



## PATIENT ADVOCACY LEADERS UNITED FOR VETERANS' HEALTH & RARE DISEASES CONGRESSIONAL HILL BRIEFING

### IMPROVING CARE FOR VETERANS WITH RARE DISEASES: ESTABLISHING A NATIONAL COMMISSION

#### HILL BRIEFING HIGHLIGHTS AND ACTION STEPS

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#### Background

Since 2021, the **Center for Patient Advocacy Leaders (CPALs)** has been convening and dialoguing with Veterans' advocates, rare disease advocates, and subject matter experts to better understand the needs of Veterans with rare diseases, comorbid illnesses and the associated social drivers of health facing those affected by rare diseases and their caregivers. A culmination of our work is captured in *Improving Care for Veterans with Rare Diseases: A Blueprint for Action (Blueprint)* developed through the **PALs United for Veterans' Health** initiative, a multi-year, collaborative effort of CPALs.

The Blueprint identifies **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 Department of Defense (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:

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



To expand on this work, CPALs hosted a Congressional Hill Briefing, **Improving Care for Veterans with Rare Diseases: Establishing a National Commission**, on July 10, 2024, at the U.S. Capitol Visitor Center in Washington, DC. This briefing was designed to:

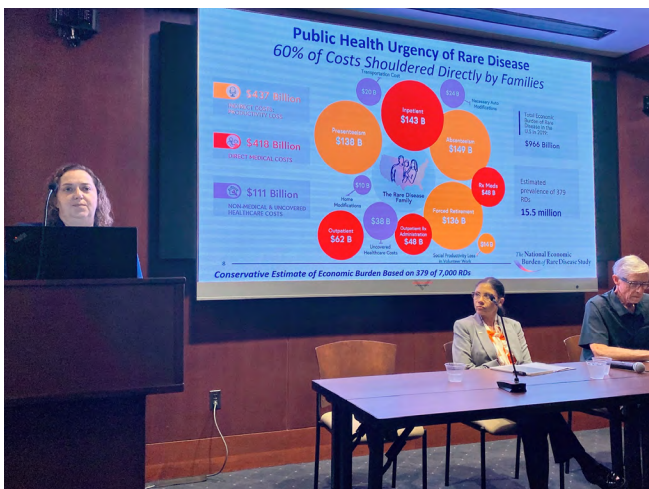
- Bring together Veterans, Veterans' advocates, rare disease advocates, patients, and congressional staff to address unmet needs of Veterans with rare diseases.
- Explore collective action to help ensure Veterans with rare diseases get the comprehensive, patient-centered care and treatment they need and deserve.
- Propose the establishment of a National Commission to further explore the issues facing Veterans and their caregivers affected by rare diseases.



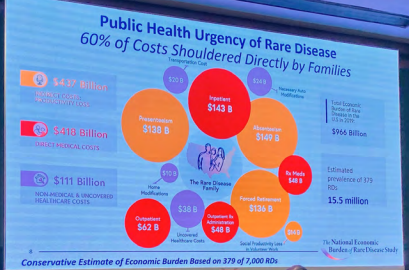
**Rep. Mariannette Miller-Meeks MD, LTC US Army, Ret. (IA-01)**, served as the briefing Sponsor and generously shared ideas stemming from her unique perspective as Chairwoman of the Subcommittee on Health, House Committee on Veterans' Affairs; member of the Rare Disease Caucus; member of the House Committee on Energy and Commerce; a Veteran, and a physician. Miller-Meeks made the following points:

- The VA should be held accountable for the effective use of the funding it receives. Funding ought to be more focused on direct service workers including nurses and Advanced Registered Nurse Practitioners (ARNPs), rather than the administrators and bureaucrats.
- Community services should be expanded, to assure easier access for Veterans in their communities.
- Research into Veterans with rare diseases should be expanded to reduce the health, emotional, and economic burdens on Veterans and the VA system. Bottom line, Veterans need to get the care they have earned.

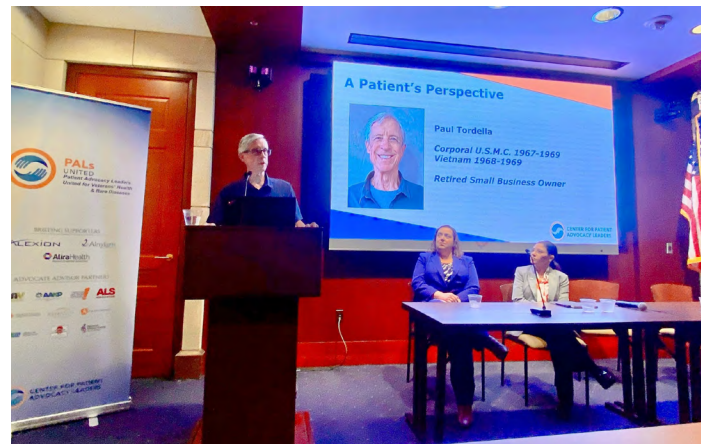
The briefing was expertly moderated by **Edward V. Hickey III, President, American Association of Kidney Patients (AAKP), Chair, AAKP Veterans Health Initiative, U.S.M.C. and PALs United for Veterans' Health Advocate Advisor**. In addition to remarks provided by Rep. Miller-Meeks, Mr. Hickey spoke to the importance of assuring Veterans get the timely care they need and that the proposed Commission could be a reasonable vehicle for helping to resolve the health issues facing Veterans with rare diseases and their caregivers. Thoughtful and passionate remarks from the Veterans community, Rare Disease Community and patients were shared by panel members.



**Jamie Sullivan, MPH, Vice President of Policy from the EveryLife Foundation for Rare Diseases**, provided powerful data on the long diagnostic odyssey many living with rare diseases and their caregivers face, causing further delays to accessing effective treatment and therapies, and increasing financial and emotional burden. Navigating a rare disease diagnosis can require more than 6 years and 17 medical interventions, on average, after symptoms begin, including emergency room visits and out-of-state specialist appointments. Highlights from Ms. Sullivan's presentation included:



- When considering rare diseases collectively, rare is not rare; millions of patients make up this class. The Orphan Drug Act defines a rare disease as a disease or condition that affects less than 200,000. Over 7,000 rare diseases affect more than 30 million people.
- Unfortunately, many do not have FDA approved treatments often because the marketplace does not incentivize creating these treatments; government must play a role incentivizing industry to address this issue.
- A 2019 study identifies that the economic burden on our economy is almost \$1 Trillion annually, for the 319 identified rare diseases. \$220,000, on average additional burden, for each individual with a rare disease.
- Improve the diagnosis by improving the access to expert care; such as policies of licensing/care across state lines.



A highlight of the briefing was the impactful stories of Veterans themselves, with **Naomi Mathis, Assistant National Legislative Director, DAV (Disabled American Veterans), USAF, Ret.,** and **Paul Tordella Corporal United States Marine Corp, retired, living with ATTR Amyloidosis,** speaking to their experiences as Veterans living with rare diseases. Their stories clearly identified the need for continued collaboration, advocacy, and action. Some specific highlights from their comments and stories included:

- Veterans should have consistent providers at the VA facility where they receive care.
- Providers need to have greater understanding and be trained in treating rare diseases.
- Care coordination with private providers and VA staff is crucial for successful treatment outcomes.
- Clear and accessible standards for prescription benefits need to be established so Veterans with rare diseases do not have to go through so many hoops.
- An ALS (amyotrophic lateral sclerosis) diagnosis in a Veteran is automatically linked to military services.
- Often times, physical conditions have comorbid mental health issues, and that cannot be overlooked, and should be treated concurrently.
- There is more evidence that exposure to toxic substances continues to reveal connections to rare disease.
- All Veterans deserve and have earned access to timely information and no less than first class, evidence-based health care.

During the **moderated Q&A** portion of the Briefing, a number of important ideas were raised:

- Additional data, mined from patient records, on rare diseases in the Veteran's community, needs to be gathered to better identify and understand Veterans' illnesses and solutions to shortening the rare disease diagnostic odyssey and burden for patients.
- It would be useful to identify the intersectional issues associated with rare diseases and race, gender, demographic issues (e.g., rural populations).
- Access to care in rural communities needs to be improved so that the travel burden on Veterans and their families is reduced.
- Streamlining access to VA care generally can help reduce the rare disease diagnostic journey.
- Clear standards for prescription benefits need to be established and accessible so Veterans are not subject to drug utilization barriers to receive FDA approved medications.
- Better sources of information should be established for Veterans so they know what is available to them and where it is available.
- Frequent turnover and inconsistency with VA clinical staff is an issue. Veterans frequently do not see the same provider therefore they have to tell their story over and over again, not always to an informed skilled clinician.
- Improved training for VA clinical staff in understanding and treating rare diseases would benefit patients greatly.
- One solution may be to establish a special branch focused on rare disease, accessible throughout the country, through the use of technology.

## ESTABLISHING A NATIONAL COMMISSION ON VETERANS WITH RARE DISEASES

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The overarching goal of the Briefing was to raise the idea of establishing a **National Commission on Veterans with Rare Diseases** to help ensure Veterans with rare diseases get the timely comprehensive care they deserve. This Commission will be designed to study and frame policies aimed at implementing and safeguarding the highest standards in rare disease care including diagnosis, treatment and supports for all Veterans both inside and outside of the VHA. The Commission may focus on the following issues raised during in the Blueprint and at the Briefing relating to Veterans with rare diseases:

- Reducing time between diagnosis and access to appropriate treatment and services to help minimize the cost burden on patients, families, and the system.
- Promoting continued access to genetic research and effective diagnostic processes for identifying rare diseases.
- Improving the availability of evidence-based and best practice care and treatment.
- Providing additional coverage including access to all FDA approved medications along with support and services addressing the social drivers of health.
- Identifying and incorporating the perspective of the patient, family, and caregiver in all advocacy work.
- Establishing a process for data collection and reporting related to rare disease, treatment, and recovery.

A number of follow-up actions were suggested to support the establishment of a Commission and assure that the collective work of the PALs United for Veterans' Health & Rare Diseases initiative moves forward. Some of the specific suggestions for action are below.

- Communicate with Representative Miller-Meeks, thank her and encourage her to consider supporting a National Commission on Veterans with Rare Diseases.
- Communicate with the House Committee on Veterans' Affairs and the Rare Disease Caucus members to encourage them to work collaboratively to improve care for Veterans with rare diseases so that Veterans with rare diseases get the treatments they need in much less time than current system offers.
- Encourage the VHA to develop and implement policy related to treating Veterans with rare diseases that is based on accurate information learned from data mining and rare disease advocates.
- Encourage the VHA to identify a department and leadership at the VHA that would have oversight to assure funds are used wisely for rare disease accurate and timely diagnosis, to use for new treatment technologies and assessment tools for treating rare disease.
- Share the possible focus areas of a National Commission relating to Veterans with Rare Diseases with legislators, in person or through a Dear Colleague letter.
- Reach out to rare disease advocates and Veterans' groups in your state/local community to share what you heard at the Hill Briefing and how all can support this initiative.
- Become familiar with the **Improving Care for Veterans with Rare Diseases: A Blueprint for Action** (Blueprint) developed through the **PALs United for Veterans' Health** initiative, and identify any action steps that might work for you and your organization.
- Share the Blueprint with local legislators, interested health advocates and providers at your local VA.
- Stay in touch with CPALs; CPALs has been dedicated to Veterans' Health advocacy for many years and our work continues as we look to collaborate on next steps that build on the Hill Briefing.

## BRIEFING SUPPORTERS

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## ADVOCATE ADVISOR PARTNERS

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**The Center for Patient Advocacy Leaders** is an evolution of a program called PALS that was developed in 2002. Since 2016 CPALs has been housed within The AIDS Institute. Work continues to be across disease states and populations, and driven by an advocate-centered model, By Advocates, For Advocates.

For more information, please visit the CPALs website at <https://www.centerforpatientadvocacyleaders.org>