January 4, 2020

The Honorable Frank Pallone
Chairman
Committee on Energy & Commerce
House of Representatives
Washington, DC 20515

The Honorable Greg Walden
Ranking Member
Committee on Energy & Commerce
House of Representatives
Washington, DC 20515

Re: Patient Access to Gene Therapy

Dear Chairman Pallone and Ranking Member Walden,

The undersigned patient organizations urge Congress to enact policies that would help ensure prompt and equitable access to transformative, potentially curative treatments, such as gene therapy. We are concerned that payers may look to limit access to transformative treatments such as gene therapies through restrictive coverage criteria and excessive patient cost sharing as a mechanism for managing the potential short-term budget impact.[1] In order to ensure access to these innovative interventions, stakeholders are exploring novel payment arrangements that balance the financial risk by tying payment for gene therapy to meaningful clinical outcomes under various structures, including a one-time payment upon administration, and payment over a period of time. Legislative action is necessary to create a pathway for these outcomes-based payment models so that patients, including children, are able to receive gene therapy and potentially cure their underlying condition.

Realizing the promise of Cures

More than four years ago during the development, consideration, and enactment of the 21st Century Cures Act, many of the undersigned organizations urged this committee to include provisions that could ensure access to the potential cures stemming from that landmark legislation. These treatments are now arriving. In the last two years, the Food and Drug Administration ("FDA") has approved gene therapy for spinal muscular atrophy and for RPE65 mutation-associated retinal dystrophy.[2] Innovators are also on the cusp of submitting to FDA biologics license applications for several more gene

[1] *See, e.g.*, Rachel Salzman et al, *Addressing the Value of Gene Therapy and Enhancing Patient Access to Transformative Treatments*, 26 MOLECULAR THERAPY 2717 (2018).

[2] *See* Letter from Mary Malarkey, Dir., Off. of Compliance & Biologics Quality, Ctr. for Biologics Evaluation & Research ("CBER") and Wilson Bryan, MD, Dir., Off. of Tissues & Advanced Therapies, CBER, to Jim Wang, Spark Therapeutics, LUXTURNA BLA Approval (Dec. 19, 2017); Letter from Mary Malarkey, Dir., Off. of Compliance & Biologics Quality, CBER, and Wilson Bryan, MD, Dir., Off. of Tissues & Advanced Therapies, CBER, to James L'Italien, AveXis, ZOLGENSMA BLA Approval (May 24, 2019).

therapies—a trend that will only accelerate. In fact, clinical trials[3] are currently underway investigating gene therapies for debilitating and life-threatening rare diseases, including:

- AADC deficiency
- alpha-1 antitrypsin deficiency
- cerebral adrenoleukodystrophy
- Duchenne muscular dystrophy
- epidermolysis bullosa
- Fabry disease
- giant axonal neuropathy
- hemophilia A and B
- Hunter syndrome (MPS II)
- Huntington disease
- Hurler syndrome (MPS I)
- Hemophgocytic Lymphohistiocytosis (HLH)
- Ichthyosis
- late-infantile neuronal ceroid lipofuscinosis type 2 (a form of Batten disease)
- Leber's hereditary optic neuropathy
- limb girdle muscular dystrophy types 2D and 2E
- metachromatic leukodystropy
- familial hypercholesterolemia
- myotubular myopathy
- ornithine transcarbamylase deficiency
- phenylketonuria
- Pompe disease
- Sanfilippo syndrome types A and B (MPS III A and B)
- severe combined immunodeficiency
- sickle cell disease
- thalassemia
- von Gierke's disease
- Wiskott-Aldrich Syndrome

For many of those conditions, gene therapy will be the first ever FDA-approved treatment. Without therapeutic intervention, several such conditions are typically fatal or severely debilitating in childhood, so these scientific advancements are addressing considerable unmet clinical need in extremely vulnerable populations. Our communities deserve access without delay to these potentially life-saving, innovative treatments.

Creating new paradigms to align payment policies with meaningful improvements in patients' lives

Gene therapy represents a new treatment paradigm, wherein a single administration has the potential to significantly alter the clinical course of serious, often life-threatening conditions and transform the lives of patients by replacing or modifying a dysfunctional gene in order to facilitate normal gene expression. The ability to treat the genetic cause of a disease with a single dose of a therapy is a meaningful scientific advancement, particularly for the many patients who face a burdensome standard of care or who lack any treatment options beyond palliative care. The impact on their families and caregivers should also be recognized. While these innovative therapies are expected to confer long-term clinical and quality of life benefit to a patient, long term durability may vary from treatment to treatment and patient to patient. New payment models are emerging to account for this uncertainty by allowing for shared risk arrangements based on patient clinical outcomes.

[3] *See* Addendum A.

It has come to our attention that existing government price reporting laws will prevent payers and biopharmaceutical companies from using these novel, outcomes-based contracting models, including those providing for payments over time, to equitably afford all patients with the opportunity to benefit from gene therapy. The current provisions in section 1927 of the Social Security Act ("SSA") related to best price and average manufacturer price ("AMP"), which Congress enacted nearly 30 years ago, at a time when one-time, potentially transformative gene therapies were not yet in clinical development, are barriers to these payment models. There is, however, growing alignment among stakeholders that our nation's pharmaceutical payment policies must keep pace with the science that is now available to deliver transformative therapies to patients.[4] For example, existing law will prevent biopharmaceutical companies from offering a full or partial refund, or otherwise making a patient and payer financially whole through a remedy for a gene therapy recipient who fails to meet a pre-defined clinical outcome post-treatment. We urge Congress to consider a targeted legislative solution that builds upon the 21st Century Cures Act, the Orphan Drug Act, and decades of other targeted, thoughtful statutes and regulations intended to ensure both the development of and access to transformative therapies.[5]

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[4] *See, e.g.*, Salzman, *supra* note 1, at 2722-2723; The Hon. Alex Azar, Sec., U.S. Dep't of Health & Human Servs., Keynote Address at the NORD Rare Summit (Oct. 22, 2019) (stating that there must be flexibility with respect to government price reporting mechanisms to allow for patients to access gene therapy); Steve Pearson, President, Inst. for Clinical & Econ. Rev., The Time is Now: Addressing Affordability While Sustaining Innovation, NORD Rare Summit (Oct. 21, 2019) (acknowledging Medicaid best price must be addressed in order to facilitate outcomes-based agreements for gene therapy).

[5] 21st Century Cures Act, Pub. L. No. 114-255, 130 Stat. 1033 (2016); Orphan Drug Act, Pub. L. No. 97-414, 96 Stat. 2049 (1983) (codified as amended at 21 U.S.C.S. §§ 360aa-360ee; 26 U.S.C.S. § 45C (LexisNexis 2019)). *See, e.g.,* Medicare Program; Competitive Acquisition of Outpatient Drugs and Biologicals Under Part B, 70 Fed. Reg. 39022, 39028-39029 (Jul. 6, 2005) (illustrating CMS recognition of the need to protect certain rare disease populations from "severe access issues" by exercising its discretion to explicitly exclude from CAP several lifesaving rare disease therapies, including IVIG, alglucerase, alpha$_1$-proteinase inhibitor, blood clotting factors, daclizumab, imiglucerase, and oprelvekin); Patient Protection and Affordable Care Act § 9008(e)(3), 124 Stat. 119, 860 2010 (codified at 26 U.S.C.S. § 4001 note prec. (LexisNexis 2019)) (excluding from the calculation of a manufacturer's annual branded pharmaceutical fee the sales of orphan designated drugs that successfully claimed the orphan drug credit); *id*. § 2501(a), 124 Stat. at 306-307 (codified at 42 U.S.C.S. § 1396r-8(c)(1)(B)(iii)(II)(aa) (LexisNexis 2019)) (significantly limiting the Medicaid outpatient drug minimum rebate percentage increase for blood clotting factors); *id*. § 6301(a), 124 Stat. at 730-731 (codified at 42 U.S.C.S. § 1320e(d)(2)(B)(ii)(III); § 1320e(d)(4)(A)(iii) (LexisNexis 2019)) (requiring the Patient Centered Outcomes Research Institute to convene disease-specific advisory panels each instance a rare disease is considered by the Institute or proposed in a grant application for a comparative effectiveness research study); Health Care and Education Reconciliation Act of 2010 § 2302, 124 Stat. 1029, 1082–1083, *amended by* Medicare and Medicaid Extenders Act of 2010 § 204, Pub. L. No. 111-309, 124 Stat. 3285, 3289 – 3290 (codified at 42 U.S.C.S. § 256b(e) (LexisNexis 2019)) (excluding orphan designated drugs from being sold at the 340B discount to the new categories of hospitals eligible for the 340B Drug Pricing Program); Food and Drug Administration Safety and Innovation Act § 908, Pub. L. No. 112-144, 126 Stat. 993, 1094-1098 (2012) (codified as amended at 21 U.S.C.S. § 360ff (LexisNexis 2019)) (establishing the Rare Pediatric Disease Priority Review Voucher Program).

Enabling outcomes-based and payment over time approaches

In order to facilitate such novel payment approaches for gene therapy, we urge Congress to enact legislation that would:

1. clarify that best price and AMP for a "potentially curative treatment intended for one-time use" sold under an outcomes based agreement exclude prices resulting from a remedial adjustment to the contract price for a non-responding patient; and

2. with respect to a "potentially curative treatment intended for one-time use" sold under an outcomes based agreement that provides for installment payments over a time certain, specify that the calculation of best price and AMP is to be determined as if the aggregate contract price scheduled to be paid over the duration of such agreement was paid in the first installment, rather than based on any single or subset of installment payments for which the contract may provide.

Such nuanced clarifying language added to section 1927 of the SSA will ensure rebates provided under the Medicaid Drug Rebate Program and discounts offered under the 340B Drug Pricing Program are based on the actual contract price of the gene therapy. For some payers, such as small or self-funded plans, outcomes-based agreements, particularly those providing for payment over time, may be the only payment model that can ensure sustainable access for their beneficiaries to gene therapies. This proposed legislative solution establishes a flexible framework that would enable biopharmaceutical companies and payers to develop customized approaches that ultimately foster patient access.

Ensuring equal access for all patients, regardless of their insurance coverage

Patients with serious diseases rely on a range of insurance types, including public and private insurance. In order to ensure access to gene therapy for *all* patients, Congress should enact a policy that would allow the flexibility to offer outcomes-based contracts, including those that allow for installment payments, to *all* payers. We urge Congress to consider a policy that would remove the existing government price reporting barriers to outcomes-based contracts, including installment payment models, through targeted clarifying language in section 1927 of the SSA.

Section 208 of S. 2543, the Prescription Drug Pricing Reduction Act of 2019, recently reported by the Senate Finance Committee, is an important starting point for creating an access pathway for gene therapy. We commend this landmark, bipartisan Senate effort, including those provisions that will help control and reduce co-payments and out of pocket costs borne by gene therapy patients. Although the scope of section 208 is limited to the Medicaid program, it represents an essential legislative breakthrough in linking payment for disruptive technologies to clinical outcomes. We believe that children and other patients with commercial health insurance should be afforded the same protection that, if enacted, section 208 promises for Medicaid beneficiaries.

Toward that end, we applaud the bipartisan amendment that was offered and withdrawn by Representatives Mullin, Schrader, and Guthrie on October 17, 2019 during the committee's mark up of H.R. 3, the Lower Drug Costs Now Act of 2019. We stand ready to continue to partner with its sponsors, members of your committee, and other stakeholders to further clarify and refine this important bipartisan proposal. We urge rapid adoption of a revised measure during this session of Congress.

Gene therapy has the potential to result in not only a better quality of life and patient health outcomes for our patients who are candidates for treatment, but also long-term savings to them individually and the larger health care system.[6] Congress must also consider the value of providing relief to all those affected by genetic diseases of the considerable financial, psychological, and other challenges that these conditions present for all family members. The benefits of these potentially life-saving innovations will not be realized until patients have a pathway to access. Time is of the essence.

Thank you for your consideration.

Sincerely,

EveryLife Foundation for Rare Diseases
Parent Project Muscular Dystrophy (PPMD)
MLD Foundation
National MPS Society
Project Alive
Rare Trait Hope Fund
International Foundation for CDKL5 Research
Huntington's Disease Youth Organization
Cure Sanfilippo Foundation
Little Hercules Foundation
National Tay-Sachs & Allied Diseases Association (NTSAD)
International Foundation for CDKL5 Research
Friedreich's Ataxia Research Alliance
Cure SMA
National PKU Alliance
Amyloidosis Support Groups Inc.
International Pemphigus & Pemphigoid Foundation
American Behcet's Disease Association (ABDA)
Alström Angels
PCDH19 Alliance
Foundation for Ichthyosis & Related Skin Types, Inc.

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[6] *See e.g.*, Stacy E. Croteau et al, *Regional Variation and Cost Implications of Prescribed Extended Half-life Factor Concentrates among U.S. Haemophilia Treatment Centres for Patients with Moderate and Severe Haemophilia*, 25 HAEMOPHILIA 668, 673 table 2 (2019) (describing annual cost of blood clotting factor therapy for severe hemophilia A patients as between $690,000 and $750,000 to cover two to three prophylaxis infusions per week).

Histiocytosis Association
Cure HHT
Children's PKU Network
Gwendolyn Strong Foundation
Cutaneous Lymphoma Foundation
VHL Alliance
Usher Syndrome Coalition
The Oxalosis and Hyperoxaluria Foundation
The Marfan Foundation
Children's Tumor Foundation
United Mitochondrial Disease Foundation
Jeffrey Modell Foundation
Tuberous Sclerosis Alliance
Noah's Hope - Hope 4 Bridget Foundation
Adult Polyglucosan Body Disease Research Foundation
CureDuchenne

cc: Representatives Eshoo, Burgess, Butterfield, Bilirakis, Bucshon, Carter, DeGette, Dingell, Guthrie, Hudson, Matsui, Mullin, Peters, Ruiz, Schrader, and Upton

Addendum A: Snapshot of Current U.S. Clinical Sites for Gene Therapy for Rare Diseases

Institution	Disease(s)	Member of Congress
University of Massachusetts Medical School	• alpha-1 antitrypsin deficiency • Leber congenital amaurosis	Rep. Jim McGovern
Massachusetts Eye and Ear Infirmary	• achromatopsia • biallelic RPE65 mutation-associated retinal dystrophy • x-linked retinitis pigmentosa • x-linked retinoschisis	Rep. Stephen Lynch
Boston Children's Hospital	• cerebral adrenoleukodystrophy • chronic granulomatous disease • hemophilia A • hemophilia B • ornithine transcarbamylase deficiency • phenylketonuria • severe combined immunodeficiency disease (x-linked) • sickle cell disease • spinal muscular atrophy (type 1) • x-linked retinitis pigmentosa	Rep. Ayanna Pressley
UConn Health	• Von Gierke's disease (glycogen storage disease type IA)	Rep. Jahana Hayes
Hackensack University Medical Center	• sickle cell disease	Rep. Bill Pascrell
Weill Medical College of Cornell University	• neuronal ceroid lipofuscinosis type 2 (CLN2 disease or late infantile Batten disease)	Rep. Carolyn Maloney
Weill Cornell Medicine-Comprehensive Center for Hemophilia and Coagulation Disorders	• hemophilia A • hemophilia B	Rep. Carolyn Maloney
New York Presbyterian/Weill Cornell Medical Center	• hemophilia B • neuronal ceroid lipofuscinosis type 2 (CLN2 disease or late infantile Batten disease) • Sanfilippo syndrome type A (MPS IIIA) • sickle cell disease	Rep. Carolyn Maloney
NYU Langone Health	• Fabry disease	Rep. Carolyn Maloney
Memorial Sloan Kettering	• beta thalassemia	Rep. Carolyn Maloney
Mount Sinai Medical Center	• hemophilia B	Rep. Adriano Espaillat
Icahn School of Medicine at Mount Sinai	• Fabry disease • ornithine transcarbamylase deficiency	Rep. Adriano Espaillat

Institution	Disease(s)	Member of Congress
	• phenylketonuria	
Columbia College of Physicians and Surgeons	• achromatopsia • choroideremia • x-linked retinitis pigmentosa	Rep. Adriano Espaillat
Columbia University Irving Medical Center	• spinal muscular atrophy (type 1)	Rep. Adriano Espaillat
Western New York BloodCare	• hemophilia B	Rep. Brian Higgins
UPMC Eye Center	• x-linked retinitis pigmentosa	Rep. Mike Doyle
Hemophilia Center of Western Pennsylvania	• hemophilia A • hemophilia B	Rep. Mike Doyle
Children's Hospital of Pittsburgh - UPMC: Program for Neurodevelopment in Rare Disorders	• Hunter syndrome (MPS II) • phenylketonuria • Sanfilippo syndrome type A (MPS IIIA)	Rep. Mike Doyle
Pennsylvania State University Milton S. Hershey Medical Center	• hemophilia A	Rep. Scott Perry
Scheie Eye Institute, University of Pennsylvania	• biallelic RPE65 mutation-associated retinal dystrophy • Leber congenital amaurosis	Rep. Dwight Evans
The Hospital of the University of Pennsylvania	• achromatopsia • homozygous familial hypercholesterolemia • Hunter syndrome (MPS II)	Rep. Dwight Evans
Children's Hospital of Philadelphia	• beta thalassemia • hemophilia A • hemophilia A w/ inhibitors • hemophilia B • Hunter syndrome (MPS II) • Hurler syndrome (MPS I) • Leber congenital amaurosis • sickle cell disease • spinal muscular atrophy (type 1)	Rep. Dwight Evans
Wills Eye Hospital	• Leber's hereditary optic neuropathy • x-linked retinitis pigmentosa	Rep. Mary Gay Scanlon
Children's National Medical Center Hematology and Oncology	• hemophilia B	Rep. Eleanor Holmes Norton
The Wilmer Eye Institute - The Johns Hopkins Hospital	• x-linked retinoschisis	Vacant
Johns Hopkins Pediatric Neurology	• spinal muscular atrophy (type 1)	Vacant

Institution	Disease(s)	Member of Congress
NIH Clinical Center	• beta thalassemia • chronic granulomatous disease • Fanconi's anemia type C • giant axonal neuropathy • GM1 gangliosidosis (type II) • severe combined immunodeficiency due to ADA deficiency • severe combined immunodeficiency (x-linked) • sickle cell disease • x-linked retinoschisis	Rep. Jamie Raskin
Lysosomal and Rare Disorders Research and Treatment Center	• Fabry disease	Rep. Gerald Connolly
Virginia Commonwealth University School of Medicine	• hemophilia A • hemophilia A w/ inhibitors • Huntington disease	Rep. Donald McEachin
UNC Hemophilia and Thrombosis Center	• hemophilia A • hemophilia B	Rep. David Price
Duke University	• Duchenne muscular dystrophy • Pompe disease • spinal muscular atrophy (type 1)	Rep. GK Butterfield
Duke Eye Center, Duke University Medical Center	• achromatopsia • x-linked retinitis pigmentosa • x-linked retinoschisis	Rep. GK Butterfield
East Carolina University Brody School of Medicine	• hemophilia A	Rep. GK Butterfield
Medical University of South Carolina	• beta thalassemia • hemophilia A • hemophilia B • sickle cell disease	Rep. Jim Clyburn
Emory University School of Medicine	• Fabry disease • hemophilia A • hemophilia B • Leber's hereditary optic neuropathy • phenylketonuria	Rep. John Lewis
Vitreo Retinal Associates	• achromatopsia	Rep. Ted Yoho
University of Florida, College of Medicine, Department of Pediatrics	• alpha-1 antitrypsin deficiency	Rep. Ted Yoho
UF Health Shands Children's Hospital	• Leber congenital amaurosis • Pompe disease	Rep. Ted Yoho
Clinical and Translational Research Building - University of Florida	• Pompe disease	Rep. Ted Yoho

Institution	Disease(s)	Member of Congress
University of Miami - Miller School of Medicine Bascom Palmer Eye Institute	• achromatopsia • biallelic RPE65 mutation-associated retinal dystrophy • choroideremia • Leber's hereditary optic neuropathy • x-linked retinitis pigmentosa • x-linked retinoschisis	Rep. Frederica Wilson
Nemours Children's Hospital Orlando	• spinal muscular atrophy (type 1)	Rep. Darren Soto
St. Joseph's Children's Hospital, Center for Bleeding and Clotting Disorders	• hemophilia A	Rep. Kathy Castor
USF Health Hemophilia Treatment Center	• hemophilia B	Rep. Kathy Castor
Vanderbilt University Medical Center	• hemophilia A • hemophilia B • homozygous familial hypercholesterolemia	Rep. Jim Cooper
University of Tennessee Health Science Center	• hemophilia B	Rep. Steve Cohen
St Jude's Children's Research Hospital	• hemophilia A • hemophilia B • severe combined immunodeficiency (x-linked)	Rep. Steve Cohen
Mississippi Center for Advanced Medicine	• hemophilia A • hemophilia A w/ inhibitors • hemophilia B	Rep. Michael Guest
University of Mississippi Medical Center	• hemophilia B	Rep. Michael Guest
UofL Health - James Graham Brown Cancer Center	• hemophilia A	Rep. John Yarmuth
UK HealthCare Hemophilia Treatment Center	• hemophilia B	Rep. Andy Barr

Institution	Disease(s)	Member of Congress
Nationwide Children's Hospital	• Becker muscular dystrophy • Charcot-Marie-Tooth neuropathy type 1A • Duchenne muscular dystrophy • dysferlin deficiency • hemophilia A • limb girdle muscular dystrophy type 2D • limb girdle muscular dystrophy type 2E • neuronal ceroid lipofuscinosis type 3 (CLN3 disease or juvenile Batten disease) • neuronal ceroid lipofuscinosis type 6 (CLN6 disease or variant late infantile Batten disease) • Sanfilippo syndrome type A (MPSIIIA) • Sanfilippo syndrome type B (MPSIIIB) • spinal muscular atrophy (type 1) • sporadic inclusion body myositis	Rep. Joyce Beatty
Ohio State University Hospital	• Huntington disease	Rep. Joyce Beatty
University Hospitals Cleveland Medical Center	• hemophilia A • ornithine transcarbamylase deficiency	Rep. Marcia Fudge
Cleveland Clinic	• x-linked retinitis pigmentosa	Rep. Marcia Fudge
Cincinnati Children's Hospital Medical Center	• alpha-1 antitrypsin deficiency • Fabry disease • hemophilia A • severe combined immunodeficiency (x-linked) • sickle cell disease	Rep. Steve Chabot
Cincinnati Eye Institute	• achromatopsia • biallelic RPE65 mutation-associated retinal dystrophy • x-linked retinitis pigmentosa	Rep. Brad Wenstrup
Indiana Hemophilia and Thrombosis Center	• hemophilia A	Rep. Susan Brooks
University of Michigan Kellogg Eye Center	• achromatopsia • biallelic RPE65 mutation-associated retinal dystrophy • Leber congenital amaurosis • x-linked retinitis pigmentosa • x-linked retinoschisis	Rep. Debbie Dingell
C.S. Mott Children's Hospital	• hemophilia A	Rep. Debbie Dingell

Institution	Disease(s)	Member of Congress
Michigan Medicine University of Michigan	• hemophilia B • Von Gierke's disease (glycogen storage disease type IA)	Rep. Debbie Dingell
University of Michigan, Pediatric Hematology and Oncology	• hemophilia A	Rep. Debbie Dingell
Wayne State University, Detroit Medical Center	• hemophilia A	Rep. Rashida Tlaib
Michigan State University Center for Bleeding and Clotting Disorders	• hemophilia A	Rep. Elissa Slotkin
Michigan State University Clinical Center	• hemophilia B	Rep. Elissa Slotkin
University of Iowa Hospitals & Clinics	• biallelic RPE65 mutation-associated retinal dystrophy • Leber congenital amaurosis	Rep. David Loebsack
Medical College of Wisconsin	• achromatopsia	Rep. Jim Sensenbrenner
Blood Research Institute at Blood Center of Wisconsin	• hemophilia A • hemophilia B	Rep. Jim Sensenbrenner
UW Health University Hospital	• hemophilia A • spinal muscular atrophy (type 1)	Rep. Mark Pocan
University of Minnesota, Masonic Clinical Research Unit, Clinical and Translational Science Institute	• hemophilia B	Rep. Ilhan Omar
University of Minnesota Masonic Children's Hospital	• cerebral adrenoleukodystrophy • hemophilia A • Hunter syndrome (MPS II) • Sanfilippo syndrome type A (MPS IIIA)	Rep. Ilhan Omar
Pangere Center for Inherited Retinal Diseases	• achromatopsia	Rep. Danny Davis
Ann & Robert H. Lurie Children's Hospital of Chicago	• beta thalassemia • hemophilia A • sickle cell disease • spinal muscular atrophy (type 1)	Rep. Danny Davis
Rush University Medical Center	• hemophilia A • hemophilia B	Rep. Danny Davis
Washington Univ. School of Medicine	• hemophilia A • spinal muscular atrophy (type 1)	Rep. Wm Lacy Clay
Tulane University Hematology & Medical Oncology	• hemophilia A	Rep. Cedric Richmond
Arkansas Children's Hospital	• hemophilia A • hemophilia B	Rep. J. French Hill
Retina Foundation of the Southwest	• x-linked retinitis pigmentosa • x-linked retinoschisis	Rep. Colin Allred

Institution	Disease(s)	Member of Congress
University of Texas Southwestern	• hemophilia B • spinal muscular atrophy (type 1)	Rep. Eddie Bernice Johnson
Baylor Scott and White Memorial Hospital	• hemophilia B	Rep. John Carter
UT Physicians Gulf States Hemophilia and Thrombophilia Center – Texas Medical Center	• hemophilia A • hemophilia B	Rep. Dan Crenshaw
Univ. of Texas Health Science Center McGovern Medical School	• Huntington disease • Von Gierke's Disease (glycogen storage disease type IA)	Rep. Al Green
Baylor College of Medicine, Texas Children's Hospital	• Sanfilippo syndrome type A (MPSIIIA)	Rep. Al Green
Baylor College of Medicine, Alkek Eye Center	• biallelic RPE65 mutation-associated retinal dystrophy • x-linked retinoschisis	Rep. Al Green
UCHealth University of Colorado Hospital	• hemophilia B	Rep. Jason Crow
The Children's Hospital Colorado	• ornithine transcarbamylase (OTC) deficiency • spinal muscular atrophy (type 1)	Rep. Jason Crow
U of Colorado School of Medicine, Hemophilia & Thrombosis Treatment Center	• hemophilia A • hemophilia B	Rep. Jason Crow
National Jewish Health	• alpha-1 antitrypsin deficiency	Rep. Diana DeGette
Colorado Retina Associates - Red Rocks Medical Center	• x-linked retinitis pigmentosa	Rep. Ed Perlmutter
University of Utah Health	• Duchenne muscular dystrophy • hemophilia B • spinal muscular atrophy (type 1)	Rep. Chris Stewart
Phoenix Children's Hospital	• hemophilia A • hemophilia B	Rep. Ruben Gallego
Orthopaedic Institute for Children Hemophilia Treatment Center	• hemophilia A • hemophilia B	Rep. Karen Bass
Children's Hospital Los Angeles	• biallelic RPE65 mutation-associated retinal dystrophy • hemophilia B	Rep. Adam Schiff

Institution	Disease(s)	Member of Congress
UCLA Mattel Children's Hospital	• cerebral adrenoleukodystrophy • Duchenne muscular dystrophy • leukocyte adhesion deficiency type 1 • severe combined immunodeficiency due to ADA deficiency • severe combined immunodeficiency (x-linked)	Rep. Ted Lieu
Ronald Reagan UCLA Medical Center	• chronic granulomatous disease • Duchenne muscular dystrophy • ornithine transcarbamylase (OTC) deficiency • sickle cell disease	Rep. Ted Lieu
University of California, San Diego – Jacobs Medical Center	• cystinosis • Danon disease	Rep. Scott Peters
Hemophilia and Thrombosis Treatment Center, UC San Diego Health - La Jolla	• hemophilia A • hemophilia B	Rep. Scott Peters
University of California, San Diego Medical Center – Hillcrest	• hemophilia B	Rep. Susan Davis
Loma Linda University Children's Hospital Pediatric Specialty Clinics	• hemophilia A	Rep. Pete Aguilar
Children's Hospital of Orange County	• Sanfilippo syndrome type A (MPS IIIA)	Rep. J. Luis Correa
UCSF Helen Diller Medical Center at Parnassus Heights	• hemophilia A • hemophilia B • x-linked retinoschisis	Rep. Nancy Pelosi
UCSF Benioff Children's Hospital (San Francisco)	• AADC deficiency • severe combined immunodeficiency (Artemis-deficient) • severe combined immunodeficiency (x-linked)	Rep. Nancy Pelosi
UCSF Benioff Children's Hospital (Mission Bay)	• ornithine transcarbamylase (OTC) deficiency	Rep. Nancy Pelosi
Lucile Packard Children's Hospital	• cerebral adrenoleukodystrophy • Fanconi's anemia type A	Rep. Anna Eshoo
Stanford Medical School	• hemophilia B • pyruvate kinase deficiency • recessive dystrophic epidermolysis bullosa • spinal muscular atrophy (type 1)	Rep. Anna Eshoo
UCSF Benioff Children's Hospital (Oakland)	• beta thalassemia • Hunter syndrome (MPS II) • sickle cell disease	Rep. Barbara Lee

Institution	Disease(s)	Member of Congress
UC Davis Medical Center	• hemophilia A • hemophilia B	Rep. Doris Matsui
City of Hope Medical Center	• sickle cell disease	Rep. Grace Napolitano
The Hemophilia Center, Oregon Health & Science University	• hemophilia B	Rep. Earl Blumenauer
Oregon Health & Science University	• hemophilia B • homozygous familial hypercholesterolemia • ornithine transcarbamylase (OTC) deficiency • spinal muscular atrophy (type 1)	Rep. Earl Blumenauer
Casey Eye Institute, Oregon Health & Science University	• achromatopsia • biallelic RPE65 mutation-associated retinal dystrophy • Leber congenital amaurosis • x-linked retinitis pigmentosa • x-linked retinoschisis	Rep. Earl Blumenauer
Bloodworks Northwest Research Institute	• hemophilia B	Rep. Pramila Jayapal
Seattle Children's Research Institute	• severe combined immunodeficiency (x-linked)	Rep. Pramila Jayapal