Norrie Syndrome
Nystagmus, Benign Paroxysmal Positional
Obsessive Compulsive Disorder
Olivopontocerebellar Atrophy
Ollier Disease
Opitz Syndrome
Opportunistic Infections
Oral-Facial-Digital Syndrome
Organic Mood Syndrome
Organic Personality Syndrome
Ornithine Transcarbamylase Deficiency
Osgood-Schlatter's Disease
Osteogenesis Imperfecta
Osteomyelitis
Osteonecrosis
Osteopetrosis
Paget's Disease
Paget's Disease of the Breast
Pallister-Killian Syndrome
Pancreatic Islet-Cell Tumors
Panic-Anxiety Syndrome
Papillitis
Paracoccidioidomycosis
Paraplegia, Hereditary Spastic
Parenchymatous Cortical Degeneration of the Cerebellum
Parkinson's Disease
Parry-Romberg Syndrome
Pars Planitis
Parsonnage-Turner Syndrome
Patulous Eustachian Tube
Pelizaeus-Merzbacher Brain Sclerosis
Pemphigoid, Benign Mucosal
Pemphigus
Penia X Syndrome
Perniosis
Pertussis
Peutz-Jeghers Syndrome
Peyronie Disease
Pfeiffer syndrome
Phenylketonuria
Pheochromocytoma
Phocomelia Syndrome
Pica
Pick's Disease
Pierre-Robin Syndrome
Pinta
Pityriasis Rubra Pilaris
Pneumonia, Eosinophilic
Pneumonia, Interstitial
Poems Syndrome
Poland Syndrome
Polyarteritis Nodosa
Polychondritis
Polycystic Kidney Diseases
Polycystic Liver Disease
Polycythemia Vera
Polymyalgia Rheumatica
Polymyositis
Polyposis, Familial
Pompe Disease (GSD II)
Porphyria
Porphyria Cutanea Tarda
Porphyria, Acute Intermittent
Porphyria, ALA-D
Porphyria, Congenital Erythropoietic
Porphyria, Erythropoietic Protoporphyria
Porphyria, Hereditary Coproporphyria
Porphyria, Variegate
Post-Polio Syndrome
Posterior Uveitis
Prader-Willi Syndrome
Precocious Puberty
Primary Lateral Sclerosis
Proctitis
Progressive Supranuclear Palsy
Prostate Cancer
Prostatitis
Proteus Syndrome
Prune Belly Syndrome
Pseudo-Hurler Polydystrophy
Pseudocholinesterase Deficiency
Pseudogout
Pseudohypoparathyroidism
Pseudomyxoma Peritonei
Pseudotumor Cerebri
Pseudoxanthoma Elasticum
Psittacosis
Psoriasis
Pulmonary Alveolar Proteinosis
Pulmonary Hypertension, Secondary
Pulmonary Hypertension, Primary
Pure Red Cell Aplasia
Purpura, Idiopathic Thrombocytopenic
Purpura, Schoenlein-Henoch
Purpura, Thrombotic Thrombocytopenic
Pyoderma Gangrenosum
Pyruvate Dehydrogenase Deficiency
Pyruvate Kinase Deficiency
Q Fever
Rabies
Radiation Syndromes
Rapp-Hodgkin's Syndrome
Raynaud's Disease and Phenomenon
Reflex Sympathetic Dystrophy Syndrome
Refsum Syndrome
Reifenstein Syndrome
Reiter's Syndrome
Renal Agenesis, Bilateral
Renal Glycosuria
Respiratory Distress Syndrome, Infant
Respiratory Distress Syndrome, Adult
Restless Legs Syndrome
Retinitis Pigmentosa (RP)
Retinoblastoma
Retinopathy, Arteriosclerotic
Retinopathy, Diabetic
Retinopathy, Hypertensive
Retinoschisis
Retrolental Fibroplasia
Retroperitoneal Dysplasia
Rett Syndrome
Reye Syndrome
Rh Disease
Rheumatic Fever
Rickets, Hypophosphatemic
Rieger Syndrome
Robert's Syndrome
Robinow Syndrome
Rocky Mountain Spotted Fever
Romano-Ward Syndrome
Roseola Infantum
Rothmund-Thomson Syndrome
Roussy-Levy Syndrome
Rubella
Rubella, Congenital
Rubinstein-Taybi Syndrome
Russell-Silver Syndrome
Saethre-Chotzen Syndrome
Sandhoff Disease
Sanfilippo Syndrome
Sarcoidosis
Sarcoma Ewing's
Schmidt Syndrome
Schwachman Syndrome
Scleroderma
Seckel Syndrome
Seitelberger Disease
Septo-Optic Dysplasia
Severe Combined Immunodeficiency
Sheehan Syndrome
Shprintzen Syndrome
Shy-Drager Syndrome
Sialadenitis
Sialidosis
Sickle Cell Disease
Simian B Virus Infection
Sirenomelia Sequence
Sjogren Syndrome
Sly Syndrome
Smith-Lemli-Opitz Syndrome
Sotos Syndrome
Spasmodic Torticollis
Spina Bifida
Split-Hand Deformity
Spondyloepiphyseal Dysplasia, Congenital
Spondyloepiphyseal Dysplasia Tarda
Stein-Leventhal Syndrome
Stenosis, Spinal
Stevens-Johnson Syndrome
Stickler Syndrome
Stiff Man Syndrome
Stroke
Sturge-Weber Syndrome
Subacute Sclerosing Panencephalitis
Sucrose-Isomaltose Malabsorption, Congenital
Sudden Infant Death Syndrome
Sutton's Disease II
Sweet Syndrome
Syphilis, Acquired
Syphilis, Congenital
Syringobulbia
Syringomyelia
TORCH Syndrome
Tangier Disease
Tardive Dyskinesia
Tarsal Tunnel Syndrome
Tarui Disease (GSD VII)
Tay-Sachs Disease
Telecanthus
Temporomandibular Joint Dysfunction (TMJ)
Tethered Spinal Cord Disease
Tetrahydrobiopterin Deficiency
Tetralogy of Fallot
Thalamic Syndrome (Dejerine-Roussy Syndrome)
Thalassemia Minor
Thalassemia Major
Thomsen Disease
Thrombasthenia
Thrombocythemia, Essential
Thrombocytopenia Absent Radius Syndrome
Thrombocytopenia, Essential
Tietze Syndrome
Tinnitus
Tolosa-Hunt Syndrome
Tongue Carcinoma
Tongue, Fissured
Tongue, Geographic
Tooth and Nail Syndrome
Tourette Syndrome
Townes-Brock Syndrome
Toxic Shock Syndrome
Toxocariasis
Toxoplasmosis
Treacher Collins Syndrome, Familial
Tricho-Dento-Osseous Syndrome
Trichorhinophalangeal Syndrome
Trichotillomania
Trigeminal Neuralgia (Tic Douloureux)
Triploid Syndrome
Trisomy
Trisomy 13 Syndrome
Trisomy 18 Syndrome
Tropical Sprue
Truncus Arteriosus, Persistent
Tuberculosis
Tuberous Sclerosis
Turner Syndrome
Typhoid
Tyrosinemia, Hereditary
Urticaria Pigmentosa
Urticaria, Cholinergic
Urticaria, Cold
Urticaria, Papular
Urticaria, Physical
Usher's Syndrome
VACTERL Association
Valinemia
Varicella Zoster Virus
Vascular Malformations of the Brain
Vasculitis
Vasculitis, Chronic Necrotizing
Ventricular Septal Defects
Vitamin B12 Deficiency
Vitamin E Deficiency
Vitiligo
Von Gierke Disease (GSD I)
Von Hippel-Lindau Disease
Von Willebrand's Disease
Vulvovaginitis
Waardenburg Syndrome
Waldenstrom Macroglobulinemia
Waldmann Disease
Weaver Syndrome
Weber-Christian Disease
Weil Syndrome
Werdnig-Hoffmann Disease
Werner Syndrome
Whipple's Disease
Wiecker Syndrome
Williams Syndrome
Wilms' Tumor
Wilson's Disease
Wiskott-Aldrich Syndrome
Wolf-Hirschorn Syndrome
Wolff-Parkinson-White Syndrome
Woltman Syndrome
Wyburn-Mason Syndrome
X-Linked Juvenile Retinoschisis
X-Linked Lymphoproliferative Syndrome
XYY Syndrome
Xeroderma Pigmentosum
Yaws
Yellow Fever
Yellow Nail Syndrome
Zellweger Syndrome
Zollinger-Ellison Syndrome

PRESERVATION PHOTOCOPY

NORD MEMBER ORGANIZATIONS

President:
Jess Thoene, M.D.

Executive Director:
Abbey S. Meyers

Member Organizations:
Acoustic Neuroma Association, Inc.
Alliance of Genetic Support Groups
Alpha1 Antitrypsin Deficiency National Association
American Carpal Tunnel Syndrome Association
American Narcolepsy Association
American Porphyria Foundation
American Society of Adults with Pseudo-Obstruction, Inc. (ASAP)
American SyringomyeliaAlliance Project
Aplastic Anemia Foundation of America
Association for Glycogen Storage Disease
Batten Disease Support & Research Association
Benign Essential Blepharospasm Research Foundation, Inc.
Charcot-Marie-Tooth Association
Chromosome 18 Registry and Research Society
Cornelia de Lange Syndrome Foundation, Inc.
Cystinosis Foundation, Inc.
Direct Link for the Disabled, Inc.
Dysautonomia Foundation, Inc.
Dystonia Medical Research Foundation
Dystrophic Epidermolysis Bullosa Research Assoc. (D.E.B.R.A.)
Ehlers-Danlos National Foundation
Epilepsy Foundation of America
Families of Spinal Muscular Atrophy
Fanconi Anemia Research Fund, Inc.
Foundation for Ichthyosis & Related Skin Types (F.I.R.S.T.)
Guillain-Barré Syndrome Foundation International
Hemochromatosis Research Foundation, Inc.
Hereditary Disease Foundation
Histiocytosis Association of America
Huntington's Disease Society of America, Inc.
Immune Deficiency Foundation
International Fibrodysplasia Ossificans Progressiva (FOP)
International Joseph Diseases Foundation Inc.
International Rett Syndrome Association
Interstitial Cystitis Association of America, Inc.
Lowe's Syndrome Association
Malignant Hyperthermia Association of the United States
Meniere's Network (EAR Foundation)
Myeloproliferative Disease Center
Narcolepsy Network,Inc.
National Addison's Disease Foundation
National Alopecia Areata Foundation
National Association for Sickle Cell Disease, Inc.
National Ataxia Foundation
National Chronic Fatigue Syndrome Association, Inc.
National Foundation for Ectodermal Dysplasias
National Fragile X Foundation
National Gaucher Foundation, Inc.
National Leigh's Disease Foundation
National Marfan Foundation
National Mucopolysaccharidoses Society, Inc.
National Multiple Sclerosis Society
National Neurofibromatosis Foundation
National PKU News
National Retinitis Pigmentosa Foundation, Inc.
National Sjogren's Syndrome Assoc.
National Tay-Sachs & Allied Diseases Association, Inc.
National Tuberous Sclerosis Association, Inc.
National Urea Cycle Disorders Fdtn.
National Vitiligo Foundation, Inc.
Neurofibromatosis, Inc.
Obsessive Compulsive Foundation
Osteogenesis Imperfecta Foundation
Oxalosis & Hyperoxaluria Foundation
Paget's Disease Foundation, Inc.

NORD MEMBER ORGANIZATIONS

Parents of Galctosemic Children
Parkinson's Disease Foundation, Inc.
PKR Foundation
Prader-Willi Syndrome Association
Reflex Sympathetic Dystrophy Syndrome Association
Scleroderma Federation, Inc.
Scleroderma Info Exchange, Inc.
Sjogren's Syndrome Foundation, Inc.
The A.L.S. Association
The American Brain Tumor Association
The EAR Foundation
The Myasthenia Gravis Foundation
Tourette Syndrome Association, Inc.
Trigeminal Neuralgia Association
T.S.A. of Illinois
United Leukodystrophy Foundation, Inc.
United Parkinson Foundation
Vestibular Disorders Association
Wegener's Granulamatosis Support Group
Williams Syndrome Association
Wilson's Disease Association

Associate Members:
Aicardi Syndrome Newsletter
Alabama Society for Sleep Disorders
A.L.S. Association Greater Philadelphia Chapter
American Behcet's Association, Inc.
American Self-Help Clearinghouse/ N.J.
ARC of Ohio
Association for Children with Russell-Silver Syndrome, Inc.
Center for Research in Sleep Disorders
Charcot-Marie-Tooth International
Children's Leukemia Foundation of MI
Christina Lazar Foundation for Juvenile Laryngeal Papillomatosis
Chronic Granulomatous Disease Association
Congenital Adrenal Hyperplasia Assoc., Inc. (CAHSA)
Devereux Foundation
Footsteps Institute
Foundation for Nager & Miller Syndromes
Freeman-Sheldon Parent Support Group
Help Hospitalized Children's Fund
Klippel-Trenaunay Support Group
Lethbridge Society for Rare Disorders/Canada
Lyme Borreliosis Foundation
Nation Association For Pseudoxanthoma Elasticum
National Coalition for Research in Neurological & Communicative Disorders
National Cushings Association
National Self-Help Clearinghouse (Israel)
North American Pediatric Pseudo-Obstruction Society
Organic Acidemia Association, Inc.
Parent to Parent of GA, Inc.
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Treacher-Collins Foundation
Tuberous Sclerosis Assoc. of IL
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• Associations are joining continuously. For newest listing please contact the NORD office.

11/92

NORD'S RESEARCH PROGRAM

NORD's Research Program is governed by its **Research Advisory Council (NRAC)** on the recommendations of **NORD's Medical Advisory Board (MAB).** The scientific grant program was initiated in 1988 through a request for proposals. These grants provide funding to scientists performing research on new **treatments** for rare disorders. NORD will not fund grants for basic research unless it is directly related to a new **treatment** for a rare disease or condition.

All of NORD's programs must develop and flourish simultaneously because one cannot be effective without the other. For example, NORD may fund a research study on a disease that affects only a handful of people in the United States. The scientist may require the participation of 25 people with that disease for the research project. Through NORD's **education** and **networking** programs, NORD must attempt to locate patients needed for the study and encourage them to contact the researcher. Each individual can then make a decision as to whether they wish to participate in the research project. Additionally, NORD attempts to relay information about important scientific studies to the medical community so that practicing physicians can refer their rare disease patients to the research scientist. Thus, adequate resources are required to carry out NORD's mission through all of its vital programs.

Your donation to NORD will provide help by expanding medical research, fostering education and ensuring that the voices of all people with rare disorders are heard in unison before all levels of the government, health related industries, and the scientific community.

NORD®

. . . out of the darkness, into the light . . .

NATIONAL ORGANIZATION FOR RARE DISORDERS®

NORD
100 Rt. 37, P.O. Box 8923,
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(203) 746-6518

Not so rare. . .

"Our son was misdiagnosed for more than two years. You cannot imagine the nightmare we lived through. The professionals we saw could not identify his illness. We spent huge sums of money searching for an answer. When his disorder was finally diagnosed, we learned that it was so rare, physicians knew little about it. There seems to be no treatment, and very little research giving us hope for his future."

Although this parent thought her story was unique, millions of Americans with rare disorders have suffered similar agonies. Names of their illnesses may be different, but most have experienced the indignity of searching for help in vain. Rare disorders are "back of the textbook" illnesses, unfamiliar to the general public and professionals alike. They can strike people of all ages, all races, and all ethnic backgrounds. Many are genetic; others are acquired through environmental causes; but for most, the cause is still unknown. Thus, even after a family obtains a proper diagnosis, they are too often left with more unanswerable questions.

NORD created to help

The National Organization for Rare Disorders (NORD) has been created by a group of voluntary agencies, medical researchers and individuals concerned about Orphan Diseases and Orphan Drugs. Orphan Diseases are rare, debilitating illnesses which strike small numbers of people. Orphan Drugs are therapies which alleviate symptoms of some rare diseases, but which have not been developed by the pharmaceutical industry because they are unprofitable.

Any disorder affecting fewer than 200,000 people is an "Orphan Disease" because products developed for these illnesses are considered by the pharmaceutical industry as "drugs of little commercial value." The cost of developing a drug in the U.S. today ranges between $50 million and $80 million. To provide incentives for commercial development of Orphan Drugs, Congress enacted the "Orphan Drug Act," which became a law on January 4, 1983.

It's just the beginning. . .

This legislation has substantially impacted upon the Orphan Drug problem by offering tax incentives to drug manufacturers who develop Orphan Drugs. In addition, the Act gives seven years exclusive marketing rights to developers of unpatentable therapies. A small pool of money is authorized by the legislation for grants to scientific investigators for research on new treatments for rare disorders.

Passage of the Orphan Drug Act, however, does not signify the end of the struggle for people with rare disorders; rather, it represents only the end of the beginning. Recognizing that more than 5,000 rare disorders affect more than 20 million Americans, NORD addresses their common concerns; people with Orphan Diseases do not suffer less pain and their families do not endure less agony simply because small numbers are affected by these illnesses.

NORD's objectives are:

- To encourage, promote and fund scientific research on the cause, control and ultimate cure of rare disorders.
- To educate the general public and medical profession about the existence, diagnosis and treatment of rare disorders.
- To act as a clearinghouse for information about rare disorders and to network families with similar disorders together for mutual support.
- To foster communication among rare disease voluntary agencies, government bodies, industry, scientific researchers, academic institutions and concerned individuals.
- To accumulate and disseminate information about Orphan Drugs and Devices, making known their availability to patients, physicians and other concerned parties.
- To assist in harmonizing and making more efficient the work of voluntary agencies and to offer technical assistance to newly organized support groups.
- To focus the attention of government, industry and the scientific community on the needs of people with rare disorders.

Your help is needed. . .

NORD's newsletter, *ORPHAN DISEASE UPDATE*, reports about progress in research on rare disorders; recent activities by government, health related industries and the scientific community; and relates personal accounts of courageous struggles by people with orphan diseases throughout the world.

NORD is dependent upon your support to carry on its vital activities, which have not been addressed by any other agency. Even if you do not suffer with a rare disorder, chances are a relative, friend or neighbor does. NO ONE IS IMMUNE FROM BEING STRICKEN BY AN ORPHAN DISEASE.

Your donation provides medical **research**, fostering **education** of the public and medical professionals so that people with rare diseases will be more readily recognized and helped, and expanding NORD's **"Networking"** program so that families with orphan diseases can be linked together with others having the same health condition.

Please help as your donation does make a difference in helping us overcome these little known painful, debilitating and, in some cases, life threatening disorders. Your support of NORD today may make a difference to someone you love tomorrow.

National Organization for Rare Disorders®
NORD, 100 Rt. 37, P.O. Box 8923,
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CUT HERE

Check appropriate membership category

Associate Membership

- ❑ Basic Individual or Family ($ 25)
- ❑ Friend ($ 50)
- ❑ Contributing ($100)
- ❑ Donor ($)
- ❑ I cannot afford dues at this time but please accept my contribution of ($)

❑ Check or Money Order Enclosed

Charge my ❑ MasterCard ❑ Visa (Minimum charge is $10.00, please.)

Card Number _____ Exp. Date _____

Name on Card _____

Signature _____ Today's Date _____

Daytime Phone No. _____

Please enclose membership dues and donation and return to: National Organization for Rare Disorders®
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Have you contacted NORD before? ❑ Yes ❑ No

Name _____

Address _____ My relationship to the person with the rare disorder is: _____ (i.e., patient, parent, etc.)

City _____

State _____ Zip _____ Phone (_____) _____

A copy of NORD's latest Financial Report may be obtained by writing to: New York State Dept. of State, Office of Charities Registration, Albany, NY 11231.

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National Organization for Rare Disorders, Inc.

NORD
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New Fairfield, CT 06812-1783
(203) 746-6518

1991 ANNUAL REPORT

The National Organization for Rare Disorders is dedicated to increasing the identification, control and treatment of orphan diseases through the following programs:

Services: Through NORD's **Networking** program, NORD links together people with the same orphan disorder for mutual support. In many instances, only a handful of people in the nation may have an illness and no voluntary agency or support group may yet exist for that condition. More over, NORD provides certain life saving Sandoz drugs to needy patients who have no health insurance.

NORD nurtures small support groups through its technical assistance program, ensuring that new support groups and voluntary agencies will evolve to serve the needs of each disease population.

Research: NORD encourages and supports research on understudied orphan diseases, creating hope for millions whose disorders are presently hopeless and untreatable.

Education: NORD educates the public and professionals so they may more readily understand and alleviate rare disorders. The organizvation acts as a clearinghouse for current information about more than 5,000 orphan diseases. Much of this data is maintained on a computerized **Rare Disease Database.**

National Organization for Rare Disorders, Inc.
MEMBER ORGANIZATIONS

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Jess Thoene, M.D.

Executive Director:
Abbey S. Meyers

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Wegener's Granulomatosis Support Group
Williams Syndrome Association
Wilson's Disease Association

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Devereux Foundation
Family Survival Project for Brain-Damaged Adults
Foundation for Negev & Miller Syndromes
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North American Pediatric Pseudo-Obstruction Society
Organic Acidemia Association, Inc.
Parent to Parent of GA, Inc.
Parent to Parent of New Zealand
Port Alberni Association for Children with Development Disabilities
Research Trust for Metabolic Diseases in Children
Shy-Drager Syndrome Support Group
Sickle Cell Association of the Texas Gulf Coast
Society for Progressive Supranuclear Palsy
Soto's Syndrome Support Group
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Tourette Syndrome Assoc. of MD
Tourette Syndrome Association of Nova Scotia
Tourette Syndrome Assoc. of OH
Treacher-Collins Foundation
Tuberous Sclerosis Assoc. of IL
Vaincre les Maladies Lysosomales/France

* Associations are joining continuously. For newest listing please contact the NORD office.

MESSAGE FROM THE PRESIDENT AND EXECUTIVE DIRECTOR

Nine years ago, the National Organization for Rare Disorders (NORD) was created by a handful of individuals, voluntary health agencies and medical researchers concerned about Orphan Diseases and the lack of Orphan Drugs to treat these disorders.

Perhaps one of the most frightening aspects of life with a rare disease, nine years ago, was the ignorance about these conditions. There was negligible governmental support for scientific investigations into these ailments, and the public was largely unaware of this enemy lurking among more than 20,000,000 Americans. Orphan diseases were outside the medical mainstream, were overlooked, undiagnosed, misdiagnosed and of little concern to the government.

The initial handful of individuals, voluntary health agencies and medical researchers had to rely on themselves. Because there were no assurances, they had to build NORD with equal measures of faith, trust and determination.

This unswerving commitment led to the passage of the Orphan Drug Act of 1983; a law which encouraged the pharmaceutical industry to develop drugs for orphan diseases by offering them tax incentives, government grants and exclusive marketing rights for developing drugs of little commercial value.

What began in a small corner of a donated office has grown in nine years to twenty-one staff members and an entire floor of an office building. Through the innovative programs of NORD, more people than ever before are being helped.

As NORD ends its first decade of service to the orphaned community, we know that, with your help, much has been accomplished but that there is still much left to be done.

Nine years ago there was no Rare Disease

Database, no Networking program, no drug assistance programs, little research being funded, and no place for those suffering with a Rare Disorder to turn for help.

Today, NORD's Rare Disease Database contains over 900 entries, more than 7,000 families have been networked with others having the same diagnosis, eight research projects have been funded, three drug assistance programs for needy patients have been established, and most importantly, people have a place to turn for information and help.

Thanks to the synergy, generosity and hard work of individuals and groups, volunteers and staff, organizations and corporations, NORD continues to grow and provide even more services and programs each year. The Orphaned Community is isolated no longer.

As we enter the final year of our first decade, we recognize that NORD has arrived at another beginning - the beginning of unimaginable biomedical advancements. A new scientific dawn will cast new light on orphan disease mysteries leading to treatments and cures never before possible. NORD remains steadfast in its pledge to continue encouragement of medical breakthroughs and assuring that important new treatments are made accessible to all who need them.

Jess Thoene, M.D.
President

Abbey S. Meyers
Executive Director

CORPORATE HONOR ROLL

The National Organization for Rare Disorders is grateful to its corporate friends for their continued generosity:

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SPECIAL CONTRIBUTORS

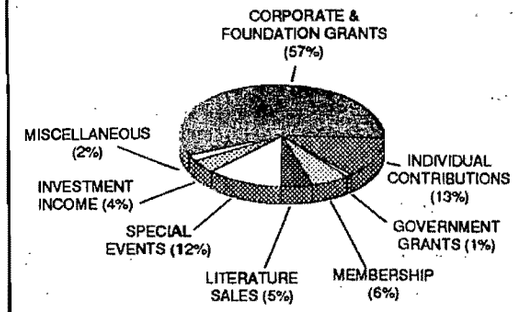
The National Organization for Rare Disorders acknowledges with thanks the following foundations, professional societies and service organizations for their generous support of NORD's programs:

American Medical Association
Catt Family Foundation
Mahadh Foundation
Raynie Foundation
Texaco Philanthropic Foundation

National Organization for Rare Disorders, Inc.
Statements of Support, Revenue, Expenses and Changes in Fund Balance for the Years Ended December 31,

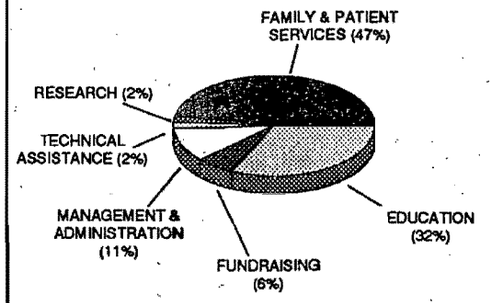
	1991	1990
Public Support and Revenue:		
Public Support:		
Contributions - individuals	$121,757	$100,443
Contributions - corporations and foundations	549,264	321,500
Special events (net of direct costs of $12,797 in 1991 and $14,496 in 1990)	117,253	146,579
Total Public Support	788,274	568,522
Revenue:		
Grants from Federal government agencies	8,000	6,000
Membership dues	58,191	50,559
Sales of materials and services to the public	50.570	36,087
Investment income	37,317	41,523
Miscellaneous	18,558	15,363
Total Revenue	172,636	149,532
Total Support and Revenue	960,910	718,054
Expenses:		
Program Services:		
Family services	414,730	344,844
Education	284,766	146,549
Technical assistance	13,213	22,756
Research	15,824	61,883
Total Program Services	728,533	576,032
Supporting Services:		
Management and general	101,776	18,305
Fund raising	56,418	61,026
Total Supporting Services	158,194	79,331
Total Expenses	886,727	655,363
Excess of public support and revenue over expenses	74,183	62,691
Fund balance, beginning of year	562,806	500,115
Fund balance, end of year	636,989	$562,806

NORD's SOURCES OF FUNDS



1991 REVENUES

WHERE NORD SPENT ITS FUNDS



1991 PROGRAM EXPENSES

Food and Drug Law Journal

The Impact of Orphan Drug Regulation on Patients and Availability

Abbey S. Meyers

Foods
Drugs
Cosmetics
Devices
Biologics
Vet. Med.

Volume 47
Number 1
1992

Official Journal of The Food and Drug Law Institute

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curate or outdated, especially because new orphan products have been, or are being developed to treat many formerly untreatable orphan diseases. This is why NORD's **Rare Disease Database** has been developed, and why we work so hard to keep it current and accurate. NORD's database has been published in book form. The **"Physician's Guide to Rare Diseases"** is written in more technical language than the actual database. It can be ordered from Dowden Publishing Company, 110 Summit Ave., Montvale N.J. 07645.

Until the **Orphan Drug Act** became law, experimental drugs were available only to patients enrolled in controlled clinical trials. When designing the law, Congress recognized that people with rare disorders often do not reside near large medical institutions where they could qualify for clinical trials. The "Treatment IND" allows treating physicians to obtain investigational orphan drugs for patients who are not participating in controlled studies. The physician must maintain adequate FDA reporting procedures during this period. The "Treatment IND" differs somewhat from the "Compassionate IND", and the patient may have to purchase the drug from the manufacturer rather than being provided the therapy at no cost. This new procedure now makes it possible for you to provide care locally to your rare disease patients if an approved or experimental orphan drug is available for use.

NORD can provide you with information about orphan drugs, their manufacturers and the diseases for which they are designated. NORD also keeps track of Orphan Product research grants (awarded annually by the FDA) including the location of current clinical trials. NORD also funds small clinical research grants for the study of new treatments for rare disorders. These grants are funded after a rigorous peer review process determines the most meritorious proposals.

The Rare Disease Database was initiated by, and continues to be expanded under a grant from the Generic Pharmaceutical Industry Association (GPIA). From time to time, additional funding for this program has been provided by the March of Dimes, the Pharmaceutical Manufacturers Association (PMA), Revco Drug Stores, and others.

NORD IS HERE TO HELP!

Refer your rare disease patients to NORD if they are searching for a support group or if they wish to be put in contact with others who have the same diagnosis. We are here to help families, patients, professionals and the public. NORD's programs of education, service and research are dedicated to the prevention, control and cure of orphan diseases.

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I am interested in more information about NORD:

Name: _____

Address: _____

_____
(City) (State) (Zip)

Phone: (____) _____

Please send NORD Literature Order Form ❑
I would like information on

_____
(name of disorder)

I am a Clinician ❑ Researcher ❑

❑ Specialist

_____
(specialty)

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National Organization for Rare Disorders

. . . out of the darkness, into the light . . .

PHYSICIAN'S GUIDE TO NORD SERVICES

NORD
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Dedicated to Helping People with Orphan Diseases

PHYSICIAN'S GUIDE TO NORD SERVICES

The National Organization for Rare Disorders (NORD) is a non-profit voluntary health agency dedicated to the identification, control and improved treatment of rare orphan diseases. Under federal law (the **Orphan Drug Act of 1983**) an orphan disease is one that afflicts fewer than 200,000 Americans. There are more than 5,000 of these disorders touching the lives of an estimated 20 million Americans. The majority of orphan diseases are genetic conditions.

WHAT IS AN ORPHAN DRUG?

NORD is the consumer oriented organization that was primarily responsible for passage of the federal **Orphan Drug Act**. An orphan drug is a pharmaceutical product (including biologics) that has little commercial value. In the past, it was difficult to locate commercial sponsors for these therapies because the market to which they could be sold was too limited, or because the products were not patentable.

The **Orphan Drug Act of 1983** provides financial incentives that have enticed pharmaceutical manufacturers into developing new orphan drugs. These incentives include exclusive marketing rights, tax credits for clinical research, and other benefits. The law has been very successful. Today, many important new therapies have been, or are being developed in response to this model legislation. The great majority of orphan drugs are designated for diseases that afflict fewer than 40,000 Americans.

HOW CAN YOU OBTAIN CURRENT INFORMATION ABOUT RARE DISORDERS AND THEIR TREATMENT?

NORD operates the **Rare Disease Database** (RDB) which is accessible to the public and health professionals through personal computers with a modem via CompuServe, the nation's largest electronic information system. Subscriptions to CompuServe can be obtained through local computer retail stores or by contacting CompuServe at: 5000 Arlington Centre Blvd., Columbus, Ohio 43220.

RDB entries are written in layman's language and reviewed by physicians before they are put on-line. Each entry is designed not to frighten patients, and to refer them to other services such as voluntary health agencies and the National Institutes of Health for more in-depth information. Each RDB entry contains a general description of the disorder, synonyms, symptomotology, etiology, affected population, related disorders, standard therapies, investigational therapies (when applicable), resources to contact for more information and references. Information about orphan drugs, orphan devices or other experimental products or procedures being used in clinical trials is contained in the investigational therapies section of each entry, when applicable. Often, the names of chief investigators, a clinic or manufacturer are included with a statement advising patients that their doctor can contact these resources.

People who do not subscribe to CompuServe can order reprints of RDB disease entries from NORD's **Literature Order Form** which can be mailed to them upon request.

HOW CAN NORD HELP YOUR PATIENTS?

NORD refers people with rare disorders to support groups when they exist. If there is no support group for a specific disease, patients may choose to enroll in NORD's **Networking** program. They must sign a written permission form allowing NORD to release their name to others with the same diagnosis. By linking people with the same disorder together, NORD encourages formation of new support groups for even the rarest disorders.

NORD's Networking program has been developed with a knowledge and understanding that people with rare disabilities can profit from the self-help peer support model, but there are usually not enough people with a specific orphan disease in one geographic area. Thus, the Networking program evolved from an informal pen-pal type program into an international self-help effort. Families have developed unique communication efforts such as exchanging video tapes, arranging an annual meeting during summer vacation periods, developing newsletters, etc., and many have taken it upon themselves to link together their health professionals, rehabilitation counselors, special education teachers, siblings, etc.

Families will also benefit from NORD's newsletter, **Orphan Disease Update**, which keeps readers abreast of new scientific developments, newly designated Orphan Drugs, and provides insight into coping with illnesses that do not ordinarily elicit understanding and compassion from the public. NORD's Patient Services department handles a variety of family concerns ranging from access to health insurance and other reimbursement programs to advice about special education, developmental disabilities services and other government run programs.

NORD is keenly aware that there is often a long delay between onset of an orphan disease and proper diagnosis. However, we are not a clinic and we cannot diagnose patients. NORD's services are aimed at people who have been diagosed with a specific rare disorder.

HOW DOES NORD HELP THE PHYSICIAN?

NORD believes that the first step in conquering rare disorders must begin by familiarizing medical professionals with the diagnosis and current treatment of these little known illnesses. Much of the medical literature about orphan diseases is inac-

ORPHAN DISEASE UPDATE©

VOL. X Ed. 3 National Organization for Rare Disorders SPRING 1993

NORD • P. O. Box 8923 • New Fairfield, CT 06812 • Phone: (203) 746-6518

...out of the darkness into the light...

ISSN 0887-0306

NORD is a participant in the Combined Federal Campaign **#0551** in the CFC Brochure.

THE ORPHAN DRUG ACT: A DECADE OF PROGRESS

Editors Note: *The following article was initially published in the March 1988 issue of the Journal of the American Medical Writers Association. NORD's founder and Executive Director, Abbey Meyers, revised and updated the article this year in recognition of NORD's 10th Anniversary and the first decade of the Orphan Drug Act.*

We share this article with readers so they can learn how and why NORD was created, and to relate how a small group of dedicated individuals and support groups were able to change the fate of millions of people throughout the world.

The Beginning

The story of the struggle for the Orphan Drug Act is testimony to all that is right and much that is wrong in America. It is encouraging, it is distasteful; it is a story of greed and of heroism. It is a Hollywood fantasy, timed and directed by the hand of God. The orphan drug story clearly illustrates that democracy works, that capitalism and social justice can enhance each other, and that the most powerful government in the world can be responsive to the needs of the few as well as the many.

The ability to move major conglomerates and impregnable government power structures lies in the hands of ordinary citizens who will never fully understand or use their freedoms more effectively than they did to solve the orphan drug dilemma. But the true power lay in the hands of the American media; if the media had not brought the story to the public's attention, the war would not have been won, and the battlefield would still be strewn with the casualties of orphan diseases. The media can also reveal the inequities and flaws that have appeared during this long decade of progress, which threaten the very foundation upon which the Orphan Drug Act is built.

For many of us, our initial involvement began in our homes where we launched separate battles against devastating diseases affecting loved ones. From these personal beginnings grew nonprofit voluntary health agencies, each devoted to the identification, treatment and cure of a single disease.

My personal involvement began when my oldest son was diagnosed with Tourette Syndrome, a rare neurological movement disorder. After three years of debilitating side effects from a drug considered the treatment of choice for Tourette, we decided to try an experimental drug. It offered major therapeutic improvement, but after a few months we were unable to obtain it. The drug (pimozide) had little commercial value in the U.S., so the manufacturer decided not to seek FDA approval. As a result, distribution of pimozide as an investigational drug was halted. The implication of this decision on my son, and others with Tourette who had benefited from the drug, was immeasurable. For my 11-year-old son, it meant he would no longer be able to use his hands to dress himself, write with a pen or eat with a fork.

The problem of orphan drugs existed in America since the beginning of the century but did not become a social policy problem until the 1960's when passage of the Kefauver-Harris Amendments to the Food, Drug and Cosmetics Act significantly increased the amount of money needed to develop a drug in the U.S. Although drugs previously had to be proven **safe** before they could reach the American market, the amended law required that drugs henceforth had to be proven **effective.** As the price of pharmaceutical research and development increased, drugs for rare diseases became known as "drugs of little commercial value" or "orphan drugs" because commercial sponsors were unwilling to adopt them.

I am an ordinary person. I had not been politically active because it had never been apparent that political decisions made in Washington, or economic decisions made in corporate board rooms, would impact on me personally or my family. When we could no longer obtain pimozide, we had to evaluate realistic choices. We could have continued to obtain the drug by purchasing it outside the U.S. and smuggling it back into the country; or we could choose to fight the inequitable system that had made this tragedy possible. I knew it would be unlikely that corporate or government bureaucracies would change their policies for one child with Tourette Syndrome, so I started writing letters to voluntary health agencies concerned about different orphan diseases in order to find out if their disease had similarly been affected by the orphan drug problem. I learned about penacillamine for Wilson's Disease, sodium valproate for subtypes of epilepsy and L-5HTP for myoclonus, among other orphan drugs.

Soon a small nucleus of consumer groups emerged and united around the orphan drug dilemma. Some had been directly affected because they could not get orphan therapies manufactured or distributed for their disease. Others, such as Huntington's disease, were still untreatable, but leaders of these groups quickly understood that the problem had to be solved; for in the absence of a legislative solution, when a cure was discovered it would never be manufactured.

A young woman with myoclonus had interested her congresswoman, Rep. Elizabeth Holtzman, in the orphan drug problem. Dr. Melvin Van Woert of the Mt. Sinai School of Medicine in New York had been manufacturing L-5 hydroxytryptophan (L-5HTP) for many years in his laboratory as a treatment for myoclonus. No manufacturer was interested in this shelf chemical because it was unpatentable, and there are only 2,000 patients with this neurological disorder in the U.S.

Rep. Holtzman had personally tried to entice a pharmaceutical firm into developing L-5HTP, but she failed. So she introduced the first orphan drug act in the Congress during 1980. This legislation would have set up a revolving pool of money to award grants and contracts for the development of orphan drugs. Because the government would subsidize the research and development phases, the bill required manufacturers to return any profits they would make to the federal government. These profits would be deposited in an orphan drug research fund so the money could be loaned again to other orphan drug developers.

The legislation hardly stirred a ripple in the great halls of Congress. No hearings were held, no interest was shown. On the other hand, the bill did have an effect on the pharmaceutical industry . . . the companies disliked it intensely. The profit motive is the major incentive for pharmaceutical development; to disregard that incentive was not logical.

Hollywood Style Miracle

We waited for the industry to negotiate for changes in the legislation. When nothing happened we sought the news media and launched a campaign to bring orphan drugs to the public's attention. By chance, and perhaps by God's hand, a miracle happened - Hollywood style - when Adam Seligman's pimozide was confiscated by the U.S. Customs Service. He had asked a friend to bring the drug from Canada. Mr. Seligman's congressman was Rep. Henry Waxman of Los Angeles. When Mr. Waxman learned that his constituent had been affected by the orphan drug problem, he held congressional hearings to investigate the matter. These hearings occurred on June 26, 1980. Consumers, including myself and Mr. Seligman, testified about the impact of orphan drugs on our families. The testimony was intensely emotional and frightening. The next day Jack Klugman's brother read a newspaper story in the **Los Angeles Times** about the hearings. He was a producer on the popular TV show, **Quincy.**

The Pharmaceutical Manufacturers Association (PMA), although invited to testify at the congressional hearing, had decided not to do so. In time it became clear that many drug companies would not acknowledge there was an orphan drug problem, and during coming months the PMA declared there were only a few orphan drugs that did not constitute a significant health problem. Within weeks of our congressional testimony the manufacturer of pimozide announced they would indeed go ahead with the development of the drug. It appeared there was no further need to be concerned. However, L-5HTP for myclonus and several other important drugs continued to be without a commercial sponsor.

On the other hand, the Klugman's had also made a policy decision. There would be a television episode of **Quincy** devoted to Tourette Syndrome and orphan drugs, and it would be broadcast on nationwide television during March 1981. It was a dramatic episode that moved the public out of its complacency. Mr. Klugman received thousands of letters from ordinary people asking how they could help, and those letters were forwarded to the Tourette Syndrome Association. From those initial letters a mailing list was developed so that a grass roots legislative network was put into place. In the aftermath of this publicly acclaimed TV show, Mr. Waxman introduced a completely new Orphan Drug Act, and he scheduled a second congressional hearing. He asked Jack Klugman to appear at the hearing, which assured a vast amount of publicity.

The pharmaceutical industry was very angry. They felt the **Quincy** episode was an overly dramatic and inaccurate portrayal of the orphan drug problem; it was a black eye on the industry, and some corporate leaders felt compelled to defend their opinions. We were delighted to learn they would testify at Mr. Waxman's hearings. We wanted to know what the industry wanted; how could we design incentives that would heighten their interest in orphan drugs? We were not prepared to hear them say that they didn't want any laws passed because they could handle the problem "voluntarily."

The Pharmaceutical Manufacturers Association announced it would develop a **Commission on Drugs for Rare Diseases.** A similar message was echoed by the government when the FDA announced they would "voluntarily" create an Office for Orphan Products Development, and the Department of Health and Human Services announced an Orphan Products Board to coordinate all federal orphan disease programs. The message seemed clear; we consumers could just go back home and be quiet, obedient citizens because the problem would be solved.

But the problems were not solved. The vast amount of publicity had reached people with rare diseases and their physicians all over the nation. We heard about more and more orphan drugs - cysteamine for 100 children dying of the fatal hereditary kidney disease, cystinosis - trien for Wilson's disease - gammahydroxybuterate for narcolepsy - one by one the orphan drugs emerged and we knew we were seeing just the tip of the iceberg. To complicate the matter, some of the industry witnesses who had testified publicly that there was no orphan drug problem - or that the problem was minimal and that we consumers were blowing it out of proportion - contacted me personally and unofficially. They urged us to keep fighting for an Orphan Drug Act. Some told us privately how serious the problem really was, and they hoped for the sake of humanity that it would be solved. It was obvious that the free enterprise system was not adequately meeting the needs of society. Because the problem was economic in nature, it needed economic solutions. These discussions reinforced our determination and gave us strength to persist. These corporate leaders felt the industry needed a conscience, and we consumers were that conscience.

Compromise Sought

During the months after that second congressional hearing it became obvious that the legislation would not be passed into law if we could not reach a compromise with the pharmaceutical industry. The companies could afford to block our advances on Capitol Hill through professional lobbyists. It was another television show that proved to be the catalyst; when **60 Minutes** began to film a segment about orphan drugs, it spurred the industry into meetings where a compromise was reached and the legislation that emerged was, I believe, the best possible solution to the orphan drug dilemma.

Like all industries, pharmaceutical corporations wanted the opportunity to make a profit if that was possible, and at the very least they wanted minimal risk of losing their investment when they committed manpower and resources to the development of an orphan drug. Addressing these problems, the Orphan Drug Act would provide the following incentives:

1. A tax credit of $.73 on every dollar spent to support clinical trials. The Tax Reform At of 1986 reduced this credit to $.50, which is still a significant incentive.

2. Seven years exclusive marketing rights during which no other company can be licensed to sell the same drug.

3. An easier approval process requiring FDA's initial and ongoing written advice to minimize the possibility that financial and human resources would be wasted.

4. An appropriation of federal funds to support grants and contracts to academic scientists and small corporations to develop orphan drugs.

5. Originally, the act defined an "orphan disease" as a condition which is "rare in the United States." An "orphan drug" was a treatment for which the cost of development outweighed its potential for profit. In 1985 this was changed to say that an orphan disease is a disorder that affects fewer than 200,000 Americans. However, drugs for more prevalent conditions can be designated as "orphan drugs" if the manufacturer can prove the drug will be unprofitable in the absence of incentives.

These incentives were chosen because we knew we needed the talents and expertise of the pharmaceutical industry to achieve our goal. Other alternatives had been considered such as asking the government to become a pharmaceutical manufacturer. But obviously, government could not possibly perform comparably to private industry. Indeed our experience indicated that once a government stepped into an area that is the rightful territory of private enterprise, it would be unlikely that many new products would emerge.

Needless to say, once these details were resolved and we had developed legislation we could all agree upon, the new proposed Orphan Drug Act moved ahead quickly and passed the House of Representatives unanimously. Subsequently, **60 Minutes** decided not to broadcast the orphan drug segment because they felt it wasn't controversial enough! The act then went to the Senate, where it languished for months until Jack Klugman decided to do a second **Quincy** episode on network TV about a U.S. Senator who was blocking the Orphan Drug Act. Miraculously, on the last day of filming this episode, the bill was called to the floor of the Senate and also passed unanimously. Then the Congress recessed for the year.

(continued on next page)

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Reprint Series
8 March 1991, Volume 251, pp. 1158–1159

SCIENCE

Curing the Orphan Drug Act

JESS G. THOENE

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NETWORKING NOTICE

Due to the large volume of requests for NORD's "Networking" services, we can no longer provide this service for free to people who are not members of NORD. Networking services are offered to individuals and families whose annual dues ($25.00) make them eligible for this and other membership privileges.

National Organization for Rare Disorders, Inc.

NORD • P.O. Box 8923 • New Fairfield, CT 06812 • (203) 746-6518

... out of the darkness, into the light ...

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Sjogren's Syndrome Foundation, Inc.
Tourette Syndrome Association, Inc.
United Leukodystrophy Foundation, Inc.
United Parkinson Foundation
Vestibular Disorders Association
Williams Syndrome Association
Wilson's Disease Association

WRITTEN PERMISSION FOR NORD'S NETWORKING PROGRAM

The National Organization for Rare Disorders (NORD) keeps names and addresses confidential unless a voluntary agency exists for the specific disorder, or a person (or parent) provides written permission for NORD to send their name to other people (or families) with the same disorder. The NORD Networking Program is meant to encourage formation of mutual self-help support groups concerned about a single rare disorder.

If you wish to be put in contact with other people (or families) concerned with the disorder you inquired about, please complete the form below and mail it to the address above. NORD will not add the names of third parties (i.e., teachers, social workers, etc.) to the NORD Networking Program. Therefore, the patient or his immediate family must sign this form in order to participate in this program.

Please Note: Do **not** sign this form if you do **not** intend to correspond with people who have your specific disability. If you do not answer their letters, you may unnecessarily disappoint them.

Name: _____

Address: _____

City: _____

State: _____ **Zip:** _____

Name of Rare Disorder: _____

_____

CIRCLE YOUR RELATIONSHIP TO THE PERSON WITH THE RARE DISORDER

Patient **Parent** **Spouse** **Relative** **Friend**

Other: _____
(specify other relationship)

Signature: _____
(required)

Please Note: *You must provide a name for your disease or condition. NORD cannot diagnose patients nor can we match names of patients according to symptoms.*

Note: *NORD cannot network without a signature on this form.*

Associate Members

Alabama Society for Sleep Disorders
Alliance of Genetic Support Groups
American Behcet's Association, Inc.
American Pediatric Gastroesophageal Reflux Association, Inc.
Angelman Research Group
Association for Children with Russell-Silver Syndrome, Inc.
Brain Impaired Adult Resource Center
Center for Research in Sleep Disorders
Charcot-Marie-Tooth International
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Friedreich's Group in America/CA
Gluten Intolerance Group of North America
Lethbridge Society for Rare Disorders/Canada
Lyme Borreliosis Foundation
Myeloproliferative Disease Research Center
National Association for Pseudoxanthoma Elasticum
National Coalition for Research in Neurological & Communicative Disorders
National Cushings Association
National Self-Help Clearinghouse/ Israel
National Sjogren's Syndrome Association
National Spasmodic Torticollis Association
North American Pediatric Pseudo-Obstruction Society
Oxalosis & Hyperoxaluria Foundation
Parent To Parent of GA, Inc.
Parent To Parent of New Zealand
Research Trust for Metabolic Diseases in Children
Self-Help Clearinghouse of N.J.
Sickle Cell Association of the Texas Gulf Coast
Soto's Syndrome Support Group
Sturge-Weber Foundation
Tourette Syndrome Assoc. of MD
Tourette Syndrome Assoc. of Nova Scotia
Tourette Syndrome Assoc. of OH
Tourette Syndrome Assoc.-PA Chapter
Tuberous Sclerosis Assoc. of IL

*Associations are joining continuously. For newest listing contact the NORD office.

Dedicated to Helping People with Orphan Diseases

NORD LITERATURE ORDER FORM

RARE DISEASE DATABASE ARTICLES

MAIL TO: NORD Literature — 100 Rt. 37, PO Box 8923, New Fairfield, CT 06812-1783

Reprints of disease articles from NORD's **Rare Disease Database** are available for $3.25 per copy, which includes postage and handling. The disorders are written in understandable language for patients and families. Each entry lists the disease name, synonyms, a general description of the disorder, symptoms, causes, affected population, standard treatments, investigational treatments (when applicable) and a list of resources that can be contacted for further information about the illness. Some of the listed disorders are not rare, but are included because **NORD** receives substantial inquiries about them.

Please use the order form below by **circling** the proper entry number and inserting the **quantity** of copies you wish to receive. Please **print** your name and address on this form and enclose the form with a check or money order for the total amount due (U.S. funds please). Expect delivery within 4 to 6 weeks.

For people with personal computers who wish to access the database directly, you can reach the **NORD Services** section of **CompuServe** by typing "GO NORD" at any prompt on the CompuServe Information System.

Name: _____

Address: _____

City, State, Zip: _____

Telephone: ( ) _____ **Card Exp. Date** | | | | |

Charge my: ☐ MC ☐ VISA

Minimum Charge is $5.00

_____

Cardholder's Signature

Cost per print	$ 3.25
Number of prints	X _____
Subtotal	$ _____
Add'l Tax Deductible Donation	_____
GRAND TOTAL	$ _____

Today's Date _____

Entry	Disease Name	Qty	Entry	Disease Name	Qty	Entry	Disease Name	Qty
641	11q Syndrome		473	Alagille Syndrome		204	Antley-Bixler Syndrome	
643	13q Syndrome		42	Albinism		254	Apert Syndrome	
639	18p- Syndrome		56	Alexander's Disease		901	Aplasia Cutis Congenita	
793	4q Syndrome		23	Alkaptonuria		190	Apnea, Infantile	
522	5-Oxoprolinuria		443	Alopecia Areata		68	Apnea, Sleep	
483	ACTH Deficiency		610	Alpers Disease		766	Apraxia	
78	AIDS (Acquired Immune Deficiency Syndrome)		53	Alpha-1-Antitrypsin Deficiency		444	Arachnoiditis	
519	AIDS Dysmorphic Syndrome		592	Alport Syndrome		312	Arginase Deficiency	
835	APECED Syndrome		431	Alveolitis, Extrinsic Allergic		311	Arginino Succinic Aciduria	
615	Aarskog Syndrome		432	Alveolitis, Fibrosing		85	Arnold-Chiari Syndrome	
106	Aase Syndrome		29	Alzheimer's Disease		221	Arteriovenous Malformation	
114	Acanthocheilonemiasis		908	Ameiblastoma		201	Arteritis, Giant Cell	
445	Acanthocytosis		501	Amelogenesis Imperfecta		86	Arteritis, Takayasu	
115	Acanthosis Nigricans		70	Amenorrhea, Primary		263	Arthritis, Infectious	
118	Achalasia		711	Amniotic Bands		899	Arthritis, Juvenile	
107	Achard-Thiers Syndrome		22	Amyloidosis		247	Arthritis, Psoriatic	
876	Achondrogenesis		57	Amyotrophic Lateral Sclerosis (Lou Gehrig's Disease)		211	Arthrogryposis Multiplex Congenita	
80	Achondroplasia		707	Anaphylaxis		851	Asherman's Syndrome	
504	Acidemia, Isovaleric		394	Andersen Disease		918	Aspartyglycosaminuria	
427	Acidemia, Methylmalonic		83	Anemia, Aplastic		680	Asperger's Syndrome	
500	Acidemia, Propionic		450	Anemia, Blackfan-Diamond		737	Aspergillosis	
818	Acne		723	Anemia, Cold Antibody Hemolytic		775	Astrocytoma, Benign	
527	Acne Rosacea		84	Anemia, Fanconi's		277	Astrocytoma, Malignant	
45	Acoustic Neuroma		771	Anemia, Hemolytic, Acquired Autoimmune		7	Ataxia, Friedreich's	
936	Acrocallosal Syndrome, Schinzel Type		770	Anemia, Hemolytic, Warm Antibody		674	Ataxia, Hereditary	
511	Acrodermatitis Enteropathica		82	Anemia, Hereditary Non-Spherocytic Hemolytic		403	Ataxia, Marie's	
613	Acrodysostosis		81	Anemia, Hereditary Spherocytic Hemolytic		406	Ataxia Telangiectasia	
51	Acromegaly		423	Anemia, Megaloblastic		138	Atrial Septal Defects	
584	Adams-Oliver Syndrome		79	Anemia, Pernicious		593	Attention Deficit Hyperactivity Disorder	
46	Addison's Disease		351	Anemia, Sideroblastic		5	Autism	
825	Adie Syndrome		596	Anencephaly				
97	Adrenal Hyperplasia, Congenital		411	Angelman Syndrome		120	Babesiosis	
43	Adrenoleukodystrophy		98	Angioedema, Hereditary		121	Balantidiasis	
940	Afibrinogenemia, Congenital		524	Aniridia		909	Baller-Gerold Syndrome	
73	Agammaglobulinemias, Primary		143	Ankylosing Spondylitis		122	Balo Disease	
355	Agenesis of Corpus Callosum		510	Anodontia		722	Banti's Syndrome	
209	Agranulocytosis, Acquired		187	Anorexia Nervosa		173	Barrett Syndrome	
155	Ahumada-del Castillo Syndrome		672	Antisocial Personality Disorder		123	Bartonellosis	
49	Aicardi Syndrome		99	Antithrombin III Deficiency, Congenital		589	Bartter's Syndrome	

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THE ETHICAL, LEGAL, AND SOCIAL IMPLICATIONS OF PREDICTIVE GENETIC TESTING FOR HEALTH INSURANCE

POLICY ANALYSIS AND RECOMMENDATIONS

Report of the Human Genome Insurance Project,
University of Florida College of Medicine

Funded by the
National Center for Human Genome Research,
National Institutes of Health

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EXECUTIVE SUMMARY

Human Genome Insurance Project Report on Health Insurance
University of Florida College of Medicine
April 1993

General Findings:

Predictive genetic tests have already been derived from basic research funded by the Human Genome Initiative. New tests and commercial applications will proliferate during the next decade. These tests will enable people to learn with a degree of specificity never before possible about their risk of developing rare genetic conditions as well as common diseases like cancer and diabetes. In some instances there will be medical or behavioral interventions available to prevent the diseases or ameliorate their course. In others, the information will have less therapeutic value. Commercial enterprises that market these tests will seek to encourage their use as part of routine medical practice. Depending on health reforms, different economic incentives may exist for their use as well. For example, under fee-for-service payment there may be an economic incentive to encourage testing as a profit center. Under a capitated system, economic incentives to pursue testing seem likely wherever cost-effective prevention is possible through medical care or behavior changes.

Information produced from genetic testing has the potential to produce serious harms including denial or prohibitive pricing of life insurance, loss or limitation of employment opportunities, inadequate informed consent for testing, and breaches of confidentiality of patient information. Emerging genetic tests, combined with ongoing technological developments in information management, have the potential to alter profoundly the way that medical information about individuals is acquired and maintained. Under America's current health care system, dominated by a segmented marketplace, indemnity insurance, and fee-for-service entrepreneurial practice, there is a great danger that people will lose their health insurance and be denied access to health services as a result of information acquired through genetic testing.

Community rating through health purchasing cooperatives or community health alliances, which is a key characteristic of a system of managed competition, may ameliorate the problems of loss of insurance and limitation of access. However, the competing prepaid health plans envisioned in managed competition will have an economic incentive to control their patient-care costs through early detection, preventive medical care and various degrees of persuasion or coercion to eliminate risk behaviors. Prepaid plans may find it profitable to encourage the widespread use of predictive tests with little regard for the patient's desire to know about genetic risks. Test results might be used to encourage behavioral changes, or even to deny benefits to persons continuing behaviors that pose excessive risks given their genetic predispositions. Without careful measures to maintain confidentiality and other safeguards, information from genetic tests may affect individuals in their attempts to buy life insurance or obtain employment. Informed consent, as the doctrine is presently understood, deals primarily with disclosing potential *medical* risks, which can be expected to be very low for most genetic tests. However, genetic test information raises issues of *non-medical* risks and requires that informed consent be broadened to account for these implications in disclosing risk.

Our full report presents 67 *specific* policy recommendations to deal with problems that may occur under various health care delivery and financing schemes. In this Executive Summary we present only

the general recommendations and recommendations for addressing problems generated under the system of managed competition currently viewed as the most likely scenario for national health care reform. These recommendations should be considered in their entirety, i.e., the recommendations for managed competition should be viewed in the context of the general recommendations. Our proposals are the result of a careful balancing of disparate concerns in an attempt to reach a solution that is both comprehensive and fair to competing interests. To separate these recommendations into discrete parts and modify them according to particular interests would, in our view, undermine their usefulness as responsible social policy. Changing circumstances such as technological developments or restructuring of the health care system may compel specific modifications. We are prepared to accommodate such developments when necessary.

General Policy Recommendations

The recommendations in this general category are applicable to all types of health care delivery and financing systems. The principal problems addressed by these general recommendations include: (1) ensuring adequate knowledge regarding medical and non-medical aspects of genetic tests; (2) protecting confidentiality of genetic information; (3) ensuring adequacy of informed consent for testing; and (4) ensuring access to a reasonable level of health care for all U.S. citizens.

- **Recommendation 1: Education of the Public and Insurers**

  The general public, insurers, and self-insured employers should be educated about the nature of genetic tests and the limitations and dangers of genetic labeling in order to avoid unfair and inappropriate use of, and possible stigmatization by, genetic information. Additional mechanisms should be developed to educate the employees of insurers, such as underwriters and medical directors, concerning the predictive values of particular genetic tests.

- **Recommendation 2: Education of Physicians**

  All physicians who engage in genetic testing of patients should be knowledgeable regarding the risks and benefits of genetic testing, including the employment and insurance implications. Medical school curricula and residency training programs should include information about both the medical and non-medical implications of genetic testing. American Medical Association specialty colleges should actively promote education of physicians regarding interpretation of genetic tests and their non-medical consequences, including employment and insurance implications.

- **Recommendation 3: Scientific Validity of Genetic Information**

  Insurers should have access only to results of genetic tests that have been approved for general use by the American College of Medical Genetics. The American College of Medical Genetics should develop formal procedures for evaluating and approving genetic tests. These procedures should include the Laboratory Practices Committee of the College, as well as other committees that the College determines appropriate.

- **Recommendation 4: Required Testing Prohibited**

  Insurers, employers, and other third party payers including governments in their capacity as health care payers, should be legally prohibited from requiring applicants for, or enrolles in, health insurance plans to undergo any genetic test.

- **Recommendation 5: Informed Consent**

  Informed consent should never be viewed as a process of obtaining signatures on forms. Appropriate informed consent consists of personal discussions between health care providers and patients about the relative risks and benefits of recommended treatments or tests and the medical alternatives, as well as the potential non-medical implications. Individuals must be asked for their informed consent prior to any genetic testing. The information provided to individuals during the consent process must include the impact of the possible test results on their insurability and employability and implications for analogous impacts on relatives and progeny. The applicable standard for disclosure to patients of information for informed consent purposes should be the reasonable person standard. Individuals should have a legally-authorized right to refuse genetic testing for any reason, after appropriate disclosure, without compromising access to health care. All individuals approached for genetic testing must be clearly notified of their right to refuse. Consent to genetic testing should never be presumed by legal, institutional, or other criteria.

- **Recommendation 6: Right of Confidentiality**

  Each individual has a right to keep his or her genetic information confidential.

- **Recommendation 7: Storage of Biological Samples**

  The storage of genetic samples or materials requires specific informed consent of the person donating the sample(s). New genetic tests on existing samples from identified individuals should not be permitted without a new, additional informed consent from that individual. The standard for informed consent should be no lower than was applicable when the specimen initially was obtained. Biological materials from which genetic information can be derived should only be stored by governmentally-licensed facilities engaged in clinical services or research for scientific purposes. Insurers should be legally prohibited from creating or maintaining storage banks of biological materials. Biological materials from which genetic information can be derived (e.g., blood samples or tissue samples) should be stored in ways that protect them from unauthorized access.

- **Recommendation 8: Genetic Counseling**

  Genetic counseling, both before and after testing, should be required before permitting any person to undergo any genetic test. All genetic counseling should meet or exceed standards set by the American College of Medical Genetics. Topics covered during counseling sessions should include information about the nature of the test, the type of information that will be derived, the significance of positive or negative test results, and ways of using test results. All genetic counseling must include disclosure of non-medical implications of test results, including potential adverse effects on insurance and employment.

- **Recommendation 9: Universal Access to Health Care**

  Any health care delivery and financing system should provide for universal access to health care for all U.S. citizens. Community rating, combined with legal restrictions on exclusions and waivers of coverage, should be mandatory for all health insurance coverage, whether provided by commercial insurers or governments. Communities should be not be narrowly

defined for rating purposes. Definitions of communities should be sufficiently broad, and communities large enough, to allow cross-subsidization of demographic and/or epidemiologic variation that may have a geographic and/or causal relationship to health care costs. Applicable demographic and epidemiologic variation includes: 1) health status based on genetic factors, 2) age, 3) gender, 4) racial or ethnic status, and 5) religious beliefs.

- **Recommendation 10: Public Insurance Guidelines**

  Public insurance should neither require genetic screening tests, deny access to useful programs and services, nor raise standards of access to health care on the basis of genetic information.

- **Recommendation 11: Medical Record-Keeping**

  Medical record-keeping systems should be designed with, or modified to include, special safeguards to protect confidentiality of genetic and other information in view of the coordinated computerized systems existing within contemporary health care systems. All health data systems maintained by commercial insurers and re-insurers, self-insured groups, governments, and other third-party payers, should have in place a reliable method for challenging, reviewing, and correcting or removing inaccurate genetic information. All such health data systems should specifically include a means to delete genetic information, whether accurate or inaccurate, that was obtained without appropriate informed consent or in other improper ways. One potential model for some of these procedures is the procedures used by the Medical Information Bureau. These procedures must include an appeals process that is independent of the organization handling the challenged information. All entities that maintain health data systems should be required to make it clearly known to all individuals whose records they keep that a record-correction and appeals process is available.

Managed Competition Policy Recommendations

While the Clinton Administration's health care reform proposal has not been completely determined as we write this Report, it appears likely that this proposal will adopt a managed competition approach. Under managed competition, two types of highly regulated entities bargain to provide health care. The first is a Health Insurance Purchasing Cooperative (HIPC), which essentially is a buying cooperative composed of government programs, large and small employers, and individuals. The second is an Accountable Health partnership (AHP) which may be an HMO, a fee-for-service plan, or other amalgamation of insurers and providers. Universal access to "basic benefits," delivered by competing AHPs, will be guaranteed by government regulation. Individuals can also purchase, from taxable income, broader coverage by selecting plans with a higher level of benefits from those offered by the various competing AHPs. Determinations of what constitutes "basic benefits" and methods for delineating membership in HIPCs are as yet poorly defined in most discussions.

Proposals for managed competition must address what counts as a "basic" plan before it will be possible to determine the extent of cost-shifting or adverse selection likely to occur. If the basic benefit level is "comprehensive care" offered by plans competing on the basis of efficient delivery, then selection bias based on medical risk seems likely to be minimal. If, however, "basic" benefits include only a minimal package that requires additional important benefits (e.g., surgery not otherwise covered, long term care, prescription drugs, etc.) to be purchased at additional premiums, it appears

likely that some persons with strong evidence of future medical need will elect to pay the higher premiums to purchase the additional benefits. Given the principle that plans will not be able to screen or select applicants (who enroll and/or switch plans through a sponsor), it seems likely that a managed competition approach based on a "minimal" rather than "comprehensive" set of services may lead to renewed concerns about adverse selection and cost spirals (or significant cost shifting if surcharges and subsidies are applied) as persons with current or expected high medical costs elect "high option" plans.

Under either definition of "basic" benefits, it is very likely that managed competition will produce significant incentives to employ genetic tests in health screening programs in order to provide early intervention for treatable conditions. Components of this system may also have an incentive to use genetic testing of enrollees in order to identify the presence of conditions for which benefits are excluded or limited. Capitated components of a managed competition system will have an economic incentive to identify conditions for cost-beneficial early interventions. Such economic incentives have the potential to be translated into requirements that participants in the program undergo testing. If fee-for-service components remain within a managed competition system, they may initially retain existing economic incentives for providers to suggest genetic tests that are of minimal benefit to the patient. However, it seems likely that such incentives will be eliminated as price competition intensifies. To avoid misuse of predictive genetic tests, a system based on a managed competition model must provide universal access to *comprehensive* health care and eliminate competition on the basis of risk selection. These goals will necessitate legal restrictions on exclusion according to pre-existing condition or type of disease, as well as legal prohibitions of churning and redlining. Competition between health care networks on the basis of quality of care, efficiency of delivery of services, and price controls should be encouraged. Stratification of pricing between communities according to relative efficiency of delivery of health care services is a legitimate means of encouraging efficient use of scarce health care resources. However, this comment should be noted in the context of General Recommendation 9.

Policy Recommendations

- **Recommendation 1: Scope of Payment Plans**

  All components of multiple payor systems should pay for, or provide, genetic testing that provides cost-beneficial information to patients. The decision to incorporate a particular genetic test or therapy into the basic package of benefits offered under a multiple payor system should be made on the basis of cost-benefit analysis used to evaluate other tests and therapies. Such a decision should not consider the "genetic" or "non-genetic" nature of the intervention as an appropriate criterion. In the event they wish to obtain genetic information for personal (non-medical) reasons, participants in multiple payor systems should be allowed to purchase genetic tests not covered by their plans.

- **Recommendation 2: Criteria for Plan Membership**

  Components of multiple payor plans should not be allowed to require applicants for membership to submit to genetic testing as a condition of acceptance into the plan or for purposes of establishing a medically underwritten premium structure.

- **Recommendation 3: Genetic Screening Programs**

  Multiple payor plans should allow population screening programs using genetic tests only when absence of test information leads to negative outcomes that are avoidable with therapy (as, for example, in sickle cell disease), and when adequate mechanisms are provided to assure that test results do not result in a loss of health benefits. Genetic tests that are cost-beneficial to specific ethnic subgroups of the population should be included in the services available to members of those groups in all system components, but may be omitted from services offered to other segments of the enrolled population. For example, it may be cost-beneficial or medically necessary to cover cystic fibrosis screening or sickle cell screening only for certain ethnic groups within a larger population. Professional judgements of financially disinterested geneticists about the usefulness of such a test may be the most appropriate criterion for determining whether such a test should be performed (refer to General Recommendation 3). If multiple payor plans institute genetic testing programs (for example, to pursue goals based on health promotion or cost-efficiency) such programs should not compel persons to submit to genetic tests. Such programs should provide a clearly-disclosed means for all enrolled persons to make an informed refusal of testing on grounds of privacy rights or religious or conscientious objections.

- **Recommendation 4: Defining Participating Groups**

  Due to the high prevalence in the general population of conditions with genetic components, groups of covered individuals under a multiple payor system should be structured in order to spread uniformly the cost of such diseases throughout the population.

- **Recommendation 5: Impact of Determination of "Basic" Package**

  When determining the level of comprehensiveness of the "basic" benefits package in a system that preserves competition, policy makers should carefully assess the impact of predictive genetic testing on the need for surcharges and subsidies across plan components in order to avoid rapid re-segmentation of the market.

- **Recommendation 6: Possible Need for Additional Regulation**

  In the event that some components of a multiple payor plan offer more comprehensive services than others, these components may be encouraged to begin stratifying the covered population on the basis of risk derived from genetic test results in existing medical records. In this instance, additional regulations (similar to our recommendations for the current system) may be necessary.

THE RARE DISEASE DATABASE

NORD maintains the Rare Disease Database, accessible to the public and professionals alike, through CompuServe. CompuServe is the nation's largest electronic information system.

- **The Rare Disease Database:**

  A searchable database containing information on hundreds of diseases. Key words can be used to locate information including the name of a disorder, symptoms, general characteristics, drugs, etc.

- **Newsletters:**

  The Newsletters of several national voluntary health agencies are available in this section.

- **Information on More Prevalent Health Conditions:**

  Most articles in this section are publications produced by the Food and Drug Administration, Centers for Disease Control, National Institutes of Health, etc. The articles tend to focus on specific health topics which are newsworthy.

- **The Orphan Drug Database:**

  This section provides a searchable database listing orphan drugs, orphan devices and medical foods.

During the year 1990, the Rare Disease Database was accessed by more than 30,000 persons.

SUPPORTERS OF THE NATIONAL ORGANIZATION FOR RARE DISORDERS

ABBOTT LABORATORIES, R & D
AGVAR CHEMICALS, INC.
ALCON FOUNDATION
AMERICAN HOME PRODUCTS CORPORATION
AMERICAN MEDICAL ASSOCIATION
AMFAR
AMGEN
AMR/AMERICAN AIRLINES FOUNDATION
ARNOLD & PORTER
BARKSDALE BALLARD
BARR LABORATORIES
BARRE-NATIONAL, INC.
BEAR STEARNS & COMPANY, INC.
BERLEX LABORATORIES, INC.
BIOCRAFT LABORATORIES, INC.
BOEHRINGER INGELHEIM PHARMACEUTICALS INC.
BRISTOL-MYERS SQUIBB COMPANY
BURROUGHS WELLCOME CO.
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CAREMARK HOMECARE INC.
CATT FAMILY FOUNDATION
CETUS CORPORATION
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COOK GROUP INCORPORATED
CORD LABORATORIES
CORTEX PHARMACEUTICALS, INC.
CURATEK PHARMACEUTICALS
E.I. DuPONT De NEMOURS & CO.
EER SYSTEMS
ELI LILLY & COMPANY
ENZON, INC.
ESPE-PREMIER SALES CORP.
F-D-C REPORTS, INC.
FISONS PHARMACEUTICALS
FLEIT, JACOBSON, COHN, PRICE, HOLMAN & STERN
FOOD MARKETING INSTITUTE
FORMULATIONS DEVELOPMENT LABORATORIES
FOX, BENNETT & TURNER
GATE PHARMACEUTICALS
GENENTECH, INC.
GENERIC PHARMACEUTICAL INDUSTRY ASSOCIATION
GENETICS & IVF INSTITUTE
GENETICS INSTITUTE, INC.
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GYMA LABORATORIES OF AMERICA
HEALTH INDUSTRY MANUFACTURING ASSOCIATION
HENRY SCHEIN, INC.
HILL & KNOWLTON
HOESCHST-ROUSSEL PHARMACEUTICAL INC.
HOFFMANN-LA ROCHE, INC.
HUGH J. ANDERSEN FOUNDATION
HULINGS FAMILY TRUST
ICI AMERICAS, INC.
ICI PHARMACEUTICALS GROUP
IMMUNOGEN, INC.
INDUSTRIAL BIOTECHNOLOGY ASSOCIATION
INTERCHEM CORP.
JACK ECKERT CORP.
JANSSEN PHARMACEUTICA INC.
JOHNSON & JOHNSON
KAPPA ALPHA THETA SORORITY/ CONNECTICUT CHAPTER
KENDALL-FUTURO CO.
KNOLL PHARMACEUTICALS
LAXALT, RERITO-DUBUC
LEET PATTERSON FOUNDATION
LEMMON CO.
LYMPHOMED
MAHADH FOUNDATION
MARCH OF DIMES BIRTH DEFECTS FOUNDATION
MARION LABORATORIES, INC.
MCNEIL PHARMACEUTICAL
MCGAW LABORATORIES
MEDICUS INTERCON PUBLIC RELATIONS
MERCK CONSUMER PHARMACEUTICALS COMPANY
MERCK & CO., INC.
MERRELL DOW PHARMACEUTICALS
MILES LABORATORIES, INC,
MYLAN PHARMACEUTICALS INC.
NMC LABORATORIES
NATIONAL ASSOCIATION of CHAIN DRUG STORES
NORWICH EATON PHARMACEUTICAL, INC.
NOVO NORDISK OF N.A., INC
NUTRO LABORATORIES, INC.
ORTHO PHARMACEUTICAL, CORP.
OSCO DRUG, INC.
PEOPLES DRUG STORES
PFIZER PHARMACEUTICALS
PHARMACEUTICAL BASICS, INC.
PHARMACEUTICAL DEVELOPMENT ASSOCIATES, INC.
PHARMACEUTICAL MANUFACTURERS ASSOCIATION
PHARMACEUTICAL RESEARCH INSTITUTE
PHARMAKINETICS LABORATORIES
PREMIER DENTAL PROD., CO.
R.W. JOHNSON PHARMACEUTICAL RESEARCH INSTITUTE
RAYNIE FOUNDATION
REED & CARNICK
REVCO DRUG STORES,INC.
ROBERTS PHARMACEUTICAL, CORP.
RUGBY LABORATORIES
SANDOZ
SCHEIN PHARMACEUTICAL, INC.
SCHERING-PLOUGH CORPORATION
SERONO LABORATORIES, INC.
SIGMA-TAU PHARMACEUTICALS, INC.
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SOMERSET PHARMACEUTICALS, INC.
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SUPER X DRUG STORES
SUTTON COMMUNICATIONS, INC.
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UPJOHN
WALLACE LABORATORIES, DIVISION OF CARTER WALLACE. INC.
WARNER-LAMBERT COMPANY
ZETACHRON, INC.

THE NORD RESEARCH GRANT PROGRAM

The NORD Research Grant program is unique in that it only funds clinical research. This means that NORD's research grants support the final phases of the research process — where the potential for positive results are most closely at hand, but where researchers often face the greatest difficulty in finding research dollars. At the clinical research stage, a treatment has been successfully tested in the lab and now faces the most critical test — its effectiveness in human subjects with the target disease. The NORD Research Grant Program awarded its first grants, totaling $140,000 in January of 1989 to four scientists pursuing new treatments for serious and life-threatening illnesses that afflict young children.

- Francine Kaufman, M.D., of the Children's Hospital of Los Angeles to study galactosemia, a genetic disease that, if not treated early, can arrest physical and mental development, cause loss of vision and even death.

- Ronald J. Sokol, M.D., of the University of Colorado Health Sciences Center to document the safety and effectiveness of a new drug, TPGS, to correct vitamin E deficiency in infants and children with liver diseases. Obstructions of the bile duct occur once in every 5,000 infants, causing degenerative, crippling and progressive neuromuscular damage.

- Saul W. Brusilow, M.D., of Johns Hopkins School of Medicine to study the effects of various treatments of urea cycle defects. Without treatment, this group of genetic diseases is fatal within the first year of life.

- Blanche P. Alter, M.D., of Mount Sinai Medical Center, New York, to conduct basic research into Fanconi's Anemia, a genetic blood disorder. Ten to fifteen percent of children with this disorder develop leukemia.

Specifically, NORD funded research has resulted in a new orphan drug to treat vitamin E deficiency in infants and children with liver disease. Work on urea cycle defects has led to new grants from the National Institutes of Health (NIH) and the Food and Drug Administration to further this research while research on a treatment for galactosemia has received a five year grant from NIH to further develop the drug Uridine.

The National Organization for Rare Disorders (NORD) was founded in 1980 as an informal coalition of small voluntary health agencies and individuals affected by orphan diseases for the purpose of working toward the passage of the Orphan Drug Act.

Incorporated in May of 1983, NORD's mission is to encourage increased identification, control and treatment of orphan diseases as well as the monitoring of the Orphan Drug Act to ensure its implementation to the fullest extent possible.

Orphan Diseases

Any disease which affects fewer than 200,000 Americans is deemed an "Orphan Disease." There are more than 5,000 of these diseases which strike people of all ages, races and ethnic backgrounds. A number of these "Orphans" fall within the realm of wide spread health conditions such as heart disease and cancer. While cancer will strike an estimated one out of every four Americans, only a few subtypes of cancer will affect more than 200,000 people at any time. Thus, many cancer patients face the same problems as those with lesser known ailments. Orphan Diseases are collectively this nation's most significant health problem affecting more than 20,000,000 Americans.

Organization

The National Organization for Rare Disorders' activities are guided by a minimum number of career professionals and support staff from offices in New Fairfield, Connecticut. NORD has 134 organizational members composed of national voluntary health agencies and over 30,000 individual members.

NORD in Your Community

NORD and its volunteers are dedicated to the common goal of raising money to fund clinical research and the programs of NORD. Support groups, educating the public and networking patients with the same illness bring NORD and the community closer together — bonded by a common purpose.

Financial Needs

The National Organization for Rare Disorders is supported by contributions from the general public. The Organization actively seeks tax deductible gifts for its research program, as well as unrestricted contributions for the other programs of NORD.

Deferred gifts in the form of wills and bequests, trusts and insurance benefits are also welcome and constitute an important source of income for NORD programs.

Tax Status

The National Organization for Rare Disorders is registered and operates as a not-for-profit corporation under the laws of the State of New York, and is not classified as a private foundation. The Organization is also exempt from United States Federal Income Taxes under Section 501(c)(3) of the Internal Revenue Code.

BEQUESTS

A **bequest** is simply a way to contribute property or money in a will. A bequest to NORD is fully deductible for estate tax purposes.

ENDOWMENT OR MEMORIAL FUND

A **memorial** or **endowment fund** guarantees that a gift will virtually last forever. The original gift is invested by the board of directors of NORD, and the interest earned each year is then used either for a purpose specified in the endowment (e.g., NORD's research program), or as a general fund to use as the board of directors determines. The principal remains intact. Gifts made to the endowment or memorial fund have the same tax savings as the others described above.

Summary

Information in this brochure suggests various ways to make tax-saving donations to NORD. Before making any decisions we **strongly recommend** that you consult with your financial planner, accountant, and/or attorney.

COURAGE

Anonymous: As the old man walked the beach at dawn, he noticed a young man ahead of him picking up starfish and flinging them into the sea. Finally catching up with the youth, he asked him why he was doing this. The answer was that the stranded starfish would die if left until the morning sun.

"But the beach goes on for miles and there are millions of starfish," countered the other. "How can your effort make any difference?"

The young man looked at the starfish in his hand and then threw it to safety in the waves. "It makes a difference to this one," he said.

— Minnesota Literacy Council.

A MESSAGE FROM NORD

The short but meaningful story on the preceding page has been a symbolic narrative that was pinned to a bulletin board in the NORD office some years ago. Every once in a while we reread the short precise words to remind ourselves about NORD's mission. With our small staff, cramped quarters, overwhelming work load and daily toil of listening to agonizing outcries of suffering from people with orphan diseases, we define our primary job as "starfish throwers." Other non-profit health agencies can appeal for the public's support claiming that they're helping "millions" of people. Although combined together orphan diseases touch the lives of 20 million Americans, we handle so many individual diseases that we often focus our attention on one disease at a time . . . one person at a time . . . one starfish thrown back into the water even though the beach is littered with millions who may die in the sun.

We wish we could work a little faster. If only we had more hands to save more starfish; but we would have to enlist an army to sweep the sand clean every day. You are the soldiers we need...you, your friends and family ...each can help us accomplish this overwhelming task and give us the courage not to give up!

NORD needs your donations to answer letters, to network families together, to support research, to focus the attention of government and health-related industries on **your** plight. We can't do it without money. We could just give up and let the starfish wither in the sun, but there is that stubborn and overwhelming sense of purpose that drives us on, day after day, year after year, on that lonely beach flinging God's creatures back to life. Join our little army, please, with your dollars and commitment.

NORD

National Organization for Rare Disorders

TM

. . . out of the darkness, into the light . . .

PLANNED GIVING: A GIFT TO NORD IS A GIFT OF LIFE

NORD
P.O. Box 8923
New Fairfield, CT 06812
(203) 746-6518

Dedicated to Helping People with Orphan Diseases

Most of us do not think ourselves capable of making a significant impact on the lives of people in need. However, with careful planning each of us **can** make a difference through support of the **National Organization for Rare Disorders (NORD)**, which is dedicated to the identification, control and treatment of rare, "orphan diseases."

Orphan diseases are rare debilitating illnesses affecting small numbers of people (200,000 Americans or less). There are more than 5,000 such conditions which strike people of all ages, races, and ethnic backgrounds; collectively these illnesses touch the lives of more than twenty million Americans.

The government provides some significant tax benefits when you make year-end and deferred gifts to NORD. Following are several ways you can help NORD to help others through charitable gifts that may reduce your taxes, now and in the future. We urge you to contact your financial advisor to determine the best gift for your situation.

A GIFT OF CASH

A gift of cash prior to the end of the year could give you a tax savings at filing time if you itemize your tax deductions. For example, if you are in the 28% marginal tax bracket, a gift of $5,000 to NORD could save you $1,400 in federal taxes, plus possible state and local income tax savings.

STOCKS/REAL ESTATE

Gifts of appreciated stock or real estate can provide you with a tax advantage. If you have owned the stock or real estate for more than one year, you can deduct the full market value of the gift as a charitable donation and by-pass capital gains taxes. (You should check with your personal tax advisor regarding the possibility of triggering the Alternative Minimum Tax on the appreciated portion of the investment.)

PERSONAL PROPERTY

Contributions of personal property related to NORD's exempt purposes are fully tax deductible at fair market value.

INSURANCE

A new life insurance policy can be created naming NORD as the owner and beneficiary, or an existing policy can be donated to NORD.

A paid-up existing policy for which the coverage is no longer needed (e.g., a policy covering a paid-up mortgage) can be donated and the amount of the charitable contribution is considered to be the replacement value or the cost basis of the policy, whichever is less. Ongoing premiums paid on a gifted policy also qualify for charitable tax deductions.

TRUST OR DEFERRED GIFT

A **trust** or **deferred gift** is a way to give a gift, receive income from it while you are alive, and take a current tax deduction. There are several types of trusts:

A **short-term** trust pays income to a charitable beneficiary for a set number of years (at least 10 years and 1 day), after which the property reverts back to the donor.

The more **traditional trust** pays income to the donor or other beneficiaries for life, after which the charitable institution owns the property. A **unitrust** is funded with an asset such as appreciated property or stocks. These assets can be sold within the trust and the proceeds reinvested to produce a greater yield for the donor(s) or beneficiaries. The income stream is a fixed percent (not less than 5%) of the asset value of the trust. As the value of the trust increases so does the income payout, thus providing a hedge against inflation.

An **annuity trust** is like a unitrust. The major difference is that the annuity trust pays a fixed dollar amount based on the trust's initial value. Both offer a current income tax deduction and by-pass of capital gains when sold, plus several future benefits.

A **deferred gift** works the same way as a trust. For example, if the gift is a home, the donor(s) or his family can continue to live in it as long as they choose. Thereafter, NORD would be entitled to manage or sell the property.