Tracking Developments in Treatment of Rare Diseases

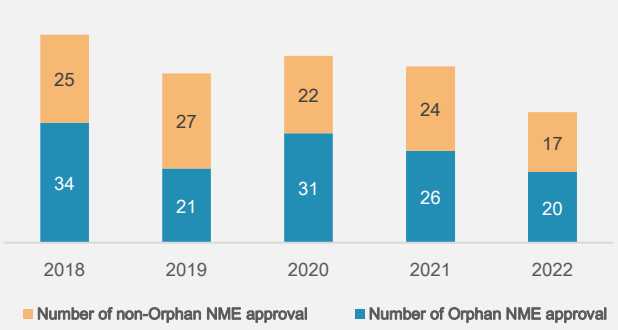
Landscape Overview And Upcoming Trends

As per the Global Rare Disease Data Platform **RARE-X, there are ~10,869 rare diseases worldwide** and ~10% people in the United States are diagnosed with a rare disease — that is about 30 Mn people. Efforts from government & private organizations are aiming to develop personalized care for affected patients. However, as the patient population is small, companies are less likely to recover development cost for medicines and hence fewer treatments are available, thus impact overall affordability & accessibility of care.

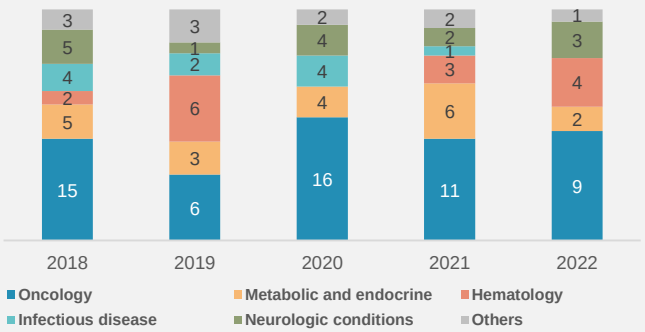
CURRENT DRUG LANDSCAPE

Only ~5% of rare diseases have US FDA approved treatments. However, the pharma industry is showing interest in developing treatments for such diseases, with oncology (34%) metabolic (15%), & hematology (11%) being therapy areas with most

Number of NME approvals (Orphan vs non-Orphan diseases)

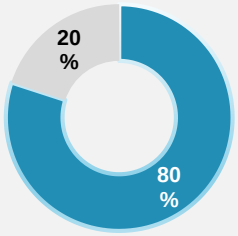


Top 5 Therapy Areas with Orphan Drug Approvals



RECENT DEVELOPMENTS

Along with increased FDA approved treatments, the field of rare diseases has seen recent developments, such as genetic discoveries, collaborative research efforts, and overall orphan drug development with the aid of government funding and support from several organizations.



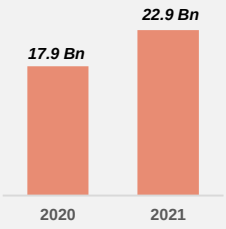
80% **of rare diseases are caused by genetic defects.**

This has led to the increased presence of gene therapy in the inline and pipeline landscape.

Zolgensma is the first gene therapy approved for spinal muscular atrophy. **Luxturna** is a gene therapy to treat a rare inherited blindness, and Glybera helps treat a rare form of lipoprotein lipase deficiency.

7/10 **largest transactions in 2021 involved rare disease** companies as target, accounting for **~29%** of all biopharma M&A deals and **~54%** of total reported deal values in 2021.

- **2019** — Vertex Pharma acquired CRISPR Therapeutics for **$9.5** Bn, gaining access to gene editing technology.
- **2020** — Sanofi acquired BioMarin for **$9.7** bn, adding orphan drugs like Vimizim and Naglazyme to its portfolio.
- **2021** — Pfizer acquired Arena Pharma for **$6.7 bn**, gaining access to orphan drugs like ravulizumab and etrasimod.
- **2022** — Amgen acquired Horizon Therapeutics for **$28 bn**, adding orphan drugs, such as Tepezza and Oxbryta, to its portfolio.



28% YoY increase in funding was seen during 2020–21 for rare disease, **totaling $22.9 Bn and surpassing most biotech and health segments.**

- **2020** — The US government and private foundations jointly contributed $700 Mn for rare disease development.
- **2021** — NIH granted $4.8 Bn for rare disease research; FDA awarded $100Mn for Orphan Products Grants Program.

Multiple strategic initiatives among rare disease companies and key pharma companies

Sanofi-Sobi

Collaboration in March 2022 to develop and commercialize innovative treatments for hemophilia A and B

Pfizer-Spark therapeutics

Collaboration in February 2022 to develop and commercialize gene therapy for diseases caused by a single gene defect

AbbVie-Takeda

Collaboration in January 2022 to develop and commercialize for CNS diseases (Alzheimer's and multiple sclerosis)

Global Genes & Genetic Alliance, International Rare Disease Research Consortium, and Rare Disease Clinical Research Network facilitate coordinated research and data sharing

UNMET NEEDS

Patients with rare diseases who experience positive effects from existing therapeutic options often encounter unmet needs, primarily related to the accessibility and affordability of these treatments.

Cost of care

- People with rare diseases incur 3–5 times higher medical costs than those with non-rare diseases.
- As per the National Organization for Rare Disorders, **~60%** only of the total approved drugs for rare diseases are covered by commercial insurance.
- About **70%** of families with a member who has a rare disease have out-of-pocket medical expense of $50,000 or more per year.
- Multiple efforts such as the expansion of insurance coverage, development of patient assistance programs, and creation of rare disease registries are been made to improve affordability of rare diseases.

Access to care

- In the US, the FDA has approved drugs for rare diseases, including Zolgensma for spinal muscular atrophy and Luxturna for inherited retinal dystrophy.
- In the UK, the National Institute for Health and Care Excellence has approved medications for rare diseases and granted positive decisions for drugs such as Soliris for paroxysmal nocturnal hemoglobinuria and Spinraza for spinal muscular atrophy.
- However, owing to lack of awareness and limited availability of specialized treatment and medications, access to care for people with rare diseases can be a challenge.

There is a need to raise awareness of these diseases and support research into new treatments. This in turn would help people get access to the care they require.

Right from understanding key issues to advising you through the right set of insights and recommendations, Aranca Research, consolidation, and insightful analysis to aid in-depth understanding of therapy and effective decision-making

HOW CAN ARANCA HELP?

01 **Market Assessment:** Disease burden/disease landscape, diagnosis and treatment paradigm, humanistic and economic burden

02 **Current and Future Landscape:** Current treatments and latest pipeline landscape assessment for a particular therapy area

03 **Strategic Initiative Analysis:** Understanding key market trends in terms of M&As, funding, deals and other pertinent strategic initiatives

04 **Health Technology Assessment (HTA):** Analysis of various HTA decisions published by various countries

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