ADDRESSING THE UNDERSERVED HEALTHCARE NEEDS OF MILITARY SERVICE MEMBERS, VETERANS, AND THEIR FAMILIES

AN APPROPRIATIONS REQUEST FOR U.S. DEPARTMENT OF DEFENSE PEER REVIEWED RESEARCH IN SPINAL MUSCULAR ATROPHY

cure SMA

www.cureSMA.org

INTRODUCTION

A devastating neuromuscular disease known as spinal muscular atrophy (SMA) affects military and veteran families across the country. SMA causes debilitating muscle and motor function loss that can require around-the-clock caregiving and aggressive medical care, including high-cost hospitalizations, ongoing therapies, and specialized equipment and services. Health interventions and daily living supports for dependents with SMA can cause enormous stress on military service members and their spouses, limit career options for service members, and strain TRICARE and other healthcare systems. Cure SMA, which represents military and veteran families impacted by SMA, seeks $10 million annually in peer reviewed research for spinal muscular atrophy at the U.S. Department of Defense (DOD).

The SMA research and treatment model has led to genetic disease-modifying therapies and served as a standard for success for other neuromuscular diseases. With new research investments, SMA is well positioned to lead again, this time in the discovery of nerve and muscle-focused treatments that could reverse symptoms and damage. These next treatments could also directly impact and benefit many other individuals with neuromuscular diseases and veterans and military service members who experienced nerve damage, spinal cord injuries, and related muscle disorders during their service to our country.

SMA AND ITS CONNECTION TO MILITARY AND VETERAN FAMILIES

SMA is a neurodegenerative disease that affects the motor nerve cells in the spinal cord and impacts the muscles used for activities such as breathing, eating, crawling, and walking. SMA is caused by a faulty gene that is critical for muscle development and function[i]. SMA affects approximately one in 15,000 births in the U.S.[ii] "Our daughter, Kassandra, was born in 1991 while I was stationed at Keesler Air Force Base in Biloxi, Mississippi," said Mike Manchester, a 26-year U.S. Air Force veteran. "My wife, Susan, and I noticed that Kassandra was missing key developmental milestones. That sent us on a diagnostic journey that included many doctors' visits and tests. In 1992, when Kassandra was one, she was diagnosed with SMA."

The Manchesters, like most military and U.S. families, had never heard of SMA before their child was diagnosed with the disease. At the time of Kassandra's diagnosis, SMA was the number one genetic cause of infant death. Babies born with SMA Type 1, the most common and severe form of the disease, often died before reaching their second birthday. Individuals with SMA who survived often required permanent ventilation, feeding tubes, and costly, intensive care and support. The Manchester's daughter, Kassandra, was diagnosed with SMA Type 2, a form of the disease where individuals typically can sit independently but require a motorized wheelchair for mobility and a caregiver for assistance with daily activities.

FACTS ABOUT SMA

- **1 in 15,000 U.S. births**
- **1 in 50 Americans is a SMA carrier, including an estimated 384,000 who are military service members or veterans**
- **Affects U.S. residents in all 50 states & U.S. Territories, including families who have served in the U.S. Armed Forces**
- **Impacts person's ability to walk, lift their arms, and perform other daily living duties**

Source: Cure SMA

More than a decade later (2006) and about 4,200 miles away (Fort Wainwright in Fairbanks, Alaska), Joshua Adams, a U.S. Army service member, and his wife, Pennie, welcomed their first child, Makayla. "She was a happy and curious baby," said Joshua Adams, a Hawaii native who joined the U.S. Army in 2004, a few years after graduating high school. "Within her first year, Makayla missed early developmental milestones, which led to tests, bloodwork, and referrals to specialists outside of Fairbanks." Shortly after their daughter's second birthday, Makayla was diagnosed with SMA Type 2. The Adams's second child, Mariah, was also diagnosed with SMA. Approximately, one in 50 Americans is a genetic carrier of SMA, often without knowing they are carriers. There are approximately 384,000 military service members and veterans who are SMA carriers, according to Cure SMA estimates. If both parents are SMA carriers, every child they have together has a 25% chance of being diagnosed with SMA, regardless of race and gender.

Joshua and Pennie Adams with their daughters, Makayla and Mariah

SMA AND ITS IMPACTS ON MILITARY AND VETERAN FAMILIES

Due to significant muscle weakness and motor function loss caused by the disease, many individuals with SMA require in-home caregiving for assistance with transfers, bathroom support, self-care, and other daily activities. For military families, caregiving responsibilities for dependents with SMA often falls exclusively on the service member and their spouse. Kameron Carpenter, a 14-year member of the Air National Guard, and his wife, Alexandra, are the parents of two young children, Zoey and Lily. Zoey, their oldest child, was diagnosed with SMA when she was 17 months old. Zoey, now age 5, is smart, caring, social, and a "great big sister," according to her parents. Because of SMA, she needs assistance with things that other children her age may take for granted, such as putting on her shoes, getting into the car, or walking through the grass. With leg braces, she can walk independently on solid surfaces, but not for extended times and long distances. Stairs are especially difficult. "She is very active with her friends and cousins. Sometimes, she requires assistance from me and my wife, like when she needed help getting up the water slide at a birthday party or keeping up with her barefooted friends," shared Kameron Carpenter.

CAREGIVING NEEDS OF INDIVIDUALS WITH SMA

- **79% of individuals with SMA rely on a family caregiver (i.e., parent, partner, family member)**
- **Most individuals with SMA require 100-168 hours per week of caregiving support**
- **Caregiving assistance includes dressing, bathroom support, food preparation, transfers (i.e., to wheelchair & bed), self-care, and household duties**

Source: Cure SMA Caregiving Report

Individuals with SMA who require in-home assistance need, on average, one to three caregivers and 100+ caregiving hours a week to assist in their daily lives. Individuals with SMA rely on family members or paid caregivers (when available) to provide this caregiving support.[iii] "Military life can be challenging—from work pressures to the difficulties associated with relocating cross-country every few years due to a base transfer. SMA adds another level of stress," said Joshua Adams, whose daughters with SMA require daily help. During his 26-year military career, Mike Manchester never served near extended family nor did his daughter with SMA ever qualify for paid caregiving support through Medicaid. "My wife and I bore the caregiving responsibility alone, on top of my military duties and caring for our other children," said Mike Manchester.

Service members are often required to be away from home, whether for weekend drills or months- and years-long trainings or deployments. When this occurs, the military spouse takes on the sole caregiving responsibilities. "It is true that the whole family serves when a military member serves," Kameron Carpenter said about his wife's extra duties when he is on assignment. "Getting Zoey to her multiple therapy and doctor's appointments, helping her up the steps, onto the toilet, and into the car, and in assisting at numerous other points throughout the day. Add this on top of the needs of our one-year-old."

> **"It's true that the whole family serves when a military member serves."**
>
> **- Kameron Carpenter**


Kameron and Alexandra Carpenter with their daughters, Zoey and Lily

With limited or no caregiving backup, many military spouses from families impacted by SMA are unable to participate in outside employment. Joshua Adams said, "We live on a single military income because my work and our daughters' needs prevent my wife from being employed. A single income can be difficult given the steep costs of having a disability, from costly vans that can accommodate two wheelchairs to increased medical and everyday costs associated with SMA." High rates of unemployment among military spouses contributed toward financial stress for military families, according to a 2022 Military Lifestyle Survey.[iv] U.S. caregivers of individuals with SMA also reported financial strain due to SMA, including 59% who took on debt, 56% who used up personal short-term savings, 47% who borrowed from family or friends, and 42% who left bills unpaid or paid late, according to Cure SMA's 2022 Understanding the Caregiver Experience Report.[v] Mike Manchester said, "While SMA only affects my daughter, the devastating disease has impacted me, my wife, and our entire family, emotionally, physically, and economically." As part of its "Taking Care of Our People" pledge, the U.S. Department of Defense recognized that military family economic well-being is critical to maintaining an all-volunteer force.[vi] Despite military initiatives to strengthen and support families, military families with dependents with SMA continue to shoulder additional responsibility, stress, and costs associated with SMA.

> **"While SMA only affects my daughter, the devastating disease has impacted me, my wife, and our entire family, emotionally, physically and economically."**
>
> **- Mike Manchester**

SMA can also negatively impact military careers, limiting duty station options and career mobility. Each year, about 400,000 service members make a permanent change of station (PCS), including those with dependents impacted by SMA.[vii] According to military families with SMA, the military branch reviews the PCS destination to ensure it is a fit for the entire family, including dependents with

SMA. Some PCS locations are not equipped for the specialty healthcare, educational supports, and other needs and accommodations required for a military family member with SMA. "My primary consideration for new work and base assignments is whether it will be a good fit for my family. In my field of medical equipment, I have foregone valuable field hospital experiences because these locations are typically more remote and do not have the resources and supports my daughters with SMA need," said Joshua Adams.

For Kameron Carpenter, his career trajectory in the military required lengthy, out-of-state trainings. "My military obligations are increasing. When I am away on military duty, the full parenting burden for our child with SMA falls on my wife." For Mike Manchester, he was unable to take his family to a PCS in Germany because the DOD school there was inaccessible. "It put me in a difficult situation. Do I go to Germany for two years by myself to further my career and leave my family behind because they don't have the medical or school services for my daughter? Or do I take another assignment to remain with my family and potentially not get promoted? It is a difficult decision that no military family should ever have to make." Yet military families impacted by SMA must regularly wrestle with these choices.

> **"When I am away on guard duty, the full parenting burden for our child with SMA falls on my wife."**
>
> **- Kameron Carpenter**

Mike Manchester and his daughter, Kassandra

THE COST OF SMA AND ITS IMPACT ON MILITARY FAMILIES

The annual economic burden of rare diseases, including SMA, was nearly $1 trillion in the U.S., according to a recent report. [viii] "There are increased medical and everyday costs associated with living with SMA. We are fortunate that we receive healthcare coverage through the military. However, TRICARE does not cover caregiving support or non-healthcare related costs," said Joshua Adams. TRICARE is the health care program for U.S. service members, retirees, and their families around the world. The DOD Defense Health Program, which includes TRICARE, has an annual budget of approximately $40 billion, which includes SMA-related costs.[x] Direct healthcare-related costs associated with SMA were often 50-fold higher than for individuals without SMA, including $100,000 in inpatient costs and $50,000 in outpatient costs for a child with SMA. [xi] About 16% of individuals with SMA reported being hospitalized within the past 12-months, higher than in previous years. Top reasons for SMA-related hospitalization included respiratory distress, pneumonia, infection, surgery, respiratory syncytial virus (RSV), and dehydration/malnutrition.[xii]

> **"There are increased medical and everyday costs associated with living with SMA."**
>
> **- Joshua Adams**

SUPPORTING MILITARY FAMILIES THROUGH SMA RESEARCH

Neurological damage caused by SMA has left children and adults with SMA with debilitating muscle weakness, significant motor function loss, and other chronic challenges. While SMA treatment breakthroughs, early diagnosis, and improved care have decreased the mortality rate and increased quality of life for individuals with SMA, unmet needs and associated healthcare costs remain high for individuals with SMA. The SMA community seeks new research and treatment development to address unmet needs related to muscle loss, motor function decline, severe fatigue, respiratory issues, and communication challenges. [xiii] Addressing these chronic complications through new SMA research would help support service members and strengthen military and veteran families impacted by SMA.

The U.S. Department of Defense is committed to addressing the needs of military service members and their families, recognizing that "people are the Department's most valuable asset." [xiv] Through its Peer Reviewed Medical Research Program (PRMRP), DOD is funding medical research that has led to impactful advances in healthcare for military service members, veterans, and their families.[xv] DOD identified SMA as a Neurological & Psychological Health research priority within its PRMRP strategic plan. [xvi] However, direct funding for DOD research in SMA is needed given the tremendous hardship SMA places on military service members, veterans and their families.

> **"Direct funding for DOD research in SMA is needed given the tremendous hardship SMA places on military service members, veterans and their families."**

Past public and private research in SMA has led to three disease-modifying treatments for SMA and research breakthroughs for other diseases. The antisense oligonucleotide (ASO) delivery mechanism used for the first SMA treatment, for example, has spurred research discoveries for Huntington's disease [xvii] and other neurological diseases. [xvii] In addition, the 2019 approval of gene therapy for SMA has led to gene therapy successes to treat other rare diseases, including Duchenne muscular dystrophy. [xix] New research in SMA through PRMRP would help address the chronic complications of individuals with SMA and unlock breakthroughs for other diseases impacting military and veteran families.

UNMET NEEDS INDIVIDUALS WITH SMA WANT ADDRESSED IN NEW RESEARCH & THERAPIES

Source: Cure SMA State of SMA

- **Gain muscle strength (97%)**
- **Achieve new motor function (90%)**
- **Stabilize motor function (88%)**
- **Reduce fatigue (85%)**
- **Improve fine motor skills (79%)**
- **Decrease dependency on mobility devices (62%)**
- **Improve breathing (59%)**
- **Improve communication (31%)**

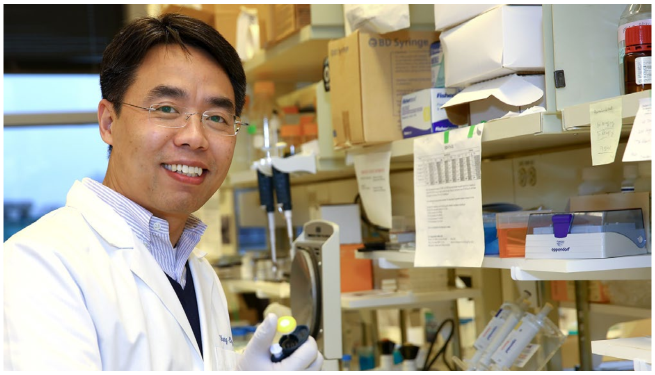
SMA COMMUNITY RESEARCH NEED & REQUEST

Past SMA research has spawned numerous discoveries —from identifying the cause of SMA and increasing knowledge of the human nervous system to the approval of disease-modifying SMA treatments and innovations in rare disease treatment delivery. In addition to addressing the needs of individuals with SMA, these breakthroughs are providing a model pathway for also unlocking new insight, findings, and hope for other neurological and neuromuscular disorders, including amyotrophic lateral sclerosis (ALS) and multiple sclerosis (MS). [xx]

SMA research success has inspired generations of scientists and grown interest in SMA research. [xxi] However, promising SMA-related research proposals have failed to advance due to a lack of research funding. Cure SMA, which has funded nearly $40 million in basic grants in the last two decades, was only able to fund fewer than 25 percent of the promising SMA research proposals submitted to Cure SMA over the past three years due to limited funds. In addition, SMA-related research at the National Institutes of Health has been stagnant for the last decade. [xxii] A lack of funding jeopardizes progress in SMA and other neurological disorders, despite being on the cusp of life-altering breakthroughs to regenerate nerves, restore muscle loss, and to cure SMA.

To help spur research discoveries in SMA and related neuromuscular disorders, **Cure SMA and the SMA community seek $10 million for peer-reviewed spinal muscular atrophy research within the DOD Appropriations bill.** "New discoveries that reverse nerve damage and restore muscle strength would, for example, provide greater functions for a military child with SMA such as eating or dressing independently. These improvements would greatly reduce the caregiving need and burden of that military member and their family," said Mike Manchester. In addition, these next nerve and muscle-focused treatments could directly benefit individuals with all neuromuscular diseases as well as veterans and military service members who experienced nerve damage, spinal cord injuries, and related muscles disorders during their service to our country.

> **"These next nerve and muscle-focused treatments could directly benefit all individuals with other neuromuscular diseases as well as veterans and military service members who experienced nerve damage, spinal cord injuries, and related muscles disorders during their service to our country"**



Appropriations Bill:	U.S. Department of Defense Appropriations Bill
Federal Department:	U.S. Department of Defense
Agency/Account:	Other Department of Defense Programs Defense Health Program (Peer Reviewed Medical Research Program)
Cure SMA Request:	$10,000,000 for Peer-Reviewed Spinal Muscular Atrophy Research The Committee remains concerned by the devastating impact SMA has on military and veteran families, including its strain on service members and military spouses. Therefore, the Committee recommendation includes $10,000,000 for the peer-reviewed SMA research program, and encourages the Assistant Secretary of Defense for Health Affairs to prioritize SMA research that improves strength and motor function and regenerates nerve damage that can also benefit individuals with other neuromuscular diseases and veterans and military service members who experienced nerve damage, spinal cord injuries, and related muscle disorders during their service to our country.

END NOTES

[i] National Institute of Neurological Disorders and Stroke: https://www.ninds.nih.gov/health-information/disorders/spinal-muscular-atrophy
[ii] Cure SMA: https://www.curesma.org/about/
[iii] Stuck Inside Caregiving Report: https://www.curesma.org/wp-content/uploads/2024/01/Advocacy_Report_Stuck_Inside_vFnl_Web.pdf\
[iv] Military Family Lifestyle Survey: https://bluestarfam.org/wp-content/uploads/2023/03/BSF_MFLS_Spring23_Finding3_Financial_Security.pdf
[v] 2022 Understanding the SMA Caregiver Experience Report: https://www.curesma.org/published-sma-research/
[vi] U.S. Department of Defense: https://www.defense.gov/News/News-Stories/Article/Article/3747144/dod-state-department-expand-employment-opportunities-for-military-spouses-stati/
[vii] Military OneSource: https://www.militaryonesource.mil/moving-pcs/plan-to-move/pcs-the-basics-about-permanent-change-of-station/
[viii] National Economic Burden of Rare Disease Study: https://everylifefoundation.org/wp-content/uploads/2021/02/The_National_Economic_Burden_of_Rare_Disease_Study_Summary_Report_February_2021.pdf
[ix] TRICARE: https://www.tricare.mil/About
[x] U.S. Department of Defense FY 2025 Budget: https://comptroller.defense.gov/Portals/45/Documents/defbudget/FY2025/FY2025_Budget_Request_Overview_Book.pdf
[xi] Economic burden of spinal muscular atrophy: https://www.ncbi.nlm.nih.gov/pmc/articles/PMC7655070/
[xii] 2023 State of SMA Report: https://www.curesma.org/wp-content/uploads/2024/06/9042024_State-of-SMA_vWeb.pdf
[xiii] 2023 State of SMA Report: https://www.curesma.org/wp-content/uploads/2024/06/9042024_State-of-SMA_vWeb.pdf
[xiv] U.S. Department of Defense FY 2025 Budget: https://comptroller.defense.gov/Portals/45/Documents/defbudget/FY2025/FY2025_Budget_Request_Overview_Book.pdf
[xv] U.S. Department of Defense Peer Reviewed Medical Research Program: https://cdmrp.health.mil/prmrp/default#:~:text=The%20Peer%20Reviewed%20Medical%20Research,retirees%2C%20and%20their%20family%20members.
[xvi] Peer Reviewed Medical Research Program Strategic Plan: https://cdmrp.health.mil/prmrp/pbks/PRMRP%20Strategic%20Plan.pdf
[xvii] PTC Therapeutics' Huntington's Treatment Gets FDA Fast Track Designation: https://www.morningstar.com/news/dow-jones/202409265639/ptc-therapeutics-huntingtons-treatment-gets-fda-fast-track-designation
[xviii] Antisense Oligonucleotides: An Emerging Area in Drug Discovery and Development, National Library of Medicine, 2004: https://www.ncbi.nlm.nih.gov/pmc/articles/PMC7355792/
[xix] National Institute of Health: https://pubmed.ncbi.nlm.nih.gov/32886442/
[xx] The American Brain Foundation: https://www.americanbrainfoundation.org/how-spinal-muscular-atrophy-research-is-fueling-advancements-for-other-neuromuscular-diseases/
[xxi] National Institutes of Health: https://directorsblog.nih.gov/tag/spinal-muscular-atrophy/
[xxii] NIH Research Funding: https://report.nih.gov/funding/categorical-spending#/

ADVOCATE FOR INDIVIDUALS WITH SPINAL MUSCULAR ATROPHY AND THEIR FAMILIES

facebook.com/cureSMA

linkedin.com/company/families-of-sma

@curesmaorg

tiktok.com/@curesmaorg

youtube.com/user/FamiliesofSMA1

Cure SMA Advocacy | 800-886-1762 | advocacy@cureSMA.org | cureSMA.org

Cure SMA is a national organization that advocates for individuals with spinal muscular atrophy, a progressive neurodegenerative disease that robs people of physical strength, taking away their ability to walk, swallow, and breathe.