



PATIENT ASSISTANCE PROGRAMS (PAPs): ESSENTIAL PATIENT ACCESS TO VITAL MEDICINES

Position Statement

Purpose

This paper sets out NORD's position on patient assistance programs (PAPs) related to the issue of increases in prescription drug spending and overall health care costs.

Introduction

Since 1987, NORD has operated patient assistance programs (PAPs) to help rare diseases patients to obtain life-saving or life-sustaining medications that they could not otherwise afford. NORD's programs (known collectively as RareCareSM Assistance Programs) provide eligible individuals medication assistance, as well as financial support with insurance premiums and co-pays, diagnostic testing assistance, and travel for consultation with disease specialists.

Recently, some health care analysts have broadly criticized PAPs as a significant cause of growth in prescription drug spending and overall health care costs.¹ These programs, they assert, artificially increase demand for certain branded medications and expensive treatments by supplanting the utilization of lower cost alternatives (such as generics).

Further, they argue that subsidies provided to individuals under the Affordable Care Act (ACA) greatly diminishes the need for PAPs. These criticisms inflate the systemic impact of PAPs on health care cost, while undervaluing the impact on individuals in need. Further, these critics demonstrate a fundamental lack of understanding of rare diseases for which treatment alternatives are scarce.

NORD Position

Charitable assistance programs do not inflate the cost of treatment – they ensure patient access and adherence to life-saving medications.

¹ <https://www.primetherapeutics.com/en/news/prime-insights/2016-insights/2016-03-08-coupons.html>

The demand for medical treatment exists independent of whether patients can afford it or not. Attributing demand for breakthrough medications to PAPs misses a critical fact: the vast majority of rare diseases do not have any other form of treatment available. In fact, only about 350 of the 7,000 rare diseases have an FDA-approved treatment at all. For those that do have a treatment, there is often only a single medicine available to them with no generic equivalent or less expensive alternative.

What's more, critics of PAPs seem to imply that all of the organizations that provide PAPs are financially motivated middlemen in a purely economic transaction designed to "game the system". This characterization is patently false for charitable PAPs provided by organizations like NORD, which offer a broad spectrum of expertise and assistance to patients that help them to obtain diagnosis and care in the beginning, as well as adherence to their treatment later.

NORD's RareCare Assistance Programs are patient-centric and designed to meet the unique aspects of the respective disorder to reduce the financial barriers to care for the rare disease community. NORD's Patient Services Representatives work closely with individuals (patients or caregivers), assessing patients' overall health care needs, their financial and insurance statuses and any other access concerns, offering supportive means for best-outcome-oriented, comprehensive care. NORD's representatives are highly engaged with the individuals and families whom we serve and are well-educated regarding their unique, and all too often devastating, medical needs.

Most rare diseases and disorders are chronic and/or life-limiting and for the small percentage that do have a treatment offering, NORD's primary intent is to ensure consistent access to these treatments for all appropriate and eligible patients. NORD's RareCare Assistance Program provisions are inclusive of but not limited to, medication copays/coinsurances/deductibles, premium assistance, diagnostic testing, physician visits, and assistive devices.

Ultimately, mission driven charitable PAPs like RareCare produce better health outcomes and lower overall costs by increasing patient adherence to their treatment.²

Patient Assistance Programs are designed to help those most in need.

After exhausting all other options (commonly including sacrifices to their other daily needs) patients turn to patient assistance in search of help. RareCare Assistance Programs are based primarily on financial eligibility; therefore these programs screen applicants through an extensive process in which patients are required to provide detailed medical and financial documentation to prove their eligibility. What's more, NORD's programs issue awards for up to 1 year, patient eligibility is monitored throughout the award period, and patients must reapply for assistance each subsequent year.

² "Patient Assistance Programs Increase Adherence to Oral Oncology Drugs." Express Scripts. January 12, 2016. <http://lab.express-scripts.com/lab/insights/adherence/patient-assistance-programs-increase-adherence-to-oral-oncology-drugs>

The Affordable Care Act has not eliminated the need for charitable PAPs.

The ACA has helped millions of people obtain health insurance who previously could not afford it or who were denied on the basis of having a pre-existing condition. Nonetheless, even with new protections on out-of-pocket limits and co-insurance, patients with a severe disease still face insurmountable cost-sharing and co-payment requirements that prevent them from accessing the treatment they need³. Charitable assistance programs make this coverage more meaningful by ensuring that it can actually be used to pay for incurred expenses.

For the rare disease community, the high cost of developing treatment for a small population means that the cost of their care will likely always be higher than the general population. In addition, specialists and providers who have an in-depth knowledge of rare disease are few and far between. Without charitable organizations like NORD to provide assistance to patients, fewer people with a rare disorder would be able to access and stay in care.

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The National Organization for Rare Disorders (NORD) is the leading independent 501(c)(3) organization representing all patients and families affected by rare diseases. NORD is committed to the identification, treatment and cure of all 7,000 rare diseases that collectively impact the lives of 30 million Americans, or 1 in every 10 people. NORD provides programs of advocacy, education, research, and financial assistance services to patients and families, as well as represents more than 250 disease-specific member organizations.

³ Claxton, G., et al. Payments for cost sharing increasing rapidly over time. Peterson-Kaiser Health System Tracker Insight Brief. April 12, 2016.