October 20, 2017

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Director of State Policy
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The Honorable Seema Verma, Administrator
Centers for Medicare & Medicaid Services
200 Independence Avenue, SW
Washington, DC 20201

Re: Massachusetts 1115 Demonstration Amendment Request

Dear Administrator Verma:

On behalf of the 30 million Americans with one of the estimated 7,000 known rare diseases, the National Organization for Rare Disorders (NORD) submits the following comments on the proposed Amendment to MassHealth Section 1115 Demonstration.

NORD is a unique federation of voluntary health organizations dedicated to helping people with rare "orphan" diseases and assisting the organizations that serve them. Since 1983, we have been committed to the identification, treatment, and cure of rare disorders through programs of education, advocacy, research, and patient services.

NORD recognizes the immense challenge facing Massachusetts to control health care costs in its Medicaid program in order to meet the needs of patients. However, after reviewing the proposed Demonstration Amendment and consulting with our member organizations, we are concerned that specific provisions of the Amendment will completely block patient access to breakthrough medical treatments for rare diseases approved by the Federal Drug Administration (FDA), particularly for medicines approved through the FDA Accelerated Approval Program. Further, the Amendment may create both short- and long-term disruptions in care for rare disease patients in the state currently receiving coverage through MassHealth. Based on these concerns, NORD strongly urges the Centers for Medicare and Medicaid Services (CMS) to reject the provisions of the Massachusetts waiver outlined below.

I. A Closed Formulary in MassHealth Will Block Patient Access to Breakthrough Treatments While Likely Failing to Have a Significant Impact on Cost Savings

The proposed Amendment seeks to institute a policy to, "[s]elect preferred and covered drugs through a closed formulary that assures robust access to medically necessary drugs."[1] Under the proposal, MassHealth will establish a "commercial-style" closed formulary that will only provide access to a single drug per therapeutic class and will exclude medicines that MassHealth determines offer limited or inadequate efficacy. The enactment of these changes would have a

[1] MassHealth Section 1115 Demonstration Amendment Request Sec. 4

devastating impact on the health and well-being of rare disease patients. NORD has seen firsthand how "commercial-style" formulary restrictions overrule the prescribing decisions of physicians; resulting in patients being unable to access the medicines best suited to treat their condition. As a result, such restrictions inhibit quality care by causing lapses in medication adherence and delays in use of medicines that provide an enhanced clinical benefit.[2] Over time, this will not only result in poorer health outcomes for MassHealth beneficiaries, but raise health care costs for the state.

Further, the underlying assumptions supporting the use of a closed formulary – that it will significantly lower costs – is not borne out by recent research analyzing the impact of orphan medications used to treat rare diseases on overall health care spending. Nationwide, the volume of prescriptions for orphan drugs is relatively low because of the small patient populations. The orphan drug share of the total volume of pharmaceutical use in the U.S. was just 0.3% in 2016. Additionally, nationwide spending on orphan drugs accounted for only 7.9% of all purchases.[3] Looking specifically at the Medicaid program in 2016, spending on rare disease medicines accounted for only 1% of all Medicaid spending.[4]

II. The Proposal to Prohibit Access to Medicines Approved Via FDA's Accelerated Approval is Potentially Catastrophic for the Health of Rare Disease Patients

We are incredibly concerned with Section 4b of this waiver which proposes to, "[e]xclude from the formulary drugs with limited or inadequate evidence of clinical efficacy."[5] In this proposed policy, MassHealth is specifically targeting, "drugs coming to market through the FDA's accelerated approval pathway."

There are many problems with this proposed policy, and we urge CMS to reject this harmful proposal. First, the Proposed Amendment's statement that drugs that "have not yet demonstrated clinical benefit and have been studied in clinical trials using only surrogate endpoints," are not incredibly impactful on the lives of individuals with rare diseases is facile and misguided. Therapies that do not meet their primary endpoints still must show efficacy in order to be approved by FDA, and often offer substantial quality-of-life improvements over other alternatives.

Second, we find it troubling that Massachusetts believes that the University of Massachusetts Medical School is better equipped to assess safety and effectiveness of therapies than FDA. To create a parallel, substandard, and unnecessary drug evaluation process entirely ignores the

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[2] Streeter, S.B., Schwartzberg, L., Husain, N.,Johnsrud, M. "Patient and plan characteristics affecting abandonment of oral oncolytic prescriptions." American Journal of Managed Care. 2011. 175 (5 Spec No.): SP38---SP44.

[3] Trends in Orphan Drug Costs and Expenditures Do Not Support Revisions in the Orphan Drug Act: Background and History. National Organization for Rare Disorders. October 2017. https://rarediseases.org/wp-content/uploads/2017/10/NORD-IMS-Report_FNL.pdf

[4] Coverage of Rare Disease Therapies in Medicaid and Medicare and the Impact on Patient Care. Jay Greissing, Dir. U.S. Government Relations and Policy, Shire. February 2016. http://www.cbinet.com/sites/default/files/files/Greissing_Jay_pres.pdf

[5] MassHealth Amendment Request Sec. 4b

unique capabilities of FDA to evaluate a therapy's safety and effectiveness. By allowing Massachusetts to create its own individual review process for evaluating efficacy, CMS will essentially be casting a vote of no-confidence for its own fellow Health and Human Services (HHS) agency, FDA.

Third, Massachusetts claims that section 4b is necessary to, "avoid exorbitant spending on high-cost drugs that are not medically necessary." To claim that the very first therapy for a rare disease is "not medically necessary" is erroneous at best. In making this claim, Massachusetts is overlooking the plight of 95% of rare disease patients who are still hoping for the very first treatment for their disease. We implore CMS to consider the dire situation in which many individuals with rare disease patients find themselves.

Fourth, the Proposed Amendment asserts that, "the 21st Century Cures Act was intended to expedite the drug approval process by reducing the level of evidence required for drugs to reach the market."[6] This is patently false, and represents a gross misunderstanding of the 21st Century Cures Act. As an organization who partnered with the House Energy and Commerce Committee and the Senate Health, Education, Labor, and Pensions Committee in the creation and enactment of the law, we can assure CMS that nothing within the Cures Act, "reduce[s] the level of evidence required for drugs to reach the market."[7] FDA must still approve every drug and biologic based upon safety and effectiveness. The Cures Act simply expands the tools FDA is allowed to use in order to assess whether a drug is safe and effective. We supported this legislation because we believe these new tools will actually improve the therapies that are approved by FDA by making them more reflective of what patients truly need in a therapy.

Fifth, Massachusetts is overlooking the unique difficulty in developing, testing, and evaluating therapies for small patient populations. It is impossible to conduct large-scale randomized, placebo controlled trials within rare diseases as there simply are not enough patients to participate. Congress has recognized this obstacle by authorizing FDA to use innovative methods to test and 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. In this section, Massachusetts is completely overlooking the distinct characteristics of small rare disease patient populations, and therefore is threatening their access to treatment.

Ultimately, the net effect of section 4b of this waiver is to turn back the clock to a time in which government bureaucracy decides what is in the best interest of the rare disease patient population without any consideration or consultation with the individuals whose health and wellbeing hang in the balance. We implore CMS to reject this harmful proposal, and reaffirm the importance of the role of FDA in approving therapies for those in need.

[6] Ibid.
[7] Ibid.

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III. The importance of Medicaid Coverage for Rare Disease Patients

Medicaid has long been a lifesaving source of health care coverage for rare disease patients in states wherein they cannot access other forms of coverage. We believe the proposed Demonstration Amendment would threaten this coverage in several ways:

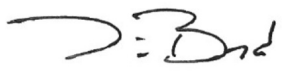
First, it will transfer non-disabled adults with incomes greater than 100% of the Federal Poverty Level (FPL) to Connector plans. While some rare disease patients may be exempt from this change as a result of their disabled status, many others would be transitioned off the program because their disease is either effectively managed or has not yet resulted in severe symptoms. This transition could result in several short and long-term disruptions in care, such as patients losing coverage for their preferred provider, losing coverage to a specialist for their specific rare disease, and experiencing an unintended increase in cost sharing or premiums that results in a drop in coverage. As written, the Amendment does not specify how to address these eventualities beyond describing that, "[i]n addition to our own direct outreach efforts, MassHealth and the Health Connector plan to provide small grants to community organizations and providers for outreach and enrollment activities for this transition."[8]

Second, the Amendment proposes to enroll non-disabled parents and caregivers with incomes up to 100% FPL in MassHealth's CarePlus Alternative Benefit Plan and block non-disabled adults with access to affordable employer-sponsored insurance (ESI) from enrolling in Medicaid. In addition to the aforementioned care disruptions that such changes will cause, this proposal does not satisfactorily take into account the unique needs of certain patient populations that would see a medical benefit from enrolling in MassHealth over an employer-sponsored plan.

Finally, the Amendment seeks to narrow available physician networks in order to promote the use of Accountable Care Organizations (ACOs) and Managed Care Organizations (MCOs). While there are potential benefits for this change to ensure better care coordination and outcomes for all MassHealth enrollees, it is critical that MassHealth make accommodations for the unique situation of rare disease patients who often struggle to find a physician with knowledge of their disease. Without enhancing specific procedures to ensure rare disease specialists can participate in MassHealth's ACO and MCO structures, many patients will suffer a lapse in care.

Thank you for the opportunity to provide comments on Massachusetts' proposed Amendment to MassHealth Section 1115 Demonstration. Once again, NORD urges you to reject the provisions of the proposed Amendment detailed in this submission.

Sincerely,



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[8] Ibid. at Sec. 3.