Immediate and Long-Term Impact of Medicare Drug Price Negotiation Program on More Than 30 Million Americans with a Rare Disease

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Medicare Drug Price Negotiation Program (MDPNP)

For the first time, empowered by the Inflation Reduction Act of 2022 (IRA), the Centers for Medicare & Medicaid Services (CMS) will **negotiate the price of some prescription drugs**.

For too many Americans living with rare diseases, out-of-pocket prescription drug costs create significant financial barriers to access. NORD strongly supports key provisions in the IRA that will ensure that more rare disease patients on Medicare will be able to afford the life-altering therapies they need. Starting in 2025, the IRA caps Medicare beneficiaries annual out-of-pocket spending at $2000 a year and spreads out monthly out-of-pocket costs.

However, other aspects of the MDPNP **threaten to reduce the likelihood of manufacturers developing new and better therapies to treat rare diseases**. By making orphan drugs eligible for negotiation as soon as they are researched (i.e., received FDA designation) for a second disease, the law threatens vital orphan drug research.

In sum, the MDPNP presents both opportunities and risks for the more than 30 million Americans that make up the 1 in 10 people in the U.S. with a rare disease.

Future Patient Access to Life-Altering Therapies Threatened

POSITIVE: In recognition of the long-standing challenges associated with bringing rare disease therapies to market, the IRA **exempts certain rare disease drugs from price negotiation**.

CONCERN: The **orphan drug exemption is extremely limited** and it could disincentivize drug manufacturers from conducting research and development on a drug to treat additional rare diseases.

Under the IRA, certain drugs relied on by people living with a rare disease, specifically orphan drugs that treat exactly one rare disease, will be exempted from price negotiation. The law also permits manufacturers to further develop these rare disease drugs for additional population subgroups, such as children impacted by the same disease, without becoming subject to negotiation.

NORD's Next Steps to Ensure MDPNP Prioritizes Patient Access to Innovative Therapies

To ensure the pace of rare disease innovation is maintained, **Congess should permit orphan drugs to be excluded from drug price negotiation until research into a second disease leads to a second FDA approval.** Failing to do so quickly will result in manufacturers deprioritizing research and development of therapies for additional rare diseases.

NORD continues to work with CMS to prioritize the needs of people living with rare disease in the MDPNP.

Making prescription drugs affordable for patients: Ensuring patients benefit from annual and monthly out-of-pocket caps starting in 2025.

Giving patients a voice in the negotiation process: Supporting rare disease patient participation in the listening sessions CMS is hosting to better understand the value of the therapies to patients.

Saving rare disease patients money at the pharmacy counter: Encouraging CMS to include all negotiated drugs on favorable tiers in prescription drug formularies.

Alone we are rare. Together we are strong.®