January 31, 2020

Commissioner Andrew Saul
Social Security Administration
6401 Security Boulevard
Baltimore, MD 21235-6401

Re: Notice of Proposed Rulemaking on Rules Regarding the Frequency and Notice of Continuing Disability Reviews, Docket No. SSA-2018-0026, RIN 0960-AI27

Dear Commission Saul:

On behalf of the 25 to 30 million Americans with one of the over 7,000 known rare diseases, the National Organization for Rare Disorders (NORD) thanks the Social Security Administration (SSA or Agency) for the opportunity to provide these comments on the Agency's proposed rule, *Regarding the Frequency and Notice of Continuing Disability Reviews* (CDRs).

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.

It is estimated that there are over 7,000 rare diseases, which are defined in the United States as diseases affecting 200,000 or fewer people. Today, over 90 percent of rare diseases still do not have an FDA-approved treatment indicated to treat the disease. Upon diagnosis, rare disease patients often find there are few medical experts who are available to treat them and limited research and information about their rare disease. Moreover, rare disease patients often require extensive, life-long medical care. Consequently, the benefits provided through SSDI or SSI and health care coverage through Medicaid and Medicare are often essential for people living with rare diseases.

Many people living with a rare disease who are on Supplemental Security Income (SSI) or Social Security Disability Insurance (SSDI) also have health care coverage through Medicaid and Medicare as a result. A significant portion of the rare disease patient community is already on or in the process of applying for SSI or SSDI.

As described in this proposed rule, the SSA conducts periodic CDRs in accordance with the diary category assigned to the person when the SSA determines the person has a disability. The current diary categories are Medical Improvement Expected (MIE) with a CDR between 6 and 18 months, Medical Improvement Possible (MIP) with a CDR at least once every 3 years, and Medical Improvement Not Expected (MINE) with a CDR between 5 and 7 years. After conducting a CDR, SSA may revise the diary category for future CDRs. SSA is proposing to add a fourth diary category, Medical Improvement Likely (MIL) with a CDR every two years. SSA also is proposing to increase the MINE CDR to at least once every 6 years.

Very little rationale is given to support adding a fourth diary category, and it is unclear how individuals would be assigned to the new MIL diary category versus the existing MIP. Under the current evaluation system, a person assigned to a MIP diary category could be reassigned to a MIE

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diary category after the first 3-year CDR, if their condition changed. Therefore, it does not seem necessary to add the MIL diary category. By adding a fourth, nebulously defined diary category, it increases the possibility that beneficiaries with rare diseases would be unduly burdened at best and lose their much-needed benefits and health care coverage at worst, which could result in devastating consequences.

The proposed rule seems to suggest that "specific impairments…*amenable to improving with treatment"* would be assigned to this new MIL diary category. However, the proposed rule does not discuss how one would interpret *"amenable to improving with treatment"* or justify why possible improvements as a result of treatment would not be more appropriate in the MIP diary category. Because the MIL diary category is not well defined in the proposed rule, it is unclear how many beneficiaries with rare diseases would be assigned, particularly if treatments are developed to help people living with rare diseases. Many rare diseases are heterogeneous, often depending on genetic mutations and other factors. There is no "one size fits all" with any treatments. While one treatment may work well in one person, it may be less effective in another. Uncertainty is to be expected and, thus, it seems that the MIP – medical improvement *possible* – diary category would be the most appropriate for situations wherein a treatment for a disease is being developed.

Additionally, as set forth in the proposed rule, it is unclear whether rare disease patients who have comorbidities would be reassigned to the more frequent reviews in the MIL diary category. Even if a treatment emerges, comorbidities may persist and prevent significant improvement. Sickle cell disease (SCD) is just one example that illustrates this concern. In 2019, the Food and Drug Administration (FDA) approved two novel treatments for SCD.[1,2] Yet, as people with SCD age, they develop complications such as pulmonary hypertension or kidney damage.[3] Rare disease patients who have a progressive rare disease with a newly available treatment, but also have progression of a disease with comorbidities, should not be subject to the more frequent reviews mandated under the new MIL category.

More frequent CDRs under MIL could jeopardize the accessibility to benefits and health care that rare disease patients often need. Consider, for example, that SSDI benefits do not actually start until six months after SSA determines a person's disability to have begun.[4] Then, in most cases, it takes another two years to obtain Medicare coverage.[5] Thus, if assigned to the MIL diary category, it is possible a beneficiary with a rare disease would be undergoing a CDR fairly soon after SSDI benefits began and before Medicare coverage even started.

Another concern with the MIL diary category is the requirement that a CDR occur at least every two years. This increase in CDRs will likely be burdensome to many people living with rare diseases or their caregivers who will have to complete the full medical review or mailer form more frequently. As mentioned above, there are over 7,000 rare diseases, and even within a rare disease there is heterogeneity. Health care for a person living with a rare disease is often costly and very complex,

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[1] https://www.fda.gov/news-events/press-announcements/fda-approves-first-targeted-therapy-treat-patients-painful-complication-sickle-cell-disease

[2] https://www.fda.gov/drugs/drug-approvals-and-databases/drug-trials-snapshots-oxbryta

[3] Derebail, Vimal K., et al. Progressive Decline in Estimated GFR in Patients With Sickle Cell Disease: An Observational Cohort Study. American Journal of Kidney Diseases. July 2019 Volume 74, Issue 1, Pages 47–55. https://www.ajkd.org/article/S0272-6386(19)30007-1/pdf. Accessed on January 28, 2020.

[4] https://faq.ssa.gov/en-us/Topic/article/KA-01777

[5] https://www.ssa.gov/planners/disability/approval.html

involving many doctor visits and even hospitalizations. Therefore, the vast majority of patients with rare diseases will require a great deal of time and likely the assistance of a caregiver to complete the form. Consider a person living with pyruvate kinase deficiency (PK deficiency) for example. PK deficiency, which is a rare disease characterized by the premature destruction of red blood cells, often requires regular blood transfusions and sometimes a splenectomy.[6] At an FDA public listening session, NORD and FDA recently heard from some patients with PK deficiency who have to undergo blood transfusions monthly or more frequently.[7] If a person living with PK deficiency were then reassigned from the MIP to MIL diary category, they would have to report on their regular blood transfusions at least every 24 months. This would be a significant increased burden to these patients.

With more frequent CDRs under MIL, the additional administrative burden placed on patients with rare diseases, caregivers, and possibly health care providers will likely increase the amount of errors or incomplete forms. The end result will either be greater costs to persons with disabilities and SSA due to appeals or the discontinuance of benefits and health care coverage for persons with disabilities. Consider that under the current diary categories and CDRs, in FY 2015 almost 35 percent of determinations to cease benefits were overturned on appeal.[8] This is yet another reason why the addition of a fourth diary category poses great harm.

NORD is also concerned about the proposal to change the frequency of CDRs in the MINE diary category to at least every six years. Although there is no data available, we expect that a sizeable number of the 25 to 30 million people living with rare diseases will eventually fall under this category. The proposed rule provides muscular dystrophy as an example of an impairment that could lead to an assignment to the MINE diary category. Duchenne muscular dystrophy (DMD) is a rare type of muscular dystrophy that results in muscle atrophy. DMD is progressive. Most patients require the use of a wheelchair in their teenage years and develop heart and breathing problems while still young.[9] Given the progressive, debilitating nature of DMD, it is difficult to understand why patients with this rare disease or others within the MINE diary category would need to undergo more frequent CDRs. Shortening the CDR timeframe for patients in the MINE diary category will only serve to cause undue burden to patients, their caregivers, and health care providers, and possibly result in errors that threaten these patients' benefits.

Finally, an overarching concern about the proposed rule is that it would pose an undue burden on SSA and consequently result in delayed or denied benefits. SSA's Compassionate Allowances program provides for expedited consideration for disability benefits if a disease is on the Compassionate Allowances Conditions list. However, there are only 90 diseases on this list currently, so most rare diseases are not on the list.[10] According to the Disability Benefits Center, SSDI and SSI claims take, on average, about 18 months to be approved.[11] CDR backlogs seem to fluctuate, for example in FY 2014 there were 900,000 backlogged and in FY 2018 there were none.[12,13] If the proposed rule is finalized, SSA projects 2.6 million additional CDRs over 10 years. It is unclear if or

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[6] https://rarediseases.org/rare-diseases/pyruvate-kinase-deficiency/

[7] https://rarediseases.org/wp-content/uploads/2020/01/NRD-2029-Voice-of-the-Patient-Report-PKD_FNL-1.pdf

[8] https://www.ssa.gov/legislation/FY%202015%20CDR%20Report.pdf

[9] https://rarediseases.org/rare-diseases/duchenne-muscular-dystrophy/

[10] https://www.ssa.gov/compassionateallowances/conditions.htm

[11] https://www.disabilitybenefitscenter.org/faq/receiving-financial-help-when-disabled

[12] https://www.ssa.gov/legislation/FY%202014%20CDR%20Report.pdf

[13] https://www.ssa.gov/open/data/Periodic-Continuing-Disability-Reviews.html#cdrBacklog

how SSA resources would be shifted to address this increase in CDRs and what the implications might be on initial benefit claims. Any further delay in access to initial disability benefits could have a negative impact on corresponding access to Medicaid and Medicare health coverage. Such delays could be detrimental to people living with rare diseases who need these benefits and health care coverage.

With the addition of a new diary category and the proposed changes in the frequency of CDR reviews for diary categories, NORD is extremely concerned that this proposed rule could result in people living with rare diseases losing access to SSI or SSDI and, consequently, their health care coverage under Medicaid and Medicare. Given the almost certain negative impacts on people living with rare diseases who are in need of disability benefits and health care coverage, NORD urges SSA to withdraw this proposed rule. NORD thanks SSA for the opportunity to comment and looks forward to working with SSA to ensure that rare disease patients have access to needed financial and health care support. For questions regarding NORD or these comments, please contact me at rsher@rarediseases.org, or 202-588-5700.

Sincerely,

Rachel Sher
Vice President, Policy and Regulatory Affairs