The objective of this review is to compare government involvement for healthcare. It is important to appreciate the regulatory status of orphan drugs in their countries to assist with patients in need.

**Background**

Rare or orphan drugs are terms used to designate drugs that affect only small numbers of individuals as so-called "health orphans". There is no unified definition of rare or orphan drugs, which varies by region depending on the population size. Rare or orphan drugs are specialty medications intended for diagnosis, prevention, or treatment of life-threatening or debilitating rare diseases. These drugs encompass a significant amount of healthcare costs and continue to increase in significance because of technological advances in areas of research and development. It is important for healthcare professionals to appreciate the regulatory status of orphan drugs in their countries to assist with patients in need.

**Method**

We conducted a comprehensive literature review using Medline, Google Scholar, and OECD (Organisation for Economic Co-operation and Development). For the different databases, we searched starting from 2000 to current. For Medline, we searched using the following MESH terms - orphan drugs, rare diseases, orphan drug legislation, and health policy. We tried to include the 10 most populous countries (populations over 200 million) based on the world health index. We used government control of pricing and policy, and variation in coverage that makes the system difficult to navigate for both patients and providers.

**Results**

Between China, India, United States, Indonesia, Brazil, Pakistan, Russia, and Japan, there was limited uniformity on orphan drug legislation, definition of orphan drug, pricing, and patient access. The Japan allows open access to rare disease medications while covering 100% of patient costs. In contrast, India, China, Indonesia, Brazil, Pakistan, and Russia have very limited official government policy and coverage for orphan medication. There is also no guarantee the actual medication is marketed and available in those countries which further limit medication access. In the United States, rather than market access, patient access is limited by the high out of pocket costs due to limited government involvement on negotiating drug prices and state and variation in coverage that make the system difficult to navigate for both patients and providers.

**Limitations**

The countries included in this review are based on population size. Few countries had very limited data. Top 10 most populous countries were originally supposed to be included, however, Nigeria and Bangladesh were excluded due to lack of information available.

**Conclusions**

This global review identified the large variation and, at times, a lack of uniform regulations regarding orphan drugs and assistance programs for patients with rare diseases to access these products. With the increasing use and relatively high cost of orphan drugs, regulatory agencies should create clear pathways to streamline improved market access for these treatments.

**References**