EXECUTIVE SUMMARY

Preparing for and Managing Rare Diseases

To discuss how to ensure timely access to orphan drugs and gene therapies for rare diseases, AMCP held a virtual multidisciplinary stakeholder forum from Sep. 8–10, 2020. Forum participants represented diverse sectors, including integrated delivery systems, health plans, pharmacy benefit managers (PBMs), employer groups, biopharmaceutical companies, patient advocacy organizations, health policy researchers, and consulting firms, and were tasked with:

- Identifying key barriers by stakeholders around planning for and managing rare disease therapies and proposing strategies to address these barriers;
- Determining relevant differences in how to manage one-time versus long-term therapies in managed care settings; and
- Recommending best practices that key stakeholders may apply to optimize rare disease for rare disease therapies.

Participants acknowledged complex challenges associated with rare disease therapies:

- **Financial impact and limited data**: Most stakeholders are concerned about the financial impact of rare disease therapies. Payers and providers are additionally challenged by limited data on their durability of effect and the potential disconnect between clinical trial data and Food and Drug Administration (FDA)-approved labeling.

- **Uncertainty around key factors**: Some employers and payers take a cautious approach to coverage of rare disease therapies due to uncertainty around the available evidence, durability of effect, predictability of the financial impact, and patient mobility. These factors also impact their interest in implementing value- or outcomes-based arrangements.

WATCH FOR FOLLOW-UP

The Partnership Forum was just the beginning of AMCP's efforts to find innovative and collaborative solutions to support timely access to orphan drugs and gene therapies for rare diseases. From here, we will:

- **Publish a proceedings document** on all findings and recommendations from the Partnership Forum in an upcoming issue of AMCP's *Journal of Managed Care & Specialty Pharmacy* and disseminate it widely to decision makers around the country.
- **Host a forthcoming webinar** to report these findings and recommendations.
- **Provide more education** around establishing coverage policies for rare disease therapies.
- **Support efforts to modernize the Medicaid Drug Rebate Program** and the Orphan Drug Act to reflect changing insurance and payment models as well as pharmaceutical innovations.
- **Continue discussions with Members of Congress** to support legislation that enhances and provides proactive and timely pharmaceutical information exchange for rare disease therapies.

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- **Patient populations:** Payers, employers, and manufacturers are challenged with identifying the right patients for treatment, supporting care coordination, and preventing missed uptake of beneficial interventions due to small patient populations and heterogeneity of rare diseases.

- **Health plan variations:** Patients, providers, and manufacturers are challenged by variations in health plan processes and coverage policies.

- **Segmentation and size:** Payer segmentation and size continue to be factors influencing rare disease treatment coverage.

Additionally, participants highlighted opportunities in several areas to address these challenges:

- **Financials/payment:** Attempt to standardize some components of value- or outcomes-based arrangements and share of best practices to benefit from economies of scale.

- **Outcomes:** Addressing both the FDA approval requirements and the payer evidence needs earlier in drug development process. Developing core outcome sets for rare disease treatments could be applied across payers and may offer a solution.

- **Communication:** Implement more transparent, timely, and truthful pre-approval communication between biopharmaceutical companies and population health care decision-makers to improve planning around investigational products for rare diseases.

- **Coverage:** Use all available evidence, including input from physician experts and patient advocates, real-world evidence, and multidisciplinary groups throughout the evidence review and coverage development process.

- **Collaboration/education:** Improve collaboration between the FDA, payers, clinical experts, patients, and patient advocacy groups to increase payer confidence in the FDA-approved label indication for determining appropriate populations to receive rare disease therapies.

- **Patient engagement:** Consider new patient incentive programs to support the collection of real-world evidence and long-term data.

- **Legislation/Regulations:** Continue efforts to modernize the Medicaid Drug Rebate Program and the Orphan Drug Act to reflect changing insurance and payment models as well as pharmaceutical innovations.