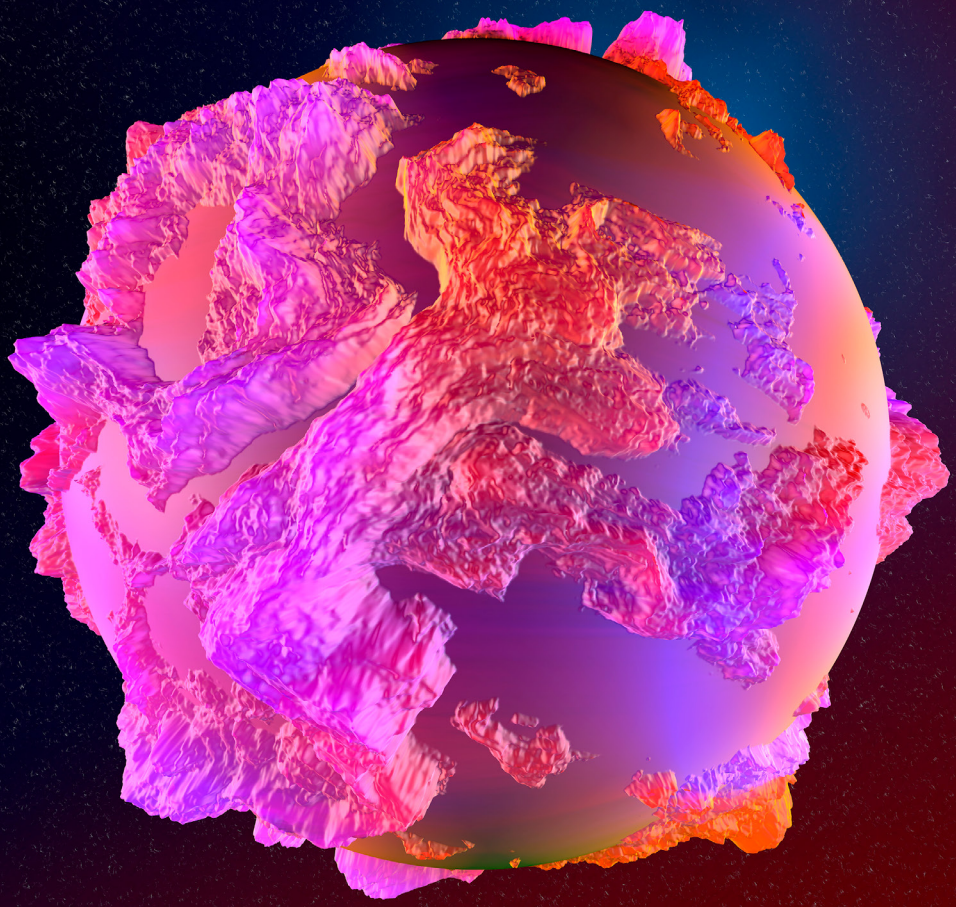
Putnam
Inizio Advisory



WHITE PAPER

Recent Trends in **Rare Diseases.**

Authored by

Mariah Hanley, PhD, Principal

Joseph Feingold, PhD, Partner, Portfolio, Licensing & Development Practice Lead & Head of Advanced Technologies Center of Excellence

Dian Huang Davis, PhD, Manager

Kelsey Kaeding, PhD, Life Sciences Consultant

Research Contributors

Kellan Kim, Associate Consultant

Rohit Katoch, Research Consulting Senior Director

Sushant Deswal, Research Consulting Manager

July 2024

ABSTRACT

The rare disease market has grown consistently over the past ten years and is expected to continue to grow, driven by regulatory incentives for orphan drugs and blockbuster commercial potential in high unmet- need diseases with little competition. Even with substantial pharma and biotech interest and investment, there remain hundreds of diseases with high unmet need for effective therapies. However, recent barriers have emerged, including increasing pricing pressure and emphasis on cost-effectiveness proven through comparative clinical trials. These have the potential to alter the historic attractiveness of drug development in rare diseases.

This work details our observations on recent global trends within the rare disease space. We also draw implications for manufacturers and recommend strategies to maximize rare disease opportunities.

Introduction

Overall Rare Disease Market Trajectory is Positive

Today, orphan drugs for rare diseases play a significant role in the global pharmaceutical market, accounting for 16% of pharma revenue. The global rare disease market has steadily increased in past years and is predicted to reach ~$336B globally by 2030. Notably, ex-US market growth is outpacing the US and is projected to comprise half of the global market by 2030.

RARE DISEASE MARKET EXPECTED TO REACH ~$336B GLOBALLY BY 2030

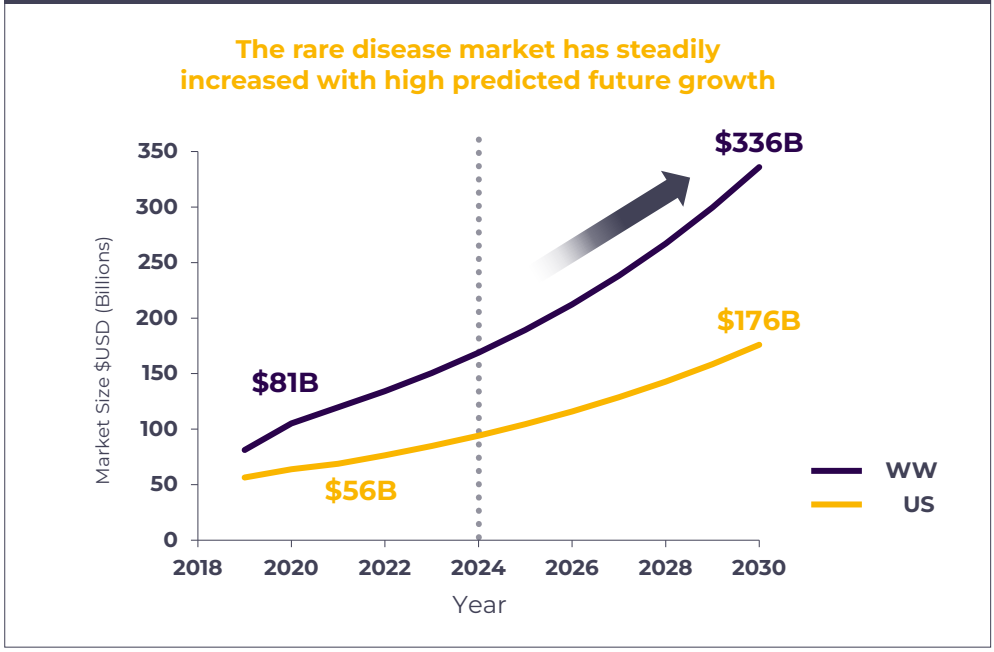


Fig. 1

Sources:
https://www.biospace.com/article/rare-diseases-treatment-market-size-share-growth-trends-report-2022-2030/ - Global.

https://www.grandviewresearch.com/industry-analysis/rare-diseases-treatment-market-report - US.

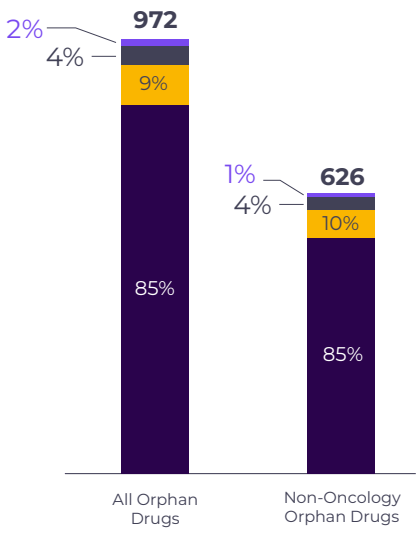
Rare disease numbers from National Organization for Rare Disease (NORD.org)

Note:
The 500 pipeline products may be partially inclusive of 350 diseases with approved therapies

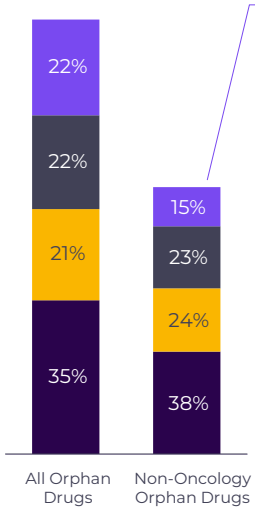
The top areas of rare disease investment are oncology, neurology, autoimmune, and ophthalmology. More broadly, the rise in biologics across the industry has also been a key driver of rare disease market growth in recent years, with notable modalities including immunotherapies, misfolded protein chaperone therapies, cell therapies, and gene replacement / gene-editing therapies. Therapies with technological innovation underpinning strong efficacy for high unmet need conditions and/or dosing convenience advantages have done particularly well, with 56 orphan drug designated therapies expected to attain >$1B in 2030 worldwide sales (34 outside of oncology) and 15 expected to achieve >$3B (7 outside of oncology).

RARE DISEASE THERAPIES CAN HAVE BLOCKBUSTER POTENTIAL, EVEN IN COMPETITIVE SPACES

Number of Orphan Drugs by Expected 2030 Sales



Contribution of Orphan Drugs to Total WW Orphan Drug Sales, 2030



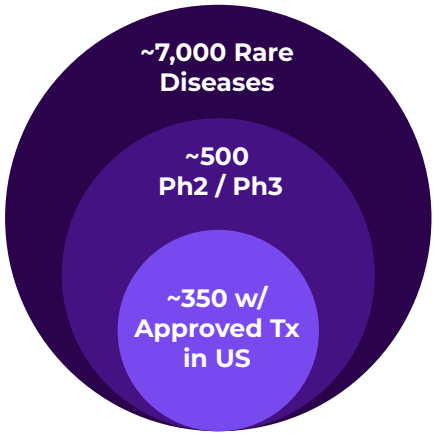
Drug	Manufacturer	Rare disease Indications	Expected Total 2030 WW Sales ($B USD)
VX-121	Vertex	Cystic Fibrosis	5.0
Winrevair	Merck	Pulmonary Arterial Hypertension	4.2
Vabysmo	Roche	Wet AMD	4.1
Kesimpta	Novartis	Multiple Sclerosis	3.6
Hemlibra	Roche	Hemophilia A	3.2
Botox	Abbvie	• Strabismus and blepharospasm associated with dystonia • Cervical dystonia • Pediatric upper limb spasticity associated with cerebral palsy	3.2
Ocrevus	Roche	Multiple Sclerosis	3.1

2030 Projected Revenue >$3B $1-3B $500K-1B <$500K

Fig. 2 **Source:** *Evaluate Pharma* **Note:** *Orphan drugs as indicated by Evaluate Pharma*

Even with the sustained growth, 95% of rare diseases currently do not have available treatments, leaving a large amount of whitespace for potential orphan drug opportunities. There are numerous critical decisions that a manufacturer looking to develop such therapies will need to make, with many considerations that are unique to rare disease.

ONLY ~5% OF RARE DISEASES HAVE THERAPIES AVAILABLE TODAY, DESPITE MANY RECENT APPROVALS



Key Biologic Modalities Driving Rare Disease Market Growth:
- HSCT / immunotherapies
- Misfolded protein chaperone therapies
- Gene replacement / gene-editing therapies

Sources:
https://www.biospace.com/article/rare-diseases-treatment-market-size-share-growth-trends-report-2022-2030/ - Global.

https://www.grandviewresearch.com/industry-analysis/rare-diseases-treatment-market-report - US

Rare disease numbers from National Organization for Rare Disease (NORD.org)

Note:
The 500 pipeline products may be partially inclusive of 350 diseases with approved therapies

Fig. 3

Key Decisions in Rare Disease Drug Development

This white paper will provide an overview of 5 important and complex topics for consideration that companies face in the drug development and commercialization process of rare disease therapies:

- Trial Design
- Market Access
- Pricing
- Innovative Contracting
- Real World Evidence (RWE) and Life Cycle Management (LCM)

Rare disease trial design considerations

EU Joint Clinical Assessment (JCA) standardization could accelerate rare disease drug access, but the focus on comparators could complicate recommendations

EMA and national health technology assessments (HTAs) will collaborate under the context of Joint Clinical Assessment (JCA) with goals of streamlining the HTA clinical evaluation process and accelerating recommendations. Beginning in 2025, Advanced Therapy Medicine Products (ATMPs), which include cell and gene therapies and tissue-engineered products, are to undergo JCA and orphan medicinal products (OMPs) beginning in 2028.

The current JCA framework proposed by EUnetHTA21 relies heavily upon comparators and randomized controlled trials (RCTs). One analysis by the Alliance for Regenerative Medicine determined almost 90% of ATMPs currently licensed and marketed in Europe would not be approved based on the current EUnetHTA21 proposed framework, though this analysis relied heavily on the assumption that randomized control trials with a comparator are required.

While shifting requirements for JCA has caused some uncertainty and concern over clinical development challenges and access for rare disease products, efforts by some national HTAs to create specialized assessments for rare disease therapies indicate a desire to ensure access to OMPs.

EU Health Technology Assessment (HTA) requirements increasingly support innovative rare disease study design

While randomized controlled trials are the gold standard for pharmaceuticals development, rare disease therapies have often been tested in open-label clinical trial designs, as situations where there is a small patient population and few to no therapies available that are reasonable, ethical comparators are common. These trials are also often small (given the small patient populations), making it difficult to properly power a randomized controlled trial. In some rare diseases, there are enough pharmacotherapy competitors where randomized controlled trials are theoretically possible (e.g., spinal muscular atrophy, with three disease-modifying therapies approved in the US). However, these diseases are the exception rather than the rule and are likely to remain so for the foreseeable future.

In Europe, randomized control trials continue to be the gold standard for all therapeutics in HTA assessments. However, some countries have made significant changes to their requirements to accommodate rare disease therapies, creating access potential even for therapies studied in open-label and/or small-size trials, given the high cost on a per-patient basis.

At least 5 EU HTAs (Germany, Scotland, Sweden, Lithuania, and the Netherlands) have approaches specific to Orphan Medicine Products (OMP) in place (defined as treating a disease with a prevalence of ≤5:10,000), including:

- Not requiring comparative studies
- Accepting lower levels of statistical significance
- Accepting surrogate endpoints to show clinical effectiveness
- Loosening or not requiring proof of cost-effectiveness

These approaches have created incentive to create strong trials that also consider the unique context often found in rare disease. Having a robust understanding of this disease context opens the possibility for novel trial designs, including those that enable early planning for collection of critical real-world data.

Across the US and EU, the growing need for RWE is playing an important role in influencing rare disease trial design

For rare disease, in cases where RCTs are not feasible, difficulties in recruiting enough patients for a control arm, and ethical concerns over the inclusion of a control arm, historical real-world data or natural history studies can be especially useful in developing an alternative trial design. For example, single arm trials or synthetic control arms (e.g., using historical data or data from electronic health records) can be used instead.

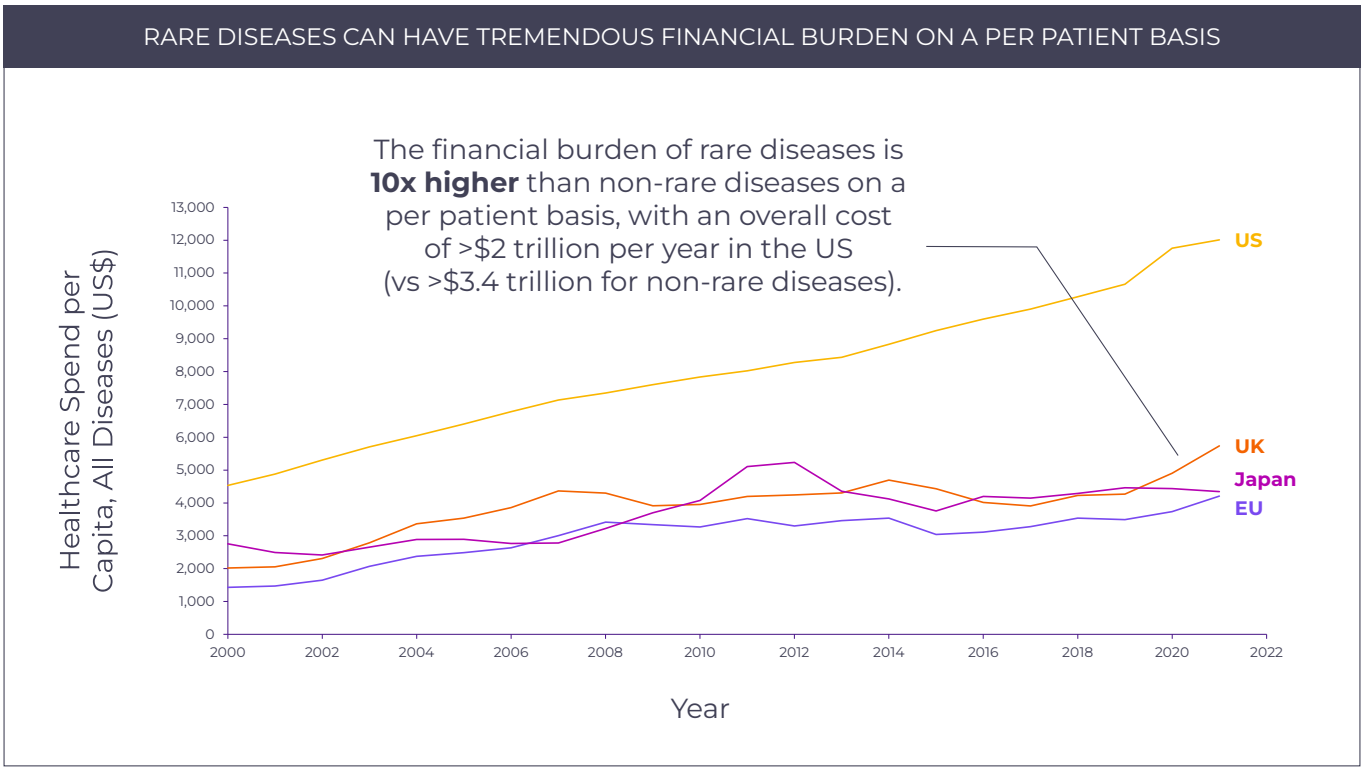


Fig. 4

Source:
WHO Global Health Expenditure Database, Chiesi Global Rare Diseases White paper – The Burden of Rare Diseases: An Economic Evaluation, chiesiglobalrarediseases.white paper-feb.-2022_production-proof.pdf (chiesirarediseases.com)

Market Access

Difficulty in Demonstrating Cost-Effectiveness Hinders Access in Europe

Market access is becoming increasingly challenging in Europe with the orphan drug reimbursement rate decreasing steadily, year over year, primarily driven by the growing importance of cost-effectiveness. Thresholds to be considered cost-effective are often more lenient for rare diseases, given the high unmet need and cost per patient. For example, in the UK, following changes introduced in April 2017, NICE set a maximum additional QALY threshold of £300,000 for highly specialised treatments, under which they will automatically be approved for routine commissioning. This is ten times higher than the standard NICE threshold of £30,000 for non-specialized treatments. The upper limit will vary according to the lifelong impact of the technology on the patient, varying from £100,000 per quality-adjusted life year for treatments that deliver less than 10 QALYs to the patient in their lifetime, up to a maximum of £300,000 for treatments that deliver more than 30 additional QALYs to the patient in their lifetime. A prominent example is Trikafta, Vertex's cystic fibrosis combination therapy, where The National Institute for Health and Care Excellence (NICE) acknowledged the effectiveness but failed to consider the medicine cost-effective in draft guidance, with the current status of the evaluation suspended, hindering UK patient access to that therapy.

Contributing to increasing access challenges in rare disease in ex-US markets is the evolution of HTA requirements and the potential for even further scrutiny under the upcoming JCA framework. Many manufacturers with rare disease products will face challenges in overcoming potential uncertainty around clinical effectiveness of their products (due to lack of active trial comparators and/or small trial sizes) and the typical high prices needed to be commanded by rare disease drugs to support an attractive commercial opportunity. Cost-effectiveness will continue to be difficult to demonstrate in the EU without adapted willingness to pay thresholds.

MARKET ACCESS IN EUROPE IS BECOMING INCREASINGLY CHALLENGING FOR ORPHAN DRUGS

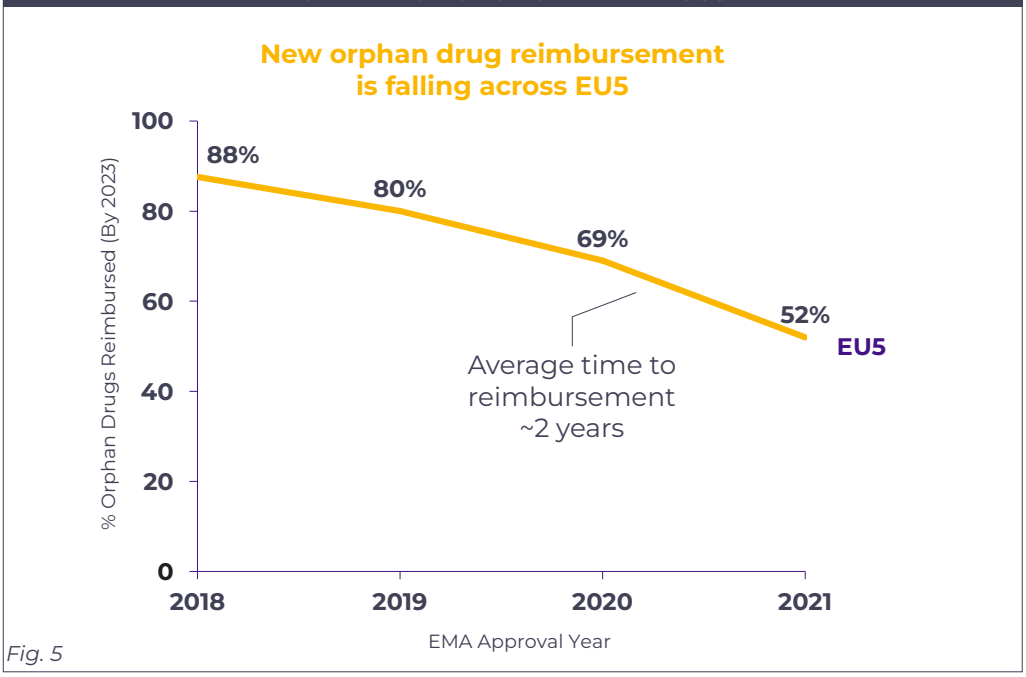


Fig. 5

Sources:
EFPIA Patients Wait Indicator 2022 Survey

Note:
Availability equates to granting access to reimbursement list

Single indication and ultra-rare diseases are favored in the evolving global access landscape

- Institute for Clinical and Economic Review (ICER) is likely to assess cost-effectiveness of drugs with ~1-2 previously approved therapies in class, potentially impacting pricing potential or formulary placement in competitive disease space.
- ICER also offers modifications to its value assessment framework for treatments in ultra-rare diseases (indications with less than 20,000 prevalent US patients).
- NICE has expanded access to the Highly Specialized Treatment review pathway for ultra-rare diseases where the QALY threshold is up to 10x that of their standard Single Technology Appraisal

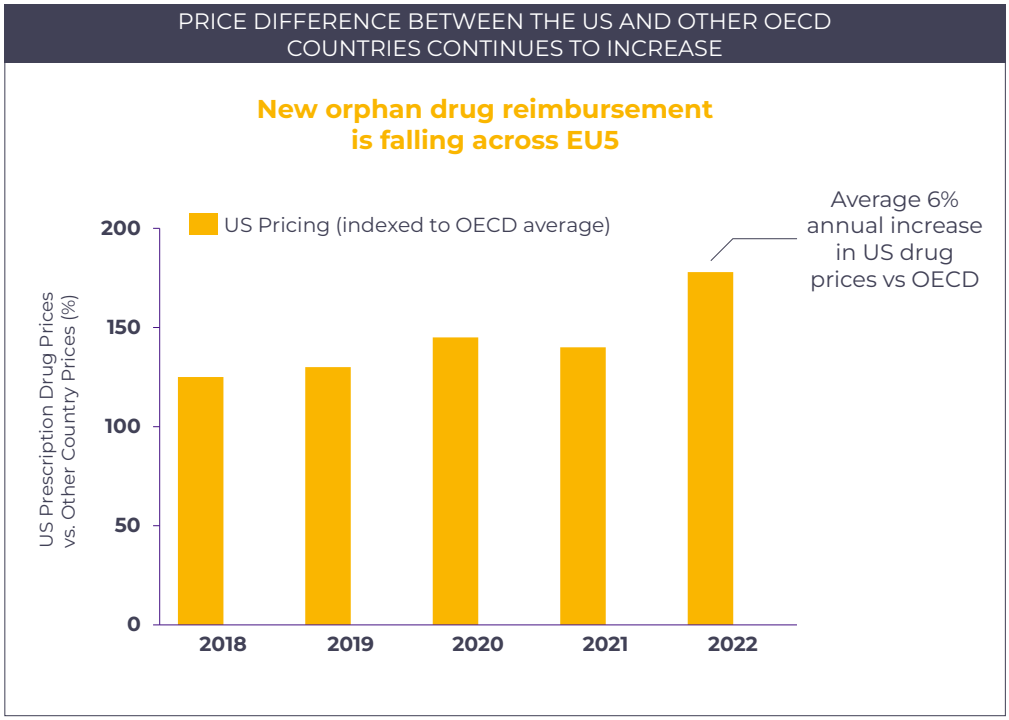
Pricing Pressure

The Inflation Reduction Act (IRA) in the US to impact rare disease drug pricing

Orphan drugs with one rare disease indication are protected from price negotiations under the IRA. While this decision was intended to incentivize companies to pursue rare disease indications, it may ultimately disincentivize development of drugs for multiple rare disease indications and create higher market risk. For example, Alnylam abandoned an indication expansion of ATTR drug Amvuttra into Stargardt disease, noting that they "will not initiate a Phase 3 study of vutrisiran in Stargardt Disease in late 2022, as previously guided, as it continues to evaluate the impact of the Inflation Reduction Act." Centers for Medicare and Medicaid Services (CMS) has clarified that the exemption includes drugs that have been FDA-approved for multiple indications within one rare disease, but drugs that are targeting multiple diseases remain at risk for price negotiation under IRA.

Additionally, the focus on Part B (biologics) in rare disease may shield many OMPs from significant impact of Part D redesign. However, manufacturer liability costs will increase for all Part D products in an attempt to close the "donut hole" to lessen the burden on Medicare patients:

- Medicare Part D beneficiary cap at $2k per year in 2025 (monthly caps will also be implemented) will prevent excessively large out-of-pocket (OOP) costs to rare disease patients
- In the catastrophic phase under the Part D redesign, manufacturers will provide a 20% discount (vs 10% prior to the IRA)



Sources:
https://aspe.hhs.gov/reports/comparing-prescription-drugs;

OECD pricing refers to a combination of 33 other countries

Fig. 6

Innovative Contracting

Innovative contracting grows in rare diseases

Innovative contracting in rare disease has become increasingly common in both the US and EU, with a variety of different approaches:

- Financial-Based Agreements – including volume-based and portfolio-linked pricing discounts
    - Primarily used for sub-acute and chronic treatment
    - Prominent across EU, especially in France
- Service-Based Agreements (i.e. the "Netflix" model) – incorporate subscriptions or additional services
    - While most utilized in chronic diseases, these agreements are often supplementary to more common financial-based agreements
- Outcome-Based Agreements (OBA) – value contingent on therapy clinical performance
    - OBAs are more logistically feasible for manufacturers in rare diseases than non-rare diseases due to lower prevalence and resulting greater ease of tracking patients.
    - OBAs are available for a majority of rare disease gene therapies that hinge on long-term durability (e.g., gene therapies), which can make high-costs more digestible to payers and public. To be competitive, manufacturers should strongly consider offering OBAs to payers at launch. However, it is important to note that not all payers will adopt OBAs and the extent to which patient access will increase is still not fully understood.
    - The rise in OBAs also elevates the importance of having a robust real-world evidence strategy. For example, treatment failure rebate of bluebird's sickle cell gene therapy, LYFGENIA, will be determined by long-term follow-up data, specifically hospitalizations for vaso-occlusive episodes. Strategic planning of outcome definitions and data collection methodology are imperative to successful negotiation and execution of OBAs. This planning should start early in clinical development to ensure collection of trial data before approval that can support clear outcomes that can be used in OBAs and easily measured and tracked outside of a trial setting.

Real-World Evidence and Life Cycle Management

For lifecycle management, real-world evidence (RWE) is playing a progressively more important role for rare disease therapies and beyond. RWE is increasingly utilized to support clinical and regulatory decisions in both new drug approvals and indication expansions:

- From January 2019 – June 2021, 116 of 378 FDA approvals included RWE as part of the submission
- 2021 FDA indication expansion of tacrolimus (Prograf) (and supplemental new drug application from the EMA) relied upon retrospective observational study data from the US Scientific Registry of Transplant Patients
- 2018 FDA marketing application approval for lutetium Lu 177 dotatate (Lutathera) relied on expanded access study data to support clinical efficacy and safety

In 2023, the FDA released guidance that biotech companies, regardless of if they are pursuing rare or non-rare indications, should seek early engagement with regulatory bodies to plan for RWE usage, data collection and analysis.

Although there are some rare-disease-specific challenges in bringing an orphan medical product to market, there are still numerous reasons for companies to pursue rare disease indications due to the overall unmet need and high severity of many of these indications that can translate to favorable commercial opportunities. Trends that theoretically could lead to increasing access challenges can potentially be mitigated through thoughtful clinical trial design and generation of real-world evidence.

Overall, there can be advantages in pursuing development of therapies for rare disease; however, it is important to be mindful of barriers and evolving trends.

BARRIERS AND DRIVERS TO RARE DISEASE THERAPY DEVELOPMENT AND COMMERCIALIZATION

Barriers

US:

- Potential for decreased access and risk of IRA price negotiation
- Rising Part D manufacturer liability

EU:

- When possible, focus on an active trial comparator in multi-country assessments (if pharmacological standard of care is available)
- Difficulties demonstrating cost-effectiveness

Global:

- Challenges in endpoint selection in under-studied disease areas

Drivers

US:

- ICER support for rare disease pricing
- Orphan drugs and biologics insulation from IRA price negotiation

EU:

- Accommodation for rare disease therapies by individual market HTAs

Global:

- Challenges in endpoint selection in under-studied disease areas
- Rising importance of real-world evidence and historic real-world data in development, commercialization, and access

Recommendations for Rare Disease Drug Commercialization

Take access and pricing implications into account when designing clinical trials, specifically:

- Feasibility of randomized comparator arm, and consider potential external controls such as real-world data that could be used to support evolving HTA assessments
- Appropriate endpoint selection and long-term follow-up data, both of which can help demonstrate cost-effectiveness and structure clear, data-backed outcomes-based agreements

Consider including the US as one of the first markets of entry given more favorable access landscape for rare diseases with less potential for near-term evolution.

Account for the catastrophic phase Part D liability in the US in profitability assessments for rare disease therapies expected to have a notable proportion of patients aged ≥65

Proactively offer outcomes-based agreements for cell and gene therapies in rare diseases, especially when entering competitive disease areas.

- Plan out an outcomes-based-agreement strategy to support trial design, ensuring appropriate selection of trial endpoints that will not only support product approval but also specific metrics and outcomes to include in outcomes-based agreements

Carefully evaluate pricing and access implications for indication expansions in the US and in particular:

- Loss of protection from price negotiation under the IRA when approved for additional indication(s)
- Expanding the addressable patient population beyond 20k prevalent patients will mean therapy cost-effectiveness is no longer assessed under ICER's adapted approach for ultra-rare conditions

Reach out to Putnam to discuss your specific needs and questions. With our decades of experience across dozens of rare disease products and manufacturers, we can help you find custom solutions to these challenges as well as many others that may be faced in the course of rare disease therapy development and commercialization.

Putnam Experience in Rare Disease

Putnam has been supporting rare disease strategy since the company's founding (a 35+ year history with exclusive focus on the life sciences)

Our work spans a wide range of project types across commercial and VPA strategy, with dozens of projects annually focused on rare disease. Example project types include (but are not limited to):

- **Commercial:** Indication assessments, competitive landscape evaluation, clinical endpoint and TPP design to maximize commercial potential, demand and segmentation, messaging and positioning strategy, launch plan development, patient registry and advocacy partnership strategies, LCM strategy
- **VPA:** Payer and market access landscape assessment, pricing strategy, contracting strategy (including innovative / value-based contracting strategies), RWE generation strategy and support
- We also offer a comprehensive suite of services to support multi-asset / multi-indication portfolio decision-making spanning both commercial and VPA practice areas

Our disease experience is both broad and deep across a wide variety of therapeutic and disease areas, including (non-exhaustive):

- **Oncology**: **20+** indications, including leukemias, lymphomas, and myelomas and ultra-rare cancers
- **Hematology**: **5+** indications (e.g., hemophilia A and B, PNH)
- **Rheumatology and Inflammatory**: **30+** indications (e.g., psoriatic arthritis, SLE and associated variants, Sjogren's, GvHD (acute and chronic))
- **Neurology**: **25+** indications (e.g., SMA, HD, DMD, rare epilepsies, leukodystrophies, dementias, and pain-centered conditions)
- **Endocrine / Metabolic / Gastrointestinal**: **15+** indications (e.g., Pompe, Gaucher, primary biliary cholangitis, osteogenesis imperfecta, HoFH)
- **Nephrology and Hepatic**: **5+** indications (e.g., Alport's, primary autoimmune hepatitis)
- **Infectious Disease**: **50+** indications across both developed and emerging markets (e.g., West Nile virus, Creutzfeldt-Jakob disease, Ebola, listeriosis)
- **Ophthalmology, Audiology, and Dermatology**: **5+** indications (e.g., Leber congenital amaurosis)
- ...and many more

We are also highly experienced in exploring rare manifestations, subtypes, and/or genetic profiles of more common diseases. Across our rare disease practice, our primary aim is to help manufacturers develop therapies as quickly as possible that can meet the often very high unmet need for often underserved patients with limited options.

We will be your strategic thought partner before, during, and after engagements to achieve this mutual goal.

If you feel you and your team would benefit from partnering with Putnam, please reach out.

Sources

- BioSpace (2022, August 25). Rare Diseases Treatment Market Size, Share, Growth, Trends | Report 2022-2030. Available at https://www.biospace.com/article/rare-diseases-treatment-market-size-share-growth-trends-report-2022-2030/
- Grand View Research (2023, January). Rare Diseases Treatment Market Size, Share & Trends Analysis Report By Therapeutic Area (Cancer, Musculoskeletal Conditions), By Route of Administration, By Drug Type, By Distribution Channel, By Region, And Segment Forecasts, 2022 - indic. Available at https://www.grandviewresearch.com/industry-analysis/rare-diseases-treatment-market-report
- National Organization for Rare Disorders (n.d.). Rare Disease Facts & Statistics. Available at https://rarediseases.org/understanding-rare-disease/rare-disease-facts-and-statistics/
    - Rare disease numbers from National Organization for Rare Disease (NORD.org)
- Inflation Reduction Act (2022). H.R.5376 - Inflation Reduction Act of 2022. Congress.gov. Available at https://www.congress.gov/bill/117th-congress/house-bill/5376/text
    - Figure 3 Sources
- Newton, M., Stoddart, K., Travaglio, M., & Troein, P. (2023, April 1). EFPIA Patients W.A.I.T. Indicator 2022 Survey. Available at https://www.efpia.eu/media/s4qf1eqo/efpia_patient_wait_indicator_final_report.pdf
    - EFPIA Patients Wait Indicator 2022 Survey
- ASPE (2024, January 31). Comparing Prescription Drugs in the U.S. And Other Countries: Prices and Availability. ASPE (The Assistant Secretary for Planning and Evaluation). Available at https://aspe.hhs.gov/reports/comparing-prescription-drugs
- Figure 6 Sources: Rudd, A. (2023, April 3). What is the joint clinical assessment and what does it entail?. Remap Consulting. https://remapconsulting.com/hta/what-is-the-joint-clinical-assessment-and-what-does-it-entail/
- Figure 7 sources: Admin. (2024, March 5). A look at three RWE supported FDA approvals. Aetion. https://evidence-hub.aetion.com/the-surge-in-fda-approvals-supported-by-rwe-a-look-at-three-recent-fda-decisions
- Center for Drug Evaluation and Research. (2021, August 4). Role of RWE in regulatory decision-making. U.S. Food and Drug Administration. https://www.fda.gov/drugs/our-perspective/fda-approval-demonstrates-role-real-world-evidence-regulatory-decision-making-drug-effectiveness
- Purpura, C. A., Garry, E. M., Honig, N., Case, A., & Rassen, J. A. (2021). The role of real-world evidence in fda-approved new drug and Biologics License Applications. Clinical Pharmacology & Therapeutics, 111(1), 135–144. https://doi.org/10.1002/cpt.2474
- Center for Drug Evaluation and Research. (2023, August 30). Considerations for the use of real-world data and real-world evidence. U.S. Food and Drug Administration. https://www.fda.gov/regulatory-information/search-fda-guidance-documents/considerations-use-real-world-data-and-real-world-evidence-support-regulatory-decision-making-drug

- Figure 8 sources: Tomassy, J., Caban, A., Hammes, F., & Toumi, M. (2019). PNS225 current status of outcomes-based agreements in selected European countries and Canada. Value in Health, 22. https://doi.org/10.1016/j.jval.2019.09.2125
- EFPIA (2023). The Pharmaceutical Industry in Figures 2023. European Federation of Pharmaceutical Industries and Associations (EFPIA). Available at https://www.efpia.eu/media/rm4kzdlx/the-pharmaceutical-industry-in-figures-2023.pdf
    - EPFIA – The Pharmaceutical Industry in Figures, 2022
- Vankar, P. (2023, September 19). Health expenditure as a percentage of GDP in select countries 2022. Statista. Retrieved March 19, 2024, from https://www.statista.com/statistics/268826/health-expenditure-as-gdp-percentage-in-oecd-countries
- Centers for Medicare & Medicaid Services. (2023, December 13). NHE Fact Sheet. CMS.gov. https://www.cms.gov/data-research/statistics-trends-and-reports/national-health-expenditure-data/nhe-fact-sheet#:~:text=NHE%20grew%204.1%25%20to%20%244.5,18%20percent%20of%20total%20NHE
- WHO Health Expenditure Database (EU)
- Castaldo, A. J., Jervelund, C., Corcoran, D., Boysen, H. B., Christiansen, S. C., & Zuraw, B. L. (2021). Assessing the cost and quality-of-life impact of on-demand-only medications for adults with hereditary angioedema. Allergy and Asthma Proceedings, 42(2), 108–117. https://doi.org/10.2500/aap.2021.42.200127
- Tisdale, A., Cutillo, C. M., Nathan, R., Russo, P., Laraway, B., Haendel, M., Nowak, D., Hasche, C., Chan, C.-H., Griese, E., Dawkins, H., Shukla, O., Pearce, D. A., Rutter, J. L., & Pariser, A. R. (2021). The ideas initiative: Pilot study to assess the impact of rare diseases on patients and Healthcare Systems. Orphanet Journal of Rare Diseases, 16(1). https://doi.org/10.1186/s13023-021-02061-3
- bluebird bio. (2024, March 11). bluebird bio Announces First Outcomes-Based Agreement with Medicaid for Sickle Cell Disease Gene Therapy. Investors & Media. Retrieved from https://investor.bluebirdbio.com/news-releases/news-release-details/bluebird-bio-announces-first-outcomes-based-agreement-medicaid.
- Andreu, P., Karam, J., Child, C., Chiesi, G., & Cioffi, G. (2022). The Burden of Rare Diseases: An Economic Evaluation [White Paper]. Chiesi Global Rare Diseases. https://chiesirarediseases.com/assets/pdf/chiesiglobalrarediseases.white paper-feb.-2022_production-proof.pdf

About Putnam

Putnam, an Inizio Advisory company, is a leading scientific and strategic consulting partner that helps the global life science community make confident decisions, build value, and bring life-changing innovations to clinicians and patients. For more than 30 years, our rigorous, bespoke approach and globally diverse team have delivered unrivaled depth across therapeutic areas, business functions, geographic markets, healthcare sectors, and technology platforms to maximize the human impact and commercial success of client innovations.