



SUBMITTED VIA ELECTRONIC SUBMISSION

Centers for Medicare & Medicaid Services
Department of Health and Human Services
Attention: CMS-2324-NC
P.O. Box 8016
Baltimore, MD 21244-8010

RE: Coordinating Care from Out-of-State Providers for Medicaid Eligible Children with Medically Complex Conditions CMS-2324-NC

June 3, 2020

To Whom It May Concern,

On behalf of the EveryLife Foundation for Rare Diseases and all patients and families impacted by rare diseases and disorders, thank you for reopening the Request for Information (RFI) regarding care for children on Medicaid with complex conditions delivered by providers outside their home state. The issue of out-of-state care is of high importance to EveryLife and the rare disease community more broadly. While children with complex conditions are not exclusive to the rare disease population, the very nature of rare diseases and how they are treated means our community is disproportionately impacted by these challenges. The Foundation and our member organizations spend countless hours navigating many of the access challenges noted in the RFI, providing us with insights we hope CMS will find of use as you continue formulating policy in this area.

With this background and perspective in mind, we are pleased to offer the following comments in response to the RFI.

Rare Diseases and Out-of-State Care

A rare disease or disorder is defined as affecting fewer than 200,000 people in the United States. The most recent estimates suggest there are about 7,000 such diseases. Yet when extrapolated across the population, rare diseases are quite common, affecting between 25 to 30 million people in the United States, or possibly as high as 10 percent of our U.S. population. Because of the relatively small patient populations, care for any single rare disease – when such care exists at all – is typically relegated to a very small number of providers and institutions. In many cases, such as very rare diseases and pediatric disorders, this universe may be one or two providers in the country.

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Because of this dynamic, it is common for families with rare diseases or disorders to routinely travel out of state for their primary healthcare services. This reality is burdensome even on patients and families who are well-resourced. For those patients who lack financial resources – such as those who depend on Medicaid or the Children’s Health Insurance Program (CHIP) to finance all or part of their healthcare – these challenges are often exponentially greater and include:

- Obtaining authorization from a state and/or Medicaid Managed Care Plan (MCO) to obtain care from a provider located out of state.
- Securing a provider from out of state who may be willing to accept another state’s Medicaid rates as compensation for care that is often highly complex and multi-faceted.
- Traveling to and from the accepting provider – often at great distances and potentially for multiple follow-up visits and multiple day visits.
- Navigating potential delays in accessing care because of onerous provider screening and enrollment requirements that only add time to the clock rather than protect program integrity needs.
- Overcoming obstacles to obtain follow-up care including prescriptions for medications, durable medical equipment and other needs.

With the care and access needs of the rare disease patient community in mind, we offer the following points for your consideration in future policy rulemaking.

Eliminate Barriers to Specialized Care Access

We urge CMS to be aggressive in knocking down barriers that impede a Medicaid or CHIP beneficiary with a rare disease or disorder from accessing clinically necessary care. Achieving this starts with a clear recognition that quality healthcare for people with rare diseases and disorders is national in scope because of the very nature of rare diseases, especially those conditions that affect very small populations. As we move toward an era of increasingly personalized and genetically targeted medications, CMS must reform or remove policies that limit access to expert care and targeted therapies.

As a starting point, CMS can prevent states from engaging in duplicative screenings of healthcare providers who are already enrolled in good standing in their home state Medicaid program and who are offering to care for a child from out of state. Such duplicative screens add significant burdens on providers and, ultimately, delay delivery of time-sensitive care. **CMS already permits states to use screenings done by other states or by Medicaid, but we need CMS to act definitely to develop and**

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support a streamlined screening and enrollment pathway akin to what is proposed in H.R. 5900, the Accelerating Kids' Access to Care Act.

Relatedly, we urge CMS to work with stakeholders including children's hospitals, payers and state Medicaid agencies to develop a system to facilitate enrollment of providers into other state Medicaid programs when called upon to provide such care. This could include, for example, common and simplified enrollment application forms and/or application criteria to help expedite the enrollment process and ensure no Medicaid beneficiary with a rare disease or disorder is forced to wait for care simply because of a bureaucratic impediment.

Finally, no discussion on access can be complete without touching on payment. We recognize that states have every interest to deliver care to their beneficiaries at home, particularly in cases where care from an out-of-state provider may be far greater than what a state typically pays. Incentives for patient communities and providers are aligned in the respect as families face exceptional personal financial costs and burden when traveling for out of state care. However, rare diseases exemplify the necessity of having out-of-state care options, particularly in cases in which the number of qualified providers is in the single digits. We encourage CMS to work with all stakeholders to consider financing innovations to address these tensions.

Pay Particular Attention to Transition Points

The transition from childhood to adulthood is particularly important for those impacted by rare diseases or disorders or other conditions that onset during childhood. Unfortunately, this point is also fraught with peril as the patient is teetering the pediatric and adult delivery systems. As care and treatment for pediatric-onset conditions improve and lifespans are beginning to expand, patients are often required to 'age out' of the pediatric care setting where the providers and specialists who are expert in their rare conditions are located. When care transitions are done poorly, these patients can fall through the gaps and see their health deteriorate significantly. Conversely, when done well, the transition from childhood to adulthood for a person with a serious condition, rare disease or otherwise, can position the person for future success. In limited instances, expert providers may be available to young adults and adults with select rare diseases but these providers are often even more scarce than the pediatric providers as these fields are still nascent.

Example of such models include Duchenne muscular dystrophy and Spina Bifida.

In Duchenne, a network of multidisciplinary care centers has been established by the Parent Project Muscular Dystrophy (PPMD) in collaboration with the Duchenne provider community. All Certified Duchenne Care Centers have met the requirements for, and agree to provide, optimal standardized care and services which were established and published by the Centers for Disease Control and Prevention

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(CDC) as the Duchenne Care Considerations¹ in 2010 and updated in 2018. To date, in the US, there are 29 Certified Duchenne Care Centers, to include 28 pediatric centers, 1 pediatrics/adult combined center and 1 adult Certified Duchenne Care Center.

The Spina Bifida Association (SBA) Clinic Care Partner program is an initiative that stems from the existing Spina Bifida Collaborative Care Network (SBCCN), a program that works to identify the needs of people with Spina Bifida, connect with health care providers, identify clinics with the best outcomes, and identify research priorities. There are 31 clinics from across the country that met 10 standards² identified as best practices to care for people with Spina Bifida. Conversely, there are over 100 clinics that offer care but do not meet these standards as of yet. Of the 31 clinics, all either have specialty care services for individuals with Spina Bifida who are transitioning into adulthood or are in the process of developing them; however, there are very few transition clinics or adult clinics to care for the ever-growing adult population, representing 65% of the people with Spina Bifida in the United States.

As CMS gives thought to health home and other best practices to manage care for children with medically complex conditions, it is essential that the agency bear in mind the needs of the population transitioning from childhood to adulthood. At a base level, this must ensure that the agency does not institute age or other limits on access to care that could preclude a person with a pediatric-onset condition from benefiting from medical management even once they attain the age of 18.

Because many genetic rare diseases and disorders onset at birth or in early life, many care programs are typically housed at children's hospitals. This means that it is not uncommon for young adults with rare diseases and disorders to still receive their core care by providers at children's hospitals. We hope more care for conditions that were once universally fatal during childhood or adolescents will be found in non-pediatric care settings, but CMS must recognize this reality and ensure policies to better manage complex conditions of childhood includes conditions that onset during the pediatric years even if the beneficiary may be over age 18.

Access to Medications, Particularly Novel Therapies

While the majority of rare diseases lack any FDA-approved disease-modifying therapies, we are grateful for recent breakthroughs that have extended the life of once-fatal conditions. These include some subtypes of lysosomal disorders and muscular dystrophy, Cystic Fibrosis, and other neurological disorders. And we are optimistic about the research that is ongoing to develop treatments for additional conditions. At the same time, we are very mindful that obtaining approval of a new therapeutic is for many patients, particularly those on Medicaid, only the start of a process to ultimately obtain access to the treatment, particularly when such therapies carry a sizeable price tag.

¹ https://www.parentprojectmd.org/wp-content/uploads/2018/06/Duchenne_CareConsiderations_2018_Part1.pdf

² <https://www.spinabifidaassociation.org/resource/clinic-care-partner-standards/>



The EveryLife Foundation recognizes the complexity of addressing the challenges associated with the costs of drugs and the need for a comprehensive and system-wide approach. With regard to the specific issue of ensuring Medicaid beneficiaries with complex conditions receive access to all necessary care, CMS must include a focus on the issue of access to medications, particularly novel gene and cell-based therapies that are only approved for administration at a limited number of specially equipped institutions. This should include working to standardize to the greatest extent possible Medicaid coverage policies of such therapies so that a beneficiary's ability to access a therapy is not determined by the state in which he or she lives. We also encourage CMS to consider this category of therapeutics for a Center for Medicare and Medicaid Innovation (CMMI) pilot or demonstration to explore the most effective model or models for providing access to gene and cell-based therapies among all Medicaid beneficiaries.

Access to Emergency Treatments

The ongoing novel coronavirus (COVID-19) pandemic exemplifies the need for adequate attention to policies pertaining to access to out-of-state emergency services. While most patients probably do not need to travel outside of their home state for emergency services, patients with rare complex conditions can and do require such care. For example, when a patient has a complex condition and multiple comorbidities and experiences a medical emergency, it is of high importance that the patient's care team – which may be at an out-of-state institution – be engaged in his or her emergency care. And assuming the patient is stabilized and able to be transported, it is best for that to occur so the care team can be directly engaged in addressing the emergency.

Unfortunately, the rare disease community knows far too many personal stories of people who experienced seriously adverse events – even death – in receipt of typical emergency care. For example, in the case of people with Duchenne muscular dystrophy, administration of oxygen without careful monitoring can result in death from buildup of carbon dioxide. In other instances, symptoms of fatty embolism syndrome can go unrecognized in a child with Duchenne and have catastrophic consequences. The coronavirus and its many unknowns – including the extent of risk on vulnerable populations and children – is a real-time reminder of the delicate issues that make emergency care even more worrisome for patients and families impacted by rare diseases. As CMS considers policies to support care by out-of-state providers to Medicaid beneficiaries with complex conditions, these considerations must ensure there are no impediments to emergency care. This needs to include ensuring that out-of-state emergency care for these patients is not thwarted by prior authorization or other regulatory barriers as well as policies that ensure that such out-of-state emergency care is appropriately paid for by the Medicaid plan or MCO of the patient.

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Conclusion

The EveryLife Foundation thanks CMS for reopening this important comment period. We hope you find our comments of value and we welcome any opportunities to work with the agency as you further shape these important policies. Please contact Annie Kennedy at akennedy@everylifefoundation.org if we or any of our patient community partners can provide additional support or information throughout this process.

Sincerely,

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CC: Mark Dant, Board Chairman
Frank J. Sasinowski, Board Vice-Chairman

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