November 15, 2017

Tim Boyd, MPH
Director of State Policy
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Felicia F. Norwood, Director
Department of Healthcare and Family Services
201 South Grand Avenue
East Springfield, Illinois 62763-0002

Re: Medicaid Patient Access to Treatment for Spinal Muscular Atrophy (SMA) and Other FDA-Approved Rare Disease Treatments

Dear Director Norwood:

On behalf of the 1-in-10 Illinois residents with one of the nearly 7,000 known rare diseases, the National Organization for Rare Disorders (NORD) writes in regard to the Medicaid prior authorization requirements for nusinersen (brand name Spinraza), a treatment for Spinal Muscular Atrophy (SMA). NORD is a unique federation of voluntary health organizations dedicated to helping people with rare "orphan" diseases and assisting the organizations that serve them. We are committed to the identification, treatment, and cure of rare disorders through programs of education, advocacy, research, and patient services.

Last month, NORD was contacted by our Illinois-based member organization, Cure SMA, with concerns that Healthcare and Family Services' (HFS) prior authorization requirements for nusinersen may be excluding SMA patients in need of treatment by restricting the approval to certain disease subtypes counter to the FDA approved indication.

NORD recognizes that prior authorization and other formulary utilization measures can promote the use of lower cost generic medicines by patients, and therefore help lower overall health care costs. However, nusineren is the first ever treatment for SMA approved by FDA, and there are no therapeutically equivalent versions of it available for patients to take. As the agency noted in granting approval for this medicine, "[t]here has been a long-standing need for a treatment for spinal muscular atrophy, the most common genetic cause of death in infants, and a disease that can affect people at any stage of life."[1] Given these circumstances, restricting use of nusineren to only certain disease subtypes counter to FDA indication for adult and pediatric SMA patients serves only to reduce costs by restricting patient access to a medically necessary treatment.

In order to remedy this issue, NORD urges HFS (via its Drug Utilization Review Board) to consult with patient groups like Cure SMA and other disease experts to ensure that Medicaid patients with SMA are not denied access to medically necessary treatment. As a national umbrella organization for rare diseases, NORD can assist in this matter by facilitating contact with appropriate patient groups and disease experts.

[1] FDA. *FDA approves first drug for spinal muscular atrophy.* Dec. 2016. https://www.fda.gov/newsevents/newsroom/pressannouncements/ucm534611.htm

HFS Concerns Regarding Medications Approved Via FDA Accelerated Approval

In addition to the state's specific actions regarding nusineren, NORD is aware that HFS is broadly concerned about its role in providing access to breakthrough medications approved by FDA via its Accelerated Approval pathway. As an organization that has worked closely with FDA and Congress to improve approval pathways for breakthrough treatments, NORD can shed light on the Accelerated Approval pathway in regard to the safety and effectiveness of new drugs to treat rare diseases.

Accelerated Approval was created to facilitate new treatment options for serious conditions that fill an unmet need by analyzing "surrogate endpoints" when it is not possible to analyze more traditional indicators. There is immense difficulty in developing and evaluating therapies for small patient populations, such as rare disease populations. It is impossible to conduct large-scale, randomized, placebo controlled trials within rare diseases as there simply are not enough patients to participate. With Congressional approval, FDA has implemented innovative methods to evaluate orphan therapies. Without these unique tools for FDA to evaluate orphan therapies, individuals with rare diseases would be left without any treatment, because standard clinical trials would be impossible to conduct.

The use of surrogate endpoints is one these innovative tools. These endpoints are clinical indications of patient health used to determine drug effectiveness. For example, every treatment for HIV/AIDS on the market was approved using a surrogate endpoint (HIV viral load and patient CD4 count), because it was not possible to identify an underlying clinical endpoint. Other examples include the use of blood pressure and cholesterol to examine the effectiveness of medications to treat heart disease. As with these examples, the surrogate endpoints used to approve breakthrough treatments for rare diseases must demonstrate substantial quality-of-life improvements over other available treatments (or no treatment at all).

If Illinois and other states reject the rigorous process used by FDA to evaluate breakthrough treatments, the net effect is to turn back the clock to a time in which patients with rare diseases have no role in determining what is best for their own health and little hope for new medical breakthroughs to fight their disease. Before making judgements on which patients should or should not benefit from new medicines, we implore HFS and other agencies to better understand FDA's process for approving breakthrough treatments and facilitate better engagement with rare disease patients and the organizations that represent them.

Thank you for your consideration of this request. Please feel free to contact me at tboyd@rarediseases.org. Maria Spencer, Cure SMA's Vice President of Policy and Advocacy, can be reached at maria.spencer@curesma.org

Sincerely,

Tim Boyd, MPH
Director of State Policy