Patient Advocacy Leaders United for Veterans' Health Roundtable: Addressing the Issues Affecting Veterans with Rare Diseases/Conditions

November 16, 2021

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Patient Advocacy Leaders
United for Veterans' Health

A program of

CENTER FOR PATIENT
ADVOCACY LEADERS

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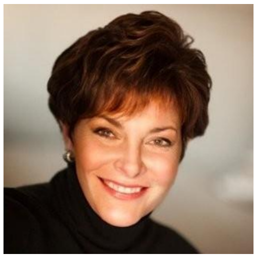
Overview

The **Patient Advocacy Leaders (PALs) United for Veterans' Health Roundtable: Addressing the Issues Affecting Veterans with Rare Diseases/Conditions** event was held on November 16, 2021. This virtual event convened a select group of over 25 veterans' advocates and health leaders to collaborate on ways to improve access to care for U.S. Veterans affected by rare diseases/conditions. The overarching goal of the roundtable event was to help build better collaboration between and among those committed to the health and well-being of veterans. Key objectives included:

- Coalesce advocates and supporters from veteran service organizations, rare disease patient health advocacy groups, caregivers, and/or provider organizations, to identify ways of working together to meet the access, diagnostic, and treatment challenges and disparities specific to rare disease within the VA Health Care system.
- Connect the group of advocates so that they can formulate a framework focused on awareness, education, and advocacy for policy development and/or procedural change within the VA to address access to care for veterans affected by rare disease/conditions.

Welcoming Remarks



Event moderator, **Glenna Crooks**, President, Strategic Health Policy International, Inc., provided opening remarks. Dr. Crooks has a long history with PALs and has a personal interest in this topic, as she has friends and family who have served in the military. She invited participants to introduce themselves, indicate if they have military service experience, and share their geographic location. Dr. Crooks provided an overview of the event agenda and introduced illustrator and graphic artist Nick Navatta, who created a graphic recording of the event (see Appendix).

Overview of Rare Disease Care in the Veterans' Community

The first speaker was **Jamie Sullivan**, Director of Public Policy for the EveryLife Foundation for Rare Diseases, a nonprofit organization dedicated to empowering the rare disease patient community to advocate for science-driven legislation and policy that advance access to diagnoses, treatments, and cures. Ms. Sullivan provided an overview on the impact of rare diseases and some challenges in diagnosing and accessing care. Over 7,000 rare diseases impact approximately 30 million individuals in the U.S. and over 90 percent of rare diseases have no approved treatment option.

The recent National Economic Burden of Rare Disease Study found that in 2019, the total economic burden of only 379 rare diseases was nearly $1 trillion including losses in productivity ($437 billion), direct medical costs ($418 billion), and non-medical and uncovered healthcare costs ($111 billion). Further research is needed to determine the impact of all rare diseases.

Rare diseases are often misdiagnosed.

- It takes 7.6 years in the U.S. to obtain a diagnosis for a rare disease.
- 40 percent of rare disease patients report receiving a misdiagnosis more than once and having to see 7.3 physicians before receiving the proper diagnosis.
- 70 percent of physicians want additional training in rare diseases.

Challenges to optimizing diagnosis and treatment for rare diseases include:

- Lack of clinical research due to small numbers of those impacted and uncertainty around outcomes.
- Limited understanding of the causes, pathophysiology, epidemiological data, and natural course of rare diseases.
- Limited medical expertise.
- Few or no classification systems (e.g., ICD codes).

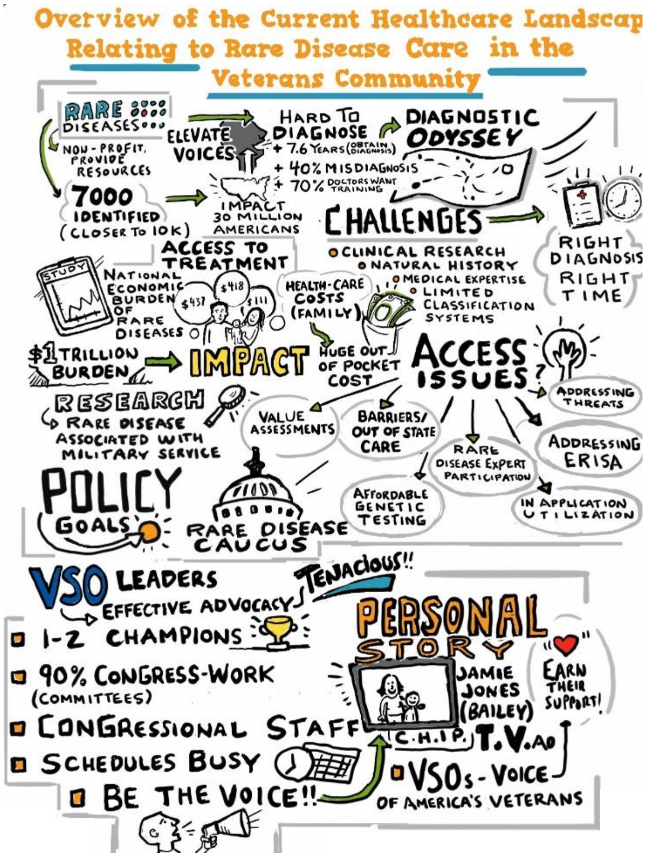
Challenges in accessing care for rare diseases include:

- Barriers to accessing out-of-state care.
- Rare disease expert representation and engagement in Medicaid DUR and P&T processes.
- Addressing ERISA/self-funded plans' ability to laser out treatments for specific diseases and identify employees or families with rare diseases.
- Use of value assessments and implementation of value-based/alternative payment arrangements.
- Access to affordable genetic testing services.
- Inappropriate utilization management practices.
- Addressing threats to the accelerated approval pathway.

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

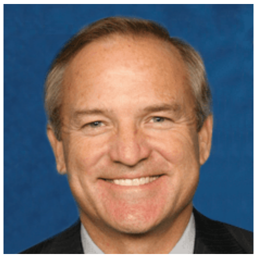
Ms. Sullivan presented the following policy goals related to rare diseases:

- Close the innovation gap for the 93 percent of rare diseases that have no FDA-approved treatment.
- Eliminate the diagnostic odyssey for rare disease patients.
- Improve the regulatory process and advance regulatory science for rare disease therapies.
- Ensure patient access to safe and efficacious therapies and cures at the earliest moment possible.
- Empower patients to develop an impactful voice in policymaking, drug development, and regulatory decision-making.



Ms. Sullivan mentioned the Congressional Rare Disease Caucus, which is comprised of 27 senators and 153 representatives. Opportunities for advocacy include the Rare Across America event and Rare Disease Week on Capitol Hill.

A Legislator's Perspective on the Advocacy Process



Chet Edwards is a former member of Congress and a partner in Edwards, Davis Stover & Associates, a government relations consulting firm located in McLean, Virginia. Mr. Edwards thanked event organizers and participants for their ongoing support of veterans. He shared lessons learned and suggestions for effective advocacy in support of veterans with rare diseases. Recommendations included:

- It is important to have at least 1 to 3 tenacious and engaged champions in Congress who will push for necessary legislation or regulatory changes.
- 90 percent or more of the work of Congress is done in committees and subcommittees. Focus should be directed to relevant committees such as the House and Senate Committees on Veterans' Affairs.
- Most of the committee work is done by Congressional staff and engaging these individuals can be important to success.
- Recognize that members of Congress have limited time; be specific and focused in your advocacy.
- Personal stories from veterans and family members are extremely impactful in advocacy work.
- Veteran service organizations are the most effective advocates.

Breakout Sessions

Dr. Crooks introduced breakout sessions focused on policy and educational issues. Participants were assigned to either the policy or education group based on their responses to the pre-event survey as well as balanced representation of veterans and rare disease advocates.

Group 1: Policy Issues

The purpose of this group was to identify policy and advocacy issues involved in building support within the U.S. Department of Veterans Affairs (VA) for access to effective and equitable treatments and support for veterans with rare diseases. Highlights of the discussion include:

- Differences between state-level and national-level determinations can lead to frustration for veterans, especially when national determinations are not adopted at the state level.
- Institute for Clinical and Economic Review (ICER) value assessments and determinations related to treatment options for rare diseases are sometimes a barrier to accessing treatment.
- There is a need for improved quality and expanded care for rare diseases in community-based settings outside of VA care settings (e.g., treatment for spinal cord injuries).
- Policy advocacy opportunities related to access to care include increasing care in certain geographic locations (e.g., rural areas), providing cultural competency training for providers, making telehealth more available, and increasing care for women veterans.
- Supply chain issues (e.g., laboratory supplies) are negatively affecting veterans.

Policy Recommendations

- Expand care in community-based and rural settings
- Improve cultural competency among healthcare providers
- Improve care for women veterans
- Increase access to telehealth
- Increase transparency of decision-making for formulary changes to allow for more effective advocacy

The group discussed the appropriate people to reach at the VA to advocate for service or policy improvements. Roscoe Butler, Associate Legislative Director for the Paralyzed Veterans of America (PVA), suggested starting with the head of the appropriate service line at the national level, who can then recommend action at more local levels. In addition, recommendations for formulary changes can be submitted to the appropriate committee for consideration, although these decisions are often made behind closed doors.

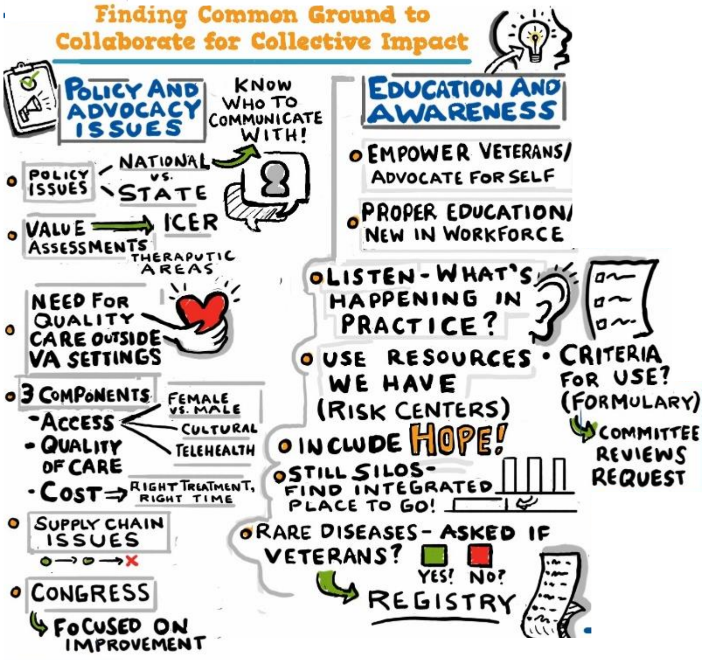
Group 2: Educational Issues

The purpose of this group was to identify educational and awareness materials and strategies for the VA to improve their understanding of rare disease patient needs and for patients/caregivers and providers to better access and advocate for science-based treatments for their rare disease/condition. Highlights of the discussion include:

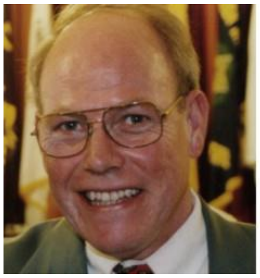
- It is important and impactful for veterans to advocate for themselves and to proactively let their providers know they are veterans.
- Providers entering the workforce should be educated about rare diseases and the specific needs of veterans.
- Learning more about the real-life experiences of veterans in getting their conditions diagnosed will inform advocacy efforts and help tell the story behind the data.
- Risk centers could create educational materials to share their successes and improve awareness among veterans of available services.
- Include messages of hope for veterans and help them to understand that they are not alone.
- Services for rare diseases are very siloed; an integrated, centralized resource or location to obtain information could be helpful for veterans.

Education Recommendations

- Build the capacity of veterans and caregivers to advocate using storytelling
- Educate healthcare providers about rare diseases and needs of veterans
- Create a centralized location for rare disease information and resources



Group Discussion: Making it Happen—What Now?



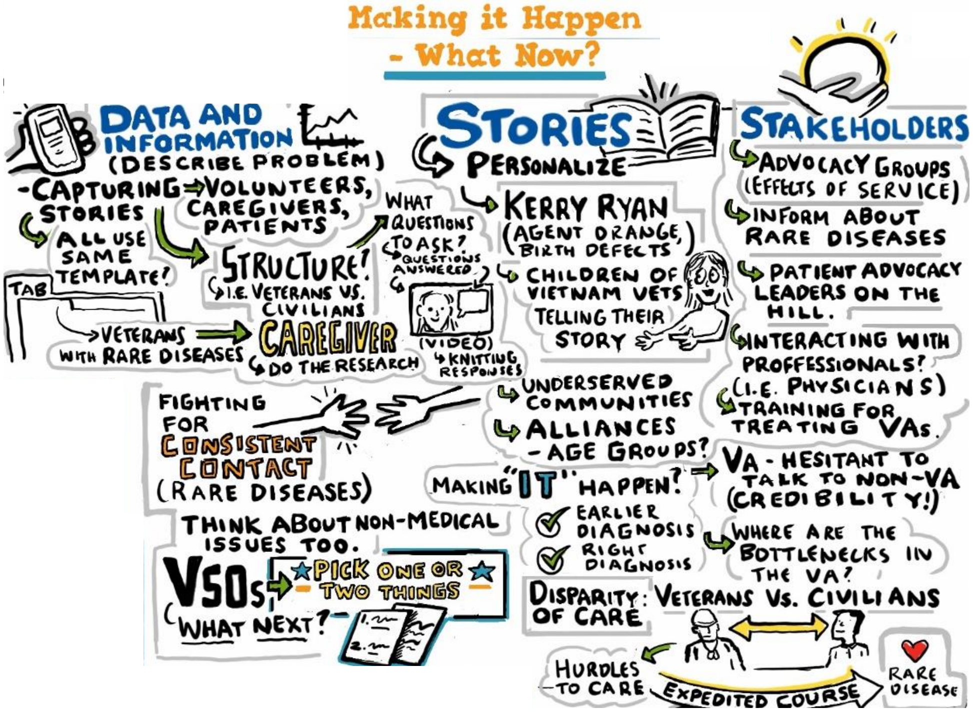
Rick Weidman, Executive Director Emeritus of the Vietnam Veterans of America, joined Dr. Crooks in facilitating a group discussion about possible next steps. Discussion topics and recommendations included:

- Additional research, data, and information are needed to more clearly describe the challenges faced by veterans with rare diseases.
- Stories from veterans and their families/caregivers, as well as organizations that provide services to veterans, are compelling and help put a human face on the issue. For example, sharing stories of the impacts of conditions such as Agent Orange were key in influencing legislation in the past.
- Capturing stories in one location and/or platform would provide ease of use and accessibility. In greater numbers, information from stories could be compiled to provide more quantitative data.
- Provide education for caregivers of veterans with rare diseases, as they often conduct research on specific conditions, in addition to providing care.
- Other stakeholders are needed to engage in advocacy. One recommendation is to conduct a presentation to raise awareness on rare diseases to a group of 40+ advocacy organizations that are convened quarterly by the Veterans Health Council.
- Connect with advocacy organizations and professional societies that may have an interest in rare diseases to educate members and raise awareness. Examples include:
    - 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.
- Develop relationships with key Congressional leaders and decision makers to have them advance legislation and champion the cause.
- Train physicians and other health professionals to routinely ask patients about military service and to better understand issues faced by veterans with rare diseases. The VA is mandated to provide education and training to health professionals; this mandate could be leveraged for this purpose.
- Veterans tend to trust other veterans, which makes it important for health and other advocacy organizations to reach out and build trust and credibility with veterans' organizations.
- Agree on several specific policy goals and speak with a common voice to make the most impact. Suggested policy goals include:
    - Develop and implement a more expedited process for evaluation and early diagnosis of rare diseases;
    - Improve access to treatment and specialists (including those outside the VA system) for veterans; and
    - Identify and address bottlenecks in the VA.

Recommended Next Steps

- Engage diverse stakeholders in advocacy efforts including:
    - Patients and caregivers
    - Health and veteran advocacy groups
    - Professional societies
    - Veterans Health Council
- Focus advocacy messaging and speak with one voice
- Connect, empower and coalesce veterans to build trust, credibility, and a network that will allow for better advocacy for rare disease care

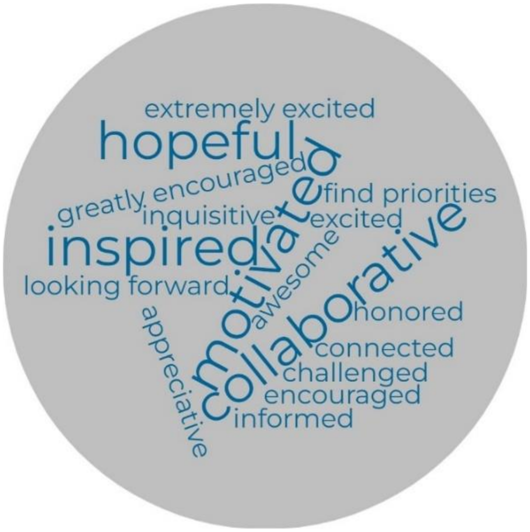
- Bring together stakeholders from both rare disease and veteran service organizations to learn from each other on a regular basis. This type of alliance can be very powerful, especially with the support of an outside convener.



> *"The PALs United for Veterans' Health Roundtable was a high quality, high impact event that combined expert presentations with key insights from some of the most effective veterans advocates in the nation. Together, we discussed maximizing our impact as a collaborative to make certain science-based research and healthcare policies are more inclusive of veteran insights and that all potential barriers to veterans' care choice and access to new diagnostics, devices and drugs, especially for rare diseases, are quickly identified and removed. The discussion was highly motivating and results-oriented, and it was a true pleasure to meet new allies in the noble fight for veterans while also seeing my old friend and advocacy legend Rick Weidman from the Vietnam Veterans of America."*
>
> **Edward V. Hickey, III, USMC**
> *Vice President & Chair of the Veterans Health Initiative*
> *American Association of Kidney Patients*
> *Recipient, Silver Helmet Award, Paralyzed Veterans of America*

Conclusion

Participants were asked to provide one or two words to describe their thoughts about the event. Responses are depicted in the word cloud below:



Anne Easter, Director of CPALs, thanked Dr. Crooks for moderating the event, speakers and subject matter experts for sharing their expertise, and all participants for their interaction. Participants were asked to complete a follow-up survey (sent after the meeting). A full report from this event, including recommendations for next steps, will be shared with all participants.

> *"As a veteran and President of RetireSafe I feel that the Roundtable was very valuable. The discussion on rare diseases and how veterans are now becoming a point of concern inspired hope. Many veterans live in rural areas where treatment is hard to come by. We need to bring this concern to our legislators and increase funding for more accessibility for these veterans. RetireSafe will continue to support efforts of PALs."*
> **Mark Gibbons**
> *President, RetireSafe*

> *"After meeting all the attendees and listening to their stories and opinions, my big take away from the meeting was a feeling that I am not alone, not unlike how our patients must feel when they come to one of our Amyloidosis Support Group Meetings. We have so many issues, but I constantly must remind myself to stay focused as we were so kindly, and often, advised by Congressman Edwards. He seemed to me to be incredibly wise indeed."*
> **Muriel Finkel**
> *President, Amyloidosis Support Groups Inc.*

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ADVOCACY LEADERS
By Advocates, For Advocates

*The **Center for Patient Advocacy Leaders** (CPALs), a collaborative initiative of The AIDS Institute, (TAI), is dedicated to improving the lives of those affected by disease and chronic health conditions of all types by educating and mobilizing health advocacy leaders to work collaboratively in developing impactful policy and advocacy solutions to timely health care issues. CPALs facilitates engagement, collaboration, and a model that promotes patient-centered, quality, affordable healthcare for all people. CPALs utilizes an advocate-centered model, "By Advocates, For Advocates" that focuses on the needs of patients and caregivers, patient advocacy leaders, their organizations, and the diverse communities they serve. The emphasis is on forming long-term meaningful partnerships involving multiple sectors and stakeholders around a common agenda.*

Appendices

Attendee List

First Name	Last Name	Organization	Title	Email
Marquis	Barefield	Disabled American Veterans	Assistant National Legislative Director	mbarefield@dav.org
Roscoe	Butler	Paralyzed Veterans of America	Associate Legislative Director	roscoeb@pva.org
Mike	Cohen	Center for Patient Advocacy Leaders (CPALs)	Associate Director	michael@mjchealthsolutions.com
Josie	Cooper	Alliance for Patient Access	Executive Director	jcooper@allianceforpatientaccess.org
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Anne	Easter	Center for Patient Advocacy Leaders (CPALs)	Director	healthadvocacy@nc.rr.com
Chet	Edwards	Edwards, Davis Stover & Associates	Partner	cedwardstx@verizon.net
Tiffany	Ellett	American Legion	Health Policy Coordinator	
Muriel	Finkel	Amyloidosis Support Groups	President	muriel@amyloidosissupport.org
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Mark	Gibbons	RetireSafe	President & CEO	mark@retiresafe.org
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Travis	Horr	Iraq and Afghanistan Veterans of America	Sr. Director of Government Affairs	travis@iava.org
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Kim	Kim Molnar	The AIDS Institute	Director, Center for Convening & Planning	KMolnar@taimail.org
Fran	Kochman	Alnylam	Director, Global/ US Patient Advocacy and Engagement, TTR	fkochman@alnylam.com
Sean	McIntosh	The AIDS Institute	Research Coordinator	SMcIntosh@taimail.org
Cheryl	Moder	Moder Communications	Vice President	cheryl@modercommunications.com
Deirdre	Parsons	Alnylam	Senior Director, Public Policy & Government Relations	dparsons@alnylam.com
Michelle	Scavnicky	The AIDS Institute	Associate Executive Director	MScavnicky@taimail.org
Artie	Shelton, MD	Veterans Health Council	Director	ashelton@vva.org
Jamie	Sullivan	Every Life Foundation	Director, Public Policy	jsullivan@everylifefoundation.org
Rick	Weidman	Vietnam Veterans of America	Executive Director Emeritus	rickweidman1@gmail.com
Angela	Weir	Paralyzed Veterans of America	Director of Medical Services	angelaw@pva.org
Tonya	Winders	Allergy & Asthma Network	President & CEO	twinders@allergyasthmanetwork.org
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Graphic Recording