

Letter from the Editors



Dear Readers, Authors, Reviewer, and Friends:

Last year we successfully introduced a new journal: The Journal of Rare Diseases and Orphan Drugs (JRDO) is a peer-reviewed open-access medical journal that publishes original research, reviews, case reports, and letters covering a broad field of its specialty. We intend to publish articles stimulating to read, educate, and inform readers with the most up-to-date research in genetics, rare diseases, and new orphan drug development in different stages of clinical trials. Journal topics are centered on patients living with undiagnosed rare diseases, the importance of a diagnosis, individual approaches to treatments. We hope that this journal will increase awareness of many difficult to diagnosed and treat medical conditions.

The mission of our journal is to facilitate the development of awareness about rare disease conditions among medical professionals; provide educational support for the medical community, patients, and families focused on rare disease conditions; support research focused on prevention and treatment for the benefit of patients with rare disorders; distribute transformative therapies using platform technology that can be deployed across multiple rare diseases.

The journal is focused on disorders with substantial unmet needs independent of their incidence.

After the 1983 Orphan Drug Act in the United States, more than 420 orphan drugs and biologic products for rare diseases have been approved by the Food and Drug Administration (FDA), compared with fewer than ten such products in the decade before the Act [1,2]. Increased attention on rare diseases has also resulted from an improved genetic, molecular, and biochemical understanding resulting from recent scientific and technological advances [3].

In the United States, the Rare Diseases Act of 2002 defines rare disease strictly according to prevalence, specifically "any disease or condition that affects fewer than 200,000 people in the United States", or about 1 in 1,500 people. This definition is essentially the same as that of the Orphan Drug Act of 1983, a federal law written to encourage research into rare diseases and possible cures [4]. The United States' Orphan Drug Act includes both rare diseases and any non-rare diseases "for which there is no reasonable expectation that the cost of developing and making available in the United States a drug for such disease or condition will [be] recovered from sales in the United States of such drug" as orphan diseases. The **Global Genes Project** estimates some 300 million people worldwide are affected by a rare disease.

Chronic genetic diseases are commonly classified as rare. Rare diseases may be chronic or incurable, although many short-term medical conditions are rare diseases [5]. The NIH's Office of Rare Diseases Research (ORDR) was established by H.R. 4013/Public Law 107-280 in 2002. The ORDR also runs

the Rare Diseases Clinical Research Network (RDCRN). The RDCRN provides support for clinical studies and facilitating collaboration, study enrollment, and data sharing.

We hope that a new 2021 year will be very fruitful for our goal of knowledge and discovery distribution. We are looking forward to reading, reviewing, and publishing your work. We also are going to preserve it and give DOI links and advertised it widely. We hope you will enjoy working with our editorial team. Manuscripts funded by NIH or their partners will be indexed as PubMed selected citations.

If you are reading this letter, you may well already be on our web. If you are interested in working with us, please, register as an author on the web: <https://spphllc.com/jrdod/user/register>. We will put our talents into work to make your paper well-edited and visible.

Editorial Team

References:

1. Haffner M.E. Adopting orphan drugs--two dozen years of treating rare diseases. *N Engl J Med.* 2006; 354: 445-447
2. Orphan Drug Act. 2049 – 2066. Available from: <http://www.gpo.gov/fdsys/granule/STATUTE-96/STATUTE-96-Pg2049/content-detail.htm>. [Accessed October 20, 2020].
3. Rare diseases and orphan products: accelerating research and development. Available from: http://www.nap.edu/catalog.php?record_id=12953. [Accessed October 20, 2020].
4. <https://www.govinfo.gov/content/pkg/PLAW-107publ280/html/PLAW-107publ280.htm>. [Accessed October 20, 2020].
- 5."Genetic and Rare Diseases Information Center". National Center for Advancing Translational Sciences, US National Institutes of Health. 2019. Retrieved 12 October 2019.
<https://rarediseases.info.nih.gov/guides/pages/120/support-for-patients-and-families>