In the **sixth edition** of the Alnylam Rare Disease Trend Report, payers and employer stakeholders share perspectives and priorities for sustainably managing rare disease and specialty therapies.

Rare disease therapies require specialized development and face complex reimbursement challenges, as stakeholders balance innovation with affordability and access. Despite this, the rare disease landscape continues to expand, with orphan drugs consistently accounting for more than half of new molecular entities. Meanwhile, payers are facing mounting financial pressure from rising drug and medical costs, policy changes such as the Medicare Part D Redesign, and broader macroeconomic forces.

This report seeks to deepen understanding of these emerging trends and challenges by equipping stakeholders with strategic insights to navigate the evolving landscape.

Key questions answered within this report include:

- How have key stakeholder priorities related to covering and managing rare disease products shifted since this report began publication in 2020?
- How are increased financial pressures affecting benefit design and management of rare disease therapies?
- To what extent are payers and employers limiting procurement and distribution models for rare disease therapies to control costs?
- How are policy changes, such as the IRA Medicare Part D redesign, influencing payer and employer strategies for rare disease therapies?
- How are stakeholders leveraging innovative technology to enhance operational efficiencies and inform coverage decisions for rare disease therapies?

To read the full report, download a complimentary copy or contact an Alnylam account director.

Only with information and through communication can we work together to overcome access barriers for the betterment of humanity.

WHAT'S NEW

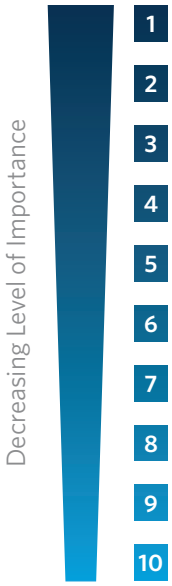
Previous editions of this report (2020, 2021, and 2022) focused exclusively on the budget impact of rare disease products on health plans. More recent editions (2023 and 2024) introduced the employer perspective on rare disease and specialty products given their critical role in coverage decisions. This year's report again integrates both payer and employer perspectives, with a focus on the impact of increasing economic pressures, recent policy changes, and resulting financial risk mitigation strategies. It also builds on previous editions by exploring the use of cross-benefit management, distribution models, and innovative technologies such as artificial intelligence.

2025 VALUE DRIVER IMPACT ON ACCESS DECISION-MAKING FOR HIGH-COST AND RARE DISEASE

CONTRACTING/ ECONOMIC FOCUSED

= No Change ▲ Increase YoY ▼ Decrease YoY

Note on Graph: 2025 YoY increase relative to 2024



2025	
EFFICACY	=
NET PRICE	=
SAFETY	=
UNMET NEED	+1 ▲
TA-SPECIFIC TREATMENT ALGORITHM	+3 ▲
TREATMENT COST VS. SoC	-2 ▼
DURABILITY	=
TOTAL BUDGET IMPACT	-2 ▼
DIRECT TREATMENT COST OFFSETS	+1 ▲
INDIRECT TREATMENT COST OFFSETS	-1 ▼

By sharing insights on current and emerging coverage and management trends, this report aims to advance dialogue around rare disease products. With a better understanding of how payers and employers perceive, evaluate, and prioritize the unique challenges of managing rare disease products, industry stakeholders can more readily adapt to market changes and help keep innovative therapies within reach.

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Alnylam Pharmaceuticals
675 West Kendall Street at Henri A. Termeer Square
Cambridge, MA 02142