

COMPASS

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SMA Care Center Network: Connecting Families to Comprehensive Care

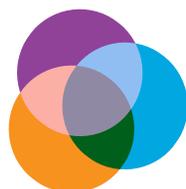
This year, Cure SMA launched the SMA Care Center Network, a collection of specialized clinics across the nation that will help ensure that all those living with spinal muscular atrophy (SMA) are able to receive high-quality, multidisciplinary care. The SMA Care Center Network is a diverse set of hospitals that share clinical data on SMA patients by connecting their electronic medical records to our registry. This will allow us to investigate the impact treatments and care have on SMA, create benchmarks, and certify centers of excellence in the future.

By connecting SMA Care Centers to one another and utilizing a united registry to collect data, healthcare providers, including neurologists, pulmonologists, physical and occupational therapies, nutritionists, and orthopedists, will be able to work together to provide complete and comprehensive treatment. Because SMA is a medically complex disease, these clinical teams must take a multi-faceted approach to treat SMA, and Cure SMA is taking a multi-faceted approach to improve care, as well.

Over the next three years, Cure SMA will invest \$9.1 million in the development of the SMA Care Center Network. The goal of the Network is to create an evidence-based standard of care to improve the lives of people with SMA. Cure SMA has developed several characteristics to identify SMA Care Centers to be a part of this network. Most importantly, SMA Care Centers must demonstrate a desire to improve SMA care both locally and nationally and offer the following:



Geographical diversity



An interdisciplinary care model



Pediatric and adult care team partnerships



The capacity to provide FDA-approved therapies for SMA



An Information Technology (IT) team to support the integration of electronic health records (EHRs)

How the SMA Care Center Network Benefits Families

Dany Sun is the mother of a 7-year old girl living with SMA type 3. Doctors originally diagnosed Dany's daughter with SMA type 2. Her family was given a packet of information and told that someone would follow up at a later date.

The night of her daughter's diagnosis, Dany connected online with other families impacted by SMA through the Cure SMA website. Dany received critical information which helped her to understand her daughter's complex medical needs and get connected with a multidisciplinary care team. Her daughter's diagnosis was eventually corrected to SMA type 3 and she was able to receive the appropriate comprehensive care that she needed.

"The stress when we were first diagnosed of physicians not knowing what SMA meant for my child was crippling. I couldn't trust them," Dany described. "Once we were connected to a center that understood SMA, I had normal worries for my kids, but I'm not worried about their wellbeing. I'm not worried the people caring for them don't understand SMA care. That should be available for everyone."

For more information, visit cureSMA.org/carecenternetwork



Dany notes that the SMA Care Center Network will ensure that families impacted by SMA can be confident in the uniformity of the care they receive. She adds that it will “completely change the landscape” by improving quality of care, limiting the impact of multiple hospitalizations and incorrect or unnecessary interventions, and relieving the stress that parents face for their children’s wellbeing.

Because of the comprehensive information that Dany received from her daughter’s multidisciplinary care team, Dany understood that her second child could be affected by SMA, as well. Her son, who is now four years old, was able to be diagnosed with SMA when Dany was just 14 weeks pregnant. This early diagnosis allowed her and her care team to identify initial signs of weakness and start immediate care.

How the SMA Care Center Network Supports Clinicians

Ten years ago, Dr. Richard Shell encountered a young patient with SMA in the Intensive Care Unit (ICU), who physicians were having difficulty treating. Dr. Shell had experience caring for children with neuromuscular difficulties, but this child’s parents asked him to think “outside the box” to help their son.

At the time, healthcare providers did not think there was much they could do to treat SMA, given that it can be fatal. Dr. Shell reached out to Dr. Mary Schroth, a renowned physician in the SMA community, to better understand how to care for people with SMA. He noted that soon, he and other clinicians realized that patients did better if they received respiratory care.

By connecting with other healthcare providers treating people with SMA, Dr. Shell was able to better understand the needs of his patient. Eventually, he helped form a multidisciplinary team to treat patients together, across specialties.

Dr. Shell says that the development of SMA Care Centers will ensure children receive good care and clinicians advance research. “It’ll help expose families to multidisciplinary care to get the answers they need in just one clinic. It will allow for better communication between families and care teams because we can analyze all aspects of care. We can also share these learnings with other SMA Care Centers in the network.”

Now, Dr. Shell’s first patient with SMA is an active sixth grader. He notes that while healthcare providers could not

do everything they wanted to treat him, the multidisciplinary team has been able to learn and improve care to meet the specialized needs of the SMA community. Dr. Shell adds that now there is not only a strong clinical center for SMA, but they can be involved in “the latest and greatest research to care for children with SMA,” as well.

A greater understanding of SMA and its treatment through the SMA Care Center Network lead to several benefits.

An Evidence-Based Standard of Care

The SMA Care Center Network is collecting large bodies of data to analyze and develop an evidence-based standard of care for healthcare providers. Because SMA affects only one in every 11,000 infants, many physicians have not cared for people impacted by the disease. This can mean that families receive insufficient information on care and treatment or travel long distances to see physicians with specialized knowledge.

But with life-extending therapies now available for SMA, the number of individuals living with this disease will continue to increase. As a result, more healthcare providers will need to be trained in appropriate SMA care to help fill the gap. The SMA Care Center Network will encourage healthcare providers across the country to efficiently share accurate and consistent information on SMA care.

“I’m pretty involved in the community and have contact with families who are in hospital settings, where providers trying to take care of them just don’t have any idea how or what they should be doing. For example, some of the pulmonary interventions contradict what a medical professional thinks they should be doing. Not all families will know to look for that or know there’s a different way.”

– Dany Sun, mother of two children with SMA

The SMA Care Center Network can give families peace-of-mind that they are receiving the most cutting-edge and reliable information on SMA care. This will prevent many parents from feeling that initial diagnoses and treatment plans are inadequate to care for their child’s complex medical needs.

Greater Access to Approved SMA Treatments

With a robust pipeline of SMA treatments currently in clinical trial, the potential for new treatments is larger than ever before. This changes the landscape of the disease and healthcare providers, in turn, must have data to rapidly adapt to this ever-changing environment and to evolving patient needs.

In 2016, the first therapy for SMA was approved by the FDA, providing children and families impacted by SMA with renewed hope. Since then, parents and physicians have reported that children receiving this therapy are maintaining their strength and motor abilities. In some cases, children are even able to regain some movement, such as the use of their fingers. Additionally, infants who can receive the treatment before the onset of symptoms are now able to meet motor milestones that were previously unimaginable, such as sitting or walking.

The SMA Care Center Network will increase the number of sites that offer access to this life-changing SMA treatment. By providing doctors with readily available data to guide the administration of treatments, SMA Care Centers will also be able to provide more people access to treatments for SMA as they are approved in the future.

“I think that having a standardized approach to any disease process will aid in making that care better. With new drugs being developed, it will be important to have centers who know how to deal with them. At the same time, as these new treatments are created, it will be important to ensure that families understand that centralized care and check-ups need to be continued. We will have a standard of care we have to follow and we can learn from the patients coming to us and share those learnings, as well.”

– Dr. Richard Shell, pediatric pulmonologist, Nationwide Children’s Hospital

As new treatments come to market, new patient data can improve prescriber and payer understanding of how they can be used for SMA care. These data can also be used to accelerate and improve coverage and reimbursement decisions. Currently, some interventions used by healthcare providers may not be specifically indicated for children or

adults with SMA, leading to financial stress on families who may already have reduced household incomes due to the demand of caring for someone with SMA.

The collection of data at SMA Care Centers will allow physicians and insurers to benchmark appropriate treatments for different patient groups. These findings can not only be used to support the effectiveness of these interventions for people living with SMA, but with added context, insurers can also more easily make coverage and reimbursement decisions.

“Information on quality improvement for SMA treatment will be disseminated and become the new standard of care. If clinicians can work together across the country and publish papers that say this is why you should provide this therapy for this patient population, and we’re showing a better quality of life and a longer life, then the cost of taking care of kids should be less.”

– Dr. Richard Shell, pediatric pulmonologist, Nationwide Children’s Hospital

Additional Sites for SMA Clinical Trials

Collecting robust data about SMA is challenging because it is such a rare disease. The SMA Care Center Network has a completely seamless and automatic data collection process.

This will help SMA Care Centers work collaboratively to answer questions about the impact of SMA and develop strategies for optimal care to better meet the needs of families impacted by SMA. Additionally, the SMA Care Center Network will allow researchers to alleviate challenges related to site capacity and patient access to clinical trial sites, as well as better meet the needs of people with SMA participating in research.

“Specialized centers will help advance research and expose more physicians to that research once it is produced. Hopefully, it will become a great tool to disseminate research protocols to get the most patients enrolled. Creating these Care Centers and collecting data will have a powerful effect on how institutions can look at research.” said Dr. Shell.

The Future of SMA Care

The needs of the SMA community will continue to grow and Cure SMA hopes to add additional Care Centers to the SMA Care Center Network to meet these needs. The goal is to continue to expand the Network until all people affected by SMA across the US have access to the best possible care.

Cure SMA plans to enroll 16 to 18 centers in 2018, with more centers added in future years to meet the growing needs of the SMA community.

While the Network grows, Cure SMA will continue to strive to improve local SMA care. This includes a physical and occupational therapy mentoring program, which is funded by Cure SMA. Through this program, an experienced SMA therapist is paired with a newer therapist. By making this connection, more individuals will have access to a trained SMA therapist and receive higher-quality care.

Cure SMA thanks all those who have supported the development of the SMA Care Center Network. From clinicians who have worked to form multidisciplinary teams, to families who have tirelessly helped disseminate SMA knowledge to others, the community effort to improve care has helped sustain this mission.

As the SMA Care Center Network continues to grow, an unprecedented level of support will be required to maintain and expand the Network.

“Having that proper and correct care will have a great impact that I don’t think families realize yet. There are so many factors that will be positively impacted, such as less financial stress due to unnecessary hospitalizations.”

– Dany Sun, mother of two children with SMA

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