

# CCM CARE Act of 2019

*Cerebral Cavernous Malformations Clinical Awareness, Research, and Education Act of 2019*

**Senator Tom Udall (NM) & Representative Ben Ray Luján (NM-03)**

Cavernous Cerebral Malformations (CCM) is an uncommon genetic blood vessel disease that impacts the brain and spinal cord. The disease, also known as Cavernous Angioma, or Cavernoma, is a devastating blood vessel disease that affects about 600,000 people in the United States. Although this disease is more common among **New Mexico** Hispanics than any other group, recent research has found that CCM is also found high rates in Hispanics in **Texas, Arizona, and Colorado**. Additionally, a second mutation, CCM2 Common Deletion, has been found to result in increased rates of the illness in **South Carolina, Georgia, Florida, Alabama, Mississippi, Louisiana, Oklahoma, Kentucky, Kansas, and northern California**.

Unfortunately, due to a lack of research and coverage funding, detection is costly and inconvenient for patients who should be tested. Lack of research also means that CCM has no other treatment available aside from brain and spinal surgery, which is only a viable option for certain patients. Additionally, a shortage of physicians trained to recognize and diagnose CCM makes matters worse. As you know, June is Cavernous Angioma Awareness month and it is important that we highlight these real issues. Although some promising work has been undertaken, such as 1) research under the Baca Family Historical Project, 2) the passage of NM HJM7 through the state legislature and 3) promising, possibly curative, research progress in microbiome and micro-RNA, we must do more to address this disease on the federal level.

**Specifically, this legislation would:**

- **Findings Section**
  - Provides updated information on scope of CCM and highlights the lack of research and treatment options for those with the disease.
- **Expansion/Coordination of NIH Research Activities Section**
  - Expands the coordination and scope of research at NIH so that it can pursue further clinical, translational, and basic research. Additionally, it directs the NIH director to build upon the success of *Clinical and Research Coordination Centers* as well as the *Participation Centers*.
  - Requires NIH Director to convene a CCM Consortium to develop and implement physician and patient training plans.
  - Provides assistance for small biotech firms to request prioritized funding to research and develop treatment options.
- **Expansion/Coordination of CDC Research Activities Section**
  - Requires the CDC Director to award grants that will help obtain better CCM data through Surveillance and Research programs.
  - Instructs HHS Secretary to award grants to entities to further CCM data gathering under the National CCM Epidemiology Program.
  - Requires HHS to provide technical assistance and coordination with Clinical Centers to award grantees.
- **Expansion/Coordination of FDA Research Activities Section**
  - Instructs the HHS Secretary to have FDA Commissioner establish and coordinate a *Biomarker Qualification Program* and a *Clinical Outcome Assessment Qualification*.
  - Requires coordination of HHS Secretary and FDA Commissioner to support *Investigational New Drug Applications* to hasten clinical trials and promote *Adaptive Trial Design and Expedited Pathways* for rare disease research.

**Support:** *Angioma Alliance*