

June 1, 2020

The Honorable Seema Verma  
Administrator  
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]**

Dear Administrator Verma,

The Institute for Gene Therapies (IGT) appreciates the opportunity to provide feedback to the Centers for Medicare and Medicaid Services (CMS or “the Agency”) regarding the Request for Information (RFI) on “Coordinating Care from Out-of-State Providers for Medicaid-Eligible Children with Medically Complex Conditions.”<sup>1</sup> IGT represents innovators, patient advocacy groups, and scientific, medical, and academic stakeholders seeking to actualize the promise of gene therapies. The overarching goal behind the creation of IGT is to translate the value of transformative therapies for patients, caregivers, the healthcare system, and society as a whole. IGT seeks to facilitate greater patient access and screening mechanisms, advocate for fair reimbursement pathways, and educate the public on the transformative nature of gene therapies.

The need for Medicaid, and the federal government in general, to enable access to gene therapies is self-evident: the Food and Drug Administration (FDA) has prioritized the acceleration of gene and cell therapy approvals and expects to receive over 200 investigational new drug applications (INDs) per year by 2020, adding to the 800 active gene and cell-based therapies currently on file.<sup>2</sup> By 2025, FDA anticipates that the agency will be approving 10 to 20 cell and gene therapy products a year.<sup>3</sup> The ability to facilitate proper patient access to gene therapies will grow in importance as the advancement of biotechnology pipelines result in increasingly specialized treatment into rarer diseases, particularly for the Medicaid population. Medicaid will be a large payer of gene therapies because many rare diseases impact pediatric patients. Thus, addressing barriers to coordinating care for Medicaid children with medically complex conditions is crucial.

To that end, IGT’s comments focus on care coordination best practices, regulatory and access barriers, and education needs for caregivers regarding out-of-state coverage parameters. The comments are based upon the collective feedback of our members who have gained significant insight as to the challenges and barriers preventing patients from accessing innovative gene therapies that hold the potential to dramatically influence their quality of life.

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<sup>1</sup> 85 Fed. Reg. 3,330 (Jan. 21, 2020).

<sup>2</sup> *Statement from FDA Commissioner Scott Gottlieb, M.D. and Peter Marks, M.D., Ph.D., Director of the Center for Biologics Evaluation and Research on new policies to advance development of safe and effective cell and gene therapies*, FOOD & DRUG ADMIN. (Jan. 2019), <https://www.fda.gov/news-events/press-announcements/statement-fda-commissioner-scott-gottlieb-md-and-peter-marks-md-phd-director-center-biologics>.

<sup>3</sup> *Id.*

## I. Care Coordination Best Practices

Care coordination for out-of-state care in the Medicaid program is pivotal to actualizing the potential of gene therapies for children with medically complex conditions. Without appropriate care coordination, undue delays in access to treatment can result in detrimental impacts on patients and their families, including increased morbidity and mortality. Both the Medicaid and CHIP Payment and Access Commission (MACPAC) and American Academy of Pediatrics have recognized the need for updating and streamlining out-of-state care for children with medically complex conditions.<sup>4</sup> Furthermore, care coordination in Medicaid is critical for children in rural areas, where there are significantly lower rates of specialists.<sup>5</sup>

To facilitate better care coordination for out-of-state care for children with medically complex conditions, CMS should take steps to reduce administrative, fiscal, regulatory, and access barriers for families; make it easier for providers to enroll, obtain adequate reimbursement, and provide care for out-of-state children; and improve transparency and education for beneficiaries, CMS, States, and manufacturers alike. IGT urges CMS to integrate best practices for care coordination into its State Medicaid Program and Medicaid Managed Care Organizations (MCOs) guidance. These programs should adhere to these best practices to facilitate access to care for children with medically complex conditions.

There are well-established best practices for care coordination that CMS should integrate into its guidance for encouraging the enhanced and efficient care coordination of medically complex children.<sup>6</sup> For example, CMS should:

- Require State Medicaid Programs and MCOs to cover and reimburse treatment provided by an out-of-state specialist or treatment center where the specialist or treatment center is properly enrolled in their home State Medicaid Program or Medicare, without further screening or enrollment burdens and barriers.
- Standardize timeframes and processes for approving prior authorization requests related to out-of-state care, as well as develop uniform documentation that is accessible to both caregivers and providers for making such requests.
- Require State Medicaid Programs and MCOs to offer travel and accommodation assistance when traveling more than 100 miles from home for care and clarify situations in which manufacturers may provide travel and lodging assistance in compliance with laws and regulations.
- Prohibit Medicaid-enrolled providers in the child's home state from retaliating against patients who receive out-of-state care.

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<sup>4</sup> Medicaid & CHIP Payment and Access Commission, *Issue Brief: Medicaid Payment and Policy for Out-of-State Hospital Services* (Jan. 2020), <https://www.law.georgetown.edu/wp-content/uploads/2018/07/Rule-18-Handout-1.Secara-1.pdf>; Council on Children with Disabilities and Medical Home Implementation Project Advisory Committee, *Patient- and Family-Centered Care Coordination: A Framework for Integrating Care for Children and Youth across Multiple Systems*, 133 PEDIATRICS e1451, e1451-60 (2014).

<sup>5</sup> *Improving Healthcare Value in Rural America*, ALTRUM HEALTHCARE VALUE HUB Res. Brief 19 (Oct. 2017), <https://www.healthcarevaluehub.org/advocate-resources/publications/improving-healthcare-value-rural-america>.

<sup>6</sup> Sara S. Bachman, Meg Comeau, & Katharyn M. Jankov, *The Care Coordination Conundrum and Children and Youth with Special Health Care*, THE CATALYST CENTER: IMPROVING COVERAGE AND FINANCING OF CARE FOR CHILDREN AND YOUTH WITH SPECIAL HEALTH CARE NEEDS (Nov. 2015), <https://ciswh.org/wp-content/uploads/2016/03/Care-Coordination-Conundrum.pdf>; Robert Book & Douglas Hotlz-Eakin, *Prospects for Care Coordination for Children with Medical Complexity*, AM. ACTION FORUM (Nov. 10, 2016), <https://www.americanactionforum.org/research/14241/>.

- Develop guidance for follow-up visits and process for long-term monitoring by the child’s in-state provider.
- Create a mechanism for referrals between the out-of-state and in state care providers can assist with data collection, as well as outcomes measurements.
- Develop and disseminate uniform educational materials for caregivers and providers on accessing out-of-state care.

## II. **Eliminating Barriers to Accessing Timely Out-of-State Care**

IGT encourages CMS to develop guidance providing children with medically complex conditions expedited access to specialist care without regard to geographic considerations.<sup>7</sup> Model guidance would mandate State and Medicaid MCO coverage for out-of-state specialist care for children diagnosed with medically complex conditions. Such guidance would allow patients with rare diseases and complex conditions to access care through specialists or highly qualified treatment centers. The guidance should incorporate the following:

**Screening and Enrollment.** Treatment delays and barriers can be driven by overly burdensome screening and enrollment processes for out-of-state Medicaid providers. In order to eliminate duplicate screening and enrollment requirements, the guidance should explicitly stipulate that Medicaid programs and MCOs cannot require out-of-state specialists or treatment centers that are properly enrolled in their home State Medicaid program or Medicare to be subject to the screening and enrollment requirements of the State financing the out-of-state care. Streamlining the screening and enrollment requirements can diminish delays in access to care, as well as limit administrative burden. CMS can address additional screening and enrollment barriers by carving out or exempting out-of-state providers and treatment centers from any Medicaid MCO in-network requirements.

**Streamlined Communications and Guidance.** Nationally-based communications and guidance could address disparate messaging and policies among State Medicaid Programs and Medicaid MCO leaders. By streamlining the guidance and accompanying communication, CMS could help position more wide-ranging, innovative payment models across states. IGT is particularly concerned about the potential payer tactics, tools, and policies that ultimately delay patient access, such as prior authorization. The guidance should provide clear policies aligned with the FDA-approved label associated with gene therapies. Furthermore, CMS should standardize timeframes and processes for approving prior authorization requests related to out-of-state care.

**Appropriate Reimbursement.** As advancement in biotechnology pipelines result in specialized gene therapies that may potentially cure medically complex conditions adversely afflicting children in the Medicaid population, the ability to obtain proper reimbursement will be vital in facilitating access. Reimbursement concerns apply to both providers and states alike. CMS should create uniform guidance addressing reimbursement for out-of-state care to prevent states from providing insufficient payment or delinquent payments. Our members have had experience with states providing adequately prompt payment for out-of-state care, while others have experienced overdue or insufficient reimbursement.

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<sup>7</sup> Kate A. Stewart et al., *Care Coordination for Children with Special Needs in Medicaid: Lessons from Medicare*, AM. J. OF MANAGED CARE (Apr. 2018), <https://www.ajmc.com/journals/issue/2018/2018-vol24-n4/care-coordination-for-children-with-special-needs-in-medicare-lessons-from-medicare>.

**Federal Regulatory Barriers.** Other regulatory barriers such as the federal price reporting methodologies, FDA manufacturing communications guidelines, fraud, waste, and abuse provisions, and HIPAA privacy protections currently limit access to gene therapies. IGT encourages CMS to consider exploring alternative price reporting mechanisms for Best Price and Average Manufacturer Price (AMP) when associated with value-based arrangements for transformative therapies. IGT also urges CMS to provide clarifying guidance on how manufacturers can incorporate Medicaid value-based arrangements in their price reporting calculations. In addition, CMS should modernize its review of manufacturer data to facilitate immediate patient access upon FDA-approval.

### III. Supporting Caregivers by Providing Information on Accessing Out-of-State Care

Caregivers attempting to coordinate care for children with medically complex conditions may lack essential information for navigating the intricate process of receiving out-of-state care via Medicaid: “depending on the severity of the child’s illness or condition, families can spend considerable time communicating among providers and across systems, assimilating recommendations, coordinating appointments, addressing insurance and financial issues, or performing therapeutic activities. In addition, families often have to juggle these tasks while caring for additional children in the household.”<sup>8</sup> Therefore, CMS should develop uniform educational materials for caregivers regarding out-of-state benefits and coverage available for children with medically complex conditions. Such educational materials may include newsletters, websites, mailings, and telephone calls. To strengthen the continuum of care, CMS should also communicate any changes in policies or processes as soon as possible to States and MCOs.

In addition, caregivers often face significant financial burdens due to the required travel for children to visit out-of-state providers. This financial burden is exacerbated by varying Medicaid benefits from state to state. To provide more certainty to caregivers, CMS should require that states post clear, accessible guidance related to Medicaid patients’ travel benefits, especially for those with medically complex conditions. States should explicitly detail the travel benefits available to Medicaid and CHIP patients. CMS should also consider the expansion of out-of-state care benefits to incorporate other caregiver necessities, such as childcare for siblings of the afflicted patient, or transportation for regular clinical trial site visits. Furthermore, CMS should create a uniform process for States and MCOs to obtain reimbursement for non-medical expenses, including travel and accommodation costs. These requirements should be publicly posted and available on state Medicaid websites, as well as CMS websites.

### Conclusion

IGT is grateful for the opportunity to provide feedback on this RFI and looks forward to collaborating with CMS to facilitate access to gene therapies for medically complex children. IGT encourages CMS to promulgate best practices and establish clear, national guidelines to streamline patient and caregiver access to specialist care. We would be pleased to answer any questions regarding the issues raised above.

Respectfully,

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<sup>8</sup> Dennis Z. Kuo, et al, *Care Coordination for Children With Medical Complexity: Whose Care Is It, Anyway?*, 141 PEDIATRICS S224, S224-32 (Supp. 3) (2018).