epocrates®

Rare Disease Pharma Marketing

A growing market that requires a unique strategy.

Introduction

Living with a rare disease, or caring for someone with one, is a challenging journey marked by a never-ending search for new information, the right care and support—and plenty of uncertainty along the way. Patients often present with puzzling symptoms that many providers struggle to make sense of, and receiving a diagnosis can often take years. Even when a diagnosis is made, effective treatments can be scarce. Fewer than 5% of rare diseases have FDA-approved treatments, however, recent market trends are signaling a seismic shift.[1] In fact, rare disease treatments now account for 72% of new drug launches, up from 51% in 2019.[2] While increased investment in rare disease portfolios reflects a significant opportunity, pharma marketers face challenges unlike those in any other market—requiring a more nuanced approach to strategy, tactics, and messaging.

1 in 10 people in the U.S. live
with a known rare disease,
50% of whom are children.
—National Organization for Rare Disorders[1]

What Are Rare Diseases?

The Orphan Drug Act, passed by the U.S. Congress in 1983, defines a rare disease as *a disease or condition that affects less than 200,000 people.*[3] Rare diseases are also defined by their impact on people's lives.[4] This can include:

- The number of years to get a diagnosis.
- Symptoms that are often similar to many other conditions.
- Symptoms that can impair daily life, the ability to work and keep a job, manage household tasks, or get through the day.
- The lack of effective treatments.
- The financial hardship on patients and their families.
- The physical and mental exhaustion experienced by patients and caregivers.

To date, there are more than 10,000 known rare diseases, affecting more than 30 million people nationwide.[5-67] Those with rare diseases often face health inequities, have a decreased quality of life, work-related challenges, uncertainty, an emotional toll, and financial hardship.[7-10]

These populations often endure a long and protracted journey until they receive a diagnosis, commonly referred to as a diagnostic odyssey. In 2019, 36% of people who had been diagnosed with a rare disease received the diagnosis in the first year, while more than a quarter (28%) said it took 7 or more years.[11]

> *Living with a rare disease doesn't just mean getting treatments and seeing doctors. Sometimes living with a rare disease means having the strength to pull yourself up and face the world after a rough day of treatment, a re-occurrence, or a doctor's visit without answers. It can take years of perseverance to get a diagnosis and start treatment and sometimes there is no treatment available. Living with and caring for someone with a rare disorder requires strength, empathy, and courage.*
>
> *—Leigh P, rare disease patient*[12]

Orphan Drugs: A Unique, But Growing Market

Rare disease drugs are often referred to as *orphan drugs.* The term came about because some rare disease treatments have been "orphaned" or discontinued because there was not enough financial incentive to continue development or production.[6]

The Orphan Drug Act of 1983 incentivizes drug development for rare diseases.[3] Companies and drug developers can receive the orphan drug designation if the drug meets certain criteria. They also can receive certain incentives such as tax credits for qualified clinical testing, waiver of the Prescription Drug User Fee, which is currently at nearly $3 million for a new drug, and potential exclusivity for 7 years. The Act also established the Orphan Product Grants Program, which provides funding to develop new products.[13]

The Act has helped to make investment attractive for drug developers. The Act's incentives—including tax credits, fee waivers, and exclusivity—have led to low development costs, a high profit margin, and a monopoly on treatment of the disease. The average annual cost for orphan drugs is twenty-five times higher than non-rare disease drugs, approximately $123,000.[14] The gross profit margin for the rare disease industry is over 80%—significantly higher than the pharmaceutical company average rate of 16%.[14]

Although they are uncommon, rare diseases represent a large, rapidly expanding pharmaceutical market. These drugs doubled their market share in global prescription drug sales, increasing from less than 10% in 2014 to nearly 20% in 2023.[15] In 2024, orphan drugs accounted for 16% of global pharma revenue.[16] In the U.S., the market was estimated to be worth over $90 billion and reach $197.31 billion in 2032.[17]

Orphan drug approvals are on the rise. In 2024, 26 of 50, or 52% of novel drug approvals were for rare diseases, up from 17 a decade earlier.[18] Since the Orphan Drug Act was enacted, the FDA has approved more than 500 orphan drugs.[19] By 2026, orphan drugs are projected to account for roughly 20% of prescription drug sales—growing approximately twice the rate of nonorphan drugs.[20] Plus, investigational therapies for rare diseases are projected to make up nearly a third (29%) of total pharma pipelines by then as well.[20] Despite this growth, 95% of rare diseases do not have treatment options.[21]

The Rare Disease market is expected to reach

~$336B

globally by

2030.

— Recent Trends in Rare Diseases - Inizio

As the orphan drug market continues to grow, it's becoming an increasingly important growth strategy for pharmaceutical companies. Large biopharmaceutical companies are expected to market or co-market nine of the top 10 best-selling orphan drugs in 2026. Those nine are expected to have orphan drug sales account for between 16% and 39% of their projected sales in 2026.[22] Plus, the top 10 orphan drugs are projected to generate $57 billion in revenue in 2028.[23]

Challenges Abound For Rare Diseases

While the rare disease market has experienced tremendous growth, numerous barriers remain for providers, patients, and pharmaceutical companies.

Provider and patient issues

Rare diseases carry a unique set of obstacles for patients and their providers. According to one survey, the greatest challenges HCPs say they face today regarding the treatment and diagnosis of rare diseases include:[24]

44%	30%	29%	28%	27%
Delays in patient diagnosis	Lack of education for physicians	Shortages of physicians specializing in rare diseases	Lack of access	Lack of awareness of rare disease symptoms

Research funding

Historically, rare disease research and drug development have faced inconsistent government funding, and recent policy shifts and pauses in federal funding have added to these challenges.[25]

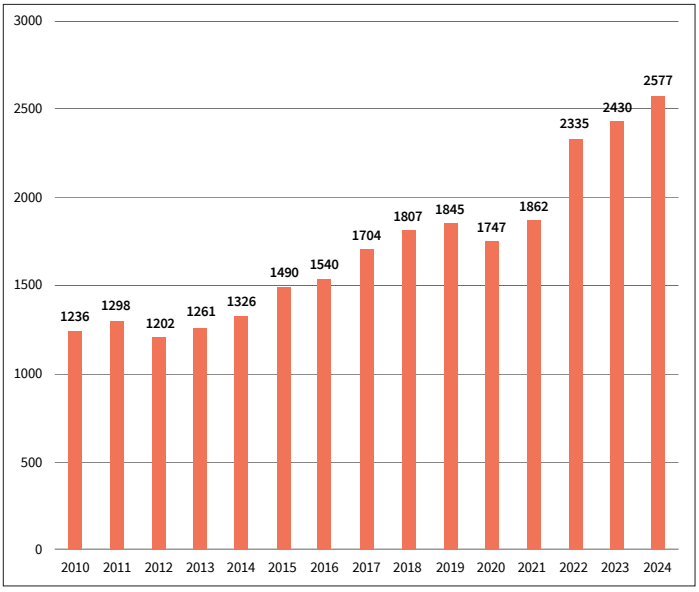
Plus, rare diseases that disproportionately affect communities of color frequently receive less research funding, which further exacerbates inequities.[26]

Clinical trials

Patient recruitment and retention for clinical trials have been a challenge for pharmaceutical companies. Small candidate pools, complex logistics, and specialized treatments make running clinical trials difficult for research sites, sponsors, and patients.[27]

To address these gaps, patient advocacy groups like the National Organization for Rare Disorders (NORD) can help find candidates as well as rare disease patient registries. Plus, Decentralized Clinical Trials (DCT) that use telemedicine, digital health technologies, and wearables, can deliver clinical supplies, ship collected specimens, and provide remote supervision—helping increase enrollment, improve patient diversity, and reduce costs.[28]

Rare Disease Clinical Trials, 2010-2024



Source: Trialtrove, January 2025

Inflation Reduction Act

The Inflation Reduction Act of 2022 has had a significant impact on rare disease drug pricing.[29] Since the Act protects orphan drugs from price negotiation, it was originally designed to incentivize companies to invest in rare disease indications. Yet it may have unintentionally disincentivized the development of drugs with multiple rare disease indications. The reason is that the IRA's orphan drug exclusion exempted drugs that are designated for only one rare disease or condition and approved for an indication (or indications) only for that disease or condition under the Medicare Drug Price Negotiation Program.[30]

The One Big Beautiful Bill, signed into law in July 2025, broadens and extends the orphan drug exclusion by allowing drugs with multiple rare disease indications to be exempt from the price negotiation program. It also delays price negotiations until the drug is approved for a non-orphan indication.[31-32] Experts say this change may encourage greater private investment in the rare disease space and increase the value of assets with multiple possible rare disease indications.[33]

Rare Disease as a Growth Strategy

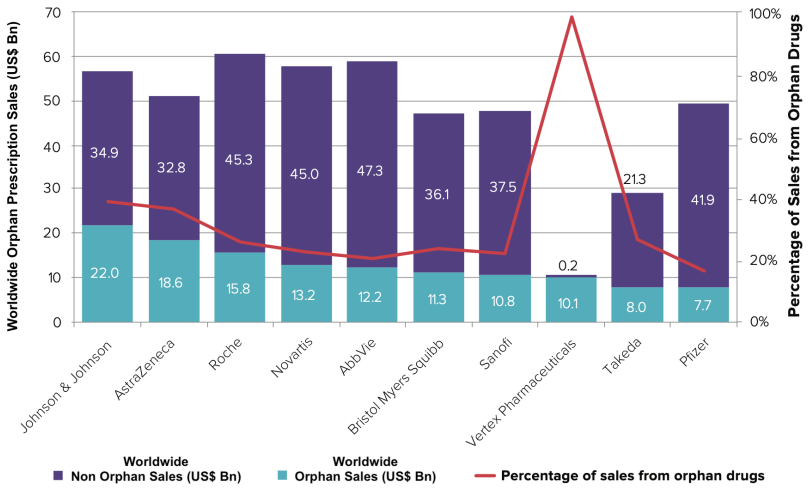
As a result of the demand for new treatments, lower investment costs, a higher probability for regulatory success, and quicker timelines, among other advantages, it's no surprise that pharmaceutical companies are making strategic moves to bolster their rare disease portfolios.[20]
In 2025, some moves included:

Novartis acquired Avidity Biosciences, Inc. for $12 billion, gaining a biopharmaceutical company focused on a new class of therapeutics enabling RNA delivery to muscle.[34]

Eli Lilly signed a deal with MeiraGTx Holdings, potentially worth over $475 million, granting it rights to an experimental gene therapy for a rare, inherited disorder that causes severe vision loss from birth.[35]

Denali Therapeutics, Inc. secured $275 million in funding for Tividenofusp alfa, its investigational TransportVehicle™-enabled enzyme replacement therapy for the treatment of mucopolysaccharidosis type II (MPS II, or Hunter syndrome).[36]

With brands accelerating investment in rare disease portfolios, we expect that pharma marketers will be prioritizing new strategies to keep pace, and focusing on targeted messaging about rare disease education, upcoming and now-approved drugs, and the mechanism of action (MOA) for new treatments.



Source: Evaluate Pharma, February 2022

epocrates®

Recently Approved Rare Disease Therapies

Pharma companies are increasing their investments in rare disease portfolios. Here are the some of the top drugs approved by the FDA last year.

Brand Name	Pharmaceutical Company	Disease	Projected Revenue in 2030/ Estimated peak sales
Grafapex	Medexus	Allogeneic hematopoietic stem cell transplantation	5-year revenue potential: $100MM
Gomekli	Springworks	Neurofibromatosis Type 1-associated Plexiform Neurofibromas	$824MM
Romvimza	Deciphera	Symptomatic tenosynovial giant cell tumor where surgery could cause severe problems	$150MM
Ctexli	Mirum	Cerebrotendinous Xanthomatosis	$128MM
Encelto	Neurotech	Idiopathic Macular telangiectasia type 2	Estimated peak sales between $250-400MM
Yutrepia	Liqudia	Pulmonary arterial hypertension and pulmonary hypertension associated with interstitial lung disease	Estimated peak sales between $685MM - $2BN
Sephience	PTC	Hyperphenylalaninemia	957MM. Estimated peak sales between $1.2 BN - $1.5BN
Modeyso	Jazz	H3 K27M-mutant diffuse midline glioma	$226MM
Dawnzera	Ionis	Hereditary angioedema	$430MM. Estimated peak sales of $500MM+
Palsonify	Crinetics	Acromegaly in patients with an inadequate response to surgery or are not surgical candidates	$1.14B. Estimated peak sales of $2.4BN

Considerations for Brand Marketers

Because the rare disease market differs fundamentally from mainstream therapeutic areas, it presents a unique set of challenges for pharma marketers.

Smaller patient populations

With lower prevalence and less geographic concentration, building brand awareness and reaching target audiences at scale is difficult.[50]

Limited awareness

HCPs, patients, and caregivers often have limited information about rare diseases and treatment options.

Lower marketing budgets

Developing and marketing therapies can be costly, and brands often have smaller budgets than those for more common conditions.

Prior authorization

Access to necessary treatments can stall due to manual processes, incomplete or inaccurate documentation, response time delays, inadequate peer-to-peer reviews, and more.[53] In fact, 94% of HCPs say prior authorization always, often, or sometimes delays patient access to necessary care.[54]

How to Develop Effective Campaigns

Given the unique challenges, rare disease marketing requires a differentiated strategy and nuanced approach. Here are some considerations pharma marketers should think about before launching a campaign.

Build awareness

Since rare diseases are, by definition, uncommon, brands must work harder to build awareness of both the condition and available treatment options among HCPs and patients.

Educate

As patients seek out the latest research, support, and recommendations, HCPs must be armed with information about patient advocacy groups, information centers, clinical trials, community resources, and upcoming treatments.[56]

42% of HCPs

say increasing provider education will have a positive impact on the rare disease space.

—Definitive Healthcare[57]

Accelerate access

To reduce prior authorization delays, brands should increase awareness about requirements, available tools, and support that can simplify the process. For example, checklists and sample clinical documentation can allow HCPs to receive approvals and speed up access to care.[50] Information about coverage, formularies, and patient support programs can also improve access.

Prioritize personalization

Campaign tactics should be focused on patient-centric content. For example, marketers may consider sharing patient stories that speak about the unique experiences and burden on patients and their caregivers. Storytelling should foster awareness, empathy, and the importance of community.

Drive engagement

Messaging must be highly relevant to effectively engage rare disease prescribers. Therefore, campaigns should include pre-approval messaging that aims to educate about symptoms and risk factors, identify potential patients, and promote emerging treatments. Campaigns with *now approved* messaging can point to clinical trial data to prove the clinical value, patient access programs, available support such as helplines, and mechanism of action (MOA). In fact, data from epocrates shows that rare disease prescribers have 45% higher engagement* on the following topics:[58]

- Dosing
- New Indication
- Safety
- Physician Resources
- MOA
- Patient Support
- Cost

Marketers who develop thoughtful campaign strategies and tactics that focus on awareness, education, and patient-centered messaging will effectively reach, engage, and support HCPs and their patients.

2026 and Beyond: Rare Disease Therapies in Development

Orphan drugs are increasingly gaining market share. Here are 10 of the top treatments by revenue currently in development.

Rare Disease Therapies in the Pipeline By Revenue[59-68]

Drug	Pharmaceutical Company	Disease	Estimated Peak Sales
Tividenofusp alfa (DNL310)	Denali	Hunter syndrome	~$546MM
Fazirsiran	Takeda + Arrowhead Pharmaceuticals	Alpha-1 antitrypsin deficiency-associated liver disease	~$4.1BN
Clemidsogene lanparvovec (RGX-121)	Regenxbio	Hunter syndrome	~$400MM
UX111 –	Ultragenyx + Abeona Therapeutics	Sanfilippo syndrome type A	~$540MM
DTX401	Ultragenyx	Glycogen Storage Disease Type 1a	~$700MM
AAV2-hAQP1	MeiraGTx	Severe xerostomia secondary to head & neck radiation	~$200MM - $1 BN
Cemdisiran	Regeneron + Alnylam Pharmaceuticals	Generalized Myasthenia Gravis	~$500MM
ST-920	Sangamo Therapeutics	Class and late-onset Fabry Disease	$350MM - $1.4 BN
WVE-N531	Wave Life Sciences	Boys with DMD who carry mutations suitable for exon 53 skipping	$250MM - $1.1BN

Engaging HCPs around Rare Disease Therapies

The rare disease landscape presents distinct challenges for treatment development and marketing, yet it offers significant revenue potential, representing a compelling growth opportunity for pharma companies. With small patient populations, lack of awareness, regulatory barriers, and limited marketing budgets, the rare disease market is complex and challenging. Yet for brands that take a thoughtful, nuanced approach that this unique market demands, they will undoubtedly come out ahead.

Before launching a strategy, pharma marketers must consider the characteristics, needs, and challenges of this market and develop campaigns that build awareness, provide education, reduce barriers, and effectively engage HCPs with the right messaging at the right time. Targeted campaigns delivered on specialized platforms that HCPs trust allow for meaningful experiences and foster greater awareness and potential adoption.

About epocrates

epocrates, an athenahealth company, is a trusted clinical intelligence platform, built to power clinical decisions across every stage of care. Designed for clarity, speed, and practical use, epocrates delivers essential drug and clinical insights at the point of care—helping healthcare professionals make confident, evidence-based decisions when it matters most. Our platform also offers opportunities for reaching clinicians through digital advertising.

Ready to learn more about digital advertising to HCPs on epocrates?

Scan the QR code or visit http://epocrates.com/discover/advertise.

Sources

1. Sroberts. (2025, December 23). Rare Disease Facts and Statistics | NORD. National Organization for Rare Disorders. https://rarediseases.org/understanding-rare-disease/rare-disease-facts-and-statistics/
2. MedPath - Medical Intelligence Platform. (2025, May 22). MedPath. https://trial.medpath.com/news/05f77dd2c80303fb/u-s-drug-prices-double-as-pharmaceutical-industry-shifts-focus-to-rare-diseases
3. Office of the Commissioner. (2024, November 21). Rare Diseases at FDA. U.S. Food And Drug Administration. https://www.fda.gov/patients/rare-diseases-fda
4. Rare and orphan diseases. (2025, June 2). Cleveland Clinic. https://my.clevelandclinic.org/health/diseases/rare-and-orphan-diseases
5. Rare Disease Diversity Coalition & National Organization for Rare Disorders. (2024). Inequities in the rare disease community: The voices of diverse patients and caregivers (RDDC survey report). Rare Disease Diversity Coalition. https://rarediseases.org/wp-content/uploads/2024/08/RDDC-Survey-Report.pdf
6. Office of the Commissioner. (2024, November 21). Rare Diseases at FDA. U.S. Food And Drug Administration. https://www.fda.gov/patients/rare-diseases-fda
7. Rare Diseases International. (2023, February 25). life with a rare disease - Rare Diseases International. https://www.rarediseasesinternational.org/living-with-a-rare-disease/
8. Mulrooney, L. (2022, June 18). Poor Health-Related quality of life, many burdens in rare diseases, study finds. AJMC - Managed Care News, Research, and Expert Insights. https://www.ajmc.com/view/poor-health-related-quality-of-life-many-burdens-in-rare-diseases-study-finds
9. Bougas, N., Kangas, T., Vanthomme, K., Solves, J. J. M., Brulé, G., Mellerio, H., Vandenheede, H., & Dumas, A. (2025). Employment and work ability in individuals living with rare diseases: a systematic literature review. Orphanet Journal of Rare Diseases, 20(1), 193. https://doi.org/10.1186/s13023-025-03691-7
10. The Translational Genomics Research Institute (TGen). (2024, December 20). The Unseen Struggles: How rare conditions affect the entire family. https://www.tgen.org/patients/tgen-center-for-rare-childhood-disorders/stories/the-unseen-struggles-how-rare-conditions-affect-the-entire-family/
11. National Organization for Rare Disorders. (2020, November 19). Barriers to rare disease diagnosis, care and treatment in the U.S.: A 30-year comparative analysis (Report No. NRD-2088). https://rarediseases.org/wp-content/uploads/2020/11/NRD-2088-Barriers-30-Yr-Survey-Report_FNL-2.pdf
12. Sroberts. (2024, February 9). Invisible Stripes: Leigh's Story in honor of Rare Disease Day - National Organization for Rare Disorders. National Organization for Rare Disorders.https://rarediseases.org/leighs-story-rdd/
13. Office of the Commissioner. (2024, October 1). Orphan Products Grants Program. U.S. Food And Drug Administration. https://www.fda.gov/industry/grant-programs-support-development-medical-products-rare-diseases/orphan-products-grants-program
14. AHIP. (2022, April 19). How big pharma makes big profits on orphan drugs. https://www.ahip.org/how-big-pharma-makes-big-profits-on-orphan-drugs
15. Van Arnum, P., & Van Arnum, P. (2024, August 2). Rare-Disease drugs: Is the market still trending upward? DCAT Value Chain Insights. https://www.dcatvci.org/features/rare-disease-drugs-is-the-market-still-trending-upward/
16. Inizio. (2026, January 2). Recent trends in rare diseases - inizio. https://inizio.com/insights/recent-trends-in-rare-diseases/
17. United States Orphan Drugs Market Size, Share | Trends Forecast 2032. (n.d.). Markets and Data. https://www.marketsandata.com/industry-reports/united-states-orphan-drugs-market
18. New drugs approved in the United States, by designations. (n.d.). Our World in Data. https://ourworldindata.org/grapher/new-drugs-approved-in-the-united-states-by-designations
19. The Orphan Drug Act: legal overview and policy considerations. (n.d.). Congress.gov | Library of Congress. https://www.congress.gov/crs-product/IF12605

Sources

20. Zs. (2025, July 25). Rare, but ready: Why the future of pharma runs through rare disease. https://www.zs.com/insights/rare-disease-pharma-innovation
21. Sroberts. (2024, May 28). Information on Clinical Trials & Research Studies | NORD. National Organization for Rare Disorders. https://rarediseases.org/living-with-a-rare-disease/clinical-trials/
22. Van Arnum, P., & Van Arnum, P. (2022, May 26). Market outlook for orphan drugs: Niche no more. DCAT Value Chain Insights. https://www.dcatvci.org/features/market-outlook-for-orphan-drugs-niche-no-more/
23. Evaluate. (2025, June 2). The New Report is Out: Are Orphan Drugs Losing their Sparkle? | Evaluate. https://www.evaluate.com/thought-leadership/orphan-drug-report-2024/
24. Definitive Healthcare. (n.d.). 2023 rare disease study. https://www.definitivehc.com/resources/research/annual-rare-disease-study
25. Khurana, N. (2025, June 16). Funding challenges in Rare Disease Research: How financial support can transform the Rare Disease Landscape — Rare Genomics Institute. Rare Genomics Institute. https://www.raregenomics.org/news-events-blogs/el9u8btf9pf30ol4yegelf6jdupvv2
26. Rare Disease Diversity Coalition. (2025). 2025 Achievement Report. https://www.rarediseasediversity.org/hubfs/RDDC/pdf/RDDC-Achievement-Report-2025.pdf?hsLang=en
27. Scrs. (2024, October 29). Enabling success in rare disease clinical trials. Society for Clinical Research Sites. https://myscrs.org/resources/enabling-success-in-rare-disease-clinical-trials/
28. Ghadessi, M., Di, J., Wang, C., Toyoizumi, K., Shao, N., Mei, C., Demanuele, C., Tang, R., McMillan, G., & Beckman, R. A. (2023). Decentralized clinical trials and rare diseases: a Drug Information Association Innovative Design Scientific Working Group (DIA-IDSWG) perspective. Orphanet Journal of Rare Diseases, 18(1), 79. https://doi.org/10.1186/s13023-023-02693-7
29. H.R.5376 - 117th Congress (2021-2022): An act to provide for reconciliation pursuant to title II of S. Con. Res. 14. (n.d.). Congress.gov | Library of Congress. https://www.congress.gov/bill/117th-congress/house-bill/5376
30. Cubanski, J. (2025, August 9). FAQs about the Inflation Reduction Act's Medicare Drug Price Negotiation Program. KFF. https://www.kff.org/medicare/faqs-about-the-inflation-reduction-acts-medicare-drug-price-negotiation-program/?entry=table-of-contents-how-many-and-which-types-of-drugs-qualified-for-price-negotiation-for-2027
31. U.S. House of Representatives. (2025–2026). H.R. 1: An act to provide for reconciliation pursuant to title II of H. Con. Res. 14 (119th Cong.). Congress.gov. https://www.congress.gov/bill/119th-congress/house-bill/1/text
32. Updates from the Capital: Implications of the One Big Beautiful Bill Act (OBBBA) on the Brain Tumor Community - National Brain Tumor Society. (2025, July 9). National Brain Tumor Society. https://braintumor.org/news/update-from-the-capital-implications-of-the-one-big-beautiful-bill-act-obba-on-the-brain-tumor-community
33. Sanzo, K. M., & Lamparelli, R. L. (2025, July 30). Orphan drugs, big breaks: The quiet carve-out in the 'One Big Beautiful Bill Act'. Morgan Lewis – As Prescribed. https://www.morganlewis.com/blogs/asprescribed/2025/07/orphan-drugs-big-breaks-the-quiet-carve-out-in-the-one-big-beautiful-bill-act
34. Novartis agrees to acquire Avidity Biosciences, an innovator in RNA therapeutics, strengthening its late-stage neuroscience pipeline. (n.d.). Novartis. https://www.novartis.com/news/media-releases/novartis-agrees-acquire-avidity-biosciences-innovator-rna-therapeutics-strengthening-its-late-stage-neuroscience-pipeline
35. Reuters. (2025, November 10). Eli Lilly signs deal for MeiraGTx's gene therapy for severe eye disease. Reuters. https://www.reuters.com/legal/transactional/eli-lilly-signs-deal-meiragtxs-gene-therapy-severe-eye-disease-2025-11-10/

Sources

36. Denali Therapeutics and Royalty Pharma announce $275 million royalty funding agreement | Denali Therapeutics. (n.d.). Denali Therapeutics. https://investors.denalitherapeutics.com/news-releases/news-release-details/denali-therapeutics-and-royalty-pharma-announce-275-million
37. Laaman S, Riad MH, Sun K, Gryga M. Jazz Pharmaceuticals PLC (JAZZ): Modeyso Launch and PRV Secured. Morgan Stanley Research; August 28, 2025.
38. Ulz ME, Novick A, Bhasin R. Ionis Pharmaceuticals Inc: Dawnzera (Donidalorsen) Approved for HAE, As Expected. Morgan Stanley Research; August 21, 2025
39. Skor M, Zhang S. Crinetics Pharmaceuticals: Palsonify Launch in Focus with Encouraging Signs from Community Uptake. Morgan Stanley Research; November 7, 2025.
40. Medexus Pharmaceuticals Inc. Medexus Pharma Investor Presentation – August 2025. Medexus Pharmaceuticals; August 2025.
41. Boutherin T, Kapila S, Hindley LA, Askin G. Merck KGaA: Acquisition of SpringWorks Therapeutics Confirmed. Morgan Stanley Research; April 28, 2025.
42. Frommer JC, Japhet ND, Patel P. PTC Therapeutics: 3Q25 Earnings – Sales Beat Supported by Strong Sephience Start. Morgan Stanley Research; November 5, 2025
43. Liquidia Corporation. R&D Day Presentation. October 29, 2025 Liquidia Corporation. 3Q2025 Earnings & Corporate Update. November 3, 2025
44. U.S. Food & Drug Administration. Approval Letter — ENCELTO (revakinagene taroretcel-lwey) Biologics License Application STN BL 125798/0. March 5, 2025.
45. Neurotech Pharmaceuticals, Inc. (March 5, 2025). [BLA approval letter to Richard Small BLA approval]. Retrieved from https://www.fda.gov/media/185733/download?attachment
46. ONO Pharmaceutical Co., Ltd. R&D Day – ROMVIMZA. May 20, 2025.
47. Boutherin T, Kapila S, Hindley LA, Askin G. Merck KGaA: Acquisition of SpringWorks Therapeutics Confirmed. Morgan Stanley Research; April 28, 2025.
48. Ulz ME, Bhasin R, Novick A. Mirum Pharmaceuticals: 3Q25 Results – In Line Quarter and Volixibat PSC Data Up Next. Morgan Stanley Research; November 4, 2025
50. Mba, R. M. (2025, August 7). Rare Disease Marketing: Strategies for niche drug launches. https://tjpagency.com/blog/rare-disease-marketing
51. Orphan drugs in the United States. (n.d.). IQVIA. https://www.iqvia.com/insights/the-iqvia-institute/reports-and-publications/reports/orphan-drugs-in-the-united-states-rare-disease-innovation-and-cost-trends-through-2019
52. MedPath - Medical Intelligence Platform. (2025, May 22). MedPath. https://trial.medpath.com/news/05f77dd2c80303fb/u-s-drug-prices-double-as-pharmaceutical-industry-shifts-focus-to-rare-diseases
53. American Medical Association & American Medical Association. (2025, April 24). Fixing prior auth: Nearly 40 prior authorizations a week is way too many. American Medical Association. https://www.ama-assn.org/practice-management/prior-authorization/fixing-prior-auth-nearly-40-prior-authorizations-week-way
54. American Medical Association & American Medical Association. (2024, July 18). Exhausted by prior auth, many patients abandon care: AMA survey. American Medical Association. https://www.ama-assn.org/practice-management/prior-authorization/exhausted-prior-auth-many-patients-abandon-care-ama-survey
55. Althobaiti, H., Seoane-Vazquez, E., Brown, L. M., Fleming, M. L., & Rodriguez-Monguio, R. (2023). Disentangling the cost of orphan drugs marketed in the United States. Healthcare, 11(4), 558. https://doi.org/10.3390/healthcare11040558

Sources

56. Resources for patients and families. (n.d.). Rare Diseases Clinical Research Network. https://www.rarediseasesnetwork.org/resources/patients-families
57. Johns, D. (n.d.). Providers' biggest challenges with rare diseases. Definitive Healthcare. https://www.definitivehc.com/blog/providers-challenges-rare-disease
58. epocrates analytics
59. Hung J, Riad MH, Sun K, Zhang S. Denali Therapeutics Inc: Denali Announces Key 2025 Milestones. Morgan Stanley Research; January 13, 2025.
60. Laaman S, Riad MH, Sun K, Madhok C. Denali Therapeutics Inc (DNLI.O): The Sanfilippo Discount; Assuming Coverage at Overweight. Morgan Stanley Research; March 6, 2025.
61. Ulz ME, Novick A, Bhasin R. Arrowhead Pharmaceuticals Inc (ARWR.O): FY4Q25 Earnings—FCS Launch Underway and Ph3 SHTG Data on Track for 3Q26. Morgan Stanley Research; November 26, 2025.
62. Skor M, Zhang S. Ultragenyx Pharmaceutical Inc: Setrusumab Readout Remains Key Focus; Management Reaffirms Confidence in Reaching Full-Year Profitability in 2027. Morgan Stanley Research; August 6, 2025.
63. Skor M, Zhang S. Ultragenyx Pharmaceutical Inc (RARE.O): Financing De-Risks Launch Prep; Orbit/Cosmic Ph3 Readouts Set Up Pivotal OI Catalyst. Morgan Stanley Research; November 5, 2025.
64. Grand View Research. Xerostomia Therapeutics Market (2025–2030) Size, Share & Trends Analysis Report by Type, Product & Region. Grand View Research; 2023.
65. Frommer JC, Japhet ND, Patel P. Regeneron Pharmaceuticals Inc (REGN.O): Strong 3Q Beat Drives Upward Revisions. Morgan Stanley Research; October 28, 2025.
66. Study details | NCT04906460 | Open-label study of WVE-N531 in patients with Duchenne muscular dystrophy (FORWARD-53) | ClinicalTrials.gov. (n.d.). https://www.clinicaltrials.gov/study/NCT04906460 https://www.clinicaltrials.gov/study/NCT04906460
67. Fabry Disease Treatment Market Size & Share Report, 2030. (n.d.). https://www.grandviewresearch.com/industry-analysis/fabry-disease-treatment-market
68. Sangamo Therapeutics reports Recent business highlights and second quarter 2025 financial results | Sangamo Therapeutics, Inc. (n.d.). Sangamo Therapeutics, Inc. https://investor.sangamo.com/news-releases/news-release-details/sangamo-therapeutics-reports-recent-business-highlights-and-13.

*versus the high end of benchmarks