RDLA Glossary of Policy and Advocacy Terms

This glossary is a one-stop resource for rare disease advocates! You will find definitions for commonly used terms, a breakdown of the U.S government structure, relevant U.S government agencies, and non-governmental agencies.

Legislative Terms

- **Act** – A bill that has passed both house of congress and has been enacted into a law.
- **Adoption** – Formally approve of; usually in reference to a change or amendment.
- **Amendment** – A formal change (or proposed change) made to a piece of legislation.
- **Appeal** – Asking a higher court to change or reverse the decision of a lower court.
- **Appropriation** – The allocation of funds for a specific purpose within government. Allows for funds to be spent but is not an actual expenditure.
- **Bill Sponsor** – A Representative or Senator who introduces a bill.
- **Bill Cosponsor** – A Representative or Senator who formally signs on to support a bill. Only the first-named Member is the sponsor, all others are cosponsors, even those whose names appeared on the measure at the time it was submitted.
- **Bicameral bill** – A bill that has been introduced in both the House and Senate.
- **Bipartisan bill** – A bill that has at least one cosponsor from both parties.
- **Cabinet** – A body consisting of the Vice President of the United State and the heads of the executive branch's federal executive departments
- **Congressional Budget Office (CBO)** – Agency within the legislative branch that produces independent analyses of budgetary and economic issues to support the Congressional process. Often calculates the cost or savings from enacting a specific bill. This is referred to as a "score".
- **Committee** – A panel with members from the House or Senate tasked with conducting hearings, examining and developing legislation, and conducting oversight.
    - *The Senate and House have separate versions of each committee, but occasionally a joint committee is made of members from both chambers. The Energy and Commerce Committee, Ways and Means Committee, and Appropriations Committee in the House and Health, Education, Labor and Pensions Committee (HELP), Finance Committee, and Appropriations Committee in the Senate have most of the jurisdiction over healthcare issues*
- **Subcommittee** – A subpanel of a committee with a more specific jurisdiction. For example, the House Energy and Commerce Committee has a Health Subcommittee.

- **Chair** – The member of the majority party on a committee or subcommittee who has formal responsibility over the panel's agenda and resources, presides at its meetings, and can, in some circumstances, act on the committee's behalf.
- **Caucus** – An informal meeting of members of a body of government (typically belonging to the same political party and/or another common interest such as the Rare Disease Caucus).
- **Companion Bill** – A bill introduced in either the House or Senate which has identical or similar language to another bill introduced in the other chamber.
- **Constituent** – Citizens within a district of a legislator; the voters that elect a representative. "Died in committee" – A bill that was considered and rejected by a committee; not returned to the house for action.
- **Enacted** – When a bill is passed by both chambers and signed into law by the President.
- **Filibuster** – A prolonged discussion of a piece of legislation that delays or prevents legislative action.
- **Hearing** – A formal meeting of a congressional committee (or subcommittee) to gather information from witnesses for use in its activities.
- **Joint session** – Meeting of both the House and the Senate in one chamber.
- **Legislator** – An elected official of a legislative body.
- **Legislature** – The branch of government responsible for enacting laws.
- **Lobbyist** – A person who attempts to influence legislation on behalf of a specific interest group.
- **Majority Party** – The political party having electoral strength sufficient to permit it to win control of a government
- **Markup** – Meeting by a committee or subcommittee during which committee members offer, debate, and vote on amendments to a bill or other measure.
- **Minority Party** – A political party whose electoral strength is so small as to prevent its gaining control of a government except in rare and exceptional circumstances
- **Motion** – A proposal asking for the Senate or House to take an action
- **Nonpartisan** – Not associated with a single political party or caucus.
- **Partisan** – Being associated with a single political party or caucus.
- **Passed** – When a bill is approved in one chamber by a majority vote (most legislation requires a 60-vote majority in the Senate).
- **Petition** – A formal written request submitted by anyone other than the legislature (individuals, boards, commissions, cities, etc.).
- **Quorum** – The minimum number of members of the legislature necessary to conduct business.
- **Ranking Member** – The most senior (though not necessarily the longest-serving) member of the minority party on a committee or subcommittee. The ranking member typically oversees minority committee staff and may coordinate involvement of the minority party members in committee activities.

        - Nominated by the president and approved by the senate with at least 51 votes
    - Other Federal Courts

Health-Related Federal Government Agencies

- **Agency for Healthcare Research and Quality (AHRQ):** The Agency for Healthcare Research and Quality (AHRQ) was established to fund and conduct research aimed at improving the quality of healthcare. Additionally, AHRQ is tasked with disseminating this evidence and translating it into clinical practice.
- **Centers for Disease Control and Prevention (CDC):** Tasked with protecting the nation from health, safety and security threats, both foreign and in the U.S. Monitors reported disease and maintains information databases on prevalence, region, etc.
- **Centers for Medicare and Medicaid Services (CMS):** Administers healthcare/ reimbursement programs including Medicare, Medicaid, and the Children's Health Insurance Program (CHIP).
    - **Medicaid:** An entitlement program administered between the federal government and state governments that provides health insurance for adults and children with limited income and resources, pregnant women, and qualifying children with complex medical conditions
    - **Medicare:** a federal system of health insurance for people over 65 years of age or for certain younger people with disabilities
- **Department of Defense (CDMRP):** The Department of Defense hosts the Congressionally Directed Medical Research Programs. CDMRP was created in 1992 via a Congressional appropriation to foster novel approaches to biomedical research in response to the expressed needs of its stakeholders. Stakeholders include the America public, the military and Congress.
- **Department of Education:** The department of the U.S federal government that administers federal programs related to education.
- **Department of Health and Human Services (HHS):** A cabinet-level department of the U.S. federal government with the goal of protecting the health of all Americans and providing essential human Services. This Department includes the below agencies, among others (12 total).
- **Food and Drug Administration (FDA):** Responsible for protecting the public health by ensuring the safety, efficacy, and security of human and veterinary drugs, biological products, and medical devices; and ensuring the safety of our nation's food supply, cosmetics, and products that emit radiation.
    - **CDER:** As part of the FDA, CDER, or Center for Drug Evaluation and Research, regulates over the counter and prescription drugs, including biological therapeutics and generic drugs.
    - **CBER:** As part of the FDA, CBER, or Center for Biologics Evolution and Research, regulated biological products for human use under applicable federal laws.
    - **CDRH:** As part of the FDA, CHRH, or Center for Devices and Radiological Health, is responsible for the premarket approval of all medical devices, as well as overseeing the manufacturing, performance and safety of these devices.

- **OOPD:** As part of the FDA, OOPD, or the Office of Orphan Products Development, evaluates information from product sponsors to determine if drugs, biologics or medical devices meet the criteria for certain incentives and administers grants to provide funding for research on rare diseases. The office also works on rare disease issues with medical and research communities, professional organizations, academia, government agencies, industry and rare disease patient groups.

- **Health Resources and Research Administration (HRSA):** The primary federal agency for improving access to health care services for people who are uninsured, isolated or medically vulnerable. This agency administers several newborn screening programs.
- **National Institutes of Health (NIH):** The nation's medical research agency tasked with making discoveries that improve health and save lives. Comprised of 27 institutes and centers.
  - National Center for Advancing Translations Sciences (NCATS): As part of the NIH, NCATS speeds the development of new rare disease treatments by focusing on scientific approaches that can address more than one disease at a time. NCATS has contributed to 55 Investigational New Drugs and 14 approved therapies.
- **Social Security Administration (SSA):** An independent agency of the U.S. federal government that administers Social Security, a social insurance program consisting of retirement, disability, and survivor benefits.

Non-Government

- **Institute for Clinical and Economic Review (ICER):** The Institute for Clinical and Economic Review (ICER) is an independent, non-partisan organization that has developed a framework for evidenced-based value assessments of new health technologies.
- **Patient-Centered Outcomes Research Institute (PCORI):** The Patient-Centered Outcomes Research Institute (PCORI) is a non-profit, government-sponsored institute. PCORI funds effectiveness research with the goal of improving outcomes by equipping patients and caregivers with the information and data they need to make informed decisions. Funding for this institute has been approved through 2029.

Frequently Referenced Legislation and Policy Terms

- **21st Century Cures Act:** The 21st Century Cures Act was signed into law on December of 2016. The law authorized $6.3 billion in funding, primarily to the National Institutes of Health (NH) and the Food and Drug Administration (FDA). The Cures Act was designed to encourage development of medical products and devices, and to streamline the process of getting them into market.
- **Accelerated Approval:** The Food and Drug Administration (FDA) initiated the Accelerated Approval Program in 1992 to allow faster approval of drugs for serious conditions that fill an unmet medical need. Drugs with accelerated approval can initially be tested in clinical trials that use a surrogate endpoint, or something that is thought to predict clinical benefit. Surrogate

endpoints typically require less time.

- **Accelerating Kids' Access to Care Act:** The Accelerating Kids' Access to Care Act would make it easier for kids with Medicaid to access providers in other state Medicaid programs for children under the age of 21. The law requires that state Medicaid programs establish a federal process permitting out-of-state providers to participate without undergoing additional screening requirements. Participating providers would enroll for 5 years and be continuously renewed if in good standing. This legislation is pending in Congress.
- **Access to Genetic Counselor Services Act:** The Access to Genetic Counselor Services Act would improve Medicare beneficiaries' access to genetic counselors. This legislation would update current Medicare law to provide beneficiaries with direct access to genetic counselors and enables genetic counselors to bill Medicare directly with a reimbursement rate of 85% of physician fee schedule amount. This legislation is pending in Congress.
- **Affordable Care Act:** The Affordable Care Act (ACA or Obamacare) was signed into law in March of 2010. It was designed to extend health coverage to millions of uninsured Americans by legally requiring them to buy health insurance. The Act had three primary goals.
    1. Make affordable health insurance available to more people regardless of their preexisting conditions by providing consumers with subsidies or "premium tax credits" that lower costs for households with incomes between 100% and 400% of the federal poverty level and by establishing marketplaces for people to shop for private health insurance plans that meet minimum coverage standards.
    2. Expand Medicaid to cover all adults with incomes below 138% of the federal poverty level.
    3. Support innovative medical care delivery models and payment policies designed to improve the quality of care and identify ways to lower the cost of health care.
- **Creating Hope Act:** The Creating Hope Act establishes an incentive for drugs to be developed for children with rare diseases. The incentive is a priority review voucher that can be earned by a company that develops a rare pediatric disease drug.
- **Federal Advisory Committee Act (FACA):** The Federal Advisory Committee Act became law in 1972 and is the legal foundation defining how federal advisory committees operate. The law has special emphasis on open meetings, chartering, public involvement, and reporting.
- **Food and Drug Administration Safety and Innovation Act (FDASIA):** The Food and Drug Administration Safety and Innovation Act, signed into law in 2021, expands the FDA's authorities and strengthens the agency's ability to safeguard and advance public health by:
    - Giving the authority to collect user feeds from industry to fund reviews for innovator; drugs, medical devices, generic drugs and biosimilar biological products;
    - Promoting innovation to speed patient access to safe and effective products;
    - Increasing stakeholder involvement in FDA processes; and
    - Enhancing the safety of the drug supply chain.

- **Guidance Document:** Non-binding advice given by an administrative agency such as FDA to the public regarding how best to comply with a law or regulation. Guidance is often used to explain the objective or interpretation of a vague or nonspecific law or requirement.
- **HELP Copays Act:** The HELP Co-Pays Act requires health plans to count the value of co-pay assistance toward patient cost-sharing requirements. The bill also closes a loophole in the Affordable Care Act that allows employer health plans to deem certain categories of prescription drugs as non-essential and thus not count any cost-sharing towards the patient's deductible or out-of-pocket maximum.
- **ICD Code:** The International Classification of Diseases (ICD) is the classification used to code and classify morbidity data from the inpatient and outpatient records, physician offices and mortality records for death certificates.
- **Inflation Reduction Act (IRA):** The Inflation Reduction Act addressed a range of policy issues and was signed into law on August 16, 2022. Parts of the IRA that focus on healthcare primarily affect people on Medicare and those who get their health insurance through the Affordable Care Act marketplace. The law extended the availability of substantial subsidies for individuals to purchase insurance on the marketplace and made several changes to lower the out-of-pocket costs that people on Medicare pay to access their prescription drugs. The IRA also created the Medicare Drug Price Negotiation Program.
- **The Lobbying Disclosure Act (LDA):** The Lobbying Disclosure Act of 1995 is legislation aimed at bringing increased accountability to federal lobbying practices. This law applies to legislative and executive brand contacts. The LDA does not apply to state or local lobbying.
- **Medicare Drug Price Negotiation Programs:** The Inflation Reduction Act passed by Congress and signed into law by President Biden he created the Medicare Drug Price Negotiation Program (MDPNP), administered by the Centers for Medicaid and Medicare Services (CMS), to negotiate the prices of certain prescription drugs as ordered by the Inflation Reduction Act (IRA). Beginning in 2026, 10 drugs covered under Medicare Part D will become eligible for negotiation, and each year, the number of drugs available for negotiation will increase. Drugs covered under Medicare Part B will also become available for negotiation beginning in 2028. The IRA exempted orphan drugs that are approved to treat only one rare disease or condition from being available for negotiation.
- **Medical Device User Fee Amendments (MDUFA):** Device user fees were established in 2002 by the Medical Device User Fee and Modernization Act. Under the user fee system, medical device companies pay fees to the FDA when they register their establishments and list their devices with the agency, whenever they submit an application or a notification to market a new medical device. These fees are used to help the FDA increase the efficiency of regulatory processes with a goal of reducing the time it takes to bring safe and effective medical devices to the market.
- **MVP Act:** The Medicaid VBPs for Patients (MVP) Act would expand access to treatments, such as gene therapy, by establishing a value-based purchasing structure. Affordability would be based on

the treatment's effectiveness, resulting in greater access for patients on Medicaid. This legislation is pending in Congress.

- **Newborn Screening Saves Lives Act:** Congress passed the original Newborn Screening Saves Lives Act in 2008, which established national newborn screening guidelines and helped facilitate comprehensive newborn screening in every state. The Act was first reauthorized in 2014.
- **Orphan Drug Act (ODA):** The Orphan Drug Act is a law that was passed in 1983 to facilitate development of orphan drugs. Orphan drugs are drugs that remain undeveloped or neglected because of limited potential for commercial gain.
- **Orphan Drug Tax Credit:** The Orphan Drug Tax Credit (ODTC), established as part of the ODA, allows companies to claim a tax credit for a portion of their qualified research expenses. Originally set at 50% of qualified expenses, the credit was reduced to 25% in 2017.
- **Patient Experience Data:** Patient experience data is defined in the 21st Century Cures Act as data that is collected with the intention to provide information about patients' experiences with a disease or condition. Patient experience data can be interpreted as information that captures experiences, perspectives, needs, and priorities.
- **Patient Focused Drug Development:** A systematic approach to help ensure that patients' experiences, perspectives, needs, and priorities are captured and meaningfully incorporated into the development and evaluation of medical products throughout the medical product life cycle.
- **Prescription Drug User Fees Amendments (PDUFA):** The Prescription Drug User Fees Act was created by Congress in 1992 and authorizes the FDA to collect feeds from companies that produce certain human drug and biological products. PDUFA must be reauthorized every five years. The last reauthorization, PDUFA VI, was passed in 2017 and included the following improvements:
  - Formalized inclusion of Patient Experience Data into the drug development and review process, including use of patient reported outcomes
  - Use of real-world evidence for regulatory decision-making;
  - Dedicated process to improve use of biomarkers as surrogate endpoints in drug development
- **Priority Review Vouchers:** A priority review vouchers are earned by pharmaceutical companies for the development and approval of drugs treating rare pediatric diseases. The vouchers grant priority review to a drug developer as an incentive to develop treatments for drugs that might otherwise not be profitable to develop because of a smaller pool of patients needing treatment.
- **Regulation:** Federal regulations are specific details directives or requirements with the force of law enacted by the federal agencies necessary to enforce the legislative acts passed by Congress.
- **Safe Step Act:** The Safe Step Act would require group health plans to provide exceptions for medications protocoled by step therapy. Often referred to as "fail first," step therapy is a practice in which health plans order patients to first try medications preferred by their insurance instead of approving the original medication prescribed by the doctor. The Safe Step Act would eliminate the potential harm patients face due to delayed access to treatments. This legislation is pending in Congress.

- **SCHIP:** SCHIP, State Children's Health Insurance Program, is a partnership between the federal government and state governments enacted by Congress in 1997. Children whose family income is above Medicaid eligibility levels may qualify for health care coverage depending on their state's upper income limits for eligibility.