January 17, 2018

Tamara Syrek Jensen, JD
Director, Coverage and Analysis Group
Centers for Medicare and Medicaid Services
Department of Health and Human Services
7500 Security Boulevard
Baltimore, MD 21244

RE: Comment on NCD entitled "Next Generation Sequencing (NGS) for Medicare Beneficiaries with Advanced Cancer" (CAG #00450N)

Dear Ms. Jensen,

On behalf of the 30 million Americans with one of the approximately 7,000 known rare diseases, the National Organization for Rare Disorders (NORD) would like to thank the Centers for Medicare and Medicaid Services (CMS) for the opportunity to provide comments on the proposed National Coverage Determination (NCD) entitled "Proposed Decision Memorandum on Next Generation Sequencing (NGS) for Medicare beneficiaries with Advanced Cancer".

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

Next Generation Sequencing (NGS) represents one of the most promising methods for diagnosing individuals with rare, genetic disorders. For many, if not most, rare diseases, NGS is the only reliable method for diagnosis. The advent of NGS technology has brought hope to millions of Americans with rare diseases still waiting for an accurate diagnosis. On average, individuals with a rare disease wait seven to ten years to obtain an accurate diagnosis, leaving many individuals with chronic conditions still waiting for a diagnosis. There are millions of patients in the U.S. who are still undiagnosed, and NGS may be their only hope.

As the voice of the rare disease patient community, we have two concerns with the NCD as proposed. First, we are alarmed that the proposal could potentially severely limit access to NGS based tests for our patients by creating an arbitrarily narrow eligibility for FDA cleared or approved NGS based tests. We anticipate this narrow eligibility for coverage could severely impede the development and access to these critical diagnostics.

In addition, this NCD sets an explicit policy of non-coverage for NGS based tests that did not receive FDA approval or clearance. This is incredibly problematic for several reasons.

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The regulation of NGS, particularly lab-developed NGS, is still evolving. Over the last several years, various regulatory schemes have been proposed by the FDA, members of Congress, and outside stakeholders, yet none of them have been promulgated nor implemented by FDA. A very careful regulatory balance must be established in order to ensure lab-developed NGS tests are valid and reliable, while also not stifling innovative labs across the country from developing such tests. None of these proposals as of yet have successfully found the equilibrium.

Since this balance has yet to be established, it makes little sense to us to finalize an NCD that relies on an essentially nonexistent regulatory scheme. Declining coverage for all NGS based tests that have not received FDA approval would result in a vast number of innovative diagnostics for Medicare beneficiaries with rare diseases going uncovered.

We ask CMS to consider the millions of Americans with a rare disease still searching for a diagnosis as it considers finalizing of this National Coverage Determination.

NORD thanks CMS for the opportunity to comment, and we look forward to working with CMS on ensuring that rare disease patients receive timely diagnoses and appropriate care. For questions regarding NORD or the above comments, please contact me at pmelmeyer@rarediseases.org or 202-545-3828.

Sincerely,

Paul Melmeyer
Director of Federal Policy