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Allies in Rare Disease

APPLYING THE ALTERNATIVE UNIT REBATE AMOUNT FOR LINE EXTENSION DRUGS TO ORPHAN THERAPIES WILL HARM INNOVATION OF RARE DISEASE TREATMENTS

Issue: The Centers for Medicare & Medicaid Services ("CMS") has proposed a Medicaid drug rebate policy that will harm rare disease therapy innovation because it failed to coordinate with other operating divisions within the Department of Health and Human Services ("HHS") – particularly with the Food and Drug Administration ("FDA") and the National Institutes of Health ("NIH"). The regulatory proposal was made in January 2012 and is expected to be finalized this May.

- ***What is the plain language and intent of the statute?*** Congress amended the Medicaid Rebate Act to require an increased rebate on "line extensions" of oral solid dosage form drugs. The intent of this policy is to prevent manufacturers from avoiding a penalty rebate on drugs for which they have taken a price increase after only making a "*slight alteration*." For the purpose of the statute, a line extension is "a new formulation of the drug, such as en extended release formulation."

- ***How does the CMS proposal for the Medicaid Drug Rebate Program exceed the plain language and intent of the statute and conflict with the Orphan Drug Act?*** In implementing this policy, CMS has proposed a significantly broader definition of "line extension," so even "a new indication for an already marketed drug" would fall within its scope. Even more troubling and arguably more illogical, the agency has explicitly proposed to include "line extensions" to which FDA has granted "orphan" exclusive approval. The explicit inclusion of drugs with "orphan" exclusive approval is at odds with the congressional intent of the provision and in direct conflict with the Orphan Drug Act because a "line extension" drug that has received "orphan" exclusive approval would have had to demonstrate it is "*clinically superior*" to the previously approved version of the drug, which means FDA has determined it "provide[s] a s*ignificant therapeutic advantage* over and above that provided by [the initial brand]" in terms of safety, efficacy, or by making a major contribution to patient care.

- ***Why will this policy discourage innovation for rare diseases?*** Nearly 7,000 rare diseases lack an FDA-approved treatment. Because the CMS policy creates a potential Medicaid

outpatient drug rebate and 340B program discount of nearly 100% upon the FDA marketing approval of a "line extension" drug, it is an economic barrier that will make developing treatments for those 7,000 conditions more difficult in direct conflict with the purpose of the Orphan Drug Act. Moreover, the CMS policy directly undermines the efforts at FDA and NIH to encourage drug repurposing, which is playing an increasingly important role in the development of rare disease therapies.

- ***What is the solution?*** Restoration of the orphan drug exclusion from this Medicaid drug rebate policy would be consistent with the purpose of the Orphan Drug Act and demonstrate a very clear commitment by Congress to the rare disease patient community, nearly all of whom are still searching for an FDA-approved treatment. When Congress originally enacted the law, it excluded "orphan" designated drugs from the policy. Congress must protect innovation in rare disease therapies by restoring the "orphan" drug exclusion, which will give hope to the millions of Americans that lack an FDA-approved treatment for their rare disease.