

Rare Disease and wAIHA Advocacy Resource Guide



A concise guide for wAIHA Warriors advocates, volunteers, and leaders

Purpose

Use this guide to understand the federal agencies, laws, funding process, research language, and access-to-care terms most likely to arise in rare disease and wAIHA advocacy.

Scope note: This is an educational resource, not legal or medical advice. Bill status, agency policy, and treatment approvals can change. Time-sensitive items are dated and should be rechecked before public use.

Quick guide: what matters most

- Rare disease policy is shaped through laws, annual appropriations, federal regulations, and agency programs.
- Authorization creates or changes a program; appropriation provides the money.
- Report language can direct federal attention even when the disease is not named in statutory bill text.
- For wAIHA, the highest-value federal targets are NIH/NHLBI research, CDC blood-disorder public health work, DoD medical research, FDA drug development, and policies that reduce barriers to clinical trials and treatment access.

1. wAIHA: essential facts

Topic	Advocacy-ready explanation
wAIHA	Warm autoimmune hemolytic anemia, the most common form of autoimmune hemolytic anemia.
What happens	The immune system produces antibodies that attach to red blood cells at or near normal body temperature, causing the cells to be destroyed too early (hemolysis).
Common effects	Anemia, fatigue, shortness of breath, jaundice, dark urine, rapid heart rate, weakness, and reduced ability to carry out normal activities. Severe disease can be urgent.
Primary or secondary	wAIHA may occur on its own or in association with another autoimmune disease, immune disorder, blood cancer, infection, medication, or other condition.
Diagnosis	Diagnosis generally combines evidence of hemolysis with antibody testing, especially the direct antiglobulin test (DAT), while excluding other causes of anemia and hemolysis.
Treatment status	As of August 3, 2026, no therapy was FDA-approved specifically for wAIHA. IMAAVY (nipocalimab-aahu) was under FDA Priority Review for a proposed wAIHA indication. Current care may use therapies off-label and should be individualized by a hematologist.
Why advocacy is needed	wAIHA has limited disease-specific evidence, no established FDA-approved therapy as of the date above, variable access to expertise, clinical-trial participation barriers, and significant quality-of-life and socioeconomic effects.

Current sources: NIH GARD wAIHA disease page; American Society of Hematology educational materials; FDA and sponsor public information on wAIHA development status.



2. Core federal agencies and programs

Term	What it means
HHS	Department of Health and Human Services - cabinet department overseeing NIH, CDC, FDA, CMS, HRSA, and other federal health agencies.
NIH	National Institutes of Health - the principal federal funder of biomedical research.
NHLBI	National Heart, Lung, and Blood Institute - the NIH institute most directly connected to blood-disorder research, including WAIHA.
NCATS	National Center for Advancing Translational Sciences - helps move discoveries toward tests, treatments, and practical health solutions; also supports rare-disease research infrastructure.
ORDR	Office of Rare Diseases Research - part of NCATS and a coordinating center for NIH rare-disease research activities.
RDCRN	Rare Diseases Clinical Research Network - NIH-supported consortia that advance collaborative natural-history studies, clinical-trial readiness, and rare-disease research.
CDC	Centers for Disease Control and Prevention - supports surveillance, education, public-health data, and prevention programs.
DBDPHG	Division of Blood Disorders and Public Health Genomics - CDC division associated with public-health work involving blood disorders.
FDA	Food and Drug Administration - regulates drugs, biologics, devices, clinical-trial requirements, and approval pathways.
CMS	Centers for Medicare & Medicaid Services - administers Medicare, Medicaid, and major coverage policies.
HRSA	Health Resources and Services Administration - supports access to care, the health workforce, and services for underserved populations.
DoD	Department of Defense - funds congressionally directed medical research, including the Peer-Reviewed Medical Research Program.
PRMRP	Peer-Reviewed Medical Research Program - a DoD program that supports research on conditions designated by Congress.
ARPA-H	Advanced Research Projects Agency for Health - funds high-risk, high-impact health research intended to produce major breakthroughs.
OMB	Office of Management and Budget - oversees the federal budget and reviews major agency policy and regulatory actions.



3. How federal policy and funding move

Term	What it means
Authorization	Creates, renews, or changes a federal program or legal authority. It does not necessarily provide funding.
Appropriation	Provides federal funding for agencies and programs, usually for one fiscal year.
FY	Fiscal year. The federal fiscal year runs October 1 through September 30.
L-HHS	Labor, Health and Human Services, Education, and Related Agencies appropriations bill, which funds NIH, CDC, HRSA, and many other health programs.
CR	Continuing Resolution - temporary funding used when regular appropriations have not been enacted.
Omnibus / Minibus	A package combining all or several annual appropriations bills.
Programmatic request	A request for a specific funding level for an existing federal program.
Report-language request	A request for written congressional direction, recognition, or encouragement concerning an agency, disease, or activity.
Report language	Explanatory or directive language accompanying legislation. It is not the same as statutory law, but agencies generally take it seriously.
Bill text	The legally operative wording of a bill.
Markup	A committee meeting where members debate, amend, and vote on legislation.
Conference	The process of resolving differences between House and Senate versions of legislation.
Policy rider	A policy direction, limitation, or prohibition attached to a spending measure.
Rescission	Cancellation of previously approved federal funding.

A practical distinction

Ask	What it accomplishes
"Fund NIH at \$X billion."	A programmatic funding request.
"Include acquired hemolytic anemias in PRMRP."	A disease-eligibility or program-direction request.
"Encourage NIH to expand the wAIHA research portfolio."	A report-language request.
"Cosponsor H.R. ____."	Support for a specific authorization or policy bill.

4. Important rare disease laws and current legislation

Law or bill	Why advocates may hear about it
Orphan Drug Act (P.L. 97-414)	Created federal incentives for development of products for rare diseases, including orphan designation and potential market exclusivity.
Rare Diseases Act of 2002 (P.L. 107-280)	Strengthened NIH rare-disease coordination and established the statutory U.S. definition of a rare disease as affecting fewer than 200,000 people.
Rare Diseases Orphan Product Development Act of 2002 (P.L. 107-281)	Authorized support for clinical research on drugs, biologics, devices, and medical foods for rare diseases.
21st Century Cures Act (P.L. 114-255)	Advanced biomedical innovation, patient-focused drug development, data sharing, research modernization, and selected multi-year research initiatives.
Clinical Trial Modernization Act (H.R. 3521 / S. 4440)	A bipartisan 119th Congress proposal intended to reduce financial and geographic barriers to clinical-trial participation, including support for travel, lodging, meals, dependent care, and technology. As of August 3, 2026, it had been introduced but not enacted.
Accelerating Kids' Access to Care Act	Designed to streamline Medicaid screening and enrollment for eligible out-of-state pediatric providers so children with complex needs can receive specialized care across state lines.
Give Kids a Chance Act	Pediatric cancer legislation intended to strengthen requirements for scientifically appropriate studies of combinations of targeted therapies in children.

Current-bill caution: Congress may amend, combine, advance, or replace bills. Confirm status on GovInfo or Congress.gov before using a bill number publicly.

5. Research and clinical-trial language

Term	What it means
NOFO	Notice of Funding Opportunity - an official announcement inviting applications for federal funding.
RFA	Request for Applications - a focused funding announcement, often tied to a defined research priority and dedicated funds.
RFI	Request for Information - an agency request for public input before or during policy development.
Natural-history study	Research describing how a disease changes over time without assigning an experimental treatment.
Registry	An organized system for collecting standardized information about people with a disease or condition.
Clinical-trial readiness	The data, endpoints, biomarkers, patient identification, sites, and infrastructure needed to conduct a useful trial.
Endpoint	A defined outcome used to judge whether a treatment is effective or safe.
Biomarker	A measurable biological characteristic associated with disease activity, risk, or treatment response.
PRO	Patient-reported outcome - information reported directly by patients about symptoms, function, treatment effects, or quality of life.
Patient-focused drug development	FDA and research approaches that systematically incorporate patient experiences, priorities, and preferences.
Phase 1 / 2 / 3	Clinical-trial stages that generally progress from early safety and dosing to evidence of effectiveness and broader confirmation.
Randomized / placebo-controlled	Methods used to compare interventions and reduce bias. Not every rare-disease trial uses both.
Informed consent	The process of explaining a study's purpose, procedures, risks, benefits, alternatives, and participant rights before enrollment.

Expanded access	A pathway that may allow use of an investigational product outside a clinical trial for a serious case.
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6. Coverage and patient-access terms



Term	What it means
Prior authorization	Insurer approval required before a treatment, service, or medication will be covered.
Step therapy / fail first	A requirement to try one or more preferred treatments before another treatment will be covered.
Medical necessity	The clinical standard an insurer uses to determine whether care qualifies for coverage.
Formulary	A health plan's list of covered medications.
Tiering	Placement of medications into different cost-sharing levels.
Utilization management	Coverage-control tools such as prior authorization, step therapy, quantity limits, and documentation requirements.
Appeal	A formal request for reconsideration of a coverage denial.
External review	Independent review of an insurer's adverse coverage decision.
Nonmedical switching	Changing a stable patient's medication for cost or coverage reasons rather than a clinical reason.
Accumulator adjustment	A policy under which manufacturer assistance may not count toward a patient's deductible or out-of-pocket maximum.
Essential Health Benefits	Ten broad categories of care that certain health plans must cover under federal law.
Medical frailty	A designation used in some Medicaid policy to identify people with serious or complex health needs who may qualify for exceptions or protections.

7. Advocacy language and Hill meetings

Term	What it means
Cosponsor	A member of Congress who formally supports a bill after introduction.
Companion bill	A substantially similar bill introduced in the other chamber.
Bipartisan	Supported by members of both major political parties.
Sign-on letter	A letter circulated for multiple members of Congress to sign in support of a funding or policy request.
Dear Colleague letter	A communication from members of Congress asking colleagues to support a bill, letter, funding request, or policy position.
Legislative assistant (LA)	Congressional staff member who handles specific policy issues, such as health.
District / state office	A member's local office, often focused on constituent services and local relationships.
Washington office	The office primarily responsible for legislative and committee work.
Leave-behind	A concise handout summarizing the issue, the request, and contact information.
The ask	The specific action requested from the legislator or staff member.
Follow-up	A brief post-meeting message providing promised information and restating the request.



A strong advocacy message in four parts

- Who we are: wAIHA Warriors is a patient and care-partner organization serving people affected by warm autoimmune hemolytic anemia.
- The problem: wAIHA is rare, serious, under-recognized, and supported by limited disease-specific research and public-health infrastructure.
- The human impact: briefly explain how delayed diagnosis, relapse, fatigue, treatment burden, travel, insurance barriers, or lack of specialists affects real people.
- The request: ask for one clear action, such as funding, report language, program inclusion, or cosponsorship.

8. What wAIHA Warriors should monitor

Area	What to watch
Annual appropriations	NIH, NHLBI, NCATS, CDC, blood-disorder public health programs, ARPA-H, VA research, and DoD medical research.
PRMRP eligibility	Whether acquired hemolytic anemias are included or otherwise eligible for DoD peer-reviewed medical research funding.
FDA activity	New wAIHA applications, priority reviews, approvals, safety communications, and patient-focused drug-development opportunities.
Clinical trials	Recruitment status, site availability, inclusion criteria, results, and practical participation barriers.
Coverage policy	Prior authorization, step therapy, Medicaid and Medicare policy, essential benefits, and patient cost-sharing.
Federal rulemaking	Proposed rules, RFIs, and public-comment opportunities affecting grants, health coverage, clinical trials, or patient access.
Selected authoritative sources:	

[NIH Genetic and Rare Diseases Information Center \(GARD\): rare disease definition and wAIHA overview](#)

[NIH GARD: Autoimmune hemolytic anemia, warm type](#)

[American Society of Hematology: anemia and immune-mediated hemolytic anemia education](#)

[GovInfo: Clinical Trial Modernization Act, H.R. 3521](#)

[FDA: information on rare-disease product development and approved products](#)

[ClinicalTrials.gov: current wAIHA study information](#)

[Federal Register: official proposed and final federal rules and notices](#)

*Concept and direction of this document by WAIHA Warriors; developed with ChatGPT assistance. Medical information is for advocacy education only and should not replace individualized medical advice.