Improving Care for Veterans with Rare Diseases: A Blueprint for Action

January 2023

PALs
UNITED
Patient Advocacy Leaders
United for Veterans' Health

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Introduction

This ***Blueprint for Action*** was developed through the ***PALs United for Veterans' Health initiative***, which is a multi-year, collaborative effort of the ***Center for Patient Advocacy Leaders*** **(CPALs)**.[1]

Over the past 2 years CPALs hosted dialogues among Veteran advocates, rare disease advocates, and subject matter experts to better understand the needs of Veterans with rare diseases and promote solutions to improve their care and quality of life via advocacy for research, awareness, education, prompt diagnosis, and appropriate, affordable, patient-centered, equitable treatment and services.

We have identified **three major unmet needs of Veterans with rare diseases** who receive care from Veterans' Health Administration (VHA) integrated delivery networks (HPIDNs), and the supplemental care provided by DoD, Federally Qualified Health Centers (FQHCs), the Indian Health Service (IHS), other Federal partners, teaching hospitals, and Community Care Network (CCN) providers across the country:

1. **Timely diagnosis, treatment, and supportive care, including in community-based settings and rural areas.**
2. **Accelerated access to treatments and supportive therapies.**
3. **Improved rare disease awareness, education, and advocacy.**

We are encouraged by the commitment to improving care reflected in the recent VA Recommendations to the Asset and Infrastructure Review (AIR) Commission …" that will:

- Cement VA as the primary, world-class provider, integrator, and coordinator of Veterans' health care for generations to come;
- Build a health care network with the right facilities, in the right places, to provide the right care for Veterans in every part of the country;
- Ensure that the infrastructure that makes up the VA in the decades ahead reflects the needs of 21st century Veterans—not the needs and challenges of a health care system that was built, in many cases, 80 years ago; and
- Strengthen VA's dual roles as the leading health care researchers in America, and the leading health care training institution in America."[2]

We believe our findings from our discussions with Veteran and rare disease advocates are aligned with these recommendations and the commitment and competence of those we have been working with on this initiative can help drive both the Report to the Commission and the goals we have outlined in this Blueprint.

Progress on each of the goals outlined in the Report to the Commission (AIR Report) and those in this Blueprint will help <u>all</u> Veterans, including those with rare diseases receive the care they deserve. The AIR Report and the Blueprint offer opportunities for collaboration with the VHA to train and educate providers and staff about rare

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[1] CPALs was established in 2019 to expand a long-standing program, the *Patient Advocacy Leaders Summit* (PALS), which was created in 2002. PALS brought together diverse patient advocacy leaders to improve their advocacy capacity and to identify ways to collaborate and unify the voices of many around common health policy issues.
[2] VA Recommendations to the Asset and Infrastructure Review Commission, March 2022. https://www.va.gov/AIRCOMMISSIONREPORT/docs/VA-Report-to-AIR-Commission-Volume-I.pdf.

diseases, to conduct research on rare diseases, and to assure that during national emergencies, any additional needs of Veterans with rare diseases are met.

CPALs' national effort focuses on all aspects of healthcare for Veterans with rare diseases, working with them to promote access to improved care via advocacy for research, awareness, education, prompt diagnosis, and appropriate, affordable patient-centered, equitable treatment and services.

Now is the time to ensure that the available tools — information about rare diseases, diagnostics, therapeutics, and other supportive programs — are available and accessible to end the devastating impact of rare diseases on those who have served the nation in the military, their families, and their communities.

Who We Are: The Center for Patient Advocacy Leaders (CPALs)

The *Center for Patient Advocacy Leaders* (CPALs)[3] was established in 2019 to expand a long-standing program, the Patient Advocacy Leaders' Summit (PALS), which was created in 2002. PALS brought together diverse patient advocacy leaders to improve their advocacy capacity and to identify ways to collaborate and unify the voices of many around common health policy issues.

CPALs programs are guided by Advocate Advisors, a pool of independent leaders from diverse health advocacy organizations. Staff, working along with Advocate Advisors, seek to ensure that CPALs processes and programs reflect our values: *Patient and Family Focus, Trust and Transparency, Diversity, Inclusion, Respect, Equity* and *Reflective Accountability.*

Programs are free of charge and delivered virtually or through in-person events, with a common purpose to help advocates develop policy and advocacy strategies, built on the voice of the patient, addressing chronic illness and complex health conditions. Our advocate-centered model, *"By Advocates, For Advocates,"* focuses on the needs of patients and caregivers, patient advocacy leaders, their organizations, and the diverse communities they serve.

What We Propose: Our Blueprint for Action

Our goals are achievable. We are optimistic about the future of innovation, given that in the past several decades, we learned much about rare diseases in the civilian population. Genomics created new opportunities to make more rapid, accurate diagnoses, to make sooner, more appropriate referrals to specialists, and to better target treatments. The Food and Drug Administration (FDA) has approved 600 medicines for rare diseases, and nearly 800 are currently in development by more than 200 companies. Ultimately, these innovations will save Veterans and their families from what is a long, frustrating, costly odyssey through the VA healthcare maze.

Our *Blueprint* builds on previous work through the *PALs United for Veterans' Health initiative* and capitalizes on a group of rare disease and Veterans' health experts to take the next steps to improve rare disease diagnosis and care for Veterans, their families, employers, and communities in which they live and work.

We are concerned about the barriers Veterans with rare diseases encounter when they seek care. We know the challenges that lie ahead. Working alongside Veterans and rare disease advocates, we are confident, however, that we can develop effective solutions.

To accelerate progress in the diagnosis and care of the 900,000 Veterans we estimate to have a rare disease, 450,000 of whom we estimate receive care in the VHA, CPALs is working with Veteran and rare disease advocates to drive and deliver on the goals identified below:

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[3] https://www.centerforpatientadvocacyleaders.org/

GOAL 1: Timely diagnosis, treatment and supportive care, including in community-based settings and rural areas

Timely Diagnosis

- **As the largest integrated health system in the nation, VHA should leverage EHR data, clinical expertise, and health risk assessments, to develop and implement protocols to expedite the diagnosis of rare diseases, particularly those which are more common to Veterans.**
- **VHA should conduct a study of Veterans diagnosed with rare diseases to explore their diagnostic journey, identify any barriers to timely diagnosis, and recommend process improvements for the benefit of Veterans who are not yet diagnosed.**
- **VHA should provide access to appropriate genetic testing and counseling when a rare disease is suspected.**
- **VHA should replicate the NIH Undiagnosed Network to address the challenges of rare disease diagnoses in Veterans.**

Access to Specialists

- **VHA should routinely support access to specialists within local communities and medical centers and should continue to maintain its provider search site (https://www.va.gov/health/ourdoctors.asp) along with the DocInfo services of the Federation of State Medical Boards (https://www.docinfo.org/), and provide Veterans access to those clinical resources regardless of their location.**
- **VHA should make widespread, easy access to telemedicine, provide parity payments for telemedicine visits, and allow low-tech options (e.g., by telephone for those who do not have access to high-speed internet or smartphones) a matter of permanent policy.**

Comprehensive Care

- **VHA should ensure that care for Veterans integrates the medical, behavioral health, and social service needs in a Veteran-centric team approach.**
- **VHA should search its electronic medical records to identify Veterans who have been diagnosed with rare diseases and should assess whether they have unmet needs.**
- **VHA should ensure that professional VHA Patient Advocates have been trained on rare diseases and the challenges patients have in securing a timely diagnosis and comprehensive care. They should be armed with information about how to reach out to rare disease groups to secure information about referral networks and should help Veterans and their caregivers who want to connect with those groups.**

GOAL 2: Accelerated access to treatments and supportive therapies and services

Quality Care

- **VHA should reach out to those already diagnosed to identify any unmet needs.**
- **VHA should establish a registry to track Veterans with rare diseases and their diagnostic and treatment experiences to identify areas of improvement.**

New Therapeutics

- **VHA should support the passage of the STAT Act.**
- **Empower patients to develop an impactful voice in policymaking, drug development, and regulatory decision-making.**

*Access to Approved Therapeutics*

- **VHA should remove all step-therapy, co-pay, and cost-sharing barriers for Veterans with rare diseases by providing first-dollar coverage and full reimbursement for any FDA-approved therapeutic or medication agreed upon by shared decision making between provider and patient.**

GOAL 3: Improve rare disease awareness, education, information, and advocacy

*Greater Visibility*

- **Veterans and rare disease organizations should collaborate to develop a repository of personal stories of Veterans with rare diseases, their caregivers, and their clinicians.**
- **Veterans and rare disease organizations should conduct awareness and education programs to inform the general public about rare diseases in Veterans and the impact on them, their families, and their employers, especially in communities with military installations and in the top five most populous States for Veterans: TX, FL, CA, PA, and VA.**

*Information*

- **VHA should develop a centralized, easily searchable resource with information about rare disease diagnosis, care, and benefits for Veterans and caregivers.**
- **Rare Disease Advocacy groups should develop health-literate information and submit those resources to the VA's National Resource Directory: https://www.nrd.gov/.**
- **Rare Disease Advocacy groups that may have Veterans within their patient population should highlight their support of Veterans on their web platforms and in their programs.**
- **Veteran and rare disease stakeholders should meet regularly to learn from one another and collaborate on finding solutions to common problems, e.g., at quarterly meetings of the Veterans' Health Council.**
- **Rare disease and Veterans' groups should jointly connect with advocacy organizations and professional societies that may have an interest in rare diseases to educate members and raise awareness, e.g., American Thoracic Society's Public Advisory Roundtable; Consortium for Citizens with Disabilities; Medical associations, including those that focus on underserved populations; AARP; and American Diabetes Association.**
- **VHA should develop a platform for Veteran-to-Veteran connections, assuring Veterans with rare diseases who want to connect with other Veterans are able to do that.**

Why We Are Committed: About Veterans

Veterans serve the nation in many ways, including in some ways that place their lives on the line. Despite that, the US has a mixed record of treating their needs, including their health needs.

It was not until 40 years after the end of the American Revolutionary War that Veterans received a pension, and not until after the Civil War that Veterans received ongoing support.[4] World War I Veterans were promised a bonus to compensate for low military pay, but Congress delayed the payment. In response, in 1932, unemployed Veterans marched on Washington, D.C., to demand it. They were driven out of the city by Army troops commanded by General Douglas MacArthur, with help from Major Dwight D Eisenhower and Major George Patton.[5] WWI Veterans received the promised payment in 1945, 27 years after the war ended. Eventually, and not without a fight, Congress passed the *GI Bill of Rights* in 1944 for WWII Veterans. Vietnam-era Veterans fought for 20 years to get Agent Orange and Post-Traumatic Stress Disorder (PTSD) benefits. Burn pits to dispose of

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[4] U.S. Department of Veterans Affairs, "History – Department of Veterans Affairs (VA)," available at https://www.va.gov/about_va/vahistory.asp.

[5] Bonus Expeditionary Forces March on Washington. http://www.nps.gov/articles/bonus-expeditionary-forces-march-on-washington.htm

waste began in 2001, mostly in Afghanistan and Iraq, and by 2007, the suspected health impact led the DAV to pilot a Burn Pit Registry. It would be another 15 years before the PACT Act would pass to expand care for those impacted by toxic exposures, but as in times past, not without a fight.

The benefits Veterans receive are administered by the Veterans' Administration (VA), which is a Cabinet-level department with an FY 2022 budget of $336 billion. It is responsible for the Veterans Health Administration (VHA), which provides healthcare and conducts biomedical and health services research. The VHA has an FY 2022 budget of $128 billion.[6] It is the largest integrated healthcare system in the nation, with 1,296 facilities, including 171 medical centers, 1,113 outpatient sites, and 1,200 healthcare facilities. Every major medical center employs a professionally trained advocate. It is also the largest provider of graduate medical education in the US and a major contributor to biomedical research, with more than 25,000 active volunteers, 113,000 health professions trainees, and 16,000 affiliated medical faculty.

VA healthcare professionals can operate in every State, and a recent initiative to establish *National Standards of Practice* is underway to standardize the practices of each healthcare occupation, protect healthcare professionals from adverse State actions, and ensure standardized electronic health records. The Veterans Choice Act of 2014[7] and the VA Mission Act of 2018[8], which superseded it, made it easier for eligible Veterans to seek healthcare options outside of the VHA because of concerns that the VHA was in a state of crisis. However, assessments by respected organizations have shown that on nearly every measure, VHA healthcare is better and more effective.[9] Wait times are shorter than in the private sector for both primary and specialty care, care quality is comparable, and behavioral health care was actually superior.[10]

The recent passage of the PACT Act is the largest healthcare benefit expansion in US history. It expands VA benefits for Veterans exposed to burn pits, Agent Orange, and other toxic substances and adds to the list of health conditions that are assumed or "presumed" to be caused by exposure to those substances, bringing the total number of presumed conditions to 103.

The number of Veterans has declined as older Veterans from WWII, Korea, and Viet Nam have passed away, but the number of Veterans receiving care has increased because of Agent Orange coverage and longer lifespans common to all Americans. Their health conditions will become increasingly complicated in the future and because of the risks faced by the military in current wars:

- They are the first since World War II where multiple deployments are likely, increasing their risk of PTSD by 50 percent and resulting in 45,000 suicides among Veterans and active-duty military[11], and at much higher rates than earlier conflicts.[12]

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[6] U.S. Department of Veterans Affairs Office of Budget

[7] U.S. Department of Veterans Affairs, "Summary: Veterans Access, Choice and Accountability Act of 2014 ('Choice Act')" (Washington: 2014), available at https://www.va.gov/opa/choiceact/documents/choice-act-summary.pdf.

[8] U.S. Department of Veterans Affairs, "VA strengthens Caregiver Support Program and expands timeline of the Program of Comprehensive Assistance for Family Caregivers," Press release, September 25, 2019, available at https://www.va.gov/opa/pressrel/pressrelease.cfm?id=5319 .

[9] Those organizations include Grant Thornton, McKinsey & Company, Mitre Corporation and Rand. The Rand study can be accessed here: Carrie M. Farmer and Terri Tanielian, "Ensuring Access to Timely, High-Quality Health Care for Veterans" (Santa Monica, CA: RAND Corporation, 2019), available at https://www.rand.org/content/dam/rand/pubs/testimonies/CT500/CT508/RAND_CT508.pdf

[10] 91 percent of Veterans who use the VA would recommend it to others.

[11] Joseph R. Hoops, "The Effects of Multiple Combat-Related Military Deployments on Post Traumatic Stress Symptoms" (St. Paul, MN: St. Catherine University and University of St. Thomas, 2012), available at https://sophia.stkate.edu/cgi/viewcontent.cgi?article=1039&context=msw_papers ; Carol Giacomo, "Suicide Has Been Deadlier Than Combat for the Military," *The New York Times*, November 1, 2019, available at https://www.nytimes.com/2019/11/01/opinion/militarysuicides.html

[12] Matthew Tull, "The Rates of PTSD in Military Veterans," Very well Mind, November 15, 2019, available at https://www.verywellmind.com/rates-of-ptsd-in-Veterans-2797430

- They benefit from advances in medical care, and those advances have dramatically increased the chances of military personnel surviving combat wounds.[13]
- They benefit from advances in medical care, and those advances have dramatically increased the chances of military personnel surviving combat wounds.[14]
- They are the first in which women (2 million service members; 17.5 percent of the military) have experienced combat and, in addition to those risks, have experienced sexual trauma.[15]
- They are the first in which the Army and Marines deployed whole units, not individuals. The practical result is that once a unit receives notice of a coming deployment, no member can leave active duty until after their unit has returned from the deployment, which often lasts a year, thus extending service beyond initial commitments.

> ***"Today we have a huge opportunity to do the right thing, to provide care for our Veterans…No matter where one stands on the war, every American wants to make sure that our Veterans get the care they deserve."***
>
> ***—Senator Patty Murray (D-WA)***

Providing healthcare services for Veterans is challenging because the VHA is hampered by several factors:

- Regional variations and shifts in demographics mean that many VA facilities are not placed to serve Veterans near where they live, nor is the VHA prepared for the shift to outpatient and telehealth care which has become more common and desirable.
- The VHA lacked leadership between 2017-2022, until the appointment of Shereef Einahal, MD.[16]
- Staff vacancies, which in 2022 numbered 50,000 total, including 2,000 physicians and 15,000 nurses, a number that has increased over time.[17]
- An aging infrastructure. The median age of VHA facilities is nearly 60 years old compared to 8.5 years in the private sector, and 69% of hospitals are over 50 years old. Maintaining the safety of those facilities costs funds that are better put to providing care.
- Confusing and unclear eligibility guidelines.
- A health records system that lags DOD systems, preventing an easy transition for Veterans as they move from active duty to the VA.[18]
- Inadequate resources to provide for the needs of two million female Veterans.
- Lack of long-term health benefits support for Reserve & National Guard units, which are deployed almost as often as active-duty forces, and who, when they return to civilian life, may lose military health benefits.

> ***"If we don't do the right thing, and young people lose confidence in their military and confidence in their government, then this will not be the country that was founded on the principles that you and I believe in. So, when people ask me, 'Do you believe Veterans are entitled to preferential treatment?' 'You bet your life I do.' They shouldn't be in our streets, homeless, jobless, and hopeless. They shouldn't have to ask for healthcare. We should be right there the same way we showed them how to get to the firing line. We should be right there to say thank you."***
>
> ***—Congressman Charles Rangel (D-NY, Retired)***

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[13] From 2.6-to-1 in Vietnam and 2-to-1in WWII to 15-to-1 today. Matthew S. Goldberg, "Updated Death and Injury Rates of U.S. Military Personnel During the Conflicts in Iraq and Afghanistan: Working Pater 2014-08" (Washington: Congressional Budget Office, 2014), available at https://www.cbo.gov/publication/49837.

[14] From 2.6-to-1 in Vietnam and 2-to-1in WWII to 15-to-1 today. Matthew S. Goldberg, "Updated Death and Injury Rates of U.S. Military Personnel During the Conflicts in Iraq and Afghanistan: Working Pater 2014-08" (Washington: Congressional Budget Office, 2014), available at https://www.cbo.gov/publication/49837.

[15] 15 percent of the women serving in Iraq and Afghanistan experienced sexual trauma during deployments. U.S. Department of Defense, "Department of Defense Annual Report on Sexual Assault in the Military: Fiscal Year

[16] https://www.militarytimes.com/Veterans/2022/03/09/white-house-names-picks-to-lead-va-health-and-benefits-efforts/.

[17] https://www.militarytimes.com/Veterans/2022/05/02/va-looks-to-fill-key-medical-posts-starting-with-the-departments-top-health-care-job/.

[18] A ten-year, $16 billion dollar overhaul of the records system is currently underway and is a priority of the current VA Secretary McDonough. https://www.militarytimes.com/video/2022/11/21/which-issue-has-the-va-secretary-pissed-off/

Why We Are Committed: About Rare Diseases

Approximately 30 million Americans have one of the 7,000 known rare diseases. The Food and Drug Administration (FDA) has approved 600 medicines for rare diseases, and nearly 800 are currently in development by 200+ companies, many of them new. Unfortunately, much work remains because there are no evidenced-based cures for rare disease, and more than 90 percent have no approved treatments at all.

The known barriers to care in the civilian sector encounter include:

- Obtaining a diagnosis. On average, it takes 7.6 years, during which time patients see an average of 7.3 physicians. During that time, 40% of patients are misdiagnosed more than once.
- Financial and logistical barriers to seeking care at major medical centers, especially when specialists are located in distant states.
- The failure of Medicaid Drug Utilization Review (DUR), Pharmacy and Therapeutics (P&T), and value assessments by some health economics groups to include rare disease experts.
- The ability of ERISA and self-funded plans to identify employees and dependents and exclude treatments for specific diseases.
- Value-based/alternative payment arrangements that do not account for the intensity of the multi-specialty care required for many rare diseases.
- Affordable genetic testing, diagnostic, and counseling services.
- Limited, if any, accelerated approval pathways to address rare disease is in place.

A *National Economic Burden of Rare Disease Study* (2019)[19] found the total economic burden of only 379 rare diseases to be nearly $1 trillion, including direct medical costs of $418 billion, non-medical and uncovered healthcare costs of$111 billion, and productivity losses of $437 billion.

Owing to the small number of people impacted by any one rare disease, research is challenging. As a result, for many rare diseases, we have a very limited understanding of their causes, pathophysiology, epidemiology, and natural course. Classification systems like ICD codes are inadequate, and expertise among clinicians to diagnose and treat is limited.[20]

Rare disease research, including research for those associated with military service, is being conducted by numerous organizations, including via Congressionally Directed Medical Research Programs, the National Center for Advancing Translational Sciences, the Rare Diseases Clinical Research Network, and the Food and Drug Administration.

The Burden of Rare Disease

Veterans face other issues that make a rare disease diagnosis and treatment more challenging: PTSD, TBI, substance abuse, and polytrauma, which may result in suicidal ideation and mental health problems in as many as 26% of returning troops. Often, barriers to getting the right care at the right time are time-consuming for Veterans and their families. The system is often plagued with clinicians overloaded with cases, workforce shortages issues and not enough clinical understanding of rare disease, which can lead to diminished access for Veterans to clinically indicated, guideline-appropriate care. Finally, rare diseases cause harm to some Veterans who will never regain the function they lost while waiting for approval or while trying and failing on a medication that does not adequately treat their condition and/or used off label in an attempt to treat the disease. Some patients find medications to be miracles, but they still face daily struggles. Side effects, the financial burden, and long-term disruption of family and work lives make it difficult for patients to complete a full course of treatment. Management of rare diseases must include helping the patient and their family through integrated, holistic ways. Until new medicines offer more rapid healing with more tolerable side effects, there will always be a need for addressing and comprehensively supporting patients, with attention to the quality of their well-being, ongoing risk behaviors, and individual factors of race, gender, and ethnicity.

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[19] Yang, G., Cintina, I., Pariser, A. *et al.* The national economic burden of rare disease in the United States in 2019. *Orphanet J Rare Dis* **17**, 163 (2022). https://doi.org/10.1186/s13023-022-02299-5.

[20] Clinicians admit to facing challenges in diagnosing and treating rare disease; 70% want additional training.

We Are Not Alone: Other Efforts Underway

Ours is not the only initiative focused on improving the health of Veterans with rare diseases. We plan to continue our collaboration with those who we have brought together, arriving at this point in time, to use this Blueprint for Action. Together we can develop a common agenda and education and policy solutions to improve accessibility to patient-centered treatment and services to address improving the health and well-being of Veterans with rare diseases. In addition, there are others doing fine work to help our Veterans with rare disease:

Rare Disease Congressional Caucus

The *Rare Disease Congressional Caucus* is comprised of 27 senators and 153 representatives and cochaired by Senators Roger Wicker (R-MS) and Amy Klobuchar (D-MN), and Representatives G.K. Butterfield (D-NC), and Gus Bilirakis (R-FL). The Caucus introduced the Speeding Therapy Access today Act (STAT Act) to improve development and access to therapies for rare diseases by making policy reforms at the FDA and creating a *Rare Disease Center of Excellence.*

Alliance for Patient Access, Rare Diseases Working Group

The *Alliance for Patient Access* (AfPA) is a national network of policy-minded healthcare providers who advocate for patient-centered care. AfPA supports health policies that reinforce clinical decision-making, promote personalized care, and protect the physician-patient relationship. The AfPA *Rare Disease Working Group* has mounted an Amyloidosis Initiative focused on the specific needs of the amyloidosis patient community, including Veterans, and the care they receive at the VA.

EveryLife Foundation for Rare Diseases

The *EveryLife Foundation for Rare Diseases* (EveryLife) is a 501(c)(3) nonprofit, nonpartisan organization dedicated to empowering the rare disease patient community to advocate for impactful, science-driven legislation and policy that advances the equitable development of and access to lifesaving diagnoses, treatments, and cures.

National Organization for Rare Disorders

The *National Organization for Rare Disorders* (NORD) is a patient advocacy organization dedicated to serving people with rare disorders, with a membership composed of more than 300 patient organizations. Their programs include education, advocacy, research, and patient services.

Congressionally-Directed Medical Research Program

Between FY 92-22, $19.4 billion has been appropriated by Congress to this Department of Defense program for research on Alcohol and Substance Abuse, Amyotrophic Lateral Sclerosis, Autism, Bone Marrow Failure, Breast Cancer, Chronic Myelogenous Leukemia, Chronic Pain Management, Women’s Health and many other diseases.

National Center for Advancing Translational Science

This is one of the 27 NIH Centers, with an FY22 budget of $879 million. Its *Clinical Trial Readiness for Rare Diseases, Disorders, and Syndromes (CTR) Program* includes activities like repurposing off-patent drugs and finding new therapeutic uses for existing drugs to expedite the time from discovery to approval. Its Council Members include several experts in rare diseases. Its *Rare Diseases Clinical Research Network* (RDCRN) facilitates collaboration, study enrollment and data sharing. Its *Genetic and Rare Diseases (GARD) Information Center* free access to information in English and Spanish for people with rare diseases and their families. Its *Therapeutics for Rare and Neglected Diseases* (TRND) supports pre-clinical development of treatment candidates for those diseases.

FDA Office of Orphan Products Development

This office, which received funding in FY 22 supports the development of new treatments for rare diseases. It provides orphan status to qualifying drugs, biologics, and medical devices, and works with the Office of Pediatric Therapeutics to determine rare disease pediatric disease designations for qualifying drugs and biologics, awards grants for clinical trials, natural history of disease studies and non-profit research consortia.

*****We Need Your Help*****

Veterans, caregivers, and families are your neighbors, friends, co-workers, employees, and constituents.

As a leader in state or national government, in research, in public health, in clinical care, in patient advocacy, in business, or in education, we ask that you JOIN US to support the goals we have described to improve the care for Veterans with rare diseases. As we work collaboratively to achieve these goals, we will share the results of with you. Rare disease is devastating for every Veteran and family member who is affected by one. We hope that as more people learn and understand the impact of rare diseases on the lives of so many men and woman, they will join us and accelerate progress toward all our goals.

*Questions or comments related to this report can be directed to **Anne Easter** at the **Center for Patient Advocacy Leaders** (**CPALs**),* ***aeaster@taimail.org.***

Appendices

A. Resources

- Alliance for Patient Access (AfPA) Rare Diseases Working Group
- American Association of Kidney Patients (AAKP) Veterans Health Initiative
- American Legion
- American Vets (AMVETS) Advocacy
- Amyloidosis Support Groups
- Caregiver Action Network (CAN)
- Center for Patient Advocacy Leaders (CPALs) – PALs United for Veterans Resources
- Congressionally Directed Medical Research Programs
- Disabled Veterans (DAV) National Legislative Department
- Elizabeth Dole Foundation
- EveryLife Foundation for Rare Diseases
- EveryLife Foundation for Rare Diseases Advocacy Tools
- FDA Office of Orphan Products Development
- Global Liver Institute Pediatric & Rare Livers Diseases News
- "ICER Partners with VA Office for Drug Coverage, Price Negotiations"
- Journal of the American Board of Family Medicine "Health of Veteran's Strengths & Challenges"
- National Center for Advancing Translational Science
    - Clinical Trial Readiness for Rare Diseases, Disorders & Syndromes
    - Rare Diseases Clinical Research Network (RDCRN)
    - Genetic and Rare Diseases (GARD) Information Center
    - Therapeutics for Rare and Neglected Diseases (TRND)
- National Organization for Rare Diseases (NORD)
- Paralyzed Veterans of America (PVA)
    - Veterans Services
    - Advocacy & Policy Priorities
- Partnership to Fight Chronic Disease (PFCD)
- Rare Disease Congressional Caucus
- RetireSafe
- The ALS Association Advocacy & Public Policy Priorities
- The House Committee on Veterans' Affairs
- The Senate Committee on Veterans' Affairs
- Veterans of Foreign Wars (VFW) Advocacy & Policy Priorities
- Vietnam Veterans of America (VVA) Veterans Advocacy
- VA Recommendations to the Asset and Infrastructure Review Commission, March 2022

B. Goal Summary

PALs
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Improving Care for Veterans with Rare Diseases:
A Blueprint for Action Summary Goals

Goal #1: Timely diagnosis, treatment, and supportive care, including in community-based settings and rural areas

Veterans' Needs	Solutions
Timely Diagnosis	• As the largest integrated health system in the nation, VHA should leverage EHR data, clinical expertise, and health risk assessments, to develop and implement protocols to expedite the diagnosis of rare diseases, particularly those which are more common to Veterans. • VHA should conduct a study of Veterans diagnosed with rare diseases to explore their diagnostic journey, identify any barriers to timely diagnosis, and recommend process improvements for the benefit of Veterans who are not yet diagnosed. • VHA should provide access to appropriate genetic testing and counseling when a rare disease is suspected. • VHA should replicate the NIH Undiagnosed Network to address the challenges of rare disease diagnoses in Veterans.
Access to Specialists	• VHA should routinely support access to specialists within local communities and medical centers and should continue to maintain its provider search site (https://www.va.gov/health/ourdoctors.asp) along with the DocInfo services of the Federation of State Medical Boards (https://www.docinfo.org/), and provide Veterans access to those clinical resources regardless of their location. • VHA should make widespread, easy access to telemedicine, provide parity payments for telemedicine visits, and allow low-tech options (e.g., by telephone for those who do not have access to high-speed internet or smartphones) a matter of permanent policy.
Comprehensive Care	• VHA should ensure that care for Veterans integrates the medical, behavioral health, and social service needs in a Veteran-centric team approach. • VHA should search its electronic medical records to identify Veterans who have been diagnosed with rare diseases and should assess whether they have unmet needs. • VHA should ensure that professional VHA Patient Advocates have been trained on rare diseases and the challenges patients have in securing a timely diagnosis and comprehensive care. They should be armed with information about how to reach out to rare disease groups to secure information about referral networks and should help Veterans and their caregivers who want to connect with those groups.

Improving Care for Veterans with Rare Diseases:
A Blueprint for Action Summary Goals

Goal #2: Accelerated access to treatments and supportive therapies

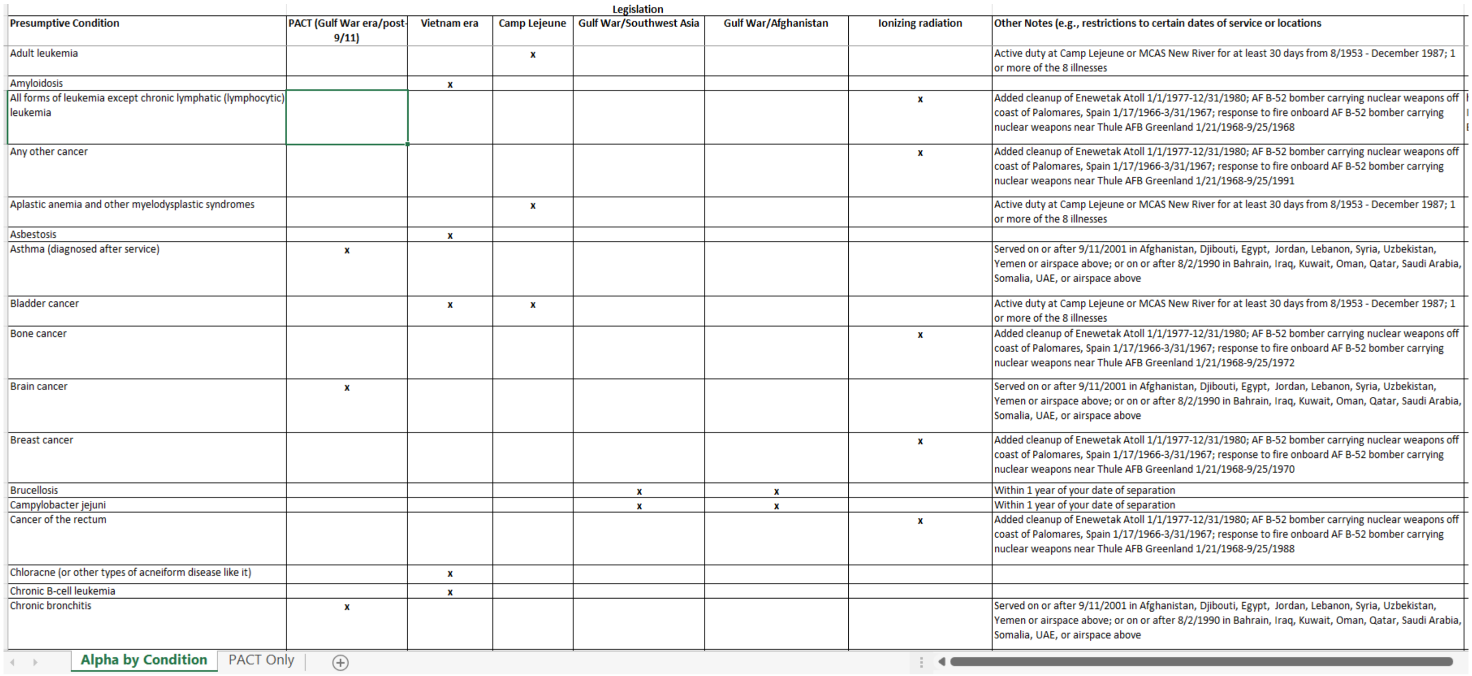
Veterans' Needs	Solutions
Quality Care	• VHA should reach out to those already diagnosed to identify any unmet needs. • VHA should establish a registry to track Veterans with rare diseases and their diagnostic and treatment experiences to identify areas of improvement.
New Therapeutics	• VHA should support the passage of the STAT Act. • Empower patients to develop an impactful voice in policymaking, drug development, and regulatory decision-making.
Access to Approved Therapeutics	• VHA should remove all step-therapy, co-pay, and cost-sharing barriers for Veterans with rare diseases by providing first-dollar coverage and full reimbursement for any FDA-approved therapeutic or other medication selected by their physician.

Goal #3: Improved rare disease awareness, education, information, and advocacy

Veterans' Needs	Solutions
Greater Visibility	• Veterans and rare disease organizations should collaborate to develop a repository of personal stories of veterans with rare diseases, their caregivers, and their clinicians. • Veterans and rare disease organizations should conduct awareness and education programs to inform the general public about rare diseases in Veterans and the impact on them, their families, and their employers, especially in communities with military installations and in the top five most populous States for Veterans: TX, FL, CA, PA, and VA.
Information	• VHA should develop a centralized, easily searchable resource with information about rare disease diagnosis, care, and benefits for Veterans and caregivers. • Rare Disease Advocacy groups should develop health-literate information and submit those resources to the VA's National Resource Directory: https://www.nrd.gov/. • Rare Disease Advocacy groups that may have Veterans within their patient population should highlight their support of Veterans on their web platforms and in their programs. • Veteran and rare disease stakeholders should meet regularly to learn from one another and collaborate on finding solutions to common problems. • Rare disease and Veterans' groups should jointly connect with advocacy organizations and professional societies that may have an interest in rare diseases to educate members and raise awareness, e.g., American Thoracic Society's Public Advisory Roundtable; Consortium for Citizens with Disabilities; Medical associations, including those that focus on underserved populations; AARP; and American Diabetes Association • VHA should develop a platform for Veteran-to-Veteran connections, assuring Veterans with rare diseases who want to connect with other Veterans are able to do that.

C. Presumptive Conditions List

Sample below. Full list available by request to Anne Easter/CPALs @ aeaster@taimail.org.



	Legislation						
Presumptive Condition	**PACT (Gulf War era/post-9/11)**	**Vietnam era**	**Camp Lejeune**	**Gulf War/Southwest Asia**	**Gulf War/Afghanistan**	**Ionizing radiation**	**Other Notes (e.g., restrictions to certain dates of service or locations**
Adult leukemia			x				Active duty at Camp Lejeune or MCAS New River for at least 30 days from 8/1953 - December 1987; 1 or more of the 8 illnesses
Amyloidosis		x					
All forms of leukemia except chronic lymphatic (lymphocytic) leukemia						x	Added cleanup of Enewetak Atoll 1/1/1977-12/31/1980; AF B-52 bomber carrying nuclear weapons off coast of Palomares, Spain 1/17/1966-3/31/1967; response to fire onboard AF B-52 bomber carrying nuclear weapons near Thule AFB Greenland 1/21/1968-9/25/1968
Any other cancer						x	Added cleanup of Enewetak Atoll 1/1/1977-12/31/1980; AF B-52 bomber carrying nuclear weapons off coast of Palomares, Spain 1/17/1966-3/31/1967; response to fire onboard AF B-52 bomber carrying nuclear weapons near Thule AFB Greenland 1/21/1968-9/25/1991
Aplastic anemia and other myelodysplastic syndromes			x				Active duty at Camp Lejeune or MCAS New River for at least 30 days from 8/1953 - December 1987; 1 or more of the 8 illnesses
Asbestosis		x					
Asthma (diagnosed after service)	x						Served on or after 9/11/2001 in Afghanistan, Djibouti, Egypt, Jordan, Lebanon, Syria, Uzbekistan, Yemen or airspace above; or on or after 8/2/1990 in Bahrain, Iraq, Kuwait, Oman, Qatar, Saudi Arabia, Somalia, UAE, or airspace above
Bladder cancer		x	x				Active duty at Camp Lejeune or MCAS New River for at least 30 days from 8/1953 - December 1987; 1 or more of the 8 illnesses
Bone cancer						x	Added cleanup of Enewetak Atoll 1/1/1977-12/31/1980; AF B-52 bomber carrying nuclear weapons off coast of Palomares, Spain 1/17/1966-3/31/1967; response to fire onboard AF B-52 bomber carrying nuclear weapons near Thule AFB Greenland 1/21/1968-9/25/1972
Brain cancer	x						Served on or after 9/11/2001 in Afghanistan, Djibouti, Egypt, Jordan, Lebanon, Syria, Uzbekistan, Yemen or airspace above; or on or after 8/2/1990 in Bahrain, Iraq, Kuwait, Oman, Qatar, Saudi Arabia, Somalia, UAE, or airspace above
Breast cancer						x	Added cleanup of Enewetak Atoll 1/1/1977-12/31/1980; AF B-52 bomber carrying nuclear weapons off coast of Palomares, Spain 1/17/1966-3/31/1967; response to fire onboard AF B-52 bomber carrying nuclear weapons near Thule AFB Greenland 1/21/1968-9/25/1970
Brucellosis				x	x		Within 1 year of your date of separation
Campylobacter jejuni				x	x		Within 1 year of your date of separation
Cancer of the rectum						x	Added cleanup of Enewetak Atoll 1/1/1977-12/31/1980; AF B-52 bomber carrying nuclear weapons off coast of Palomares, Spain 1/17/1966-3/31/1967; response to fire onboard AF B-52 bomber carrying nuclear weapons near Thule AFB Greenland 1/21/1968-9/25/1988
Chloracne (or other types of acneiform disease like it)		x					
Chronic B-cell leukemia		x					
Chronic bronchitis	x						Served on or after 9/11/2001 in Afghanistan, Djibouti, Egypt, Jordan, Lebanon, Syria, Uzbekistan, Yemen or airspace above; or on or after 8/2/1990 in Bahrain, Iraq, Kuwait, Oman, Qatar, Saudi Arabia, Somalia, UAE, or airspace above