Communities That Have Benefited from the PRV Program

This is a running list of the disease areas that have treatments that received a PRV following approval as of March2025.

- Achondroplasia
- Acid Sphingomyelinase Deficiency (ASMD)
- Activated Phosphoinositide 3-kinase Delta (PI3Kδ) Syndrome (APDS)
- Alagile Syndrome
- Alpha-mannosidosis
- Aromatic L-amino Aacid Decarboxylase Deficiency
- Biallelic RPE65 Mutation
- Bile Acid Synthesis Disorder (BASD)
- CDKL5 Deficiency Disorder
- Cerebral Adrenoleukodystrophy
- CHAPLE Disease
- Classic Congenital Adrenal Hyperplasia
- CLN2 Disease
- Congenital Athymia
- Congenital Thrombotic Thrombocytopenic Purpura (cTTP)
- Cystic Fibrosis
- Dravet Syndrome
- Duchenne Muscular Dystrophy (DMD)
- Epidermolysis Bullosa
- Familial Intrahepatic Cholestasis
- Fibrodysplasia Ossificans Progressiva
- Friedreich's Ataxia
- Hereditary Orotic Aciduria
- Hutchinson-Gilford Progeria Syndrome
- Hypophosphatasia
- Hypoplasminogenemia
- Lennox Gastaut Syndrome
- Lysosomal Acid Lipase Deficiency
- Metachromatic Leukodystrophy (MLD)
- Molybdenum Cofactor Deficiency Type A
- Morquio A Syndrome
- Neimann Pick Type- C
- POMC
- Primary Hemophagocytic Lymphohistiocytosis
- Primary Hyperoxaluria
- Processing-Deficient Progeroid Laminopathies
- Rett Syndrome
- Sickle Cell Disease
- Sly Syndrome
- Spinal Muscular Atrophy (SMA)
- ß-thalassemia
- Tuberous Sclerosis
- WHIM Syndrome
- X-linked hypophosphatemia