

MEMORANDUM

To: Subcommittee on Health Members and Staff
From: Committee on Energy and Commerce Majority Staff
Re: Health Subcommittee Hearing on February 29, 2024

The Subcommittee on Health will hold a hearing on Thursday, February 29, 2024, at 10:00 a.m. (ET) in 2123 Rayburn House Office Building. The hearing title is “Legislative Proposals to Support Patients with Rare Diseases.”

I. Witnesses

- **Dr. Terence Flotte, MD**, Provost and Dean of UMass Chan Medical School, Vice President of American Society of Gene and Cell Therapy (ASGCT)
- **Dr. Alexander Bassuk, MD, PhD**, Physician-in-Chief, University of Iowa Stead Family Children’s Hospital; Chair and Professor, Stead Family Department of Pediatrics
- **Dr. Aaron Kesselheim, MD, JD, MPH**, Professor of Medicine, Harvard Medical School; Director, Program On Regulation, Therapeutics, And Law (PORTAL) at Brigham and Women’s Hospital
- **Dr. Jeromie Ballreich, PhD**, Associate Research Professor, Johns Hopkins Bloomberg School of Public Health
- **Dr. Alice Chen, PhD**, Senior Fellow, USC Schaeffer Center for Health Policy and Economics; Associate Professor and Vice Dean for Research, USC Sol Price School of Public Policy
- **Ms. Khrystal Davis, JD**, Founding President, Texas Rare Alliance

II. Background

Rare Disease Day takes place annually on the last day of February, and it provides an opportunity to recognize the impacts of rare diseases on the lives of Americans and those around the world, as well as the progress that has been made in treating and curing these diseases. Under the Orphan Drug Act of 1983, rare diseases are defined as diseases or conditions that affect less than 200,000 people in the United States.¹ As many as 80 percent of all rare diseases are believed to have genetic origins,² and all pediatric cancers are considered rare.³ Today, there are an

¹ Orphan Drug Act, Pub. L. No. 97-414, § 5. See also Rare Diseases Act of 2002, Pub. L. No. 107-280, § 3.

² National Human Genome Research Institute. “Rare Genetic Diseases.” <https://www.genome.gov/dna-day/15-ways/rare-genetic-diseases>

³ National Cancer Institute. “Rare Cancers of Childhood Treatment (PDQ) – Health Professional Version.” <https://www.cancer.gov/types/childhood-cancers/hp/rare-childhood-cancers-pdq#:~:text=Therefore%2C%20all%20pediatric%20cancers%20are%20considered%20rare.&text=Most%20cancers%20in%20subgroup%20I,aged%2015%20to%2019%20years.>

estimated 10,000 known rare diseases that affect approximately 30 million Americans,⁴ and cost the health care system up to \$400 billion per year in direct medical expenses.⁵ Only about 500 of these diseases have approved treatments.⁶

In order to support patients with rare diseases, Congress has taken critical steps to support the development of treatments and improve patient access to care. For instance, by funding foundational research and creating a hospitable regulatory environment for innovation, Congress has facilitated investment in the rare disease space to bring more breakthrough cures to market. Once these therapeutic and curative products are commercialized, legislators have worked to ensure that payers are able to cover new treatments so that patients can access the best care possible.

Congress passed the Orphan Drug Act of 1983, which encourages manufacturers to enter smaller rare disease markets by providing incentives for the development of treatments for rare diseases. Under this law, drug developers may request orphan drug designations early in the development of such drug, which qualifies sponsors for tax credits, user fee waivers, and the potential for 7 years of market exclusivity if the drug is approved by the U.S. Food and Drug Administration (FDA). The law also established the Orphan Product Grants Program to support clinical trials for rare disease products. Since the passage of the Orphan Drug Act of 1983, the FDA has granted orphan designation to nearly 7,000 drugs and has approved over 1,200 drugs to treat rare diseases, impacting millions of patients.⁷ In more recent years, Congress has furthered this work in the Food and Drug Omnibus Reform Act of 2022 and the 21st Century Cures Act,⁸ which among other programs, established the Rare Disease Endpoint Advancement (RDEA) Pilot Program,⁹ extended the Pediatric Priority Review Voucher Program,¹⁰ modernized the accelerated approval pathway,¹¹ increased patient involvement and input in the drug development and review process,¹² and created platform therapy designations.¹³

Despite such progress, significant challenges stand in the way of meeting the needs of patients with rare diseases and their families. The Inflation Reduction Act (IRA), which was signed into law on August 16, 2022, reportedly has disincentivized developing innovative

⁴ National Institutes of Health. "What is a rare disease?" <https://rarediseases.info.nih.gov/about>

⁵ National Center for Advancing Translational Sciences. "NIH Study Suggests People with Rare Diseases Face Significantly Higher Health Care Costs." 2021. <https://www.nih.gov/news-events/news-releases/nih-study-suggests-people-rare-diseases-face-significantly-higher-health-care-costs>

⁶ National Institutes of Health. "Rare Diseases" <https://www.nih.gov/about-nih/what-we-do/nih-turning-discovery-into-health/promise-precision-medicine/rare-diseases>

⁷ Food and Drug Administration. "Search Orphan Drug Designations and Approvals" <https://www.accessdata.fda.gov/scripts/opdlisting/ood/>.

⁸ Consolidated Appropriations Act, 2023, Pub. L. No. 117-328; 21st Century Cures Act, Pub. L. No. 114-255.

⁹ Consolidated Appropriations Act, 2023, Pub. L. No. 117-328, § 3208.

¹⁰ 21st Century Cures Act, Pub. L. No. 114-255, § 3013.

¹¹ Consolidated Appropriations Act, 2023, Pub. L. No. 117-328, § 3210.

¹² 21st Century Cures Act, Pub. L. No. 114-255, § 3001-2.

¹³ Consolidated Appropriations Act, 2023, Pub. L. No. 117-328, § 2503.

treatments for rare diseases.¹⁴ For example, one provision in the IRA exempts orphan drugs from government price setting through the Drug Price Negotiation Program, but only if that drug has an orphan designation for one indication. If the drug shows potential in treating another rare disease under the orphan drug designation, and the company pursues that designation, the drug could become subject to price controls. This outcome fails to embrace the potential that further innovation could offer to patients awaiting new cures. This provision discourages companies from researching additional drug indications, such as those for rare diseases and children, due to the uncertainty surrounding price negotiations.¹⁵

Additionally, the IRA also encourages delayed launches for new cures.¹⁶ The timeline for price-setting selection under the law begins on the FDA approval date for a drug, regardless of the incidence of the disease that receives the initial indication. As written, this structure may result in companies delaying product launches until additional, more prevalent disease indications are market ready, so that there is a larger patient population to market treatments to – bypassing the rare disease community.¹⁷ This provision, and others, have the potential to be harmful to patients with rare diseases.

There are other challenges that Congress can address to help further ensure that patients can receive the care that they need. Patients with rare diseases often need to travel to receive treatments for their care. For example, novel cell and gene therapies for rare diseases may only be available at certain centers of excellence across the country, creating financial and logistical barriers for Americans who may live hundreds of miles away from the only hospital that can otherwise treat their rare disease. Similarly for providers, existing Medicaid rules make it difficult for doctors in one state to bill another state's Medicaid program, forcing doctors to spend time filling out paperwork just to treat patients with rare diseases rather than letting them spend those crucial hours actually taking care of their patients.

In this hearing, the Committee will examine every aspect of the rare disease treatment pipeline. This includes assessing the requirements for bringing a treatment or cure to market, in order to ensure that patients can ultimately get the care that they need. This hearing will provide an opportunity to discuss bipartisan solutions to strengthen patient access to care so that future Rare Disease Days can celebrate more cures.

¹⁴ Goldman, Dana, et al. "Mitigating the Inflation Reduction Act's Adverse Impacts on the Prescription Drug Market" *USC Schaeffer Center White Paper*. 2023. <https://healthpolicy.usc.edu/research/mitigating-the-inflation-reduction-acts-potential-adverse-impacts-on-the-prescription-drug-market/>

¹⁵ Chambers, James. "Follow-On Indications for Orphan Drugs Related to the Inflation Reduction Act." 2023. <https://jamanetwork.com/journals/jamanetworkopen/fullarticle/2808362>

¹⁶ Health Affairs Forefront. "How The IRA Could Delay Pharmaceutical Launches, Reduce Indications, And Chill Evidence Generation." 2023. <https://www.healthaffairs.org/content/forefront/ira-could-delay-pharmaceutical-launches-reduce-indications-and-chill-evidence>

¹⁷ Patterson, Julie, et al. "Unintended Consequences of the Inflation Reduction Act: Clinical Development Toward Subsequent Indications." *The American Journal of Managed Care*. 2024. <https://www.ajmc.com/view/unintended-consequences-of-the-inflation-reduction-act-clinical-development-toward-subsequent-indications>

III. Legislation

H.R. 1092, Better Empowerment Now to Enhance Framework and Improve Treatments (BENEFIT) Act (Reps. Matsui and Wenstrup)

This legislation would modify the new drug approval process by requiring the Food and Drug Administration (FDA) to utilize relevant patient-experience data as part of the benefit-risk assessment framework.

H.R. 3433, Give Kids a Chance Act (Reps. McCaul and Eshoo)

This legislation authorizes the FDA to require pediatric cancer trials for new drugs that are used in combination with active ingredients that meet the standard of care for targeting pediatric cancer or have been approved to treat adult cancer and are directed at molecular targets for pediatric cancer.

H.R. 4758, Accelerating Kids Access to Care Act (Reps. Trahan and Miller-Meeks)

This legislation would promote enrollment under the Medicaid program for eligible out-of-State providers. Specifically, the bill would enable providers to enroll in State Medicaid programs, besides their home state's program, without additional screening requirements.

H.R. 5539, Optimizing Research Progress Hope And New (ORPHAN) Cures Act (Reps. Joyce and Nickel)

This legislation would expand and clarify the exclusion for orphan drugs under the Drug Price Negotiation Program, allowing drugs that treat more than one rare disease to maintain their orphan drug status and exemption from price setting through the Drug Price Negotiation Program.

H.R. 5547, Maintaining Investments in New Innovation (MINI) Act (Reps. Nickel and Joyce)

This legislation would modify the criteria for certain single source drugs to qualify for the Drug Price Negotiation Program after being approved for 11 years, instead of 7 years, given their similarity to larger, biological products that are subject to the 11-year timeframe under the IRA.

H.R. 5663, ALS Better Care Act (Reps. Schakowsky, Quigley, and Fitzpatrick)

This legislation would expand Medicare coverage to include relevant services for people diagnosed with ALS. The supplemental facility-based payment system established by this bill would cover outpatient services administered by a qualified provider.

H.R. 6020, Honor Our Living Donors Act (Reps. Obernolte and DelBene)

This legislation would prevent an organ recipient's income from being considered when providing reimbursement for qualifying expenses incurred by a living organ donor during the donation process.

H.R. 6094, Providing Realistic Opportunity To Equal and Comparable Treatment for (PROTECT) Rare Act (Reps. Matsui and Dunn)

This legislation would expand the definition of medically accepted indications in Medicare Part D and Medicaid to include treatments for rare diseases that are supported in peer-reviewed literature and clinical guidelines and do not otherwise have unfavorable reviews in the United States Pharmacopoeia or other similar compendia. Additionally, the bill would require expedited processes for private health insurers to similarly review coverage for such treatments.

H.R. 6465, Preserving Life-saving Access to Specialty Medicines in America (PLASMA) Act (Reps. Hudson and Davis)

This legislation would modify how plasma derived medicinal products are treated in relation to changes to the Part D cost-sharing structure. This change would phase-in the increase in manufacturer rebates over 5 years under the existing pathway for small biotechnology manufacturers.

H.R. 6664, Innovation in Pediatric Drugs Act (Reps. Eshoo and McCaul)

This legislation would remove the existing exemption for orphan drugs to be studied in children before approval and authorize the FDA to penalize companies that have not completed required pediatric studies on time. Additionally, the bill would reauthorize and increases funding for the National Institutes of Health Best Pharmaceuticals for Children's Act (BPCA) program, currently authorized at \$25 million through FY2027, to \$50 million from FY2023 to FY2027.

H.R. 6705, Effective Screening and Testing for Tuberculosis Act (Reps. Moolenaar and Dingell)

This legislation would require the Secretary of Health and Human Services (HHS) to treat certain tests for tuberculosis as breakthrough devices eligible for expedited development and priority review. It would also require certain establishments that perform donor screening or testing to screen or test for active and latent tuberculosis.

H.R. 7188, Shandra Eisenga Human Cell and Tissue Product Safety Act (Reps. Moolenaar and Dingell)

This legislation would require the Secretary of HHS to conduct a national, evidence-based education campaign to increase public and health care provider awareness regarding the potential risks and benefits of human cell and tissue products transplants. The Secretary would also be required to report any actions that could be taken to improve the safety of human cell and tissue products and update existing donor eligibility guidance accordingly. The bill also establishes civil monetary penalties for any person who violates human cell and tissue product requirements established in section 361 of the Public Health Service Act.

H.R. 7248, FDA Modernization Act 3.0 (Reps. Carter and Barragán)

This legislation would establish a process for the qualification of nonclinical testing methods to replace the use of animals in nonclinical research. Specifically, the FDA would prequalify non-animal testing methods before the technologies are integrated into an application.

H.R. 7383, Retaining Access and Restoring Exclusivity (RARE) Act (Reps. Matsui and Bilirakis)

This legislation would clarify the FDA's interpretation of limiting orphan drug exclusivity to the approved indication rather than the potentially broader designation.

H.R. 7384, Creating Hope Reauthorization Act of 2024 (Reps. McCaul and Eshoo)

This legislation would extend the FDA priority review voucher program from FY2024 through FY2028 to incentivize the development of drugs for rare pediatric diseases.

H.R. 7436, Antimicrobial Resistance Research Assessment Act (Rep. Griffith)

This legislation would require the Government Accountability Office (GAO) to report on the efforts of the federal government to address antimicrobial resistance, including the roles of each federal program in these efforts and recommendations to improve coordination.

H.R. ____, Patient Access Act (Rep. Guthrie)

This legislation would prevent antikickback penalties from being applied to certain travel and lodging arrangements, made between a drug manufacturer and an individual who is prescribed such drug, as a means to improve access to treatments that may otherwise require traveling to centers of excellence to receive necessary care.

H.R. ____, Sickle Cell Disease Comprehensive Care Act (Rep. Burgess)

This legislation would allow State Medicaid programs to establish health homes for eligible beneficiaries with sickle cell disease.

IV. Staff Contacts

If you have questions regarding this hearing, please contact Emma Schultheis of the Committee staff at 202-225-3641.