Results

To determine whether drugs that are:
I. Approved under the Orphan Drug Act and
II. Solely indicated for the treatment of a rare disease and
III. Do not have existing patents and exclusivity preventing generic entry have genetic products available to increase access of affordable medications to patients affected by orphan diseases.

Methods

1. A comprehensive list of all approved orphan drugs and their indications from January 1983 to August 2017 was used to include the FDA Orphan Drug Product Designation Database.
2. The FDA-published list of Off-Patent, Off-Exclusivity Drugs without an Approved Generic was used to establish which orphan drugs are both off-patient and off-exclusivity, and whether genetic versions exist for each of these products.
3. The indication(s) for each off-patient, off-exclusivity product were extracted using the “Indications and Usage” section of package inserts found on the National Institutes of Health (NIH) Dailysheet website. To minimize the effect of confounding variables, the results of this study primarily focus on products that are solely indicated for a rare disease.
4. The Micromedex® RED BOOK Online® was used to determine the average wholesale price (AWP) of all off-patient, off-exclusivity products without an approved generic.

Conclusions

Findings suggest that at least 18.1 percent of orphan drugs do not have generic product availability, demonstrating a significant area of unmet need. Orphan drugs with no generic alternatives are indicated for a broad range of diseases, from acute to chronic illnesses.

- These orphan disease states may impact anywhere from 200 to 1.6 million patients, although most orphan drugs are indicated for specific subsets. There is a wide range in per-unit AWP of orphan drugs without generic drug competition. Note that some of these drugs may be used acutely, while others may require lifetime use.
- AWP costs per unit of drug range from $1.63 to $24,512.40.
- The majority (84%) of orphan drugs are marketed for more than $100 per unit.

Limitations

Limitations to data analysis of the FDA list of Off-Patient, Off-Exclusivity Drugs without an Approved Generic:
- Drugs that have been withdrawn from the market because newer or more effective products have been approved may still be included in the study.
- The list does not account for generics that were once approved, but are no longer marketed. Thus, additional drugs currently lacking competition may exist.
- Limitations to patient population data:
- These data are based on rough estimates compiled from multiple sources.
- Patient population sizes may not reflect actual subsets the drugs are used in.
- Limitations to cost data:
  - AWP does not necessarily reflect the actual price paid by the patient after insurance coverage and manufacturer coupons are accounted for.
  - AWP may not reflect the cost burden to patients because some drugs may only be used once during the patient’s lifetime, while others may be used chronically.

Future Direction

Generic competition plays a critical role in improving patient access and providing more affordable options. Future studies on potential regulatory or scientific barriers to generic drug approval for orphan drugs could help identify pathways for bringing lower cost alternatives to the market.