# What Makes an Orphan Drug? Determining Characteristics of Non-Oncology Orphan Approvals from 2015-2022

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## OBJECTIVES

Orphan drugs are pharmaceuticals developed to treat rare diseases affecting less than 200,000 individuals in the US. The Orphan Drug Act incentivizes the development of these medicines by providing financial and regulatory benefits to manufacturers including tax benefits, longer market exclusivity, and reduced FDA application fees. Beyond these benefits, there are other factors that can incentivize manufacturers to develop orphan drugs, namely greater pricing power given high value stemming from more limited competition and high unmet need with limited existing treatment options.

This research aims to understand and segment new molecular entity (NME) approvals according to their product attributes and key market factors. In doing so, we aim to illuminate the nature of the orphan drug market and create evidence which may support the development of new medicines for orphan diseases.

## METHODS

The FDA's Center for Drug Evaluation and Research (CDER) publishes a list of new molecular entity (NME) products approved each year. This list excludes biologics and cell & gene therapies. CDER NME approval lists from 2015-2022 were documented, from which non-orphan and oncology products were removed to control for the unique factors of the oncology market. For products with multiple indications, only the first-approved indications were considered. We then referenced secondary sources to collect the prevalence, typical treatment duration (limited or chronic), route of administration, and number of FDA-approved alternatives for each product. We also calculated the annual price per patient based on WAC price at launch.

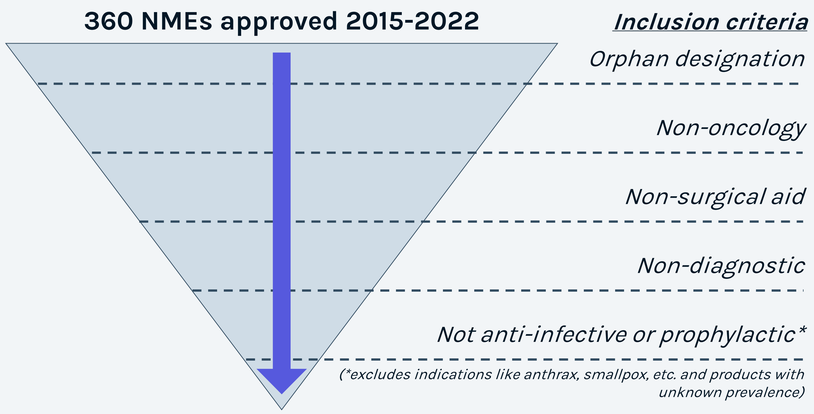


*Figure 1. Inclusion criteria used to filter NME approvals from 2015-2022.*

## RESULTS

n=76 orphan medicines met screening criteria for analysis. 55% of medicines were ultra-orphan (<10,000 individuals in the US), and 45% were not. Annual per patient prices were distributed accordingly: 21% <$100k; 22% $100k-$250k; 33% $250k-$500k; 17% $500k-$1M; and 7% >$1M. 91% of products were dosed chronically while 9% had a limited duration of therapy. 55% were first in indication therapies. The average number of on label competitors for the 45% of products which were not first-in-indication was 2 at launch. 54% were first in class products, and 46% were not. Most products were orals (41%), followed by infusions (30%), subcutaneous injections (28%), and topicals (1%). 65% of products were patient administered and 35% were HCP-administered.

### Annual per Patient Price of Orphan Drugs

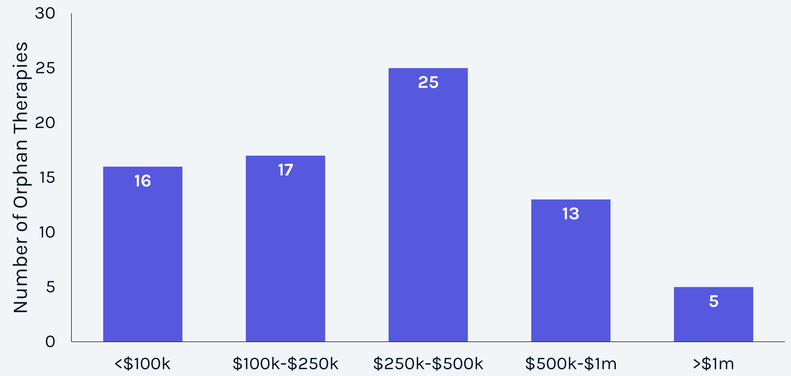


*Figure 2. Number of orphan drugs bucketed by price segments*

### Orphan Drug Route of Administration

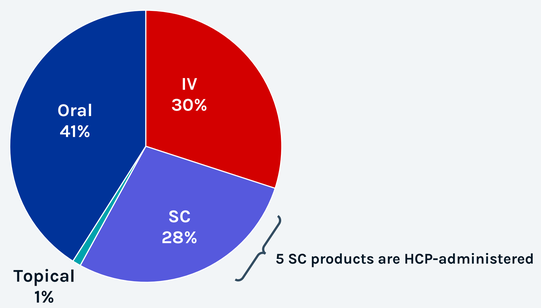


*Figure 1. Percentage of orphan drugs corresponding to different routes of administration; HCP: healthcare provider; IV: intravenous; SC: subcutaneous*

### Other Orphan Drug Characteristics

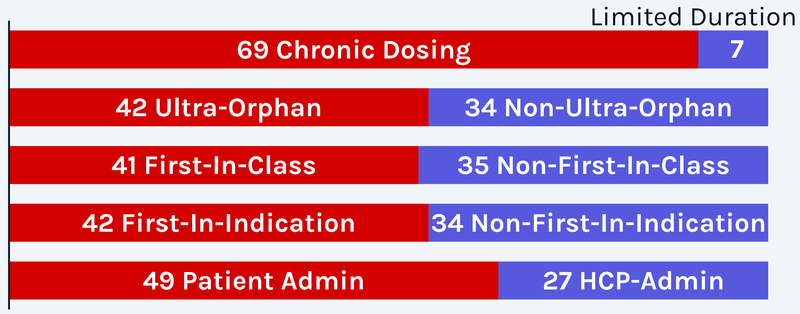


*Figure 4. Number of orphan drugs split by other characteristics; HCP: healthcare provider*

## CONCLUSIONS

The median orphan medicine is a first-in-class, first-in-indication, patient-administered, chronically dosed therapy for an ultra-orphan indication priced between $250k and $500k. Still, the recent focus on orphan drug development has resulted in a remarkably varied set of therapies which reflect the drug market at large. The orphan drug pathway remains an attractive development route for manufacturers regardless of product attributes.

## FUTURE IMPLICATIONS

Previous understandings of orphan drugs as an ultra-specialized and challenging market segment may evolve as more orphan drugs are launched and overall characteristics begin to reflect the pharmaceutical market at large. Self-administered oral medications form the largest segment of orphan drug approvals, and almost half of orphan drugs are not first-in-indication, suggesting an orphan indication is achievable for many assets and potentially worth further exploration.

## REFERENCES

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