



CELEBRATING THE ORPHAN DRUG ACT

By Michael Eging, Executive Director, RAAP

On January 4, 1983, the Orphan Drug Act (ODA), which created incentives for the development of rare disease medicines, was signed into law by President Ronald Reagan. This achievement came after a focused advocacy effort that was led by Abbie Miers and the newly formed patient advocacy organization the National Organization for Rare Disorders (NORD). Bipartisan leadership in the House, championed by Henry Waxman (D-CA), and in the Senate by Orrin Hatch (R-UT) changed the trajectory of rare disease research and development. An orphan disease, under the legislation, was defined as affecting under 200,000 Americans. Currently there are approximately between 7,000 and 8,000 identified rare diseases affecting as many as 25 million Americans.¹

In 1986, my father was diagnosed with a rare cancer. Yet, three years after the passage of the ODA, there were very few treatment options, and my father's care was mainly palliative. Five months after his diagnosis, he had passed away. In 1991, I began work for Senator Hatch and soon found out that I and my family were not alone in both our loss and our search for treatments. The ODA now provides a window of hope to families like mine and with over the 40 years of being in place has delivered treatments that can change the course of disease, and in some instances, provide a cure.

Prior to 1983, only 38 drugs were approved in the USA specifically to treat orphan diseases. Between 1983 and 2019, a total of 5099 drugs and biologics received orphan drug designation. Designations more than doubled between the 1980s and 1990s, almost doubled between the 1990s and 2000s, and almost tripled in number between the 2000s and 2010s. The top three therapeutic areas represented in the orphan drug designations were: oncology (1910, 37%), neurology (674, 13%), and infectious diseases (436, 9%). The broad disease categorization found that the proportion of designations for pediatric-onset diseases has increased in the most recent decade to 27%.²

An orphan drug designation does not automatically translate into approval. Since the inception of the program, approximately 16% of therapies that received the designation later gained FDA approval.³ Overall, the Orphan Drug Act of 1983 has resulted in the approval of over 650 orphan

¹ Roberts AD, Wadhwa R. Orphan Drug Approval Laws. [Updated 2022 Jun 12]. In: StatPearls [Internet]. Treasure Island (FL): StatPearls Publishing; 2022 Jan-. Available from: <https://www.ncbi.nlm.nih.gov/books/NBK572052/>

² Miller, K.L., Fermaglich, L.J. & Maynard, J. Using four decades of FDA orphan drug designations to describe trends in rare disease drug development: substantial growth seen in development of drugs for rare oncologic, neurologic, and pediatric-onset diseases. *Orphanet J Rare Dis* **16**, 265 (2021). <https://doi.org/10.1186/s13023-021-01901-6>

³ Maragkou, Irina. Rare Disease Spotlight – Tracing the rise of orphan drug designations over almost 40 years. Pharmaceutical Technology, June 29, 2022. <https://www.pharmaceutical-technology.com/features/rare-disease-spotlight-tracing-the-rise-of-orphan-drug-designations-over-almost-40-years/>

drugs, demonstrating its effectiveness in increasing drug development for rare conditions or those affecting fewer than 200,000 individuals.⁴ In 2021, over half of FDA's approvals were orphan drugs intended to treat rare diseases. By 2026, orphans will make up a fifth of all prescription drug sales, and almost a third of the global drug pipeline's value.⁵ However, we still have therapies for approximately 10 percent of the identified orphan diseases, and once approved by the FDA, those that are available face significant challenges.

Rare patient access to therapies has become an increasingly challenging part of the rare patient journey. For patients who have waited for these therapies to become available only to be told by their health plan or insurer that they remain out of reach due to coverage policies can be devastating. With the amount of investment and time needed to identify drug candidates, perform clinical trials, and seek approval, the United States and the biopharma community invests significantly in patient identification and drug candidate viability, only to have that therapy denied to the patient once it is approved.

At RAAP, we are launching a celebration of the 40th anniversary of the ODA, by looking back at the therapies and communities impacted by FDA approvals. In addition, we will be highlighting the hope of the near-term pipeline, made possible by the ODA. And we will be focusing in on access solutions for the next 40 years of the ODA that will improve patient access to therapies and care. Our **40 for 40** project will be highlighted throughout the year in our social media, our Spring workshop and annual meeting, as well as in our state and federal advocacy efforts.

We look forward to celebrating with the rare disease community and highlighting key solutions that can make a difference for the next generation of rare patients to access the therapies they need. As with experience of the ODA, we can make a difference in rare patient treatment; and ensure that we meet the promise of the ODA through ensuring patient access to rare therapies.

⁴ Miller KL, Lanthier M. Investigating the landscape of US orphan product approvals. *Orphanet J Rare Dis.* 2018 Oct 22;13(1):183. [[PMC free article](#)] [[PubMed](#)]

⁵ Miller KL, Lanthier M. Investigating the landscape of US orphan product approvals. *Orphanet J Rare Dis.* 2018 Oct 22;13(1):183. [[PMC free article](#)] [[PubMed](#)]