



August 21, 2017

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Marylou Sudders  
Secretary of Health and Human Services  
One Ashburton Place, 11th Floor  
Boston, MA 02108

*Transmitted via email to [kaela.konefal@state.ma.us](mailto:kaela.konefal@state.ma.us) in the EOHHS Office of Medicaid*

**Re: 1115 Demonstration Amendment Request (Public Comment)**

Dear Secretary Sudders:

On behalf of the 1-in-10 Massachusetts residents with one of the nearly 7,000 known rare diseases, the National Organization for Rare Disorders (NORD) thanks the EOHHS for the opportunity to provide comments on its 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. We are committed to the identification, treatment, and cure of rare disorders through programs of education, advocacy, research, and patient services.

NORD recognizes the immense challenges facing HHS to control health care costs in order to meet the needs of Massachusetts patients, especially given uncertainty around ongoing federal efforts to reform the Medicaid program. However, after reviewing the proposed Demonstration Amendment and consulting with our member organizations, NORD is concerned that specific provisions of the Amendment will create both short and long-term disruptions in care for rare diseases patients in the state currently receiving coverage through MassHealth. Further, the proposal to adopt a closed formulary and supplement the Food and Drug Administration's (FDA) determinations about the safety and efficacy of medicines poses a specific threat to rare disease patients who are benefitting from FDA's accelerated approval of breakthrough treatments.

**I. The importance of MassHealth Coverage for Rare Disease Patients**

MassHealth has long been a lifesaving source of health care coverage for rare disease patients in the state who 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.”

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 to 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 EOHHS 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.

## **II. A Closed Formulary in MassHealth Threatens Patient Access to Treatment**

The proposed Demonstration Amendment seeks to institute a “commercial-style” closed formulary that only provides access to a single drug per therapeutic class and would exclude medicines that the state determines offer limited or inadequate efficacy. The enactment of these changes would have a 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 thus 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. Over time, this will not only result in poorer health outcomes for MassHealth beneficiaries, but will raise health care costs for the state.



Further, NORD is troubled by the HHS' proposal to institute the state's discretion as to the clinical efficacy of medicines above and beyond that of FDA, particularly for medicines that received an accelerated approval. The accelerated approval of new breakthrough medicines has enabled rare disease patients to benefit from research into diseases where no treatment currently exists. These approval decisions are made in close consultation with patients, expert advisory committees, and manufacturers to determine whether a new medicine meets the specified clinical end-points for approval. At this time, we do not believe that MassHealth has the capacity or expertise to overrule FDA decisions regarding the safety and efficacy of new medicines.

Thank you once again for the opportunity to provide comments on the proposed Amendment to MassHealth Section 1115 Demonstration. For questions on these comments, please contact me at [tboyd@rarediseases.org](mailto:tboyd@rarediseases.org).

Sincerely,

A handwritten signature in black ink, appearing to read "T. Boyd".

Tim Boyd, MPH

Director of State Policy